



House of Commons
Health Committee

National Institute for Health and Clinical Excellence

Eighth Report of Session 2012–13

Volume II

Additional written evidence

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The Health Committee

The Health Committee is appointed by the House of Commons to examine the expenditure, administration, and policy of the Department of Health and its associated bodies.

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Additional written evidence may be published on the internet only.

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¹ Mr Stephen Dorrell was elected as the Chair of the Committee on 9 June 2010, in accordance with Standing Order No. 122B (see House of Commons Votes and Proceedings, 10 June 2010).

List of additional written evidence

| | <i>Page</i> |
|--|-------------|
| 1 Weight Watchers UK Ltd | Ev w1 |
| 2 Mrs Ingrid Hardacre | Ev w2 |
| 3 British Association of Dermatologists | Ev w5 |
| 4 Specialised Healthcare Alliance | Ev w6 |
| 5 The Work Foundation | Ev w7 |
| 6 Baxter Healthcare | Ev w8 |
| 7 Beating Bowel Cancer | Ev w10 |
| 8 The Royal College of Midwives | Ev w11 |
| 9 British Thoracic Society | Ev w12 |
| 10 HEART UK | Ev w13 |
| 11 British Society of Gastroenterology | Ev w15 |
| 12 Alzheimer's Society | Ev w15 |
| 13 Faculty of Pharmaceutical Medicine | Ev w17 |
| 14 Merck Serono Ltd | Ev w18 |
| 15 British Society for Rheumatology | Ev w19 |
| 16 Rare Disease UK | Ev w20 |
| 17 National Heart Forum | Ev w22 |
| 18 Genzyme Therapeutics | Ev w22 |
| 19 Johnson & Johnson | Ev w23 |
| 20 Ethical Medicines Industry Group | Ev w26 |
| 21 UK Council for Psychotherapy | Ev w30 |
| 22 Deltex Medical | Ev w33 |
| 23 Association for Family Therapy and Systemic Practice | Ev w37 |
| 24 British Heart Foundation and the British Cardiovascular Society | Ev w40 |
| 25 Bayer HealthCare | Ev w41 |
| 26 The Hepatitis C Trust | Ev w44 |
| 27 European Medicines Group | Ev w45 |
| 28 The Karen Clifford Skin Cancer Charity | Ev w48 |
| 29 AstraZeneca | Ev w49 |
| 30 BioIndustry Association | Ev w50 |
| 31 MS Society | Ev w51 |
| 32 Bristol-Myers Squibb Pharmaceuticals Limited | Ev w55 |
| 33 British Association for Counselling and Psychotherapy | Ev w58 |
| 34 British Association for Behavioural & Cognitive Psychotherapies | Ev w60 |
| 35 Novo Nordisk | Ev w61 |
| 36 Roche Diagnostics Limited | Ev w63 |
| 37 Association of British Healthcare Industries | Ev w66 |
| 38 Royal National Institute of Blind People | Ev w68 |
| 39 Cystic Fibrosis Trust | Ev w70 |
| 40 National Infertility Awareness Campaign | Ev w72 |
| 41 Arthritis and Musculoskeletal Alliance | Ev w73 |

| | | |
|----|---|---------|
| 42 | Medtronic Ltd | Ev w74 |
| 43 | Rarer Cancers Foundation | Ev w76 |
| 44 | Prostate Cancer UK | Ev w80 |
| 45 | Astellas Pharma Ltd | Ev w81 |
| 46 | Shire plc | Ev w84 |
| 47 | British Intra Diagnostics Association | Ev w88 |
| 48 | GlaxoSmithKline | Ev w89 |
| 49 | Neurological Alliance | Ev w92 |
| 50 | Novartis Pharmaceuticals UK Limited | Ev w96 |
| 51 | Myeloma UK | Ev w97 |
| 52 | Medical Marketing Consultants | Ev w100 |
| 53 | British Medical Association | Ev w103 |
| 54 | Karl Claxton, Steve Martin, Marta Soares, Nigel Rice, Eldon Spackman, Sebastian Hinde, Peter C Smith and Mark Sculpher | Ev w107 |
| 55 | Pfizer Ltd | Ev w110 |
| 56 | The British Psychological Society | Ev w114 |
| 57 | The British Cardiac Patients Association | Ev w115 |
| 58 | Roche | Ev w117 |
| 59 | Ovarian Cancer Action | Ev w120 |
| 60 | NHS Confederation | Ev w122 |
| 61 | UK Faculty of Public Health | Ev w123 |
| 62 | The Royal College of Radiologists | Ev w125 |
| 63 | Cambridge Weight Plan | Ev w126 |
| 64 | LighterLife | Ev w127 |
| 65 | National Obesity Forum | Ev w129 |

Written evidence

Written evidence from Weight Watchers UK Ltd (NICE 03)

Weight Watchers welcomes the opportunity to provide feedback on the valuable work of the National Institute for Health and Clinical Excellence (NICE). Firstly, Weight Watchers firmly believes that the work of NICE is world renowned, highly respected and a vital part of our national networks of clinical governance and healthcare improvement. Some potential improvements are summarised below, to develop and leverage this strong position:

1. Tackling obesity is the number one public health priority—yet NICE clinical guideline is six years out of date.
2. Dissemination and implementation of NICE’s clinical guidelines on obesity is weak.
3. There is no formal national monitoring or regulation of implementation of NICE guidance.
4. Cost effectiveness appraisals often useless to decision makers.
5. Patient views and preferences for service options not taken into account by NICE.

1. TACKLING OBESITY IS THE NUMBER ONE PUBLIC HEALTH PRIORITY—YET NICE CLINICAL GUIDELINE IS SIX YEARS OUT OF DATE

Being a healthy weight is central to good health. Being overweight or obese has a severe impact on a person’s health—both associated with an increasing risk of diabetes, cancer and heart and liver disease amongst others, and the risks get worse the more overweight people become. These illnesses put pressures on families, the NHS and society more broadly, with overall cost to society forecast to reach £50 billion per year by 2050 (Foresight, 2007). The Foresight team described obesity as the “climate change of public health”. For all these reasons, helping the 26 million people who are currently overweight or obese to achieve and sustain a healthy BMI should be core to NICE’s work programme and Weight Watchers is concerned that NICE’s most relevant clinical guidance on obesity management (NICE, 2006) is outdated. The Health Technology Assessment systematic review, on which much of NICE guidance is based (Avenell *et al*, 2004) was conducted almost 10 years ago. Over the last decade there has been significant expansion in the evidence base underpinning obesity management through NHS interventions, yet recent consultation from NICE was positioned as “no need to update current obesity management guidance”.

2. DISSEMINATION AND IMPLEMENTATION OF NICE’S CLINICAL GUIDELINES IS WEAK

NICE employs different channels to disseminate its guidance from publications on its website to distribution to local decision makers. Adequate and timely communication and implementation is crucial, as guidance is only effective if it is indeed used by decision makers to make health policy and for provision decisions. However, existing evidence suggests that the uptake of NICE guidance on obesity is slow, patchy and without adequate incentives for implementation.

For example:

- The Office of Health Economics survey of Primary Care Trusts (PCTs) in 2010 revealed that 10% of PCTs, responding to the questionnaire, said that they did not follow the NICE guideline on obesity (CG43—NICE, 2006). Only 40% interpreted this guidance stringently. Weight Watchers knows that there is huge regional variation in implementation resulting in a “postcode lottery” of obesity services to patients (OHE, 2010).
- In Kingston a review of maternal weight management care was carried out in 2010 (http://www.kingston.gov.uk/obesity_in_pregnancy_needs_assessment.paf). This survey revealed that GPs and pharmacists were not aware of NICE’s (2010) guidance on weight management in pregnancy. The Head of Midwifery said that referral of obese women was well implemented, although advice for obese women about the risks their weight posed to their pregnancy and weight management discussion and support within the postnatal period was less well enforced. Health Visitors in Kingston did not understand the evidence base around maternal obesity or how to raise the sensitive issue of weight. Furthermore, in November 2010 the Royal College of Midwives (RCM) together with Netmums released the results of a survey of 6,226 mother’s experiences of weight management and obesity issues. It revealed huge gaps in relevant postnatal care from midwives (RCM, 2010). Key findings included:
 - 84% rated the overall care that they received from midwives regarding healthy eating and weight management as “Neutral”, “Poor” or “Very Poor”; and
 - 89% of women said that after giving birth they did not have an opportunity to discuss healthy eating and weight management issues with their midwife, despite this being an explicit recommendation in NICE’s (2010) guidance on weight management before, during and after pregnancy.

3. THERE IS NO FORMAL NATIONAL MONITORING OR REGULATION OF IMPLEMENTATION OF NICE GUIDANCE

Processes for ensuring guidance from NICE is effectively implemented appears to be a local responsibility. There seems to be a wide variation in audit/monitoring processes across health service localities, but methodologies often rely on self reported practice which is subjective and tends to lack sensitivity and validity. It seems to be a huge waste of effort to invest significant resource into generating high quality theoretical guidance without formal national evaluation and reporting of its local implementation.

4. COST EFFECTIVENESS APPRAISALS OFTEN USELESS TO DECISION MAKERS

NICE guidance is often based on assessments of clinical effectiveness and cost effectiveness of different NHS interventions. However, reports on economic analyses are often complicated and very technical, making their application to commissioning decisions problematic. NICE could do much more to bridge the gap between cost effective evidence and policy making, plus service provision decisions at local level. For example NICE could:

- Summarise cost effective and economic analyses in plain English which is understandable to local health service commissioners.
- Separate out cost effective analyses from appraisals of clinical effectiveness to make the information more usable
- Include simple recommendations that commissioners compare the costs of different treatments/interventions. In terms of weight management services for adults in primary care, it is surprising that commissioners often omit to make basic service cost comparisons when evaluating service provision.

5. PATIENT VIEWS AND PREFERENCES FOR SERVICE OPTIONS ARE NOT TAKEN INTO ACCOUNT BY NICE

NICE generates guidance from systematic reviews and cost effective analyses. Patient's views seem rarely to be represented or referenced within NICE's guidance documents. However, improving and promoting patient choice of services and treatments provided by the NHS is the cornerstone of the NHS reforms which are currently being put in place. Weight Watchers would like to see surveys on patient satisfaction and preferences or qualitative data on patient views included as a much stronger strand within NICE's appraisal process, not just patient representatives on high level panels.

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National Institute of Health and Clinical Excellence (2010). Public Health Guidance 27, Weight management before, during and after pregnancy, NICE: London.

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October 2012

Written evidence from Mrs Ingrid Hardacre (NICE 04)

I refer to some of your bullet points in your Select Committee Announcement.

- The role of NICE Quality Standards.
- The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome.

Ref. NICE guidelines G 40 for the treatment of urinary incontinence.

I am writing as an individual on behalf of all surgical mesh implant sufferers.

I would like to draw your attention to the plight of surgical mesh implant sufferers who have been and are still being harmed by implant devices:

- TVT/TOT = transvaginal tape or tension free vaginal tape and other mesh implant products used for POP (Pelvic Organ Prolapse) conditions.
- These mesh products are not fit for purpose!

- According to NICE guidelines issued 2006 (these are out of date since they should be updated every three years!) these mesh products are being recommended as the best treatment for urinary incontinence despite the, now known, statistics on unsatisfactory outcomes and complications that arise from these operations.

These complications are recorded and researched all over the world and they include:

- Death, erosion, pain, urge incontinence, haematomas, nerve damage, inability to have sexual intercourse, auto immune reactions and life long disabilities.

Surgeons and consultant have devised these NICE guidelines G40 as a panel (my consultant was part of one of the early panel) in conjunction with the British Society of Urogyneacology with one aim only in mind to promote this type of implant operation, simply because it was deemed the fastest operation time and therefore cheaper compared to more traditional surgery.

These guidelines echo all the leaflets that are available all over the British Isles for this type of surgery for patients seeking help. What these guidelines omit are the complications that can arise. Consequently the patient is not fully informed as to what can happen with this operation and they remain ignorant to all the adverse facts, even as they sign their consent forms!

Complications arise because:

1. Operation is not performed properly (Technique/badly trained or badly executed).
2. From products that are not fit for purpose.
3. No robust clinical trials were conducted to support the theory that this operation delivers what it says on guidelines issued and being passed on to the MHRA agency who regulate and approve the use of devices.

My letter—and other sufferers' letters—to the MHRA showed a complete lack of interest in the problems relating to the TVT device. Yet one would expect such an organisation to be pro-active in its activities to monitor the safety of devices and protect the public from harm.

The MHRA recently stated on their website:

- In light of an increasing number of adverse events and patient concerns being reported, the MHRA has launched an investigation to better understand the use of these devices and the complications associated with their use.
- The “NHS Information Centre for Health and Social Care” recently provided Hospital Episode Statistics data indicating there are about 13,000 operations a year implanting tapes for stress urinary incontinence and about 600 ie 4.6% partial or total removals of these SUI tapes. The MHRA is currently working closely with the NHS Information Centre on further statistics related to vaginal mesh and tapes and interpretation of their data.

Since most NHS hospitals regard this operation as:

- Routine “minimally invasive day surgery” in such a cavalier fashion, that registrars are:
 - Using under-informed patients as guinea pigs and performing these operations under the umbrella-ship of the clinical lead.
 - Are inserted by many consultants, but removed by few because they cannot deal safely with the mesh mess that women are left with.
 - When things go wrong, patients see the consultants who are not interested.
 - Many medics tell sufferers that the problem is in their head! Women are regularly counselled and supported by self help volunteers. These are women whose life has been devastated by mesh complications, and it is clear that their consultant is totally unaware of—or unwilling to see—the problems that mesh can cause.
 - When mesh surgery goes wrong, there are multiple trips to the GP, hospital clinic, costs of tests including ultrasound scans, urodynamics, MRI scans, and cystoscopy, and then eventually repeat surgery (and sometimes multiple surgeries) and hospital inpatient treatment to remove part or all of the tape which is eroding or causing obstruction or pain.
 - Add the cost of medication for pain, antibiotic treatment, trips to the pain clinic for treatment or injections. Throw in the cost of incontinence products, such as catheters, and you might have a better idea of the real cost to the NHS of failed TVT surgery.
 - Some sufferers find it hard to recover from the stress of actually getting their mesh problem recognised and acknowledged.

The NHS must also bear the cost of remedial surgery when there are problems with TVT or other synthetic meshes as they have to with failed PIP implants. This subject has received more publicity, but is, in fact, exactly the same problem-failed implant surgery!

I therefore feel the following points should be included in any new leaflets:

1. The patient should only be put forward if all else has failed; and

2. Only used when women are given full details of the potentially devastating complications in frank and open disclosure:
3. Including death, erosion, extreme pain, urge incontinence, haematomas, nerve damage, inability to have sexual intercourse, auto immune reactions such as responses to mesh eg fibromyalgia, lupus, sensitivity to strong smells, skin reactions, poor healing.

The risks stated in most hospital leaflets that have been copied from RCOG directions leaflet are fundamentally dishonest, inaccurate, and underplayed particularly for TVTs/TOT. Like many sufferers, I, would have declined this operation had I known more about the risks.

- This opinion is brought forward by every single mesh sufferer and they feel they have been misled, as they really trusted their consultants.
- These leaflets are still being used in hospital around the country, reassuring women that the risks associated with mesh surgery is minimal, which is untrue because according to some studies from BMJ data:

“Observational reports found that complications associated with TVT include death (10 deaths associated with TVT; [54] from unrecognised bowel perforation in 8/10 [80%] cases and haemorrhagic complications in 2/10 [20%] cases). Case reports of serious complications included bowel injuries, [55] [56] [57] [58] [59] [60] [61] [62] necrotising fasciitis, [63] [64] Fournier’s gangrene, [65] urethrovaginal fistula, [66] and nerve injuries, [55] [63] which might cause problems even after removal of the TVT. The most common complications were urethral injuries during surgery, and urethral erosion up to 5 years later. [55] TVT might eventually erode into the bladder, [55] [67] [68] which might require surgical opening of the bladder to rectify. Other surgical complications reported in prospective and retrospective cohort studies include bladder perforation, injury to iliac vessels, bleeding, urinary tract infection, retropubic haematoma, and vaginal tape erosion [69] [70] [71] [72] [73] [74] [75] incisional hernia of the inguinal canal [76] and urethral diverticulum. [77]”.

BMJ clinical evidence on stress incontinence 2008:
<http://clinicalevidence.bmj.com/ceweb/conditions/woh/0808/0808-get.pdf>

Read:

The “promotional pitch” by leading manufacturer of the UK’s “gold standard” in SUI mesh.

Ethicon’s often-quoted statistics of 11.5 years of success is based on a long term study (Eleven years prospective follow-up of the tension-free vaginal tape procedure for treatment of stress urinary incontinence. Nilsson CG, Palva K, Rezapour M, Falconer C) where 77% of the initial cohort of 90 women and 89% of those alive and capable of cooperating were assessed 11.5 years after the TVT operation. This means that just 69 women were used as a statistically significant sample to sell this product as a long term success story. The subjective findings showed 77% success rate. Thus nearly a quarter of the TVTs were unsuccessful in a small sample. Is that a highly significant success story?

<http://www.ethicon360.com/products/gynecare-tvt-retropubic-system-tension-sup-incont>

- These products are made of petroleum polypropylene woven mesh. Whatever brand or size, whether for SUI or prolapse repair, this mesh is proven to shrink, twist, leach and degrade in the body—mine felt inside like sitting on razor wire.
- I could not walk, sit, stand or travel in a car and was in constant pain and my life has never been the same since.
- Donald Ostergard has shown in several studies how mesh behaves in the body:
<http://www.ncbi.nlm.nih.gov/pubmed?term=Ostergard%20D%2C%20Prolene>

I quote here information which came from one tireless campaigner, who, along other self help websites has invested a great deal of her time to collate an enormous amount of information to lend support and hope to fellow sufferers in this country.

This information below is from the US and it states:

- July 2012 a judgement against Bard, manufacturers of Avaulta and other pelvic mesh products, led to the plaintiff being awarded \$5.5 million following transvaginal mesh complications. Details should be read in full: <http://www.wvsc.uscourts.gov/mdl/2187/pdfs/FinalMasterComplaint.pdf>
- Johnson and Johnson, of which Ethicon and Gynecare/TVT is a subsidiary company and resulting product, have plaintiff claims made against them that summarise very neatly the research that has been collected over the last 18 months and stories heard from women affected by mesh.

Despite these strong and successful actions in the US nothing is done in the UK to take notice about this serious lack of interest in the parts of the NHS, Government and other health organisations.

Will things change with NICE guidelines and those that are passed down the line by the RCOG?

While the RCOG and NICE continue to minimise the risks of mesh as a pelvic/SUI repair solution, the MHRA avoid a compulsory register and while almost all gynaecologists in the UK continue to use such mesh

without giving any hint of the real risks or devastating complications, women will continue to be harmed. This must stop. You have the power to affect this.

Although you are not able to comment on individual cases and I have kept my personal comments to a minimum, because I believe in getting better information out there, so women have the opportunity to make an informed choice. We are aware that all operations carry risks. But this operation should have a Health Warning on the leaflets that are given to patients.

In my case, all I was handed, was a leaflet. The consultant saying: “this explains everything and under NHS guidelines you will hear from us within 16 weeks”. He still does now!

Please also look into the NHS Trusts practises on how consultants spend their time (or not, as in my case) with patients under their care, when patients come back to the wards.

In my case I never saw my consultant after my operation. There was never a ward round with him attending. I was one of those complications, with very heavy haematoma (I took pictures!) Statistically this happens four in 100 in this operation. I had also all the other related complications as described. My day case surgery turned into a four-day stay in Hospital. The NHS Trust denies any problems or complications. I have raised my concerns and followed all the right steps according to patient’s rights reporting their experience and the disappointing outcome.

In contrast, the centre that removed my tape had consultant, registrars, junior doctors and nursing staff on their ward rounds, everyday during my four-day stay.

BBC News, this week, broadcast that the Government will look into the way some hospitals work and that a daily ward round should include consultant, surgeons, all training staff including a nurse at the bedside of each visited patient

I am grateful that I can contribute to this enquiry as an individual but on behalf of thousand of sufferers in the UK.

I would like to draw attention to the various website links—listed below—to give you insight of the problems faced by fellow surgical mesh implant sufferers

Please start by listening to the very brief podcast on Woman’s hour, where Jenny Murray is interviewing a fellow sufferer. This interview was also attended by Dr Mark Slack, head of Gynaecology at Addenbrooks Hospital, who was at one time part of the NICE panel and co- author of the RCOG guidance on the use of mesh. He was also a “travelling doctor” promoting mesh and paid by mesh manufacturers.

<http://www.bbc.co.uk/iplayer/console/b019gy9r>
<http://www.tvt-messed-up-mesh.org.uk/petitions.html>
www.tvtinfo.wordpress.com

Unheard voices are actively creating awareness by the following actions:

- Petitions, one having been taken to the House of Commons.
- Developing a detailed blog of research and information eg tvtinfo.wordpress.com
- Demonstrating outside the 2012 conference of the UK Continence Society in Liverpool.
- Meeting with the MHRA and contacting NICE.
- Writing to MPs.
- Appearing in local and national media to raise awareness.
- Liasing with support groups around the world.

I may be one of many unheard voices, but I urge you to bring about change so there will be:

- Regulation on complaint registration.
- A national register of adverse mesh implant incidents.
- This information must be accessible to any patient seeking help.

October 2012

Written evidence from the British Association of Dermatologists (NICE 05)

1. NICE’s role as a national body has been to reduce postcode lottery in the provision of services by establishing clear guidelines about what should and should not be available as an NHS treatment. It has clearly defined and transparent recommendations based on Quality of Life Years to decide how to spend a finite resource in the NHS and to counter “shroud waving”. This role is envied by many health services overseas who also find it difficult to make these decisions.

2. The committees are respected and the format fairly rigid in producing recommendations. One concern is that, particularly with novel drugs, the experts are those who have been involved in the trials and may have consultancies or financial incentives which, even though declared as potential conflicts of interest does not

make them appear to be completely unbiased. Similarly those who know most about a novel treatment are often advocates for its use and their view may seem not entirely balanced.

3. NICE Quality Standards are welcomed in the new commissioning environment as a means of benchmarking existing providers and assessing new ones but the predicted time course is long and therefore many commissioning decisions may be made in advance of the Standards becoming available, but we think that they will still be of value when they are produced.

4. NICE clinical guidelines on treatment of specific conditions produced a valuable review of existing evidence to support different treatments. Sometimes however the evidence in reliable controlled trials is lacking, basically because the studies have not been done or they are not done in a scientifically robust way, so the evidence to support a treatment, or say that it is not of value, just does not exist. This has resulted in some guidelines producing conclusions that would not always have been the instinctive ones of clinicians working in the field. This is perhaps inevitable.

5. We think that NICE is a very valuable body and the fact that NICE approved drugs have to be funded, and IOGs implemented, makes it easy as clinicians to ensure that these drugs are available equitably and resource to comply with IOGs available. However the reliance of the system on NICE recommendations does create a group of “second-class” diseases and treatments which have not and, because of their relative rarity are unlikely to be, a subject for a NICE assessment. Specialists dealing in these areas consequently spend huge amounts of time—and thus resource—battling the PCTs to justify such treatments through the IFR system. This is to the detriment of patients as their treatment is delayed and, in some cases, refused by PCT’s. Thus NICE has been very helpful for the NHS in regulating spend on expensive drugs for common diseases but an unintended consequence is that it has resulted in a bureaucratic and inflexible process for approval of drugs that are not in its purview. Also, PCTs under financial pressure funding NICE approved drugs increasingly look at the non-NICE approved treatments as places where they can save money and increasingly turn down IFRs or make the process exceedingly difficult.

6. NICE’s new responsibilities in relation to evaluating social care interventions will hugely increase its work overall. We think that this will be valuable in integrating health and social care services which is a major feature of the recent Health and Social Care Act. It is important however that NICE is given sufficient resource that existing initiatives are not lost.

October 2012

Written evidence from the Specialised Healthcare Alliance (NICE 06)

The Specialised Healthcare Alliance is a coalition of 81 patient-related organisations, supported by 12 corporate members, which campaigns on behalf of people with rare and complex conditions. This response focuses on NICE’s future role in assessing high-cost, low-volume “ultra-orphan” drugs for very small patient populations from April 2013. The Alliance was instrumental in drawing up a framework of ethical considerations for funding decisions around these products in 2009, which helped to inform the development of a decision-making framework by the Advisory Group on National Specialised Services (AGNSS) the following year.

On 18 July 2012, Earl Howe announced that NICE would take on the assessment of very high-cost, low-volume drugs from April 2013 in conjunction with the disbandment of AGNSS as a result of the health reforms. In making this announcement, the Minister noted potential concerns about NICE’s ability to assimilate the unique considerations around drugs for highly specialised services into its appraisals.¹ Indeed, in 2006, NICE concluded that its existing assessment methodology would be inappropriate for consideration of ultra-orphan drugs.²

To address such concerns, Earl Howe stated that NICE’s assessment of drugs for highly specialised services would “build on the decision-making framework that AGNSS uses at the moment.”³ The framework, he explained, “balances health gain, best clinical practice, societal value and reasonable cost. In addition, recommendations from NICE will not be based solely on a cost per QALY figure.”⁴

The Alliance strongly welcomes this commitment from the Minister. AGNSS’s remit has applied to products for patient populations in England usually fewer than 500 per year, where development costs tend to be large in proportion to market size. The Alliance would urge the retention of health gain, best practice and societal gain in any decision-making framework developed by NICE for its assessment of ultra-orphan products. This would build on the assurances from the Minister as quoted above, and would give a clear indication that NICE does not intend merely to adjust QALY thresholds within its existing appraisal processes when considering these products.

¹ 1 *Hansard* HL Deb 18 July 2012, vol 739, col 307.

² 2 NICE, “Appraising Orphan Drugs”, (2006), http://www.nice.org.uk/aboutnice/whoweare/seniormanagementteam/seniormanagementteammeetings/2005/12july2005/appraising_orphan_drugs.jsp

³ 3 *Hansard* HL Deb 18 July 2012, vol 739, col 307.

⁴ 4 *Hansard* HL Deb 18 July 2012, vol 739, col 307.

The Alliance's members are anxious that the advances achieved through the development of the AGNSS framework should be secured with NICE. The Health Select Committee may wish to explore:

- NICE's proposals to engage with stakeholders in the development of its interim assessment framework for the appraisal of ultra-orphan products before it begins its full consultation on methods in 2013–14.
- The role, if any, that Incremental Cost Effectiveness Ratios (ICERs) will play alongside the considerations encompassed by the AGNSS framework.
- The application of the new arrangements to patient populations of 500, reflecting the AGNSS arrangements, or 1000, reflecting NICE's definition of ultra-orphan products as having a prevalence of less than 1 in 50,000.
- The expertise required to assess ultra-orphan products, as distinct from the expertise required to appraise drugs for more common conditions.
- The anticipated relationship between two distinct methodologies within a single organisation predicated on patient population, especially at the boundary between the two.

October 2012

Written evidence from The Work Foundation (NICE 07)

- Changes in UK demographics mean that coordinated healthcare, welfare and labour market interventions to maximise labour market participation should have higher priority.
- NICE Quality Standards have the potential to help ensure patients receive appropriate support to help them remain in, or return to work, and the National Quality Board should ensure Quality Standards are prioritised for long-term conditions that have an impact on employment.
- The Work Foundation is concerned that employment of people with long-term conditions no longer features in the latest recommendations to Commissioning Outcomes Framework. To exclude employment for long-term conditions is to neglect a major avenue both for speeding up patient recovery and reducing costs to the UK's healthcare and welfare systems.
- NICE Quality Standards should help encourage a more joined-up approach, but this also requires a broader Government view of smarter public spending.
- The Work Foundation supports the Health Select Committee's earlier call for NICE to be able to comment on wider societal benefits as part its technology appraisal process.
- Value-Based Pricing has the potential to recognise wider societal benefits of treatments, but it would appear there is some way to go before a model can be agreed.

1. The Work Foundation has, for several years, been conducting research on the employment experiences of people with long-term conditions including back pain, rheumatoid arthritis (RA), multiple sclerosis, mental illness and inflammatory bowel disease. We have consistently found that enabling those with such conditions to remain in or return to work as soon as they are ready significantly improves their quality of life. It provides an income, a sense of purpose, dignity and social connectivity. With an ageing workforce, later retirement and a growing proportion of the UK workforce (up to 50% by 2030) likely to have at least one long-term condition, coordinated healthcare, welfare and labour market interventions to maximise labour market participation should have higher priority.

2. NICE Quality Standards have the potential to help ensure patients receive appropriate support to help them remain in, or return to work, and the National Quality Board should ensure Quality Standards are prioritised for long-term conditions that have an impact on employment. Organisations such the British Society of Rheumatology, for example, are already calling for Quality Standards to recommend that people with RA be asked about the impact the disease is having on their ability to work.⁵

3. At the same time, the Work Foundation has previously expressed concern that employment of people with long-term conditions no longer features in the latest Commissioning Outcomes Framework (COF), which is key to incentivising Clinical Commissioning Group (CCG) behaviour under the new NHS structures.

4. To exclude employment for long-term conditions is to neglect a major avenue both for speeding up patient recovery and reducing costs to the UK's healthcare and welfare systems. By focussing on short-term indicators to save money, this type of planning would create a false economy by shifting the burden onto the DWP in the long term.

5. With the DWP prioritising return to work for people with chronic conditions via the Work Capability Assessments, this would represent a disconnect in strategic planning between Government departments.

6. NICE Quality Standards should help encourage a more joined-up approach, but this also requires a broader Government view of smarter public spending. The economic modelling for the 2009 National Audit Office report into *Services for People with RA* found that productivity gains could be achieved and patient quality

⁵ British Society of Rheumatology, *Top Ten Quality Standards for RA* (2012) www.rheumatology.org.uk/includes/documents/cm_docs/2012/t/top_10_quality_standards_for_ra.pdf

of life improved through better integration and coordination of services, leading to quicker diagnosis and earlier treatment.⁶

7. The NAO reported that only 10% of RA patients were seen within three months. While increasing this number to 20% could initially increase costs to the NHS by £11 million over five years, due to higher expenditure on drugs and the associated costs of monitoring people with the disease, this increase in earlier treatment could, however, result in productivity gains of £31 million for the economy due to reduced sick leave and lost employment. On average, this could also increase quality of life by 4% over the first five years, as measured by quality adjusted life years (QALY) gained. After around nine years, earlier treatment could also become cost neutral to the NHS.

8. The NAO showed that rheumatoid arthritis costs the NHS around £560 million a year in healthcare costs and that the additional cost to the economy of sick leave and work-related disability is £1.8 billion a year. The Work Foundation's *Fit for Work* report in 2007 put the cost of lost working time due to MSDs at £7 billion.⁷

9. In October 2012 I chaired the Fourth Annual "Fit for Work Summit" in Brussels which brought together over 200 delegates from across the world and saw eminent clinicians, patients, policy-makers and health economists discuss the need for national and regional plans to provide a logic, standards, pathways and outcome measures for health, welfare & employment support for people with MSD. The issue of siloed budgets was a recurring theme and the UK is no exception. There was consensus that a cross-Government approach is needed so that savings such as through reduced welfare benefits and increased productivity can offset spend by other departments such as health.

10. The Health Select Committee itself has called for wider societal benefits, such as increased productivity, to be considered by NICE when carrying out Health Technology Appraisals.⁸ The Work Foundation supports this call and NICE, where possible, should be free to comment on wider societal benefits as part its technology appraisal process.⁹

11. Value-Based Pricing (VBP) has the potential to recognise these wider societal benefits, but it would appear there is some way to go before a model of VBP can be agreed. Should there be a "cap" on the amount paid for a treatment, despite potentially significant benefits, or would this undermine a proposal for a "value-based" approach.

12. At the same time, there is ongoing discussion about persistent variations in the implementation of existing NICE guidance. Reports of some PCTs restricting treatments have since been met with criticism from senior clinicians as well as NICE Chairman Sir Michael Rawlins.¹⁰ Despite proven benefits of NICE-approved technologies, financial pressures at a time of NHS reform means there is a real risk that progress on reducing this variation of patient care will be slowed. We would look to the Department of Health, the NHS Commissioning Board and NICE to ensure the appropriate action is taken so that patients across the country have equity of access to appropriate treatment, and that appropriate clinical guidance enables people to remain in, or return to work, play an active role in supporting the UK economy, and improve health outcomes.

October 2012

Written evidence from Baxter Healthcare (NICE 08)

INTRODUCTION

As Sir Michael Rawlins prepares to step down from his long stewardship of NICE in March 2013, we welcome this opportunity to comment on the role of NICE at this important time in the history of the NHS. Since it was set up in 1999, NICE has achieved much and been key to evolving an ethos of evidence-based practice in the NHS.

As NICE moves into non-departmental public body (NDPB) status, it is important that the role of NICE is redefined and it is clear exactly what NICE is accountable for and how it should operate.

Baxter has and continues to be actively engaged as a stakeholder in the development of NICE Guidelines and Quality Standards. These include standards on Chronic Kidney Disease, Nutrition and IV Fluids. Baxter has been operating for over 50 years in the UK offering over 7,500 products and services to the NHS and patients. We are a major contributor to the UK life science and health care industry in the UK.

⁶ National Audit Office, *Services for People with Rheumatoid Arthritis* (2009) www.nao.org.uk/publications/0809/services_for_people_with_rheum.aspx

⁷ The Work Foundation, *Fit for work? Musculoskeletal Disorders and Labour Market Participation* (2007) www.theworkfoundation.com/DownloadPublication/Report/44_44_fit_for_work_small.pdf

⁸ Health Select Committee, *National Institute for Health and Clinical Excellence* (2008) www.publications.parliament.uk/pa/cm200708/cmselect/cmhealth/27/27.pdf

⁹ The Work Foundation, *Adding Value: The Economic and Societal Benefits of Medical Technology* (2011) [www.theworkfoundation.com/DownloadPublication/Report/296_Adding value—The Economic and Societal Benefits of Medical Technology.pdf](http://www.theworkfoundation.com/DownloadPublication/Report/296_Adding_value—The Economic and Societal Benefits of Medical Technology.pdf)

¹⁰ Sir Michael Rawlins, *Health Service Journal* (2012) www.hsj.co.uk/opinion/columnists/michael-rawlins-playing-fair-on-treatments/5047276.article

RECOMMENDATIONS

- NICE is an integral part of achieving the Government’s life sciences growth and NHS innovation agendas. It is therefore important that appropriate relationships established with UK health care companies are maintained and further developed in the future.
- Following the announcement that AGNSS¹¹ is to be disbanded, NICE needs to incorporate the learning from the AGNSS decision-making framework into its current way of working. Many years of work went into AGNSS and this important background should not be lost.
- The NHS needs to be more strongly incentivised to actively implement NICE guidance, quality standards and care pathways. The tariff and financial incentives should be adjusted to ensure trusts are appropriately reimbursed or incentivised to implement NICE guidance.

INNOVATION IN UK

The Prime Minister has committed the UK to becoming “the best place in the world to invest and innovate in life sciences”.¹² Innovation Health and Wealth committed the NHS to supporting adoption of innovation and a successful life sciences sector. The UK has long been a favoured place for investment by US companies because the Government understands and recognises the complexities of the pharmaceutical business. Indeed, the NHS has supported competitive commercial behaviour by industry, while ensuring reasonable prices for the NHS. It has met the needs of industry, the NHS and patients. However, the UK commercial environment is causing international concern. Low prices and low and slow patient access to innovative medicines and services can negatively impact the potential investment into healthcare companies in the UK.

The UK has one of the lowest prices for medical technologies in Europe. Further downward pressure on costs will damage international competitiveness. Therefore companies will review the launch of new products early in the UK if it has the effect of damaging global positions.

Groundbreaking innovation in health technologies comes very rarely. However many of the existing technologies being used by the NHS could be re-deployed in a more efficient and effective manner to improve patient outcomes and experience. For example dialysis treatment at home and innovative delivery systems that enable medication or therapies to be administered at home. These cost effective service redesign projects are often limited to small localities and thus their benefits are not disseminated widely. NICE should play a vital role in supporting uptake of such innovations through the development of support and guidance as well as the spread of good practice within the NHS.

ORPHAN MEDICINES

Baxter supported the work of the Advisory Group for National Specialised Services (AGNSS) for people with very rare conditions. However, it was announced in July 2012 that NICE will be taking forward this work and we hope and anticipate that the framework that has been set up by AGNSS will continue to be used by NICE.

AGNSS’s remit applied to products for patient populations in England usually fewer than 500 per year, where development costs tend to be large in proportion to market size. The inclusion of health gain, best practice and societal value in its ethical decision-making framework must be retained by NICE, resisting the temptation merely to adjust the QALY threshold for these products.

TARIFF VS GUIDANCE

Baxter supports the work that NICE has recently conducted developing national guidance and quality standards, particularly in relation to therapies for long term conditions and patient experience. Much of the guidance already published has been helpful in encouraging the delivery of care closer to or in the patient’s own home.

As a major provider of home based treatments, therapies and care, we often see that the implementation of NICE guidance is slow and variable within the NHS. The system needs to increase incentives for local NHS organisations to adopt it more swiftly to ensure that patient outcomes are improved. There is still a clear disconnect between the uptake of NICE guidance and quality standards and implementation at a local commissioner level (as highlighted by the low uptake of home dialysis).

To help implementation we believe that commissioning guidance should be published that follows the recommended NICE clinical guidance or Quality Standard. This needs to be supported by a national pricing structure that rewards or incentivises the implementation of NICE guidance and quality standards through a “result based” reimbursement method as opposed to the current system of activity based payments.

October 2012

¹¹ AGNSS decision-making framework. <http://www.specialisedservices.nhs.uk/info/agNSS>

¹² Prime Minister at the Global Health Summit in August 2012: <http://www.number10.gov.uk/news/global-health-policy/>

Written evidence from Beating Bowel Cancer (NICE 09)

Beating Bowel Cancer is dedicated to saving lives by working in partnership with individuals, local communities, clinical communities and Government to improve public awareness of bowel cancer and to increase the rate of early diagnosis. We help patients access the treatment they need and provide emotional and practical support to improve the lives of everyone affected by bowel cancer.

ABOUT BOWEL CANCER

- Bowel cancer is the UK's second biggest cancer killer—claiming a life every half an hour.
- Over 41,000 people are diagnosed with bowel cancer each year.
- Over 110 people are diagnosed with bowel cancer every day, that is someone every 15 minutes.
- Every 32 minutes someone dies from bowel cancer in the UK—just under 16,000 each year.
- If diagnosed early, over 90% of bowel cancer cases can be treated successfully.
- Around one in 17 people will get bowel cancer.
- It affects men and women almost equally.
- Most bowel cancer cases are in the over 50s, however the number of cases of bowel cancer in younger people is increasing rapidly, particularly in the under-30s which has increased by 120% in the last decade.

OUR RESPONSES TO THE SELECT COMMITTEE'S INQUIRIES PARTICULAR POINTS ARE AS FOLLOWS:

1. *NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS*

1.1 Beating Bowel Cancer welcomed the initial intention of the health technology appraisal process to eliminate regional variations in access to treatments and ensure that value for money is delivered to the tax payer. A number of treatments for bowel cancer have not been recommended by NICE and it is only through the Cancer Drugs Fund that bowel cancer patients have been able to access two types of chemotherapy for bowel cancer that are clinically effective and supported by clinicians.

1.2 Beating Bowel Cancer would urge NICE to learn from the Cancer Drugs Fund, which has demonstrated that NICE's costing templates do not necessarily reflect the actual cost of treatment.

1.3 Beating Bowel Cancer is keen to ensure that the patient perspective is incorporated into value-based pricing. The charity has set up a Commission on the Value of Treatments and its group of expert patients will be making recommendations on what matters to patients in terms of bowel cancer treatment. We would urge NICE to ensure that whatever role it takes in supporting value-based pricing, that patients are a central component of the process.

2. *The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities*

2.1 Beating Bowel Cancer has welcomed the publication of the quality standard for bowel cancer. NICE should actively work to ensure that quality standards are fully implemented. Recent news has suggested that the NHS Commissioning Board may be looking to other groups for this expertise. Beating Bowel Cancer believes that this step is welcome, if it means that guidance will be made available more swiftly. For example, on the issue of cancer chemotherapy. This was a topic that was initially referred to NICE by the Department of Health but no progress has been made in terms of its development.

3. *The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome*

3.1 NICE clinical guidelines should be reviewed regularly to take into account developments in care and treatment for a particular condition. NICE should also consider data that are made available, such as those from the National Bowel Cancer Audit to assess the extent to which NICE clinical guidelines are being adopted.

4. *The effect of the new public health system architecture on NICE's continued role in respect of public health guidance*

4.1 NICE's public health guidance should take into consideration the shared responsibilities across the public health and health services to encourage coordination where it is needed. For example, if an awareness campaign is undertaken by the public health service, NHS resources should be made available to ensure that patients can get access to testing and advice services on the NHS.

Written evidence from The Royal College of Midwives (NICE 10)

1. The Royal College of Midwives (RCM) is the trade union and professional organisation that represents the vast majority of practising midwives in the UK. It is the only such organisation run by midwives for midwives. The RCM is the voice of midwifery, providing excellence in representation, professional leadership, education and influence for and on behalf of midwives. We actively support and campaign for improvements to maternity services and provides professional leadership for one of the most established clinical disciplines. The RCM welcomes the opportunity to respond to this consultation and our answers to the relevant consultation topics are set out below.

SUMMARY

2. The RCM values the work of NICE in the production of Quality Standards and Guidelines in driving forward commissioning. However we also recognise their limitations and continue to have concerns about the methodologies used.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

3. The RCM thinks that NICE Quality Standards will be very useful in determining commissioning priorities, but that there should be a clearer link to the relevant NICE guideline, so that other key recommendations can have a role in driving up the quality of care.

4. It is also important that commissioners recognise that these are only guidelines and that there will be circumstances where users' needs may over ride NICE guidance and there is room for variation and individualisation of care based on user/clinician discussion. It should be acknowledged that the absence of evidence does not always mean that something should not be done, as has been the recommendation for example, on debriefing after a difficult birth experience.

5. In some NICE guidance, we have found a tendency for bias towards the status quo or to be written in a rather risk averse way. Examples of this are in the intrapartum guidelines in the recommendations made about the place of birth and the use of water for pain relief. These recommendations therefore tend to imply an opt out of the accepted system.

6. There is also the problem of the frequency of updating the guideline when new important evidence emerges, that should have an impact on the recommendation. This is exacerbated by the "forensic" approach taken to literature searching, limited to "the assessment of abstracts from high-level randomised controlled trials" when reviewing the need to update a guideline, as was the case with the Post Natal Guideline. We were aware of significant new evidence from surveys of women's views of their care that was not picked up through this method. We consider that the two week consultation period for proposals on updating guidelines is too short, as this does not allow stakeholders enough time to undertake their own literature searches.

7. NICE clinical guidelines remain limited in the context of midwifery care, by the grading of the evidence and the consequent dominant status of findings from randomised controlled trials.

8. So overall, we recognise the value of NICE guidelines, particularly in driving good commissioning. However we are aware of their limitations in terms of their methodology. They must be used in conjunction with other considerations, which should include expert opinion in the commissioning decisions.

The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

9. As discussed above, we think that NICE clinical guidelines have an important role. We think that NICE could undertake more work in encouraging implementation through audit and consideration of the barriers to implementation. It has not been clear how effective the field work undertaken by NICE has been. More resources might be needed for the field workers or NICE fellows to facilitate this important activity.

The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

10. The current public health architecture outlined below, is confusing, particularly in relation to commissioning public health activities.

- Local authorities will be responsible for health improvement activities, such as smoking cessation, sexual health and drug/alcohol services. They can commission these services directly or they may choose to commission them jointly with clinical commissioning groups (CCGs).
- The NHS Commissioning Board will commission some public health services nationally as agreed with the Secretary of State eg immunisation and national screening programmes.
- CCGs will be responsible for commissioning public health services for nought to five year olds.

- Public Health England will be responsible for commissioning some services in relation to prevention, infectious diseases, emergency preparedness and health intelligence.
- There are many areas where different agencies could be responsible for commissioning services. For example, in respect of tobacco control: local authorities will be responsible for commissioning local activities, including smoking cessation services; CCGs will be responsible for commissioning brief interventions in secondary and maternity care; the NHS Commissioning Board will be responsible for commissioning brief interventions in primary care.

11. With commissioning arrangements that are so complex there is clearly the potential for some confusion. In this context RCM has the following concerns:

- (a) whether all the respective agencies will use NICE guidelines and quality standards in a consistent manner;
- (b) whether guidelines/standards will cut across commissioning responsibilities ie smoking cessation and, if so, how will they be applied; and
- (c) whether NICE will need to duplicate guidance for different agencies.

October 2012

Written evidence from the British Thoracic Society (NICE 11)

- NICE has an accurate and robust system that enables difficult decisions on new therapies to be made; it may be too slow.
- The NICE system should fit well with Value-based pricing provide value is measured appropriately.
- NICE guidelines and quality standards are essential to improve healthcare; scope might need to be limited.
- NICE guidelines need to recognise the potential variation in priorities for implementation in different populations.
- NICE is ideally placed to provide a scientific evaluation of social care interventions, but the effectiveness on NICE should be evaluated early.
- NICE should commission other bodies to produce high quality guidance in areas that NICE cannot cover; limited funds should be available to ensure a high quality process.

1. NICE has an accurate and robust system for evaluating the effectiveness and cost effectiveness of drugs and other clinical interventions. This process has been criticised as being too slow and NICE have been accused of preventing patients from having access to treatments that are available elsewhere in the world. However, there are limited resources and therefore it is important that each therapy is evaluated as accurately as possible and decisions (often tough ones) are made in the best interests of all patients and the NHS as a whole. NICE does this well. NICE has made some attempts to fast track some appraisals.

2. Value-based pricing may not have much impact on the NICE Technology Appraisal process unless the parameters by which value is currently measured are changed. It would seem inappropriate to do this as the measures currently used are scientifically based. Where some drugs are very high cost but have a very high value to patients, there will still need to be a ceiling. NICE have used these limits effectively in the past to encourage pharma to reduce costs of some drugs and this important function of NICE should not be lost.

3. Guidelines are an important tool to improve the standards of healthcare but if they are not implemented effectively and comprehensively, they do not achieve the full benefit. The NICE Quality Standard process (as well as the excellent NICE implementation tools) serves to bring recommendations in guidelines to the attention of those in the NHS who commission services so that decisions can be made on the best contemporary advice. The NICE guideline development process is one of the most respected in the world and should continue to be at the centre of what NICE does. Standards must not be compromised. There may be a role for limiting the scope to topics where change in practice might have the largest potential impact on health rather than, as is currently the case, on the largest patient populations.

4. If NICE is to continue to help drive up standards then it is important that local healthcare infrastructure does not prevent implementation of important changes recommended in NICE guidance. However, the ability to prioritise intervention according to local needs can be a good way to maximise the value obtained from limited resources. NICE will need to recognise that some recommendations and Quality Standards might have different values in different socio-demographic settings.

5. NICE's new responsibilities with regard to social care interventions should provide a more evidence based approach to this area. It will no doubt substantially increase the work of NICE and consume considerable resources. An early evaluation of the cost-effectiveness of this aspect of NICE's work should be undertaken to identify areas where a scientific approach is of most value. NICE will be in a good position to ensure effective integration of recommendations that overlap health and social care.

6. NICE is unable to provide guidance and quality standards in all areas and should in the future be more open to adopting guidance by other professional bodies provided that those bodies maintain appropriate

standards. Consideration should be given towards the provision of limited funds to commission guidelines in areas of need where NICE is unable to complete a guideline. These funds should be used to enable other bodies to achieve the same standards in guideline production as NICE. The British Thoracic Society would be supportive of this approach.

October 2012

Written evidence from HEART UK (NICE 12)

HEART UK is the nation's cholesterol charity. HEART UK is responding on the role of NICE clinical guidelines in improving the quality of healthcare and the role of NICE Quality Standards in the new NHS architecture.

THE ROLE OF NICE CLINICAL GUIDELINES IN IMPROVING THE QUALITY OF HEALTHCARE

HEART UK believes that NICE clinical guidelines present an opportunity to make genuine improvements in healthcare. The guidelines are based on the best available evidence for high quality treatment. However, in some instances, it is clear that NICE guidelines are not being implemented.

A case in point is the wholesale failure to implement the NICE Guidelines on Familial Hypercholesterolaemia (FH) in England (CG71), published in 2008. There are an estimated 120,000 people living with FH in the UK. Yet only 15–20% of these have been formally diagnosed. Children of an individual with FH have a 50% chance of inheriting the condition. Left untreated, FH may lead to premature death from CVD. 50% of males with untreated FH will develop coronary heart disease (CHD) before the age of 50 (for females, 50% have CHD before age 60). 50% of untreated males will die before they are 60. These deaths are avoidable. Unlike many genetic conditions, FH can be diagnosed relatively easily and, with inexpensive treatment, people with FH can lead normal, healthy lives.

The NICE guideline recommends identifying cases of FH, using cholesterol measurements and genetic testing of families, by a method known as cascade screening. Referral to specialist lipid clinics is recommended for confirmation of the diagnosis, patient counselling and in order to initiate cascade screening.

The devolved countries each have a national directive or initiative specifically targeting FH, which has helped them achieve higher standards of care for their FH patients than for those in England.

Despite the NICE Guidelines, we know that little has been achieved in England. Indeed, in 2010, to better understand the extent to which the Guideline has been implemented in England, HEART UK conducted a study in which Freedom of Information requests were sent to Primary Care Trusts, asking about their progress to date. The study showed that little has been done to implement the Guideline in England. The findings demonstrate a lack of formal planning for FH and incomplete provision of clinical services and education about FH. There is a paucity of specialist services, including provisions for paediatric, obstetric, as well as adult patients. The NHS Health Checks programme recommends that people identified with total cholesterol >7.5 mmol/L should be considered as a possible case of FH, with referral advised according to clinical guidelines. However, identification continues to take place on an *ad hoc* basis and the commissioning of FH services remains very limited.

Translating the NICE FH Guidelines into practice has not worked for a number of reasons, including:

- Issues associated with the localised commissioning structure have hampered the development of FH services and access to genetic testing.
- Lack of clinical awareness and understanding of FH.
- Lack of appropriate IT software that would aid the process of cascade screening and registering patient data.
- Lack of lipid clinics to help treat people with complex lipid disorders.
- Management/financial blocks to purchase an FH genetic test, as the test is considered by some to be a “new development”.

A number of elements need to work effectively in unison in order for the NICE Guidelines on FH to be implemented. HEART UK believes that the FH Guidelines can most effectively be implemented with national leadership.

HEART UK recommends an England national programme should be established to improve the diagnosis and treatment of people with FH. In February 2012, HEART UK published a new report, *Saving lives, saving families: The health, social and economic advantages of treating Familial Hypercholesterolaemia*. (Download the report at: http://heartuk.org.uk/files/uploads/HUK_SavingLivesSavingFamilies_FHreport_Feb2012.pdf). The report includes new economic modelling that demonstrates the health and cost savings that can be made through improved identification and treatment of FH.

Key findings of the research include:

- High intensity treatment, compared to low intensity or no treatment, results in greater reduction of low density lipoprotein (LDL) cholesterol and major cardiovascular events, which translates into more quality adjusted life years and life years gained.
- High intensity treatment will mean 101 cardiovascular deaths avoided per 1,000 FH patients (aged 30 to 85 years), when compared with no treatment.
- The UK could save £378.7 million from cardiovascular events avoided if all (100%) relatives of FH index cases are identified and treated optimally over a 55 year period, or £6.9 million per year.

Examining the evidence and FH programmes in other countries, HEART UK suggests the following actions to help realise these improvements:

1. A national programme for FH in England under the NHS Commissioning Board or similar body. This is the best means of ensuring that access to FH services is available beyond the limited boundaries of a PCT or clinical commissioning group. A national programme should have ring-fenced funding and include the following:
 - (a) A dedicated network of involved professionals, including lipid clinics, primary care and genetic services.
 - (b) Clear referral pathways at local level.
 - (c) Employment of FH nurses to rollout the cascade screening process.
 - (d) Measurement of outcomes as the programme is rolled out at local level.
2. A UK-wide national patient register and database for FH to aid better cascade screening across the country.
3. Improved capacity of lipid clinics to manage patients with possible or definite FH.
4. Increased education and training programmes that have been developed to nationally agreed standards and contain nationally agreed content.

A National programme for FH could emulate the work already taking place in Wales. Wales has established the FH All Wales Cascade Testing Service. This features a diagnostic service for FH combined with family cascade testing. The Wales service uses the NICE Guidelines on FH as the evidence base for its work. The Welsh Assembly is providing long-term funding for the project.

The service is multidisciplinary, and links with elements of current lipid clinic provision, clinical genetics, paediatrics and laboratory testing. The service is hosted by Cardiff and Vale Health Board and managed by the All Wales Medical Genetics Service with oversight from a multidisciplinary all Wales steering group.

A key benefit of the service is that it enables wider availability within Wales for referral to specialist lipid services and for genetic testing for FH. It provides a pathway to assist diagnosing FH for use in primary and secondary care and also sets out a system for family testing for FH. As a result, a Welsh national database of patients with FH is being developed. Based on experience from the Dutch testing programme, the service aims to identify 60% of those affected in Wales over a 10-year period.

The Welsh experience demonstrates the value of national leadership to aid the implementation of the clinical gold standards contained in the NICE FH Guidelines. This leadership is critical for the implementation of the same Guidelines in England.

THE ROLE OF NICE QUALITY STANDARDS IN THE NEW NHS ARCHITECTURE, IN PARTICULAR THE STATUS OF NICE GUIDELINES IN DETERMINATION OF COMMISSIONING PRIORITIES

Defining high standards of care for specific conditions presents another useful opportunity to demonstrate and encourage optimum care. HEART UK is pleased that a Quality Standard is being developed for FH. However, the implementation of that Quality Standard after it is completed remains to be seen.

For better detection and treatment of FH, a national programme is the best means of delivering improvements in patient outcomes. Localised commissioning structures cannot accommodate the long-term view required to realise the gains of cascade testing for FH.

Written evidence from the British Society of Gastroenterology (NICE 14)

NICE has brought a lot of benefits and has had some notable successes. Technology Appraisals are often prompt and provide clear outputs. Clinical guidelines and quality standards have also proved to be very useful to our members' everyday clinical practice.

As NICE moves forward into a new phase some consideration is needed as to how to ensure its decisions are complied with. A new guideline on acute upper GI bleeding, for example, recommends that all busy hospitals should have arrangements for 24/7 access to services, but from informal surveys we believe that only 50% of hospitals in England & Wales are able to meet this standard of care. NICE will lose credibility if it pushes for change in one area while turning a blind eye to inactivity in others.

- Moving forward NICE needs to form partnerships to ensure that its guidance can cover the ambitious areas it has set itself. It should seek to work closely with the specialist societies in this regard.
- NICE needs to help join up advice and guidance to public health and to NHS sectors.
- We have some concerns that as quality standards develop they will become generic and seen as the guideline for clinical management, rather than the more detailed guideline that sits below it. Attention is needed to ensure that quality standards don't become too generic and overshadow its specific details and dedicated guidelines.
- We have some concerns that recent guidelines tend to focus on "average" patients. The new guideline on Crohn's disease is a case in point. In complex cases, and in many settings, patient management is not ordinary and strict adherence to care management as described in a quality standard may mean patients not receiving the care and treatment they need. Guidelines should not replace or prevent the expert advice provided by consultants for all other patients.
- As Quality Standards are developed NICE needs to take a broad view of how services are provided. There are concerns within the membership that healthcare providers will strive to meet standards at the expense of quality and safety of care as happened with the target regime of the 2000s.
- At present NICE guidance is defined by its remit which is often very focused. The guidelines developed are often less-inclusive of connected issues. The development of Quality Standards is an opportunity to broaden the focus.

October 2012

Written evidence from Alzheimer's Society (NICE 15)

ALZHEIMER'S SOCIETY'S SUBMISSION TO THE HEALTH COMMITTEE

Alzheimer's Society is pleased to submit evidence in relation to the Health Committee's inquiry into the National Institute for Health and Clinical Excellence.

ALZHEIMER'S SOCIETY

Alzheimer's Society is the leading care and research charity for people with Alzheimer's disease and other forms of dementia, their families and carers. The Society has expertise in providing information and education for people with dementia, carers and professionals. It provides a helpline and support for people with dementia and carers, runs quality day and home care, as well as funding medical and scientific research. It campaigns for improved health and social services and greater public understanding of all aspects of dementia.

1. NICE's role in relation to evaluating the effectiveness and cost effectiveness of drugs and other clinical interventions including how its role will be affected by the intended introduction of value based pricing for drugs purchased by the NHS

1.1 Alzheimer's Society believes NICE has a vital role to play in supporting fair access to clinically effective treatment. Patients, carers and healthcare professionals require access to independent and comprehensive evaluations of drugs and other clinical interventions.

1.2 Careful scrutiny and monitoring of the implementation of guidance is essential as NICE take forward their work programme. Implementation of NICE guidance has been an ongoing concern for Alzheimer's Society. We continue to receive calls from people with dementia being denied access to the four medicines licensed for Alzheimer's disease even after the publication of NICE guidance recommending access to the treatments. This is despite the legal duty on PCTs to fund treatments that have been positively appraised by NICE.

1.3 It is vital that individuals have appropriate access to treatments that are clinically and cost effective. Accordingly, Alzheimer's Society welcomed David Nicholson's recent announcement that NHS organisations will be required to publish information which sets out which NICE Technology Appraisals are included in their local formularies. There must be firm action taken against any local areas that are failing to implement NICE guidance.

1.4 It is difficult to comment on the introduction of value based pricing without more details of how it will work in practice. One of the many complexities of NICE appraisals of Alzheimer's drug treatments was the inclusion of the wider benefits of the drug treatments, particularly carer benefits. These complexities will no doubt be a challenge to the development of value based pricing. Considerable work will be required to develop definitions of "value", to understand how to measure value and how to set prices according to value. Patients and carers must be closely involved in that work.

1.5 The assessment and decision making process in NICE appraisals is likely to become more complex once value based pricing is adopted. Ensuring that this process is transparent and understandable will therefore be a significant challenge. But it is important that the public understand how decisions about care and treatment are reached if they are to have confidence in the fairness of the process.

1.6 Alzheimer's Society urges NICE to continue to involve patients and the public in their decision making. However, we would like NICE to provide clearer guidance on how they use patient group evidence within their appraisals and guidelines. It is not clear at the moment whether the evidence we provide is in a format that can be used by NICE or how the evidence is used within the appraisal process. Although NICE produce guidance and their Patient and Public Involvement Programme has always been very helpful, it is difficult to understand how our evidence has been used. In our experience the balance is weighted heavily towards evidence from clinical trials and it is not clear how other evidence might influence decision making.

1.7 We recommend that NICE publish specific examples of how patient evidence has informed and influenced their decision making process. This would provide helpful clarity when producing evidence for NICE appraisals. Producing submissions for NICE is an expensive and time consuming process and we want to make sure NICE are able to use the patient and carer evidence we provide to maximum effect.

2. The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

2.1 We support the commitment in the National Quality Board's draft report *Quality in the new health system* (2012) that "The Quality Standards of today will need to become the essential standards of tomorrow." This is the goal that the NHS as a whole should be working towards. Developing a timetable for achieving this would be a strong driver for continuous quality improvement. It is important that the Quality Standards move beyond the aspirational and become the usual standard of care as soon as possible.

2.2 Clinical Commissioning Groups will be working to the Commissioning Outcomes Framework, which will be developed as Quality Standards are produced. Therefore, it is important that NICE are adequately resourced to produce Quality Standards, alongside their other duties.

