Brussels, 22 January 2021

Subject: Ongoing evaluation of the Orphan Medicinal Products Regulation

Dear Vice-Presidents, Dear Commissioners,

The EU's Orphan Drug Regulation has incentivised public and private investment in biotechnology, created an encouraging environment for research into rare diseases, and served as a catalyst for the development of life-changing treatments. Trying to alter the Regulation puts this flagship EU success story at risk.

New medicines often begin in the lab as an idea. The researchers believe they may be on the cusp of identifying a new way to improve the quality of life for patients with a certain condition. Although many such ideas hold promise, the reality, unfortunately, is that many of these investigational therapies bear no fruit upon undergoing the rigorous scientific research required to bring them to life.

The pathway from the lab "bench to bedside" is a long, difficult, and challenging process. It can be especially cumbersome for smaller biotech companies, whose scientists and researchers strive to transform their hypothesis into an effective therapy.

This is particularly true in the field of rare diseases. Much less is known about these diseases compared to more common conditions, thus requiring more extensive research. The scientific complexity is costly, making the development of a medicine for a rare disease a rather difficult undertaking. External funding to conduct this research is often essential for many biotech companies, particularly SMEs. These companies need to make a very strong case to convince any potential investors to incur the high economic risk of early-stage research and lengthy therapeutic development.

Acknowledging the significant costs and complexity in rare disease research, the European Union adopted the OMP Regulation in the year 2000. The aim of the Regulation was to create a positive environment for the development of new treatments for these conditions. This proved to be a great success. Prior to 2000, there had been just 8 orphan-like products available in the EU. Since the introduction of the legislation, the number of medicines in development for rare conditions has increased consistently with 8% year-on-year growth between 2000-2015 (EMA, 2017) and over 2000 medicines for rare diseases in development by the end of 2019 (EMA, 2020). Behind those numbers are thousands of patients, who today have a treatment option, where there previously was none.

The increased focus on rare diseases means our understanding of them is increasing day by day. This has also led to the creation of a myriad of new SMEs, generating high-quality jobs for people working to bring new therapies to patients who need them. SMEs are responsible for nearly half of all orphan medicines in development in Europe (European Commission, OMP Evaluation 2020). This all points to the fact that European science can be world-class— given the right conditions.

Despite the success of the legislation, there are indications coming from public officials that amendments to this Regulation are being considered to address issues which do not fall within its scope. It is our conviction, that any changes to the legislation will endanger the progress made thus far, creating unnecessary unpredictability, and will negatively impact our ability to attract the capital needed to fund our research.

We understand there are concerns regarding the differences in availability of certain medicines among the different EU Member States. These concerns are often the result of varied and fragmented data requirements, processes, and bureaucratic timelines, which fall exclusively within the national competence. These concerns can, and must, be addressed without jeopardising EU legislation that successfully encourages much-needed research into conditions with unmet medical need.

This can be achieved without changing the Regulation, but by developing a high-level dialogue, encompassing all stakeholders: patients, payers, policy makers, regulators, and industry. We stand ready to engage in constructive discussion on non-legislative means to ensure equity of patient access in Europe while maintaining a world-class environment for innovative research.

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