Cystic Fibrosis Trust Response: Medicines and Medical Devices Bill

1. Cystic fibrosis is the most common life-limiting genetic disease affecting over 10,500 children and adults in the United Kingdom. 1 in 25 of people carry a copy of the faulty gene that causes cystic fibrosis. The wide range of progressive symptoms and co-morbidities affect multiple organs in the body and require a rigorous medicine and physiotherapy regime to manage. Last year, the median age at death in the UK was only 32.

2. The Cystic Fibrosis Trust is the only UK-wide charity dedicated to fighting for a life unlimited by cystic fibrosis for everyone affected by the condition. We support people with cystic fibrosis and their families by:
    - funding a variety of research programmes including digital care and stem cell research
    - hosting a Clinical Trials Accelerator Platform to support CF centres in recruiting suitable participants for trials and to ensure new drugs reach the market more quickly
    - supporting clinical cystic fibrosis teams in the NHS to improve delivery of care and staffing
    - campaigning on key issues including access to new medicines

3. **Introductory Comments:** The Cystic Fibrosis Trust is supportive of the introduction of the Medicines and Medical Devices Bill. It is vital that appropriate regulation is in place to avoid disruptions to accessing medicines and medical devices, particularly with the UK's departure from the European Union. We also welcome Government recognition of the importance of bringing new medicines and medical devices to market post-Brexit and ensuring the attractiveness of the UK as a place to conduct clinical trials.

4. **Data and Digital Innovation:** We commend the Bill for highlighting the importance of data and digital innovation in healthcare and the potential for the bill to support this. It is widely recognised that cutting-edge technology is developing quickly and there is a huge role for data in many areas, including in pharmacovigilance under a strengthened Medicines and Healthcare products Regulatory Agency (MHRA) and decision making via 'app-based data collection'. The outbreak of COVID-19 has highlighted the importance and value of technology in finding solutions across government and the NHS, from texting patients, updating guidelines, empowering home monitoring of chronic conditions, and the use of efficient technology to speed up diagnosis. However, it is imperative to also consider the impact that health technology will have on patient safety and ensure there is balance between the swift approval of health technology and its safety.

5. The Cystic Fibrosis Trust has provided grants to support digital health research projects. One example is Project Breathe which is exploring the use of home monitoring to detect lung exacerbations with the aim of (1) using this technology to avoid unnecessary visits to hospital, saving on NHS resources and (2) early detection so that people with cystic fibrosis get care sooner to avoid further permanent lung damage.

6. **Clinical Trials:** Clinical trials are vital for people with cystic fibrosis to ensure access to ground-breaking treatments that are safe and effective. The UK is leading the way in early-stage clinical research into new medicines and vaccines, with more than 600 commercial clinical trials taking place in the NHS.[1] Further, patients in the UK and Europe both benefit from the UK's participation in EU trials and this has helped improve access and patient recruitment in rare disease and paediatric trials. Over 4,800 UK-EU trials were conducted between 2004 and 2016.[2]

7. We commend the Bill for recognising the important of clinical trials and the desire to ensure the UK remains a leader in this field. We would urge the UK to continue collaboration with the EU by ensuring the MHRA

[1] Cancer Research UK, Future of Clinical Trials after Brexit, 2018: https://www.cancerresearchuk.org/sites/default/files/future_of_clinical_trials_after_brexit.pdf
[2] ABPI, Clinical trials: How the UK is researching medicines of the future, October 2019: https://www.abpi.org.uk/publications/clinical-trials-how-the-uk-is-researching-medicines-of-the-future/

maintains a close working relationship with the European Medicines Agency (EMA). Otherwise, we are concerned that UK/EU misalignment could risk the UK becoming a less attractive area to do research. This could result in the costs of medicine increasing within the UK, which will hinder NICE recommendations of medicines due to cost-effectiveness, particularly for rare diseases such as cystic fibrosis. It also risks the UK facing barriers to participate in multi-country clinical trials which provide researchers with access to the large population base required; this can particularly hinder rare disease clinical trials where researchers already struggle to recruit suitably sized cohorts. To increase participation, there is also a need for home-based monitoring in trials to enable people with cystic fibrosis to participate in trials whilst decreasing the possibility of cross-infection.

8. To help increase participation on cystic fibrosis clinical trials, the Cystic Fibrosis Trust has implemented the Clinical Trials Accelerator Platform (CTAP) to increase awareness and supporting clinical trial opportunities. Even though we appreciate the risks mentioned above will depend on the type of deal the UK makes with the EU, mitigation plans should be formulated to prevent any adverse effects through avenues such as Clinical Trials Accelerator Platform. The Cystic Fibrosis Registry is also working on storing clinical trial results such as CF START, the first registry based clinical trial which is assessing the safest and most effective way to treat infants diagnosed with cystic fibrosis with antibiotics.

Further clarity on how the Government will address alignment with the EU and plans going forward to safeguard our position as an attractive place for research going forward would be welcome.

9. **Conclusions:** The Cystic Fibrosis Trust supports the Medicines and Medical Devices Bill but would urge the Government to implement a mechanism for allowing AI and machine learning to directly influence diagnosis and treatments. The Cystic Fibrosis Trust is in a unique position to offer data and lived experience from a patient group who encapsulate the barriers facing rare disease communities regarding access to medicine and treatments due to limitations in clinical trials. The Cystic Fibrosis Registry also holds healthcare data on 99% of the CF community with 100% participation from CF centre with 20-year longitudinal data. We would be interested in discussing possible solutions to the issue we have outlined, please contact us at policy@cysticfibrosis.org.uk.