2.3 The risk is that the priority given to particular conditions by commissioners will depend on whether the condition has a clinical guideline and a Quality Standard. NICE and the NHS Commissioning Board need to keep this under review to ensure conditions that do not have a Quality Standard are not ignored. We support the "NICE accreditation scheme", which allows other bodies to produce their own guidance, as it can help to fill any gaps where there is no NICE guidance.

3. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

3.1 Clinical Guidelines are important in the promotion of good dementia care. The dementia clinical guideline is useful to health and social care staff, as well as people with dementia and their carers, for a number of reasons. It covers the whole pathway of care, from initial identification to end of life care. It also brings together evidence and expert opinion in a comprehensive way that can guide decision making and explain to individuals the care and health interventions that should be available to them. Furthermore, clinical guidelines are used as the basis for Quality Standards which will help guide commissioning and drive up the quality of care. It is important that Quality Standards have a strong evidence base that the NICE Clinical Guideline can provide.

3.2 It is important that NICE continue to produce clinical guidelines and update them as the evidence base develops.

4. What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services?

4.1 Evaluating social care interventions will require NICE to work with the different types of evidence available with regard to social care. It is important they are flexible in their approach to using this evidence.

4.2 NICE social care evaluations present a useful opportunity to develop the evidence base around social care because they may highlight gaps in existing evidence and demonstrate what data need to be collected. Where the evidence base is inadequate, NICE could lead work, with relevant organisations and experts, to develop ways of assessing outcomes and rigorously evaluating interventions.

4.3 We recommend that NICE produce joint health and social care guidelines and Quality Standards in relation to specific conditions in order to promote well-coordinated care and support. As well as promoting an integrated approach to services amongst staff and commissioners, it would help people with dementia and carers understand the full package of care that they should be receiving.

October 2012

Written evidence from the Faculty of Pharmaceutical Medicine (NICE 16)

The Faculty of Pharmaceutical Medicine (FPM) of the Royal Colleges of Physicians of the UK welcomes the opportunity to respond to the Health Select Committee's call for evidence to its Inquiry into the work of NICE. The FPM is a professional membership organisation with approximately 1,400 members of whom about 1,100 are based in the UK. The members are practising or retired Pharmaceutical Physicians or those with a professional interest in the specialty. The FPM's mission is to advance the science and practice of pharmaceutical medicine by working to develop and maintain competence, ethics and integrity and the highest professional standards in the specialty for the benefit of the public. The FPM seeks, through its many activities, to bring about improvement in the health of the public and thus has an interest in the future role of NICE.

1. NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

1.1 Since its inception NICE has played an important role in advising the NHS on the clinical and cost effectiveness of new medicines and other technologies. Essential to this activity has been the requirement on Hospital and Primary Care Trusts to introduce NICE's recommendations without delay. In the new commissioning based NHS from April 2013, it is vital that NICE's recommendations on new medical technologies including medicines continue to be introduced without delay.

1.2 In the last few years, NICE has introduced a system of scientific advice for companies developing potential new medicines into the NHS. The FPM hopes that this system will continue and be enhanced thus providing more opportunities for pharmaceutical companies to interact with NICE in planning development programmes thus enabling those programmes to address the needs of all stakeholders including patients.

1.3 NICE has a worldwide reputation as a major Health Technology Assessment organisation and its decisions are carefully monitored and often followed in other countries. The FPM therefore believes that the system of scientific advice and interaction with pharmaceutical companies outlined in 1.2 is vital with the development of new medicines becoming more and more costly.

1.4 In 2014, a new system of value-based pricing for new medicines is due to be introduced and NICE will play an important role in advising the Secretary of State on the value of new medicines. The FPM broadly supports the concept of value-based pricing as it is committed to ensuring that the best value medicines are made available to patients. The pharmaceutical industry's current major research priorities including cancer, heart disease and stroke, dementia, diabetes and other long term conditions such as rheumatoid arthritis align very well with the NHS's priorities for healthcare. As indicated in 1.3, the cost of new medicine development is escalating considerably and therefore it is vital that value-based pricing provides sufficient short to medium-term return on R&D investment for the continuation of research into innovative medicines. If the return is inadequate, it is likely to continue to drive pharmaceutical R&D away from the UK which will inevitably have an adverse effect on the future health of the UK public.

1.5 The FPM believes that it is vital to recognise that much innovation is incremental and that the day of new "blockbusting" medicines has probably ended. Nevertheless, these increments can be of significant benefit to patients and NICE will play an important role in determining the value of these increments. The FPM believes that if only downstream value is considered and upstream investment along with inevitable pipeline attrition ignored, then there is likely to be a failure to conserve a sustainable industry within the UK.

1.6 The FPM believes that the proposed new pricing structure for medicines should reflect the long term gains of having new medicines available as when they go off-patent society gains from having them available for many years as cheaper generic medicines. It should be remembered that most of the WHO essential medicines were once innovative new medicines.

1.7 The FPM notes that NICE will become the National Institute for Health and Care Excellence from April 2013 and believes that just using Quality Adjusted Life Years (QALY) as a measure of effectiveness for a medicine may be inadequate. The QALY does not measure changes in health status effectively particularly in end of life care where prolongation of life is not well represented. The potential improved impact on carers of a new medicine will need to be considered and that isn't the case when just using the QALY.

1.8 The FPM believes that where NICE have provided pre-launch advice for a new medicine then that advice and the accompanying cost/value discussions should generally be binding. If the company delivers what has been requested then the price should be justified and not challenged.

1.9 The UK is the only country in Europe that allows a new medicine to be prescribed on its day of launch. The FPM believes that under no circumstances should the value-based pricing system prevent that advantage for UK patients from continuing.

2. The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

2.1 The FPM believes that NICE Clinical Guidelines are probably the most important output of NICE as they are evidence-based best practice guidelines. It is vitally important that commissioners take them into account positively when commissioning services so that patients can be assured that they will be receiving the best available care. Commissioners should also consider decommissioning services where NICE advises through their guidelines. This is one of the original remits of NICE that has not been pursued as well as it should.

3. The continuing role of NICE clinical guidelines in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

3.1 As outlined in para 2.1, the FPM believes that Nice Clinical Guidelines are one of the most important outputs of NICE. They indicate current best practice in the relevant condition and although clinicians are treating the individual patient in front of them, they should be able to justify why they have stepped outside a guideline in treating that specific patient. There will of course be variations but it is that variability that confirms the heterogeneity of patients and that will be of interest to research based companies as they develop future stratified medicines. This will be where the electronic patient record will be useful as these variations will be recordable and then researched from the anonymised record.

4. What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services?

4.1 The FPM believes that a systems-based approach is required for health/social care with full integration of medicines development, optimising treatment guidelines, prescribing, patient compliance, social care and environmental impact. There is an opportunity for NICE's new responsibilities to bring quantitative research into social care interventions and more qualitative research methods into healthcare. However care must be taken not to downgrade healthcare as NICE considers the potential tradeoffs in priorities for funding and resources.

October 2012

Written evidence from Merck Serono Ltd (NICE 17)

1. NICE is currently performing a critical role into developing the most complete clinical and health technology guidelines, shaping and advising clinicians' everyday practice.

We believe that NICE has done a great job up to today, work that is nowadays replicated in many countries around the world.

Nonetheless, we believe there could be improvements made to the NICE process to ensure more consistency with regard to making recommendations about particular health technologies. Set out below are few observations and suggestions about the process:

- Pharmaceutical companies should be given more of a voice at the committee meetings.
- Academics should be challenged more vigorously.
- Academics should also perhaps leave the Committee when decision making is taking place (Part 2 of Committee meeting).
- Academics contracted by NICE should not be able to use the work they have been commissioned to do for publication purposes.
- There should be consistency across the four Committees as to whether to recommend health technologies. We have observed inconsistency whereby some Committees are less likely to recommend the use of treatments against cancer.

It would be an opportunity for the new leadership to review the NICE process accordingly.

2. Overall, Merck Serono would make the following recommendations about governance and implementation:

- For the future there should be a separation between the organisation developing the NICE framework and the one applying it.
- NICE's remit should be to apply the future framework to their process and outcomes.
- NICE's remit is also to ensure implementation of positive guidance, having absorbed the MPC, the synergy should ensure that medicines are listed in all local formularies.

- Failing to implement guidance and guidelines should result in clear accountability.

October 2012

Written evidence from the British Society for Rheumatology (NICE 18)

1. ABOUT THE BSR

1.1 The BSR is an organisation of and for clinicians, health professionals, and scientists that advances rheumatology through providing clinical leadership, programmes of education, advocacy and practice support.

1.2 The BSR has joined with two patient organisations to create the Rheumatology Commissioning Support Alliance to provide combined clinical and patient advice and support to policy makers and commissioners. It has been set up to develop a new commissioning support tool, which will be deployed with a health economy over the next two years to improve the commissioning of services in rheumatology.

1.3 “Rheumatology” or “rheumatic disorder” refers to an acute or long term condition which is characterised by inflammation and reduced function of connecting or supporting structures such as the joints, tendons, ligaments, bones and muscles. In some cases this can go on to cause permanent disability without treatment. The therapeutic interventions in such disorders is called Rheumatology, a multidisciplinary branch of medicine that deals with the investigation, diagnosis and care of patients with one or more of over 200 disorders affecting joints, bones, muscles and soft tissues, including inflammatory arthritis and other generalised autoimmune disorders. Often other organs are involved and require input.

1.4 Rheumatology is in the midst of a period of exponential growth in knowledge of the mechanisms of rheumatic and auto-immune disease, knowledge which is transforming and advancing our treatment options.

2. SUMMARY OF KEY POINTS IN SUBMISSION

- Full support for new approach to developing clinical guidelines and commissioning guidance.
- BSR willing to explore active participation in new model.
- Concerns over: value based pricing; less than universal funding of NICE-approved therapies; lack of capacity for smaller organisations being able to participate in NICE-led processes, for resource reasons.
- Strong commitment from BSR to continuing involvement of patient and clinical expertise.

3. FULL SUBMISSION

3.1 The British Society for Rheumatology (BSR) www.rheumatology.org.uk welcomes the opportunity to provide evidence to the Health Committee inquiry into the functions of NICE. We would like to make the following comments.

3.2 The BSR strongly supports NICE continuing its role in producing technology appraisals (TAs), clinical guidelines and developing Quality Standards. We believe Specialist Societies with accreditation from NHS Evidence could play a more formal role in supporting NICE, through assisting its programme of developing clinical guidelines, including specialty level commissioning, best practice models and guidelines. Indeed, Sir Bruce Keogh said, “People like the Royal Colleges and specialist associations have [in the past] worked on their own to produce guidance for their members. We need to think about how we quality assure that guidance, and where it is good and appropriate, to integrate it into the mainstream commissioning system.” The BSR fully endorses this approach and stands ready and willing to participate in such a process, including possible pilot schemes in the shorter term.

3.3 We have recent experience in this area which we believe will be useful to NICE and other stakeholders going forward. For example, we have led on developing new clinical guidelines in gout; whilst the BSR has also worked closely of late with the British Orthopaedic Association to partner in the development of guidelines on musculoskeletal issues (eg knee, hip, hands).

3.3 It is in our view critically important that NICE continues to use expert professional opinion, including specialist societies such as the BSR, for the above. We understand that NICE is introducing a new process for developing Quality Standards to speed up production, using standing committees to which experts are invited, rather than recruiting a new Topic Expert Group. NICE needs to ensure that guideline production and STAs have adequate expert representation and a transparent process for inviting and selecting expert nominations.

3.4 We have serious concerns about the lack of clarity around some aspects of value based pricing and its potential effects on commissioning, as well as on the wider relationship between the pharmaceutical industry and the NHS.

3.5 We also have serious concerns about the potential lack of obligation on the part of commissioners to fund therapies in accordance with NICE TAs.

3.6 It is essential in our view that NICE includes appropriate patient and professional opinion in relation to its new roles in public health and social care.

3.7 We would also wish to see more support provided to smaller organisations with remits relevant to TA topics, but who often find it difficult to devote the human resources and time needed for formal submissions.

4. CONCLUSION

4.1 The BSR believes that despite some concerns articulated above, NICE still has an important role to play in ensuring that patient outcomes in the area of rheumatology are optimised within available resources.

4.2 We are also willing partners in exploring new ways of ensuring that expert clinical leadership plays its part in the process of developing the sort of guidelines and commissioning guidance which lead to better clinical outcomes for patients, no matter where they live.

October 2012

Written evidence from Rare Disease UK (NICE 19)

SUMMARY

- RDUK's response relates to the announcement that as of April 2013, NICE will appraise high cost, low volume drugs for rare diseases.
- RDUK would like clarification about what interim method NICE will use to appraise high-cost, low volume drugs.
- RDUK is keen to ensure that the ability of the current AGNSS decision making framework to take into account a wide range of factors is not compromised by an increased emphasis on cost/QALY analysis.
- RDUK calls for resources to be put in place to enable active patient engagement in the decision making process to be undertaken.
- RDUK calls on NICE to conduct a full consultation on a new process for appraising ultra-orphan drugs as soon as possible (should it wish to use a system other than the current AGNSS system).
- RDUK would like clarification on whether, in the event of a "not recommended" by NICE, whether the NHS Commissioning Board would be able to commission a drug as part of a service, if it has identified a need for the drug in question.
- RDUK would like clarification on how the expertise developed by AGNSS in this field will be utilised by NICE.

ABOUT RARE DISEASE UK

1. Rare Disease UK (RDUK) is the national multi-stakeholder alliance for people with rare diseases and all who support them. Our membership of over 1,200 includes:

- Over 220 patient organisations.
- 13 pharmaceutical companies.
- Clinicians.
- Professional bodies.
- Health professionals.
- Researchers.
- Individual patients and families.

2. RDUK is an initiative of Genetic Alliance UK, the national charity representing 155 patient organisations supporting people affected by genetic disorders.

NICE'S NEW ROLE ASSESSING MEDICINES FOR RARE DISEASES

Background

3. In July this year, it was announced that, as of April 2013, NICE will assess high cost drugs for rare diseases.

4. Until recently, NICE could refer drugs for fewer than 500 people in England to the Advisory Group for National Specialised Services (AGNSS) for assessment. AGNSS developed a framework to aid the decision-making process in recognition that the standard NICE HTA process is not suitable for these drugs, which are often high-cost and have greater evidential uncertainty due to the challenges of developing treatments for very small patient populations. AGNSS uses this framework to make recommendations to Ministers about whether the drug in question should be available to patients on the NHS.

5. The use of the AGNSS's decision-making process for medicines is in its infancy; only two medicines have gone through the process and the final decision now lies with Ministers about whether these medicines should be available. A moratorium on AGNSS's work was announced earlier this year and AGNSS will cease to exist when the NHS Commissioning Board becomes operational in April.

NICE's appraisal of ultra-orphan drugs

6. RDUK welcomed the announcement that NICE would take over the assessment of drugs for very rare diseases in terms of clarifying who would have the responsibility for assessing these drugs, and that a separate process to that of NICE's usual HTA process would continue to be used. NICE responsibility for these decisions could also be beneficial in terms of having a body making decisions which is independent from Ministerial control. RDUK does, however, have a number of issues we would like clarification on:

6.1 It was announced that NICE will develop "interim methods for the first for the first few drug assessments". RDUK would like clarification about what these methods will be, whether these methods will be based on the current AGNSS framework and what NICE's plans are to consult with relevant stakeholders in developing this process, should it be a different process to the AGNSS framework. RDUK believes that the AGNSS process should be used in the interim as opposed to a process that is un-tested and has not been subject to wide-consultation.

6.2 RDUK is keen to ensure that the ability of the current AGNSS decision making framework to take into account a wide range of factors is not compromised by an increased emphasis on cost/QALY analysis.

6.3 AGNSS recognised that when dealing with small patient populations which may not be represented by a well-established patient organisation, pro-active steps have to be taken in order to find patients and to support patients or patient groups to make effective patient submissions. Resources were in place to enable this work to be undertaken. It is crucial that NICE continues to take this approach.

6.4 RDUK understands that a consultation will take place to develop a new system to appraise drugs for very rare diseases; however, this consultation may not take place until 2014. This could mean an interim process that has not been consulted on is used for well over a year. As a result RDUK calls on NICE to conduct a full consultation as soon as possible.

6.5 Many specialised services for very rare diseases commissioned on a national basis have been closely aligned to the delivery of a drug, for example, enzyme replacement therapy for lysosomal storage disorders. This approach has led to a multitude of benefits including:

- ensuring proper adherence to protocol;
- ensuring high-quality prescribing of what are often very high-cost drugs;
- enables accurate record-keeping, including the collection of data on patient registries; and
- the delivery of the drug becomes a component of a co-ordinated service.

Under the new system, the body making the decision on a drug (NICE) will be separate from the body commissioning services (NHS Commissioning Board). RDUK would like clarification on whether, in the event of a "not recommended" by NICE, whether the NHS Commissioning Board would be able to commission a drug as part of a service, if it has identified a need for the drug in question.

6.6 RDUK would like clarification on how the expertise developed by AGNSS in this field will be utilised by NICE.

NICE's appraisal process for other orphan drugs

7. AGNSS had the ability to give advice on drugs intended to treat fewer than 500 patients in England (one in 106,000 of the general population) whereas to qualify for orphan designation, a drug must be intended to treat a condition affecting fewer than five in 10,000 of the general population. This amounts to around 26,500 patients in England.

8. Drugs intended to treat more common rare diseases might be able to be appropriately appraised using NICE's standard HTA process, however for drugs to treat rarer diseases it may not be appropriate. A drug treating 800 patients in England, for example, is likely to have many of the same characteristics as a drug treating 400 patients in terms of the high-cost and higher levels of evidential uncertainty.

9. As part of the consultation on the appraisal process for drugs for very rare diseases, NICE should examine the appropriate threshold for the application of a separate appraisal process, or whether there should be flexibility in the application of the process, as opposed to a set threshold which will inevitably be arbitrary to some extent. This is particularly so as the number of patients affected by a particular condition is often difficult to ascertain.

Written evidence from the National Heart Forum (NICE 20)

ABOUT THE NATIONAL HEART FORUM

The National Heart Forum (NHF) is a leading charitable alliance of 70 national organisations working to reduce the risk of coronary heart disease and related conditions such as stroke, diabetes and cancer. NHF is both a UK forum and an international centre for non-communicable disease (NCD) prevention. Our purpose is to co-ordinate public health policy development and advocacy among members drawn from professional representative bodies, consumer groups, voluntary and public sector organisations. Government departments have observer status. The views expressed here do not necessarily reflect the opinions of individual members of the forum.

INTRODUCTORY REMARKS

The National Heart Forum restricts its submission to comment on the effect of the new public health system architecture on NICE's continued role in respect of public health guidance.

NHF and NICE staff have had a productive working relationship over a number of years. NHF staff and trustees have contributed to NICE guidance through programme development groups on cardiovascular disease prevention, obesity, spatial planning, diabetes and behaviour change.

NHF RESPONSE ON THE EFFECT OF THE NEW PUBLIC HEALTH SYSTEM ARCHITECTURE ON NICE'S CONTINUED ROLE IN RESPECT OF PUBLIC HEALTH GUIDANCE

1. We stress the importance of ensuring that under the new arrangements, we continue to achieve a robust evidence-based approach to prevention of disease and the promotion of population health.

2. Over the years, NICE has established a solid track record in producing evidence and evidence appraisal that is trusted by health service commissioners, practitioners, academic and professional organisations and civil society. It has a well-developed structure with the necessary competencies and expertise that allows it to perform an important leadership role in evidence appraisal across the whole spectrum of public health issues including immunisation and screening.

3. We recognise that new structures under the devolved public health arrangements, such as Health and Wellbeing Boards (HWBs), will need to access evidence and evidence appraisal of the highest possible standard in order to determine local health and social care strategies.

4. We see NICE as a principal resource to HWBs alongside the Cochrane Collaboration and other professional organisations and charities to provide good advice that goes beyond randomised controlled trial evidence but is subject to the most rigorous standards to ensure elimination of bias.

5. The evolving remit of NICE is well-adapted to the integration between health and social care policy and delivery.

6. We see the future roles of NICE and Public Health England (PHE) as complementary. NICE should continue to provide a national repository of evidence while PHE has a clear role to play in the development of bespoke solutions for local level public health practice.

7. We would like to see a Memorandum of Understanding between NICE and PHE to ensure clarity about their respective functions and responsibilities and the avoidance of any duplication of effort.

October 2012

Written evidence from Genzyme Therapeutics (NICE 21)

1. Genzyme Therapeutics has pioneered the development and delivery of transformative therapies for patients affected by rare and debilitating diseases for over 30 years. We are focused on rare diseases and multiple sclerosis, and are dedicated to making a positive impact on the lives of the patients and families we serve.

2. From April 2013, the National Institute for Health and Clinical Excellence (NICE) will be assessing new medicines for people with extremely uncommon medical conditions, often high-cost, low-volume drugs, commonly referred to as "ultra-orphan drugs". Previously, the Advisory Group on National Specialised Services (AGNSS) undertook a multi-criteria decision analysis (MCDA) assessment of ultra-orphan drugs, which takes account of many factors. AGNSS was set up, in part, after NICE had concluded that its existing methodology—the use of the Quality Adjusted Life Year (QALY)—would not be appropriate to assess ultra-orphan drugs.¹³

3. The Government also intends to introduce a system of value-based pricing for branded pharmaceuticals with NICE undertaking the health technology assessment for all new drugs. Details on the value-based pricing

¹³ NICE. Appraising Orphan Drugs. Draft v3. 2006. <http://www.nice.org.uk/niceMedia/pdf/smt/120705item4.pdf>

scheme—due to start on 1 January 2014—are very limited in general and non-existent for ultra-orphan drugs.^{14, 15}

4. At this stage, we are unconvinced that a single process of value-based pricing can take adequate account of innovative drugs, such as enzyme replacement therapy, which are unable to expand into different disease indications as they have been developed for very specific rare diseases. We believe that NICE should retain a separate process for assessing new ultra-orphan drugs not try to assimilate their assessment into existing plans to develop a new system of value-based pricing for branded pharmaceuticals.

5. In our experience a QALY based approach to assessing ultra-orphan drugs, albeit with modifiers, as adopted in Scotland results in negative decisions for reimbursement. This has huge impact for patients and we are aware of patients that have moved to England in order to gain access to drugs such as enzyme replacement therapies.

6. While we would prefer to see the AGNSS MCDA process retained within NICE for the assessment of new ultra-orphan drugs we are keen to see NICE's proposals for assessing such drugs and will engage constructively in the consultation which is expected in 2013–14.

7. There is no universally accepted definition for orphan or ultra-orphan drugs. In the past, NICE has used the definition for an ultra-orphan drug as covering a patient population of 1,000, whereas AGNSS has used the figure of 500. Clarity on this point will be helpful to patient associations, industry and the broader global healthcare community which looks towards NICE as a respected source of authority.

8. The pricing of drugs for multiple indications is an important issue which needs to be actively considered by NICE. While we recognise the practical difficulties this raises for the NHS, the advent of personalised medicine, and the rise in the use of therapies such as monoclonal antibodies, means that many single products will be used for multiple indications, with each indication representing a different value to the NHS.

9. Moving the assessment of ultra-orphan drugs into NICE creates an inherent policy tension with the broader principles of the NHS. Through the use of the QALY, NICE seeks to ensure cost-effectiveness of new treatments and consequently helps deliver the greatest health gain for the NHS's fixed budget. But some academics suggest that devoting resources to the treatment of very rare diseases would mean that there would be fewer resources for the treatment of common conditions and so treatments for very rare diseases should not be funded by the NHS. However, one of the commonly understood NHS principles is equity in access to treatment, which means patients suffering from rare conditions should be entitled to the same opportunity of receiving treatment as other patients with more frequently occurring disorders. Reconciling these policy tensions within NICE will be a significant challenge for its incoming Chairman.

October 2012

Written evidence from Johnson & Johnson (NICE 22)

1. EXECUTIVE SUMMARY

1.1 Johnson & Johnson (J&J) is the world's most broadly-based manufacturer in health care. With significant presence in the pharmaceutical, medical devices and diagnostics and consumer healthcare markets, J&J has potentially the most diversified experience of NICE.¹⁶ With an interest in over 15% of published Technology Appraisals, our interest includes appraisals of pharmaceuticals, medical devices and surgical procedures. We also have a direct interest in many Clinical Guidelines, Public Health Guidance, and now Quality Standards.

1.2 J&J is also a major economic investor in the UK Economy. In a typical year, over 24,000 jobs are supported by J&J in the UK (including 5,700 direct and 18,500 indirect jobs). We invest in 10 facilities in England, two in Scotland, one in Wales; covering Commercial Offices, R&D, Manufacturing and Warehouses/Distribution Centres. In 2010, J&J invested £71 million in R&D and conducted 222 clinical studies, enrolling nearly 16,000 patients at more than 850 UK sites.

1.3 Summary of Issues and recommendations

1.4. We recognise that NICE has evolved significantly under Sir Michael Rawlins leadership, and adapted to the many and varied challenges encountered during that time. Given the next stage evolution of NICE, into an executive non-departmental public body, there are a number of issues and recommendations we request the Committee consider:

1. We believe the construction and operation of a Value-Based Pricing system is fraught with issues, and may be insurmountable. We recommend evolution of the existing system, not revolution, providing a predictable, sustainable environment for all parties in the UK.

¹⁴ Department of Health. A new value-based approach to the pricing of branded medicines—a consultation. December 2010. http://www.dh.gov.uk/en/Consultations/Liveconsultations/DH_122760

¹⁵ Department of Health. A new value-based approach to the pricing of branded medicines: Government response to consultation. September 2011. http://www.dh.gov.uk/en/Consultations/Liveconsultations/DH_122760

¹⁶ The Johnson & Johnson family of companies include: Janssen, Ethicon, Ethicon-Endo Surgery, DePuy Synthes, LifeScan, Ortho Clinical Diagnostics, Crucell, and the Johnson & Johnson Consumer companies.

2. NICE's decision-making to be inclusive beyond cost/QALY considerations, the baseline QALY threshold to evolve over time in line with NHS costs, and decisions to recognise the limitations of the QALY methodology.
3. NICE to have a clearer strategy on non-drug Medical Technologies; be reviewed under one "programme", and address issues of inappropriate application of product specific evidence to a class ("inappropriate evidence genericisation").
4. NICE to be accountable to DH post status-change to executive non-departmental public body, and to be aligned with the objectives of other relevant Government bodies, such as DH and BIS Life Science Strategy which seeks to foster a vibrant Life Science ecosystem to stimulate economic recovery.

2. NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

2.1 NICE's methods have evolved significantly since its inception, and it has tried to adapt its methods to address the changing needs of the environment it is seeking to inform. Given the intended introduction of Value-Based Pricing (VBP), it is timely that a number of methodological and policy issues be addressed. These issues include: An over-reliance on the QALY, resulting in the use of adjustments that highlight its limitations; methodology that does not consider the value of the product delivered over its lifetime (dynamic efficiency); a QALY threshold that has not changed since NICE was created; a lack of clarity on how medical devices will be reviewed by NICE.

2.2 Value Based Pricing

2.3 J&J believes that the current medicines pricing system, the PPRS has delivered a predictable, sustainable environment that provides companies with a number of elements that can be used to support the uptake and adoption of innovation. We believe that a PPRS system, followed by a NICE HTA appraisal has the potential, with some modifications, to provide a framework that can deliver value for money to the NHS, speed the uptake of innovation and make the UK a leader in the adoption of new technologies. The key to making this a success will be reform the current value assessment in the NHS HTA process. J&J believes that the operational and methodological issues involved in the construction of a VBP system are many, and may be ultimately insurmountable. We remain deeply concerned that the proposed VBP system could delay access to medicines in the UK, further erode prices (already amongst the lowest in Europe), will create medium term unpredictability and could cause some medicines not to be launched here at all. We call for a fundamental rethink around VBP. Instead we believe that an evolution of the existing NICE appraisal system, alongside a maintained PPRS agreement, is the most effective option and provides a way forward for all parties. J&J calls for prudent, thoughtful evolution and not a revolution that threatens to destabilise the UK's pricing environment. Furthermore, the methodological challenges associated with the construction of a VBP system are many and varied. As an illustration, any rudimentary evaluation of NICE TAGs shows that economic models tend to be used to make binary "yes/no" type approval decisions. This is because at best they give plausible cost per QALY ranges under different assumptions. Modelling provides an indicative direction of travel rather than a definitive answer, which means agreeing on a single set of assumptions to create a single price will, in many evaluations, be impossible.

2.4 Some discussions surrounding the development of VBP, whilst theoretically logical, miss the realities of the real-world context. It is expected that between 2012 and 2016, branded drugs accounting for approximately £2Bn will go generic.¹⁷ The QALY approach only considers the relative cost-effectiveness during the patent period of a drug, whereas in reality the NHS continues to reap significant value from once-innovative products many years after patent expiry, through the use of generics. This "dynamic efficiency", or lifetime perspective is not considered in the cost-per QALY evaluation.

2.5 An over-reliance on cost per QALY based decision-making—Flawed for use in VBP

2.6 NICE decisions give undue weight to cost per QALY estimates derived through computer simulation modelling. Whilst it has been frequently stated that the QALY is a "tool not a rule", recent policy decisions by NICE would tend to suggest otherwise.

2.7 Cost-effectiveness ratios derived through computer simulation modelling are almost always estimates of what the future cost-effectiveness of the technology might be, rather than robust primary evidence. Despite the uncertainty inherent in simulation models, the answers they produce dominate the decision at the expense of other important factors such as the innovative nature of the technology, the level of unmet need and the disease under consideration.

2.8 The reliance of NICE on the QALY threshold can be deduced by two recent policy "adjustment factors" applied to QALY estimates. First, where the threshold has caused issues with drugs having a short term impact, and the second, where the impact is many years away.

¹⁷ IMS Health MIDAS Market Segmentation MAT December 2011.

2.9 Recognising the £20-£30k per QALY threshold was becoming increasingly difficult to achieve for many new therapies providing near term relief, eg cancer, supplementary advice was issued to the Appraisal Committees in August 2009 on appraising life-extending, end of life treatments.¹⁸ A review of decisions taken under that advice implies a multiplier of around 1.7 appears acceptable to QALY estimates fulfilling the criteria set. Hence, rather than using judgment on a case by case basis, the QALY threshold was effectively adjusted to fit a Cost per QALY-based decision criteria.

2.10 In August 2011 NICE issued clarification on discounting in appraisals, when benefits “*are both substantial in restoring health and sustained over a very long period (normally at least 30 years)*”, indicating that a lower discount rate of 1.5% should be used for benefits in this situation.¹⁹ This has the impact of increasing the benefit (QALY) estimate, and so reducing the “Cost per QALY” estimate. This adjustment was used in the considerations resulting in the recommendation for TA 235 (Osteosarcoma), October 2011.

2.11 These two adjustments, in very different settings, demonstrate that the QALY is the central decision making criteria for NICE appraisals.

2.12 *The QALY Threshold has not changed over time*

2.13 It is difficult to comprehend how the threshold has not changed over time, even though NHS experiences inflation like all other sectors. Estimates are easily produced using the Hospital & Community Health Services (HCHS) index for general NHS inflation, the health service cost index (HSCI), referencing the changing cost of goods, or indeed HM Treasury’s GDP deflator index, which is used to manage reimbursed prices in the Drug Tariff.

2.14 One of the implications of no increases in the “Cost per QALY threshold” is that a technology previously deemed cost-effective could indeed now be deemed not cost-effective, merely because the cost of administration (staff) has increased due to pay inflation. Where benefits are constant and relative costs increase due to inflation, the cost-effectiveness ratio will increase, and hence may tip above the “threshold” over time.

2.15 While the example may be illustrative, it does help explain why not increasing the threshold is counter-intuitive, and why the threshold is becoming increasingly challenging for new innovative technologies to achieve. In real terms, the NHS in 2012 is willing to pay less for new innovations than it was in 2000, and with a static threshold is effectively willing to pay less and less every year.

2.16 *Other limitation of the QALY approach*

2.17 The limitations of the QALY were raised with the HSC at the last review of NICE. Given the interest in moving to VBP, it is worth referencing briefly the key issues here. The QALY cannot readily capture the degree of unmet need, the innovative nature of the technology, or the severity of the underlying condition.

2.18 In conditions where few new treatments have been launched, new entrants into a class must compare against generic treatments which have been on the market for many years, rather than against other branded products. These are often the areas where innovation is most valued, but where achieving acceptable cost per QALYs can be most challenging. Such comparisons present an impossibly unfair hurdle for comparative cost-effectiveness assessments.

2.19 The scope of costs considered in determining the cost per QALY for technologies is still restricted to those incurred by the NHS & PSS. This excludes significant costs that can be incurred by patients and carers.

2.20 We believe that if NICE’s decisions are to become more accessible to the general public, it is imperative that the obsession with predictive computer modelling of potential future benefits be balanced with a more considered view on the wider societal and clinical benefits of the treatment.

2.21 *Future Governance of NICE*

2.22 As NICE moves to an executive non-departmental public body, what will its Governance be, specifically in terms of its accountability to the Department of Health for its role in any future pricing system. We would recommend there be a mechanism by which NICE would still be accountable to the DH in this capacity.

2.23 We would also request the Committee scrutinise how joined-up NICE will be with other Government Departments. For example, NICE is not altogether unrelated to the objectives of the DH and BIS Life Science Strategy which seeks to foster a vibrant Life Science ecosystem to stimulate economic recovery. Does NICE, consider—at all—the impact its decisions may have on the Objectives of the Life Science strategy? These objectives are led by the Prime Minister himself who wants to see a thriving Life Science and Pharmaceutical industry contribute to UK economic recovery.²⁰

2.24 We have also noticed a change in the prevailing “culture” of the NICE appraisal committees towards industry, in that they do not seem to appreciate the model of pharmaceutical innovation, ie risk and reward. Instead there is all too often suspicion which is disconcerting. The risk is this may become exaggerated under

¹⁸ <http://www.nice.org.uk/media/E4A/79/SupplementaryAdviceTACEoL.pdf>

¹⁹ http://www.nice.org.uk/media/955/4F/Clarification_to_section_5.6_of_the_Guide_to_Methods_of_Technology_Appraisals.pdf

²⁰ Speech to the *Financial Times* Conference, 5 December 2011, London.

VBP. In contrast, J&J is impressed by the pro-industry tone taken by the Life Science Strategy and NHS leaders taking part in the Innovation, Health and Wealth “task and finish groups.” There is a shared, equal view that innovative practices and technologies are better for patients, the clinicians and industry. J&J asks why NICE appraisal committees cannot have the same outlook.

2.25 Evaluation of non-pharmaceuticals in the era of VBP

2.26 If VBP for drugs is implemented, it is evident that the differences between NICE’s approach to pharmaceuticals and other medical technologies will only increase. Even now, it is evident that NICE struggles with the evaluation of non-drug technologies. The “Single Evaluation Pathway” for medical technologies is actually a single entry point, which then passes technologies in to one of several different evaluation pathways.

2.27 The MTG programme only reviews products which are net cost saving/neutral, and it is unclear how technologies that are “cost-effective” will continue to be assessed. However, there have been limited evaluations of medical technologies in the TA programme recently, and it is unclear what capacity will exist to develop TA’s for devices once VBP arrives.

2.28 An added complication regarding medical technologies is the conundrum on how evidence is used, both in NICE appraisals and clinical guidelines, where product-specific evidence is taken, either implicitly or explicitly, to support the use of other similar products that do not have a similar evidence base. This “inappropriate genericisation”²¹ of the evidence base is a disincentive to evidence generation, as it incentivises fast-followers to compete on price rather than generate evidence of effectiveness and ensure equivalent value.

3. The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

3.1 We recognise the intention of introducing Quality Standards (QS), and the idea is supported. Our view however is that the first group of standards that have been released are rather broad, difficult to quantify (perhaps lacking a “SMART” approach) and it is unclear what acceptable levels of achievement are in some of the standards. We would like to see QS made more specific such that the commissioning standards required in the given areas are much more specific.

3.2 Given the introduction of QS, the status of other disease areas without QS is unclear: how the NHS should prioritise them, especially in areas of significant unmet need or variation in clinical practice? Of concern to us is the area of Hepatitis C. This is a condition in which huge variation exists and yet no quality standard is planned. In its absence, how will the NCB ensure that outcomes-based, high quality care is delivered in such instances across the whole of the country?

4. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

4.1 When NICE Clinical Guidelines were first introduced, it was expected these would influence the NHS more than individual technology appraisals. However, whilst they clearly have some impact, our experience suggests they are considered aspirational and not consistently implemented across the NHS. This also impacts technology adoption where a TA is incorporated into a guideline. Efforts should be made to ensure Clinical Guidelines are definitive and implemented. The most frustrating scenario is the apparent optional status today.

October 2012

Written evidence from the Ethical Medicines Industry Group (NICE 23)

In response to the Health Select Committee’s call for evidence, the Ethical Medicines Industry Group (EMIG) has prepared the following submission into the work of the National Institute for Health and Clinical Excellence (NICE). Please note that EMIG has only provided a response to the questions for which it is appropriate to do so.

By way of background, EMIG is the pharmaceutical trade association that represents the interests of over 200 companies and allied organisations based in the UK. EMIG member companies range from start-ups to highly developed research-intensive businesses delivering essential products to patients, while continuing to invest heavily in the fight against disease.

EMIG members are commonly, but not exclusively, small to medium-sized enterprises (SMEs) which specialise either in the discovery and development of medicines to treat rare and underserved diseases, or focus their efforts on discrete disease areas, for example diabetes and respiratory disorders. EMIG member companies employ approximately 20,000 people in the UK and have a combined annual turnover of £4 billion.

²¹ Assuming product specific data applies equally to all members of the class of products without any evidence of equivalence.

INTRODUCTION

EMIG welcomes the opportunity to present our views to the Committee on the workings of NICE at what we believe is a watershed moment for healthcare provision in England with the implementation of the reforms set out in the Health and Social Care Act now fully underway.

The NHS is currently undergoing an unprecedented period of reform which will see clinicians take responsibility for much of the NHS budget with clinical-decision making being at the fore of provision. Within this context, NICE will see its role transformed and by April 2013, will become a Non Departmental Public Body, whose remit will expand to include, amongst other things, quality standards for the social care sector. With the commencement of Government-industry negotiations on the future of the Pharmaceutical Price Regulation Scheme (PPRS) and the broader shape of value-based pricing (VBP) imminent; we believe there is a unique window of opportunity for industry to reflect on the workings of the medicines regulator and its future role in supporting the new commissioning environment.

To assess the true impact of the forthcoming changes to the pricing and reimbursement landscape, further clarity is required on the shape of VBP before the impact of the new system on NICE can be truly considered. We look forward to playing a full role in the discussions surrounding the development of VBP and the development of pricing and reimbursement for medicines in the UK. VBP must create an environment to stimulate patient access to medicines, a commitment to pharmaceutical R&D and investment in the UK economy.

EMIG has focused its response to the committee's call for evidence on the major areas of policy where we believe NICE's operations will have most impact on our members. We will touch on the new functions of NICE in public health and social care, however, in the main we are concerned with the impact of NICE's operations on the ability of the small and medium sized pharmaceutical sector to flourish in the UK and the environment within which commercial and investment decisions are made.

Key recommendations:

- Further clarity is required on the shape of VBP before the impact of the new system on NICE can be fully considered.
- To ease the burden on small companies of the Health Technology Assessment (HTA) process EMIG believes there must be a transparent and consistent approach to assessment across the four nations to prevent costly duplication of submissions.
- Within the new NHS framework there should be no additional assessment of medicines with a NICE approval. Clinical commissioners must respect the outcome of decisions made on a national basis and focus on delivering better outcomes for patients based on patient need.
- For rare conditions, currently assessed by the Advisory Group for National Specialised Services (AGNSS), we recommend that the QALY should not be the finite measure of cost-effectiveness and other data should be considered.
- To be an effective and efficient tool for clinicians, Quality Standards must be short, focused and clear on good practice expectations and outcomes that will be delivered for patients.
- For NICE guidelines to continue to be effective within the new NHS, local organisations should be held accountable to their use of guidelines.

1. NICE's role in relation to evaluation of the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

1.1 Fast and appropriate access to innovative medicines is a critical factor in the delivery of good quality health outcomes for patients and efficient use of NHS resources. Moreover, expedient access to medicines and a robust but efficient regulatory process has a direct impact on global commercial decisions around the UK as a location for industry investment in research and development (R&D), clinical trials, and commercial operations.

1.2 We welcome recent commitments made by the Government in *Innovation Health and Wealth: Accelerating adoption and diffusion in the NHS* to reduce variation and strengthen compliance through the NICE compliance regime, the NICE Implementation Collaborative, the Innovation Scorecard and the 90 day implementation window; all of which seek to improve uptake of vital medicines across the NHS.

1.3 In general, EMIG and its member companies enjoy a good working relationship with NICE and cooperate with the appraisal process. However, for some smaller companies the cost and time resource burden of submitting data can be prohibitive. EMIG is particularly concerned that the current system for HTA as conducted by NICE (and the SMC in Scotland) puts small and medium sized companies at a disadvantage and impacts negatively on the low volume, high value medicines produced by some EMIG companies.

1.4 EMIG strongly believes in the importance of establishing a level playing field when assessing new treatments. For small and medium sized companies, preparing submissions under the current HTA structure is time and resource intensive. Results from a survey of our members show that an HTA could cost up to £250,000

or higher for more complex developments. For companies with a turnover of less than £50 million this is a significant burden.

1.5 To ease the burden on small companies of the health technology assessment process, EMIG also believes that there must be a transparent and consistent approach to assessment across the four nations to prevent costly duplication of submissions.

1.6 NICE currently assess only 40% of all new medicines and the remaining 60% therefore become subject to the vagaries of local assessment groups around England. These groups assess medicines in different ways and, despite assessing the same data, arrive at differing conclusions regarding the value of particular medicines. This contributes to postcode prescribing.

1.7 The adoption and diffusion of new medicines across the NHS in England is variable and, in some parts of the country, extremely slow. Not only does this restrict patient access to innovative treatments, it creates significant uncertainty for companies planning to invest in developing new medicines in the UK. It is widely known that bringing a new medicine to market requires huge investment and, if we are going to continue to have high quality R&D in the UK, it is vital that the NHS does not act as a barrier to patients wishing to access appropriate treatments.

1.8 Within the new NHS framework there should be no additional assessment of medicines with a NICE approval. Clinical commissioners must respect the outcome of decisions made on a national basis and focus on delivering better outcomes for patients based on patient need.

1.9 Whilst EMIG is supportive of the proposed role of NICE in assessing value and VBP we are still unsure of the definition of value; how it will be assessed, who will assess it and how value will be measured in terms of clinical use. Moreover we question whether NICE can capture value outside of constraints of the QALY—especially societal benefits, for which there is no definitive agreement on how benefits will be classified and measured.

1.10 As previously stated, NICE currently assesses only 40% of medicines—therefore there is a question over how NICE will resource the timely assessment of all medicines launched after January 2014 that will require assessment under the new principles of VBP. We are concerned that under VBP, the “hurdle” of completing and compiling lengthily health technology assessments could further impact on the long-term viability of low volume, high value medicines produced by EMIG companies—this could lead to the loss of many incremental innovations on these important products.

1.11 Significant issues also remain for those medicines that will not be assessed under VBP. Far more detail is required to understand how the value of many essential, everyday medicines will be captured and harnessed in a system which requires NICE approval to ensure uptake and access within the NHS. For many low cost, high volume products access challenges within the NHS will remain.

1.12 It needs to be confirmed that medicines with a VBP will gain automatic access on NHS formularies (as with products supported by a NICE TAG)—and indeed there are limitations of the current system whereby all medicines with NICE approval must be included in formularies within 90 days of approval.

1.13 We also have concerns around the specialist considerations required for the assessment of orphan therapies (to treat rarer diseases which affect approximately 5 in 10,000 patients) where detailed health economic data may not be readily available.

1.14 NICE will also assume the work of AGNSS from April 2013. However, this raises questions regarding whether NICE will assess all Orphans/Ultra Orphan drugs as there appears there will be a gap in their responsibility. Also, there are inconsistencies in the recognition of Orphan/Ultra Orphan status:

1.14.1 Orphan in the UK—less than 1:10,000.

1.14.2 Ultra Orphan in the UK—1:50,000.

1.14.3 AGNSS—<500 patients in England.

1.14.4 EMA Orphan—less than 5:10,000 and there is no EU Ultra Orphan status.

1.15 We welcome the move within the Health and Social Care Act 2012 for NICE to assess very high cost drugs for people who suffer with rare conditions, which is currently managed by AGNSS. However, we believe that in such circumstances the QALY should not be the finite measure of cost-effectiveness and other data should be considered, such as budget impact analysis and/or multi decision criteria analysis (MCDA) to identify a validated range of patient symptom responses to assess total outcome benefits.

1.16. This is particularly important within the context of value-based pricing. The assessment of orphan and ultra-orphan medicines is extremely difficult due to the small patient populations and lack of comparator treatments. It is vital that a strong degree of pragmatism is therefore built into the new pricing system to ensure patient access to these essential medicines. From an industry perspective, it is imperative that the price paid for Orphan and Ultra-Orphan drugs under VBP should adequately reward the significant investment that is required to develop new therapies, which would otherwise not be available to patients on the NHS.

2. The role of NICE Quality Standards in the new NHS system structure, in particular the status of NICE guidelines in determination of commissioning priorities

2.1 The intention of NICE Quality Standards to drive and measure priority quality improvements within a particular area of care is laudable. However their impact in practical terms is largely untested. Moreover, we have some concerns over the ability of NICE to produce the expected 150 Quality Standards over the next five years. We suggest this is an area that the Committee may wish to explore over the course of the inquiry.

2.2 We welcome the intention for the quality standards to improve the consistency of NHS decision making across the NHS as this remains a major area of concern. Currently the lack of consistency in local decision making regarding the adoption of new medicines at present and further devolved decision making could exacerbate this situation.

2.3 We believe that the link to clinical benefit is the key driver in assessing value to any one patient. National guidance, in the form of Quality Standards, could help to ameliorate variations in local decision making and increase transparency. In cases where NICE has sufficient information on clinical effectiveness, even for those medicines that are not cost effective, this evidence should be utilised. However, this is unlikely to address the issue of those medicines not considered by NICE. In all circumstances commissioning decisions should be clinically led.

2.4 To be an effective and efficient tool for clinicians, Quality Standards must be short, focused and clear on good practice expectations and outcomes that will be delivered for patients.

3. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness

3.1 Much variation remains in the local use of NICE guidelines because they do not have mandatory status. In the main, we are supportive of this variation in that it allows for clinical decision making and effective response to local population health needs.

3.2 The new NICE “Evidence Summaries; New Medicines are an excellent attempt for NICE to address this issue. However, clinicians will not go looking for these and they are difficult to find on the NICE website. NICE needs to be more proactive on this issue and these Summaries potentially affect those medicines that do not undergo NICE assessments because they are low cost/low volume medicines—these, therefore, could offer significant value to the NHS.

3.3 For NICE guidelines to continue to be effective within the new NHS structures we believe that local organisations should be held accountable to their use of guidelines (and indeed Quality Standards).

4. The effect of the new public health system architecture on NICE’s continued role in respect of public health guidance

4.1 In the context of the new health service structure we believe that there will be an important role for NICE in developing guidance in respect of public health to ensure that the NHS, local authorities and other organisations in the public, private, voluntary and community sectors have clear direction on how to plan services in response to local needs.

4.2 We are, however, concerned as to whether NICE will have the resource and expertise to carry out its increasingly expanding role in this area.

5. What effect NICE’s new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

5.1 The significant reforms and intended integration of the health and social care service have further increased NICE’s role with new responsibilities to develop quality standards and other guidance for social care in England. Given that this process is in its embryonic stages we feel it is right to reserve judgement on NICE’s abilities in this area until the current pilot programme for developing social care quality standards are complete and this initial phase of work can be evaluated.

Written evidence from the UK Council for Psychotherapy (NICE 24)

This submission will focus on the important role the National Institute for Health and Clinical Excellence (NICE) plays in determining the availability of psychological therapies to treat mental health problems in the NHS.

The UK Council for Psychotherapy (UKCP) is recognised as the leading professional body for the education, training and accreditation of psychotherapists and psychotherapeutic counsellors. As part of our commitment to protecting the public, we work to improve access to psychotherapy and psychotherapeutic counselling, to support and disseminate research and to improve standards.

SUMMARY

- NICE has played a key role in focusing the medical profession on evidence-based treatments, but has not performed as well when assessing psychological therapies;
- NICE guidelines try to “pick winners” between different types of psychological therapy when instead its focus should be on patient choice and quality of service;
- NICE guidelines adhere to a hierarchy of evidence which, for psychological therapies, over-value randomised controlled trials and undervalue the importance of a range of other qualitative and quantitative evidence types; and
- As a result, many highly effective psychotherapists are not able to practice on the NHS and patient choice of therapy is restricted.

1. *NICE's history*

1.1 At this point of change for NICE, it is useful to briefly recall the circumstances of its birth. The organisation was founded in 1999 with responsibility for publishing national guidance for a range of medical conditions, partly to try and end the “postcode lottery” for NHS treatments. NICE’s early role included making judgements about expensive new drug treatments, particularly cancer drugs. As a new public sector body it had to choose between drugs developed by international pharmaceutical companies and to do so in the face of hostility. This embattled and hostile environment was the one in which NICE was established.

1.2 Since the embattled circumstances of its birth, NICE has developed into an institute recognised around the world. It has succeeded where many thought it would fail and established itself as a key part of the national healthcare system.

1.3 Its role has now evolved following the Health and Social Care Act. Its remit has expanded to include social care and a new and welcome emphasis has been placed on NICE promoting quality. Equally, it still bears the marks of its difficult birth and they remain evident in its approach to mental health.

1.4 Over the last 13 years there has been a systematic expansion in the availability of basic “talking therapies” for the treatment of psychological distress in the NHS. This is an extremely welcome development and NICE has played its part in delivering this important change in the way health policymakers and medical professionals approach mental health.

1.5 Drug treatments do have a role to play in the treatment of psychiatric conditions but it is disturbing that over the last 20 years the number of antidepressant prescriptions in England has increased by over 500% and in 2011 stood at 46.7 million (HC *Hansard*, 2012). This is an unsatisfactory state of affairs and is at odds with a professional and public demand for greater use of non-pharmaceutical approaches.

1.6 NICE has played a crucial role in focussing the medical profession on evidenced treatments. That role must continue. At the same time, UKCP hold the view that while NICE does many things very well its approach to evaluating, comparing and recommending treatments for psychological distress has been too narrow.

1.7 The main way NICE has influenced the availability of psychological therapies in the NHS is through publishing clinical guidelines for specific mental health conditions such as depression, anxiety and schizophrenia.

1.8 NICE, having spent its early years picking the best treatments between different drugs, then approached mental health with the same mindset. Its guidelines have sought to “pick winners” between the different psychological therapies available. So, even though there has been an expansion in the availability of talking therapies in the NHS through the Improving Access to Psychological Therapies (IAPT) programme, it has been on a very narrow model which has predominately meant the delivery of a manualised form of Cognitive Behavioural Therapy. This model works well for some patients but excludes many others models that are beneficial.

1.9 As IAPT has expanded it has rigorously adhered to NICE’s guidelines. These guidelines have had the unintended effect of restricting access to the full range of high-quality, evidenced and effective psychotherapeutic treatments available. As a consequence, NHS patients have been denied meaningful choice in the treatment of their psychological distress.

1.10 The change in leadership at NICE presents an opportunity for the organisation to develop a new approach towards supporting psychological wellbeing. Change will improve patient choice, transform the quality of care and bring meaning to the commitment to parity of esteem between physical and mental health that was introduced into law this year.

2. *Why therapy works*

2.1 Going to therapy is a very personal experience, usually preceded by significant and/or sustained emotional distress. Psychotherapy requires an active engagement on the part of the client in a process which seeks to deliver lasting change in the way that client experiences their lives.

2.2 The interrelationship between client and therapist is built on trust, and the relationship is central to the success of therapy. An investigation into evidence-based therapy relationships by the American Psychological Association (APA, 2011) concluded that: “the therapy relationship makes substantial and consistent contributions to psychotherapy outcome independent of the specific type of treatment”.

2.3 The strength and nature of the therapeutic relationship is shaped by several important factors. These factors include: the length and frequency of therapy, the chronicity of the condition for which a client is seeking psychological support, the social and cultural context which brings both client and therapist into the therapy room and, significantly, the characteristics of both the client and the clinician involved in the therapy. In therapy a client is not the passive recipient of an external treatment. Instead it is helpful to see therapy as a dialogue which requires engagement on both sides to have a successful outcome.

2.4 This approach recognises that to focus on the specific brand of therapy delivered and ignore the importance of the therapeutic relationship fails to appreciate the very essence of therapy. The same APA study (2011) also concluded: “the therapy relationship accounts for why clients improve (or fail to improve) at least as much as the particular treatment method”. In the words of Bohart and House (2008:195): “Therapy “works” through the interrelational presence of the therapist, rather than through specific technological, or skill-based operations.”

3. *The current NICE approach*

3.1 It is our contention that NICE’s current guidelines on mental health treatment both:

- neglects the importance of the therapeutic relationship in promoting psychological wellbeing; and
- places undue emphasis on which brand of therapy should be made available to NHS patients.

3.2 At the moment when NICE develops a new guideline for a particular medical condition it creates a guideline development group which examines available research evidence for the efficacy and cost-effectiveness of treatments for that condition. It is a rigorous process which applies academic standards to ensure that all NICE recommendations are “evidence-based”. And, yet, there are several problems with its approach.

3.3 UKCP does not believe that NICE’s guideline development approach of “picking winners” between different therapeutic models is what is most important. As research shows (Stiles, *et al*, 2008) that different therapy models have almost equally positive outcomes, it does not make sense for NICE to focus so much attention on picking between marginal differences.

3.4 By adopting this approach, NICE guidelines can miss what is important. Outcomes are the key criteria for determining the effectiveness of therapy. To an extent, how that therapy works is less important than what impact it has. NICE’s guidelines (2009:162) on depression illustrate this point. In relation to one study on the effectiveness of psychotherapy it was observed that “it is difficult to determine whether or not the long-term benefits associated with psychodynamic psychotherapy resulted specifically from the therapy or the prolonged contact with the therapist during that time”. NICE’s approach is unsatisfactory (see point 2.4) and sees this as a reason to reject the validity of the findings.

3.5 The allegiance to a rigid hierarchy of evidence in modern therapeutics has been challenged by the current NICE chair, Sir Michael Rawlins. Despite this, a hierarchical approach still governs NICE’s guideline development approach.

3.6 At the summit of the evidence hierarchy sits randomised controlled trials (RCTs) which in a typical example (Rawlins, 2008:2) have a 1++ rating for the strength of their evidence. Nestling in the foothills (to borrow Sir Michael’s phrase) sit other well-designed quantitative and qualitative studies which NICE does not fully value.

3.7 RCTs are one of the best tools that modern science has to establish the efficacy and effectiveness of specific interventions. UKCP do not contest their value in many areas of medical research. However, in the area of psychological distress their limitations are substantial.

3.8 In the words of the current NICE chair (Rawlins, 2008:2), “The notion that evidence can be reliably placed in hierarchies is illusory. Hierarchies place RCTs on an undeserved pedestal for... although the technique has advantages it also has significant disadvantages.”

3.9 Of the disadvantages around RCTs that Sir Michael identified, two are particularly relevant to psychotherapy:

- Generalisability of the results.
- Resource implications.

3.10 RCTs seek to establish the efficacy of a treatment in controlled conditions. It is assumed that these results can be generalised across whole populations who are viewed as essentially uniform. However, in therapy (as in life), the particular factors that underpin the relationship of individual therapist-client pairs are central to the success of psychological therapy. It is extremely hard to design an RCT trial that could capture this.

3.11 After progressive attempts to make RCT methodology unassailable, it has become so prohibitively expensive to run an influential study that the cost is only easily met by the pharmaceutical industry.²² This problem was recognised in the Health Committee's 2008 report on NICE when it stated that "in some areas, without commercial sponsors, notably public health and many physical and psychological therapies, there is little research about the cost-effectiveness of different interventions" (Health Committee, 2008:43). In relation to psychological therapy, this point was not addressed in either NICE's or the Government's response.

3.12 An overreliance on RCT-type evidence has resulted in the approval of manualised treatments over ones that give space to the professional judgement of the therapist.²³ One of the key assumptions of RCT methodology is that treatment can be standardised in order to eliminate the impact of the therapist. To manualise implies that the therapist's role can be encompassed within a set of prescriptive instructions and that there is no significant level of professional judgement involved. This does not correspond to a real world understanding of how therapy delivers change.

3.13 Commenting on the deleterious impacts of hierarchies of evidence, Sir Michael (2008:34) stated that: "Hierarchies attempt to replace judgement with an over-simplistic—pseudo-quantitative—approach to the assessment of the totality of the available evidence. Decision-makers need to incorporate judgement as part of their appraisal of the evidence in reaching their conclusions. Hierarchies should be replaced by embracing a pluralistic approach to evidence".

3.14 In America, this pluralistic approach to evidence has embraced psychotherapy as a highly effective, evidenced and cost-effective treatment for a wide variety of mental health conditions. The APA is the world's largest association of psychologists. Its *Resolution on the Recognition of Psychotherapy Effectiveness* is an unequivocal statement of the value of psychotherapy. It concludes:

As a healing practice and professional service, psychotherapy is effective and highly cost-effective. In controlled trials and in clinical practice, psychotherapy results in benefits that markedly exceed those experienced by individuals who need mental health services but do not receive psychotherapy. Consequently, psychotherapy should be included in the health care system as an established evidence-based practice (APA, 2012).

3.15 The APA has recognised that psychotherapy is an evidence-based and effective treatment and is in many instances preferable to pharmacological treatment. It is time for NICE to do the same.

4. A way forward

4.1 NICE guidelines currently focus on approving specific psychological treatments for use in the NHS. When NICE looks at mental health services again it needs to recognise that the brand of therapy is not the most important aspect determining quality. Instead NICE should embrace a more pluralist approach which acknowledges the quality of a range of evidence types for the effectiveness of psychotherapy.

4.2 NICE has a critical role to play in the healthcare system. From the difficult circumstances of its birth it has secured a vital position in promoting evidence-based and cost-effective treatments in the NHS. But, when it comes to mental health, NICE must move away from the focus on "picking winners". The evidence is there for the effectiveness of psychotherapy, but an outdated commitment to rigid evidence hierarchies is getting in the way. This is excluding high-quality treatments and therapists from playing a greater part in improving the psychological wellbeing of our society.

4.3 UKCP would like the committee to take this opportunity to ask the prospective chair to review how NICE works in relation to mental health.

²² Sir Michael Rawlins (2008) estimated that the average cost of an RCT was now £3.2 million.

²³ This pattern is captured in NICE's latest guideline for depression (NICE, 2009:90).

SUGGESTED QUESTIONS

Q. Does the prospective chair agree with the outgoing chair that hierarchies of evidence attempt to replace judgement with overly simplistic approaches and it is necessary, especially when dealing with psychological therapies, for NICE to adopt a more pluralistic approach to the evidence base around effective treatments in relation to mental health?

Q. Is the prospective new chair prepared to review the way in which NICE assesses the effectiveness of psychological therapies in developing guidelines on mental health treatment?

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October 2012

Written evidence from Deltex Medical (NICE 25)

SUMMARY

- This is a timely inquiry not just ahead of the retirement of NICE’s Chair but also following the *Innovation, Health and Wealth* (IHW) report and Government emphasis on NHS compliance with NICE guidance.
- Deltex Medical’s CardioQ-ODM was one of the first devices to undergo evaluation under the NICE Medical Technology Evaluation Programme. We have concerns in three main areas, as follows.
- We feel the mechanisms in place to support implementation of NICE Medical Technology Guidance are insufficient. We also regret that guidance on medical devices carries less weight under the NHS Constitution than guidance on pharmaceuticals. Finally, we have concerns about the way the Department of Health is implementing IHW without due regard for NICE evidence.
- Throughout this submission we make recommendations about ways to strengthen the current policy framework around NICE. Separately, we have identified the implementation of the IHW commitment to roll-out “ODM or similar” fluid management monitoring as one worrying example of the disconnect between NICE evidence and NHS implementation.

1. Introduction

1.1 Deltex Medical welcomes the opportunity to respond to the Committee’s inquiry into NICE. The inquiry comes at an important moment for NICE—not just ahead of the departure of its current Chair, but also following the publication of the Department of Health’s *Innovation, Health and Wealth* report (December 2011), which established a “NICE compliance regime” for the NHS in England.

1.2 We approach this inquiry from the perspective of a small British medical technology company that has first-hand experience of undergoing a NICE evaluation. This consultation submission sets out our proposals to strengthen the application of NICE Medical Technology Guidance. We also take *Innovation, Health and Wealth* as a case study of where compliance with NICE evidence could be tightened.

1.3 The benefits of improved compliance would be wide-spread, with the opportunities to improve NHS efficiency, deliver better patient outcomes and support an innovative life sciences sector through greater use of clinically and cost effective innovations.

1.4 We would be happy to answer any questions the Committee may have and would welcome an opportunity to give oral evidence should any such sessions take place.

2. NICE and the CardioQ-Oesophageal Doppler Monitor (ODM)

2.1 Deltex Medical is a small British medical technology company that pioneered Oesophageal Doppler Monitoring (ODM). Our CardioQ-ODM provides an innovative solution to fluid management in surgery and critical care, enabling clinicians to accurately and safely optimise blood flow. The CardioQ-ODM was one of the first medical devices to undergo evaluation under NICE's Medical Technology Evaluation Programme. Guidance published in March 2011 (MTG3)²⁴ recommended the CardioQ-ODM for use in major and high-risk NHS surgery and found that use of ODM saved on average two bed days and around £1,100 for every relevant surgical patient. With a potential patient population in excess of 800,000, NICE figures suggest that the NHS in England could save over £800 million each year.

2.2 ODM was subsequently cited as an innovation case study in the NHS Operating Framework and prioritised as one of six high-impact innovations in the NHS innovation review, *Innovation, Health and Wealth*. Under a reformed CQUIN payment scheme, there will in future be financial penalties for hospital trusts that fail to adopt fluid management technology. To support procurement, the CardioQ-ODM was awarded the tender by NHS Supply Chain for cardiac output monitoring. The Department of Health has also sponsored the Enhanced Recovery Partnership's 2012 guide²⁵ to implementing enhanced recovery programmes for major surgery in the NHS. This emphasises the importance of intra-operative fluid management and ODM as a modern best practice approach to surgery.

2.3 We understand that this level of endorsement represents a high level of support for a medical device in the NHS. However, we have concerns about the way in which NICE guidance has been interpreted and the levers in place to support implementation. These are:

- NICE guidance on medical technologies is not treated as equivalent to pharmaceutical products;
- Mechanisms for implementing NICE Medical Technology Guidance could be stronger to support innovation uptake; and
- Department of Health plans to implement "ODM or similar fluid management" technologies in the NHS risks diluting NICE evidence.

3. Medical technology under the NHS Constitution

3.1 The Department of Health's draft Mandate to the NHS Commissioning Board requires it to "promote access to clinically appropriate drugs and technologies recommended by NICE, in line with the NHS Constitution". However, at present a patient's right under the NHS Constitution to be treated with NICE-recommended technologies covers only medicines assessed under NICE's Health Technology Appraisal programme. This excludes those recommended under the Medical Technology Evaluation Pathway.²⁶ As a result, there are weak incentives to adopt money-saving technologies, and the bar for adoption of medical devices is set much higher than for pharmaceuticals even though the clinical and economic benefits of some medical devices are far greater than those of the vast majority of pharmaceuticals.

3.2 Medical technologies that have undergone a robust NICE evaluation should not be relegated unfairly to a second class status. NICE has successfully evolved a medical technology function since its inception, and this should be upheld by a supportive policy framework. To assist the adoption and use of innovative, money-saving devices, medical technologies should be given the same status as medicines in the NHS Constitution, with mandatory funding for NICE-recommended technologies providing an incentive to procure those that will improve NHS efficiency.

3.3 The same clinical evidence leading to a recommendation under NICE's Medical Technology Evaluation Programme would often also support a recommendation under NICE's Health Technology Appraisal programme, yet the evaluation pathway used to assess the evidence base materially alters Government's requirements for NHS implementation. In the case of ODM, in 2008 the National Institute for Healthcare Research reported significant benefit from ODM when evaluated under a Quality Adjusted Life Year Basis and this finding would underpin a NICE Health Technology Appraisal recommendation and inclusion within the NICE compliance regime.²⁷ Furthermore there is highly compelling evidence that the avoidance of post-operative complications, a proven benefit of ODM, has a profound effect on patients' long term survival and quality of life.²⁸

²⁴ NICE (March 2011). CardioQ-ODM (MTG3): <http://guidance.nice.org.uk/MTG3>

²⁵ NHS Enhanced Recovery Partnership. *Fulfilling the Potential: A Better journey for patients and a better deal for the NHS*: http://www.improvement.nhs.uk/documents/er_better_journey.pdf

²⁶ Assessed under the NICE Medical Technologies Advisory Committee (MTAC).

²⁷ <http://www.hta.ac.uk/execsumm/summ1307.shtml>

²⁸ Khuri, S F *et al.* "Determinants of long-term survival after major surgery and the adverse effect of postoperative complications". *Ann Surg* 2005; 242(3):326-41.

4. Mechanisms for implementing NICE Medical Technology Guidance should be strengthened to tackle low and slow uptake of innovation

4.1 In the 18 months between publication of the NICE guidance and 30 September 2012, the number of CardioQ-ODM monitors in NHS operating theatres increased by 97 (31%). We note that the NHS has only purchased 16 of these monitors and that the installation of the other 81 has had to be financed by Deltex Medical. It is astonishing to us that the NHS seems to expect a small company to fund a change programme that is being promoted nationally by the Department of Health and potentially saves the NHS over £800 million a year.

4.2 NICE estimated that NHS hospitals would realise a full return on their investment in CardioQ-ODM after just 10 days, but despite their recommendation we continue to find the NHS response lacking. A number of steps could be taken to address this situation, and we are now awaiting a Department of Health implementation plan for ODM and the other high-impact innovations set out in IHW.

4.3 In line with the Department of Health's plans for a "NICE compliance regime", we recommend that Trusts be required to publish implementation plans within a fixed time period (eg 90 days) of the publication of NICE Medical Technology Guidance. Subsequently, they should monitor progress to be published in a transparent manner. Performance could then be scrutinised and benchmarked.

4.4 Clinicians and NHS managers must feel confident about the range of devices that could make more efficient use of NHS resources. Professional associations should therefore be encouraged to provide and promulgate advice and guidance to their members on the use of NICE-recommended technologies. Surgical "checklists" should be widely promoted to ensure all patients receive a recommended standard of care. The checklist model would incentivise purchase and use of relevant equipment as well as facilitating audit. We would like to see his approach promoted by the Committee as a means of strengthening NICE's role.

4.5 In our case, the checklist would ensure that clinicians are incentivised to use ODM on surgical patients, in line with NICE guidance. There may be questions as to whether this checklist approach impedes clinical autonomy. To improve monitoring of uptake while maintaining clinical freedom, we recommend that doctors be required either to tick a box on patients' notes to demonstrate that they have managed fluids in line with NICE guidance, or else to explain in full their decision to adopt an alternative approach. The compliance element of such a "comply or explain" model, using patient notes, has been implemented successfully in NHS London to allow an audit of compliance with local fluid management CQUIN targets.

4.6 NHS Supply Chain should play an important role in streamlining the procurement of NICE-recommended technology. Our CardioQ-ODM monitor is listed by Supply Chain for cardiac output monitoring, and we see this as an opportunity to accelerate the implementation process. However, we note that ODM devices would be listed alongside devices without equivalent levels of evidence. Clinician autonomy is important, but public bodies also have a responsibility to highlight the underlying evidence base for NHS decisions, especially where there is NICE guidance. NHS Supply Chain should therefore have a duty to prioritise and promote NICE-backed technologies. This could include a circular letter to all Chief Executives and procurement leads notifying them of new NICE Medical Technology guidance and options to purchase through Supply Chain.

5. Department of Health plans to implement "ODM or similar fluid management" technologies in the NHS risks diluting NICE evidence

5.1 The Department of Health and NHS senior leadership have prioritised fluid management for rapid NHS adoption and cited "ODM or similar fluid management monitoring" as one of six high-impact innovations.²⁹ Sir David Nicholson, Chief Executive of the NHS Commissioning Board, writing in the Foreword to *Innovation, Health and Wealth*, described the opportunities arising from improved fluid management as "tantalising" and says that 800,000 patients could benefit. This 800,000 figure derives directly from the NICE evaluation¹ of our CardioQ-ODM device.

5.2 At first glance, then, IHW would seem to be a DH-led effort to support implementation of the relevant NICE guidance on ODM. However, we have serious doubts about the pace, scale and evidence base used to support the IHW implementation plan. We in fact believe that the current DH approach is a dilution of NICE evidence.

5.3 We have three concerns about the DH approach to meeting its IHW commitment on "ODM or similar fluid management monitoring technologies":

- the scale is un-ambitious;
- the pace is too slow; and
- the NICE evidence base has been diluted.

5.4 Although the Foreword to IHW noted that 800,000 patients could benefit from fluid management, the Technology Adoption Pack³⁰ (TAP) published to support the implementation programme identified just 77,560

²⁹ Innovation, Health and Wealth: Accelerating adoption and diffusion in the NHS, P26 http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_134597.pdf

³⁰ IOFM Technology Adoption Pack, 08/05/2012, http://www.ntac.nhs.uk/NewsAndEvents/IOFM_Technology_Adoption_Pack_Published.aspx

patients a year in the NHS “against which the CQUIN pre-qualification is most likely to be measured”. The scale of ambition is therefore disappointingly low, at just 10% of the potential envisaged by NICE and the IHW report. The pace is also too slow, with no sight of an implementation plan for the high-impact innovations almost a year since the launch of IHW, contrary to the timeline proposed in the original report which committed to launch a national drive on ODM within three months.

5.5 This is a perfect example of NICE evidence being diluted at the point of implementation. For our part, we are ready to increase rapidly our manufacturing capacity and we are committed to providing free training and implementation to support the NHS. We would urge the Committee to scrutinise the interactions between the NICE team behind the CardioQ-ODM guidance and the NTAC team that produced the Technology Adoption Pack to determine:

- What evidence base was used to arrive at the 77,560 patient figure;
- Why the figure is so much lower than the NICE estimate of 800,000;
- Whether there are ambitions to meet the upper estimate in the medium or long term—and the proposed pace; and
- What conversations took place with NICE in developing the Technology Adoption Pack.

5.6 *NICE evidence and IHW*: While the NICE guidance on intra-operative fluid management relates specifically to the CardioQ and the use of ODM, IHW took a more generic approach to supporting “ODM or similar fluid management monitoring technologies”. We are unconvinced by the use of the word “similar” in IHW. Minimally-invasive Doppler probes operate in a fundamentally different way to other invasive (often catheter-based) fluid monitoring techniques. There is an important point of evidence here that IHW should have recognised. Regrettably, the generic “or similar” language was replicated in the Technology Adoption Pack and manufacturers were not invited to review the Pack in draft. As a result, it contains material errors and omissions and treats a number of technologies as if they were similar to ODM despite evidence to the contrary. Deltex Medical sent comments on the Pack to the National Technology Adoption Centre (NTAC) on 24 May 2012, but no corrections have yet been made. In our view, the entire approach to supporting fluid management in IHW has been contrary to the available NICE evidence. We see this as a further example of the NICE evidence base being diluted and disregarded.

5.7 We understand the Department’s hesitation at being seen to promote individual products (the CardioQ). However, in recommending ODM, IHW is recommending a *class* of fluid monitoring technology, not an individual product. Yet the generic “similar fluid management” language allows technologies without *any* NICE evaluation to claim an equivalent status—in effect piggy-backing on NICE guidance without any of the supporting claims for clinical and cost benefits. This kind of loose wording would never be made in, for example, the setting of pharmaceuticals.

5.8 More worryingly, the dis-similar fluid management technologies could cost the NHS more in financial terms—without any proven clinical or cost benefits, they could in fact cost more when used on patients while at the same time allowing a Trust to pre-qualify for lucrative CQUIN payments. We note, for example, that the TAP promotes at least one technology which has been submitted to the NICE Medical Technology Evaluation Programme but was determined to have insufficient evidence to be evaluated. Furthermore, five of the eight listed alternatives in the TAP use a technology where NICE did compare evidence of outcome improvement to CardioQ-ODM and concluded that ODM was “dominant” as a result of delivering both better outcomes and lower costs. It is perverse that such material competitive advantages as delivering better care, lower cost and being the only technology with either a robust evidence base or a NICE recommendation should be arbitrarily discounted.

5.9 We are not asking for a Government endorsement of our product. Rather, we are asking for an explicit acknowledgement of the evidence base and the guidance published by NICE. The definition of “ODM or similar” should be evidence-based and made explicitly clear. NICE could have a role in determining what “ODM or similar” means in any future IHW implementation plan. We recommend that all suppliers wishing to remain listed in the Intra-Operative Fluid Management Technology Adoption Pack be required to notify NICE of their technology and undergo an evaluation. Under NICE’s Medical Technology Evaluation Pathway, it would be possible for NICE to decide within weeks which technologies, if any, were “similar” to ODM. Final guidance on those could be available within 10 months.³¹

6. CONCLUSION

6.1 UK policy on the evaluation of health technologies leads the world and NICE is by far the most respected and most influential body of its type. The Committee now has a timely opportunity to scrutinise NICE’s activities, to consider whether the supporting policy framework is fit for purpose, and to probe the wider NHS relationships that ultimately determine whether or not NICE guidance is effective.

6.2 As set out in this submission, we feel that the policy framework supporting implementation of NICE Medical Technology Guidance could and should be strengthened. The benefits will be many and varied: NICE

³¹ Deltex Medical, Interim results for the 6 months ended 30 June 2010, <http://www.deltexmedical.com/downloads/interim2010.pdf>.

evidence trusted and followed, leading to better quality patient care, improved NHS efficiency, and a world-leading platform from which to grow the UK life sciences.

October 2012

Written evidence from the Association for Family Therapy and Systemic Practice (NICE 26)

1. SUMMARY

- The extension of NICE’s remit is a welcome opportunity to establish a wider perspective of its roles and procedures.
- Until now NICE has applied the processes it developed for medical treatments to all of its tasks, which many feel limits its usefulness.
- There is usually no clear evidence for superiority of one psychotherapy over other established treatments, NICE should change from rejecting all but one, to recommending against any treatments that are demonstrably less effective or more damaging.
- We strongly urge NICE to attend to the ways its advice is used in practice.
- Conclusions.

2. THE EXTENSION OF NICE’S REMIT IS A WELCOME OPPORTUNITY TO ESTABLISH A WIDER PERSPECTIVE OF ITS ROLES AND PROCEDURES

2.1 We enthusiastically welcome the extension of NICE’s remit because our experience and frameworks of thinking point to a continuity from the psychological distress of individuals, through to the whole issue of social welfare. So an authoritative body that gives guidance across this continuum, as we hope NICE will become, will be of great value. We³² are writing from 50 years’ experience of our 2,000 members gained from intensive working with families in all conditions, and knowing that the provision of therapy for families has been severely damaged because the variety and flexibility of the work cannot be captured by research that fits the narrow criteria currently enforced by NICE. Because of the deserved status that NICE has achieved in promoting evidence based medicine, the damage extends beyond the UK NHS to other contexts of practice.

2.2 The extension of its remit to cover the integration of health and social care services creates a unique opportunity for NICE, and for the authorities that will specify its work, to step back from its current core concern with diagnosed physical medical conditions and their treatments, and specify its methods so as to fulfil a wider objective of the well-being of the population. However, we anticipate that the core business of NICE will continue to be the recommendation of medical treatments within the NHS, and for it to have credibility we assume that the new Chair will have to be closely associated with the medical professions. Our submission is written to support a proposal that the person be selected to have a willingness and commitment, to creating new perspectives and procedures within NICE that will give proper attention to this wider brief; preferably having already started thinking about what would be required. The requirements being first, to start from an explication of the research question of what constitutes evidence based psychological practice (APA, 2006); and then to think through the implications of how the existing NICE apparatus could be adapted to generate research-based guidance.

2.3 We feel we have to urge a significant re-conceptualisation of the operation of NICE because our experience over the past decade has not been of a willingness to adjust the processes based in its medical origins to cope with the realities that systemic family therapists encounter every day. We point to recent examples which we hope will be seen as designed to invite the decision process to the sense of urgency we have that this new opportunity be taken forward in a way that equips NICE to make its full contribution to the wider needs. For this to succeed, we feel that NICE needs to extend its assumptions beyond the dominant medical model.

3. UNTIL NOW NICE HAS APPLIED THE PROCESSES IT DEVELOPED FOR MEDICAL TREATMENTS TO ALL OF ITS TASKS

3.1 A clear example: In its current consultation about the formation of Guideline Development Groups (NICE, 2012), every single example in its 154 pages of how it needs GDGs to function is of medical conditions for which there is a clear diagnosis and an intervention that can be rigorously defined and delivered in a standard form for the purposes of research. There is no questioning by NICE of whether these processes are applicable to all of the areas for which it is responsible, or consideration of which research methods are most appropriate for psychological conditions. Indeed, Craig *et al* (2008) raised these questions for the Medical Research Council in relation to developing and evaluating complex interventions which take into account the “difficulty of standardising the design and delivery of the interventions, their sensitivity to features of the local context, the organisational and logistical difficulty of applying experimental methods to service or policy change, and the length and complexity of the causal chains linking intervention with outcome”. It is therefore crucial to consider what are the appropriate research questions and studies for effective intervention for psychological disturbance that arises in the context of family relationships.

³² Please checkout our website for descriptions of Systemic Couples and Family Therapy: www.aft.co.uk

3.2 The single-minded use of medical examples allows NICE to claim unequivocally in all its documents that “the best study design is an RCT” (randomised control trial). But this design is extremely difficult to apply to many of the psychiatric/psychological conditions which the psychological therapies have been developed to treat, especially because of the limitations of diagnosis (APA, 2012).

3.3 NICE’s role has been to recommend the best available cure, which works well in medicine. We wish for a wider perspective which is to use the full range of research so patients receive the best possible support and treatment. As the brief for NICE has extended to mental health, and now to social care, it has become increasingly obvious that a constricted focus on “the best available cure” (based entirely on RCTs) will create a very poor fit to the needs of the population.

3.4 The people of this country badly need NICE to look beyond what can be achieved by matching defined treatments for individual patients to diagnoses of their illness. Within the GDG proposals, under “Guidance by type” (NICE, 2012 Section 1.1, p 11) is the clear statement that: “*NICE clinical guidelines make recommendations to the NHS on treating and caring for people with specific diseases and conditions.*” And Section 4.3.2, p 48: “*A diagnostic test is a means of determining whether a patient has a particular condition (disease, stage of disease or subtype of disease).*”

3.5 This is in fact a fair representation of how it has worked until now, but with increasing loss of credibility in areas such as family therapy. How should a therapist diagnose and then choose a treatment for a patient who is violent in the home with a history of depression and current addiction to drugs? Especially when it is their child who has been referred for their anxiety and “school refusal”. How could any approach be useful without recruiting the whole family into the treatment process? This is why there is an urgent need for NICE to broaden its perspective on evidence based practice in psychology problems (APA 2006).

4. WHEN ALL ESTABLISHED TREATMENTS ARE EQUALLY EFFECTIVE, NICE SHOULD CONCENTRATE ON IDENTIFYING THOSE THAT ARE DEMONSTRABLY LESS EFFECTIVE

4.1 Research tells us that “the general or average effects of psychotherapy are widely accepted to be significant and large” and “are quite constant across most diagnostic conditions” (APA, 2012). Patient characteristics such as social support, chronicity, and clinician and context factors, have been found to more heavily influence variations in outcome than diagnoses or different therapeutic approaches (APA, 2012). More specifically, research has most often found little difference in the effects of different types of therapy and that contextual and relationship factors often mediate or moderate outcomes.

4.2 On the basis of its extensive review the APA concludes that “*most valid and structured psychotherapies are roughly equivalent in effectiveness and patient and therapist characteristics, which are not usually captured by a patient’s diagnoses or by the therapists’ use of a specific psychotherapy, affect the results.*” This well researched conclusion would point to a different opportunity for NICE reviews. One could start, as at present, by searching for clear evidence that there is one form of therapy that is so superior to all others that it can be universally recommended. But such cases are very rare. Then, the well structured and developed therapies could each be examined to determine whether there is evidence that it is less effective or cost-effective than others. In the absence of such evidence, it would be included in current provision. An advantage of this orientation derives from another strand of research which shows that effective therapists regularly draw on two or more models of therapy in order to adapt their psychotherapy to the particular needs of each client. So we need a Chair who will guide NICE towards the reality that it should accept the wisdom that has grown up during a century of practice of psychological treatment and social care, and concentrate its efforts on identifying and excluding approaches where there is evidence that they are less effective than others.

5. WE STRONGLY URGE NICE TO ATTEND TO THE WAYS ITS ADVICE IS USED IN PRACTICE

5.1 Beyond the procedures by which NICE informs its judgements is the way that the status of the advice is presented. NICE has had to push hard in medicine to have its guidance seen as definitive and to establish that good practice consists in following this guidance. Unfortunately this successfully created image has carried over to areas such as psychological disturbance arising within the context of peoples’ relationships, in which the idea of there being a definitive recommendation of a single treatment is entirely inappropriate. In practice, the detail of NICE guidance in these areas does contain useful caveats, but these are routinely ignored especially when commissioners of services feel that they will avoid question and criticism if they strictly follow NICE summary guidelines.

5.2 It is the way that people implement NICE that has resulted in the current UK destruction of patient choice of psychotherapy. This situation contrasts with USA managed care in which Family Therapy is 14% of all treatments, and the most cost-effective (Crane & Payne, 2011). Until now, despite our pleas, NICE has not felt it had responsibility to adapt the way it phrases its advice in order to avoid these consequences. So agencies like IAPT take the summary guidance as an instruction to exclude any approach to therapy not explicitly recommended. As NICE (2012) recognises, “*Many people read only the recommendations*” (9.3, p.113). Some statements by NICE are designed to prevent any provision that is not specifically recommended. For example that therapists providing any other therapy should warn every patient that they are using an approach that is not supported by evidence, and NICE 2012, 9.2, p 113 “*If evidence of effectiveness is either lacking or too weak for reasonable conclusions to be reached, the GDG may recommend that particular interventions are*

used within the NHS only in the context of research”. So we are urging attention to actual consequences, not just good intentions. We therefore request that one question posed to potential Chairs is how they view the ways that NICE guidance has been used to restrict psychotherapies and whether they see a way that NICE could in future present its advice in ways that avoid such damaging consequences.

5.3 NICE already nods towards patient choice and the possibility of comparable effectiveness: “For all recommendations, a general principle of NICE clinical guidelines is that patients should be informed of their choices and be involved in decisions about their care... There might be little evidence of differences in cost effectiveness between drugs within a class, and the clinician and patient might choose between these drugs on the basis of side-effect profile.” (Guidelines Manual Consultation section 9.1, p 109–110.) If this criterion was expanded to the needs of psychotherapeutic treatments and then implemented, it would be found that in almost all cases there is “little evidence of differences” (APA 2012) so all should be made available to patients. But of course patients will not be able to choose any of the treatments that are being eliminated from the NHS through the current rigid interpretation, for example in IAPT, of NICE summary recommendations.

5.4 Effective mental health and social welfare provision should measure outcomes, not whether a specific procedure has been applied. This is the shift from evidence-based treatment to practice-based evidence, and has empirically been shown to increase effectiveness; providing guidance when little difference in effectiveness of particular treatments is to be found. Until now NICE has not accepted practice based evidence such as that generated by CORE (Barkham *et al* 2006). If NICE could be persuaded to place particular emphasis on the admittedly less “pure” evidence generated in everyday practice, practitioners would be encouraged to monitor their own effectiveness thereby enhancing both practice and the research base.

6. CONCLUSIONS

6.1 The UK population need NICE to stand back from its focus on the purity of particular research procedures, so that it can take fuller account of what makes a difference in mental health and social welfare. Sir Michael Rawlins (2008) talked in detail of the necessity of using different forms of evidence in NICE. We would like the new Chair to be asked whether she/he plans to take forward their predecessors intention.

6.2 We are hoping the development of NICE will be undertaken so as to incorporate current valuable Government commitments to wellbeing and quality of life and use the full spectrum of available empirical research. Major advances in research processes to evaluate such objectives are already available (Fujiwara & Campbell, 2011; ONS 2012). Practice-based evidence of outcome focused services will underpin the funding of services in proportion to their achievements for clients rather than the delivery of a prescribed treatment. Until now service commissioners have tried to impose the NICE procedures and criteria that were successfully developed for physical medicine, on each new area. It will take a strong captain, with a clearly articulated commitment, to steer this tanker into a different direction within the necessary time scale.

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Written evidence from the British Heart Foundation and the British Cardiovascular Society (NICE 27)

1. INTRODUCTION

1.1 The British Heart Foundation (BHF) is the nation's leading heart charity. Our vision is of a world in which no one dies prematurely of heart disease. We fund pioneering research into the causes and treatment of heart disease, provide a range of support to people living with heart disease and campaign for change. The British Cardiovascular Society (BCS) supports and represents those working in cardiovascular care and research and sets standards of clinical excellence for the benefit of patients.

1.2 We welcome the opportunity to respond to this inquiry. Our comments are restricted to those areas where we have most expertise. In summary:

- NICE plays a valuable role in evaluating the effectiveness of clinical interventions.
- There is a lack of clarity over the status of quality standards and how they are being used to inform the development of the Commissioning Outcomes Framework.
- Further efforts are needed to ensure NICE guidance is fully implemented across the NHS.
- The new public health players need to champion NICE public health guidance.

2. NICE'S ROLE IN RELATION TO EVALUATING EFFECTIVENESS AND COST-EFFECTIVENESS OF DRUGS AND OTHER CLINICAL INTERVENTIONS

2.1 We believe that NICE has played a valuable role in evaluating the effectiveness of clinical interventions and indeed, the work of the Institute is viewed as an example of best practice internationally. We value the work that NICE has done in comprehensively and consistently assessing new interventions as they become available. However, in some cases the guidance could be clearer around what is clinically effective and what is cost-effective given that these factors can change independently of each other.

2.2 We commend the approach that NICE has taken to stakeholder engagement with strong use of open consultation processes and efforts to involve patients as far as possible. Both the BHF and the BCS have responded to numerous relevant consultations and have received constructive feedback from NICE about how our comments have been taken into account. The BHF has also been able to offer opportunities for people living with heart disease to become involved with NICE's work. In some cases, it may have been useful for NICE to engage with professional societies like the BCS earlier in the process, for example in identifying experts to advise on new technologies and care pathways, rather than only at the point of consultation.

3. THE ROLE OF NICE QUALITY STANDARDS IN THE NEW SYSTEM ARCHITECTURE, IN PARTICULAR THE STATUS OF NICE GUIDELINES IN DETERMINATION OF COMMISSIONING PRIORITIES

3.1 In our experience, there is a lack of clarity over the status and purpose of quality standards. There also seems to be a disconnect between development of these standards and engagement with clinical practice. Professional societies such as the BCS could play an important role in closing this gap.

3.2 In particular, NICE has stated that quality standards will inform the development of Commissioning Outcomes Framework indicators³³ but this does not appear to be the case for cardiac indicators. There has been a general paucity of cardiac indicators in the proposed COF list for 2013–14 but even those that are included do not reflect the existing quality standards on chronic heart failure and stable angina. The BHF and the BCS are working with NICE and the Department of Health to address the lack of cardiac indicators to ensure that tackling heart disease is given sufficient priority by commissioners.

3.3 NICE is also working through a substantial list of proposed quality standards. While the National Quality Board has a role in prioritising the proposed standards there is a danger that this workload is unwieldy. The lag that will inevitably occur in developing these standards could therefore have a knock-on effect on the development of indicators in the Commissioning Outcomes Framework and the decisions made by commissioners more generally.

3.4 For example, one of the proposed quality standards in NICE's library of topics is secondary prevention of myocardial infarction and cardiac rehabilitation. There is no timescale for when this quality standard will be developed. The discussions of the Commissioning Outcomes Framework Advisory Committee concluded that an indicator on cardiac rehabilitation could be usefully included in the framework but as there is no existing standard no indicators have been proposed for this area.

4. THE CONTINUING ROLE OF NICE CLINICAL GUIDELINES IN IMPROVING THE QUALITY OF HEALTHCARE

4.1 While NICE guidance is helpful in setting parameters and consistent use of clinical interventions, NICE has limited power to get this guidance fully implemented in the NHS. We welcome the efforts that the Government has recently made to ensure that all NICE approved drugs are available to everyone who needs them through the NICE compliance regime for local formularies.³⁴

³³ <http://www.nice.org.uk/aboutnice/cof/cof.jsp>

³⁴ <http://www.dh.gov.uk/health/2012/08/nice-technology-appraisals/>

4.2 Although this may improve consistent implementation of guidance on technologies, this will not affect implementation of guidance relating to other interventions. For example, the guidance on chest pain of recent onset published by NICE in 2010 remains largely aspirational. While the changes that it proposes cannot happen overnight, NICE and the DH should be supporting relevant parts of the NHS to make this a reality.

5. THE EFFECT OF THE NEW PUBLIC HEALTH SYSTEM ARCHITECTURE ON NICE'S CONTINUED ROLE IN RESPECT OF PUBLIC HEALTH GUIDANCE

5.1 Given the difficulties that NICE has had in getting universal adoption of clinical guidance across the NHS, it is difficult to envisage how they can ensure consistent implementation of public health guidance when the levers to achieve this reside largely outside of the NHS. More clarity is needed on how Public Health England, local authority directors of public health and other key players in the new public health system will adhere to, and champion, NICE public health guidance. Organisations such as the BHF and the BCS could also have a role to play in publicising relevant NICE public health guidance, for example on the prevention of cardiovascular disease or reducing the harm caused by smoking.

October 2012

Written evidence from Bayer HealthCare (NICE 28)

1. EXECUTIVE SUMMARY

1.1 Bayer HealthCare welcomes the opportunity to provide information to the Health Committee in relation to its inquiry into NICE. Bayer is one of the ten largest specialty pharmaceutical companies in the world, and market our products in more than 100 countries.

1.2 Despite the broad ranging remit of this inquiry, our response will focus solely on NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals).

1.3 We believe that Bayer has experienced a specific challenge in relation to evaluating the effectiveness and cost-effectiveness of drugs through the technology appraisal system which will be of interest to the Committee and has the potential to require that the *Guide to the methods of technology appraisal* be amended.

1.4 Key points from our evidence are as follows:

- NICE should only allow the use of off-label or unlicensed treatments as a comparator product when no licenced alternative exists.
- NICE should define "routine use" so that it is made clear which treatments can be considered as appropriate comparators in the technology appraisal system.
- NICE should ensure that its guidance and methods are consistent with recommendations of other bodies such as the Medicines and Healthcare products Regulatory Agency (MHRA), European Medicines Agency (EMA) and guidelines produced by professional regulators such as the General Medical Council (GMC).
- The on-going appraisal of our medicine aflibercept solution for injection (hereafter aflibercept) for the first line treatment of wet age-related macular degeneration (wet AMD) is an example where selecting an inappropriate comparator may fundamentally change the outcome of the technology appraisal.

2. NICE's role in evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisal)

2.1 NICE has a critical role to play in ensuring that rational and transparent decisions are made about the clinical and cost-effectiveness of treatments, to allow difficult decisions to be made about how the finite healthcare budget is allocated. We believe that this is an important part of the health system in England and one which should continue in the future.

2.2 There are some specific areas where we believe that the system could be improved which are outlined in this response. Specifically, this response focuses on the selection of appropriate comparators for inclusion in a technology appraisal.

Selecting the most appropriate comparator(s)

2.3 The selection of suitable comparators is of critical importance when setting the scope of any technology appraisal. The comparator becomes the benchmark against which the treatment being assessed in the technology appraisal is compared, in order to determine if the treatment can be considered clinically and cost-effective for use within the NHS. It is therefore essential that, to conduct a fair and robust technology appraisal, suitable

comparators are selected as this decision will frame the entire technology appraisal. NICE itself recognises that the choice of comparator can change the cost-effectiveness of a treatment entirely.³⁵

2.4 Section 2.2.4 of the *Guide to the methods of technology appraisal* issued in June 2008, describes comparator technologies. It states that:

*“Relevant comparators are identified, with consideration given specifically to routine and best practice in the NHS (including existing NICE guidance) and to the natural history of the condition without suitable treatment. There will often be more than one relevant comparator technology because routine practice may vary across the NHS and because best alternative care may differ from routine NHS practice. For example, this may occur when new technologies are used inconsistently across the NHS.”*³⁶

2.5 We understand that there may be a number of different appropriate comparators, but would suggest that where there is a licenced comparator used by the NHS, this should take precedence over use of other medicines which may be unlicensed.

Use of unlicensed and/or off-label comparator treatments

2.6 The *Methods Guide* goes on to state that:

*“Relevant comparator technologies may also include those that do not have a marketing authorisation (or CE mark for medical devices) for the indication defined in the scope but that are used routinely for the indication in the NHS.”*³⁷

2.7 We accept that in some cases there will be good reason to use a comparator medicine which is used off-label or where a medicine is unlicensed entirely. However, we believe that these comparisons should be limited to situations where there is no licenced comparator (for example in extremely rare conditions where conducting trials to secure a licence are difficult) or where unlicensed usage of a medicine is the most commonly used treatment in the NHS for a particular condition or disease (this may be the case where an older medicine is still best practice in the NHS).

2.8 Using comparators which are off-label or unlicensed can present a number of challenges in a technology appraisal:

- There is no clear reimbursement price set for the use of these treatments on the NHS. Indeed clinical practice, dose, treatment schedule and follow up can vary widely.
- Data about the clinical effectiveness of these treatments are likely to be more scarce and less robust than for licenced treatments used in a setting where they have been granted a marketing authorisation.
- Safety data are likely to be less mature and therefore patients could be exposed to unnecessary harm by using these treatments as a comparator, particularly where a licenced alternative is available for comparison.

2.9 These challenges render any cost-effectiveness assessment and subsequent recommendation extremely uncertain.

2.10 It is therefore correct that once comparators are selected, the appropriateness of these treatments as the benchmark for current best practice in the NHS is then scrutinised during the scoping process of any technology appraisal. It is important that the manufacturer of the product and other registered stakeholders involved in the technology appraisal have the opportunity to comment on the selected comparator treatments and to suggest where these are not appropriate.

3. THE CASE OF AFILBERCEPT

3.1 Bayer HealthCare is currently involved in the ongoing technology appraisal of aflibercept for first line treatment of wet age-related macular degeneration (wet AMD) as the manufacturer of this product.³⁸

3.2 During the initial scoping of this appraisal the Committee decided to include two comparators in scope of the appraisal:

³⁵ National Institute for Health and Clinical Excellence, *TA Methods Guide Review: supporting documents, Comparators non workshop briefing paper*, 7 June 2012. Accessed on 22 October 2012, via <http://www.nice.org.uk/media/C67/40/TAMethodsGuideReviewSupportingDocuments.pdf>

³⁶ National Institute for Health and Clinical Excellence, *Guide to the methods of technology appraisal*, June 2008. Accessed on 22 October 2012, via <http://www.nice.org.uk/media/B52/A7/TAMethodsGuideUpdatedJune2008.pdf>

³⁷ National Institute for Health and Clinical Excellence, *Guide to the methods of technology appraisal*, June 2008. Accessed on 22 October 2012, via <http://www.nice.org.uk/media/B52/A7/TAMethodsGuideUpdatedJune2008.pdf>

³⁸ National Institute for Health and Clinical Excellence, *Macular degeneration (wet age-related)—aflibercept (1st line)*. Accessed on 22 October, via <http://guidance.nice.org.uk/TA/Wave0/623>

- Lucentis (ranibizumab), which is licenced for the treatment of wet AMD. It was recommended by NICE for NHS use in August 2008, and the guidance was re-issued in May 2012 after a change to the Patient Access Scheme.³⁹
- Avastin (bevacizumab), which is unlicensed for the treatment of wet AMD (although used in a small minority of NHS centres to treat this condition) but is licenced to treat a number of different types of cancer.

3.3 We are concerned that the Committee has decided to include bevacizumab as a comparator treatment in this appraisal for the following reasons:

- Inconsistency with *NICE Methods Guide*
 - The Methods Guide states, “Relevant comparator technologies may also include those that do not have a marketing authorisation (or CE mark for medical devices) for the indication defined in the scope but that are used routinely for the indication in the NHS.”³ The use of bevacizumab can never become routine or best practice for treating wet AMD unless the product receives a marketing authorisation. As a medicine unlicensed for wet AMD, bevacizumab can only be legally supplied, dispensed and administered under certain exemptions from the medicines licensing requirements. Such exemptions are engaged only if there is an unmet special need of the patient (ie where no licensed alternative is available). The Court of Justice of the European Union (CJEU) recently confirmed this, making clear that cost considerations do not result in an unmet special need.⁴⁰
- Unsuitability for intra-ocular use
 - When bevacizumab is used to treat cancer patients it is administered via an intravenous infusion and is licenced for this type of usage. When bevacizumab is used to treat patients with wet AMD it is reformulated and administered intravitreally (ie, an injection directly into the eye). Since the draft scope for aflibercept was published the European Medicines Authority (EMA) has changed the summary of product characteristics (SPC) for bevacizumab to explicitly state that “Avastin is not formulated for intravitreal use”. It also states that “It has not been developed or made for injection into the eye. It is therefore not authorised to be used in this way,”⁴¹ and details that:
 - “Individual cases and clusters of serious ocular adverse events have been reported following unapproved intravitreal use of Avastin compounded from vials approved for intravenous administration in cancer patients. These events included infectious endophthalmitis, intraocular inflammation such as sterile endophthalmitis, uveitis and vitritis, retinal detachment, retinal pigment epithelial tear, intraocular pressure increased, intraocular haemorrhage such as vitreous haemorrhage or retinal haemorrhage and conjunctival haemorrhage. Some of these events have resulted in various degrees of visual loss, including permanent blindness”⁴¹
- Regulatory concerns

MHRA

 - The inclusion of bevacizumab as a comparator in this appraisal undermines the regulatory framework for medicines. Before a medicine can be authorised for use in the UK and the European Union, the medicine must have undergone extensive testing to demonstrate that the product is safe, efficacious and of suitable quality. The manufacturer of bevacizumab has not undertaken any such studies, and those studies that have consider unlicensed use of bevacizumab for eye conditions have a number of flaws.

General Medical Council (GMC)

 - The GMC guidance on off-licence/unlicensed prescribing⁴² does not allow doctors to factor in the cost of a medicine. Rather doctors must be satisfied that it would better serve the patient’s needs than the licensed alternative and be satisfied that there is a sufficient evidence base and/or experience of using the medicine to demonstrate its safety and efficacy. Therefore the unlicensed use of bevacizumab cannot be said to be ethically appropriate, routine or best practice for treating first-line wet AMD.
- NICE procedural inequality

³⁹ National Institute for Health and Clinical Excellence, *TA155: Ranibizumab and pegaptanib for the treatment of age-related macular degeneration*, May 2012. Accessed on 22 October 2012, via <http://publications.nice.org.uk/ranibizumab-and-pegaptanib-for-the-treatment-of-age-related-macular-degeneration-ta155>

⁴⁰ Case 185–10 *Commission v Poland*, 29 March 2012. There is also a pending case before the CJEU from Germany regarding the legality of reformulating bevacizumab (Case C-535/11 *Novartis Pharma GmbH v Apozyt GmbH*)

⁴¹ European Medicines Agency, *Annex 1—Summary of product characteristics, bevacizumab*, last updated 6 September 2012. Accessed on 22 October 2012, via http://www.ema.europa.eu/docs/en_GB/document_library/EPAR_-_Product_Information/human/000582/WC500029271.pdf

⁴² See Good practice in prescribing medicines—guidance for doctors, September 2008, available at: http://www.gmc-uk.org/guidance/ethical_guidance/prescriptions_faqs.asp#9

- Using bevacizumab off-label is cheaper than using treatments which have been developed and are licenced specifically to treat wet AMD. Therefore including this treatment inside the scope of the appraisal will skew the cost-effectiveness assessment of aflibercept, potentially undermining clinical effectiveness assessments. As outlined in point 2.3. above NICE itself recognises that the choice of comparator can change the cost-effectiveness of a treatment entirely⁴³ and the inclusion of bevacizumab in this appraisal will have perverse consequences.
- In the absence of a specific direction from the Secretary of State for Health requesting NICE to review bevacizumab for wet AMD, we believe (without of course accepting the legality of such a direction) that including it as a comparator in this appraisal could be viewed as conducting an appraisal of bevacizumab “through the back door”. Such a move could be used by PCTs to justify illegal policies recommending reformulated bevacizumab for wet AMD.

4. SUMMARY

4.1 We believe that aflibercept is an important example of where the *The Guide to the Methods of Technology Appraisal* needs to be changed and be made more specific. We will aim to address these points directly in the forthcoming NICE assessment of aflibercept, but we have highlighted this specific example to the Committee as we believe that this could have relevance to other future appraisals and is an area where action could be taken quickly to ensure that appropriate comparators are selected from the outset in technology appraisals.

October 2012

Written evidence from The Hepatitis C Trust (NICE 30)

Comments on NICE’s role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS:

1. The Hepatitis C Trust has found NICE to be responsive and helpful when evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions. There are a number of clinically and cost-effective treatments for hepatitis C that have received positive technology appraisal guidance from NICE.
2. We know however that uptake of NICE-approved treatments is not consistent across the board and we fear that this will be exacerbated if the mandatory nature of NICE appraisals are compromised in any way. For example, in the report, *The Extent and Causes of International Variations in Drug Use* (2010, by Professor Sir Mike Richards for the Secretary of State), the UK was the 13th out of 14 comparable countries for usage of hepatitis C treatments.
3. Although this low uptake is partly attributable to problems with service configuration, resources should be appropriately directed to ensure that patients get equal access to NICE-approved treatments.
4. It is vital that patient representatives and patient groups are closely involved with the value based pricing discussions on relevant drugs. It is welcome that the mandatory funding directive will apply to value-based pricing. Any hepatitis C treatments that are either NICE-approved or have received a value-based price, should continue to be made routinely available to eligible patients with hepatitis C.
5. It would be beneficial for NICE to offer free training to patient groups on the health technology appraisal process, how NICE works, how the introduction of value-based pricing will work and how to engage most effectively with relevant processes.

Comments on the role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities:

6. NICE Quality Standards feed into outcome measures and other important commissioning incentives and levers. The Hepatitis C Trust is therefore very concerned that NICE does not have plans to develop a hepatitis C quality standard. We question the decision process for quality standards as mortality rates from hepatitis C are rising and appropriate service configuration could reverse this trend.
7. Recent news reports suggest that the NHS Commissioning Board may be looking to other professional organisations for advice on commissioning. If delays in the NICE process continue, The Hepatitis C Trust may look to support this kind of initiative for standards for hepatitis C.

⁴³ National Institute for Health and Clinical Excellence, *TA Methods Guide Review: supporting documents, Comparators non workshop briefing paper*, 7 June 2012. Accessed on 22 October 2012, via <http://www.nice.org.uk/media/C67/40/TAMethodsGuideReviewSupportingDocuments.pdf>

Comments on the effect of the new public health system architecture on NICE's continued role in respect of public health guidance:

8. The Trust welcomes the NICE public health guidance on ways to promote and offer testing for hepatitis B and C that is due to be published by the end of 2012. This will give valuable detailed guidelines to local commissioners and providers of public health services, directors of public health, primary care practitioners and many other stakeholders. It will be useful for the uptake and impact of this guidance to be monitored and evaluated to ensure uptake.

9. NICE's public health guidance should take into consideration the shared responsibilities across the public health and health services to encourage coordination where it is needed. This is particularly relevant for hepatitis C where awareness and testing campaigns could result in greater pressure on NHS resources. Coordination is vital to ensure that there is capacity in the NHS to support testing for hepatitis C.

October 2012

Written evidence from the European Medicines Group (NICE 31)

This submission is made on behalf of the members of the European Medicines Group (EMG).

The EMG is fully supportive of the response made by the ABPI to this consultation and would like to take this opportunity to emphasise some of the points within the ABPI submission and to build upon others.

1. THE EUROPEAN MEDICINES GROUP

1.1 EMG was formed to enable the UK affiliates of research-driven pharmaceutical companies headquartered in Continental Europe to give their perspective on UK health policy. The EMG has 15 members. Together we employ over 10,000 people in the UK and are some of the biggest investors in medicines research in the UK, investing more than £500 million pounds in research and development every year. Between us we have developed some of the most innovative medicines available in the UK, having launched over 100 in the last decade.

1.2 The EMG is a separate organisation from the ABPI but works alongside it; the majority but not all of our members are also members of the ABPI.

2. SUMMARY

- As the reach of NICE grows, regulations should be put before Parliament to define clearly the future accountabilities of NICE, making the distinction between policy decisions for elected Ministers and operational decisions for NICE.
- Regulations on the role and responsibilities of NICE should include framing expectations about its role in supporting delivery of the life sciences growth and innovation agendas.
- With respect to Health Technology Appraisal (HTA):
 - There is a disproportionate focus on economic rather than clinical perspectives on the value of medicines.
 - NICE should incorporate the learnings from AGNSS into its approach to consideration of the value of medicines for rare conditions.
 - The situation with respect to factors that generate inconsistency in decision-making and affect NICE's ability to be fully accountable for decisions need to be addressed, eg providing a proper framework within which academic centres conduct evidence evaluations and Appraisal Committees weigh evidence.
- Implementation of positive NICE HTA guidance remains slow and while the recommendations of *Innovation Health and Wealth* and the introduction of inclusion of medicines subject to a positive NICE HTA are very welcome, other levers and incentives need to be considered to ensure that Clinical Commissioning Groups (CCGs) are encouraged to implement NICE guidance and are appropriately reimbursed.
- As there has never been an expectation that all new medicines would be subject to NICE HTA, it is important to note there are many that have not nor will likely ever go through the HTA process. EMG estimates that over half of the medicines launched by its members in the last decade have not been selected for HTA. These medicines are not "value unproven", they have merely not been through NICE HTA and they still have a valuable role to play in the treatment of patients. Patient access to these medicines then becomes a local issue. EMG experience is of variable quality in local decision making. The absorption of the National Prescribing Centre (now the Medicines and Prescribing Centre (MPC)) into NICE in April 2012 means NICE now has a direct interest and an important role in improving quality and supporting fair and equitable use of these medicines locally. EMG encourages the new Chair of NICE to take up this challenge and find solutions.

- This Inquiry provides a timely opportunity to consider creative mechanisms which might allow a medicine not otherwise designated for appraisal to be considered for HTA where there is significant interest from a key stakeholder (including the manufacturer).
- A significant concern of EMG is the recommendation in NICE clinical guidelines and HTA guidance of off-label and unlicensed medicines when appropriately licensed alternatives are available. Straying into the area of endorsing medicines that have not been subject to the rigorous scrutiny of the Medicines and Healthcare products Regulatory Agency (MHRA) and European Medicines Agency (the competent authorities) for the conditions in which they are being recommended undermines the medicines regulatory system that exists to protect patients. Relevant clinical guidelines and HTA guidance should be corrected.

3. CLEARLY DEFINING ACCOUNTABILITIES

3.1 As regulations are being drafted in preparation for the future of NICE as an executive non-departmental public body (NDPB), the Inquiry is a timely opportunity to consider the impact that NICE has on patients' access to medicines, the NHS, the research-based pharmaceutical industry and the wider economy.

3.2 The importance of NICE and its impact on the well-being of our citizens are enormous. As its reach grows, EMG believes that regulations must be put before Parliament to define clearly the future accountabilities of NICE and how it should operate as its status evolves to a NDPB. The future increased independence of NICE is recognised, and this makes it imperative that it is clear which are policy decisions for elected Ministers and which are operational decisions for NICE. Like the ABPI, EMG has concerns, for example, that in the recent review of the NICE *Guide to the Methods of Technology Appraisal*, NICE has strayed into defining what elements constitute "value", which EMG believes is not within the remit of a politically unaccountable organisation. No matter how unintended, "mission creep" must not be allowed to take place in an organisation which is so important to UK citizens.

3.3 NICE decisions have a direct effect on the adoption of innovation in the NHS and on the achievement of the life sciences growth agenda. Within the new regulations, therefore, EMG believes there exists an important opportunity to frame expectations of the role of NICE in supporting delivery of these key Government policies.

4. HEALTH TECHNOLOGY APPRAISAL

4.1 In its submission ABPI has extensively covered issues relating to NICE's role in HTA. Given the constraints of document length this is not repeated here, though EMG is fully supportive of these comments. . In particular we share concerns that there is a disproportionate focus on the economic rather than clinical perspective of the value of medicines.

Medicines for rare diseases

4.2 Our concerns about NICE methods are particularly pertinent to the appraisal of medicines for rare conditions. NICE has recently been asked to take on appraisal of medicines formerly undertaken by the Advisory Group for National Specialised Services (AGNSS), ie medicines for conditions generally affecting up to 500 people in the UK. We believe a fundamental tension exists between the approaches to the assessment of value adopted by NICE and AGNSS.

4.3 Development costs of a medicine tend to be similar regardless of whether there are many or few patients who may benefit from treatment. In rare conditions this means that these costs are recouped from a much smaller population resulting in a higher "per patient" medicine acquisition cost. The use of the economic measure (the Incremental Cost Effectiveness Ratio (ICER) as the measure of cost-effectiveness, which is the NICE standard, is not appropriate for these medicines as the value of the ICER will invariably and necessarily exceed NICE's cost effectiveness threshold. It is inequitable that patients be denied an effective treatment merely because the condition from which they suffer is rarer than conditions affecting other people.

4.4 The challenges of appraisal of these medicines had been considered at length by AGNSS which developed a framework that recognised a range of factors determining value, including patient need/disease severity and societal benefits. Given the expertise in rare conditions within AGNSS, we call on the new Chair of NICE to incorporate this learning into the NICE approach for assessment of medicines for rare conditions; indeed there is much to be said for this broader consideration of value for all medicines.

4.5 The new Chair might also wish to consider the appropriate place and method of appraisal for diseases affecting between 500 and 1000 people. Currently, NICE generally undertakes appraisal in patient populations of over 1,000 and the AGNSS brief related to populations of 500 or below.

Timeliness of appraisal

4.6 Timely access to new medicines is a key priority. Whilst EMG supports NICE in maintaining rigour around the integrity of technology appraisals, consideration should also be given to expediting the process in order to ensure patients do not face the delay in access that is caused by a system which takes 12–15 months to reach a decision.

5. CONSISTENCY OF DECISION-MAKING

5.1 EMG is concerned about factors inherent in NICE HTA which generate inconsistent decision-making. In theory one should expect a high degree of consistency in the outcomes of a process which on the face of it has many standards and absolutes. Yet despite this, different Appraisal Committees considering the same evidence can reach different conclusions—an issue which has been acknowledged by NICE itself.

5.2 The situation with respect to the academic centres that act as Evidence Review Groups (ERGs) and conduct evidence evaluations for NICE is troubling especially when looking to a future where NICE might play a role in determining value in a new pricing scheme. Industry experience is that different approaches to evidence evaluation are adopted by these academic centres, which are neither funded by nor report to NICE, meaning it has a limited ability to determine how they work. As the ABPI has said in its submission, the ethos of “academic freedom” exercised by these units cannot be allowed to continue to manifest itself in the level of variability that has characterised NICE HTA thus far. To ensure a fair and equitable system for patients, EMG supports the ABPI in its assertion that the current system, whereby academic centres adopt variable approaches to evidence evaluation and Appraisal Committees to weighing evidence, undermines the ability of NICE to be accountable for decisions made on behalf of the patients it serves.

5.3 Furthermore, an effective research-based pharmaceutical industry which can contribute both to the health and well-being of our citizens as well as the UK economy needs consistency, clarity and certainty of approach. The current level of variation is unacceptable, particularly so should NICE play any role in a new pricing scheme.

6. IMPLEMENTATION OF NICE GUIDANCE

6.1 The implementation of NICE HTA guidance remains slow and variable and the system needs to increase incentives for local NHS organisations to adopt it (and introduce penalties for failure to do so). Mandatory funding has been in place for some years and compliance with NICE HTA guidance is a commitment in the NHS Constitution, so when the outgoing Chair of NICE needs to suggest (as has been reported) that patient groups resort to judicial review to assert these rights, a radical reconsideration of measures to ensure implementation is probably justified. Amongst potential measures, the tariff should be adjusted to ensure that trusts are appropriately and adequately reimbursed when implementing NICE guidance. The recommendations of the *Innovation Health and Wealth* review are a welcome step towards effective implementation.

6.2 NICE has an Implementation Directorate which provides resources on putting guidance into practice. Given the challenges to date, we encourage the new Chair of NICE to consider how this function can be made more effective.

7. MEDICINES THAT HAVE NOT BEEN SUBJECT TO A NICE HTA

7.1 There has never been an expectation that all medicines in England would be subject to a NICE HTA; EMG experience is that over half of the medicines introduced by its members over the last decade have not been appraised by NICE. These include medicines for cancer, in conditions that affect only small numbers of patients, and for conditions where no other effective therapy exists (but often in areas with large numbers of treated patients). These medicines should not be assumed to be “value unproven” (many have been appraised and approved in Scotland and in other European countries for example); merely because they have not been selected for NICE appraisal.

7.2 Patient access to and the use of these medicines then becomes a local issue. The absorption of the National Prescribing Centre (now the MPC) into NICE in April 2012 means NICE now has a direct interest in both national and local medicines decision-making.

7.3 EMG experience is that the quality of local decision making to determine which medicines (old and new) are included in local formularies can be variable (in terms of evidence evaluation and decision-making processes) with lack of transparency of some processes and duplicative/multiple assessments occurring. While we respect the move to local determination by CCGs, EMG does not believe that good practice should be defined locally; this risks reinforcing the current variations in quality and compounding inequity of access. EMG believes NICE has an important role in improving the quality of local decision-making. EMG therefore calls on the new Chair of NICE to take up this challenge and find solutions—an issue with which the EMG would be very willing to engage.

7.4 Whilst emerging mechanisms (such as MPC Evidence Summaries: New Medicines) may help reduce variation in local evaluation, it should be noted that they do not cover the same mandate for implementation as is the case with a technology appraisal. This has the potential to lead to an increasing divide between those medicines subject to NICE HTA and those that are not. EMG encourages NICE to consider a mechanism for ensuring medicines (be that new chemical entity, indication or formulation) can be subject to HTA where there is significant pressure from any key stakeholder, including the manufacturer.

8. ADVOCATING USE OF OFF-LABEL AND UNLICENSED MEDICINES

8.1 EMG fully accepts that there will be situations of compelling clinical need where no appropriately licensed alternative exists and the use of an off-label or unlicensed medicine is in the interests of the individual patient. NICE has a potentially valuable contribution to make in providing clarity and consistency about use of medicines in these circumstances.

8.2 EMG agrees with the position of the ABPI on use of unlicensed and off-label comparators in the development of HTA guidance.

8.3 A significant concern is recommendation in NICE clinical guidelines and, less frequently, HTA guidance of off-label or unlicensed medicines when an appropriately licensed alternative exists.

8.4 Recommendations of this type undermine the regulatory system that exists to protect patient safety. The MHRA is, by law, the authority on deciding whether the balance of risk and benefit of medicines is acceptable after full scientific scrutiny of the evidence. We are concerned that NICE has strayed into areas of endorsing routine use of some medicines which have not been subject to the rigorous scrutiny of the competent authority in the conditions for which their use is being recommended by NICE.

8.5 EMG calls for existing clinical guidelines and technology appraisal guidance, which recommend the use of unlicensed and/or off-label medicines where an appropriately licensed alternative exists to be corrected.

8.6 EMG believes that a recent judgement made by the European Court (in relation to Poland)⁴⁴ has helped clarify the legal position about government-sponsored organisations advocating the use of unlicensed medicines in circumstances other than for compelling clinical need.

We would be happy to comment further on any of the points raised above.

October 2012

Written evidence from The Karen Clifford Skin Cancer Charity (NICE 32)

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

- The Karen Clifford Skin Cancer Charity (Skcin) supports the intention of the health technology appraisal process to eliminate regional variations in access to treatments and ensure that value for money is delivered for the NHS. However, NICE has yet to issue guidance on the new treatments for melanoma, having previously issued draft negative guidance. Currently patients are only able to access these treatments through the Cancer Drugs Fund and there is, as yet, no clarity about the arrangements for funding these treatments after 2014.
- Skcin is keen to ensure that the patient perspective is incorporated into value-based pricing. Our experience of engagement with the technology appraisal process has not been that patients are at the centre of the decision. We would recommend that NICE ensures that, whatever role it takes in supporting value-based pricing, patients are heavily involved in the process.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

- NICE should actively work to speed up the development of quality standards. Skcin has welcomed the inclusion of skin cancer, (including melanoma) in the library of quality standards that will be developed but we are disappointed that no progress has yet been made on developing it. Recent news reports have suggested that the NHS Commissioning Board work with other groups to get the expertise to develop guidance and Skcin believes that this is welcome, if an appropriate validation process is in place and it means that guidance will be made available more swiftly.

The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

- NICE's public health guidance should take into consideration the shared responsibilities across the public health and health services to encourage coordination where it is needed. There are a number of initiatives that could be taken to prevent skin cancer and raise awareness of the signs and symptoms of melanoma. It is important that, if public health services carry out an awareness campaign, NHS resources are also available to ensure that patients can get access to diagnosis and advice through the NHS. It is also important all interested parties/charities are involved/consulted.

October 2012

⁴⁴ Judgement of the Court (third Chamber), case C-185/10 Action under Article 258 TFEU. Judgement 29.03.12.

Written evidence from AstraZeneca (NICE 33)

ABOUT ASTRAZENECA

AstraZeneca is a UK based global biopharmaceutical business. It is a significant contributor to UK science and innovation investment, to economic prosperity and to patient health. Some key facts about our UK presence are:

- We have 7,500 employees, including 3,000 scientists.
- Alderley Park in Cheshire is our leading global centre for cancer research.
- Around 45% of AstraZeneca's global Research & Development is invested in the UK.
- AstraZeneca accounts for around 3% of the UK's export of goods.
- We have had two medicines approved by NICE since 2010, treating non small cell lung cancer and Acute Coronary Syndromes.

SUBMISSION BY THE ASSOCIATION OF THE BRITISH PHARMACEUTICAL INDUSTRY (ABPI)

AstraZeneca supports the views set out in the submission of the ABPI, particularly the introduction of a clearer accountability framework. We have added some supporting comments below on the specific issues raised by the Committee.

NICE'S ROLE IN RELATION TO EVALUATING THE EFFECTIVENESS AND COST-EFFECTIVENESS OF DRUGS AND OTHER CLINICAL INTERVENTIONS

Academic Input to the Appraisal Process

The discharging of NICE's duties has been made more difficult due to the nature of the relationship it has with the independent academic centres which are contracted with the Department of Health through the National Institute for Health Research (NIHR) Health Technology Assessment programme. The Groups are not accountable to NICE and often exceed their remit of critiquing the evidence in line with NICE methods, resulting in inconsistency. It is currently unclear how the role of the Evidence Review Groups (ERGs) will be affected during the intended introduction of value based pricing.

Implementation of NICE's Health Technology Assessment Guidance

The NHS should be more strongly incentivised to implement NICE Health Technology Assessment (HTA) guidance using appropriate levers and incentives (eg. CQUIN, Commissioning Outcomes Framework). It is recognised in the UK and abroad that NICE produces well respected and robust evidence based technology assessments of new medicines. Its guidance is backed with a statutory funding direction, to ensure the NHS makes funding for these technologies available. Despite this, there is still considerable variation in the implementation of its guidance across the NHS.

One year after it was recommended by NICE, underpinned by a significant mortality benefit over the current standard of care, uptake for AstraZeneca's medicine ticagrelor, for the treatment of Acute Coronary Syndromes (ACS), provides a clear example. While a significant proportion of patients now have routine access to it in the North of England, only a comparatively small number of hospitals in London and the South are making it routinely available to patients. The main reason for this variable uptake is that there are still too many local processes and barriers which seek to duplicate the NICE assessment process. These include reviews by formulary committees and clinical networks. In cases where the medicine is initiated in secondary care and then used in primary care, the need for inclusion on treatment protocols provides another sometimes lengthy delay as agreement for funding is sought between a hospital and local commissioners. These local barriers and processes delay or sometimes block entirely patient access to NICE recommended medicines. We welcome the steps being taken through the "Innovation, Health and Wealth" implementation process to improve the situation. But, as the ABPI submission states, there is a long way to go before the NHS willingly and actively adopts NICE guidance.

THE ROLE OF NICE QUALITY STANDARDS IN THE NEW NHS SYSTEM ARCHITECTURE

We understand the role of Quality Standards is to ensure that patients can expect to receive a high quality of care from the NHS. However, with provider organisations incentivised to achieve Quality Standards, there is a concern that other aspects of patient care will be overlooked.

THE CONTINUING ROLE OF NICE CLINICAL GUIDELINES IN IMPROVING THE QUALITY OF HEALTHCARE

In the reformed NHS system, much of the focus seems to be on Quality Standards, while clinical guidelines appear to have been marginalised. The NHS should find a way of incentivising NHS organisations to implement clinical guidelines and the associated best practice rather than just those recommendations that are stated in

the related Quality Standards. It is critical that the system avoids inadvertent perverse incentivisation of poor clinical care in those areas not covered by the Quality Standards.

October 2012

Written evidence from the BioIndustry Association (NICE 34)

1. Established in 1989, the BioIndustry Association (BIA) is the trade association for innovative healthcare focused bioscience enterprises. BIA members include emerging and more established bioscience companies, pharmaceutical companies, academic research and philanthropic organisations, and service providers to the UK bioscience sector. Our members are responsible for over ninety% of biotechnology-derived medicines currently in clinical development in the UK and are at the forefront of innovative scientific developments targeting areas of unmet medical need. This innovation will lead to better outcomes for patients, to the development of the knowledge-based economy, and economic growth.

2. The BIA welcomes this short inquiry by the House of Commons Health Select Committee into the National Institute for Health and Clinical Excellence (NICE). It comes at an opportune time when the roles and responsibilities of NICE are in transition and it will be important for the Committee to give consideration to the possible effects of such changes to the life sciences sector and patients.

3. The comments below are focused primarily on the first part of the Committee's inquiry. This focuses on NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals).

4. It is worth noting from the outset that the UK has significant strengths in medical research and development. Government figures demonstrate that the life sciences sector generates a turnover of over £50 billion and employs 166,000 people in 4,500 companies.⁴⁵ Biopharmaceutical companies alone invest almost £5 billion per annum in research and development in the UK, more than any other sector.

5. The Government has recognised the importance of the sector to future UK growth and innovation through the launch of the Strategy for UK Life Sciences.⁴⁶ It is important to have a positive environment for clinical research and development at every stage to ensure an ecosystem that is conducive to life science activity. The role of NICE is one such important component of the development pathway of a new medicine.

6. The BIA values and recognises the expertise that NICE has developed and welcomes its openness to engagement.

7. The BIA is aware of other responses from interested stakeholders to this call for evidence. This includes the response of the Association of the British Pharmaceutical Industry which the BIA also supports. With this in mind, the comments below focus on some core issues currently of interest to BIA members.

8. There are three issues in particular the BIA would like to make the Committee aware of: a) the global impact of NICE activities; b) NICE's new evidence summaries of unlicensed medicines; and 3) NICE's new role in taking over the assessment of rare diseases from the Advisory Group for National Specialised Services (AGNSS).

9. A) NICE is an internationally renowned and respected organisation and its expertise in health technology assessments are of particular importance. The message from NICE is therefore heard globally by other jurisdictions who often rely upon its assessments of new medicinal products.

10. NICE should remain aware of this wider global impact particularly as it relates to innovative life science investment in the UK. The Strategy for UK Life Sciences clearly outlines the government's ambition to make the UK an attractive location for industry to invest and it is important to also demonstrate that products of such research are also readily adopted. This helps to avoid the situation whereby the UK is perceived to be welcoming to medical research but poor at adoption.

11. B) NICE is now providing evidence summaries, which do not constitute formal NICE guidance, on unlicensed or off-label drugs. In July 2012 NICE released its interim progress statement⁴⁷ and on 15 October published its first evidence summary on Tranexamic acid.⁴⁸ A list of upcoming evidence summaries is available on the NICE website.⁴⁹

12. The BIA has expressed its interest in these evidence summaries to NICE and is grateful to be given the opportunity to engage going forward. It is understood that such evidence summaries are, in part, an attempt to better educate clinicians regarding an available medicinal product to guide usage.

13. The BIA is keen to understand the process involved in the formation of evidence summaries and the opportunity a company may have to engage with the authorities if its product is subject to such a review. The

⁴⁵ <http://www.bis.gov.uk/assets/biscore/innovation/docs/s/11-p90-strength-and-opportunity-2011-medical-technology-sectors>

⁴⁶ <http://www.bis.gov.uk/assets/biscore/innovation/docs/s/11-1429-strategy-for-uk-life-sciences>

⁴⁷ http://www.nice.org.uk/media/01B/92/ESUOM_Interim_Process_Statement_July_2012.pdf

⁴⁸ <http://www.nice.org.uk/newsroom/pressreleases/NICEPublishesFirstEvidenceSummaryUnlicensedOrOffLabelMedicine.jsp>

⁴⁹ <http://www.nice.org.uk/mpc/evidencesummariesunlicensedofflabelmedicines/home.jsp>

use of unlicensed and off-label medicines is an area which requires sensitivity and does not automatically lend itself to the decision making tools that NICE have to hand.

14. In particular, it is worth considering the possible affect the new initiative may have on the regulatory pathway for the development of medicines. The safety and efficacy testing required for new medicines are necessary to support patient and clinician confidence with the product. Any evidence summaries of unlicensed or off-label medicines clearly have an impact on these regulatory and safety issues, where clinical trials may not be completed or may not have taken place, and may cause confusion within the sector.

15. It will also be important to ensure that such evidence summaries are not used for the purpose of legitimising the use of off-label drugs over authorised products on the basis of cost. NICE have acknowledged this issue and indeed a recent case, *European Commission v Republic of Poland*, made clear that the use of off-label or unlicensed medicines cannot be done on the basis of cost alone. The BIA is also unclear how this will interact with the proposed Earlier Access to medicines Scheme announced in the Strategy for UK Life Sciences.

16. C) It was further announced this year that NICE would take over the roles and responsibilities of AGNSS in the assessment of orphan drugs for rare diseases. This expansion of NICE's role will again require careful planning and sensitivity to ensure it does not negatively impact upon innovative research and development.

17. AGNSS developed a great deal of expertise in the assessment of orphan drugs recognising that traditional methodologies and tools applied by NICE in health technology appraisals are not appropriate. In the past NICE has been unable to reconcile its methodology with the data supplied for review given that the patient populations are so small and the clinical studies are, therefore, based on a smaller cohort of patients.

18. BIA members who are researching and developing innovative products for rare diseases have a concern therefore to avoid losing the expertise built up during the period of positive progress under AGNSS. NICE should also interact with the new Specialised Services Commissioning Innovation Fund which was again established recently with the recognition that specialist care for rare conditions requires a national approach.

October 2012

Written evidence from the MS Society (NICE 36)

1. ABOUT MS

1.1 Multiple sclerosis (MS) is one of the most common disabling neurological conditions affecting young adults. Around 100,000 people in the UK have MS. For most people, MS is characterised by relapses followed by periods of remission, while for others it follows a progressive pattern. Even those with relapsing-remitting MS typically experience increasing disability and morbidity. The causes of MS are unknown, there is no cure and the treatments that are available are effective in only certain cases and for some of the time. MS symptoms include loss of mobility, pain, fatigue, visual impairment, numbness, loss of balance, depression and cognitive problems. MS can lead to severe and permanent disability.

2. ABOUT THE MS SOCIETY⁵⁰

2.1 The MS Society is the UK's largest charity for people living with MS, with approximately 38,000 members and more than 300 branches. The MS Society is the UK's largest charitable funder of research into MS. Since 1956, the MS Society has been providing information and support, funding research and fighting for change. We provide grants to individuals, for home adaptations for example. We are committed to bringing high quality standards of health and social care within reach of everyone affected by MS.

3. SUMMARY

- NICE must adopt a process of precedent when considering the use of comparators for a condition area in health technology.
- There needs to be an easy and transparent “whistleblowing” system, that patients and clinicians can take to raise their concerns with NICE and the NICE Implementation Collaborative when obligatory guidance is not being carried out.
- The NICE scorecard should include Fingolimod or an alternative MS NICE recommended treatment to improve limited access to MS treatments.
- The government must ensure the focus on NICE approved medicines does not result in poor access to non-appraised but licensed and effective treatment options.
- We recommend NICE works with the MS Society and the researchers of the MS measure and MS register⁵¹ to ensure both can be applied in future research and HTAs.

⁵⁰ Multiple Sclerosis Society. Registered charity nos 1139257 / SC041990. Registered as a limited company in England and Wales 07451571

⁵¹ www.ukmsregister.org

- NICE must engage with stakeholders beyond the Guideline Development Group when producing guidance.
- NICE must develop the library of Quality Standards as soon as possible to ensure a broad Commissioning Outcomes Framework and provide commissioners with guidance.
- NICE should produce integrated guidelines and Quality Standards covering health and social care.

4. INTRODUCTION

4.1 The MS Society has had extensive engagement with NICE in the last few years through NICE health technology appraisals (HTAs), consultations on Commissioning Outcomes Frameworks (COF), Quality Standards and the MS Clinical Guideline. Through this work a number of issues have been raised.

5. EVALUATING THE EFFECTIVENESS AND COST-EFFECTIVENESS OF DRUGS AND VALUE BASED PRICING

5.1 **Lack of precedent:** The NICE appraisals of Tysabri, Fingolimod and the current MS multiple technology appraisals all demonstrate that NICE is repeatedly failing to apply a precedent regarding comparators. NICE continue to argue that “Best Supportive Care” should be the comparator without defining what this means and assessing whether this is a fair approach. As this was considered an inappropriate comparator for Tysabri and Fingolimod, we are saddened to see NICE are once again suggesting that this should be the comparator for the multi technology appraisal of four MS drugs. This will result in an unnecessary waste of valuable resources and time not only for the MS Society but also NICE.

- NICE must adopt a process of precedent when considering the use of comparators for a condition area.

5.2 **Transparency and time:** It is not clear what difference the MS Society or its patient experts have made to NICE decisions. The MS Society asked over 1,000 people with MS for their views and experiences of different methods of treatment; it is unclear to what extent this evidence was taken into account or weighted in the final decision on Fingolimod. Factors such as whether a medicine is innovative, can be taken into consideration but as it’s not a formal part of NICE’s statutory remit and or methods guide, does not have to be made explicit.

5.3 In the final decision on Fingolimod, the committee acknowledged that the economic analysis did not capture all the potential benefits: “The Committee recognised that including all of the benefits of Fingolimod which may not be adequately captured in the QALY calculation (as suggested by the manufacturer and the patient experts) could decrease the ICER to a level that would be considered a cost-effective use of NHS.”⁵² NICE errs on the side of caution; this is understandable but risks ignoring potentially positive impacts. This inconsistent approach must be addressed.

5.4 The TA for Fingolimod took an unusually long time; three committee meetings and two Appraisal Consultation Documents before the FAD was published. If the committee had checked from an early stage that they were taking a balanced approach valuable resources for NICE, patient groups and other organisations could have been saved.

- NICE must constantly and consistently check that they are taking a balanced view and are not ignoring positive impacts.
- NICE must be able to communicate to patient groups how they have taken their evidence into account and how this has been weighted in the final decision.

5.5 **Acknowledging Expertise:** We experienced a bias towards clinicians’ expertise above patient group and patient experts’ evidence. For example, in our experience there was an inconsistent approach when inviting the patient expert with MS who was not invited to all committee meetings.

5.6 Clinicians’ expertise is important as the committee does not always have a member with relevant expertise. Despite the need for this expert advice during the HTA for Fingolimod the committee did not always treat the clinician or the patient group with respect and brought into question their approach and ethics without direct knowledge and understanding.

- Committees must always invite a patient expert to committee meetings during an appraisal or communicate why this is not appropriate.
- Committee chairs and members must foster a helpful and constructive dialogue with the experts to ensure that the meeting is inclusive and professionally conducted.

5.7 **Limitation of accurate evidence:** NICE prides itself on the use of evidence, however, in MS drug appraisals, NICE committees continue to use an outdated longitudinal data set. The MS Society is funding an MS register⁵³ and would strongly encourage NICE to work with us to ensure the MS register can be used for HTAs. The MS register is an online survey and seeks to combine information collated from people with MS, neurologists and data collected by the NHS to build a national picture of how MS affects each individual and

⁵² NICE Final Appraisal Determination Fingolimod, TA254, 2012.

⁵³ www.ukmsregister.org

what impact it has on lives. It would be expedient to actively seek involvement from external organisations at the scoping stage to check the most recent datasets are used. This will help to reduce discrepancies at later stages in the appraisal process. Similarly, the MS Society is funding research to develop a more sophisticated EQ5D measure for MS. The EQ5D is currently limited in its ability to capture changes in mobility, fatigue or cognitive dysfunction in MS.

- NICE must engage with external organisations to check the most recent datasets are used and use patient registers whenever possible.
- We recommend NICE works with the MS Society and the researchers of the MS measure and MS register to ensure both can be applied in future research and HTAs.

5.9 Implementation of TAs and guidance: Even once a positive NICE appraisal is given there is not rapid implementation of the recommendation despite statutory obligation to provide the medicine. For MS, the UK ranks 25 out of 27 EU countries in access to treatment with approximately 11% on treatment despite MS treatments having a funding directive since 2002.⁵⁴

5.10 The NICE implementation team is miniscule in comparison to the rest of the organisation with approximately six people within the team. Despite NICE's remit growing the implementation team has not kept pace. Monitoring work and whistle blowing rests with patient groups and in some cases clinicians although it is not easy for clinicians to raise their concerns.

5.11 The NICE implementation Collaborative (NIC) and the scorecard have the potential to improve implementation of TAs. However, the scorecard will not be able to monitor all HTAs and the work of the NIC will rest on the willingness of partner organisations.

- The NICE scorecard should include Fingolimod or an alternative MS NICE recommended treatment to understand barriers to implementation.
- There needs to be an easy and transparent “whistleblowing” system, that patients and clinicians can take to raise their concerns with NICE and the NIC when implementation of obligatory guidance is not being carried out.

5.12 Value Based Pricing: The possible development of VBP presents new opportunities for the appraisal of drugs which could include wider societal factors as recommended in the Kennedy Review. However, patient groups have already noticed possible implications of VBP that are concerning. There is a risk that if early discussions continue without change it would result in reduced access to medicine for vulnerable groups who are not able to work or “contribute to society”.

5.13 Early discussions have been opaque and have not allowed patient groups to actively engage with the research and highlight potential issues. There is growing concern that decisions are being made in isolation between NICE, the Department of Health and the ABPI with cursory engagement with patient groups rather than meaningful consultation. NICE must use its voice to raise the issue of patient involvement and assert its authority in evidence based practice.

5.14 To date there has been no engagement with patient groups regarding what questions the research should ask, who should be asked and whether it is capturing all the concerns of people affected. Without a clear understanding of what the research aimed to achieve, how it supports the new VBP approach and how it will safeguard against unfair implications for vulnerable groups in society it would be irresponsible to press ahead.

- We strongly encourage NICE to use its voice to raise the issue of patient involvement and assert its authority in evidence based practice.
- NICE must ensure that safeguards are in place to prevent vulnerable groups from being discriminated against in the appraisal of treatments.

5.15 NICE Blight: For people with MS there are two NICE recommended treatments however, there are a number of other licensed MS symptomatic treatments that have not been appraised by NICE, these include Sativex and Fampyra, and will not undergo an HTA. For people with MS this is frustrating as there are effective licensed treatments for MS symptoms which they cannot access as they have not undergone an HTA.

5.16 Little has been done to reduce “NICE blight” identified in earlier reports and whilst VBP may seek to address this issue in the future it will not necessarily address existing licensed treatments. In many cases health bodies are not providing treatments unless they are mandated to do so because it is a statutory duty.

- The government must ensure the focus on NICE approved medicines does not result in poor access to those medicines not considered by NICE but licensed and effective treatment options.

6. THE CONTINUING ROLE OF NICE CLINICAL GUIDELINES

6.1 The MS Society has been calling for a review of the MS clinical guideline for some years and in 2012 the review of the 2003 guideline began. The new guideline will not be published until 2014 when the original guideline will be 11 years old. With an extending remit NICE must ensure that progress continues and guidance and quality standards are developed and revised in a timely manner.

⁵⁴ Kobelt and Kasteng, 2009, *Access to Innovative Treatments in Multiple Sclerosis*.

6.2 To date the engagement with NICE regarding the MS clinical guideline has been poor. There is a tendency to develop the draft guideline in isolation of the wider stakeholders. Without direct membership of the Guideline Development Group there is limited impact that patient groups can have during the development of the guideline. There is no formalised process to inform stakeholders of discussions as they take place reducing the opportunity to share additional expertise and evidence at the earliest opportunity.

6.3 The MS Society coordinated an extensive response to the draft scope for the MS guideline and collaborated with professional groups and other organisations calling for changes to the scope. Despite this there were very few changes and children, disease modifying treatments and social care remain outside of the scope questioning the inclusivity and comprehensive nature of the guideline.

6.4 Without the opportunity to engage with the development of the guideline the consultation on the draft guideline is the sole way to improve the final document. However, our experience leaves us with little confidence that the consultation on the final document will be any different. Developing the guideline in isolation of stakeholders risks a final document that is not supported by stakeholders.

- NICE must regularly update clinical guidelines to ensure that people affected by conditions have access to an up to date guideline to understand what their pathway of care should look like.
- NICE must engage with stakeholders beyond the GDG during the development of guidelines to ensure that the final document is robust, relevant and supported beyond the GDG, this could be through an observer status at GDG meetings or by sharing papers with stakeholders.

7. What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

7.1 Integration and Quality Standards: In our response to the consultation on the scope for the MS clinical guideline, in light of the forthcoming extension of NICE's remit into social care, we called for an integrated guideline covering health and social care. Disappointingly, NICE ignored joint calls to develop an integrated guideline despite widespread support from a number of professional groups. If integration is to become reality clinicians and commissioners will need integrated condition specific guidance as well as broader guidance or integration will remain political rhetoric.

- NICE needs to lead by example and provide integrated guidance and Quality Standards covering health and social care on specific conditions not just overarching themes as early indications show.

8. The role of NICE Quality Standards in the new NHS system architecture and the status of NICE guidelines in determination of commissioning priorities

8.1 Not all NICE guidance is compulsory, but should act as a guide for commissioners. Consequently, implementation and monitoring is limited. Under the new system the COF provides one mechanism to encourage implementation of guidance as indicators can be derived from Quality Standards. However, with only 60 indicators there is a limit to the range of conditions and overarching themes that can be incorporated in the COF.

8.2 The delay to the revised MS clinical guideline and the consequential delay in developing an MS Quality Standard is an early warning sign. If NICE fails to develop guidelines and Quality Standards in a timely manner there is a risk of a culture of neglect within NICE and beyond where commissioners prioritise solely areas with COF indicators. The deadline for completing the library of Quality Standards was 2015 but this has now extended to 2019 causing concern for how commissioners will be informed of how best to commission high quality standards.

8.3 NICE must improve the implementation of guidance and quality standards by developing mechanisms and incentives. Without such an approach there is a great risk that extensive resources will be used to develop guidance which is only minimally implemented. As patient groups we have a role to play in encouraging the implementation of guidance but it cannot be patient groups alone that carry this responsibility.

- NICE must improve the monitoring and implementation of guidance and quality standards by developing mechanisms and incentives for recommended guidance.
- NICE must develop the library of Quality Standards as soon as possible to ensure a broad COF and prevent neglect of areas without COF indicators.

Written evidence from Bristol-Myers Squibb Pharmaceuticals Limited (NICE 38)

SUMMARY

BMS welcomes this inquiry into the work of NICE prior to the appointment of a new NICE Chair. NICE has a significant impact on patients' access to new and innovative medicines and BMS has an active and constructive relationship with the organisation. However, we believe that this is an opportunity to make significant improvements to the roles and responsibilities of NICE.

- NICE should undertake a more balanced assessment of the various elements of value. It should further hold its appraisal committees accountable to ensure that all aspects of value are fully considered in decision making processes.
- NICE is becoming increasingly conservative when judging uncertainty and we would welcome a more pragmatic approach.
- In cases where generic medicines become the comparators of choice in health technology assessments (HTAs), it is impossible for new medicines to demonstrate cost-effectiveness at a price that allows the manufacturer a fair return on investment.
- Appraisal committees and academic review groups should be held accountable by a clear governance hierarchy.
- When an appeal is upheld, the appraisal should be referred to a different appraisal committee than the one that undertook the original appraisal.
- We believe stronger incentives must be put into place to encourage NHS organisations to take up NICE recommended medicines.
- We request that the work of NICE International be re-considered.
- In the context of value based pricing, BMS believes that NICE should not be the single body assessing all aspects of value of a medicine. Organizations which have the strongest expertise in assessing broader aspects of value beyond HTAs, eg societal benefit, should be responsible for the assessment.

INTRODUCTION

Bristol-Myers Squibb (BMS) is a global BioPharma company whose mission is to discover, develop and deliver innovative medicines that help patients prevail over serious diseases. Around the world, our medicines help millions of people in their fight against cancer, cardiovascular disease, diabetes, hepatitis B, HIV/AIDS, rheumatoid arthritis and psychiatric disorders. BMS has a significant presence in the UK, with sites in Uxbridge and the North West of England and a comprehensive clinical trial programme, in all phases of development, taking place across the country.

BMS welcomes this inquiry into the work of NICE prior to the appointment of a new Chair. BMS is a member of the Association of the British Pharmaceutical Industry (ABPI) and has been involved in the development of the ABPI's submission to this inquiry. BMS supports the ABPI's submission.

To avoid duplication we outline below some additional points that we wish to be considered.

NICE'S ROLE IN RELATION TO EVALUATING THE EFFECTIVENESS AND COST-EFFECTIVENESS OF DRUGS

NICE has a significant impact on access to medicines. In the 13 years since it was created, NICE has conducted ground-breaking work and has established itself as an internationally recognised leader for HTAs. During this time it has also demonstrated that it is able to adapt to change and has become more flexible. BMS has an active and constructive relationship with NICE, and represents a substantial part of NICE's workload. However, we believe that this is an opportunity to make significant improvements to the roles and responsibilities of NICE.

Focus on cost-effectiveness as an assessment of value

NICE's "guide to the methods of technology assessment"⁵⁵ states that in undertaking appraisals, NICE is expected to take into account directions from the Secretary of State for Health as follows:

- The broad balance of clinical benefits and costs.
- The degree of clinical need of patients with the condition of disease under consideration.
- Any guidance issued to the NHS by the Secretary of State that is specifically drawn to the attention of the Institute by the Secretary of State and any guidance issued by the Secretary of State.
- The potential for long-term benefits to the NHS of innovation.

However, NICE's current approach to HTAs focuses mainly on cost-effectiveness rather than the broader aspects required by its methods guide.

⁵⁵ National Institute for Health and Clinical Excellence. *Guide to the methods of technology appraisal*. June 2008.

BMS believes that if NICE were to follow these directions more closely, its approach would provide a more balanced assessment of the various elements of value. It should further hold its appraisal committees accountable to ensure that all aspects of value are fully considered in decision making processes.

Value assessment vs. cost-minimisation

As highlighted above, NICE's methods guide requires NICE to conduct a broad value assessment of the value of medicines.⁵⁶ Whilst we above criticise that NICE's appraisal committees focus too much on cost-effectiveness, we would like to draw the attention here to these committees use cost-minimisation arguments forcing manufacturers to submit ever increasing patient access schemes. By doing so, these committees are straying outside their remit.

BMS believes that NICE's appraisal committees should recognise the value of the medicines they assess and not just oversimplify a decision problem by applying cost-minimisation.

Dealing with uncertainty

Uncertainty in science, health and economics is pervasive, entering the technology assessment process at every stage.⁵⁷ Data on new medicines is by definition immature, and any economic evaluation using such data results in wide ranges of cost-effectiveness results. The methodology to address such uncertainty has evolved substantially and is still evolving. This adds to the complexity of appraisals itself, but has also the potential to increase the ranges of cost-effectiveness.

BMS believes that NICE is becoming increasingly conservative when judging uncertainty. Furthermore, the academic groups undertaking the work to inform NICE apply increasingly complex methodologies that move further away from clinical decision making. In addition to increasing uncertainties, this also increases the burden for manufacturers of medicines developing HTA submissions. By not accepting a greater level of uncertainty about innovations to addressing serious diseases, there is a risk to miss innovations that set a foundation as standard of care for future therapeutic advances.

We would welcome a more pragmatic approach to decision making, ie accepting less complex approaches to address uncertainty, which balances the needs of physicians, patients and payers.

Choice of comparators

HTAs require that a new medicine has to demonstrate cost-effectiveness to specified comparator treatments. The choice of comparator is therefore critical. NICE's reference case, as laid out in its methods guide, states that the comparators used in appraisals should be therapies routinely used in the NHS, including technologies regarded as current best practice.

Some new medicines enter therapeutic areas where no treatment advances have been made for a long time. In such cases generic medicines become the comparators of choice, making it impossible for the new medicine to demonstrate cost-effectiveness at a price that allows the manufacturer a fair return on investment. BMS believes the comparators used within HTAs should represent current standard of care. Comparators which are not standard of care not reflecting clinical practice should not be used with the sole purpose of putting pressure on innovative medicines manufacturers to decrease their prices.

Furthermore, where economic evaluations are conducted, HTAs should consider the short, medium and longer term implications of innovation and not undervalue the cost of bringing new innovations to market and the benefits that result from these innovations for patients and society. If older technologies represent the standard of care and serve as the comparison for new technologies, then efforts should be made to consider the incremental cost of bringing the new innovation to market and the short and long-term value such innovations provide so that comparisons are meaningful.

Lack of governance of academic assessment groups and appraisal committees

NICE's methods guide⁵⁵ states that the appraisal committees are independent advisory bodies. These committees are subsequently informed by independent academic groups commissioned by the NIHR, not NICE. Therefore decisions are not accountable and the NICE Executive appears to have no remit to challenge the decisions made by its committees. This independence also creates a climate in which individual committee members with strong views can drive decision making in an inappropriate and unbalanced direction.

BMS believes that the appraisal committees and academic review groups should be held accountable by a clear governance hierarchy.

⁵⁶ Ibid

⁵⁷ Briggs A, Gray A. *Handling uncertainty when undertaking economic evaluation of health care interventions*. Health Technology Assessment 1999; Vol. 3: No. 2

Appeal process

BMS welcomes the recent changes to the make-up and appointment of the appeal panel announced by the Department of Health. However, we believe, that when an appeal is upheld, the appraisal should be referred to a different appraisal committee than the one which undertook the original appraisal. Furthermore, we also believe that, when an appraisal goes through a rapid review of the original appraisal decision, for example after the submission of a patient access scheme, manufacturers should have the right to have the appraisal referred to a different committee than the one which undertook the original appraisal.

Slow implementation of NICE guidance

BMS welcomes the Government's initiatives to speed up the uptake of new medicines. However, uptake of positive NICE guidance remains slow and variable across the country. We believe stronger incentives should be put into place to encourage NHS organisations to make NICE recommended medicines available to those patients who would benefit from them.

NICE International

NICE International recently developed a health technology cost-effectiveness threshold for Romanian authorities at three times GDP per capita, ie £23,000/QALY vs. NICE's £30,000/QALY for non-cancer medicines and £50,000/QALY for End-Of-Life medicines. This threshold is extremely low and has far-reaching consequences on medicines pricing decisions internationally.

NICE international is now also involved in a number of projects in China and South America.

BMS supports NICE offering its expertise on aspects of its work where it is a recognized leader, for example in the methods of HTAs. However, BMS does not support projects such as that undertaken in Romania where spurious methodology has been used to fundamentally undermine the value of medicines. We therefore request that the work of NICE International is re-considered.

NICE quality standards

NICE's quality standards are developed independently by NICE, in collaboration with NHS and social care professionals, their partners and service users.

They are central to supporting the Government's vision for an NHS and Social Care system focussed on delivering the best possible outcomes for people who use services. Therefore BMS believes that, as in HTAs, that is important to take into account a broad assessment of value, which is not only focusing on resource implications, but on the best outcomes.

HOW NICE'S ROLE WILL BE AFFECTED BY THE INTRODUCTION OF VALUE BASED PRICING (VBP)

The development of value-based pricing (VBP) in the UK could be a significant opportunity if it secures improved access for patients to new medicines and allows the wider aspects of the value of medicines to be captured fully.

BMS is pleased that VBP is intended to broaden the definition of the value of medicines to include consideration of the burden of disease and unmet need, along with therapeutic improvement/innovation and societal benefits. However, the detail of how this system will work has yet to be determined. BMS would be supportive of the introduction of weightings within the system for these additional considerations, but the current thresholds within the system should not be lowered, as suggested by the recent work funded by the Medical Research Council/National Institute for Health Research.⁵⁸ We believe that where value assessments are conducted, these should allow for an appropriate recognition of innovation. In contrast to Claxton and colleagues, we found that medicines that offer a step change in innovation are worthy of a higher cost-effectiveness threshold.⁵⁹

- Additionally, it will be important that the system is able to recognise incremental innovation as well as breakthrough innovation. Incremental innovation is more common and can often lead to subsequent breakthroughs, for example, combination therapies, which would not have been achieved in one step. The system needs to have sufficient flexibility to recognise innovations even where the evidence is limited initially. Additionally, BMS believes that organizations which have the strongest expertise in assessing broader aspects of value beyond HTAs should be made accountable for these.

October 2012

⁵⁸ Claxton K, Martin S, Soares M, et al. *Methods for the estimation for the NIOCE cost-effectiveness threshold*. Draft final report. 24 October 2012.

⁵⁹ *Assessing innovation of end-of-life treatments appraised by NICE*. Hutchings A, Harris M, Pericleous L, Batty A, Briggs A, Prenzler A, Lebmeier M. June 2012, 9th HTAi annual meeting, Bilbao, Spain

Written Evidence from the British Association for Counselling and Psychotherapy (NICE 39)

1. EXECUTIVE SUMMARY

- The NICE guideline development process, which makes recommendations about the commissioning of cost effective treatments, is a robust process and an important aid to decision making.
- The current hierarchy of evidence utilised by NICE results in guidelines based on a high quality but narrow evidence base. BACP believes that, in addition to the findings of RCTs, high quality practice-based evidence should inform the NICE review process.
- NICE needs to review its evidence evaluation process to admit a range of quantitative and qualitative evidence in the evaluation of psychological therapies, including controlled studies, case studies and effectiveness studies.
- NICE's limited evidence base for psychological therapies disadvantages patients through restricting patient choice and access to a range of interventions. It currently leads to an over emphasis on cognitive behavioural therapy (CBT) for patients with depression or anxiety.
- In the context of the new commissioning environment, NICE guidelines should be used to inform and not replace clinical judgment.
- Future development groups set up by NICE for mental health guidelines should have a broader balance and cross-section of professional stakeholders and peer reviewers to try to prevent researcher/intervention-allegiance bias.
- The Department of Health should work with NICE, the professional bodies in psychological therapies and mental health charities, to agree a national research programme, which identifies the gaps in the evidence.
- A better system of monitoring of whether clinical guidelines are having beneficial impacts on patient care should be developed including the DH, professional bodies, mental health charities and NICE.

2. INQUIRY RESPONSE

The Health and Social Care Act 2012 has introduced a large-scale reorganisation of the NHS against a backdrop of rising demand, changing need, efficiency drives and economic pressures. The Government has devolved decision-making for health and care to new local leaders, namely local authorities and GP-led Clinical Commissioning Groups.

Evidence-based psychological therapies are an important part of the delivery of health care within this new commissioning environment. The NICE guideline development process, which makes recommendations about the commissioning of cost effective treatments, is a robust process and an important aid to decision making. The inclusion of guidelines on mental health and behavioural conditions within the wider framework for evaluation by NICE means that treatment of mental health problems has directly benefited from additional investments in service development and education.

Within the new NHS structure it is unclear how effective the mechanisms designed to encourage the commissioning of psychological therapies over other local health priorities will be. NICE guidelines and quality standards will be used as a basis for new commissioning strategies or for re-designing existing psychological therapies. Commissioners of NHS services may quite reasonably look to guidelines from NICE to inform their decisions.

NICE's rigorous evidence review process puts great value on randomised controlled trials (RCTs) and systematic reviews, which are costly to carry out. Without the resources of the pharmaceutical industry, with competing priorities for limited health research funding and with no specific monies for mental health research there is consequently limited evidence for NICE to draw upon when developing its guidelines. As a result, the guidelines that do exist, and underpin the Improving Access to Psychological Therapies (IAPT) programme, lean heavily on a narrow evidence base which privileges cognitive behavioural therapy (CBT), an effective intervention which enjoys a robust evidence base.

CBT lends itself to RCTs, as a manualised treatment that fits with the biomedical paradigm. A key factor in the efficacy of treatment which is hard to capture in trials is the importance of the therapist-patient relationship; the influence of this tends to be best captured by qualitative research and case studies.

The heavy focus of recommendations in NICE guidelines on CBT results in a narrow range of treatments that patients can access, and therefore less patient choice and access to a limited range of interventions. Studies have shown that giving patients a choice of treatment can itself contribute to the recovery process. CBT is a groundbreaking technology and has the capacity to help great numbers of people, but it can be unsuitable for some people and some conditions (Enright, 1997). Routine outcome data collection and analysis has shown that counselling for depression is equally as effective as CBT (Glover, Webb & Evison, 2010), but this is not reflected in guidance.

Thus the NICE approach can underplay the importance of methodologies that complement trial data by assessing—through audit, benchmarking and quality evaluation, whether and how a treatment works in practice. Studies that show a therapy can work in the trial context ought to be complemented by other methodologies (such as audit and benchmarking) that can assure that their delivery in routine settings is still producing positive outcomes.

NICE does acknowledge the limitations to its guidelines and that “they are not a substitute for professional knowledge and clinical judgement”. Commissioners can therefore still design psychological therapy services that are evidence based and use a broader range of treatments than those recommended in NICE’s guidelines. If commissioners look beyond NICE guidelines when designing services, they must still ensure that the practitioners providing treatments are competent and ethical. All BACP members sign up to an ethical framework, which informs their practice and BACP promotes an accreditation scheme which identifies those of its members who have demonstrated mature competence in counselling and psychotherapy training and practice. Commissioners can use this system as a robust basis for determining which practitioners are suitable for the delivery of NHS-funded services.

RECOMMENDATIONS

The Government should set up a review of the evidence hierarchy which NICE relies on for its guidelines on mental health and behavioural conditions, to investigate the impact of current criteria for evaluating research into psychological therapies and consequent clinical guidelines on patient choice, innovative services, and patient care.

The relationship between RCT evidence and systematic data collection from routine settings (audit, benchmarking, quality evaluation) and the role of qualitative research need to be reviewed in order to improve the NICE evaluation process, so as to make NICE guidelines relevant and applicable to the NHS.

Future guideline development groups set up by NICE for mental health guidelines should involve a combination of expert clinical researchers, clinicians, service users, carers and methodologists. It is important that individuals who contribute to guideline development groups are appointed on the basis of their specific expertise. These appointments should be transparent and decided by elected representatives from the stakeholder organisations.

The Department of Health should work with NICE, the professional bodies in psychological therapies and the mental health charities, to agree a national research programme, which identifies the gaps in the evidence (across all the guidelines mental health and behavioural conditions), and priorities for research, and provide funding for these to be undertaken as an important part of the development and implementation programme for NICE guidelines.

NICE and the Department of Health should work with the professional bodies, with research departments for psychological therapies and with mental health research charities to establish an evaluation and audit infrastructure within NHS services which will enable ongoing improvements in practice, and better monitoring of whether clinical guidelines are having beneficial impacts on patient care.

3. BRITISH ASSOCIATION FOR COUNSELLING AND PSYCHOTHERAPY

As way of background, The British Association for Counselling and Psychotherapy (BACP) is the leading and largest professional body for counselling and psychotherapy in Europe, with a membership of over 38,000 practitioners, drawn from the various professional disciplines in the field of counselling and psychotherapy and based in a range of settings.

All BACP members are bound by the Ethical Framework for Good Practice for Counselling and Psychotherapy and within this, the Professional Conduct Procedure.

BACP is a regular respondent to NICE guideline consultations and has worked with NICE as a consultee in their development of mental health guidelines as well as a representative on NICE Scoping and Guideline Development Groups.

We use our experience as well as our research expertise and the knowledge of our practitioner members to assist the NICE in developing workable and evidence-based guidelines.

4. FURTHER INFORMATION

Should the Committee be seeking further oral evidence, BACP would be delighted to provide additional information about the development and impact of NICE guidelines on the delivery of psychological therapies.

5. REFERENCES

Enright SJ. Cognitive-behaviour therapy-clinical applications. *BMJ* 1997;314:1811–16

Glover G, Webb M, Evison F. Improving access to psychological therapies: a review of the progress made by sites in the first roll-out year. July 2010. <http://www.iapt.nhs.uk/wp-content/uploads/iapt-year-onesites-data-review-final-report.pdf>

October 2012

Written evidence from the British Association for Behavioural & Cognitive Psychotherapies (NICE 40)

1. *NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS*

BABCP is strongly in favour of maintaining the role of NICE in evaluating the effectiveness and cost effectiveness of drugs and clinical interventions.

The inclusion of mental health and psychological therapies within the wider framework for evaluation by NICE means that treatment of mental health difficulties has directly benefited from additional investments in service development and education.

Thus NICE has had a very positive impact on the overall provision of psychological therapy and mental health services within England. This has had the effect of increasing patient choice of effective treatments for mental health problems.

In addition, the comprehensive approach which NICE has taken in relation to mental health problems is very welcome. This has helped identify high quality research on psychological therapies and overall has had a positive effect on the provision of a range of treatments for severe mental illnesses eg schizophrenia, bipolar disorder

NICE guidelines have also highlighted the importance of recognising the psychological impacts of long term and acute physical health problems and of providing psychological care to patients alongside treatment for their physical health problems.

BABCP does not have a specific view about the introduction of value based pricing for drugs because BABCP is concerned specifically with the development and evaluation of Cognitive Behaviour Therapy.

2. *The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities*

BABCP welcomes the use of the NICE Quality Standards in guiding NHS practice, including commission.

NICE are in a strong position to impact positively on commissioning as they operate within an evidence based system and recommend best practice.

3. *The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome*

BABCP strongly supports the methods of critical appraisal used by NICE to evaluate the evidence base for healthcare intervention. NICE methodological standards are internationally recognised as maximising the validity and generalizability of research to enhance patient care and outcomes. BABCP also commends NICE on their transparent processes of evidence appraisal and their systematic methods for engaging with professional stakeholders and service user/carer groups.

4. *The effect of the new public health system architecture on NICE's continued role in respect of public health guidance*

BABCP does not have a specific view on the role of NICE in relation to public health guidance. However, BABCP is of the view that NHS practice should be based on transparent, robust and systematic reviews of the best available evidence.

5. *What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services*

In general BABCP welcomes this broader role for NICE. This has the potential to improve research appraisal in relation to social care interventions and to introduce new ways of evaluating social care research methods.

Using similar or equivalent standards to evaluate interventions across the health and social care domains to evaluate effectiveness and cost effectiveness has the potential to help integration of services.

October 2012

Written evidence from Novo Nordisk (NICE 41)

1. INTRODUCTION

1.1 Novo Nordisk welcomes the opportunity to respond to the Health Select Committee's inquiry into NICE. Novo Nordisk has nearly 90 years of experience in developing medicines for people with diabetes. Our ambition is to defeat diabetes by driving better outcomes for patients and for the National Health Service (NHS). Novo Nordisk is committed to working in collaboration with the NHS and NICE to help improve the quality of care for patients with long term conditions, including people with diabetes. In addition, Novo Nordisk has leading positions within haemophilia care, growth hormone therapy and hormone replacement therapy.

1.2 In this response, we have concentrated only on those areas of the Committee's inquiry where we feel that we have a particular contribution or comment to make.

1.3 We have also contributed to the response submitted by the Ethical Medicines Industry Group (EMIG) to this inquiry. EMIG is the trade association that represents the interests of small to medium-sized pharmaceutical companies in the United Kingdom.

2. NICE'S ROLE IN RELATION TO EVALUATING THE EFFECTIVENESS AND COST-EFFECTIVENESS OF DRUGS

2.1 We value the role that NICE plays in assessing the cost-effectiveness of medicines and have found NICE to be a constructive partner in work to ensure that patients are able to receive new treatments that are appropriate to them which will help improve health outcomes.

2.2 The current economic environment has resulted in considerable financial pressures on the NHS and on its partners. There is a potential risk associated to this in that the evaluation mechanisms used by NICE could run counter to what should be their key objectives: identifying medicines that bring the greatest benefit to patients, ensuring early access to these medicines and allowing choice among medicines of value. There are examples of health technology assessment which have led to increased access for patient to medicines in multiple areas such as diabetes, cancer and cardiovascular diseases but we are concerned that cost pressures could lead to cost-effectiveness decisions being made on a narrow basis.

2.3 In its methodology, we are keen to see that NICE demonstrates a comprehensive understanding of the full range of benefits of a medicine in disease management. We believe therefore that the perspectives on a medicine's value which are considered should be broad. Even where added benefit is identified objectively on the basis of agreed criteria, it may not be taken sufficiently into consideration within the framework of disease management needs and priorities. A comprehensive look, involving the opinions and experiences of the medical profession and individual patients is needed to identify where unmet need in the real-life management of a particular disease exists, how quality treatment can be increased and how the optimal, appropriate and efficient use of a medicine can be ensured.

2.4 In addition, evaluations of cost-effectiveness should take into account the indirect benefits of a new therapy, such as productivity gains, and reduction in carer and personal time costs over the long-term.

2.5 It is important that all stakeholders who have an interest in guidance being developed by NICE—including patients and industry—are involved in an early and inclusive dialogue. This inclusive process should allow at least an advisory function for patients and the pharmaceutical industry. The views, experiences and expertise of patients must be integrated into the evaluation process to allow for a better evaluation of the balance between benefits, costs and risk.

2.6 Assessment of highly specialised medicines by NICE

2.7 We wanted to draw the Committee's attention to the forthcoming expansion of NICE's role to take on the assessment of very high-cost, low-volume drugs from April 2013 when the Advisory Group on National Specialised Services (AGNSS) will cease this role.⁶⁰

2.8 We have some concerns about NICE's ability to assimilate the unique considerations around drugs for highly specialised services into its appraisals. This is because the current cost per quality-adjusted life year that NICE operates for its appraisals of drugs would rule out highly expensive drugs for small numbers of people with rare conditions. Therefore, we welcome the commitment made by the Department of Health in July 2012 that *NICE should build on the decision-making framework that AGNSS has developed to ensure that the needs of people with rare and very rare conditions are properly considered.*⁶¹

⁶⁰ Department of Health, *NICE to assess high cost low volume drugs*, press release, 19 July 2012, accessed on 20 October 2012 online via <http://www.dh.gov.uk/health/2012/07/very-high-cost-drugs/>

⁶¹ Hansard HL Deb 18 July 2012, vol 739, col 307, accessed on 20 October 2012 online via <http://www.publications.parliament.uk/pa/ld201213/ldhansrd/text/120718-0002.htm#120718132000263>

2.9 We would welcome the Committee's interest in this area which, although it is relatively small in terms of patient numbers the impact on the health outcomes for the patients concerned is significant. We would invite the Committee to ensure that the framework developed by NICE balances health gain, best clinical practice, societal value and reasonable cost and that recommendations from NICE will not be based solely on a cost per QALY figure.

2.10 Evidence summaries of new medicines

2.11 We would also invite the Committee to consider how evidence summaries of medicines, which NICE has recently started to develop, are being used within the NHS.⁶² The intended purpose of these summaries is that they will support commissioners, budget holders and other health professional to make decisions and aid local planning around the introduction of key new medicines or of existing medicines with new indications.

2.12 Although these summaries of evidence for selected new medicines, or existing medicines with new indications, are not formal guidance they could be influential in local decisions about whether patients can access certain medicine. These summaries will not be compiled with the same rigour as a technology appraisal. Therefore, the methodology used to select evidence which should be included within them merits further consideration, and the scope of evidence which is considered eligible for inclusion could be broadened to support decisions better.

2.13 Quality-adjusted life years (QALYs)

2.14 The Committee will be aware that research work is being carried out at the University of York into whether the QALY threshold utilised by NICE in its cost effectiveness analysis should be altered to address future healthcare cost pressures. There has been some discussion whether this work could potentially lead to differential QALY thresholds being used in the future for different conditions. This is an important area which would benefit from consideration by the Committee in its work.

2.15 Implementation of NICE guidelines and guidance

2.16 From our own discussions with healthcare professionals and our partners in the NHS we understand that there are some concerns around the costs associated with implementing some measures within NICE guidelines. For example, NICE's public health guidance on preventing type 2 diabetes⁶³ (*Preventing type 2 diabetes: population and community-level interventions*) suggests that there should be a widening of screening and health checks. These are very sensible recommendations which we support but commissioners and managers in the NHS may benefit from more detailed guidance on the cost impacts of implementing these measures.

2.17 The role of NICE Quality Standards in the new NHS system

2.18 Novo Nordisk supports the development of quality standards by NICE. As clear and succinct markers of high-quality, patient care, quality standards should be a very useful resource for both professionals and patients to help drive and measure quality improvements.

2.19 The *Quality standard for diabetes in adults*⁶⁴ was published in March 2011, and was one of the first quality standards to be introduced in the NHS. Novo Nordisk supports the fact that the condition has been prioritised in this way and hopes that this quality standard will make a significant contribution to addressing the burden of diabetes.

2.20 Diabetes is a chronic condition which leads to high blood sugar levels which can have serious implications for the long-term health of people. 2.9 million people in the UK are currently affected by diabetes.⁶⁵ In addition, it is estimated that about 850,000 people in the UK have the condition but are unaware they are suffering from diabetes, during which the body is being damaged by excess blood glucose.⁶⁶

2.21 The prevalence of diabetes is also rapidly increasing. Changes to lifestyle patterns, dietary factors and reduced physical activity are all associated with the rise in diabetes.⁶⁷ It is estimated that by 2025 around 5 million people in the UK will have diabetes.⁶⁸ In addition, there are striking variations in the provision of diabetes services across England. This is clearly evident in the *NHS Atlas of Variations for People with Diabetes* which shows that there are considerable differences in processes and outcomes of care. For example, fewer

⁶² National Institute for Health and Clinical Excellence, *Evidence summaries: new medicines*, NICE website, accessed on 20 October 2012 online via <http://www.nice.org.uk/mpc/evidencesummariesnewmedicines/home.jsp>

⁶³ National Institute for Health and Clinical Evidence, *Preventing type 2 diabetes: population and community-level interventions (PH35)*, May 2011, accessed online on 26 October via <http://publications.nice.org.uk/preventing-type-2-diabetes-population-and-community-level-interventions-ph35>

⁶⁴ National Institute for Health and Clinical Evidence, *Quality standard for diabetes in adults*, March 2011, accessed on 20 October 2012 online via <http://www.nice.org.uk/media/FCF/87/DiabetesInAdultsQualityStandard.pdf>

⁶⁵ Diabetes UK, *Diabetes in the UK 2011/2012: Key statistics on diabetes*, December 2011, accessed on 20 October 2012 online via <http://www.diabetes.org.uk/Documents/Reports/Diabetes-in-the-UK-2011-12.pdf>

⁶⁶ Diabetes UK, *Understanding diabetes—your essential guide*, June 2011, accessed on 20 October 2012 online via https://www.diabetes.org.uk/upload/How%20we%20help/catalogue/Understanding%20diabetes_Jan12.pdf

⁶⁷ International Diabetes Federation, *Diabetes Atlas, fifth edition*, accessed on 20 October 2012 online via <http://www.idf.org/diabetesatlas/5e/what-is-diabetes>

⁶⁸ Diabetes UK, *Diabetes in the UK 2011/2012: Key statistics on diabetes*, December 2011, accessed on 20 October 2012 online via <http://www.diabetes.org.uk/Documents/Reports/Diabetes-in-the-UK-2011-12.pdf>

than 53% of people with type 2 diabetes⁶⁹ received all nine key care processes recommended by NICE guidance.^{70,71}

2.22 There is not yet sufficient evidence setting out how the *Quality standard for diabetes in adults* has been implemented by commissioners, providers and healthcare professionals. Therefore, we would support the initiation of an audit of the implementation of the *Quality standard for diabetes in adults* to create a clearer picture of how it is being used across the NHS and how effective it has been in reducing variations in care. In addition, it would be beneficial to consider how this quality standard will evolve, how it will be used in the future and whether further guidance around its use would benefit both professionals and patients.

3. ABOUT NOVO NORDISK

3.1 Novo Nordisk is a global healthcare company with 89 years of innovation and leadership in diabetes care. The company also has leading positions within haemophilia care, growth hormone therapy and hormone replacement therapy. Headquartered in Denmark, Novo Nordisk employs about 32,500 people in 74 countries and markets its products in 179 countries.

October 2012

Written evidence from Roche Diagnostics Limited (NICE 42)

SUMMARY

- Despite commitment and joint working by NICE and Government with the medical technology industry sector, the adoption of innovative technology within the NHS remains poor, at a time when innovative approaches are key to “Nicholson’s Challenge.”

INTRODUCTION

1. The National Institute for Health and Clinical Excellence (NICE) defines diagnosis as, “the process of identifying whether the patient has a disease at the time of testing. It is performed for patients with specific complaints or in whom signs or symptoms have been noted that may indicate a disease. Tests can have several different uses in the process of diagnosis, for example:

- Ruling in or out a specific disease.
- General examination looking for clues to the cause of the symptoms.
- Staging, or additional testing to assess how advanced or severe the disease is.
- Monitoring a patient over time to determine changes in their condition.
- Screening tests to look for conditions in patients without signs or symptoms of the specific condition”.⁷²

NICE AND DIAGNOSTICS

2. It is recognised by Government that innovative medical devices and diagnostics have the potential to contribute to the delivery of significant improvements in health outcomes and in the patient experience.⁷³ NICE is central to ensuring that the case for the adoption of such devices and diagnostics by the NHS is supported by the evidence. However, the evaluation of medical devices and diagnostics presents a number of challenges; if testing is linked to treatment or can improve disease management, then it has “downstream” impact on the outcome of extended life and/or improved quality of life. Diagnostics also have an impact on patients’ utility outside the QALY-oriented health outcomes used to evaluate drugs and therapies. These aspects are not reflected in commissioners’ decisions, which are usually based on narrow “medical” quality of life measures.

3. The established evaluation process and criteria used for drugs and therapies is not considered appropriate by NICE or industry. NICE and industry representatives have been working together over a number years to develop and refine the evaluation process, the Medical Technologies Evaluation Programme (MTEP)—and in particular the Diagnostics Assessment Process (DAP) which focuses specifically on innovative diagnostics. NICE states that “the aims of the Diagnostic Assessment Programme are:

⁶⁹ NHS RightCare, *NHS Atlas of Variations in Healthcare for People with Diabetes*, 2012, accessed on 20 October 2012 via <http://www.rightcare.nhs.uk/index.php/atlas/diabetes/>

⁷⁰ National Institute for Health and Clinical Excellence, *Type 2 Diabetes—National clinical guideline for management in primary or secondary care*, (CG 66), May 2008 (updated by CG 87) accessed on 20 September via <http://guidance.nice.org.uk/CG66>

⁷¹ National Institute for Health and Clinical Evidence, *Quality Standards Programme: NICE cost impact and commissioning assessment for diabetes in adults*, March 2011. Available at URL: <http://www.nice.org.uk/media/109/95/costingcommissioningimpactassessmentfinal.pdf>
Last accessed: October 2012.

⁷² NICE website www.nice.org.uk accessed October 2012

⁷³ Correspondence between Earl Howe, Parliamentary Under-Secretary of State for Health and Roche Diagnostics 23rd January 2012

- to promote the rapid and consistent adoption of innovative clinically and cost-effective diagnostic technologies in the NHS;
- to improve treatment choice or the length and quality of life by evaluating diagnostic technologies that have the potential to improve key clinical decisions; and
- to improve the efficient use of NHS resources by evaluating diagnostic technologies that have the potential to improve systems and processes for the delivery of health and social care”.⁷⁴

4. DAP recognises that the evaluation of diagnostics differs from that of treatments, mainly because diagnostics do not have a direct impact on health outcomes. However, the current DAP approach does not allow the decision maker to consider a broad set of outcomes, including the value of information on patients’ conditions independent of health gains.

5. The MTEP and DAP identifies medical technologies that have the potential to offer substantial benefit to patients and/or the NHS; the premise for the programme is that such medical technologies are more likely to be adopted more consistently and more rapidly by the NHS if NICE develops guidance on them. Consistent and rapid adoption by the NHS however is rarely realised.

6. Roche Diagnostics Limited, alongside industry associations BIVDA and ABHI, has and continues to participate in a number of initiatives by both Government and NICE to try to address the poor uptake of NICE recommended technology. The commitment of NICE and Government (in particular the Office of Life Sciences) to addressing this issue is not in doubt and has been demonstrated for example by the initiatives identified in *Innovation, Health and Wealth: Acceleration adoption and diffusion in the NHS* (DH June 2012). Despite such initiatives, the inertia within the NHS with regards to adoption (which in many ways is shared by the QIPP programme) continues to frustrate that commitment.

7. The barriers to adoption are long established and include: silo budgets and financial incentives (the real world impact is not aligned to budgets), “day to day” realities of procurement, “real world” evidence.

8. In order to try to address the impact of financial incentives and silo budgets, each SHA holds a legal duty to promote innovation,⁷⁵ raising the profile of innovation and encouraging a more rapid adoption of innovation throughout the health service. “Innovation leads” are employed in each Authority to deliver this requirement. Supporting this legal duty, an Innovation Fund was created (worth £220 million over five years). It was envisaged that this fund would support faster innovation and more universal diffusion of best practice—innovation would be encouraged, recognised and rewarded. Whilst laudable, the results have been mixed and such an approach does not embed the uptake of innovative technology within the NHS.

“REAL WORLD” IMPACT AND VALUE-BASED PRICING

9. Whilst there has long been recognition of the social determinants of health (Chadwick, Booth Rowntree et al in the latter half of the C19th) in the context of the “modern NHS” it was “The report on Inequalities in Health Care” commissioned by Government in 1977⁷⁶ to address why the NHS had failed to reduce social inequalities in health. Despite the welfare state, there was evidence that social class difference with regard to health had widened. The expert group was chaired by Sir Douglas Black, former president of the Royal College of Physicians. The “Black Report” showed that there had continued to be an improvement in health across all the classes during the first 35 years of the National Health Service but there was still a correlation between social class and infant mortality rates, life expectancy and inequalities in the use of medical services. Similar findings were published by Sir Donald Acheson in 1998.

10. The DH and NICE in recent years have sought to address how to consider other values besides absolute health gain can be incorporated within assessments. Other sources of value such as distributional issues, and further social goals, are widely considered important by the public, yet do not explicitly figure in NICE assessments. If so, then from the public point of view—the taxpayers—the NHS is not achieving the best for its budget. The remedy for this problem is to incorporate a wider “societal perspective” which is driven by societal approval for policies that may diverge from maximizing total health gain.

11. The value-based pricing (VbP) proposal by the DH aims to reflect the values that are worth funding through the state in general and the NHS in particular

12. The VbP proposal should address some aspects of the existing narrow approach by considering other factors not related to the quality-adjusted life year (QALY), including: burden of disease (defined as unmet need and severity), the degree of therapeutic innovation, and health related benefits to patients not measured by the QALY.⁷⁷ The DH also advocates that a societal perspective should be taken, these might include benefits and costs outside health gain and health system costs such as productivity, tax revenues, welfare benefits, social inclusion and cohesion, reduction of inequalities and environmental impact.

⁷⁴ NICE DAP Manual (December 2011)

⁷⁵ High Quality Care for All, DH April 2009

⁷⁶ Inequalities in health: report of a research working group. (The Black Report). DHSS 1980

⁷⁷ A new value-based approach to the pricing of branded medicines: Government response to consultation. DH July 2011

13. In May 2012 at the annual NICE Conference, Andrew Lansley, Sec. of State said, “NICE will examine the evidence on the potential clinical and cost effectiveness of new drugs as they become available; drawing on its world-leading expertise in the field.

“And, importantly, under the new system of VbP, NICE will no longer be obliged to make yes/no decisions on access, based on its own cost per QALY thresholds.

“Instead, you’ll be free to focus on the rigorous appraisal of evidence to show the relative benefits of a new medicine.

“There will be price ranges under VBP, reflecting the contributory aspects of value, including the additional therapeutic benefits, the quality of innovation, the response to unmet need, and societal benefits.

The resulting pricing thresholds will be set as part of the VBP pricing mechanism, by Government, rather than by NICE.”

COMPANION DIAGNOSTIC TECHNOLOGY

14. NICE defines companion diagnostic technology as a “diagnostic technology that identifies people who are likely to benefit from a specific therapy for their condition. It may also help in stratifying disease status, selecting the proper medication and tailoring dosages to patients’ needs. In some cases, the use of companion diagnostic technologies may be necessary to comply with the licensed indications of pharmaceuticals.”

15. As long ago as 2007, Dr. Severin Schwan, Roche CEO declared that “For every single Pharma product... the Biomarker research and the development of potentially companion diagnostics is a standard part of the development process”. By then Roche had for several years been developing the Personalised Healthcare approach, “fitting the treatment to the patients”.

16. However, the process of evaluation and how best to fund the combination of diagnostic and therapy remains a challenge. Whilst the value of the test or therapy may separately (or may not all) have value, it will be less than when partnered. The matter is further complicated by the development of “in-house” tests by hospitals which may have different technical characteristics, including accuracy, which will compromise the initial general evidence and have an impact on the overall health gains of the combined diagnostic test and therapy. Such unregulated scenarios are a major concern and reduce the incentive to industry to produce the evidence to support such developments.

EVIDENCE

17. In addition to a different value proposition to that of drugs and therapies, there are other considerations that set medical technology and diagnostics apart, including:

- the medical technology industry currently has limited experience of health technology assessment;
- there are significant differences between devices, in-vitro diagnostics and imaging; and
- technical considerations (safety, compatibility, procurement, maintenance, calibration, training, upgrades) need careful consideration.

More significantly:

- typically the evidence base is light (compared to pharmaceutical assessments);
- there is generally a lack of studies linking diagnostic test results to final patient outcomes (clinical utility)—requires economic modelling to link intermediate outcomes (eg diagnostic test accuracy, test side effects, etc.) to patient outcomes (eg length and quality of life); and
- working with weak or absent data in estimating economic model parameters (eg data available but from a different population).

18. Against the background of the end of Professor Sir Michael Rawlins term of office as Chair of NICE and the expanding remit of the regulator to include public health and social care, the issue of evidence is key to the uptake of innovative technology. In 2008, Professor Sir Michael gave the Harveian Oration, “De Testimonio—On the evidence for decisions about the use of therapeutic interventions”. In it he concluded that “Experiment, observation and mathematics—individually and collectively—have a crucial role to play in providing the evidential basis for modern therapeutics. Arguments about the relative importance of each are an unnecessary distraction. Hierarchies of evidence should be replaced by accepting—indeed embracing—a diversity of approaches.”

CONCLUSION

19. The diversity of approach advocated by Professor Sir Michael is arguably key, not only with regard to the nature of evidence and the expanded remit of NICE but also to the culture within the NHS insofar as making decisions about the use of taxpayers’ money. Until the mind-set changes from one of “cut this budget” to “how do we improve value for money for the taxpayer”, the use of innovative technology and services will be seen by the NHS as too expensive an option. This is a shame as the NHS is the perfect “shop-window” to

demonstrate innovation services and technology to the rest of the world—a fact that the Prime Minister and Department of Business, Innovation and Skills are very aware of.

ABOUT ROCHE DIAGNOSTICS

20. Roche Diagnostics is the world's largest in vitro diagnostics company. We supply high quality products and services which are used to diagnose and monitor medical conditions and to facilitate medical research. The NHS carries out around two million tests on our equipment every day.

21. We have a track record of developing new and innovative devices and have around 1200 tests for medical use, many of which have had a radical impact on the way patients are treated. For example, we pioneered the development of meters used by people with diabetes to measure their blood glucose levels, and meters used by patients on long-term anticoagulant care to measure their blood clotting time. We have also developed a test to rule out heart failure and monitor the effectiveness of treatment, so patients can be tested by their doctor locally instead of having to go to hospital for tests. In addition to our diagnostic tests and devices, we have also developed Optimal Managed Laboratory Services which have been designed to support every aspect of a modern laboratory: clinical, operational, financial and strategic and operate in over 100 sites across UK and Ireland.

22. Roche Diagnostics is one of two core businesses in the wider Roche Group, the other being Roche Pharmaceuticals which is a leading supplier of medicines used in the treatment of cancer, virology and transplantation. Roche is committed to developing innovative solutions to address unmet medical needs. We have alliances and research and development agreements with numerous partners, and a presence in over 150 countries around the world. Further details can be found at: www.roche-Diagnostics.co.uk.

October 2012

Written evidence from the Association of British Healthcare Industries (NICE 43)

ABOUT THE ASSOCIATION OF BRITISH HEALTHCARE INDUSTRIES (ABHI)

The ABHI is the industry association for the medical technology sector in the UK. Our purpose is to promote the rapid adoption of medical technologies to ensure optimum patient outcomes throughout the UK and in key global markets. With over 250 company members ABHI is the voice of the medical technology sector, championing industry to the NHS, Government, regulators and stakeholders. The UK sector employs 64,000 people and has a turnover of £15 billion.

SUMMARY

1. Manufacturers of medical device technology require stronger intellectual property protection on the clinical evidence for their particular device type. This will lead to more willingness to invest in the development of evidence.

2. The incorporation of NICE guidance into the payment by results tariff—both technology appraisals and MTEP—would help manufacturers of medical device technology understand the value of the process and enable patient access to the appropriate technology.

3. Wider definitions of value—other than clinical and cost—should be adopted by NICE.

4. NICE requirements on the type and amount of clinical and cost effectiveness evidence are well suited to the pharmaceutical industry, but this does not always compliment manufacturers of medical device technology. Solutions to this problem require closer working between NICE and the medical technology industry.

INTRODUCTION

1.1 The ABHI welcomes the opportunity to contribute to the Health Select Committee's inquiry into the National Institute for Health and Clinical Excellence (NICE). NICE was established in 1999 to establish a means of assessing medicines, therapies and clinical practice on clinical and cost effectiveness grounds. Its role has continued to evolve and expand, and is now set to gain a number of new roles and responsibilities.

1.2 This submission addresses in particular the first point of the Select Committee's stated interest:

“NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS.”

MEDICAL TECHNOLOGIES EVALUATION PROGRAMME

1.3 Over the last three years NICE taken on board the specific task of appraising medical device technologies through the “Medical Technologies Evaluation Programme” (MTEP), which uses different methods from the long-established Technology Appraisal programme. MTEP was created in response to one of the recommendations of Lord Darzi's “High Quality Care for All” report in 2008:

“For new clinical technologies, we will simplify the way in which they pass from development into wider use by creating a single evaluation pathway, and will develop ways to benchmark and monitor their successful uptake.”⁷⁸

1.4 While the new process was designed to be a route for manufacturers to obtain approval for a single medical device or technology, there is concern that NICE have adopted requirements on the type and amount of clinical evidence that are better suited for the pharmaceutical industry. For example, a key question is how far the structure of NICE systems takes into account the impact on the market place of those products. A submission is required to show how the device might assimilate into existing NHS clinical pathways. However, in many cases the true value of a technology might only be realised through changing existing pathways. Thinking is well-established and widely discussed in the drugs field but arguably less so in medical device technology where modalities of operation are much more diverse than for pharmaceuticals.

1.5 The amount of clinical data required also presents a challenge for many manufacturers (especially small and medium sized enterprises). Medical technologies offering real promise but are at an early stage in their life cycles are likely to have limited clinical utility evidence.

1.6 This in turn raises questions about the hierarchy of evidence and how this will develop. Technology Appraisals have a Secretary of State funding direction and legal redress for patients under the NHS Constitution. Below that are Clinical Guidelines, and below that the Interventional Procedures programme which has no such impact and a review in two years before it can reach a technology appraisal. This is likely to become increasingly blurred as more guidelines are informed by TAs and as the Quality Standards library begins to assume greater significance in underpinning the NHS Outcomes Framework. Further, as the Medical Technologies and Diagnostics appraisal pathways develop and mature, there will be need to be a clearer place in the hierarchy for products which have been thus acknowledged as cost saving.

INTELLECTUAL PROPERTY

1.7 The approach taken through MTEP is of a single technology and is relatively blind to questions of equivalence of evidence. A manufacturer may secure approval through this channel and another manufacturer may wish to use the approval for their own technology, whether or not they have invested in evidence development and/or submitted to NICE directly themselves. Greater clarity is needed as to where value is identified by NICE and what the implications are for competing technologies.

1.8 This question arises in the context of competing technologies historically experiencing rapid price erosion, from which the NHS may benefit but which does not reward investment in evidence. It is arguable that stronger protection of evidence over a period of time would be of value in terms of encouraging further manufacturer investment in development. This would also help the NHS secure benefits of that technology, which could be quantified. At present, quantification happens only at the procurement stage, and as regards price paid per item, rather than any value being set on the commercial relationship in a way that would reward investment in evidence for improvement in clinical results.

NHS TECHNOLOGY ADOPTION

1.9 There continues to be wide debate about the extent of uptake of NICE approved medicines and technologies. Some of this was addressed in “Innovation, Health & Wealth” published in 2011.

1.10 This gap between assessment of value and how it is taken in the NHS is not helped by the existing relationship between NICE appraisal and assessment products and the NHS. There is a long term NHS failure to create any kind of linkage, transmission or gearing that leads firmly and in a timely way from appraisal—including Technology Appraisal for example—into “payment by results” tariff with its full coding and costing infrastructure. It is vital that this is addressed in creation of new tariff structures, whatever they may be.

1.11 As there is no mandatory element to NICE approval through the MTEP—nor direct guarantee of NHS purchasing—many SMEs have to weigh up the cost/benefits of participation.

RECOGNISING WIDER VALUE

1.12 The current NICE analysis—for both technology appraisals and through the MTEP—looks at clinical effectiveness, cost effectiveness and cost consequences. They only assess direct benefits to patients and the NHS. There are wider societal benefits such as the returning to work more quickly and less dependency on welfare and social care that should be taken into account as well as improved efficiency of delivery of care. Savings across a number of Government departments could be realised.

October 2012

⁷⁸ High Quality Care For All—NHS Next Stage Review Final Report—Department of Health 2008

Written evidence from Royal National Institute of Blind People (RNIB) (NICE 44)

1. ABOUT ROYAL NATIONAL INSTITUTE OF BLIND PEOPLE (RNIB)

1.1 RNIB is the largest organisation of blind and partially sighted people in the UK. We are a membership organisation with over 10,000 members who are blind, partially sighted or the friends and family of people with sight loss. Eighty% of our Trustees and Assembly Members are blind or partially sighted. We encourage members to get involved in our work and regularly consult with them on Government policy and their ideas for change.

1.2 As a campaigning organisation, we fight for the rights of people with sight loss in each of the four nations of the UK. This includes engaging with NICE to ensure patients get timely access to new treatments on the NHS; as well as making sure guidelines and Quality Standards are developed for conditions that impact on eye health.

1.3 RNIB is pleased to have the opportunity to respond to this consultation, and has provided comments on four out of the five questions posed by the Committee.

2. SUMMARY OF MAIN POINTS:

2.1 NICE deserves its reputation as an international leader in evaluating the cost-effectiveness of new medicines. It also produces evidence based guidelines and Quality Standards which help patients receive high quality care.

2.2 The following provides a summary of the key points we would like the Health Select Committee to consider as part of its inquiry:

- The role of the patient voice in technology appraisals is unclear and at times undervalued.
- NICE's Patient and Public Involvement Programme is very effective and an asset to the organisation.
- NICE's decision making can lack transparency.
- There is a serious problem with the implementation of NICE guidance.
- The development of the library of Quality Standards is worryingly slow.
- The forthcoming Value Based Pricing policy could disadvantage vulnerable groups.
- NICE guidance is not always updated at the specified regular intervals.
- NICE's move into social care provides the opportunity to join up health and social care guidance.
- NICE's expanding role could put a strain on the organisation's resources.

3. *NICE's role in relation to evaluating the effectiveness and cost effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS*

The patient voice in technology appraisals

3.1 The extent to which the patient voice can influence the outcome of a technology appraisal seems to be limited.

3.2 For example, in the recent NICE appraisal of Lucentis for the treatment of diabetic macular oedema (DMO) (rapid review of TA 237). The Committee recognised patient's concerns about the substantial negative impact DMO has on quality of life, especially in relation to loss of independence and employment. It also acknowledged that diabetes is managed with self-care and that visual impairment can affect a person's ability to manage their own condition. While RNIB welcomed these comments, it was clear that the clinical evidence and, more importantly, the manufacturer's revised Patient Access Scheme actually led to NICE issuing positive draft recommendations (on 5 October 2012) approving Lucentis in a sub-group of people with DMO.

3.3 NICE should issue clear guidance on where the patient voice can add the most value/have the most impact. It should also outline how the appraisal Committee uses patient input. Perhaps NICE could re-evaluate the importance it places on patient comments and how they could be given more weight.

Patient and Public Involvement Programme

3.4 NICE is to be applauded for its Patient and Public Involvement Programme. It offers free training to patient groups (to help them understand NICE and its processes) as well as assistance from a dedicated team.

3.5 Staff working in the Patient and Public Involvement Programme are extremely helpful and offer:

- advice on what to do during NICE appraisals (a valuable service for those new to NICE);
- a meet and greet service at appraisals (which is particularly important for individual patients who are often very nervous); and

- timely answers to enquiries, for example, “what does innovation mean in a NICE technology appraisal?”

NICE decision making and transparency

3.6 There are occasions when NICE could be more transparent about its decision making.

3.7 In terms of eye health, there is an unlicensed treatment (Avastin) which has been used as a comparator in several NICE technology appraisals. In the NICE guide to the methods of technology appraisal (June 2008) it states that: “relevant comparators are identified, with consideration given specifically to routine use and best practice in the NHS”. NICE has concluded that Avastin is in routine use in the treatment of several eye conditions; however, it has never defined what “routine use” actually means. This makes their decision making very subjective. We would like NICE to review this matter and issue a definition of “routine use”.

3.8 In a related point, we would also question whether using an unlicensed medicine as a comparator could be considered “best practice”. With Avastin, there are still question marks over the safety of its use in the eye. We feel that NICE is sending out the wrong signals using an unlicensed comparator and that this ultimately undermines the purpose of its guidance.

3.9 NICE’s use of the EQ-5D in calculating quality-adjusted life years (QALY) is inappropriate in appraisals dealing with sensory impairment. We believe NICE should review the way it calculates QALYs by considering other measures; for example, the VQF-25 could be used to assess the quality of life in patients with visual impairment.

3.10 NICE has made illogical decisions in the past. In the case of Lucentis for the treatment of wet Age-related Macular (wAMD) Degeneration, NICE initially said it could only be used by someone who had gone blind in one eye (June 2007). Following a public outcry, the organisation revisited the evidence and made an agreement with this manufacturer (Novartis agreed to pay the drug costs of any patient that needed more than 14 injections per eye). In August 2008, NICE re-issued guidance approving the use of Lucentis in NHS patients for the treatment of wAMD. This was a welcomed U-turn but it is important to note that during the time lag (between 2007 and 2008) wAMD patients had been left to lose their sight unnecessarily.

Lack of implementation of NICE guidance:

3.11 Implementation of NICE guidance is poor and there is no accountability in the system to ensure uptake is achieved. It is often left to patient groups to monitor and challenge PCTs/hospital trusts that are not implementing NICE approved treatments (see Ozurdex example below).

3.12 RNIB is aware that the NICE Implementation Collaborative (NIC) aims to help local NHS organisations implement NICE guidelines and spread best practice. However, it is unlikely that the NIC will have enough resource to really monitor what is going on and challenge poor implementation. We are also concerned that the new “scorecard” will mean that providers focus on implementing the latest NICE-approved drugs listed on the card and overlook other approved medicines.

Poor implementation of NICE guidance: Ozurdex case study

3.13 NICE approved a corticosteroid implant (Ozurdex) for the treatment of macula oedema following retinal vein occlusion in July 2011. This meant that PCTs and hospital trusts had until October 2011 to ensure a full Ozurdex service was in place and that NHS patients had access to the treatment.

3.14 In February 2012, RNIB wrote to all PCTs/hospital trusts to ask them whether an Ozurdex service was in place. Over 80% of PCTs responded to our request and we found that (as of April 2012):

- 12% had no service;
- 40% had a sub-standard service (often requiring each patient to complete an Individual Funding Request to get access to Ozurdex); and
- 48% had a full service.

3.15 Following conversations with ophthalmologists across the country, it appears that the reasons behind the failure to comply with the three month deadline are varied but include:

- delays in approving contracts and business cases to establish an Ozurdex service;
- miscommunication between PCTs and hospital trusts resulting in services not being set up; and
- continued use of an unlicensed drug (Avastin).

4. The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

Slow development of the library of Quality Standards

4.1 We are disappointed at the slow development of the NICE library of Quality Standards. NICE has informed RNIB that the cataract and macular degeneration standards will not be considered for development

before the 2014–2015 financial year; and that the full library of topics will not be complete before 2020. This is worrying as the standards are set to play a central role in the new commissioning system.

4.3 In addition, we are aware that arbitrary thresholds are being used to unfairly restrict access to cataract surgery. In the absence of the cataract Quality Standard for this condition, this situation is likely to get worse.

4.4 We are also concerned about the prioritisation of the development of the Quality Standards. It appears that NICE will produce a Quality Standard for a specific indication if there is an up-to-date NICE guideline in place. Diseases without guidelines (that possibly lack a strong evidence base) will be pushed to the bottom of the list. This is problematic as it means the development of standards will be based on process decisions rather than need. A lack of evidence does not mean a lack of importance—it could signal an urgent need to start collecting the evidence to ensure patients with a certain disease do not fall through the gaps.

Value Based Pricing (VBP) and vulnerable groups

4.5 We are concerned that VBP is not being developed in a transparent manner which allows stakeholders, such as patient groups, to be involved in the process. There is little information about the initiative which makes it hard for stakeholders to critique the scheme and outline how it may affect the groups they represent.

4.6 Of the small amount of information that has been issued, it appears that the policy may disadvantage certain groups—particularly those who do not directly contribute to the economy such as the elderly, children and those with learning disabilities. This needs urgent clarification and review.

4.7 We would expect the NHS Constitution and the funding directive to apply to new treatments that are approved under VBP.

5. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

5.1 We have noticed that clinical guidelines can be out of date—having not been revised at the specified interval (usually three years). This is problematic if the development of new Quality Standards rests on having a current NICE guideline in place.

6. What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

6.1 As NICE's role expands we are concerned that the organisation's resources (staff and financial) will not be sufficient enough to undertake all the work within its new remit.

6.2 Its move into social care provides an opportunity to produce integrated guidelines that join up health and social care. There are occasions in eye care where patients are treated in the hospital, discharged and not put in touch with the social services they need. Hopefully, integrated guidelines could prevent this from happening in the future.

October 2012

Written evidence from the Cystic Fibrosis Trust (NICE 45)

SUMMARY

- NICE should appraise all CF treatments and technologies to ensure access to treatments that offer better quality of life, improve treatment burden and improve outcomes.
- The NICE assessment process for appraising treatments remains prolonged and creates delays in getting effective treatments to the patient. NICE processes should ensure that effective treatments are made available quickly.
- The key factors in the AGNSS ethical decision making framework of health gain, best practice and societal value must be retained by NICE.

1. The Cystic Fibrosis Trust is the UK national charity for people living with cystic fibrosis (CF). The Trust funds world-leading medical research, ensures safe and appropriate clinical care, and offers direct support for people with CF and their families.

2. The Cystic Fibrosis Trust is a member of the Specialised Healthcare Alliance (SHCA) and supports the SHCA's submission to this inquiry.

3. CF is an inherited and progressive life-limiting disease which affects internal organs (particularly the lungs and digestive system) by clogging them with thick sticky mucus. This makes it harder to breathe and to digest food. The mucus in the lungs provides an ideal environment for pathogenic bacteria, promoting recurrent and increasingly frequent respiratory infections. There are over 9,000 people with CF in the UK and in 2010 the average age at death was only 29.

4. The Cystic Fibrosis Trust welcomes this inquiry into the work of National Institute for Health and Clinical Excellence (NICE). We are focusing our comment on NICE's role in appraising treatments for the whole CF patient population and the future role of NICE in appraising treatments for people with CF who have specific gene mutations, which classifies these treatments as 'ultra-orphan' treatments.

APPRAISING TREATMENTS FOR THE WHOLE CF POPULATION

5. The Cystic Fibrosis Trust welcomes the appraisal of CF treatments by NICE. So far very few treatments and technologies for CF have been appraised by NICE. This has led to regional variations in how treatments are prescribed and a lack of transparency about when and whether new treatments should be prescribed. Therefore there is a lack of consistency and confidence in prescribing to patients, resulting in a postcode lottery relating to CF treatments.

6. This postcode lottery is slowly being addressed through a number of processes, ie the implementation of a national tariff for CF from April 2013 and a national commissioning policy for CF specific drugs. A handful of CF treatments are currently being assessed by NICE. These include Mannitol (Bronchitol), colistimethate sodium and tobramycin, all dry powder inhalers.

7. There is an urgent need to make sure that a broader range of newly developed/developing, life enhancing treatments for CF are made available, particularly treatments that are quick and easy to use. People with CF have a huge burden of care often having to do hours of treatments and physiotherapy every day. Each treatment that becomes available and is proven to be effective in treating infections and symptoms of CF is a huge step forward in helping people with CF to stay well and live longer. The latest treatment and technologies must be appraised to ensure access to treatments that offer better quality of life, improve treatment burden and improve outcomes.

8. We are, however, concerned that the process for assessing treatments remains prolonged and creates delays in getting effective treatments to the patient. For example, Mannitol was licenced in October 2011 and we received the final recommendation on Friday 26 October 2012.⁷⁹ Tobramycin podhaler was licenced in September 2010⁸⁰ and the final recommendation will be issued in 2013. In contrast, the appraisal process for the new treatment, Kalydeco, carried out by the Clinical Priorities Advisory Group has been very swift. Kalydeco was licenced in July 2012 and a recommendation is expected in December 2012.

APPRAISING "ULTRA-ORPHAN" TREATMENTS

9. On 27 July a new revolutionary treatment was granted its licence in the EU. This treatment corrects the basic gene defect in people with CF who carry the G551D gene mutation. This affects around 250 people in England. Kalydeco (Ivacaftor) has been described as "a significant new step in managing cystic fibrosis" which "provides both a long term quality of life and survival benefit to patients."⁸¹

10. The NHS is currently in a transition period and there is no formal system in place for assessing treatments that affect under 500 patients, as the Advisory Group for Specialised Services (AGNSS) is in moratorium and NICE is unable to assess this treatment because the patient population is too small. It has therefore fallen to the four Specialised Commissioning Groups in England to devise a process for appraising this treatment.

11. A Health Technology Assessment (HTA) of Kalydeco was commissioned in July to consider the available evidence and provide an assessment of the clinical effectiveness. A Clinical Priorities Advisory Group (CPAG) met in September and agreed that Kalydeco provided clinical benefit but deferred discussion of the drug's cost effectiveness to a further meeting with Vertex at the end of October. This meeting will make recommendations to Specialised Commissioning Groups and Primary Care Trusts.

12. The Cystic Fibrosis Trust has welcomed the work of NHS commissioners and advisors in England to accelerate the system of approval, and their recognition of the drug's clinical benefit. We would like an approach such as this where committed experts have shown that timescales can be radically scaled down to less than six months to appraise a treatment.

13. As outlined in full in the SHCA submission, the Cystic Fibrosis Trust welcomes Earl Howe's announcement on 18 July 2012 that NICE, in its new role, would "build on the decision-making framework that AGNSS uses at the moment."⁸²

14. The Cystic Fibrosis Trust strongly welcomes this commitment from the Minister and supports calls for key factors in the AGNSS ethical decision making framework of health gain, best practice and societal value to be retained by NICE.

October 2012

⁷⁹ <http://www.pharmaxis.com.au/bronchitol>

⁸⁰ <http://www.novartis.com/newsroom/media-releases/en/2010/1446760.shtml>

⁸¹ Clinical Priorities Advisory Group Meeting, Minute of the Meeting held on Tuesday 25th September 2012

⁸² Hansard HL Deb 18 July 2012, vol 739, col 307

Written evidence from the National Infertility Awareness Campaign (NICE 46)

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

1.1 No comment.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

2.1 Holding Clinical Commissioning Groups (CCGs) to account will be one of the main functions of the NHS Commissioning Board (NHSCB) when it takes on its full statutory responsibilities in April 2013. The transposition of NICE Quality Standards into the Commissioning Outcomes Framework is welcomed by the National Infertility Awareness Campaign (NIAC). However we have serious concerns surrounding the future of NICE clinical guidelines.

2.2 The recommendations contained within NICE guidelines are intended to inform the commissioning decisions of local commissioners, yet too often they are ignored or implemented in a piecemeal fashion. This is perhaps more true for fertility treatment than any other NHS service.

2.3 The 2004 NICE guideline recommended that three full cycles of IVF be provided to eligible couples. Ever since its publication, NIAC has been seeking to increase adherence amongst commissioners. Significant progress has been made but even now, eight years after its publication, variations in provision are still widespread.

2.4 In 2011 the All Party Parliamentary Group on Infertility published a report entitled "Holding Back the British IVF Revolution". The report highlighted the fact that over 70 percent of Primary Care Trusts (PCTs) were not providing the recommended three cycles of IVF treatment to eligible couples.⁸³

2.5 NIAC routinely writes to those Primary Care Trusts (PCTs) who have suspended or reduced funding for fertility treatment. As a matter of course, we quote the recommendations contained within the 2004 NICE guideline. Many PCTs acknowledge the guideline and express their regret at not being able to "afford" three full cycles, whilst others simply state that the guideline is not mandatory. In addition, many PCTs are not adhering to the eligibility criteria as stated in the guideline with often arbitrary eligibility criteria being used instead to cut down the number of referrals for treatment.

2.6 In the words of the Health Select Committee, the "net effect in most PCTs is half-hearted endorsement of NICE guidelines, with agreement that it is the right direction of travel, but little active encouragement and especially where new resources are required."⁸⁴

2.7 NIAC accepts that financial constraints often prevent the immediate implementation of NICE guidelines but in the case of infertility, PCTs have been "working towards" full implementation for eight years. This is in our view, an unacceptable length of time.

2.8 That is not to say that progress has not been made in recent years. The 2004 guideline on fertility has had a marked effect in increasing awareness of infertility and there has been an overall increase in the number of cycles of IVF commissioned on an annual basis. We are however far from confident that this upward trend will continue. Investments by one PCT have in many cases been countered by disinvestments in another. Given that age is one of the most significant factors effecting IVF success rates, patients requiring treatment continue to be adversely affected by inconsistent, ever changing policies.

2.9 NIAC fears that without further impetus from Government, the new 2012 NICE guideline on fertility will suffer the same fate as its predecessor, that is to be an excellent guideline that is routinely ignored by the Service.

2.10 This could be prevented, in part, if greater importance was attached to central intervention. We believe that central intervention can play a key role in increasing adherence to NICE guidelines, especially in fields such as infertility where differences in provision are widespread. In 2010, NIAC witnessed a wave of disinvestments in IVF across the country. In early 2011, this wave was halted and in some cases reversed after the Deputy Chief Executive of the NHS wrote to PCTs to remind them of their responsibility to work towards full implementation of the 2004 NICE guideline on fertility.

2.11 We believe that similar reminders, on a regular basis, will be necessary to ensure greater uptake of the new guideline.

2.12 Another problem of recent years has been the lack of central monitoring of implementation. Last year's APPG on Infertility report had to base its findings on freedom of information requests. NIAC is often made aware of commissioning changes through press reports and from patients that have been denied treatment. This

⁸³ APPGI, *Holding Back the IVF Revolution* (June, 2011), available here: <http://www.infertilitynetworkuk.com/uploadedFiles/InfertilityAwareness/appg%20IVF%20report.pdf>

⁸⁴ Health Select Committee, First Report of the 2007–08 Session, paragraph 266: <http://www.publications.parliament.uk/pa/cm200708/cmselect/cmhealth/27/2702.htm>

not only causes great confusion and distress for patients but it also prevents NICE from tracking the implementation of its guidelines. Without this information, NICE cannot hope to accurately measure the effectiveness of the advice that it offers.

2.13 It would help enormously therefore if NICE or the NHSCB were to host a central database containing information relating to local provision. This way resources could be concentrated and assistance offered in those areas that are struggling the most to implement the guideline.

2.14 The recent announcements surrounding the NHS Scorecard system are a welcome development and should go a long way in increasing the transparency of commissioning decisions. It is our understanding that this would compel commissioners to publish the reasons why they are not following NICE guidance (we use the word “guidance” but it is our understanding that this scheme would cover NICE guidelines as well). In the case of IVF it would also help if commissioners were required to publish the number of cycles they provide and the eligibility criteria they employ as opposed to simply stating whether or not they follow the NICE guideline.

2.15 We have used the example of fertility to highlight the lack of adherence to NICE’s recommendations, but we are aware of other clinical guidelines that have also been implemented in a piecemeal fashion.

The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

3.1 With regard to variations in outcome, please see NIAC’s comments above.

The effect of the new public health system architecture on NICE’s continued role in respect of public health guidance

4.1 No comment.

What effect NICE’s new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

5.1 No comment.

October 2012

Written evidence from the Arthritis and Musculoskeletal Alliance (NICE 47)

1. INTRODUCTION

The Arthritis and Musculoskeletal Alliance (ARMA) is the umbrella body providing a collective voice for the arthritis and musculoskeletal community in the UK.

Together, ARMA and its member organisations work to improve quality of life for around 10 million people in the UK with these conditions.

We aim to foster co-operation between charities and professional organisations, working to enhance understanding and mutual support between individuals and organisational bodies.

2. SUMMARY

ARMA supports the future development of NICE as a body that

- Takes full account of existing expertise and engages meaningfully and proactively with all relevant stakeholders, including both professional and patient organisations.
- Takes much greater account of social factors and social benefits in its appraisals, and in defining “quality of life” drawing on existing evidence and the expertise of our community.
- Produces robust Quality Standards covering all main cohorts of MSDs, and clearly set out how these should be implemented in practice.
- Ensures that as its remit widens all quality standards and guidance take an integrated approach to health and social care.
- Works with other bodies such as the CQC and Monitor to ensure the monitoring and implementation of NICE guidance in the new Health and Social Care structure.

3. NICE’s role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

3.1 The place of both professional and patient organisations in the NICE TA process needs to be clear and quantifiable in terms of the scope of the role in influencing final TA decision.

3.2 In setting out final decisions on value-based pricing NICE should give a clear explanation of what factors have been considered and how these have informed TA decisions.

3.3 The appeal process should include the possibility of challenging the assumptions used by the TA where there is evidence to show that these are incorrect.

3.4 The introduction of value-based pricing should mean that NICE takes into account wider benefits than within its current remit. However the nature of the wider factors should be defined and explicit.

4. The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

4.1 It is significant that Musculoskeletal Diseases are the 4th largest area of spending in the NHS and each year 20% of the general population consults a GP about a Musculoskeletal disease, and yet there is no quality standard for MSKs and hence no COF for commissioning groups in this important area. As Quality Standards underpin the COF to which clinical commissioning groups are accountable there is a risk that MSK conditions will be given a lesser priority by CCGs.

4.2 It should be a priority for NICE to identify where there are gaps in quality standards and work patient and professional bodies to gather the evidence base and draw up standards accordingly.

4.3 NICE should produce robust Quality Standards covering all main cohorts of MSDs, and clearly set out how these should be implemented in practice.

4.4 There is presently a lack of clarity on the requirements in terms of implementing/adhering to NICE guidance in an increasingly devolved NHS, and how CCGs will be both supported and held to account for this.

5. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

5.1 Clinical guidelines cover all aspects of care from diagnosis to end of life care. As such it is essential that they should incorporate both the treatment and social care needs that a person might need at different times in the course of living with a particular condition. It is also essential that they are regularly updated in the light of evidence.

5.2 At present NICE Clinical Guidance does not address social care and although guidelines may be reviewed every three years they are not necessarily updated. Guidance currently also focuses on pathways that include only NICE approved treatments and exclude other licensed and effective treatments already in use.

6. What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

6.1 As it takes on new responsibilities in evaluating social care NICE should produce integrated quality standards and guidance that incorporates evidence from health and social care practice.

6.2 NICE should work with CQC and Monitor to ensure the monitoring and implementation of NICE guidance in the new Health and Social Care structure.

October 2012

Written evidence from Medtronic Ltd (NICE 48)

ABOUT MEDTRONIC

Medtronic is the global leader in medical technology, alleviating pain, restoring health and extending life for millions of people around the world. With deep roots in the treatment of heart disease, Medtronic now provides a wide range of products and therapies—every four seconds, somewhere in the world, another life is improved by a Medtronic product or therapy.

The company was founded in 1949 in Minneapolis, Minnesota, USA, by Earl E Bakken and Palmer J Hermundslie. In the UK, Medtronic has been based in Watford for over 25 years.

SUMMARY OF RECOMMENDATIONS

- NICE need to keep abreast of the medical device innovation landscape and ensure the assessment processes are fit for purpose.
- There needs to be a closer working relationship between NICE and industry when it comes to when technologies should be assessed.
- International clinical trial should be considered relevant for NICE submissions.
- NICE evaluation procedures should include appropriate methods for the assessment of devices.

- NICE should have role in developing an effective system of monitoring local compliance with Quality Standards.
- Re-establish the link between the NICE process and the National Horizon Scanning Centre in relation to medical technologies.

1. INTRODUCTION

1.1 Medtronic (MDT) welcomes the opportunity to contribute to the Health Select Committee inquiry on the role and work of NICE.

1.2 Since its inception NICE has become a commonly recognised NHS body which frequently receives a high media profile. It has also acquired a reputation as an international role model for the development of guidance on technologies and therapies. It was established to improve the quality and availability of care across England and Wales—within the limited resources of the NHS—by assessing the clinical and cost effectiveness of treatments. This has worked well. However, the mandatory element of the technology guidance developed by NICE is often not implemented by local Trusts, and enforcement of this is weak.

1.3 There have been seven NICE technology appraisals therapy areas in which Medtronic devices feature. In each of these areas, the device has yet to reach the levels of uptake recommended by NICE. There are several recommendations contained in Innovation, Health and Wealth (IHW) which seek to address this which are welcomed by MDT. While this cannot be seen as a failure of NICE, it is worth recognising that much of the value in improving the way it works and the guidance it produces will be wasted without effective implementation by local NHS Trusts and commissioners. It is therefore welcome that NICE will become responsible for creating an appropriate compliance regime.

1.4 This short submission will focus on those areas where we feel the work of NICE can improve. It will focus on the role of the organisation in relation to medical technologies.

2. KEEPING PACE WITH INNOVATION

2.1 Medical devices are evolving technologies that often change quickly. Improvements are characterised by a continuous and incremental product development process. A large proportion of the device types used in the NHS today will not have been available just two years ago, and each variation will often have a very short life-cycle. Unlike pharmaceutical products which remain as market leaders for extensive periods of time, medical device are regularly superseded by new innovations within short periods of time. This is an important way medical technologies differ from pharmaceutical products and this presents a challenge for NICE and for the industry.

2.2 If a technology is assessed too early, there is a risk NICE may not recognise the continuous improvements that are likely to develop—as well as the learning curve associated with the use of device. However, delays in assessment will limit the availability of the technology and the associated benefit for patients. The timing of assessment of innovations should be done on a case by case basis in collaboration with industry.

2.3 The National Institute for Health Research Horizon Scanning Centre (NHSC) has a remit to provide advanced notice to the Department of Health and NICE, of selected key new and emerging pharmaceuticals that might require evaluation due to meeting an unmet need or providing significant advances in therapies. This is particularly useful in ensuring that an identified product is quickly assessed by NICE and can allow pharmaceuticals to be planned into assessment waves. However, there are considerable differences in the way innovations are presented. Detailed “pharmaceutical technology briefings” are prepared in the case of drugs, which are used for national NHS priority setting and decision-making. NICE has broken the link which allows NHSC to horizon scan technologies and plan them into work programmes, instead relying on a proactive notification process rather than the active search process that pharmaceuticals receive. NICE do not use this route in the same way as ‘pharmaceutical technology briefings’ to identify future work streams. We recommend the re-establishment of the link with horizon scanning to level the playing field with pharmaceuticals.

3. ASSESSMENT APPROPRIATE FOR DEVICES

3.1 There have been too few technology appraisals focussing on medical technology and some may argue that pharmacological products have dominated the work of the agency in the past as they are easier to assess.

3.2 Renal denervation is a new technology to treat resistant hypertension where pharmacological management has failed to bring blood pressure down. This effects up to 500,000 people in the UK and is a leading cause of heart attack and stroke. The procedure has been used in over 4,000 patients, has evidence of efficacy up to three years and was even referenced in the Government’s Life Science strategy. It is inconceivable that a new drug which is launched and addressed an unmet medical need of 500,000 would not immediately receive a full NICE Technology Appraisal.

3.3 Unlike technology appraisals, the interventional procedures programme—under which many medical devices have been assessed—do not carry a mandatory duty for funding. Moreover the “Medical Technologies Evaluation Programme” (MTEP) gives no certainty of NHS adoption. This inequitable treatment for devices

when compared to pharmaceuticals, combined with the research costs and intellectual property risks is enormous barrier for the medical technology industry when engaging with NICE.

3.4 There is no incentive for medical device manufacturers to invest in the large amounts of clinical research required by the NICE evaluation system if another manufacturer can simply use this to justify the use of a similar technology without the associated cost and resource implications. This type of practice would not be tolerated within the pharmaceutical industry.

3.5 There are further features of the NICE assessment process which does not appropriately recognise the differences between the pharmaceutical and the medical technology sector. Medical technology is generally based on engineering rather than chemistry and a NICE evaluation system—which often focuses on evidence from randomised controlled trials—will not be appropriate or possible for all kinds of medical technology. Moreover, there are other factors such as susceptibility to inter-patient variation and clinical proficiency with a new device.

3.6 The necessity for clinical trial data from the UK is also a barrier to participation in the NICE assessment process and ultimately to device availability for patients. Devices have often undergone extensive clinical trials in other countries before being introduced to the UK market. International clinical evidence, from countries with respected healthcare institutions, should be considered relevant for a submission.

3.7 Cost savings associated with the use of new technologies will often require changes to the way services are delivered in order to be realised. This is something that should be also be recognised through NICE assessment processes.

4. NICE Quality Standards

4.1 The development of 'Quality Standards' is designed to drive and measure improvements in certain areas of care and this could have potential benefits for patient access to the most appropriate technology. The key—as with all NICE guidance—will be local implementation by commissioners and providers.

4.2 Like NICE's new role in ensuring compliance with technology appraisals, the body should also have a role in developing an effective system of monitoring local compliance with NICE Quality Standards.

October 2012

Written evidence from the Rarer Cancers Foundation (NICE 49)

SUMMARY

- The combination of existing drug pricing arrangements and NICE processes has not been fit for purpose for rarer cancers, necessitating the Cancer Drugs Fund (CDF).
- A longer term solution to cancer drugs is required, including determining how the CDF drugs will be made available. In developing this, lessons should be learnt from the CDF.
- NICE has an important role to play in encouraging high quality clinical practice for very rare conditions and this should be encouraged.
- The way in which NICE promotes and facilitates patient involvement engagement needs to be strengthened.
- The Committee might wish to make a range of recommendations to improve the operation of NICE (see Section 6).

1. INTRODUCTION

1.1 The Rarer Cancers Foundation (RCF) offers advice and information to people with rare or less common cancers and to their carers, families and friends. We facilitate supportive networking, raise awareness, provide information and resources and work to ensure that people with rarer cancers have access to the best possible services, often in partnership with other charities or NHS organisations.

1.2 People with rarer cancers have experienced particularly poor outcomes including reduced survival⁸⁵ and poorer patient experience.⁸⁶ Rarer cancers are therefore an area of high unmet need, with a particular requirement for the development of more effective treatments, research and services.

1.3 In recent years there has been important progress in the development of new treatments, many of which have been appraised by NICE. Since 2009–10:

- 39 treatments for rarer forms of cancer have been appraised.⁸⁷

⁸⁵ Office for National Statistics (ONS), Cancer Survival in England: Patients Diagnosed 2006–2010 and Followed up to 2011, October 2012

⁸⁶ Department of Health, Cancer Patient Experience Survey 2011/12, August 2012

⁸⁷ Hansard, 11 June 2012: Column 123W

- Representing 48% of NICE’s total technology appraisal workload.⁸⁸

1.4 As a result, rarer cancers has formed a disproportionate part of NICE’s workload. We welcome the opportunity to respond to this inquiry, which we hope will provide an opportunity to reflect upon ways in which the service NICE provides to the NHS and patients can be improved.

2. CHALLENGES IN THE CURRENT SYSTEM

2.1 Despite progress in the development of new treatments for rarer cancers, a number of challenges have emerged in enabling patients to gain access to new clinically effective treatments:

- Delays in developing guidance have resulted in a postcode lottery of access, with availability subject to often inconsistent local processes.
- Treatments have often been rejected on the grounds of cost-effectiveness, meaning that clinicians have not been able to prescribe the treatments they feel could most benefit their patients.
- It has not been possible to develop guidance for the use of treatments outside their licensed indication, which is a necessary and accepted part of clinical practice for very rare cancers.

2.2 These problems are not all the making of NICE. The Pharmaceutical Pricing Regulation Scheme (PPRS) includes features which have made it more difficult to gain approval for cancer treatments to be routinely used in the NHS, including:

- Providing only limited scope for price variation during the lifecycle of a product, meaning that prices have been set at the initial launch of a medicine when data are often immature, thus inhibiting an accurate assessment of the value—and therefore appropriate price—delivered by a treatment.
- Encouraging price cuts to be focused on treatments in therapy areas where there is intense competition rather than those in areas of high unmet need. Given the rapid developments that have occurred in cancer care, many new cancer medicines have been licensed in areas where no previous effective treatments existed and therefore competition has been minimal.
- Ensuring transparent pricing and the early launch of new medicines in the UK. Although superficially desirable, this has resulted in the UK being used as a reference point by other countries considering the appropriate level of pricing for new medicines, thus introducing a strong commercial incentive for manufacturers to avoid reducing list prices because of the potential adverse impact that this could have on international pricing.

2.3 These factors have often combined with the NICE process to delay the availability of guidance to the NHS on how a drug should be used, limiting access to many new cancer treatments.

3. NICE’S RECORD IN RELATION TO RARER CANCERS

3.1 We wholeheartedly support the development of clear national standards to inform NHS services. The benefits of this approach have been seen through the *Improving Outcomes Guidance*, which have reorganised cancer services to ensure that patients are seen by specialist teams with the appropriate level of expertise. We believe that NICE has an important role to play in this area, including through the development of:

- Quality standards to inform commissioning and ensure accountability.
- Evidence summaries to support clinical practice for very rare conditions.
- Clinical guidelines, to enable consistency of practice.

3.2 However, NICE’s role has been less beneficial in the appraisal of treatments for rarer cancers:

- NICE’s methodology does not capture the full impact of factors important to cancer patients, such as exhaustion or ability to maintain independence.
- Data from cancer clinical trials are often immature at the point of launch, meaning that the full benefits of treatments (such as overall survival impact) are unknown.
- Issues such as a crossover in clinical trials can confound results.
- Clinical practice in the real world is often somewhat different from that in trials.
- The Quality Adjusted Life Year (QALY) “threshold” is not well suited for conditions where there has either been very little progress (and so the comparator is old, ineffective, inexpensive and does not reflect the standard of care) or where, conversely, there has been rapid progress (meaning that the new drug will be used in combination with expensive modern treatments).
- NICE has been overly restrictive in interpreting and applying its own end of life criteria, which provide additional flexibility to treatments used in small patient populations.

⁸⁸ National Institute for Health and Clinical Excellence (NICE), Published technology appraisals, 25 October 2012. Available at: <http://www.nice.org.uk/guidance/ta/published/index.jsp?p=off> (accessed on 26 October 2012)

3.3 For the first time in 2011–12, a majority of cancer treatments appraised by NICE were not recommended, with 57.89% receiving a rejection.⁸⁹

3.4 On average, the cost per QALY of cancer drugs appraised by NICE has not varied significantly and is actually somewhat lower at £44,956 in 2011–12 than it was in 2009–10 (£51,797).^{90,91} This is actually below the limit suggested for medicines eligible for consideration under NICE’s end of life criteria,⁹² implying that a more flexible interpretation of the guidance could result in more cancer drugs being recommended for use in the NHS.

3.5 It is also notable that the appraisal process no longer appears to be encouraging flexibility on the part of manufacturers in relation to the cost of new cancer drugs. The proportion of technology appraisals where a patient access scheme was referenced has declined from 56.25% to 36.84% since 2010. Explanations for this might include:

- Inflexibility on the part of those proposing patient access schemes, with manufacturers instead preferring to negotiate with CDF committees.
- Inflexibility on the part of those considering patient access schemes, with the Department of Health and NICE being more restrictive in the type of schemes they are willing to permit.

3.6 It is welcome that the CDF has meant that patients have had another option for securing NHS access to life-extending treatments. To date, over 21,000 patients have gained access to treatment they would have otherwise been denied as a result of this policy⁹³ and this number should increase now that a similar mechanism is being established to fund new radiotherapy techniques. Over the lifetime of the policy, we estimate it will enable 44,000 patients to gain access to clinically effective cancer drugs which would otherwise not have been available.⁹⁴

3.7 Whilst providing an alternative access mechanism for cancer drugs, the CDF is only a temporary solution until the introduction of value-based pricing in 2014. However, value-based pricing is intended to apply to new drugs licensed in 2014 onwards. As a result, those treatments currently available through the CDF risk being made unavailable in the new system.

3.8 It is imperative that access to cancer drugs does not go backwards as a result of value-based pricing. In designing policies to avoid this, there are several important lessons that can be learned from the CDF:

- In the past NICE has consistently overestimated the eligible patient population, and therefore overall cost, of new cancer treatments. Usage figures for every drug made available through the CDF suggest that would qualify as being applicable to a small patient population.
- It is possible to make rapid decisions on availability of new treatments
- Clinicians can be trusted to make decisions on drug availability in a responsible manner. Indeed the CDF is underspent.
- Accountability for prescribing decisions is an important lever in ensuring prescribing responsibility.

3.9 The future of those treatments currently funded through the CDF is inextricably linked to that of NICE. Without clarity on a longer term funding mechanism for these medicines, NICE’s current approach would become untenable.

4. THE IMPORTANCE OF GUIDANCE FOR VERY RARE CANCERS

4.1 NICE is unable to conduct technology appraisals on drugs used outside their licensed indication. This is right and proper, as there is likely to be a paucity of data on the efficacy of such treatments, let alone their cost-effectiveness. Nonetheless, off-label treatment plays an important role in the treatment of rare conditions, including cancer.⁹⁵

4.2 Although individually the need for such treatments is rare, collectively it is common. Off-label usage is a particular issue for some blood cancers, where there may be no licensed treatments, but clinicians may wish to prescribe a drug licensed for a more common form of malignancy which has a similar biology.

4.3 There is a spectrum of clinical plausibility for the use of drugs off-label, ranging from highly plausible (eg using a drug licensed for one form of lymphoma to treat another, rarer, form which has a similar biology of disease) to less plausible (eg using anti-psychotic drugs to treat dementia). This is recognised in some commissioner funding policies.⁹⁶

⁸⁹ Hansard, 11 June 2012: Column 123W

⁹⁰ Rarer Cancers Foundation, *There when you need it the most? The Cancer Drugs Fund: 2011–12 annual report*, August 2012

⁹¹ Rarer Cancers Foundation, *Exceptional England? An investigation of the role of Primary Care Trusts in making cancer medicines available through exceptional cases processes*, October 2008

⁹² National Institute for Health and Clinical Excellence, *Appraising life-extending, end of life treatments*, January 2009

⁹³ Hansard, 15 October 2012: c151W

⁹⁴ Rarer Cancers Foundation, *There when you need it the most? The Cancer Drugs Fund: 2011–12 annual report*, August 2012

⁹⁵ Rarer Cancers Foundation, *Rare but common: a discussion paper on how to improve the evidence base on off-label*, June 2012

⁹⁶ NHS Birmingham East and North, *Individual Funding Requests Policy*, September 2009

4.4 In circumstances where a clinician wishes to use a drug off-label, and in the absence of NICE guidance, it can be difficult for commissioners to determine whether this is appropriate:

- By their very nature, such applications are likely to occur in small numbers, meaning that expertise cannot be developed.
- Expertise is likely to reside with the prescribing clinician rather than with the commissioner.
- There may be an absence of published or accessible information to guide decision-making.

4.5 It is therefore very welcome that NICE has now been asked to commission the development of treatment guides for the use of off-label drugs, which we believe can play an important role in supporting high quality clinical practice and focusing clinical efforts on treatments where there is more plausibility of benefit. We hope that NICE focuses on treatments:

- For rare conditions where there is no licensed alternative.
- Where there is evidence of variation in clinical practice.
- Where inequalities in access might occur.

4.6 It is important that NICE's work is supported by appropriate longer term commissioning policies for off-label treatments. In the field of cancer, the CDF has helped to avert these issues as off-label treatment is explicitly within its scope. To date, over 10% of treatments which have been funded have been for use in an off-label setting, benefitting between 1,131 and 1,391 patients in the first year of the policy.⁹⁷ Taken together, off-label treatments are the third most commonly-approved treatment in the CDF.

5. PATIENT INVOLVEMENT

5.1 Decisions about the availability of treatments or what constitutes a high quality service should be informed by the experience of patients and carers. We welcome the steps that NICE has taken to encourage patient and citizen involvement, but more needs to be done to make this meaningful.

5.2 We have participated in many different NICE processes. Unfortunately, too often the process is:

- Highly technical, making it difficult for patients to engage meaningfully.
- Adversarial, often proving to be an intimidating experience.
- Of marginal impact, with patients feeling that their views are discounted compared to those of other experts.
- Impractical, with insufficient scope for substitutions in patient witnesses in the event of illness, incapacity or death.

5.3 These issues must be addressed if public confidence in NICE's current and future role is to be bolstered and it is to be supported in maximising the contribution it can make to improving health and social care services.

5.4 Charities and patients often do not have the resources and expertise to engage with all aspects of the NICE process. Given the imperative to secure effective involvement, we believe there is a strong case for developing an independent advocacy service to support charity engagement, provide training and mentoring where it is required and to ensure there is adequate compensation for expenses incurred in fully participating in the appraisal.

6. IMPROVING NICE

6.1 In considering how NICE could improve in its functions, we hope the Committee will consider recommending that:

1. The Government proceeds with value-based pricing without delay as a means to developing a system which more sensitively reflects issues such as severity of disease and unmet need.

2. As part of this, manufacturers should be encouraged to take steps to improve the value and affordability of new treatments through commercially confidential value agreements.

3. The long term availability of treatments currently available through the CDF needs to be assured, otherwise access to cancer treatments will go backwards.

4. In reforming access to medicines arrangements, lessons should be learned from the largely successful clinically-led approach adopted through the CDF.

5. The complexities of appraising drugs for rarer conditions should be recognised and a flexible, pragmatic approach should be encouraged.

6. Data collection on the usage, benefits, side effects and costs of cancer drugs in routine NHS practice should be placed at the heart of any improved system.

⁹⁷ Rarer Cancers Foundation, Rare but common: A discussion paper on how to improve the evidence base on off-label treatments, June 2012

7. Future NICE work should be informed by a more realistic assessment of the size of patient populations likely to benefit from a treatment, and therefore the costs associated with this.

8. The work on developing evidence guides for off-label treatments should be applauded and continued, with appropriate levels of resource devoted to it.

9. Efforts to support more meaningful patient involvement in NICE's processes should be made a priority. Developing an independent advocacy support service for charities engaging with NICE should be considered.

October 2012

Written evidence from Prostate Cancer UK (NICE 50)

1. OVERVIEW

1.1 Prostate cancer is the most common form of cancer in men in England. Almost 35,000 men are diagnosed with prostate cancer every year and 215,000 men are currently living with the disease. Each year over 9,000 men in England die from prostate cancer. Of all the men diagnosed with prostate cancer in England and Wales, more than three quarters (77%) will live for at least five years after diagnosis. In the UK, one in nine men will get prostate cancer at some point in their lives. Older men, men with a family history of prostate cancer and men of African and Caribbean descent are more at risk.

1.2 Prostate Cancer UK is the UK's leading charity for men with prostate cancer and prostate problems. We support men and provide information, find answers through funding research and lead change to raise awareness and improve care. The charity is committed to ensuring the voice of people affected by prostate disease is at the heart of all we do.

1.3 In this submission Prostate Cancer UK provides written evidence about the National Institute for Health and Clinical Excellence (NICE).

1.4 Prostate Cancer UK would also welcome the opportunity to provide a witness to give oral evidence to the Committee on the issues raised.

1.5 Prostate Cancer UK is a member of Patients Involved in NICE (PIN) and fully supports its submission to this inquiry.

INTRODUCTION

1.6 The Charity welcomes the opportunity to contribute to the Health Committee's Inquiry on NICE. Our response makes following points:

- There are a number of flaws in the current system for appraising drugs which we hope Value Based Pricing will address:
 - It is vital that men with prostate cancer, as well as other people with cancer, are able to have an influence on the way in which VBP system is designed and operated.
 - The new system needs to reflect the priorities of men with prostate cancer when it comes to defining the value of new drugs. We also need to carefully consider the impact on men with prostate cancer of the introduction of any new weightings designed to capture the "wider societal benefits" of new drugs.
- In terms of the future role of NICE, it should continue to have a strong voice, but there needs to be more opportunity for patients to be fully involved in its work.

2. VALUE BASED PRICING

2.1 From January 2014, all new branded drugs will be subject to a revised process of drug pricing known as Value-based Pricing (VBP).⁹⁸ The new process will have a significant impact on men with prostate cancer and on NICE. Prostate Cancer UK, working in partnership with other leading cancer charities, has commissioned research to ensure the views of people affected by cancer are put at the heart of the Government's new policy of VBP.

2.2 Our research has found that men and women affected by cancer see the move to VBP as an opportunity to radically improve a system of new drug pricing and assessment that currently has many failings.⁹⁹ However, it is important that we get this right and the move to VBP must lead to an improvement in access to clinically effective drugs across the UK. People affected by cancer and the organisations that represent them will not support a system which cannot be shown to improve and promote timely and equitable access to the best possible medicines.

2.3 The Department of Health (DH) and the Association of British Pharmaceutical Industry (ABPI) are currently the only groups involved in deciding how new drugs should be assessed in the future. Men and women

⁹⁸ http://www.dh.gov.uk/en/Consultations/Liveconsultations/DH_122760

⁹⁹ A new value based approach to the pricing of branded medicines, P7, The Department of Health, December 2010.

affected by cancer have the experience, the knowledge and above all the desire to help lead on developing a better system to the Pharmaceutical Price Regulation Scheme (PPRS) and in assessing the value of new drugs. People affected by cancer and the organisations that represent them deserve an equal role in the design of VBP. The DH and NICE should commit to involving patients effectively in this process and beyond.

2.4 In terms of value, our research has identified that people affected by cancer believe additional factors such as reduced fatigue and emotional wellbeing should be included. This would be additional to the current “quality of life” assessments which are currently used by NICE in appraising new medicines. Also small but valuable differences made to quality of life should be included. A drug, for example, that enables people to get to the bathroom unaided can make all the difference to someone who was previously bed bound.

2.5 As part of VBP, the Government had promised to give greater consideration to the “wider societal benefits” of new drugs. We are pleased that the Government had made this commitment. Implementation of this will need to be carefully considered however. Much of the debate to date around the “wider societal benefits” of drugs has focused on the importance of helping people get back to work. This is an important goal but it isn’t the only one. Many people affected by cancer have already either retired or aren’t in a position to enter the workforce. If the new system prioritises drugs aimed at helping people back to work, then there’s a grave risk that many people affected by cancer will lose out. It’s vital that VBP does not lose sight of the contribution that many of these people have already made to our economy and society or the role that younger people will play in the future. We also have to ensure that drugs that allow people with cancer to spend quality time with their friends and family are given the value they deserve. Nor should we forget that value of drugs that help to give hard pressed carers much needed respite and the time and space to get on with other things.

3. FUTURE ROLE OF NICE

3.1 From January 2014, NICE will no longer have the final say over which medicines should be approved for use in the NHS. The final decision will instead rest with the Secretary of State for Health. Without an objective, independent, arms length body such as NICE making the final decision, there is an inherent risk of the system responding to “who shouts the loudest”. In creating another layer of decision making, there is a danger that value based pricing will add to the period of “NICE blight”.

3.2 Prostate Cancer UK are very disappointed that NICE have delayed producing a quality standard for prostate cancer until at least until the end of 2014. It is important that in future NICE has the capacity necessary to undertake and complete key pieces of commissioning guidance—such as quality standards—in a timely fashion. Opportunities for working collaboratively with external experts such as professional bodies and charities need to be taken. Partly due to the delay in developing a quality standard for prostate cancer.

3.3 Prostate Cancer UK has developed our own set of patient centred standards. These set out the care and support that men with prostate cancer should expect to receive at every step from the point of diagnosis. Our “Quality Checklist: your standards of care”¹⁰⁰ is based on extensive consultation with men with prostate cancer. We hope this patient focussed checklist will empower men across the UK to seek the best possible care and support and complement the NICE quality standard when it is finally published.

CONCLUSION

For Prostate Cancer UK the priority is to make sure patients are able to play a meaningful role in the system of appraising new medicines. It is also vital that patients have access to the best possible medicines. This is a central point of our “quality checklist: your standards of care”. However it is crucial that there is an independent body at which patients have equal representation in which to make decisions. We believe given the move towards VBP NICE still has a major and meaningful role to play, but that role needs to be clearly identified.

October 2012

Written evidence from Astellas Pharma Ltd (NICE 51)

1. INTRODUCTION

1.1 Astellas Pharma Ltd (“Astellas”) is a research and development-driven global pharmaceutical company that develops and markets clinically effective therapies for a number of therapy areas where there is an unmet clinical need including urology, oncology, dermatology and transplantation. The European and UK operation is based in Chertsey, Surrey and the company has a UK workforce of over 250.

1.2 Astellas welcomes the opportunity to respond to the Health Select Committee’s inquiry into the National Institute for Health and Clinical Excellence (“NICE”). Astellas is committed to working in partnership with the NHS and NICE to deliver high quality treatment and care and support the best possible outcomes for patients.

1.3 Our evidence focuses on the experiences and insights derived from our urology business where we have been working with patient, clinical and NHS partners over a number of years to build consensus on the core

¹⁰⁰ http://prostatecanceruk.org/media/1620003/a_quality_checklist_-_your_standards_of_care.pdf

components of high quality care for patients with lower urinary tract symptoms (LUTS) including urinary incontinence.

1.4 This submission addresses the following areas which fall within the remit of this inquiry:

- The development of guidelines and quality standards to inform high quality, cost effective commissioning and delivery of services.
- Evaluating the clinical and cost effectiveness of new treatments now and in future.
- Improving the way NICE guidance is acted upon.
- NICE's role in evaluating social care interventions.

2. THE DEVELOPMENT OF GUIDELINES AND STANDARDS TO INFORM HIGH QUALITY, COST EFFECTIVE COMMISSIONING AND DELIVERY OF SERVICES

2.1 Astellas supports NICE's work in overseeing the development of clinical guidelines and, more recently, quality standards to underpin the delivery of high quality, cost effective care. This work is more important today than ever before. There is a risk that the organisational changes to the NHS combined with heightened financial pressures could destabilise the delivery of high quality care in spite of the Government's laudable ambition to secure continuous improvements in the outcomes of patients.

2.2 Continence is one area of healthcare that has never been a priority for NHS improvement or investment and, as a result, there has been limited progress in establishing the high quality services and support that people with continence problems rely on to lead a normal life. Recent assessments of the provision of continence services such as the *National Audit of Continence Care* led by the Royal College of Physicians¹⁰¹ and *Commissioning for incontinence, lower urinary tract symptoms and bowel symptoms—an audit*¹⁰² highlight issues such as:

- under-diagnosis of continence problems;
- a lack of specialist care and support;
- a failure to evaluate performance in line with national guidelines and conduct local needs assessments; and
- a lack of local NHS leadership for quality improvement.

2.3 As commissioners begin to assume their new responsibilities they will rely even more heavily on high quality guidance and standards to help them plan and deliver the best possible services for their local population in a cost effective way. For LUTS including urinary incontinence, a lack of concerted action to support the commissioning and provision of services could pose serious risks to the quality of care patients receive.

2.4 By providing accessible summaries of the core components of high quality care for time pressured commissioners and providers, NICE quality standards will play an important role in addressing this challenge. However there is a concern that the length of time taken to develop quality standards does not match the speed at which the reforms are taking place.

2.5 The decision to refer quality standards to NICE on urinary incontinence in women and LUTS earlier this year is a welcome step forward. However, due to the initial delays to the development programme (so far only 22 out of the proposed 150 have been published),¹⁰³ there were concerns within the clinical and patient community about the timeliness of the standards given the need to rapidly address shortfalls within continence provision and translate the elements of good practice in the post-reform context. As such, a group of clinicians and patients with an interest in LUTS including urinary incontinence, chaired by the Director of the British Urological Institute, Professor Paul Abrams, met in April to discuss and agree the core standards of high quality continence care.¹⁰⁴ The purpose of the initiative was to help foster consensus on the content of the LUTS quality statements, and accelerate NICE's development process.

2.6 The readiness of expert patients and clinicians to support the process of guidance development is echoed by the recent report in the *Health Service Journal* revealed that the NHS Commissioning Board is considering a role for charitable and professional bodies in the development of commissioning guidance (to support the implementation of NICE quality standards).¹⁰⁵ Astellas would support this as long as the process supports the availability of timely, high quality guidance for commissioners which incorporates appropriate validation and patient involvement.

2.7 In addition to assuring both the quality and timeliness of quality standards, Astellas would also like the Committee to consider a number of additional recommendations with regard to the development process:

¹⁰¹ Royal College of Physicians, *National Audit of Continence Care*, September 2010

¹⁰² P Abrams et al, *Commissioning for incontinence, lower urinary tract and bowel symptoms—an audit*, April 2012

¹⁰³ National Institute for Health and Clinical Excellence, *NICE quality standards*, 11 October 2012. Available at: <http://www.nice.org.uk/aboutnice/qualitystandards/qualitystandards.jsp> (accessed on 24 October 2012)

¹⁰⁴ Astellas provided funding to support the facilitation of the meeting

¹⁰⁵ West D. Outside groups could vie with NICE over guidance. *Health Service Journal (HSJ)*, 2012 [cited 17 October 2012]. Available from: <http://www.hsj.co.uk/news/commissioning/outside-groups-could-vie-with-nice-over-guidance/5050678.article>

- NICE should use the opportunity posed by the quality standard programme to think imaginatively about the way that it selects topics. It may not necessarily be most effective to develop standards on the basis of historic guideline topics. For example, the distinction between LUTS in men and urinary incontinence in women established within two NICE clinical guidelines^{106, 107} should not be carried through to two separate quality standards when one quality standard could effectively capture the core components of effective care for men and women. This would expedite the process and reduce unnecessary demands on stakeholders.
- Quality standards should include a sufficient number of outcomes measures (or proxies for outcomes) to insure that their impact can be effectively evaluated.
- Reports of delays in the development of quality standards,¹⁰⁸ increases the likelihood that important topics and measures for those conditions are without a quality standard, such as LUTS and urinary incontinence, will continue to be excluded from the COF. Appropriate metrics to evaluate the performance of commissioners on improving outcomes for patients with long term conditions including LUTS through the Commissioning Outcomes Framework should be developed as soon as possible and should not be dependent on the existence of quality standards.
- It is important to ensure that disease specific quality measures are reflected across the quality improvement system—for example through the development of appropriate incentives such as CQUINS and best practice tariffs. NICE quality standards will not achieve their intended purpose if they stand alone.
- Commissioners should be held accountable for their performance in meeting the expectations set out in quality standards through the publication of data under each statement. We would support the inclusion of relevant questions within the National Audit of Continence Care to assess how the quality standard is being used and how effective it has been in reducing variations in care.

3 EVALUATING THE CLINICAL AND COST EFFECTIVENESS OF NEW TREATMENTS NOW AND IN FUTURE

3.1 Astellas recognises that a high performing health technology appraisal process is a critical component of any publically funded healthcare system that aspires to give patients access to the best new treatments available, while managing a finite resource and securing value for the taxpayer. The challenges inherent in this task are amplified by the current financial climate. Astellas believes that this creates a number of imperatives for the evolution of NICE's role, and for any future system of value-based pricing.

3.2 Firstly, the way that value is assessed in future must incorporate a full assessment of the societal costs and benefits of interventions. To use the example of LUTS, a failure to effectively manage symptoms costs society dearly. Among older people, it heightens the risk of falls and fractures¹⁰⁹ and increases their reliance on long-term care.¹¹⁰ As we move towards a more integrated health and social care system, it will become increasingly important that treatments and interventions which support independence and reduce the cost of caring are evaluated on broader terms.

3.3 Secondly, patients should play a central role in the process of determining the value of treatments. It is important that the complexities of a new system do not preclude patients from being involved in current negotiations, and in future decision-making. Evaluating the burden of disease in terms of the severity of a condition and the level of unmet need is by its nature a subjective process. For a person living with incontinence which affects almost every moment of their life, their condition is severe but to outside observers, it is less so. There is a risk that a system based on thresholds and criteria that are too rigid could end up failing to deliver the fair determination of value to which it aspires.

3.4 Thirdly it is important that in future the system builds on the increased flexibility afforded by the introduction of Patient Access Schemes which improve value and affordability while maintaining commercial confidentiality.

4. IMPROVING THE IMPACT OF NICE GUIDANCE

4.1 The NHS Constitution¹¹¹ stipulates that all patients have the right to access to drugs and treatments recommended by NICE for use in the NHS if a clinician feels they are clinically appropriate. Despite these clear principles and values, there are clear instances of variation in the implementation of NICE technology appraisal guidance which serves as a barrier to the delivery of high quality care. The forthcoming public consultation aimed at strengthening the Constitution offers an important opportunity to ensure that the NHS

¹⁰⁶ National Institute for Health and Clinical Excellence (2006) *The management of urinary incontinence in women*. CG40. London: National Institute for Health and Clinical Excellence

¹⁰⁷ National Institute for Health and Clinical Excellence (2010). Lower urinary tract symptoms; *The management of lower urinary tract symptoms in men*. CG97. London: National Institute for Health and Clinical Excellence

¹⁰⁸ Calkin S. Quality standards hit by arm's length body underspends. *Health Service Journal (HSJ)*. 2012 [cited 15 February 2012]. Available from: <http://www.hsj.co.uk/news/policy/quality-standards-hit-by-arms-length-body-underspends/5041521.article>

¹⁰⁹ Jensen J et al, 'Falls among frail older people in residential care', *Scandinavian Journal of Public Health*, 2002, 30; 54–61

¹¹⁰ Department of Health, *Good practice in Continence Services*, April 2000

¹¹¹ Department of Health, *The NHS Constitution for England*, March 2012

becomes more responsive to the individual needs and preferences of patients and improve the quality of services, by granting greater patient choice and control.¹¹²

4.2 Astellas welcomes proposals to introduce innovation scorecards to enable patients to see which hospitals and commissioning bodies are adopting the latest NICE-approved treatments and drugs most quickly. The proposals aimed at providing greater transparency set out in *Innovation, health and wealth: Accelerating adoption and diffusion in the NHS*¹¹³ will help to ensure greater consistency in the implementation of NICE clinical guidance and improve compliance.

4.3 As well as ensuring the consistent access to the full range of effective treatments, patients must also be supported to exercise greater choice and control over their care. Astellas supports the Government's commitment to involve patients fully in their own care, with decisions made in partnership with clinicians.¹¹⁴ The principle of shared decision-making between patients and healthcare professionals is particularly important where clinical guidelines recommend a range of treatments in the same classification, as is the case with anticholinergic drugs for LUTS. It is important that clinicians are able to support patients by explaining their treatment options, the potential side-effects and outcomes of interventions, and agreeing a process for routine monitoring and feedback from the patient, before settling on a specific course of treatment. When considering switching to an alternative treatment, the potential consequences of the change must be explained and decisions should be made in partnership with the patient.

4.4 The introduction of a dedicated quality standard for shared decision making would help to embed these practices and ensure patients and clinicians work together to make informed decisions about their care which reflect their individual needs and preferences.

4.5 In addition, fostering an effective dialogue between patients and their healthcare professionals is particularly pertinent for patients with long-term conditions such as LUTS. It is important that clinicians have the skills they need to be able to make shared decision making a reality. NICE should consider partnering with Health Education England to take forward work in this area.

5 NICE'S NEW RESPONSIBILITIES IN RELATION TO EVALUATING SOCIAL CARE INTERVENTIONS

5.1 Care for patients with LUTS takes place across different NHS and social care settings, it is therefore imperative that they are able to access fully integrated services. We welcome the expansion of NICE's role to include responsibility for developing guidance and quality standards in social care. As with the quality standards for health care, those in social care must be translated into key levers and mechanisms within social care quality framework to help improve outcomes for services users. It is also important that any supporting statements and metrics for social care are closely aligned with those included in health standards to ensure consistency in the provision of care across both settings.

October 2012

Written evidence from Shire plc (NICE 52)

Shire warmly welcomes the Health Select Committee's inquiry into NICE and is pleased to be able to respond to the call for evidence. The Committee has asked for written submissions in several key areas of interest and we have set out our thoughts on those areas where we feel we have particular expertise and experience below.

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

The assessment gap—the absence of NICE guidance can distort local clinical priorities

1. We would firstly like to draw the Committee's attention to one of the unintended consequences of the NICE topic selection process and the very well-intentioned statutory funding directive¹¹⁵ (ie that NHS organisations in England are obliged to make funding available for the treatment of patients whose clinicians recommend treatments in line with NICE appraisals within three months of the appraisal being published)—that is, that some innovation is at risk of not being taken up and funded because of the lack of a NICE review.

2. NICE currently reviews approximately 40% of new medicines launched onto the UK market per year.¹¹⁶ Therefore, there are significant numbers of innovative new medicines launched each year that do not receive any appraisal from NICE and might not additionally benefit from a retrospective multiple technology appraisal

¹¹² Department of Health, *Join in the conversation on how to strengthen the NHS Constitution*, September 2012, available at: <http://www.dh.gov.uk/health/2012/09/constitution-blog/> (accessed on 24 October 2012)

¹¹³ Department of Health, *Innovation, health and wealth: Accelerating adoption and diffusion in the NHS*, December 2011

¹¹⁴ Department of Health, *Liberating the NHS: No decision about me, without me*, July 2012

¹¹⁵ http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_114087.pdf Accessed 8.10.12

¹¹⁶ Nayanah Siva, "The drug price is right—or is it?" *The Lancet* 2009; 373, Issue 9672, 1326–1327

or inclusion in a clinical guideline. These are not medicines that have undergone a review and been found lacking in clinical or cost effectiveness, rather they have simply not been reviewed at all.

3. NICE itself does not decide on the topics for guidance and appraisals. Instead, topics are referred to NICE by the Department of Health, selected on the basis of a number of factors, including the burden of disease, the impact on resources, whether there is inappropriate variation in practice across the country, and factors affecting the timeliness or urgency for guidance to be produced.¹¹⁷

4. It is our experience that when a medicine does not meet the selection criteria for appraisal and is marketed without a positive review and therefore no funding directive, some NHS organisations might feel that the absence of guidance is notable and that—despite the innovation and improved patient outcomes that might be provided by that technology—they struggle or indeed decline to make the funds available to support the uptake of that innovation. We refer to this as the “assessment gap”, whereby a medicine’s uptake suffers because its clinical and cost effectiveness merits have not been assessed.

5. Whilst we applaud the initiatives set out in the 2011 Department of Health paper, *Innovation, Health and Wealth*,¹¹⁸ to support NHS organisations in England to accelerate the adoption and diffusion of innovation we note that these initiatives are in large part only applicable to NICE-approved technologies. We fully support the drive to reduce variation in practice and improve compliance with NICE technology appraisals, but we also feel that the emphasis contained within this document might exacerbate the current situation by concentrating scarce resources into the funding directive at the expense of other innovation.

6. Innovation is provided by many medicines—not only those which have met the selection criteria for NICE appraisal—and due to this “assessment gap” patients are at risk of missing out on improved outcomes and experience because of the distortions resulting from such well intentioned policy initiatives.

NICE’s new role in assessing high cost, low volume drugs—why conventional HGTA methodologies are not suitable for orphan drugs

7. In July 2012, the Department of Health announced that from April 2013 NICE would take on the assessment of high cost, low volume drugs,¹¹⁹ taking over this work from AGNSS, the Advisory Group for National Specialised Services. The decision was taken as part of the reforms under the Health and Social Care Act 2012, which sees AGNSS being disbanded. Lord Howe commented: “In taking this work over from AGNSS, NICE will wish to build on the decision-making framework that AGNSS has developed to ensure that the needs of people with rare and very rare conditions are properly considered.” NICE will develop interim methods for the first few assessments and will take forward a consultation exercise in 2013–14.

8. Shire takes a particular interest in this decision, as we were pleased to work with AGNSS in piloting the development of a new assessment process for products and technologies for ultra-rare diseases, (ie conditions with a prevalence of less than one in 50,000 patients) that offers a way forward in this area and is subject of considerable international interest.

9. Firstly, we were pleased that in asking NICE to build on the AGNSS decision-making framework there is a recognition that NICE’s standard approach to health technology assessment (HTA) is not appropriate or suitable for ultra-orphan products.

10. However, we also recognise that NICE’s current wealth of expertise lies in assessing “conventional” medicinal products against standard methods of HTA, therefore we feel the following points might be of use to the Committee in understanding why there should be a distinct methodology for orphan drugs. Current methodologies for the HTA of conventional medicines are mainly based on two sets of criteria: the clinical efficacy (effectiveness) and the cost-effectiveness of a new medicinal product compared to other products in the same therapeutic field, where they exist.

11. Although standard methods of HTA have an important role in optimising the efficiency of healthcare provision, they tend to result in restricted and inequitable access to new orphan therapies for several reasons, including:

- (i) Orphan drugs differ from conventional medicines as they are used to treat rare conditions for which there may be no alternative treatments available;
- (ii) R&D costs have to be covered by sales to a limited number of patients worldwide, raising acquisition costs per patient for any successful therapy;
- (iii) Higher acquisition costs raise cost-effectiveness ratios for orphan drugs sometimes by orders of magnitude. Consequently, however innovative and effective they are orphan drugs are almost always unable to meet standard cost-effectiveness thresholds in HTAs for reimbursement purposes.¹²⁰ Indeed, the scale of difference of ICER values of “conventional treatments” versus

¹¹⁷ <http://www.nice.org.uk/media/96A/B2/TopicSelectionProcessManualv25.pdf> Accessed 8.10.12

¹¹⁸ http://www.dh.gov.uk/prod_consum_dh/groups/dh_digitalassets/documents/digitalasset/dh_134597.pdf

¹¹⁹ <http://www.dh.gov.uk/health/2012/07/very-high-cost-drugs/> Accessed 4.10.12

¹²⁰ Michael D Rawlins & Anthony J Culyer, “National Institute for Clinical Excellence and its value judgements” *BMJ* 2004;329:224–227

treatments for rare diseases is worth highlighting ie approximately £20–40,000 per QALY for conventional treatments vs £500,000-£1 million per QALY for rare diseases;

- (iv) The absence of comparators in many instances can also inflate cost effectiveness ratios, as the cost of treatment is being compared with the cost of no treatment;
- (v) While HTA is typically an “evidence-based” process, the evidence base for orphan medicines is usually more limited at the time of marketing authorisation due to the rarity of the condition. The expansion of evidence follows with use in clinical practice. Further orphan drug evidence is associated with small heterogeneous populations, short duration of follow-up of studies, limited scientific understanding or consensus on clinical endpoints, absence of hard clinical outcomes such as survival and limited natural history data which create major challenges if following standard HTA methods;
- (vi) Conventional HTA methods often do not take sufficient account of societal values such as the fact that societies may be willing to pay more to help patients with rare, severe, life-limiting/threatening often genetic conditions where no alternative treatment exists.¹²¹

12. For all these reasons, current HTA methodology risks denying patients access to potentially life-saving drugs. Therefore, we believe that NICE needs to broaden the range of criteria they consider in their assessment if these patients are to avoid being left behind.

13. One way of doing this would be to use multi-criteria decision analysis (MCDA). MCDA in HTA is an analytical framework that systematically considers additional criteria beyond cost effectiveness, such as societal preferences, equity, benefits to caregivers, disease rarity and severity etc.

14. AGNSS’ ethical decision-making framework is the first example in the EU of an MCDA for ultra-orphan drugs. It was developed in 2010 through a series of multidisciplinary workshops involving clinicians, payers, patient representatives, health economists and ethicists, and as such has broad cross-sectoral support. Shire was pleased to work with AGNSS in developing and piloting the framework before its entry into operation.

15. Traditionally, HTA conducted by NICE is based on the incremental cost-effectiveness ratio (ICER) ie the cost per quality-adjusted life year (QALY) gained by recipients of the treatment. The AGNSS framework takes into consideration many more factors as part of a transparent process centred on patients’ needs.

16. The AGNSS assessment is a two stage procedure, whereby product applications are first checked against their suitability for national commissioning (so called “outline applications”) and secondly against the 12 core AGNSS criteria. These criteria are organised into four groups: i) health gain; ii) societal value; iii) reasonable cost; iv) best practice.

17. Crucially, the framework requires that no single criterion should hold sway but a holistic view be taken, balancing how the different criteria work together in reaching a decision. More expensive products will be expected to benefit nearly all patients treated, ie have a “number needed to treat” (NNT) approaching 1.¹²²

18. Against that background, the AGNSS framework offers the following benefits in assessing orphan products relative to conventional HTA:

- (i) AGNSS avoids a rigid quality-adjusted life year (QALY) ceiling like NICE. Instead it takes into consideration a broader societal perspective.
- (ii) It does not make assumptions about the evidence base but allows flexibility depending on the technology and the patient group concerned.
- (iii) Given the difficulty of collecting data for “ultra-orphan” and rare disorders, AGNSS assumes further data collection will occur in the five years after recommendation, and assesses the ability of the applicant to do this.¹²³
- (iv) It encourages submissions from patients and healthcare professionals, on the basis of the consideration that patients and caregivers can provide the best information on the impact of a disease on daily living, as well as on benefits or unwanted side-effects of treatments.
- (v) It provides room for narrative or contextualisation of clinical data.
- (vi) It provides support to patients/patient groups in developing their submission, through assistance on literature review, focus groups, writing, survey design and interviews.

19. In conclusion, in assuming responsibility for evaluating ultra-orphan products and technologies from April 2013, NICE has an opportunity to adopt the AGNSS framework and work to ensure that the practice of HTA evolves in order that patients in England and Wales can access these potentially life-saving medicines.

¹²¹ Societal attitudes towards cost effectiveness have been explored in a number of reports produced by NICE’s Citizens’ Council

¹²² The “number needed to treat” (NNT) is an epidemiological measure reflecting the average number of patients who need to be treated to prevent one additional bad outcome. The ideal NNT is 1, where everyone improved with treatment compared to control. The higher the NNT, the less effective the treatment.

¹²³ Anu Bharath, AGNSS—Is There Really a Need For a NICE of Rare Disorders? Feb 6 2012 <http://healthcare.blogs.ihc.com/2012/02/06/agnss-is-there-really-a-need-for-a-nice-of-rare-disorders/>

The introduction of Value Based Pricing

20. The government has signalled its intent to introduce a system of Value Based Pricing (VBP) to all new medicines launched onto the UK market from January 2014, replacing the current system under which the UK enjoys some of the lowest medicine acquisition costs in the EU.¹²⁴ Under the proposals as currently set out, a value assessment would be carried out against a set of value measures, including burden of illness, therapeutic improvement and innovation, cost effectiveness and wider societal benefits. This value assessment would be translated into a price, seemingly using a set of thresholds.

21. Firstly, it is clear that a body or bodies are required to undertake the value assessment on behalf of the Department of Health, and it would seem that NICE is in a sensible place to take part or all of this duty.

22. However, as referenced above, NICE currently only appraises approximately 40% of new medicines launched each year. Taking every new medicine through an extended value assessment and price threshold calculation would therefore seem to indicate a much higher workload and therefore resource requirement for NICE. The current NICE appraisal process is consultative, but can be lengthy; and it will be important to ensure that the new assessment process under VBP is equally consultative, but speedy with minimal delays to patient access.

23. Secondly, the value measures proposed by the Department of Health are wider than those currently used by NICE. It will be important to ensure that the right experts are engaged in the assessment process in order to provide a thorough and robust assessment of a new medicines' contribution to therapeutic improvement and innovation, the burden of illness, and wider societal costs. It is perhaps this last point that has proved challenging for NICE in the past and it will be imperative to define what wider societal costs include and how these might be measured to give clarity to industry when putting together an evidence package.

24. Finally, the Department of Health signalled in its consultation response that it might be willing to look at medicines for rare and very rare conditions differently under the VBP process. We agree that a different process would be needed for such medicines and we would strongly urge the Committee to look carefully at this in its inquiry. We hope it is evident from the points set out above (under why conventional HTA methodology is not appropriate for orphan drugs) that it is vital that these medicines are considered separately from other drugs under VBP, at the very least utilising the MCDA framework pioneered by AGNSS.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

25. NICE Quality Standards are a concise set of statements designed to drive and measure priority quality improvements within a particular area of care, and focus on the treatment and prevention of different diseases and conditions. To date NICE has published 22 sets of Quality Standards¹²⁵ and proposes that up to 150 of these should be developed over 5 years.¹²⁶ Quality Standards for the NHS will be reflected in the Commissioning Outcomes Framework and will inform payment mechanisms and incentive schemes such as the Quality and Outcomes Framework (QOF) and Commissioning for Quality and Innovation (CQUIN) Payment Framework.

26. We would echo our comments made under section 1 above with regard to the “assessment gap” caused when a medicine has not met the criteria for selection for a NICE technology appraisal and can be perversely affected by the lack of NICE review and subsequent funding directive. As Quality Standards do not cover every clinical area, it will be important to ensure that they do not become the only means by which commissioning priorities are determined as otherwise many innovative and potentially useful interventions could be left in a form of NICE “limbo” from which there is no escape.

ABOUT US

As one of the world's leading specialty biopharmaceutical companies, Shire is focussed on a single purpose: to enable people with life-altering conditions to lead better lives. We focus on developing treatments for rare diseases and symptomatic conditions treated by specialist physicians.

Through our Shire Human Genetic Therapies (HGT) business, we pursue opportunities on behalf of patients and families facing such rare diseases as Fabry disease, Hunter syndrome, Gaucher disease, hereditary angioedema, and San Fillipo—patients whose very lives often hinge on the discovery and delivery of extraordinary medicines. Through our Specialty Pharma business, we develop and distribute an innovative portfolio of treatments for patients with ADHD, and gastro-intestinal disorders such as ulcerative colitis and chronic constipation.

October 2012

¹²⁴ OHE, 11th Report (2004–2010); 5th Report (2000–2003); 2011

¹²⁵ <http://www.nice.org.uk/guidance/qualitystandards/qualitystandards.jsp> Accessed 4.10.12

¹²⁶ <http://www.nice.org.uk/guidance/qualitystandards/QualityStandardsLibrary.jsp> Accessed 4.10.12

Written evidence from the British In Vitro Diagnostics Association (NICE 53)

Our general sense of engaging with NICE is that our relationship with them has been steadily improving as we seek to assist them in establishing an evaluation programme for diagnostics which is fit for purpose and gives sufficient merit to the value of diagnostic information in guiding therapy decisions and other forms of care. A summary of our main points includes:

- NICE has an essential role in providing evidence-based recommendations of innovative medical technologies.
- One of the main challenges to the role of NICE in encouraging innovation is the slow and varied implementation of its guidance.
- Although we welcome the establishment of two new diagnostic evaluation programmes, BIVDA members are concerned about the use of QALY in these programmes.

1. The British In Vitro Diagnostic Association (BIVDA) welcomes the opportunity to respond to the Health Select Committee's inquiry into NICE. BIVDA is the industry association for British suppliers and manufacturers of in vitro diagnostic (IVD) products. We pursue a strategy of raising awareness of the clinical and cost utility of innovative IVDs in the provision of effective healthcare. We also provide valuable support services to our 100 strong membership which includes a number of small and medium sized enterprises and UK subsidiaries of multi-nationals. UK IVD sales to NHS in 2011 were approximately £700 million which gives an indication of the scale of the industry and the strength of its relationship with the NHS.

2. 70% of clinical decisions in the NHS are based on the results of laboratory or point of care tests, using IVD products. These are used to diagnose disease and monitor and manage treatment. Increasingly IVDs can be used to predict and prevent onset of disease and they can help ensure the appropriate and effective use of drugs and other NHS resources. IVDs are recognised as being able to improve patient outcomes and experience of care. They also have the capability to create savings in the healthcare system through innovative service redesign.

3. NICE has an essential role in making new treatments and technologies available to patients. Its role in approving and recommending new technologies makes it an essential gateway to healthcare innovation. However, the evaluation of medical devices and diagnostics presents a number of challenges; if testing is linked to treatment or can improve disease management, then it has "downstream" impact on the outcome of extended life and/or improved quality of life. Diagnostics also have an impact on patients' utility outside the quality adjusted life years (QALY)-oriented health outcomes used to evaluate drugs and therapies. These aspects are not reflected in commissioners' decisions, which are usually based on narrow "medical" quality of life measures.

4. BIVDA has long been concerned about the scale of variation in the implementation of NICE guidance across the country. Despite new technologies being a key part of "Nicholson's Challenge", adoption of new NICE approved technologies is slow and inconsistent. A significant barrier to the adoption of new IVDs is that, although IVDs will come from the pathology budget, the savings will often be felt by another department. In order to gain a more accurate sense of the value of an IVD, it is necessary to take a wider pathway perspective and move away from a "silo budget" mentality.

5. There have been steps from within the Office of Life Sciences and the Innovation, Health & Wealth work to address inconsistencies in adoption of NICE guidance. BIVDA is pleased to be engaged in this work but the results of these efforts are still pending. The transition from 151 PCTs to 212 CCGs also creates the potential for greater variation in service provision. BIVDA hopes that with the recent announcement about medical revalidation—which placed an emphasis on new technologies in the appraisal of doctors—will contribute to rapid and consistent implementation of NICE guidance.

6. BIVDA has enjoyed a steadily improving relationship with NICE over a number of years. As part of this relationship we have welcomed the recognition that assessment programmes designed for drug therapies are not suitable for diagnostics. From this recognition have come two programmes specifically for the evaluation of new diagnostics—the Medical Technologies Evaluation Programme (MTEP) and the Diagnostics Assessment Programme (DAP). These programmes are clearly extremely positive outcomes of our relationship with NICE, although our members have observed that the programmes have mainly concentrated on imaging diagnostics until now. Increased assessment of IVDs—to create an equal balance of *in vitro* and *in vivo* diagnostics—would of course be welcomed.

7. MTEP is intended to provide the fast evaluation of a specific high impact diagnostic. MTEP evaluates the clinical effectiveness and cost consequences of that one diagnostic, taking 38 weeks from topic selection to guidance publication. MTEP requires notification of technologies through the active engagement of industry. DAP is more complex. DAP assessments include the cost effectiveness analysis (this requires the evaluation of outcome benefits—QALYs) of a single or multiple related diagnostic technology(s). No formal submissions are accepted from industry, although they can give evidence. The whole process requires 62 weeks from topic selection to guidance published.

8. The establishment of these evaluation programmes, and their differing functions, have been a welcome acknowledgement of the specific assessment requirements of diagnostics. Unlike drug therapies, diagnostic tests do not *directly* impact on health outcomes. Rather, tests provide essential information which enable

clinicians to make appropriate decisions regarding care. At present, the use of QALYs in the DAP does not allow the decision-maker to consider a broad set of outcomes including the value of the information provided by the test. Although QALYs may be appropriate for the evaluation of companion diagnostics, the continuing use of QALYs in the assessment of single or multiple similar IVDs is of concern to BIVDA members.

9. The MTEP and DAP identify medical technologies that have the potential to offer substantial benefit to patients and/or the NHS; the premise for the programmes are that such medical technologies are more likely to be adopted more consistently and more rapidly by the NHS if NICE develops guidance on them. Consistent and rapid adoption by the NHS however is rarely realised.

10. Overall, BIVDA is very pleased to enjoy an ever improving relationship with NICE. The inclusion of two NICE representatives on the UK Diagnostics Forum is an example of excellent partnering between NICE and industry. However, the good work of NICE as an independent organisation (and in its collaborations with industry) can be undermined by deep rooted cultural barriers to innovation which exist within the NHS. Rather than concentrating on year in year savings, NHS financial managers need to recognise the potential for innovative technologies to save the NHS money over a number of years. There also needs to be a recognition of the savings and improvements which can be made via IVDs, outside of the pathology silo. A broader 'pathway perspective' of value is essential if innovation can be achieved in NHS services. The role of NICE in enabling innovation through evidence-based recommendations cannot be underestimated, but we need to start seeing results from the work currently underway to decrease variation in implementation of guidance.

October 2012

Written evidence from GlaxoSmithKline (NICE 54)

1. INTRODUCTION

1.1 GSK supports a system where evidence-based value judgements are made about technologies for us in the National Health Service (NHS). NICE performs a valuable role in assessing technologies and developing clinical guidelines, and its decisions have a significant impact on access to medicines and uptake of innovation in the NHS. Therefore, NICE Health Technology Assessments (HTAs) and corresponding value judgements should be fair, transparent and predictable. They also need to take into account the entire value a medicine delivers, including rewarding innovation.

1.2 GSK welcomes the opportunity to provide evidence on how NICE has operated to date and what improvements should be sought going forward.

2. THE NEED FOR A POLICY FRAMEWORK TO OVERSEE THE WAY NICE WORKS

2.1 NICE plays an important role in appraising the value of new technologies to the NHS. However, the value of an individual medicine must be appraised in the context of a number of factors including how value and innovation are defined. GSK believes that these are matters of policy and that a distinction should be made between the policy issues, determined by Government, that together form a framework for NICE decision making (the "what") and the methods to deliver this policy framework (the "how"), determined by NICE.

2.2 Currently this distinction is not made. This can be seen clearly in the NICE Methods Guide which has been a central document to guide how health technology appraisals are conducted. The current version covers, in our view, not only issues of methodology but also of policy.

2.3 For example:

- Agreement of what is considered as part of an HTA (ie the reference case).
- How evidence is appraised and decisions made.
- Which factors should be taken into account (eg innovation).
- The perspective taken (eg NHS or wider societal perspective).
- The willingness to pay for health benefit (ie setting the threshold).

2.4 We believe such issues go beyond NICE's core remit and should form part of a Government policy framework that NICE implements. To ensure there is accountability for implementing the framework, the NICE Executive should be able to challenge decision-making by Appraisal Committees if it believes the framework has not been followed.

2.5 The framework should include the following issues:

- The broader value of a medicine—how the decision-making process should account for elements such as innovation, disease severity, and unmet need
- Opportunity for NICE and its Appraisal Committees to consider higher thresholds where technologies demonstrate innovation and value outside cost/QALY
- Uncertainty—A pragmatic approach is taken towards the inherent uncertainty in evidence at the time of launch

- Choice of comparator—the need for a pragmatic approach and a focus on treatments which reflect current clinical practice
- Multiple layers of independence within the appraisal system

3. THE COMPONENTS OF THE POLICY FRAMEWORK

(a) *Consideration of the broader value of a medicine—innovation and other value elements*

3.1 We are concerned that the current system is almost exclusively dominated by cost-effectiveness (ie cost/quality-adjusted life year methodology) and there should be a clear process by which other elements of value are considered within the decision-making process.

3.2 The Government's proposal for Value Based Pricing (VBP) and the *Innovation: Health and Wealth* report highlight the need to reward innovation in healthcare and we believe NICE has an important role to play in this. The current NICE HTA process allows for manufacturers to suggest to what degree a medicine is innovative or what additional elements of value have not been captured by cost/QALY methodology; however, there is no clear indication of how this has been quantified or how this has impacted the Appraisal Committees' decision-making process.

3.3 NICE HTAs should, as part of a policy framework, consider more explicitly a broader definition of a medicine's value (such as addressing significant burden of disease, delivering innovation above that captured in current cost-effectiveness methodology, and consideration of a broader societal perspective, where appropriate).

3.4 Appraisal Committees should be instructed to consider these broader factors, demonstrate to what degree they have impacted their decision, and be held accountable for their decisions.

3.5 NICE and Appraisal Committees should consider higher thresholds where a technology demonstrates broader elements of value.

(b) *Treatment of uncertainty*

3.6 There are fundamental challenges with regards uncertainty:

- The approach towards the inherent uncertainty in evidence at the time of launch.
- The move towards more complex analytical approaches within appraisals is increasing complexity and analytical burden, which will add to the uncertainty.
- The variability in decision making as a result of uncertainty and the need for consistency in decision making.

The approach towards the inherent uncertainty in evidence at the time of launch

3.7 It is not possible for all uncertainty to be removed from the decision as at the time a product is launched, evidence on the full value of the medicine will not be available. This is because it will only have been used in clinical trials and evidence on clinical effectiveness (real-life data) and longer term benefits will need to be collected/demonstrated.

3.8 Where all reasonable attempts are made to address uncertainty, Appraisal Committees should be guided to focus on the most plausible estimate of cost-effectiveness. It is currently not clear that this is happening. Furthermore, where treatments are innovative, address a significant unmet need, and deliver other aspects of value not captured by cost/QALY methodology, the Appraisal Committee should be more accepting of inevitable uncertainty, particularly where there is significant unmet need and/or limited budget impact, for example, in a small patient population where obtaining evidence is particularly challenging.

3.9 *GSK recently launched Benlysta (belimumab), offering the first treatment for lupus for 50 years and addressing significant unmet need. Lupus is a progressive disease, and while Benlysta demonstrated positive results in the Phase 3 trials, evidence of long-term benefits was not available at launch, resulting in some uncertainty. NICE issued negative guidance for Benlysta partly based on the Appraisal Committee's concerns over this uncertainty.*

3.10 *GSK's Arzerra (ofatumumab) received regulatory approval for the treatment of Chronic Lymphocytic Leukaemia (CLL) based on a single-arm Phase II trial. This approval acknowledged the existing unmet need and the rarity of CLL patients, and concluded that these factors were sufficient to compensate for any uncertainty in our data. In contrast, NICE considered that the uncertainty in the data was too great and issued a negative decision.*

The move towards more complex analytical approaches within appraisals

3.11 The NICE Methods Guide details how uncertainty should be dealt with when conducting economic evaluation (Section 5.8); however, we believe an increasingly more complex and analytical approach is being recommended and used, which hinders practical clinical decision-making.

3.12 In recommending a new medicine, NICE is committing the NHS to fund it and it is therefore appropriate to exercise caution in considering evidence. However, we believe that there should be a balance between academic rigor and pragmatic clinical decision-making; in our experience, the trend towards more academic approaches can increase the analytical burden on a manufacturer beyond what is necessary to make a decision, and can introduce more uncertainty into an appraisal as further analyses are undertaken. This means it is even more difficult for an Appraisal Committee, comprising a range of expertise not limited to health economics, to reach a decision and can result in economic analysis dominating clinical pragmatism. Ultimately this can limit patient access.

The variability in decision-making as a result of uncertainty and the need for consistency in decision-making

3.13 Appraisal Committees are provided with guidance in considering evidence and the corresponding uncertainty (Section 6.1–6.3); however there remains significant potential for variability in decision-making between different Appraisal Committees, resulting in a lack of predictability for manufacturers and inconsistent outcomes for patients.

3.14 Fundamental to a sustainable pharmaceutical industry is predictability of access decisions; the approach to uncertainty described above does not deliver predictability nor, we believe, the best outcomes for patients. We hope VBP will introduce a process where the outcomes are predictable. Furthermore, we believe, in advance of VBP, that improvements should be made to the current system to address the issue of inconsistency in the treatment of uncertainty by Appraisal Committees.

3.15 There should be a requirement to consider uncertainty in a more consistent way, with decisions made on the balance of clinical and cost-effectiveness evidence, and a more pragmatic approach taken to the inherent uncertainty in data at launch.

(c) *The choice of comparator*

3.16 The choice of comparator is crucial to an appraisal as the new technology must demonstrate incremental benefit relative to the costs associated with using it. This is highlighted when comparing a new innovative medicine against a cheap generic comparator; and in circumstances of being required to provide evidence of effectiveness against an unlicensed medicine, when no such evidence exists. This may lead NICE to issuing guidance that leads to non cost-effective use of NHS resources.

3.17 There is therefore a need to ensure pragmatism around selection of the comparator; the choice should not be based on the most cost-effective comparator, but rather what is representative of current clinical practice in the NHS. This is important in order to ensure guidance is relevant to healthcare professionals in NHS.

3.18 *GSK's Tyverb (lapatinib) for treatment of relapsed metastatic breast cancer was more cost-effective (with a patient access scheme) than trastuzumab, unlicensed in this indication but currently being used in NHS. Due to concerns over trastuzumab's relative clinical effectiveness and its cost-effectiveness, the Appraisal Committee did not consider it a relevant comparator and concluded that publication of guidance should be postponed until trastuzumab and lapatinib could be reviewed together. Patient access to Tyverb is currently provided through the Cancer Drugs Fund.*

3.19 NICE should adopt a pragmatic approach to consideration of comparators, based on current NHS clinical practice. In situations where the comparator is a cheap generic, this should be acknowledged and taken into account as part of the decision-making process.

(d) *Multiple layers of independence within the appraisal system*

3.20 Under the current system, Government delegates the HTA process to NICE, which in turn delegates to independent Appraisal Committees with independent chairs. These Committees are themselves informed by reports from independent Evidence Review Groups (ERGs). Independence is an important element of the decision-making process, and it is appropriate that appraisals are conducted at arm's length from Government and from the submitting manufacturer. However, we believe, based on direct company experience, that these multiple layers of independence within the same system raise questions about accountability, consistency and transparency of decision-making.

Appraisal Committees

3.21 There is currently insufficient transparency to understand how decisions are made by Appraisal Committees, such as how evidence was considered and what importance was placed on various aspects of value. The NICE Guidance Executive is limited in its ability to challenge Appraisal Committee decisions and in certain examples the only option has been to halt the appraisal (as seen for Tyverb above).

3.22 *Our submission of Revolade (eltrombopag) exemplifies our concern. Revolade for Idiopathic Thrombocytopenic Purpura (ITP) was licensed around the same time as romiplostin; both medicines have identical licensed indications and treat an orphan disease. The cost of Revolade to the NHS is significantly less than romiplostin. The medicines were reviewed by different NICE appraisal committees; romiplostin was approved and Revolade was not.*

3.23 The NICE Guidance Executive should have greater powers to hold Appraisal Committees to account to ensure a consistent decision-making approach across Committees; and that Appraisal Committee Chairs demonstrate how decisions were made, how various factors (such as those in our proposed policy framework) were taken into account in the decision-making process, and the impact this had on the decision.

3.24 Within the context of the proposed policy framework, it should be possible to appeal the outcome of an HTA appraisal if the various elements of value have not been considered and how they have influenced the decision; at present, it is only possible to appeal the process.

Evidence Review Groups (ERGs)

3.25 The ERGs are responsible for reviewing a manufacturer's submission and for providing a critique of the evidence for the Appraisal Committee to consider. There is significant variability in the outputs from different ERGs, which translate into inconsistency in the evidence Appraisal Committees have to consider and therefore inconsistency in NICE recommendations.

3.26 We are not aware of any guidelines on standard approaches to reviewing evidence or any mechanism to systematically review outputs from ERGs. While NICE is responsible for liaising with ERGs, it is limited in its ability to challenge outputs. When significant concerns are raised about the quality of the work produced by an ERG, it is referred to the Decision Support Unit (DSU).

3.27 The ERGs should report directly to NICE and greater guidance given to them (such as standard approaches to evaluating a submission and standard proforma for presentation of results). There should also be a process put in place to regularly review ERGs.

3.28 Within the appraisal, the manufacturer's submission is central to the decision-making process; however, there is limited opportunity for dialogue between manufacturers and ERGs. There is the decision-problem meeting and clarification questions process; however these both offer only limited opportunity to resolve issues, which may at a later stage have an impact on the decision-making process, either through a delay or through a decision being reached which the manufacturer later appeals.

3.29 There should be greater opportunity for dialogue (eg formal meeting around modelling approaches) between NICE, ERGs and the manufacturer to ensure the evidence presented to Appraisal Committees allows informed and consistent decisions to be made.

3.30 The selection of appropriate experts to validate clinical assumptions and how patients are treated is critical in order to ensure that the guidance issued by NICE is relevant to healthcare professionals. We have experience of an ERG gaining clinical input from an unrepresentative source, only for their views and assumptions to be corrected by the clinical experts in attendance at an Appraisal Committee meeting (this occurred during the Benlysta (belimumab) Appraisal Committee meeting regarding the management of Lupus).

3.31 NICE should ensure the appropriate clinical experts are able to input during the process to ensure the ERGs' interpretation of the clinical evidence is reasonable.

4. UPTAKE OF MEDICINES IN THE NHS

4.1 Local implementation of positive NICE HTA guidance and subsequent usage of NICE-approved medicines is slow and low. The *Innovation: Health & Wealth* measures are an important part of the solution to overcome local barriers and enable uptake of medicines; however their impact is dependent on excellent execution.

4.2 Planned steps to increase visibility of compliance with NICE guidance—including the innovation scorecard and the requirement for local formularies to be published by April 2013—are pivotal; however the IHW measures must be underpinned with appropriate and aligned incentives.

4.3 We also believe NICE itself should advocate the importance of implementing its guidance, to address demonstrated clinical resistance to using the newest medicines.

October 2012

Written evidence from the Neurological Alliance (NICE 55)

1. ABOUT THE NEUROLOGICAL ALLIANCE

1.1 The Neurological Alliance is the only collective voice for more than 70 national and regional brain and spine organisations working together to make life better for 8 million children, young people and adults in England with a neurological condition.

1.2 Our mission is to raise awareness and understanding of neurological conditions to ensure that every person diagnosed with a neurological condition has access to high quality, joined up services and information from their first symptoms, throughout their life.

2. SUMMARY

2.1 We welcome the opportunity to submit evidence to the Health Select Committee's inquiry on NICE. Our submission focuses on the role of NICE quality standards.

2.2 Recent reports by the National Audit Office (NAO)¹²⁷ and Public Accounts Committee (PAC)¹²⁸ have vividly described the parlous state of neurological services in England. Both reports conclude that care for people with neurological conditions is currently characterised by fragmentation, a lack of coordination and regional variation resulting in poor outcomes for service users and poor value for money for the NHS. The need for improvement in this complex and historically neglected area is urgent but following the Government's rejection¹²⁹ of many of the PAC's recommendations on how the outcomes and cost effectiveness of neurological care could be enhanced, there are concerning signs that neurology is once again being sidelined under the reformed NHS.

2.3 Together with the strategic clinical network (SCN) for mental health, dementia and neurological conditions, the 15 confirmed neurology-specific NICE quality standards are currently the central drivers for improvements in neurological care under the new NHS arrangements. We do not regard the SCN and quality standards as capable alone of achieving improvements of the scale and at the pace necessary; they must be part of a broader strategy if the NHS Commissioning Board (NHS CB) and Department of Health are to successfully address the legacy of failure ahead of the neurological services progress review, which the PAC has indicated it intends to direct the NAO to conduct in 2014. Nonetheless we do believe that, once developed, quality standards could play a vital role in providing commissioners with much needed support in giving due priority to neurological conditions in their commissioning decisions and in delivering high quality, cost effective neurological care.

2.4 However, we have a number of concerns relating to quality standards, specifically the:

- slow pace at which quality standards are being developed and undetermined process by which quality standards will be prioritised for development;
- fast tracking of quality standards for a condition or condition area that has an existing NICE guideline, which will have the effect of prolonging neglect in areas without NICE accredited guidance;
- extension by four years of the deadline for all 180 quality standards to be developed from 2015 to 2019;
- status of quality standards as one of only two means by which a condition or condition area can be included in the key commissioning accountability framework, the Commissioning Outcomes Framework (COF), which will have a substantial influence over commissioning priorities and from which neurology is currently largely omitted; and
- unreasonable weight of expectation placed upon neurological quality standards, in the absence of any other quality, accountability and improvement mechanisms under the reformed NHS, to deliver urgent, large scale improvements to neurological services.

2.5 It is clear that clinical commissioning groups (CCGs) are not being equipped to tackle the huge challenge that neurology represents and that in the absence of additional quality accountability, incentive mechanisms or national neurology strategy, neurological quality standards will perform an absolutely critical role both prompting CCGs to commission, and be supported in the delivery of, appropriate services for people with neurological conditions.

2.6 As such, we will be calling on NICE to prioritise the unscheduled neurological quality standards for development in 2013.

3. THE ROLE OF NICE QUALITY STANDARDS IN THE NEW NHS SYSTEM ARCHITECTURE

NICE quality standards and neurological conditions

3.1 Of the 180 quality standards that NICE is due to develop by 2019, a deadline recently extended from 2015, a total of 15 relate specifically to neurology¹³⁰ with 11 focussing on individual conditions. In addition, a quality standard for rarer neurological problems has also been confirmed; this is an extremely important development for the substantial number of neurological conditions for which there has never been NICE generated guidance. We strongly support the announcement of all 15 neurological quality standards and are ready to work with NICE where appropriate to assist in their development and promotion.

¹²⁷ Service for people with neurological conditions—National Audit Office, December 2011

¹²⁸ Services for people with neurological conditions—House of Commons Committee of Public Accounts, Seventy-second report of Session 2010–12, 27 February 2012

¹²⁹ Treasury minutes: Government responses on the Sixty Eighth, the Seventieth, the Seventy Second and Seventy Fourth reports from the Committee of Public Accounts: Session 2010–12—pp. 14–20

¹³⁰ Autism (adults); autism (children and young people); epilepsy (adults); epilepsy (children); head injury; headaches/migraine (young people and adults); multiple sclerosis; Parkinson's disease; faecal incontinence; delirium; management of transient loss of consciousness in adults; motor neurone disease; neurological problems (relatively uncommon neurological problems e.g. muscular dystrophy); dementia; stroke

The pace of quality standard development

3.2 However, the positive role that quality standards could play within the new commissioning model is being fundamentally undermined by both the speed at which they are being developed and the continued lack of clarity on the development timetable.

3.3 To date, just two neurological quality standards have been published,¹³¹ with only a further two in development;¹³² none of the remaining neurological quality standards have been scheduled in the development programme. In addition to impacting detrimentally on the ability of CCGs to commission high quality, cost effective services for these conditions, the lack of published neurological quality standards effectively debars neurology from inclusion in the COF, the key commissioning accountability framework (see paragraphs 3.9–10).

3.4 NICE and the Department of Health have yet to establish the basis on which undeveloped quality standards will be prioritised and, from this, publish a development timetable. The only criterion for prioritisation identified to date is that those conditions or areas with a confirmed quality standard that have an existing NICE guideline will be fast tracked for development.

3.5 As NICE guidelines will continue to act as a source of guidance to commissioners under the new system, we do not consider this criterion is an appropriate basis for quality standard prioritisation; its unintended consequence will be that those conditions for which there is no guideline at present will continue to be unaddressed in the early period of CCG operation, leaving commissioners unsupported often in the most complex and challenging areas. Indeed, as the vast majority of neurological conditions have no NICE guideline, so the creation of all the confirmed neurological quality standards, including the overarching standard for rarer neurological problems, needs to represent an urgent priority for NICE.

3.6 We note with great concern that an additional four years has been added to the original timetable for quality standard development, with the full suite now not due to be completed until 2019. This presents a considerable problem for commissioners, who will not be able to use the full suite of quality standards to support their commissioning decisions and the delivery of comprehensive, value for money care and support as intended for six years from the date CCGs become operational.

3.7 In consideration of the acute need for support that commissioners will have in respect of driving neurological improvements and the dearth of neurology specific accountability and incentive mechanisms in the new system (see paragraphs 3.11–22), we will be calling on NICE to commit to develop in 2013 all neurological quality standards on which work has not yet commenced.

3.8 The imperative to deliver service improvements and enhance outcomes for neurological conditions is particularly urgent in light of the progress review on neurological services that the PAC has expressed an intention to instruct the NAO to undertake in 2014. If neurological quality standards are to play a substantive role in delivering improvements of the scale and at the pace necessary, they will need to be developed as soon as possible; waiting until 2014–15, let alone until 2015 onwards, will be too late.

Quality standards and the Commissioning Outcomes Framework

3.9 The importance of publishing the quality standard development timetable, which must prioritise quality standards on the basis of a clear rationale, and increasing the pace at which standards are developed is underscored by the relationship between quality standards and the COF. Having a quality standard represents one of only two routes by which it is possible for a condition or condition area to qualify for inclusion in this key accountability framework; the other is having an indicator in the NHS Outcomes Framework.

3.10 In defining the key areas that CCGs must report against in respect of improvement to the NHS CB, the COF will be central in determining the commissioning activities of CCGs. Unless additional channels through which indicators can be integrated into the COF are opened, further delays to the development of neurological quality standards will have a double impact in both denying CCGs access to vital support and guaranteeing that neurology remains outside the formal NHS accountability frameworks for the foreseeable future.

Quality standards and the broader NHS quality, accountability and improvement architecture

3.11 The role of NICE quality standards in supporting commissioners assumes a heightened importance in respect of neurology when considering the broader context of the new NHS quality, accountability and improvement architecture. Throughout these arrangements, neurological conditions are profoundly underrepresented. This approach is particularly concerning given that the recent NAO¹³³ and PAC¹³⁴ reports on services for people with neurological conditions identified a lack of accountability at national and local level as central to the current status of neurological services as delivering poor outcomes for patients and poor value for money.

¹³¹ Dementia and stroke

¹³² Epilepsy (adults) and epilepsy (children)

¹³³ Service for people with neurological conditions—National Audit Office, December 2011

¹³⁴ Services for people with neurological conditions—House of Commons Committee of Public Accounts, Seventy-second report of Session 2010–12, 27 February 2012

3.12 Despite neurology accounting for £4.3 billion of NHS spend annually¹³⁵ and representing the seventh largest NHS budget spending category, neurology-specific accountability and incentive mechanisms are virtually non-existent under the arrangements due to become operational in April 2013.

3.13 There are no references to neurology in the draft NHS mandate, by which the Secretary of State will hold the NHS CB to account. Of the 60 indicators in the 2012–13 NHS Outcomes Framework, through which the Department of Health will hold the NHS CB accountable, only three are neurology-specific, with one each allocated to stroke, dementia and epilepsy.¹³⁶ Similarly, of the 44 indicators NICE has recommended populate the COF, which CCGs will report against to the NHS CB, the 10 neurology specific indicators again relate only to stroke, dementia and epilepsy.

3.14 Consequently, together with the recently announced strategic clinical network (SCN) for mental health, dementia and neurological conditions, NICE quality standards are the second of just two parts of the reformed system that give neurological conditions specific focus and provide CCGs with any support and guidance on how to commission high quality, cost effective and appropriate care for this notoriously complex and historically neglected group of conditions.

3.15 Indeed, the SCN and NICE quality standards represent the only explicit indication under the reformed NHS that CCGs will be the key player in reversing spiralling neurological costs, reducing disproportionate emergency admission rates and driving urgent service improvements in this area. Even Joint Strategic Needs Assessments (JSNAs) on which local commissioning plans will be based routinely omit the vast majority of neurological conditions due to the chronic shortage of centrally and locally collated neurology data.

3.16 The need to properly equip CCGs to tackle neurology is further emphasised by the generally poor levels of understanding of neurology amongst health and social care professionals, resulting from the complexity and relative rarity of the vast majority of neurological conditions. Despite the clear and significant support needs commissioners will have if they are going cater for the care and support needs of people with neurological conditions, the new system architecture will require that they do so in the absence of:

- a national outcomes strategy for neurological conditions;
- a dedicated national neurological advisor to the NHS CB to lead on and coordinate improvements in neurological services;
- mandatory accountability and incentive mechanisms; and
- NICE guidelines, which exist for only a small number of neurological conditions.

3.17 In view of this, NICE quality standards, together with the SCN for mental health, dementia and neurological conditions, will need to play a pivotal role in encouraging CCGs to take neurological conditions into proper account in their commissioning plans.

3.18 We do not regard this situation as adequate given the non-mandatory status of quality standards. Additionally, their intended purpose is as one of a range of sources from which commissioning priorities should be determined, alongside JSNAs, Joint Health and Wellbeing Strategies, the COF, national level outcomes strategies and NHS CB commissioning guidance. As these instruments currently give little or no profile to neurological conditions, it would be unrealistic to expect CCGs to give priority in its commissioning decisions to neurology or any other condition area on the basis of quality standards alone.

3.19 The NHS CB and Department of Health need to go further to ensure that due consideration is given to neurological conditions in commissioning decisions, starting by appointing a national clinical advisor for neurology and devising an NHS CB outcomes strategy for neurological conditions.

3.20 We believe that NICE quality standards could play a positive role in the new NHS system architecture but that this potential will only be fulfilled if NICE and the Department of Health rapidly arrive at an agreement on a development timetable, based on robust criteria. From a neurological perspective, quality standards bear a great deal of responsibility for driving service improvements and, as such, their early development and publication is an imperative for patients, the NHS, Government and taxpayers alike.

October 2012

¹³⁵ Programme Budget Expenditure 2010/11—Department of Health

¹³⁶ In under-19s

Written evidence from Novartis Pharmaceuticals UK Limited (NICE 56)

Novartis is a leading global healthcare company providing a broad and diverse portfolio of healthcare solutions, including innovative medicines, eye care products, cost-saving generic pharmaceuticals, consumer health products, preventive vaccines and diagnostic tools.

The UK is a major centre for Novartis research, development, sales, marketing and manufacturing and the company employs over 3,200 people across the UK. Novartis medicines have been the subject of some 25 health technology appraisals (HTAs) by NICE, since its inception in 2000. Over the next two to three years we expect up to 30 new products or new indications of existing products to be appraised. Our submission to the Health Select Committee is based on this extensive experience of working with NICE. Novartis has contributed to and fully endorses the ABPI submission. However, there are some additional points that we feel should be raised with the Committee in more detail.

COMMERCIAL IN CONFIDENCE—CONFIDENTIALITY

Novartis continuously looks for ways in which to ensure speedy access for patients to innovative medicines particularly through Patient Access Schemes (PASs). In order to allow this practice to continue, Novartis believes that it is important that all pricing other than the published list prices should remain confidential. Not only does this mean that patients can have faster access to the most innovative medicines, but it also maintains a commercially competitive environment which encourages inward investment in R&D and clinical trials.

We would therefore expect NICE to continue to develop robust systems and processes to ensure that all pricing information remains confidential. We would argue that it is unnecessary for NICE to comment on any type of agreement, PAS or otherwise, as by nature of its approval, positive NICE guidance confirms that the treatment is cost effective.

APPRAISAL OF MEDICINES FOR RARE DISEASES

NICE has recently been asked to take on the work of the Advisory Group for National Specialised Services (AGNSS) to appraise medicines for conditions affecting up to 500 people in the UK. NICE has acknowledged that its appraisal methodology, most particularly the use of the Incremental Cost Effectiveness Ratio (ICER) as the sole measure of value, is not appropriate for these medicines, whose clinical development costs are on a par with medicines for more common conditions but where a return on investment has to be covered by a very small patient population. AGNSS worked within a framework that recognised a range of factors determining value, including patient need/disease severity and societal benefits, recognising that a broad balance is required to establish the value of the medicine. We would call on the new Chair of NICE to incorporate the learning from AGNSS into the NICE approach for all medicines.

We would also urge that NICE engages with all relevant stakeholders in the development of its interim assessment framework for the appraisal of ultra-orphan products before it begins its full consultation on methods in 2013–14.

QUALITY STANDARDS

Quality Standards play an important role in NHS performance management through the NHS Outcomes Framework, the NHS Commissioning Outcomes Framework, QOF, etc. There is considerable concern about NICE's capacity to develop the set of 150+ Quality Standards that it has been tasked to produce within five years. What is more, the choice of Quality Standards appears to rely too much on the availability of existing clinical guidelines and measurable indicators. This has the potential to skew local prioritisation decisions as priorities are driven by available Quality Standards, not necessarily local health needs. NICE should audit the implementation of its Quality Standards and their impact on services in disease areas that are not covered by Quality Standards.

UNLICENSED COMPARATORS

When a product undergoes an HTA, the manufacturer is asked to prove cost effectiveness against an existing treatment. In some instances, companies are asked to prove cost effectiveness against unlicensed medicines as comparators where no licensed alternative exists. Novartis believes that the use of such unlicensed medicines within HTAs cannot be justified either from a methodological or equity perspective. Decisions on the cost-effectiveness of licensed medicines should be based upon comparisons against medicines that have undergone the full rigour of regulatory risk-benefit evaluation and post-marketing safety monitoring.

Vital data about unlicensed comparators is often missing and this significantly limits the robustness and value of like-for-like comparisons. The presumption by HTA bodies that routine NHS use of unlicensed or off-label medicines, built up from years of custom and practice, automatically represents good practice is flawed, particularly where a licensed alternative exists; neither is the presumption valid that the full costs and risks of their use are known.

For a medicine to receive a licence (marketing authorisation) in the UK, the competent regulatory body, the MHRA (working in alignment with the EMA), must be satisfied as to its safety, efficacy and quality following

a scientific evaluation of the evidence and a robust risk/benefit assessment. The evidence is collected in extensive clinical trial programmes over many years, involving hundreds or thousands of patients (depending on the prevalence of the condition) and substantial investment in scientific and clinical expertise and resources. The licence stipulates therapeutic use(s), dose, dosage form and route of administration, together with full “labelling” including a patient information leaflet. A licence is also associated with obligations on companies to maintain a robust pharmacovigilance framework, which requires individual adverse event monitoring and reporting and periodic safety update reports to evaluate and inform any change to the risk-benefit ratio.

Where a medicine is used, for example, for both a different indication and in a different dosage form it is regarded as unlicensed.

October 2012

Written evidence from Myeloma UK (NICE 57)

Myeloma UK is pleased to be offered the opportunity to respond to the Health Select Committee Inquiry into the remit and work of the National Institute for Health and Clinical Excellence (NICE).

We are particularly pleased by the commitment of Health Select Committee to open the debate on how the operation of NICE will be affected by value-based pricing (VBP) which will come into effect on 1 April 2014. Given the limited detail that has been released by the Government on this subject to-date, we consider this a very timely Inquiry and hope it is used to inform the development of the VBP reforms, as well as the work programme of the incoming NICE Chair.

We would like to offer the following comments to the Inquiry. Please note that we have not responded to all topics the Inquiry has put forward to stakeholders, only to those that we feel we have expertise on.

SUMMARY

- There is an opportunity under the direction of the new NICE Chair to continue to build and develop relations with external stakeholders, in particular with patient organisations and industry, to ensure a collaborative approach to overcoming access to treatment barriers in England and Wales.
- NICE should further develop their capacity to work with industry to ensure that clinical trial design, data collection and economic modeling undertaken by industry is fit-for-purpose and up to the standard required to receive a positive NICE recommendation.
- Stakeholders—including NICE, patient organisations, industry, Government (both UK and European), the Department of Health (DH) and commissioners—need to work together to ensure that the system from clinical trial development through to approval of treatments and local implementation is seamless.
- Stop-gap measures such as patient access schemes (PAS) and end-of-life modifiers do not address the “root causes” of why treatments sometimes receive a negative recommendation by NICE. Improving the sophistication of data collection techniques and economic modeling, particularly as we move towards VBP will reduce the need for temporary measures. It will also serve to “fine-tune” the timeliness of NICE appraisals.
- There is wide-spread concern at the lack of detail released by Government on what the role of NICE will be in the new system of VBP. We would welcome detailed clarification on the responsibilities of NICE in the new pricing system, how this will impact on the devolved regions and how NICE guidance will be implemented by local health bodies.
- More information is needed on how the Government plans to reform the QALY threshold(s) to reflect “burden of illness” and “therapeutic innovation” and how the NICE procedural system will take into account “wider societal benefit”. This should be underpinned by strong empirical evidence to support the reasoning and be subject to peer-review.
- Whilst we understand and support the need to make it compulsory for local health bodies to fund NICE guidance for relevant patients, we strongly believe that hospitals and trusts should be encouraged to look at more flexible ways of prescribing treatments.

FULL COMMENTS

1. Relationship with NICE

1.1 Since its formation in 1999, and under the Chair of Professor Michael Rawlins, Myeloma UK has seen the work and the decision-making procedures/capacity of NICE go from strength-to-strength.

1.2 Over the years, we have had a number of high-cost novel myeloma treatments that have been through the NICE assessment process. These have included Velcade™ (bortezomib) and Revlimid™ (lenalidomide) both of which initially received a negative recommendation following an assessment by NICE and were subsequently overturned through the application of a PAS.

1.3 Whilst in the past we have not always agreed with the decisions reached, over the years we have developed a very open and honest working relationship with NICE and have come to understand the reasons why in certain situations NICE have had to make negative decisions on new medicines. Under the leadership of Michael Rawlins, NICE has developed a more flexible/pragmatic approach to decision-making and a strong willingness to work and develop relationships with both the pharmaceutical industry and patient organisations to overcome the issues that exist in approving new medicines for use on the NHS. We hope that this continues and positively evolves under the new NICE Chair.

1.4 Moving forward, there is a great need and opportunity for strategic collaboration between NICE and patient organisations such as Myeloma UK on areas and issues of mutual benefit—for example, as the discussion evolves about adaptive licensing, early engagement with industry and on the development of more sophisticated methods of evidence collection.

2. Clinical and cost-effectiveness data collection

2.1 Despite previous NICE reforms, there are still a number of treatments that do not get through its rigorous health technology assessment (HTA) processes. The reasons for this are often complex and multi-factorial, but in the majority of cases negative decisions stem from uncertainty about the clinical evidence and data supporting the medicine, combined with an unjustifiably high price.

2.2 In the case of Velcade in 2007, despite NICE accepting the clinical effectiveness of the drug, it was unable to approve it for use on the NHS due to the comparators used and participant cross-over during the trial. For these reasons, the pharmaceutical company was unable to adequately justify the cost-effectiveness of the treatment.

2.3 Since then NICE has developed more sophisticated ways of working with the pharmaceutical companies and signaling to them what is expected of their data prior to their submission. NICE are now able to review trial protocol and design (even back at Phase II to III) to provide advice to industry about their product, evidence collection, their economic modeling and on suggested areas for improvement.

2.4 Whilst this has improved the situation, this advisory relationship involving NICE and industry is voluntary and there are still situations that arise where treatments receive a negative decision due to uncertainty. There is therefore room for NICE to further make industry aware of the services that they offer in this respect and to proactively engage with pharmaceutical companies in the earlier stages of development.

2.5 As an aside to this, the Government also needs to recognise that this is not a situation that NICE can “fix” on their own, it is an issue that needs strong policy backing and joint-thinking between relevant stakeholders to resolve.

2.6 The problem usually stems back to the initial trial design, where the clinical questions being asked are not targeted to fit into an NHS clinical setting—something that is seen often in the larger commercial trials. Treatments then receive their European licence on this basis and by the time the treatment reaches NICE, it is difficult for NICE to assess clinical and cost-effectiveness.

2.7 It is clear that researchers, industry, government (both UK and European), licensing and HTA bodies need to work together to ensure that the system works seamlessly and that by the time treatments get to the approval processes there is very little uncertainty about their clinical and cost-effectiveness and how they fit into clinical practice. More consideration could also be given by industry, Government and NICE to academic research and NHS “bolt-on” studies and how these can be used to plug the gaps in the NHS relevant trial data.

2.8 VBP offers an opportunity to work towards improving the clinical evidence and data submitted to NICE and also to link NHS clinical evidence and real-life data into NICE appraisals. However, in light of the lack of detail within the development of VBP about how to best do this, this remains a critical question that needs further thought and discussion amongst stakeholders led by Government.

3. Stop-gap measures

3.1 In an attempt to resolve some of the problems outlined above, temporary “stop-gap” measures have been put in place to try and get more treatments through the NICE process and available to patients on the NHS—in particular in the form of PAS and end-of-life modifiers.

3.2 Whilst these have been effective in the short-term at getting treatments through the NICE process, in the long term they are not attempting to resolve the “root causes” as to why some treatments are not approved for use on the NHS. The application of PAS can actually serve to delay the NICE appraisal process and extend the time it takes for patients to obtain access to it.

3.3 Myeloma UK strongly believes that if we improve the trial design and the clinical and cost-effectiveness data collected by pharmaceutical companies, it would reduce the need for measures such as PAS and end-of-life modifiers, something that we hope to see under VBP. This would also promote a more equitable approval process across all diseases/conditions.

4. *Timeliness*

4.1 In recent years, a number of policy initiatives have been put in place to improve the time it takes from the licensing of new medicines by the EMA to having them approved by NICE and made available to patients on the NHS, for example NICE's single technology appraisal process. As a result, the timeliness of NICE appraisals has increased dramatically and where delays do occur in the process this is often due to legal action or commercial decisions (such as PAS).

4.2 NICE has recently received further criticism about the length it takes to complete appraisals. Whilst there is no robust evidence to suggest this is the case or detrimental to patients, it is important that NICE continually keep on top of their performance and always look to ways to improve working.

4.3 Under VBP if NICE are able to increase their ability to engage early with industry over issues such as trial design and data collection it may be possible to get treatments through the appraisal process faster due to the reduction of uncertainty and need for PAS.

5. *The process post-2014*

5.1 Myeloma UK has been very involved in VBP as it has developed. Although we accept that pricing methodology needs reform to incorporate better value judgements, we are minded to think that building on the reforms undertaken to the PPRS, when it was last renegotiated in 2009, as well more clearly defining the parameters by which NICE determines value, may have been a more sensible and workable approach. However, as VBP policy is imminent we are keen to make it the best it can be to the benefit of all stakeholders and most importantly to patients.

6. *NICE and VBP*

6.1 Myeloma UK has frequently expressed concerns to the UK Government and to the DH in England that the continuing lack of detail that has been published about VBP and the role of NICE in the new system is concerning. We would strongly welcome clarification from the Government on the specifics of VBP, given that 2014 is imminent.

6.2 Under current publically-known VBP proposals, NICE will assess a treatment according to specific pricing thresholds (on the basis of "burden of illness" and "therapeutic innovation"). Once NICE have undertaken this assessment, it will pass over to the DH in England to attach a final price to the treatment and to approve its use on the NHS.

6.3 The impact that this proposed amendment will have on the role of NICE raises a lot of unanswered questions, in particular (1) is NICE currently equipped with the necessary information from the Government to be able to take on this role in 2014? (2) are NICE being given an adequate role in the development of VBP? (3) what impact will the additional layer of approval have on the time it takes from licensing to NICE approval? (4) what impact will the amended role of NICE have on patient access in the devolved regions of the UK (particularly Scotland) given that value assessment of treatments will take place during pricing?

6.4 More specifically the operation of the QALY and the implementation of NICE guidance by local health bodies needs to be established in a lot more detail.

7. *QALY and wider societal benefit*

7.1 The system of assessing treatments on the basis of Quality Adjusted Life Years (QALY) has often faced wide-ranging criticism from stakeholders, in particular, due to the arbitrary nature of the threshold (which is around £30,000) and the rigidity of the tool in taking account of other elements aside from physical health such as wider value judgments.

7.2 Under VBP the proposal is to put in place QALY thresholds or "weightings", which will enable treatments to be attributed a price based on the value that the treatment will bring to the NHS. Treatments will be eligible for higher pricing thresholds if they are able to demonstrate, "therapeutic innovation" and the ability to address diseases with a greater "burden of illness." NICE are also looking into ways of incorporating and factoring in wider-societal benefit into their assessment process.

7.3 Whilst there is room to improve the capacity of the NICE process and the QALY to take these types of factors into account, Myeloma UK thinks that more empirical research needs to be undertaken into how NICE plan to assess these, what the QALY thresholds will consist of and how they will operate. In addition, any plans to amend the operation of the QALY or to take into account wider societal benefit in the assessment process should be subject to extensive peer review to ensure that the new system is "fit-for-purpose."

7.4 In addition, the terms "value" and "innovation" need to be adequately defined by the Government and there needs to be an acceptance that taking into account diseases with a higher "burden of illness" and assigning value on the basis of what patients bring to wider society may be potentially controversial and could widen health inequalities.

7.5 Finally, Myeloma UK believes that the development of VBP and the issues outlined above would benefit greatly from further NICE involvement in the development process due to their extensive experience in this area. The Government should make efforts to facilitate this in the lead up to 2014.

8. *Local uptake*

8.1 The Secretary of State for Health has recently confirmed the publication of the NHS Innovation Scorecard, which will rate local health boards on their uptake and implementation of NICE guidance.

8.2 Whilst we welcome this commitment to further monitor the health board implementation of guidance, we do have slight concerns about the messages that this sends to health boards about the rigidity of NICE guidance.

8.3 NICE guidance was designed to allow room for local clinical interpretation, but this has not been reflected in local commissioning decisions or processes to-date. Allowing flexibility in prescribing is particularly important for relapsing and remitting diseases such as myeloma where doctors rely upon using treatments in different combinations and innovative ways to treat patients at different disease stages.

8.4 We have evidence that health boards implement NICE guidance to the exact wording, and patients have difficulty in accessing treatments outside of the way NICE prescribes, even if deemed clinically the best option by their clinicians and supported by evidence. In order to improve outcomes and achieve value for money, the Government should encourage hospitals and trusts to look at more flexible ways of commissioning, which would better meet the interests of patients, clinicians and commissioners.

8.5 Finally, we would welcome clarification on whether the Innovation Scorecard will be used when VBP is implemented and if not, how is the Government going to encourage the local uptake of NICE guidance and flexible prescribing.

9. *Moving forward*

9.1 I hope that these comments are useful to the Health Select Committee as they conduct their Inquiry into the work of NICE. Please do not hesitate to contact us if we can provide any further information to support the Inquiry.

October 2012

Written evidence from Medical Marketing Consultants (NICE 58)

1. NICE TECHNOLOGY APPRAISAL PROCESSES AND VALUE-BASED PRICING

- From our survey of stakeholders on what they value about medical treatment and there is a huge disparity between what NICE and the NHS consider and what patients feel important. NICE appraisals need to be changed to reflect patient and tax-payers views of value. These include (detailed lists below):
 - Effectiveness/efficacy and safety, patient dignity, care needed by friends and family, convenience, side effects, time away from work, time to feeling completely well and the invasiveness of treatment as being very important, as well as costs to their employers and the economy.
 - There should be more patient and clinician involvement in NICE appraisals and reviews and QALYs and thresholds should be changed to better reflect patient needs and experience.
- The implications for patients are likely to be slower uptake and access to new medicines and treatments due to the uncertainty over pricing and increased costs to the pharmaceutical industry.
- The savings suggested by OFT are unrealistic and unlikely to be achievable.
- Non-compliance by patients, particularly to older medicines with unpleasant side effects have not been considered.
- Patient outcomes should be pegged against those of other leading EU countries and considered in NICE recommendations.

2. NICE QUALITY STANDARDS AND CLINICAL GUIDELINES

- Research carried out with the patient group FEmISA and the Medical Technology Group on all English PCTs and acute NHS Trusts showed that:
 - Some PCTs ignore NICE Clinical Guidelines and do not allow patient access to a less invasive, less expensive treatment which allows women to retain their fertility.
 - Few PCTs or Acute NHS Hospitals have mechanisms to ensure patients are fully informed about their treatment options.
 - Most women in the survey wanted much more information about their treatment options.

- Most women [60–70%] receive the most expensive—to the NHS and patients, most invasive, oldest hospital treatment, with the longest recovery time, which leaves women infertile—abdominal hysterectomy.
- The Government should consider making NICE Clinical Guidelines mandatory for PCTs and CCGs and acute NHS Trusts.
- There should be a requirement and mechanisms to ensure all patients and properly and fully informed about treatment options.
- Outcomes and treatment uptakes should be monitored by PCT/CCG and compared with outcomes from leading EU countries and published so the public can scrutinise.

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

We carried out a project for a health think tank on value-based pricing, which included unique research into how the various stakeholder groups value medical treatment, including patients, clinicians, suppliers and NHS managers. As far as we could discover there has been no previous research into this. We also conducted 35 individual interviews with patient groups, Government and the NHS, NICE, health economics, academics and the Royal Colleges.

There is a huge disparity between what NICE and the NHS “value” and what patients and tax-payers “value”. Effectiveness/efficacy and safety rank top with all stakeholder groups researched, but patients and other stakeholders also consider their dignity, care needed by friends and family, convenience, side effects, time away from work, time to feeling completely well and the invasiveness of treatment as being very important, as well as costs to their employers and the economy. Also, consideration of value for treatments for acute (short-term), chronic (long-term) and end of life disease differ and this should be taken into account.

Thirty five stakeholders interviewed were conducted about the implications of the new PPRS scheme: patient groups; Government and NHS, including NICE, Department of Health (DH) and OFT; Royal Medical Colleges; academic health economists and policy makers; medical and pharmaceutical trade associations; marketing professionals and pharmaceutical, biotechnology and medical companies.

Non-Government respondents felt that “value” should reflect value to patients and other stakeholders and many also thought wider costs to society and the economy. Patient access schemes were thought a useful access route by some patient groups, but the risk was borne by the company and not shared. They should be seen as an opportunity to collect more data on the treatment and data on outcomes. It is very important that a process for this is set up.

NICE should include wider patient costs in their appraisals and that QALYs (Quality Adjusted Life Years—the assessment used by NICE) should be modified to take this into account. It should also be recognised that different ages, severity of disease, and whether it was acute, chronic, rare or terminal should all be taken into account. One equation does not fit all! Many wanted greater patient participation in NICE appraisals of value, one patient group advocated greater inclusion of clinicians, as they not only understand patient needs, but also the treatments. There was wide agreement among non-government stakeholders that cost effectiveness assessment criteria need to change.

Some saw value reviews as an opportunity to demonstrate the value of a product after launch in order to command a higher price and to show that it would promote medical innovation. Others felt that it would have the opposite effect. Some commented that when a new technology is approved by NICE the NHS is slow to decommission old ones, especially where there is little evidence of clinical effectiveness.

NICE Technology Appraisal Processes and Value-Based Pricing: we recommended the following changes:

- Wider Social and Economic Costs and Benefits Should be included to patients as well as the NHS including.
- The QALY Should be Reviewed:
 - Categorising Diseases—five different categories of disease to be regarded differently:
 - Acute diseases eg infections.
 - Chronic—long-term disease.
 - End of life disease.
 - Rare disease.
 - Paediatric disease.
- NICE thresholds should be reviewed.
- Medical technologies and diagnostics should have redesigned appraisal processes.
- Outcomes should be made part of the appraisal and pegged to outcomes in other leading EU countries.
- NICE appraisals should be subsidised, especially for smaller companies.

- Price cap increase review.
- Explanation of different prices for different indications.
- *Increasing the patent life of medicines should be explored with EU.*

The implications of value-based pricing are to increase costs to companies above those of other EU and developed countries to launch a new medicine and possibly new medical technologies in later years. A NICE appraisal is *estimated to cost an additional £4.5 million for each product, while the appraisals themselves cost an average of £350,000* [source ABPI]. This is prohibitive to small companies.

The implications are likely to be:

Higher prices of new treatments at launch to cover higher costs from:

- Additional NICE appraisals.
- *Further delays to adoption, use and patient access to new treatments—the UK is one of the slowest developed countries to adopt new medical treatments—medicines and technologies* [ABPI, Medical Technology Group].
- Greater pricing instability, as list prices may be reduced post-launch thus affecting ROI (return on investment).
- Considerably more expense and bureaucracy for medical companies.
- An impact on world prices—as the UK is used for international price benchmarking thus.
- Some companies may choose to launch late in the UK, leading to:
 - A reduction in UK clinical trials and research.
 - Commercially funded clinical research in UK—may be reduced.
 - New treatments would not be available in the UK or be delayed.
 - Reduced employment in UK from medical companies.
 - Reduced investment in the UK by medical companies.
- Parallel Exporting and supply problems for UK patients.

The original OFT report that recommended value-based pricing suggested significant savings of £500 million. This is unrealistic and is unlikely to ever been realised. In addition to the issues raised above the cost of patient non-compliance/adherence has not been considered.

The WHO report that:

- Adherence among Patients suffering from *chronic diseases averages only 50%:*
 - *Among non-adherent patients, 76% of patients consciously choose not to be adherent.*

The National Community Pharmacists Association [USA] survey found that (% of respondents):

- 49% admitted forgetting to take a prescribed medicine.
- 31% had not filled a prescription they had been given.
- 29% stopped taking a medicine before the supply ran out.
- 24% had taken less than the recommended dose.

Non-adherence is due to a number of issues one being unwanted side effects, particularly from older drugs, which are not sufficiently taken into account by NICE. The cost of patient non-adherence has widely different estimates of costs to the UK tax payer, but few consider the additional costs in the burden of disease.

Here is a link to the summary of the report—<http://www.medicalmarketingconsultants.co.uk/index.php?/valuing-medical-treatments-and-value-based-pricing.html>

Here is a link to the complete report—<http://www.medicalmarketingconsultants.co.uk/images/stories/documents/value%20based%20pricing%20report.pdf>

- *The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities.*
- *The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome.*

We support a voluntary patient group FEMISA [Fibroid Embolisation: Information, Support and Advice www.femisa.org.uk] FEMISA is a member of the Medical Technology Group, whose aim is to improve patient access to new medical technologies. We carried out a recent Freedom of Information request to all English PCTs and acute NHS Trusts about the patient access to uterine artery/fibroid embolisation. It is an interventional radiology treatment for uterine fibroids, which is a less invasive and less expensive to the NHS and patients. Despite this some PCTs do not allow patient access and do not commission it, and there are great disparities between treatments. Most women are not fully informed about their treatment choices and not offered less invasive treatments. Most women 96–700% receive the oldest, most invasive and most expensive treatment,

which makes them infertile and has a long recovery time—abdominal hysterectomy and great cost to the women and the NHS.

The NHS could save in excess of—approx £3million and 70,000 bed days

Society could save:

| | |
|---|---------------------|
| Return to work/normal life 1–2 weeks with embolisation c.f. Hysterectomy 3 months | |
| 2 weeks off work versus 3 months—working days saved | 1,251,327 |
| Average weekly earnings Jun '12 [Office for National Statistics] | £468.00 |
| Potential economic saving from earlier return to work from UFE | £117,124,207 |

See full cost saving details here on the FEmISA web site—<http://www.femisa.org.uk/index.php/cost-comparisons>

The FOI survey results show:

- significant variation among the PCTs and Acute Trusts who responded in the numbers of woman undergoing UFE as a treatment for fibroids;
- in a number of PCT areas in England UFE is not being routinely commissioned for women; and
- some PCTs were unaware of new Best Practice Tariff for UFE and there is currently a lack of patient group involvement in commissioning fibroid treatments.

The FEmISA patient survey shows:

- that many women did not feel that they had been given enough information about treatment choices or options for their fibroids from their GP or gynaecologist.

A set of recommendations for the Government, NHS commissioners and healthcare professionals have been developed to address the challenges identified by this survey:

Link to full report here—<http://www.mtg.org.uk/index.php/policy-initiatives/mtg-campaigns/8-a-review-of-the-provision-of-and-access-to-uterine-artery-fibroid-embolisation-a-treatment-for-fibroids-for-women-in-england>

October 2012

Written evidence from the British Medical Association (NICE 59)

The British Medical Association (BMA) is an independent trade union and voluntary professional association which represents doctors from all branches of medicine all over the UK. It has a total membership of over 150,000.

EXECUTIVE SUMMARY

- While the BMA commends the role that NICE plays in evaluating the clinical and cost-effectiveness of new treatments, their health technology assessments only apply to a small proportion of the NHS' offering. Access to the vast majority of treatments in use in the NHS remains at the discretion of local NHS commissioners.
- NICE recommendations are not accompanied by new funding and the recent requirement for NHS commissioners to fund NICE-approved technologies immediately will put even more pressure on commissioners than at present.
- There should be greater emphasis on helping NHS commissioners and providers to implement NICE's "do not do" recommendations as this could lead to improvements in relation to costs, quality and equity.
- New value based pricing proposals mean that in the future it will be the government, not NICE, who will determine the appropriate threshold in order for new drugs and technologies to be made available on the NHS. NICE will focus predominantly on clinical effectiveness, which the BMA considers to be a beneficial change.
- How reliably the 44 indicators that NICE has suggested for inclusion in the Commissioning Outcomes Framework (COF) can assess the performance and contribution of commissioners is questionable. Furthermore, the COF should not be used for the allocation of the quality reward for Clinical Commissioning Groups.
- There is a lack of clarity on how and whether local authorities will be supported and assessed in their new role as commissioners of health care services previously commissioned by Primary Care Trusts.
- Given the complexity of the new public health system, NICE's public health guidance will need to be mindful of the many different players involved.

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

1. The most high-profile aspect of NICE's work is that of health technology assessments (HTA). Currently, once NICE has approved a new drug or surgical intervention for use in the NHS, largely on the basis of relative cost-effectiveness, a funding mandate obliges NHS commissioners to provide access to it if clinicians believe it would benefit their patients.¹³⁷ Before 2005, this decision was down to local discretion. However, Primary Care Trusts (PCTs)—and from April 2013 Clinical Commissioning Groups (CCGs)—still determine access to the vast majority of treatments in use in the NHS. This is because the majority have not and will not be subject to NICE HTA.

2. While the BMA commends the role that NICE plays in evaluating the clinical and cost-effectiveness of new treatments, in reality these principles only determine a relatively small proportion of the NHS' offering. In 2007 the BMJ estimated that just 13% of treatments offered are known to be clinically effective and that 48% have uncertain effectiveness.¹³⁸ These figures are very poor and the NHS should consider working towards improving them over a specific timeframe, for example by achieving 26% known clinical effectiveness within four years.

3. Compliance with the funding mandate is not a given and the latest national directive to NHS commissioners comes in the form of the "innovation scorecard".¹³⁹ Previously, commissioners were expected to fund a new technology within three months of the NICE recommendation. Now access is expected to be immediate. Given the combined pressures of the NHS spending freeze, the Quality, Innovation, Productivity and Prevention programme and rising demand, the weight of this obligation on NHS commissioners' should not be underestimated. Furthermore, NICE recommendations are not accompanied by new funding.

4. A by-product of NICE's HTA work, Quality Standards and clinical guidelines is their "do not do" recommendations database, listing interventions that it advises against being used in the NHS, on the basis of lack of evidence on the benefits.¹⁴⁰ As NHS commissioners face increasing cost pressures, the decommissioning of existing services will become vital if they are to continue to fund new, expensive drugs and technologies in the future. For this reason, NICE's "do not do" recommendations are just as important, if not more so, than its HTA work. At present, most PCTs engage in priority setting activities¹⁴¹ on an individual basis in order to exclude certain services from the local NHS benefits package. These exclusion lists tend to differ from PCT to PCT and in 2011 it was estimated that there were around 250 different treatments or procedures, which were subject to some kind of restriction in the country.¹⁴² NICE's "do not do" work is relatively unknown and a greater emphasis on helping NHS commissioners (and providers) to implement their recommendations could lead to improvements in relation to costs, quality and equity.

5. Another area of controversy relating to NICE's HTA function arises from methodological concerns, namely the use of the Quality Adjusted Life Year (QALY) as a measure of cost-effectiveness. QALYs have been criticised on the basis of being biased against the old and disabled¹⁴³ and there remain a number of unresolved issues with valuing health and estimating disability weights.¹⁴⁴ To address the fact that QALYs may not capture all elements of social value, NICE considers other factors alongside cost-effectiveness such as the lack of or inadequacy of alternative treatments, the seriousness of the condition, affordability from the patient perspective were the NHS not to fund an intervention and equity objectives. However debate continues around the validity of there being no empirical basis for the implicit threshold used by NICE of £20–30,000 per QALY.¹⁴⁵

6. The government's solution to this problem is to place the onus for affordability onto the pharmaceutical industry by introducing value based pricing (VBP). Under this arrangement, the calculation of affordability will no longer regard the cost of medicines as exogenous. Instead NICE will evaluate the effectiveness of a medicine or treatment and this will be combined with price thresholds determined by the government to produce the maximum amount which should be paid by the health care system for a medicine or technology with a given health outcome. The industry will then face a choice between supplying at or under that price, or not supplying to the NHS.

7. The BMA has been generally supportive of the HTA role of NICE and, in many ways VBP will operate in similar fashion. However one concern has been that the threshold used by NICE was too inflexible and there

¹³⁷ Department of Health (2012). *The NHS Constitution for England* London: Department of Health.

¹³⁸ BMJ (2007). *BMJ Clinical Evidence Handbook* London: BMJ Group

¹³⁹ Department of Health (2012c). *Patients to get the latest drugs and treatments more quickly* press release accessed online on 5 September 2012 <http://mediacentre.dh.gov.uk/2012/08/28/patients-get-latest-drugs-treatments-more-quickly/>

¹⁴⁰ NICE website (2012). *NICE "do not do" recommendations* accessed on 18 August 2012 at <http://www.nice.org.uk/usingguidance/donotdorecommendations/index.jsp>.

¹⁴¹ Nuffield Trust (2011). *Setting priorities in health: the challenge for clinical commissioning* London: Nuffield Trust.

¹⁴² Audit Commission (2011). *Reducing Spending on Low Value Clinical Treatments* Health Briefing April 2011.

¹⁴³ Oliver A, Sorensen C (2005). "The limits and challenges to the economic evaluation of health technologies" in Costa-Font J, Courbage C, McGuire D (eds.) *Economics of Innovation and New Health Care Technologies* Oxford: OUP.

¹⁴⁴ Dolan P (2000). "The measurement of health-related quality of life for use in resource allocation decisions in health care" in Culyer A J, Newhouse J P (eds.) *Handbook of Health Economics* Amsterdam, Oxford: Elsevier.

¹⁴⁵ Klein R, Maybin J (2012). *Thinking about rationing* London: King's Fund.

were also grounds for thinking it might be too high.¹⁴⁶ Under the value based system of course too high translates into too low since the government is setting a price threshold and not a cost one. The government rather than NICE will in the future be the judge of the appropriate thresholds and whilst it has indicated that there will be price ranges under VBP, reflecting the contributory aspects of value, including the additional therapeutic benefits, the quality of innovation, the response to unmet need, and societal benefits, the financial climate is now considerably worse than when the system was first mooted and it will inevitably be seen as a cost-constraint measure.

8. VBP will only apply to new medicines introduced after 2014 and might only involve 20 to 30 per year. The NHS in England will continue to fund existing drugs that have been recommended by NICE and presumably continue to deny access to those which have hitherto failed the existing cost-effectiveness test. Critically for NICE though, going forward it will be able to concentrate on gathering the evidence to determine clinical effectiveness and will no longer need to establish a cost threshold. The BMA considers this to be a beneficial change.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

9. While NICE's HTA work is binding on the NHS through the funding mandate, NICE's Quality Standards are not binding on individual doctors, providers or commissioners. The current list of 22 is expected to expand to 150 by 2015. While currently the National Quality Board recommends topics to NICE, in the future this will become part of the NHSCB's role. This appears to be the most appropriate place for this function to lie provided that the NHSCB maintains a dialogue with CCGs on potential topics given that they will be commissioning the majority of services in the NHS.

10. The implementation of NICE Quality Standards and clinical guidelines can be slow and in recognition of this a new NICE Implementation Collaborative will be established later this year, to support faster adoption of such guidelines in the NHS.¹⁴⁷ The BMA would welcome any efforts to improve adoption of evidence-based, best practice in the NHS. However, it should also be noted that problems can and do arise as a result of NICE guidance. An example of this is clinical guideline CG88, which states that interventions such as radiofrequency facet joint denervation should not be offered to patients with low back pain. However, we are aware of a situation whereby some PCTs misinterpreted the guidance as also being applicable to chronic back pain, which is a distinct condition. As a result, this intervention, which can be appropriate for some patients with chronic back pain, was no longer offered, causing distress amongst patients and an increase in follow-up appointments. The usual timeframe for NICE guidance to be reviewed does not necessarily allow for problems such as this one to be resolved quickly.

11. The new Commissioning Outcomes Framework (COF) is currently being designed in order for the NHSCB to assess the performance of CCGs in the future. As part of the process, NICE has recommended 44 indicators in total: 14 based on the NHS Outcomes Framework and 30 on NICE Quality Standards and other indicator collections. The NHSCB is currently deciding which of these indicators to include in the COF and how it will be used in practice. While all 44 indicators have been through a very thorough and transparent process of assessment and may be appropriate for measuring aspects of the performance of providers, how well they can assess the performance and contribution of commissioners is questionable. In our response to the NICE consultation on a draft set of indicators in February, the BMA voiced concern that the list was too detailed and could restrict the freedom of commissioners to respond to local need as they aim instead to meet centralised targets. It is also BMA policy that the COF should be used for patient and peer information and not for the allocation of the quality reward for CCGs.¹⁴⁸

12. In keeping with the current focus in the NHS, the majority (29) of the indicators are outcome measures and the remaining 15 clinical process indicators. However, the validity of many outcome measures is debatable. While they are undoubtedly more meaningful, disadvantages include that they require large sample sizes in order to be statistically significant, some take many years to materialize and there are many confounders. Furthermore, attribution of outcomes to the activities of the health service can be problematic as can the interpretation of differences between providers—let alone commissioners—in relation to measures such as mortality or complications as they may not indicate differing quality.^{149,150} Process and clinical process measures on the other hand are more actionable, reflect compliance with good practice and are easier to attribute directly to health services. That said, process measures are easier to manipulate, and the development of clinical process measures schemes can be subject to bias, for example through the (subjective) selection of

¹⁴⁶ Martin S, Rice N, Smith P (2007). *The Link Between Health Care Spending and Health Outcomes: Evidence From English Programme Budgeting Data*. Centre for Health Economics, University of York.

¹⁴⁷ Department of Health (2011). *Innovation, Health and Wealth: accelerating diffusion and adoption in the NHS* London: Department of Health.

¹⁴⁸ Annual Conference of Local Medical Committees 2012

¹⁴⁹ Smith P C, Busse R (2010). Chapter 5.1 "Targets and performance measurement" in Smith P C, Mossialos E, Papanicolas I, Leatherman S (eds.) *Performance Measurement for Health System Improvement: experiences, challenges and prospects* Cambridge, Cambridge University Press.

¹⁵⁰ Nolte E, Bain C, McKee M (2010). Chapter 2.1 "Population Health" in op. cit. *Performance Measurement for Health System Improvement: experiences, challenges and prospects*.

conditions of interest and review of clinical evidence, which is subject to publication bias (only positive findings may be published).¹⁵¹

13. There are well-recognised risks inherent in any performance measurement framework, which apply equally to the COF. Systems can lead to “tunnel vision” whereby there is distorted focus and behaviour so that what is measured, matters.¹⁵² Another problem that can arise is that what is important cannot be measured, and the necessary process of selecting indicators means that the performance of a complex organisation is assessed through only a few targets or objectives.¹⁵³ This is certainly the case with the emerging COF as the clinical areas the 44 indicators cover are limited and do not account for the full range of services for which commissioners will be making provision and the associated activities they will undertake, such as the promotion of integration. Strategies for minimising the unintended consequences of performance measurement schemes include that staff should be involved in their development, systems should be flexible and kept under constant review and countering gaming should be integral to the design process.¹⁵⁴

The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

14. See paragraphs 1–2, 4 and 9–10 above.

The effect of the new public health system architecture on NICE’s continued role in respect of public health guidance

15. NICE is currently considering how it can work more closely with local government and developing a guide to how NICE’s public health guidance maps on to the new Public Health Outcomes Framework (PHOF).¹⁵⁵ The BMA agrees that it is important for the appropriate relationship to develop between NICE and local authorities (LAs) as not only will LAs will be responsible for the public health function from April 2013, but they will also take on new commissioning responsibilities previously undertaken by PCTs. These include the majority of sexual health services, alcohol and drug misuse services, public mental health services, children’s public health services from five to 19 years and from pregnancy to five years from 2015.¹⁵⁶ However, there is a lack of clarity at present on how LAs as commissioners of NHS services will be supported and assessed. Given that CCGs will be subject to the COF it would seem logical that equivalent arrangements also apply to LAs.

16. Given the complexity of the new public health system, NICE’s public health guidance will need to be mindful of the many players involved, and which of them are expected to have regard to the guidance should also be made more explicit. The new section of NICE’s website “NICE pathways” has started to bring together all of its work on HTA, Quality Standards, clinical and public health guidance and implementation tools in one place. Hopefully this will make it clearer to LAs, which aspects of NICE’s work will be relevant to them in the future.

17. It is also worth noting that the accountability arrangements relating to the PHOF are more complex than those for the NHS Outcomes Framework. For example, progress against the individual PHOF indicators is expected to be built into Joint Strategic Needs Assessments and Joint Health and Wellbeing Strategies. And LAs in partnership with Health and Wellbeing Boards are also expected to demonstrate improvements. Further there will be a link between the PHOF and the health premium for LAs which will incentivise certain of the indicators. The new national body Public Health England (PHE) will also have a role in delivering a number of the outcomes.¹⁵⁷

What effect NICE’s new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

18. There are currently two pilots underway within NICE to create social care Quality Standards: care of people with dementia and the health and wellbeing of looked-after children. These topics were decided by the Departments of Health and Education however who is involved in future recommendations to NICE should be considered in the context of the new NHS architecture. Presumably as is the case with the NHS, social care Quality Standards will not be binding on commissioners or providers of social care.

19. While there are 128 quality standards for NHS care currently in the pipeline, it is not clear if any such targets might be set for the development of social care Quality Standards and guidance in the future. It is also

¹⁵¹ McGlynn E A (2010). Chapter 2.3 “Measuring clinical quality and appropriateness” in op. cit. *Performance Measurement for Health System Improvement: experiences, challenges and prospects*.

¹⁵² Smith P C (1995). “On the unintended consequences of publishing performance data in the public sector”. *International Journal of Public Administration* 18: 277–310.

¹⁵³ Bevan G, Hood C (2006). “What’s measured is what matters: targets and gaming in the English public health care system”. *Public Administration* 84(3): 517–38.

¹⁵⁴ Op. cit. “On the unintended consequences of publishing performance data in the public sector”.

¹⁵⁵ NICE website (2012) accessed online on 16 October <http://www.nice.org.uk/localgovernment/Localgovernment.jsp>

¹⁵⁶ Department of Health (2012). *Healthy Lives, Healthy People: Update and Way Forward* London: Department of Health.

¹⁵⁷ Department of Health (2012). *Improving outcomes and supporting transparency. Part 1: A Public Health Outcomes Framework for England, 2013–16*. London: Department of Health.

unclear whether NICE's predominant focus will remain health rather than social care, or whether the intention is to give more equal attention to the two over time. It would certainly be useful to have more evidence in general on what kinds of social care interventions are effective and those that are not. The Care Quality Commission (CQC) has recently announced that it intends to start gathering and using evidence and information on social care to better effect.¹⁵⁸ It is important therefore that NICE and the CQC work more closely together in the future.

20. Regarding the integration of health and social care, there may be some scope for NICE to develop Quality Standards for common pathways of care that span both NHS/health and social care services. Much work is already underway within the Department of Health, Monitor, the NHSCB, PHE and the NHS Institute for Innovation and Improvement as to how to facilitate the implementation of greater integration of health and social care services. Were NICE to contribute to this work programme, it should of course complement rather than duplicate work already underway.

October 2012

Written evidence from Karl Claxton, Steve Martin, Marta Soares, Nigel Rice, Eldon Spackman, Sebastian Hinde, Peter C Smith and Mark Sculpher (NICE 61)

SUMMARY

- Amongst others, the Health Select Committee has previously called for more research into the NICE cost effectiveness threshold.
- We report on research funded by the National Institute for Health Research (NIHR) and Medical Research Council (MRC) Methodology Research Programme to develop methods to estimate the threshold.
- We estimate the relationship between changes in overall NHS expenditure and changes in mortality and extend this to changes in broader health effects in the form of quality-adjusted life-years (QALYs).
- Our central estimate of the threshold is £18,317. The probability that the overall threshold is less than £20,000 per QALY is 0.64 and the probability that it is less than £30,000 is 0.92.
- Although other assumptions and judgments are possible that retain some level of plausibility, they do not all favour a higher threshold. Indeed, when considered together, they suggest that, on balance, the central estimate is, if anything, likely to be an overestimate.

1. INTRODUCTION

1.1 NICE's comparison of the incremental cost effectiveness ratio (ICER) of a new technology, which is more costly than existing alternatives, with the cost-effectiveness threshold is important in assessing whether the health expected to be gained from its use exceeds the health expected to be forgone elsewhere as other NHS activities are displaced (ie whether the new technology is cost effective).

1.2 When NICE issues positive guidance for a new intervention which imposes additional costs on the NHS, the resources required to deliver it must be found by disinvesting from other interventions and services elsewhere. This displacement will result in health decrements for other types of individual. Thus the threshold represents the additional cost that has to be imposed on the system to forgo 1 QALY worth of health through displacement.

1.3 Currently NICE uses a threshold range of £20,000 to £30,000 QALY gained. Amongst others, the Health Select Committee has asked for further research on the value of the threshold.¹⁵⁹

1.4 This submission provides a summary of a draft report on a two-year project funded by the NIHR and MRC Methodology Research Programme to develop methods to estimate the NICE cost effectiveness threshold.¹⁶⁰

1.5 Given NICE's remit, empirical research on the threshold requires some key characteristics:

- Reflect the expected health effects (in terms of length and quality of life) of NICE guidance through the displacement decisions taken across the NHS rather than what specific services are (or could have been) displaced.
- Facilitate regular updates, based on routinely available data, to reflect NHS changes such as real overall expenditure and productivity. This would encourage accountability through scrutiny by stakeholders and provide predictability for technology manufacturers' investment decisions.
- The nature of service displacement and the magnitude of the health forgone will depend on the scale of the budget impact which should, ideally, be reflected in the value of the threshold.

¹⁵⁸ Care Quality Commission (2012). *CQC: The next phase. Our consultation on our strategy for 2013 to 2016.*

¹⁵⁹ House of Commons Health Committee. *National Institute for Health and Clinical Excellence: First report of the Health Committee 2007–2008. HC27-I.* London: Stationery Office, 2008.

¹⁶⁰ Full draft report available at <http://www.york.ac.uk/che/research/teehta/methodological-research/#tab-4>

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- Methods should recognise the inevitable uncertainty relating to the evidence currently available for the threshold and reflect its implications for policy.

2. STUDY METHODS

2.1 The aim was to develop methods to estimate the NICE cost-effectiveness threshold making use of routinely available data. Objectives were:

- (i) Informed by relevant literature, to provide a conceptual framework to define the threshold and the basis of its estimation.
- (ii) Using programme budgeting data for the English NHS, to estimate the relationship between changes in overall NHS expenditure and changes in mortality.
- (iii) Extend the measure of benefit in the threshold to QALYs by estimating the quality of life (QoL) associated with additional years of life and the direct impact of health services on QoL.
- (iv) Present the best estimate of the cost effectiveness threshold for policy purposes.

2.2 Earlier econometric analysis estimated the relationship between differences in primary care trust (PCT) spending and associated disease-specific mortality.¹⁶¹ Expenditure came from programme budgeting data which allocates the entire volume of health care expenditure to broad programme budget categories (PBCs) according to primary diagnosis.

2.3 The recent research extended this in several ways including estimating the impact of marginal increases or decreases in overall NHS expenditure on spending in each of the 23 PBCs. These were linked to changes in mortality outcomes by PBC across 11 PBCs.

2.4 The results of the econometric analysis were translated into broader effects in terms of QALYs. The first stage linked estimated effects on mortality to life years taking into account the “counterfactual” deaths that would have occurred if the population in a given PBC faced the same mortality risks as the general population. The second stage accounted for the health (QALY) effects of changes in mortality due to changes in expenditure reflecting how QoL differs by age and gender. The third stage incorporated those effects on health not directly associated with mortality and life year effects (ie, the “pure” QoL effects) to estimate an overall cost per QALY threshold. This effectively used the estimates of mortality and life year effects as a “surrogate outcome” for a more complete measure of the health effects of a change in expenditure.

3. CENTRAL OR “BEST” ESTIMATE OF THE THRESHOLD

3.1 The most relevant threshold is estimated using the latest available data (2008 expenditure, 2008–10 mortality). The central or “best” threshold is estimated to be £18,317 per QALY.

4. WHICH PBCs HAVE THE GREATEST INFLUENCE ON THE OVERALL THRESHOLD?

4.1 Although the 11 PBCs where a mortality effect of changes in expenditure could be estimated only account for 36% of the change in overall expenditure, they account for 80% of the overall health effects. The other 12 PBCs, where mortality effects could not be estimated, account for the greater part of a change in overall expenditure (64%) but only 20% of the overall health effects, ie, the cost per QALYs estimates associated with a change in expenditure in these PBCs are, in general, much higher.

4.2 Insofar as investment and disinvestment opportunities in these PBCs might have been more valuable (offered greater improvement in QoL) than suggested by the implied PBC thresholds, the overall QALY effects will tend to be underestimated and the overall cost per QALY threshold will be overestimated.

4.3 The overall threshold of £18,317 may be especially conservative (ie, likely to be overestimated) with respect to health effects in PBC5 (Mental Health Disorders), which accounts for a large proportion of the change in overall expenditure (25%) and contributes most to the overall health effects (9%) compared to these other PBCs. The cost per QALY associated with this PBC is based on an extrapolation rather than observations of the direct impact of changes in expenditure on QoL. Available evidence suggests that the investment and disinvestment opportunities in mental health are likely to have been much more valuable than its implied cost per QALY.

5. HOW UNCERTAIN ARE THE ESTIMATES AND WHAT ARE THE IMPLICATIONS?

5.1 Simulation methods were used to reflect the combined uncertainty in the various estimates from the econometric analysis. This indicated that the probability that the overall threshold is less than £20,000 per QALY is 0.64 and the probability that it is less than £30,000 is 0.92.

5.2 As the consequences of overestimating the threshold are more serious than underestimating it in terms of population health, a *policy threshold* will be lower than the mean of the cost per QALY threshold (ie, lower than £18,317) to compensate for the more serious consequences of overestimating the “true” value.

¹⁶¹ Martin S, Rice N, Smith P C. Does health care spending improve health outcomes? Evidence from English programme budgeting data. *Journal of Health Economics* 2008; 27:826–42.

5.3 There were other (“structural”) sources of uncertainty associated with the estimated threshold, specifically relating to the choice of econometric models and identification of causal effects. Although all the models passed the relevant tests of validity, there remained some uncertainty about the validity of the instruments. This structural uncertainty constituted a greater part of the overall uncertainty associated with the mortality effects of changes in expenditure, but the central estimate of the cost per QALY threshold was robust to this uncertainty.

5.4 The methods of analysis used to link the effects of changes in expenditure on mortality to a fuller measure of health expressed in QALYs was also subject to uncertainty. A preferred analysis (or scenario) was identified as making the best use of available information, with assumptions appearing more reasonable than the available alternatives and providing a more complete picture of the likely health effects of a change in expenditure.

5.5 A critical issue is whether, on balance, the central or best estimate is likely to be an underestimate or overestimate of the cost per QALY threshold. Although other assumptions and judgments are possible that retain some level of plausibility, they do not all favour a higher threshold. Indeed, when considered together, they suggest that, on balance, the central or best estimate of £18,317 is, if anything, likely to be an overestimate.

6. THE IMPACT OF INVESTMENT, DISINVESTMENT AND NON-MARGINAL EFFECTS

6.1 The central estimate of the cost per QALY threshold is based on estimates of the health effects of changes in expenditure across all 152 PCTs, some of which will be making investments (where expenditure is increasing) and others making disinvestments (where expenditure is reduced or growing more slowly).

6.2 The threshold is, however, likely to differ across these different types of PCT. It would be expected that, other things equal, more expenditure would increase health but at a diminishing rate. Therefore, the amount of health displaced by disinvestment would be expected to be greater, and the associated threshold lower than the central estimate. Conversely, the health gained from investment would be expected to be lower, and the associated threshold higher.

6.3 This was examined by re-estimating the outcome and expenditure effects separately for those PCTs where their actual budget is under the target allocation from the Department of Health resource allocation formula (ie, those under greater financial pressure and more likely to be disinvesting than investing), and those that are over target (under less financial pressure and more likely to be investing than disinvesting).

6.4 The results confirm these expectations: the health effects of changes in expenditure are greater when PCTs are under more financial pressure and are more likely to be disinvesting than investing. The analysis suggests that budget impact not only displaces more valuable activities within each PBC but that overall expenditure tends to be reallocated to PBCs which can generate more health. Although further research might enable a quantitative assessment of how the relevant threshold should be adjusted for the scale of budget impacts, the qualitative assessment seems clear: the central estimate of the threshold is likely to be an overestimate for all technologies which impose net costs on the NHS (almost all technologies appraised by NICE); and the appropriate threshold to apply should be lower for technologies which have a greater impact on NHS costs.

7. HOW DOES THE THRESHOLD CHANGE WITH OVERALL EXPENDITURE?

7.1 The same methods are used to consider how the cost per QALY threshold is likely to have changed from 2007 to 2008 as overall expenditure has increased. This provides some insights into how the threshold might be expected to change over time as, for example, overall expenditure and NHS productivity changes.

7.2 This has implications for a judgement about the appropriate frequency of periodic reassessment of the cost per QALY threshold. Other things equal, the threshold would be expected to increase following a rise in overall expenditure, although this will depend on whether there is discretion over how additional resources can be spent. However, insofar as the productivity of those activities that are valuable to the NHS also improves through innovation, the threshold will tend to fall. So the net impact of these two countervailing effects on the threshold cannot be determined *a priori*.

7.3 Differences in the estimated thresholds between 2007 and 2008 are assessed. Although overall expenditure increased by 6% between 2007 and 2008 which represented real growth of 2% in 2007 prices, the overall threshold for all 23 PBCs fell by 2% in nominal terms and by 5% in real terms.

7.4 The reasons are complex but reflect changes in productivity, which differ across PBCs, but also a general reallocation of a change in overall expenditure towards those PBCs that appear more valuable in 2008. Given the uncertainty in estimation, subtle differences between 2007 and 2008 should not be over interpreted. This analysis does suggest, however, that the overall threshold will not necessary increase with growth in the real or even nominal NHS budget. This suggests that the threshold is more likely to fall at a time when real budget growth is flat or falling and PCTs find themselves under increasing financial pressure.

8. WHAT TYPE OF HEALTH IS FORGONE BY APPROVAL OF A NEW TECHNOLOGY?

8.1 The methods of analysis can identify not only how many QALYs are likely to be forgone across the NHS as a consequence of approving a technology which imposes additional costs on the NHS, but also where those QALYs are likely to be forgone and how they are made up, ie, the additional deaths, life years lost and the QoL impacts on those with disease.

8.2 As an example, based on the 2008 central estimate of the cost per QALY threshold (£18,317), the approval of Ranibizumab for the treatment of diabetic macular oedema (prior to the patient access scheme agreement) would have imposed additional annual costs of up to £80m on the NHS each year and been likely to displace 4,367 QALYs elsewhere in the NHS. This forgone health is likely to be made up of 295 additional deaths and 1,337 life years forgone, most of which are likely to occur in Circulatory, Respiratory and Cancer PBCs. However, much of the total health effect of these additional costs (3,509 QALYs) is associated with QoL forgone during disease which is most likely to occur in Respiratory, Neurological and Mental Health PBCs.

9. CONCLUSIONS

9.1 The methods of analysis presented here go some way to providing an empirically-based and explicit quantification of the scale of opportunity costs the NHS faces when considering whether the health benefits associated with new technologies are expected to offset the health that is likely to be forgone elsewhere in the NHS.

9.2 The study also starts to make the other NHS patients, who ultimately bear the opportunity costs of such decisions, less abstract and more “known” in social decisions. Since who happens to be known or unknown is only a matter of perspective, time and ignorance, ethical and coherent social decisions require that both should be treated in the same way. These methods contribute to removing some of the “ignorance” and making the unknown more real.

9.3 This work has implications for the Government’s proposals to move to a system of value-based pricing for new prescription pharmaceuticals. These proposals include a widening of the measure of benefit to be used in evaluating new products including wider social benefits such as carer time and a consideration of the unmet need. Implementation of an augmented measure of benefit will also need to be reflected in terms of benefits forgone through service displacement. In principle, this will be feasible given the methods used in this study.

October 2012

Written evidence from Pfizer Ltd (NICE 62)

EXECUTIVE SUMMARY

This inquiry is very timely given regulations being drafted to establish NICE as a non-departmental public body and with negotiations with industry on a future value-based pricing scheme developing momentum, the coming months represent a unique opportunity to revisit how NICE operates.

NICE has made significant progress in the previous 13 years working towards ensuring high quality care is delivered across the NHS and has much to be proud of. It has developed robust processes for credible engagement with stakeholders, an appeals process and programmes to support better patient outcomes such as Quality Standards and input into the Outcome Frameworks development.

Whilst it’s assessment of technologies has made great strides in allowing comparative appraisals across different therapy areas, we believe this is an area that requires careful review to ensure continued access to future innovative medicines, particularly in areas such as rare diseases, cancer, personalised medicines and where there is high unmet medical need.

1. ACCESS TO TECHNOLOGIES

Medicines deliver good value for money to the NHS with UK prices being low compared to comparable European markets in terms of total spend and cost per prescription. NICE must work to narrow the gap between England and other European countries with respect to access to new and innovative medicines.

Appropriate HTA and appeals processes applied consistently will result in timely and appropriate access to new and innovative technologies. It is critical that the system used for assessing the value of new medicines to the NHS should fully capture health gains of all patients and wider benefits that achieve NHS objectives, and benefit society as a whole. Reliance on the QALY as the sole measure of benefit does not capture these gains adequately.

Relying on the QALY as the single measure of the value of a medicine is restrictive and doesn’t take into consideration numerous other important factors which more appropriately define the value a medicine brings to society and the NHS. Technology assessment needs to be fundamentally addressed to reflect a broader definition of value and improve HTA decision-making to ensure equitable access to new medicines for all patients.

We strongly believe that to continue its role as an integral part of the healthcare landscape, NICE must be as effective as possible in not just appraising the cost effectiveness of technologies, but also encouraging their uptake and access. It is crucial that NICE plays its role in supporting adoption of innovation in the NHS.

2. UPTAKE OF TECHNOLOGIES

NICE has a very broad remit, most notably technology assessment, but also a range of other work programmes such as Clinical Guidelines, Public Health Guidelines, Quality Standards and so on. There is currently a disproportionate focus from NICE on the cost effectiveness and health technology assessment of new medicines in the UK. Whilst this is an extremely important role for NICE, Pfizer would like to see a greater focus on other “clinical excellence” work programmes and improved implementation of NICE guidance.

A more systematic implementation of NICE guidelines, clinical and public health, will produce better and more consistent uptake of NICE approved medicines. In addition, NICE has put in place tools and resources to help further facilitate better uptake of NICE approved medicines such as the NICE Implementation Teams and a suite of materials on its web site. In spite of these, implementation of positive NICE guidance remains low and variable and Pfizer would want to see NICE take further steps to improve this.

Recommendations

- NICE should adopt a broader definition of value to improve access to medicines that builds upon the use of the QALY by:
 - Including dimensions of value beyond quality of life and survival benefit that achieve wider NHS objectives.
 - Recognising that QALYs are not necessarily valued equally by society and a greater weight may be desired in different therapy areas or particular patient circumstances, such as high severity and unmet need.
 - Allowing the inclusion of costs and savings accruing outside of the NHS and Social Services budgets when relevant.
 - Adequately reflecting the value versus the viability, risks and costs of research and development for medicines used to treat rare diseases, areas of unmet need, and specialised, advanced applications of science such as oncology, biologics and personalised medicines.
- NICE’s appeals process needs revising to allow an independent review for a challenge of scientific interpretation of evidence.
- NICE must work to improve uptake of, and reduce variation in uptake of, NICE approved technologies.
- There needs to be stronger incentives in place to encourage adherence to NICE work programmes which improve access and uptake of technologies.

1.0 ACCESS TO TECHNOLOGIES

1.1 *Limitations of the QALY*

NICE focuses almost exclusively on cost effectiveness for its technology appraisals rather than clinical effectiveness and the broader value to society. To that end the QALY (as measured in the £/QALY metric) has become the dominant assessment tool intended to capture the entire value of new medicines. The QALY, whilst useful for comparing health gains across different therapy areas, is a formulaic approach that has widely recognised limitations and does not include value beyond quality of life and survival benefit. It therefore fails to capture the full value medicines can bring to patients and society. The reliance on the QALY as the all-encompassing, single arbiter of value and rigid approach to a fixed value of a QALY, results in the current failure of the HTA system for some medicines. In particular, these HTA issues are most evident in medicines for cancer and rare diseases.

Despite efforts allowing adjustment to the QALY to reflect some additional value under certain conditions, many specialist treatments in cancer are still not recommended, which in England has led to the establishment of the Cancer Drugs Fund (to fund medicines not approved as cost effective by NICE). Also, a completely separate process of evaluation has been established, undertaken by the Advisory Group for National Specialised Services (AGNSS), for the assessment of medicines for very rare conditions. NICE will assume responsibility from AGNSS in April 2013 and have confirmed that the QALY alone is not suitable for assessment of rare disease medicines and will build on the ethical framework developed by AGNSS.

Although most apparent for rare diseases and oncology, the limitations of the QALY approach to HTA are widely applicable and systematically underestimate the value that medicines can bring to the NHS and society.

The following are some of the significant limitations to the QALY approach to HTA which should be addressed.

1.1.1 The QALY fails to include dimensions of value beyond quality of life and survival benefit that achieve wider NHS objectives

The objectives of the NHS are wider than health gain alone. Patient experience of care is an important objective as reflected in the NHS Patient Experience Framework. Integration of healthcare across the health and social care system can lead to benefits through the re-location and simplification of process of care. Medicines can contribute significantly to achieving these goals and enable care to be transitioned closer to the patient and encourage self care. Medicines can deliver value related to patient experience of care, such as convenience, independence, and dignity, which is not captured within the QALY. Furthermore, it has been widely recognised that certain aspects of quality of life such as vitality, stigma, pain and psychological distress are not well represented in current approaches.

Any measure of health care benefit should be supplemented by consideration of these additional factors.

1.1.2 QALYs are not necessarily valued equally by society

HTA as currently practiced in the UK places significant emphasis on cost-effectiveness, which in its purest form seeks to maximise the number of QALYs gained from a given budget. While seeking cost-effectiveness (or value for money) is clearly a legitimate policy goal, it is not the only goal of the NHS. We also contend that NICE are too crude and rigid in the way that they approach this element of the appraisal. Taken together, we believe that this has led to a lot of the public disquiet about NICE recommendations.

The calculation of the cost-effectiveness estimate currently assumes that society views all *absolute* QALY gains as being equivalent and are indifferent to the characteristics of the patients in question. We believe this is an over-simplification and that society also places value on other considerations, including improving the position of the least advantaged.

The introduction of the NICE End of Life Criteria was a response to Society placing a greater value on interventions that prolong life than is estimated by the current NICE approach. Society in effect values these QALY gains more highly than equivalent absolute QALY increases to patients who are not approaching their end of life.

There are other circumstances where we believe that society places a greater value on QALY gains than NICE estimate, including treatments: for patients who currently have a poor quality of life; for severe conditions; and where there are no existing treatment options. We would also contend that QALY gains from interventions in diseases areas that are a national priority should be given additional weight by NICE.

The Department of Health Value Based Pricing proposal in effect recognises the limitations of the current HTA model and proposes that medicines for severe condition and in areas of highest unmet need should be given additional weighting.

While the introduction of the end of life criteria was a welcome development, we believe this is only a partial solution to a wider problem. Furthermore, while the NICE deliberative process provides an opportunity for the broader considerations to be taken into account, we remain to be convinced that this is done adequately or systematically.

1.1.3 The failure to include costs and savings accruing outside of the NHS and Social Services budgets

It is important for a HTA system to consider benefits beyond health gain per se to include factors such as equity, helping parents and carers and getting people back to work. These factors are not routinely considered by HTA bodies and there are increasing calls for these to be considered in HTA. Inclusion of costs and savings beyond the current NHS and Social Services should be an option for consideration by HTA bodies.

1.1.4 Lack of consideration of industry supply-side factors

The HTA approach as currently practiced by NICE makes no allowance for the economics of drug development and in effect appears to assume it is possible to bring medicines for different illnesses to market below the existing cost-effectiveness threshold. In reality, this is not possible.

It has been widely reported that the costs of successfully developing a new medicine are substantial. The total cost for an individual medicine will vary depending on factors including the length of the development process and the number and size of clinical trials that need to be conducted in order to secure regulatory approval.

For a commercial organisation, the costs of drug development need to be recouped over the lifetime of the product as well as securing an acceptable rate of return on the capital employed. Within the revenue side of the equation, the two key variables are the price the medicine is sold at and the total number of product sales, which is heavily influenced by the number of patients who have the condition.

Importantly, the cost of drug development does not vary in direct proportion with the number of patients who have the condition. This is why commercial organisations are able to launch medicines for common conditions at relatively low prices while medicines for rare conditions are typically significantly more

expensive. The costs of drug development are in effect being recouped over a much larger number of sales for a common condition compared to a rare condition, which is reflected in the price of the medicine.

The HTA approach used by NICE includes having a common cost-effectiveness threshold for all medicine and makes no allowance for difference in drug development costs. In doing so we are creating a system where it is harder for medicines for rare conditions to gain positive approval than it is for medicines for common conditions, as witnessed by the high cost-effectiveness estimates and negative NICE recommendations. This is also a major contributing factor that led to the creation of the AGNSS framework, which is seeking to look at criteria beyond simple cost-effectiveness estimates.

In reality the problem is continuous as opposed to affecting one group, such as orphan or ultra medicines although it is most extreme in these groups of medicines.

1.2 NICE Appeals Process

1.2.1 The recent DH decision that NICE must appoint a NICE Appeals Committee Chairperson who is independent of NICE is a welcome and necessary step to retain the credibility of this process.

1.2.2 Pfizer does, however, still have concerns that the grounds for appeal remain unchanged. In particular, the grounds for appeal on perversity should be reviewed to enable an independent review in the event that it is reasonable to challenge the scientific basis and/or interpretation of significant data that would lead to a different decision.

1.2.3 In the event a medicine is appraised within a clinical guideline, Pfizer believe that manufacturers should have the opportunity to appeal a clinical guideline decision, especially given medicines can be appraised either for the first time or their original guidance reviewed within this process.

1.2.4 If an appeal is upheld, the appraisal should not be referred back to the appraisal committee that made the original decision to the process and procedure driven appeals process.

2.0 UPTAKE OF TECHNOLOGIES

2.1 Implementation of NICE Guidance

2.1.1 Access to medicines following a positive appraisal by NICE is only part of the process of getting cost effective medicines to patients. Mechanisms need to exist and be used to ensure appropriate uptake of a medicine once it has been positively appraised by NICE. There is still significant variation in the uptake of medicines across the UK as was highlighted in the recent Health and Social Care Information Centre national metric report, which showed that approximately half of the disease areas explored had uptake levels below what was expected.

2.1.2 NICE has worked to address this, for example adoption of the Innovation Scorecards to highlight availability of NICE-approved medicines. Alongside the announcement of this initiative was the setting up of a group to help local NHS organisations implement NICE guidelines and spread information on best practice to improve the speed of uptake of new medicines and treatments. We welcome these initiatives alongside the availability of tools on the NICE web site to help facilitate uptake.

2.1.3 The report, “Innovation Health and Wealth: Accelerating Adoption and Diffusion in the NHS,” highlighted a number of areas for improvements in the NHS, which included the adoption of innovation as a key principle. Following this report, the NHS is adopting innovation into its ways of working and thinking, and given NICE’s fundamental role with regard to improving access and uptake of innovative technologies, NICE should also adopt the principles and tone laid out here.

2.1.4 Greater emphasis should be placed on NICE’s work programmes that facilitate uptake of approved technologies. Clinical Guidelines, Public Health Guidelines, Development of Local Formularies and Pathways should be aligned to metrics and incentives in the NHS to accelerate uptake of approved medicines for the right patients to improve and reduce variation in uptake across the country.

2.2 Clinical Guidelines and Quality Standards

2.2.1 Clinical guidelines should provide clear direction for improving uptake of NICE approved medicines along an evidence based patient treatment pathway. Currently guideline implementation is not mandated; consequently some organisations continue to cherry pick guidelines and implementation based on affordability, perceived cost effectiveness and ease of implementation rather than what is best for the patient. There is still some way to go before the NHS consistently adopts and implements these programmes. We would like to see stronger incentives put in place to encourage adherence to these work programmes in order to improve their implementation and subsequent uptake of technologies.

2.2.2 Additionally there should be a more pragmatic stance to guideline production when a clinical guideline does not support a Quality Standard. If there is a clear advantage in developing a clinical guideline that does not align to a Quality Standard then we would like to see a mechanism which will allow this flexibility.

October 2012

Written evidence from the British Psychological Society (NICE 63)

ABOUT THE SOCIETY

The British Psychological Society, incorporated by Royal Charter, is the learned and professional body for psychologists in the United Kingdom. We are a registered charity with a total membership of just over 50,000.

Under its Royal Charter, the objective of the British Psychological Society is “to promote the advancement and diffusion of the knowledge of psychology pure and applied and especially to promote the efficiency and usefulness of members by setting up a high standard of professional education and knowledge”. We are committed to providing and disseminating evidence-based expertise and advice, engaging with policy and decision makers, and promoting the highest standards in learning and teaching, professional practice and research.

The British Psychological Society is an examining body granting certificates and diplomas in specialist areas of professional applied psychology.

NATIONAL INSTITUTE FOR CARE AND HEALTH EXCELLENCE—NEW ROLES

The British Psychological Society (the Society) thanks the Health Committee for the opportunity to respond to this call for written evidence. The Society would welcome the opportunity to address the select committee on some or all of these issues.

Q.1 NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

- The Society believes it is important that NICE continues to evaluate a whole range of interventions and not focus unduly on pharmacological interventions.
- Whether in guidelines or technology appraisals, the Society believes that NICE should continue to focus on psychological interventions both as stand-alone interventions but also as part of complex, multi-component interventions such as pain management programmes. This is particularly important in the area of chronic, long-term health conditions where psychological and psychosocial interventions are crucial in ensuring good outcomes which focus not just on symptoms but also on functioning and quality of life.

Q.2 The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

- We believe that, overall NICE Quality Standards have the potential to be a positive development in support of the implementation of NICE guidance but we have a number of concerns.
- The existing national library of NICE Quality Standards may limit NICE's ability to develop new guidance and hence new quality standards.
- We are concerned that this may limit access to important guidance on mental health and psychological interventions more specifically.
- In particular, we are concerned that a number of vulnerable groups, for example children and young people with mental health and neurodevelopmental disorders will be excluded as there few Quality Standards for them. The situation is similar for adults with learning disabilities.
- NICE Quality Standards will focus only on a few areas/interventions for each condition/disorder (and in particular in the associated Commissioning Outcomes Framework) specifically on what can be more easily measured. We are very concerned that this may both limit and distort commissioning priorities.
- The Society would propose that NICE should develop more explicit evidence-based commissioning guidance for Clinical Commissioning Groups (CCG's).

Q.3 The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

- We believe NICE guidelines have an extremely valuable role in the development of health care practice in general and the impact on psychological care particularly in mental health has been substantial and positive.

- The Society, in collaboration with other stakeholders, has drawn attention to the fact that the evidence-base is more extensive for some psychological therapies than others and that absence of evidence in no way equates to evidence of ineffectiveness. We have highlighted the need for additional research to address gaps in the evidence base through the use of well-conducted randomised clinical trials of both novel and under-researched psychological therapies to establish their efficacy.
- We have also highlighted the need to identifying innovative methods for analysing evidence from a range of studies including, for example, from high quality cohort studies, to consider in conjunction with the evidence obtained from efficacy studies such as Randomized Controlled Trials (RCTs).
- With an increasing emphasis on long-term conditions we believe there needs to be a greater emphasis on the role of evidence-based psychological interventions in physical health care.
- There is a concern that the focus on National Quality Standards could limit the development of new guidance and we are concerned that without adequate resources the guideline programme could see a substantial number of its guidelines becoming out of date and so undermining their clinical value.
- The Society believes that NICE guidelines should continue to be concerned with not just individual interventions but also with the evidence for the systems concerned with the effective delivery of existing and new interventions. As mentioned previously, we believe that service-level guidance should be integrated with guidance on clinical interventions.
- The Society suggests that more consideration should be given to the presentation of NICE guidance from a range of sources (clinical, social care and public health guidance, along with technology appraisals in a manner which is integrated and hence more accessible to clinicians, patients and commissioners.

Q.4 The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

- The Society believes that NICE public health guidance has already proved to be of real value and stands to have a significant impact on the general health of the population (eg the recent guidance on alcohol).
- However, if there is not greater clarity about the respective roles of NICE and Public Health England, and clear policy direction from the DH then there is a considerable danger that the wider consumer base for PH guidance (eg local authorities, educational bodies and the public health provider community) will be uncertain about the value to be placed on NICE public health guidance.
- With the development of social care guidance careful thought about the linking of this guidance with the work of PH England is also required.

Q.5 What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

- If properly resourced, we do not expect there to be a significant impact of NICE's new responsibilities on its work programme.
- In our view, social care guidance should be closely integrated into the other NICE guidance programmes as social care is an important part of the care of many people with long-term conditions (eg dementia).
- Whilst we support greater integration of clinical guidance, we expect the major challenges to be faced in developing social care guidance will be the relative paucity of good quality evidence on social care interventions. It seems likely that considerable work on behalf of NICE will be needed to ensure that there is effective implementation of the guidance in an area where evidence-based practice is less well-established than in areas of healthcare.

October 2012

Written evidence from The British Cardiac Patients Association (NICE 64)

The British Cardiac Patients Association, a national registered charity made up totally of volunteers, aims for encourage the provision of the best possible care for those with a cardiac condition.

This includes ensuring the best possible in ongoing new treatments and technologies.

Only occasionally, and solely due the restriction placed by being volunteers, has the Association engaged with NICE. This is one instance where hopefully a submission to the Select Committee may have some influence on the way in which NICE gives the best possible advice to ensure good practice in the light of developing technologies.

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

- There are a number of clinically and cost-effective treatments for cardiovascular diseases that have received positive technology appraisal guidance from NICE. We know however that uptake of NICE-approved treatments is not consistent across the board. The introduction of the innovation scorecard for local NHS organisations is an important step in driving compliance with NICE guidance by providing greater transparency.
- Patients should be fully involved in the process of determining the value of treatments and clinical interventions. It is imperative that under the planned new system of value-based pricing, the views and experiences of cardiac patients are proportionately incorporated within guidance decisions. There also needs to be greater flexibility in the new system to ensure a broad and holistic assessment on the value of a given treatment which includes the associated societal costs and benefits which can be overlooked.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

- The BCPA supports NICE's work in producing evidence-based clinical guidelines and quality standards to inform the provision of high quality, cost effective care. This role has greater relevance during a period when the NHS is undergoing significant organisational changes and faces budgetary constraints. These environmental factors have the potential to disrupt the delivery of high quality care and hinder the Government's commitment to achieving improved patient outcomes.
- The BCPA welcomes the decision by the Department of Health to refer a range of CVD-related topics such as secondary prevention of myocardial infarction and cardiac rehabilitation, to NICE for quality standard development. In the last decade there has been significant progress in reducing CVD mortality,
- By setting out clear markers for high quality care, these standards should help patients to understand the kind of care and support they should expect to receive and build of the recent progress made in reducing CVD mortality by helping to identify areas for local improvement. Continued delays in the development of quality standards, increases the likelihood that areas of CVD care will be neglected and overlooked for inclusion within future iterations of the Commissioning Outcomes Framework. Recent reports that the NHS Commissioning Board may be considering outsourcing responsibility for developing commissioning guidance to other professional or charitable organisations if delays in the publication of quality standards persist. The BCPA would support any alternative arrangements as long as the proposed process results in timely, high quality guidance and guarantees patient input.
- Although we acknowledge that quality standards are intended to set out aspirational but achievable levels of care, in order to effectively support local benchmarking of current service performance and identify priorities for improvement, all standards should include a sufficient number of outcomes measures (or proxies for outcomes) to insure that their impact can be gauged.
- Commissioners should be held accountable for their performance in achieving the markers set out in quality standards through the publication of data under each statement. We would support the alignment of specific questions on cardiac rehabilitation within the Myocardial Ischaemia National Audit Project (MINAP) with the content of the proposed quality standard focused on secondary prevention to assess how it is being implemented and its effectiveness in reducing variations in care.
- To help improve outcomes for cardiac patients and reduce variations in care, it is important to ensure that those measures contained within quality standards are translated into the various other levers and incentives within the NHS quality improvement framework such as CQUIN payments for service providers.

The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

- NICE's public health guidance should reflect the shared responsibilities across the NHS and public health for commissioning services. Education programmes and awareness campaigns for both primary and secondary prevention of heart attacks are likely to place additional strain on NHS capacity. A joined-up approach to these activities is important to ensure that patients can access all of the appropriate services.

Written evidence from Roche (NICE 65)

1. INTRODUCTION

1.1 Roche is a leading manufacturer of medicines for a range of conditions, including cancer, inflammatory arthritis, hepatitis, mental health and neurology. As such, we have considerable experience of engaging with NICE. Since 2011, we have engaged in the development of:

- 14 STAs.
- Six MTAs.
- Four clinical guidelines.
- Six quality standards.

1.2 We also have experience in making medicines available through the Cancer Drugs Fund, which will be relevant in considering how NICE develops.

1.3 We welcome the opportunity to contribute written evidence to this inquiry. We have focused our evidence on:

- Defining service quality and informing commissioning.
- Appraising new technologies.
- Methodological challenges in appraising new medicines.
- Ensuring the uptake of NICE guidance.
- The future for NICE.

2. DEFINING SERVICE QUALITY AND INFORMING COMMISSIONING

2.1 Although much of the focus on NICE's role relates to technology appraisals, developing guidance to support high quality commissioning is critical. In order to be effective, guidance needs to be:

- Developed in a timely manner.
- Based on recommendations which are accepted as good clinical practice.
- Measurable, enabling service improvement and accountability.
- Consistent with other forms of guidance and public policy priorities.
- Translatable into quality levers and incentives.
- Able to adapt to developments in the evidence base.

2.2 NICE's planned library of quality standards is therefore welcome. However, we have concerns relating to:

- The length of time taken to develop quality standards. To date, only 22 quality standards have been completed out of a planned 180. The initial deadline to develop the standards of 2015 has been postponed to 2019 although many stakeholders were already frustrated with the initial five year development window, feeling it was too lengthy in the context of NHS reform.
- The process for selecting and prioritising topics for quality standard development. For example, to date, hepatitis C has not been identified as a quality standard topic, despite being a major cause of liver mortality, identified as an improvement area in the NHS Outcomes Framework.¹⁶²
- The extent to which quality standards focus on process rather than outcomes and the ability of quality standards to be translated into levers and incentives, such as CQUINs or COF indicators.

2.3 Without high quality clinical and commissioning guidance, there is a danger that the system will be destabilised, with insufficient focus on clinical quality to balance efficiency imperatives. Gaps in the library of quality standards could lead to distortions in priorities, with insufficient attention paid to some conditions. The risk posed by this can be found in neurological conditions or rheumatoid arthritis.^{163,164}

2.4 Recent news¹⁶⁵ that the NHS Commissioning Board is to utilise guidance produced by other bodies, such as charities or royal colleges, is therefore interesting. It would be helpful for the Committee to consider:

- What performance management measures should the NHS Commissioning Board establish to ensure timely and high quality delivery of commissioning and clinical guidance.
- The validation arrangements that should be established to support the use of guidance by other providers.
- The accountability processes for determining quality standard topics.

¹⁶² Department of Health, *NHS Outcomes Framework 2012–2013*, December 2011

¹⁶³ National Audit Office, *Services for people with rheumatoid arthritis*, July 2009

¹⁶⁴ National Audit Office, *Services for People with Neurological Conditions*, 16 December 2011

¹⁶⁵ Health Service Journal, *Outside groups could vie with NICE over guidance*, October 2012

3. APPRAISING NEW TECHNOLOGIES

3.1 In evaluating NICE's role in appraising new medicines it is important to consider:

- The original purpose of technology appraisals in eliminating “the postcode lottery”.
- The impact on clinical practice compared to other countries.
- The extent processes and decisions have been consistent and fair, reaffirming public confidence in the NHS.
- The ability to secure good value for money for the taxpayer.

Tackling the postcode lottery

3.2 When NICE recommends a treatment, there is good evidence to support a convergence in clinical practice, although regional variations do remain for some time. However, when NICE does not recommend a treatment or has yet to appraise it, evidence suggests high levels of variation.^{166,167} Despite improvements in local decision-making processes, a significant postcode lottery remains for medicines which have yet to receive a positive NICE recommendation, necessitating initiatives such as the Cancer Drugs Fund. Addressing this situation will be a key challenge for value-based pricing.

International variations

3.3 Over the past three years, a good deal of evidence has been developed on variations in clinical practice, when compared to other countries. *Extent and causes of international variations in drug usage* found that:

- A negative technology appraisal can lead to very low levels of usage in the UK. For example, the UK was a very low user of clinically effective newer cancer medicines, many of which had been rejected by NICE on the grounds of cost-effectiveness.
- A positive technology appraisal does not necessarily lead to high levels of uptake, if other factors to support high quality services are not in place. For example, the UK was a low user of hepatitis C medicines, despite them being assessed as clinically and cost effective, due to problems with diagnosis, referral and treatment capacity. A recent report from the Health Protection Agency found that treatment rates for hepatitis C have been decreasing but that by increasing the proportion of patients treated by as little as 10%, among those people with moderate hepatitis C and to 20% among those with more advanced disease, the number of new cases of cirrhosis or liver cancer could be reduced by more than 2,000 over the next 10 years.¹⁶⁸

3.4 Therefore, the existence of the technology appraisal process has not, in itself, been able to ensure that clinical practice in the NHS keeps pace with that in other countries. Indeed, in the field of cancer, the NICE process has acted as a barrier, resulting in levels of uptake in the UK being significantly below that in comparable European countries. Although initiatives such as the Cancer Drugs Fund have enabled some catch-up, usage in England remains significantly lower than elsewhere¹⁶⁹ and lower still in Scotland and Wales.¹⁷⁰ It is likely that this historic deficit in England will take many years to address and it is important to note that the Cancer Drugs Fund, which is due to expire in 2014, will therefore not alone provide a long term mechanism for achieving this.

3.5 The Committee may wish to consider what longer term steps could be taken to ensure that clinical practice in the NHS is able to keep pace with that elsewhere and what the longer term arrangements should be for those treatments currently reimbursed by the Cancer Drugs Fund which will not be covered by value-based pricing.

Quality and consistency of decision-making

3.6 NICE's current structure relies on autonomous appraisal committees, which are supported by NICE's technical staff and independent evidence review groups (ERGs). The ERGs are academic centres commissioned by NICE to conduct a separate clinical and health economic assessment. In our experience there is some variation in how different appraisal committees and ERGs approach evidence and we cannot rule out the possibility that this variation may have contributed to material differences to final guidance. It may be useful for the Committee to consider what steps NICE intends to take to improve the quality and consistency of ERG processes.

3.7 In our experience appraisal committees often apparently lack sufficient technical expertise, particularly in statistics and health economics, to appropriately evaluate the evidence provided by manufacturers, ERGs

¹⁶⁶ Rarer Cancers Foundation, *Funding cancer drugs: An evaluation of the impact of policies to improve access to cancer treatments*, June 2011

¹⁶⁷ Rarer Cancers Foundation, *Exceptional Progress?—Assessing the progress made in improving access to treatment for people with rarer cancers*, March 2010

¹⁶⁸ Health Protection Agency, *Hepatitis C in the UK: 2012 Report*, July 2012

¹⁶⁹ P Stephens, D Thomson, *The Cancer Drug Fund 1 year on—success or failure?* www.thelancet.com/oncology Published online June 29, 2012 [http://dx.doi.org/10.1016/S1470-2045\(12\)70273-5](http://dx.doi.org/10.1016/S1470-2045(12)70273-5)

¹⁷⁰ Rarer Cancers Foundation, *Nations divided? An assessment of variations in access to cancer treatments for patients in England, Scotland and Wales*, August 2012

and others. We believe that having only one independent health economist on a typical appraisal committee, and potentially no individuals with the relevant biostatistics experience, prevents appropriate scrutiny and can result in suboptimal decisions. It would be helpful to consider whether the make-up of appraisal committees should be reassessed.

3.8 An area of particular inconsistency has been in the way in which different ERGs and appraisal committees consider eligibility for NICE's end of life criteria. NICE's appeal board has also observed this variability. However, it is our belief that the variability applies more broadly.

3.9 The appraisal consultation process often involves the exchange of large volumes of new analysis and evidence. This exchange often takes place under significant time pressure and we have experienced situations where the appraisal committee's demands for analysis could not be met by the ERG in time for the next appraisal committee meeting. The Committee may wish to consider how NICE could improve its opportunities for redress, with the objective of minimizing the need for time consuming and expensive appeals on the grounds that important information has been overlooked.

3.10 These inconsistencies can have a material impact on the outcomes of appraisal processes, but also on public confidence in NICE and the wider NHS. Given this, taking steps to ensure greater consistency should be a key focus for NICE as it considers its future development.

4. SECURING VALUE FOR MONEY

4.1 We recognise that it is imperative that the NHS secures value for money from new medicines. It is therefore welcome that NICE is now able to consider Patient Access Schemes (whereby a manufacturer introduces measures to improve the value delivered by a medicine) under full commercial confidentiality. Nonetheless, greater flexibility is required.

4.2 In our experience, access to medicines has been improved by the introduction of schemes to allow greater flexibility around HTA recommendations and reimbursement. In particular, NICE's willingness to consider Patient Access Schemes (PAS) and recommendations in sub-groups has improved access. However, the Cancer Drugs Fund was still required to enable access to clinically-effective treatments, demonstrating that PASs and the NICE process was, in isolation, insufficient as a mechanism to ensure access and value.

4.3 In this respect, we believe that there is an opportunity to do more through value-based pricing, providing this enables greater flexibility. One such opportunity is through the use of improved NHS data collection systems such as the Systemic Anti Cancer Therapy dataset (SACT) to validate the outcomes delivered by medicines in routine NHS practice and adjust prices accordingly, as well as to provide a mechanism to effectively and efficiently administer rebate schemes.

5. METHODOLOGICAL CHALLENGES IN APPRAISING NEW MEDICINES

5.1 There are methodological challenges in appraising the value delivered by new medicines, particularly cancer medicines where the evidence base is often not well-suited to making lifetime estimations of QALY gains. In such complex situations, the NICE Guide to Methods offers some advice about what analyses might be carried out. But in our experience there is rarely consensus or clarity between ERGs, manufacturers and the appraisal committees about which analyses should be carried out to maximise the Institute's confidence in situations when the evidence base is complex or challenging.

5.2 Given that certain issues come up repeatedly, for example how to extrapolate survival in cancer trials, which routinely allow patients in the "placebo" arm to "cross over" and receive the new medicine, we feel it would be helpful if the Institute published further checklists for appraisal committees to help ensure that all plausible approaches have been applied and evaluated before a decision is made.

5.3 The appraisal process is well equipped to evaluate incremental innovation in that many therapy areas have seen frequent but small advances in treatment, together with comparable increases in price. However in areas where there has been little or no development in treatment for many years, it can be difficult to prove cost-effectiveness. Comparator treatments are old, relatively ineffective and cheap. They are invariably out of patent and are sometimes used outside their licensed indication. Given their low cost, it is impossible to demonstrate sufficient QALYs to meet cost-effectiveness thresholds

5.4 NICE's "end of life" criteria stipulate an upper limit on the eligible patient population size. This effectively limits the number of medicines which could benefit from the end of life provision. We have evidence that the eligible patient population size may have been overestimated for many cancer drugs which were otherwise eligible for the end of life provision. Data from the Cancer Drugs Fund suggest that no treatment has yet incurred levels of usage which are above the small patient population criterion.¹⁷¹

6. ENSURING THE UPTAKE OF NICE GUIDANCE

6.1 There is no point in NICE incurring significant effort and cost in developing the different forms of guidance for which it has responsibility if this guidance is then not implemented. Equally, in the context of

¹⁷¹ Rarer Cancers Foundation, *There when you need it the most? The Cancer Drugs Fund: 2011–12 annual report*, August 2012

technology appraisals, there is no incentive for manufacturers to meet NHS value needs if it is unlikely to lead to rapid and high levels of uptake. Therefore it is important that NICE and others devote commensurate resources to promoting uptake and reducing variation in clinical and commissioning practice.

6.2 We note that a number of NHS initiatives are underway in this area, such as innovation scorecards, which offer the potential to support uptake, alongside the mandatory funding direction which accompanies a technology appraisal.¹⁷² However, we consider the primary challenge in promoting high levels of uptake to have been the absence of accurate, detailed and publically-available data on how medicines are being used in the NHS.

6.3 In this sense, the development of initiatives such as SACT is extremely important, if overdue. We hope that this will enable pseudonymised patient-level tracking of treatment and the evaluation of the outcomes delivered. This will enable:

- Analysis of uptake of guidance and variations in usage.
- Assessment of the outcomes delivered.
- Adjustments in pricing and reimbursement.
- Clinical accountability.
- Patient choice.

7. THE FUTURE

7.1 NICE's role has expanded significantly in recent year. Unfortunately its capacity to deliver timely, high quality products has not always kept pace, creating short term pressures and delays. In considering NICE's future role it is important that these mistakes are not repeated.

7.2 This is especially the case given the development of value-based pricing, which it seems likely that NICE will play a role in. We note that detailed discussions about value-based pricing have only just begun. However, we would make the following observations:

- In developing value-based pricing, we need to look again at every aspect of the technology appraisal process.
- However, the current proposals build on rather than address the structural flaws in the current system.
- We need a better system, which more sensitively reflects unmet need and therapeutic benefit.
- It will not be possible to find perfect solutions for every challenge—therefore a guiding principle should be flexibility is encouraged, enabling all sides to work creatively to enable access.
- Recognising that cancer has been particularly challenging for NICE, it should be a key early test of whether value-based pricing is work.

7.3 Given the scale and complexity of the issue, the Committee may wish to give further consideration to the issue of value-based pricing.

October 2012

Written evidence from Ovarian Cancer Action (NICE 66)

1. Introduction

Ovarian Cancer Action (OCA) welcomes the opportunity to put forward a written submission as part of the Health Select Committee's inquiry into NICE. OCA is a national charity whose aim is to stop women dying from ovarian cancer. OCA works towards achieving this aim by focussing on two main areas, funding research into understanding this complex disease so that better treatments can be developed; and raising awareness of the symptoms of ovarian cancer as part of our drive to ensure women are diagnosed at an earlier stage when the disease is easier to treat.

Our efforts at improving survival rates for women has meant that we have had to engage with GPs and healthcare professionals to ensure that patients get a high standard of care, are diagnosed without delay and are treated appropriately. As part of our work in improving the care of patients we have had experience with NICE which has developed an ovarian cancer quality standard, published clinical guidance on the primary care of ovarian cancer and a key messages document for healthcare professionals. Because of the experience that we have had with NICE we would like to make some short comments on NICE's current function.

In summary Ovarian Cancer Action holds the following views which will be expanded in sections 2, 3 and 4:

TECHNOLOGY APPRAISAL PROCESS

- We believe that patients should play a greater role in NICE's technology appraisal process to ensure that their experiences are adequately captured.

¹⁷² Department of Health, *Innovation, health and wealth: Accelerating adoption and diffusion in the NHS*, December 2011

- We would advocate that where there is strong evidence to support the efficacy of an individual drug at a different dose to the license, NICE should be able to consider that evidence, particularly if this is likely to make the treatment more cost effective to the NHS and therefore enable routine access for every patient who could benefit.

NICE QUALITY STANDARD

- NICE should look to develop additional standards and metrics relevant to other aspects of the patient pathway—including supportive care, adjuvant treatment post-surgery and end of life care.
- We would support an initial audit and then continuous assessment of the ovarian cancer quality standard to understand how the ovarian cancer quality standard is being implemented and how effective it has been in reducing variations in care.
- We would welcome some clarity on what measures have been put in place if a quality standard is not being implemented so there is an understanding of how it can be enforced.

NICE CLINICAL GUIDELINES

- Additional information and further outreach is needed to ensure that primary care professionals are aware of published NICE guidelines.
- From an ovarian cancer perspective additional information and further outreach is needed to understand the challenges primary care professional face in implementing the NICE Ovarian Cancer Primary Care Guideline and support should be given to overcome these challenges.
- The key messages of any national or local public awareness campaigns should be aligned with the key components of the NICE clinical guidance to ensure consistency of public health messages.

2. NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

Ovarian Cancer Action supports the aim of reducing variations in access to treatments through the NICE technology appraisal process. Decisions regarding access to treatments or defining high quality care should be informed by the input from patients and carers. At present, there are lay members on the appraisal committees and evidence is invited from patient representatives, however, frequently the aspects of treatment and care that patients value the most, such as having a choice of treatments, are often neglected, or overlooked compared to those of clinical or economic experts. NICE has taken positive steps to address this and encourage greater patient and public involvement, however, as the new value-based pricing regime is developed, we would advocate a greater role for patients to ensure their experience and insights are adequately captured and fully informed guidance decisions are made.

Although NICE has been commissioned to produce a series of evidence summaries for off-label and unlicensed medicines, NICE is not able to conduct HTA assessments on medicines outside of their licensed indication. This can serve to limit access to clinically effective treatments where there is clinical demand to use a treatment at a different dosage to its licensed indication. We would advocate that where there is strong evidence to support the efficacy of an individual drug at a different dose to the license, NICE should be able to consider that evidence, particularly if this is likely to make the treatment more cost effective to the NHS and therefore enable routine access for every patient who could benefit.

3. The role of NICE quality standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

We welcome the recent introduction of the quality standard for ovarian cancer and the important role they play in defining high quality, cost-effective care for people with ovarian cancer. At present, evaluation of the impact of the quality standard on patient outcomes is difficult because metrics are focussed on assessing processes rather than outcome measures.¹⁷³ As such NICE should look to develop additional standards and metrics relevant to other aspects of the patient pathway—including supportive care, adjuvant treatment post surgery and end of life care. Nonetheless, in order to benchmark the performance of NHS services and monitor the impact of the quality standard, we would support an initial audit being undertaken to assess how the quality standard is being implemented and how effective it has been in reducing variations in care, following this we would support continuous assessment of the quality standard to ensure it is being implemented. We would also welcome some clarity on what measures have been put in place if the standard is not being implemented so there is an understanding of how it can be enforced.

¹⁷³ MHP Health Mandate, An assessment of the prioritisation in quality and incentive frameworks in the new world: internal presentation, August 2012

4. The continuing role of NICE clinical guidelines (on treatment for specific conditions) in improving the quality of healthcare, in particular in the context of analysis of effectiveness of established treatment procedures and review of variations of outcome

Clinical guidelines remain important resources for improving the standards and consistency of services, however we feel that many GPs are still unaware of the current guidelines for ovarian cancer guidelines. Additional information and further outreach is needed to help inform primary care professionals to help support prompt referrals and early diagnosis of ovarian cancer patients.

The key messages of any national or local public awareness campaigns such as the Be Clear on Cancer initiative should be aligned with the key components of the NICE clinical guidance for ovarian cancer regarding disease awareness and early identification of symptoms. As well as providing clear and consistent information, if local authorities are to run targeted awareness campaigns, adequate resources should be available to ensure that patients can get access to obtain appropriate advice and support from NHS services.

October 2012

Written evidence from the NHS Confederation (NICE 67)

1. The NHS Confederation represents all types of organisations that commission and provide NHS services. It is the only membership body to bring together and speak on behalf of the whole of the NHS. We are pleased to have the opportunity to submit evidence to this inquiry.

2. The NHS Confederation strongly supports the role of the National Institute for Clinical Excellence (NICE). It performs an extremely important function in independently developing quality standards and reviewing the value of healthcare interventions, helping the NHS to assess cost and clinical effectiveness. Its international reputation is well deserved.

3. This brief submission sets out our view on how NICE's role could develop further to support the NHS as it faces its greatest ever financial challenge. However, it is not intended to be a comprehensive commentary on NICE's work.

SUPPORTING COMMISSIONERS' DECISION MAKING

4. NHS commissioners have finite resources to invest in the full range of treatments and services required by the populations and patients they serve. In this context, the impact of the growth in the number of treatments that commissioners are legally required to fund (ie over which they have no ability to apply local discretion regarding their relative priority) needs to be recognised. Such a requirement applies to all medicines and treatments recommended by NICE's technology appraisals. This is valuable to the system in minimising uncertainty about patients' rights to access certain treatments that have been independently assessed as being cost-effective. However, it must be acknowledged that if the cost of paying for all the treatments recommended by NICE's appraisals increases, the funding available for other services and treatments inevitably decreases. This could mean less funding being made available for services and treatments that are equally, or even more clinically or cost effective than those recommended by NICE, but which have not been assessed. (Treatments may not be assessed either because they are relatively new or under-researched and it has not yet been possible to build the comprehensive evidence base required, or because they are older treatments, whose value is clearly established, which do not need to be assessed by NICE.)

5. The Government should take into account the overall cost of treatments that commissioners are legally obliged to fund when setting the NHS' budget, and when holding local commissioners to account for their performance in improving overall health outcomes. We also believe there is a case for NICE to do more to support and enable commissioners to manage finite resources. In particular, NICE's knowledge and expertise could play an important role in supporting national and regional decision-making around identifying and disinvesting in services and treatments that are less cost effective, no longer clinically appropriate, or not financially sustainable.

VALUE-BASED PRICING

6. The NHS Confederation supports the principle of introducing a pricing system for drugs which takes into account their value and operates in the context of the overall NHS budget. However we expressed concerns about the value based pricing proposals on which the Government consulted in 2011. In particular, we were concerned that the proposals may drive up the cost of drugs to the NHS without increasing access to treatments, resulting in less investment in treatments that have been objectively judged to be more cost effective, simply because they do not meet certain narrowly defined criteria around "innovation". We also question whether it is feasible to introduce this new system by 2014 given the Government has acknowledged that extensive testing of the system will be necessary.

7. We welcome the fact that, following consultation, the Government has acknowledged that NICE should be given a central role in implementing the new "value-based" pricing system. We still believe that NICE

should also be tasked with designing an approach to value based pricing, given its internationally renowned expertise in this area.

NICE'S POTENTIAL ROLE IN RECONFIGURATIONS

8. The NHS Confederation has been arguing for some time that significant service redesign is necessary in many parts of the NHS on both quality improvement and financial sustainability grounds. It is essential that this change is genuinely evidence based and that decisions are taken efficiently and transparently.

9. The NHS currently wastes time and effort by expecting local organisations to collate and present the full evidence base for service change locally. While interpretation will always have to be done in the local context, much more can be done to give authoritative advice on evidence at the national level. We believe Ministers should task the NHS Commissioning Board with taking the lead in working with NICE to deliver clear, authoritative advice on the evidence to back clinical service re-design. Both organisations should also work with the medical Royal Colleges and other academic institutions in developing this advice.

October 2012

Written evidence from the UK Faculty of Public Health (NICE 68)

ABOUT THE UK FACULTY OF PUBLIC HEALTH

The Faculty of Public Health (FPH) is the standard setting body for specialists in public health in the UK. FPH is the professional home for more than 3,000 professionals working in public health. Our members come from a range of professional backgrounds (including clinical, academic and policy) and are employed in a variety of settings, usually working at a strategic or specialist level.

FPH is a joint faculty of the three Royal Colleges of Public Health Physicians of the United Kingdom (London, Edinburgh and Glasgow). In addition, FPH advocates on key public health issues and provides practical information and guidance for public health professionals, aiming to advance the health of the population through three key areas of work: health promotion, health protection and healthcare improvement.

This response has been prepared by FPH's Health Services Committee which provides advice and expertise on the third domain of public health related to health service commissioning and provision—including healthcare needs assessment, priority setting, clinical and cost-effectiveness, clinical outcomes, audit and evaluation.

EXECUTIVE SUMMARY

1. NICE's role in advising on value to the NHS has been fundamental. However, the competing pressures that NICE has to balance in its decisions have not always allowed NHS commissioners to prioritise for funding a range of good value interventions that have not been assessed by NICE or not had a high public profile.

2. It is difficult to imagine what value based pricing will look like and that it will achieve anything better than NICE making solid and good value judgments based on realistic measures.

3. In these times of particular financial restraint for the NHS, NICE's cost effectiveness level (its use of the cost per QALY) may need to go down for the benefit of patients across the whole NHS. There has been regular pressure for this measure to creep upwards to the detriment of interventions with good value but a low public profile.

4. NICE's quality standards may turn out to have a limited role in the long term as there has not been a good understanding of how standards work in practice.

5. Clinical guidelines are useful but need to be put in the context of NHS commissioners using a full range of patient outcomes to understand and improve a clinical service.

6. If done well then NHS commissioning should be about structured health improvements, which is a perspective that public health specialists would look to develop.

CLINICAL EFFECTIVENESS AND VALUE

7. NICE has an important role in evaluating the effectiveness and cost effectiveness of drugs and other clinical interventions. It is usually seen as being relatively objective and puts considerable resource into trying to make fair and reasonable decisions. However, there are some important caveats.

8. NICE's role in evaluating effectiveness and cost-effectiveness will be affected by the intended introduction of value-based pricing for NHS drugs and looks potentially confusing in an area of critical importance to the affordability of the provision of health services. In some senses NHS commissioning's key role is about the management of value. NICE should have a clear role in helping to control the use of new interventions that are of marginal or poor value.

9. However, NICE's decisions may still skew priorities at a local level. The prioritisation of funding is a key task for commissioners and it is important that NICE helps to get the right balance. NICE does not make decisions in the context of the real NHS budget in the sense that it is not directly accountable for the budget. If NICE makes a positive technology appraisal at the margins of cost effectiveness for the NHS then this may oblige those directly managing the NHS budget, such as commissioners in PCTs, or now CCGs, to make decisions not to fund other health care interventions or developments. This will be the case if funding is tight, even though those organisations judge the developments to be of higher value than those assessed by NICE.

10. It is not clear how value based pricing will work as there is insufficient detail available in the public domain to assess whether any scheme will offer a better process than the current one of NICE evaluations and judgments. It is difficult to imagine a better process than good assessments and hard-nosed judgments that feed back key messages to the commercial sector on what is of real value to the NHS.

11. It should be recognised that there are political influences which NICE has to deal with. This is an area where NICE has to maintain its credibility amongst commercial and media interests and those making national political decisions. This sometimes produces guidance from NICE that is seen as a compromise and it would be useful to separate the role of obtaining best value for the NHS from that of also having to support commercial interests to the possible advantage of the UK economy.

NICE GUIDELINES AND QUALITY STANDARDS

12. The quality standards published by NICE represent an interesting development, but are potentially misleading. The majority of quality standards to date are not readily measurable. This creates an illusion of certainty and pushes responsibility downwards on to NHS organisations with little resource or guidance about how to bridge the gap between the demands at the top and the complexity of delivery. To be effective no quality standards should be released without a measurement being piloted and the resources associated with it quantified.

13. Whilst the intentions of the quality standards developed by NICE are good, they are unlikely to make substantial changes to clinical practice in the short term. The quality standards may well come to be seen as relatively narrow managerial targets that in themselves end up as having unintended consequences. There are alternatives to these sorts of standards that may appear to need more resource but will deliver much more in the long run.

14. It would be more productive for commissioners to assess fully a much wider range of outcomes than are captured in the quality standards. In practice these sorts of clinical outcomes are often complex and interdependent and need both clinical engagement and commissioner insight.

15. Clinical outcomes are available in the established national clinical databases although it may need a national database to be put in place where this sort of data is not collected. NHS commissioners have been poor in using this sort of data, but this may reflect that commissioning has only recently established itself with a recognised structure and process. Whilst databases have a running cost they are likely to more than repay this in terms of improving the quality of patient care, outcomes and the associated efficiencies.

16. These databases, where they exist (and there are a large number already established) are underused by commissioners but do represent an opportunity to build in clinical improvements to the NHS commissioning process. This could happen on an annual basis by incorporating recognised improvements to the service specifications and contracts that are used with clinical providers. However, many more clinical services may need model service specifications to be developed on a national basis for use by the Clinical Commissioning Groups (CCGs) as individually these Groups are unlikely to have the resources to develop these.

17. The NHS Commissioning Board represents an opportunity to facilitate these sorts of developments both for specialised services commissioning and by supporting pan-Clinical Commissioning Group work.

CLINICAL GUIDELINES

18. Evidence based guidelines are helpful and should be used to guide good clinical practice. It may be useful to separate out how clinical guidelines can be used by commissioners and providers.

19. It is not always appropriate for commissioners to specify in detail to providers exactly what clinical standards should be followed in delivering the key patient outcomes unless it happens to be a specific standard with serious consequences if not met. Broadly, commissioners will be more effective by using patient outcomes to specify a service. These outcomes can be supported by reference to a body of service guidelines and it is then the role of the provider to judge how to deliver the service to the accepted standards within the resources available. This is not to undermine the place of guidelines and quality improvement or the appropriate resources to support this. Rather it is to ensure that value in service delivery is optimised. Contracting arrangements should not be reduced to details such as specifying the number of clinical staff to deliver a particular service when this is not necessary.

20. The use of clinical guidelines can be a particular issue for commissioners when clinical guidelines have not been assessed for value for money. Whilst apparently worthy, some guidelines may not represent good value for the wider NHS with its fixed budget.

21. There may need to be further attention to the extent to which guidance genuinely reflects common clinical problems rather than focusing on areas because that is where evidence exists. Guidelines should start with a description of the common clinical presentations or scenarios they are addressing, rather than just the extent of the health problem. An example of this is the guidelines on depression which are extensive but do not readily map on to the management problems faced by general practitioners day to day.

The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

22. NICE public health guidance has proven to be authoritative and thorough—where good randomised trial evidence is not available particularly where there are complex multifaceted responses needed on legislation, behavioural economic and social measures, NICE has sought to offer the best available evidence for action. The guidance has therefore formed more the basis for professional standards for local implementation rather than straight forward guidance for the implementation of single therapeutic regimens. This work is of vital importance and should continue. It is not confined to health improvement measures but does cover treatment—for example in the area of targeted cardiovascular intervention and it also health protection. Local authorities need to be able to call on more of such authoritative guidance in the future if local policies are not to be infused with personal prejudices and political dogma. Evidence based healthy public policy is essential if the public health role in councils is to succeed. The guidance also needs increasingly to take account of the resource implications of many of its recommendations. Opportunity costs must never be forgotten or ignored.

What effect NICE's new responsibilities in relation to evaluating social care interventions might have on its work overall and how this will relate to the integration of health and social care services

23. Some of the descriptive guidance from NICE has been some of the least useful- tending to revert to general principles rather than provide any real stimulus for action. It is far from clear at the moment that NICE will have a significant impact here.

The joining of NICE with the Social Care Improvement and Excellence SCIE is a welcome move to increase the evidence base for the practice of social work and social care intervention. This was a strong recommendation from the first Munro report. SCIE have made notable efforts to rise to this agenda and should be fully supported in the new arrangements to increase the evidence base for social care and to improve the effectiveness of preventive interventions which benefit social care, safeguarding of adults and children and the public health overall.

October 2012

Written evidence from The Royal College of Radiologists (NICE 69)

SUMMARY

This evidence refers to two issues in respect of the functions of NICE, as follows:

- the role of NICE in evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions through its medical technology appraisal strategy; and
- the role of NICE Quality Standards and the relationship with the work of other bodies.

The Royal College of Radiologists (RCR) commends NICE, under the chairmanship of Professor Sir Michael Rawlins, for the breadth and scope of its work both nationally and internationally and is appreciative of the annual review meetings with the Chair which have proved invaluable in building and improving relationships between NICE and the RCR's two specialties of Clinical Oncology and Clinical Radiology.

THE ROLE OF NICE IN RELATION TO VALUE-BASED PRICING OF DRUGS PURCHASED BY THE NHS

1. The RCR is keen to endorse the role of NICE in evaluating the efficacy and cost-effectiveness of drugs and other clinical interventions through its medical technology appraisal strategy. Whilst being encouraged that NICE will continue to have a role in evaluating the cost effectiveness of drugs under the value-based approach, we are uncertain as to how the new system will operate in detail. Whilst it is assumed that value-based pricing will empower clinicians to treat patients according to clinical need, the complexity of the process to enable this within the new health architecture leads to doubt as to whether the stated aims of the policy can be achieved for NHS patients in a global marketplace.

THE ROLE OF NICE QUALITY STANDARDS AND THE RELATIONSHIP WITH THE WORK OF OTHER BODIES

2. The RCR commends the work of NICE in publishing clinical guidelines and quality standards. In respect of the RCR's two specialties, the Quality Standards for the treatment of various cancers are much respected for the improvement in treatment methodologies with the concomitant improving outcomes for patients. The technology appraisals, diagnostics guidance and endorsement of interventional radiology procedures have also had a welcome positive impact on patient care.

3. However, NICE cannot be expected to cover the full range of medical practice and treatments. The RCR therefore hopes that NICE will continue to look to the guidance, standards and similar information developed by other bodies in order to avoid a duplication of effort.

October 2012

Written evidence from Cambridge Weight Plan (NICE 71)

SUMMARY

Cambridge Weight Plan (CWP) offers a number of flexible weight management programmes, which provide the overweight and obese with an effective and sustainable way to achieve a healthy weight. CWP offers flexible programme options 440 and 1500 kcal/day, all using a nutritionally balanced formula food as their foundation. Our products contain carefully formulated amounts of energy, protein, carbohydrate, fat, fibre and all essential micro-nutrients. CWP products and programmes are only available through specially trained and accredited Cambridge Consultants, who provide initial screening and advice to clients, and individual support throughout their programme.

CWP recognises and fully appreciates the excellent job that NICE generally does, which has earned it a worldwide reputation for the quality of its work. Its guidance is mainly thorough, comprehensive and evidence based and its engagement with stakeholders is an example for all bodies associated with the health system.

We do, however, believe that given the new responsibilities that it is to assume, NICE will have to work hard to ensure that it performs its duties in an inclusive, thorough and effective manner, while at the same time ensuring that it does not take unreasonable amounts of time to produce its guidance documents.

We would particularly like to make the following points, which have a focus on NICE's role in the new public health system.

- In the current economic climate, NICE's role in evaluating the cost-effectiveness of clinical interventions has become ever more important.
- NICE needs to be open to updating its guidance at more regular intervals, as only this will ensure that the organisation keeps up with new medical technology and up-to-date clinical research.
- NICE's current approach to updating guidance can create the risk of endorsing recommendations on treatments which either are obsolete or do not reflect the latest results of clinical research.
- NICE should make sure that it continues to formulate accurate guidance, underpinned by clear evidence and thorough research, but it should make sure that this approach does not cause unnecessary delays and long waits.
- In the new Public Health system, NICE's role will become more important than ever as inexperienced commissioners in local authorities seek expert guidance.

INTRODUCTION

1. CWP offers a variety of weight management options, including Low Calorie Diet (LCD) and Very Low Calorie Diet (VLCD) programmes, for those who are overweight and clinically obese. They are primarily aimed at those with severe weight problems, typically with a Body Mass Index (BMI) greater than 30.

2. CWP would like to thank the Health Select Committee for this opportunity to comment on the work of NICE.

NICE's role in relation to evaluating the effectiveness and cost-effectiveness of drugs and other clinical interventions (through medical technology appraisals) including how its role will be affected by the intended introduction of value-based pricing for drugs purchased by the NHS

3. CWP supports the activity of NICE and believes that its work is crucial to ensure that those working in the NHS, local authorities and the wider community deliver high-quality healthcare. In the current economic context characterised by financial constraint, NICE's role in evaluating the cost-effectiveness of clinical interventions has become ever more important and we hope that NICE will continue to produce high quality evidence-based guidelines highlighting the most effective and cost effective interventions.

The role of NICE Quality Standards in the new NHS system architecture, in particular the status of NICE guidelines in determination of commissioning priorities

4. CWP does agree that evidence-based guidelines on the most effective ways to diagnose, treat and prevent disease and ill health are very important in helping healthcare professionals in their work. However, we believe that NICE needs to be open to updating its guidance at more regular intervals, as only this will ensure that the organisation keeps up with new medical technology and up-to-date clinical research.

5. We believe that NICE's current approach to updating guidance can create the risk of endorsing recommendations on treatments which either are obsolete or do not reflect the latest results of clinical research. We can provide the example of NICE's initial refusal to update Clinical Guideline 43 on Obesity, which was originally published in 2006 and which the Institute was not going to update, despite a considerable amount of new data on effective interventions on obesity had been brought to its attention. NICE was eventually forced to take into account the new data and a decision to update the Clinical Guideline was taken in December 2011.

6. CWP welcomes relatively recent moves by NICE towards openness and engagement with all stakeholders, including private providers and industry, through the provision of both stakeholder meetings and individual meetings. It appears clear that NICE is making an effort to change the previous situation of limited transparency and lack of engagement, and we hope that this trend will continue in the future.

7. We would like to note that CWP is greatly supportive of NICE's approach to formulating accurate guidance. We believe that these procedures should always be underpinned by clear evidence and thorough research, but the Institute should make sure that this approach does not cause unnecessary delays and long waits.

8. As an example of this, we would like to mention the draft guidance on "Managing overweight and obesity in adults—lifestyle weight management services," which is being developed by NICE.¹⁷⁴ This guidance will not be available in its final form until 2014, something which, quite clearly, will create serious issues for commissioners after the transfer of competencies to local authorities in April 2013.

The effect of the new public health system architecture on NICE's continued role in respect of public health guidance

9. With the implementation of the welfare reforms and the new architecture of the public health system, the role of NICE and its expertise will become more important than ever as inexperienced commissioners in local authorities seek expert guidance. This will increase the need of the Institute to produce wide-ranging yet straightforward guidance.

CONCLUSION

10. We hope that our submission has been useful in identifying some of the issues with the way in which NICE carries out its work.

October 2012

Written evidence from LighterLife (NICE 72)

SUMMARY

LighterLife is a UK company offering weight loss and weight-management programmes for people who are clinically obese or overweight. Our programmes are based on the recognition that lasting weight-management success can only come by addressing the underlying reasons behind weight gain. Without this understanding, weight re-gain is highly likely.

The LighterLife programme was researched and developed over many years before finally being launched in 1996 and was developed because of a profound understanding and decades of experience of how it feels to be obese. LighterLife differs from other weight loss plans because it fundamentally understands that in order to change the body you needed to start with the head—the approach based on thinking in line with addictive behaviour models and a profound understanding of food addiction. The company challenged the status quo, by developing a real solution to a serious and growing health problem. As a result, LighterLife is a unique combination of three powerful dimensions, a change programme for self development, small group support facilitated by a qualified LighterLife Weight Management Counsellors and fully nutritious food and lifestyle options. LighterLife also provide the only national weight-management programme developed specifically for men.

LighterLife currently offers obese clients a personalised weight loss programme called Total which is delivered using a Very Low Calorie Diet (VLCD) and a programme called Lite for overweight clients using a Low Calorie Diet (LCD), both of which are followed on by the Management Programme. LighterLife understands our client's weight management journey with all its ups and downs and knows it takes time, practice and support and we therefore provide a programme to focus on long term weight management, healthy eating and active living to help clients sustain and manage their own weight loss for life.

LighterLife is supported by over 270 qualified weight management Counsellors around the UK, expert teams, including nursing and nutrition advisors; a medical director; psychotherapist support and a medical advisory board comprised of international experts in obesity, metabolism, endocrinology, clinical nutrition and psychology. The board's role is to review all clinical aspects of LighterLife, and provide advice and direction

¹⁷⁴ <http://guidance.nice.org.uk/PHG/67>

to ensure compliance with best practice applicable to the use of very-low-calorie diets (VLCDs) and low-calorie diets (LCDs), current evidence-based research and health guidelines, including NICE guideline 43 on obesity. This ensures the best possible programmes are delivered, thus facilitating safe and effective weight loss.

LighterLife fully supports NICE as an authority which provides independent, authoritative and evidence-based guidance on the most effective ways to prevent, diagnose and treat disease and ill health, reducing inequalities and variation. In particular we appreciate the manner in which NICE works through a rigorous process that is centred on using the best available evidence but also includes the views of experts, patients and carers, and industry.

However, we do believe that NICE will have to redouble its efforts to guarantee that it continues to operate with the same level of professionalism and effectiveness, while at the same time ensuring that procedures do not result in unacceptable delays in the publication of its guidelines. In particular, we would like to highlight the following to be taken into consideration:

- LighterLife believes that NICE has to take greater account of the cost-effectiveness of clinical interventions. By taking into account the latest developments in research, NICE would ensure that patients have access to the best choice and interventions at all times alleviating an excessive cost on the health service.
- NICE's current approach to updating guidance may run the risk of leaving patients without access to the most up-to-date care interventions and limit patient choice. Unnecessary long periods of guidance development may not always enhance the quality of the final guidance, while risking compromising the delivery of high-quality services for patients.
- It is essential that NICE guidelines are updated and elaborated and in particular take into consideration the ramifications of such guidelines around the rate and amount of weight-loss and health care professionals approaches to VLCDs and the perceptions around safety.
- NICE should do more to further improve public engagement with health professionals and organisations and educate health professionals as to all the options available.
- NICE's role will be particularly important in the new Public Health system, with inexperienced commissioners in local authorities seeking expert guidance.

INTRODUCTION

1. LighterLife offers multi-component programmes, with increased physical activity, behavioural change and healthy eating being key features. The emphasis is on identifying personal psychological drivers of obesity and overweight. This enables individuals to achieve a healthier and manageable BMI by making sustainable changes to the way they eat, think and live.

2. Weight loss is initiated via either:

- LighterLife Total—a VLCD for the obese (BMI $\geq 30\text{kg/m}^2$) or for women/men with BMI $\geq 28\text{--}29.9\text{kg/m}^2$ and a waist circumference $>88\text{cm}/102\text{cm}$, using four nutritionally complete Foodpacks per day, which include soups, shakes, bars and porridge.

OR

- LighterLife Lite—a LCD for the overweight (BMI $25\text{--}29.9\text{kg/m}^2$), combining three nutritionally balanced Foodpacks (including soups, shakes, bars and porridge) per day with a meal from a selection of specified foods to provide key nutrients and energy during weight loss.

3. In conjunction, LighterLife Weight-Management Counsellors work with participants in single-sex, weekly groups (maximum 12) to facilitate techniques from transactional analysis (TA) and cognitive behavioural therapy (CBT). Developed for behavioural modification in weight management, these techniques aim to help participants understand their relationship with food and develop new skills to support healthier eating and lifestyle behaviours, including being more active.

4. Following weight loss, LighterLife's Management Programme focuses on establishing a healthier lifestyle through the continued development of a healthier psychology. It empowers people with coping mechanisms developed in the weekly group meetings using cognitive behavioural therapy to support ongoing change, both physical and emotional. This enables sustainable weight management and a reduction in the risk of weight-associated co-morbidities. The Management Programme progresses individuals to a healthy, balanced and varied diet, consistent with current advice on healthy eating, and support meetings.

5. LighterLife would like to thank the Health Select Committee for the opportunity to comment on the work of the National Institute for Health and Clinical Excellence (NICE).

COMMENTS

6. LighterLife considers the work of NICE to be characterised by high quality, rigour and expertise. We believe that NICE's role in helping health and social care professionals to deliver the best possible care based on the best available evidence is highly commendable and we support the organisation's effort to continually improve its guidance and its other products.

7. LighterLife believes that NICE has to take greater account of the cost-effectiveness of clinical interventions, an element particularly important in the current economic climate. In the past, we have found that innovative, cost-effective interventions which are backed by peer-reviewed research have not triggered reviews of existing clinical guidance, even when a considerable number of pieces of research agreeing on the effectiveness of certain interventions were available. By taking into account the latest developments in research, NICE would ensure that patients have access to the best interventions at all times without an excessive cost on the health service.

8. Regular review of guidance is crucial to ensuring that the organisation is up to speed with new medical technology and clinical research. LighterLife therefore believes that NICE should be more open to regular updates of its guidance, especially when guidelines are too restrictive and do not reflect recent advances in clinical interventions. NICE's current approach seems to be based more on considerations related to procedural safety, but this does not take into account the importance of providing effective interventions as soon as reliable evidence is made available.

9. LighterLife believes that NICE's current approach to updating guidance may run the risk of leaving patients without access to the most up-to-date care interventions. We would like to mention the example of Clinical Guideline 43 on Obesity,¹⁷⁵ which was originally published in 2006 and which NICE refused to update after the first two years of operation, despite it being clear that a considerable amount of new data on effective interventions on obesity was available. This is a clear example of how an excessively conservative approach to the review of guidance may render it obsolete, denying patients the access to the best available interventions.

10. LighterLife welcomes NICE's efforts to engage with stakeholders on a more regular basis, including the health sector industry. This has not always been the case and we hope that this trend towards improved openness and transparency will continue in the future. We would welcome a move towards more regular engagement, both through open consultations and face to face meetings with relevant stakeholders, which would undoubtedly facilitate a more frank constructive discussion with NICE.

11. LighterLife is supportive of NICE's role in the formulation and provision of guidance based on the best available evidence. However, we believe that guidelines should also be produced taking into account the needs of the health profession and of the patients. If too much time is taken by NICE to produce or review its guidance, this may have negative consequences for patients who are denied access to the latest developments.

12. For example, the development of Public Health Guidance on "Overweight and obese adults—lifestyle weight management,"¹⁷⁶ which officially started in 2011, will not come to an end until May 2014. This long period of development will mean that local public health commissioners will not have access to high-quality guidance in this area for a whole year. In fact, health reforms provide that public health competences will be devolved to local authorities from April 2013. This situation should have been avoided by making sure that the schedule for the development of this guidance was adjusted to the needs of patients and health professionals.

13. Finally, we would like to note that, with the implementation of the welfare reforms and the new architecture of the public health system mentioned above, NICE's expertise will be ever more important. The new public health commissioners who take up their duties in April 2013 are bound to have limited experience of commissioning, and they will need to be appropriately assisted by NICE through well-designed and wide-ranging guidelines. We do hope that this increased level of responsibility will be matched by a more open approach to collaboration with external stakeholders when drafting and revising guidance.

CONCLUSION

14. We hope that LighterLife's submission has been useful in identifying some of the challenges with the way in which NICE carries out its work.

October 2012

Written evidence from the National Obesity Forum (NICE 73)

SUMMARY

The National Obesity Forum (NOF) was established in May 2000 to raise awareness of the emerging epidemic of obesity and the effect that it was going to have on both individuals and the NHS. NOF works to create recognition of obesity as a serious medical problem, provide education and training on obesity management and convince Government and healthcare workers to give obesity a high priority nationally and locally.

We greatly appreciate the role of NICE and its work in making sure that quality and excellence are always at the centre of healthcare provision in the UK. We do believe, however, that NICE will have to redouble its efforts to guarantee that it continues to operate with the same level of professionalism and effectiveness, while at the same time ensuring that bureaucratic procedures do not result in unacceptable delays in the publication of its guidelines.

¹⁷⁵ <http://publications.nice.org.uk/obesity-cg43>

¹⁷⁶ <http://guidance.nice.org.uk/PHG/67>

Please find below a summary of the main arguments which we have set out in our submission:

- NICE could consider taking a more flexible approach in updating its guidelines, as this would help it better accomplish its objectives of providing effective and cost-effective guidance.
- A more open approach by NICE to revising its own guidelines would be welcomed by many healthcare professionals and providers, and could lead to effective interventions being implemented more quickly.
- Long periods of guidance development may not always result in higher quality guidance, while at the same time they may have a negative impact on the delivery of high-quality services for patients.
- The new structure of the public health system will pose a considerable challenge to NICE and it will be important to make sure that its public health guidance is appropriately tailored to the needs of local commissioners.
- In recent times, NICE has improved the way in which it engages with relevant stakeholders and we hope that it will continue to engage in a positive manner with the public, health professionals and health organisations.

SUBMISSION

1. The NOF would like to thank the Health Select Committee for this opportunity to comment on the work of NICE.

2. The NOF is convinced that the role of NICE is crucial to help healthcare organisations, local authorities and others ensure that the best interventions are made available to patients, while at the same time guaranteeing the financial viability of the health service. We also believe that NICE's assessments of the cost-effectiveness of drugs and interventions is absolutely crucial at a time in which reforms to the health service and efficiency savings requested to meet the "Nicholson challenge" are constraining the financial resources available. NICE will undoubtedly need to continue providing guidance which is both up-to-date with the latest research and demonstrably cost-effective.

3. The NOF believes that NICE would be able to better accomplish its objectives of providing effective and cost-effective guidance by making sure its approach to new evidence and research is adequate. We believe that NICE should provide more flexibility in the way it updates its own guidelines and allow these to be updated more frequently, ensuring that the latest updates to medical interventions and clinical research are taken into account.

4. At the present time, NICE appears to rely on a rather conservative approach to the review and update of its guidance, something that is certainly due to the need to ensure that the evidence on which the guidelines are based is sound and tested. However, this approach can become frustrating for healthcare professionals and providers who sometimes observe how some very effective interventions are not put into practice as NICE is reluctant to review its guidelines.

5. A notable example of this conservative approach is provided by Clinical Guideline (CG) 43 on Obesity, first published in 2006, which NICE only agreed to update at the end of 2011. NICE came to this decision only after several organisations had showed that much new scientific evidence was available, highlighting how research on effective and cost-effective interventions on obesity had substantially developed. For instance, the evidence base for obesity-related interventions has moved rapidly in the last few years on issues such as sarcopenia in elderly patients; the obesity paradox; epigenetics; and metagenomics. We believe that innovation and research should play a bigger role in NICE's decisions about whether or not to update its CGs.

6. A similarly conservative approach, we believe, is taken by NICE in the formulation of new guidance. As outlined previously, the NOF is convinced that guidelines always need to be based on peer-reviewed evidence and authoritative research, but it is equally important that guidelines are produced in a timely manner, responding to the needs of the health profession and of the patients. Unnecessary long periods of guidance development may not always enhance the quality of the final guidance, while risking compromising the delivery of high-quality services for patients.

7. In this case, the NOF can mention the example of the draft guidance on *Managing overweight and obesity in adults—lifestyle weight management services*, which is currently being developed by NICE.¹⁷⁷ The latest updates on the draft guidance's webpage indicate that NICE's work will continue until the spring of 2014. While we appreciate that guidance specifically aimed at giving advice to local authorities on who should be involved as potential partners and stakeholders in weight management service provision will be available shortly, we believe that the delay in updating this and other obesity-related guidelines could create issues for local commissioners during the period between April 2013 and May 2014. Indeed, during this initial period of activity local commissioners would certainly value the provision of accurate and up-to-date guidance from NICE.

8. As briefly outlined above, the new, localised public health system which will be implemented from April 2013 will see many challenges for local commissioners, who will more than ever need the close support of

¹⁷⁷ <http://guidance.nice.org.uk/PHG/67>

NICE through its expertise. The NOF believes that NICE will have to take this into account and make sure that its public health guidance is appropriately tailored to the needs of local commissioners.

9. The NOF notes that in recent times NICE has implemented measures aimed at improving its engagement with stakeholders and we greatly appreciate this development. We do hope that more will be done to further improve public involvement.

November 2012
