Statement

Access to treatments is not only about incentives

EURORDIS responds to the European Pharmaceutical Strategy

8 December 2020, Brussels - EURORDIS-Rare Diseases Europe – an alliance of 944 patient organisations - welcomes the European Commission's Pharmaceutical Strategy, published on 25 November, but urges greater ambition in the scope of the Strategy as it is implemented to improve both development and access to treatments for people with rare diseases in Europe.

The long-awaited Strategy, part of the push from the European Health Union to prioritise and centralise actions around health under the European Health Union, sets out some crucial initiatives that could prove pivotal in improving the environment for patient access to treatments in Europe. This includes a proposal to review the EU Regulation on orphan medicinal products through more tailored incentives by 2022.

The Pharmaceutical Strategy was eagerly awaited by the 30 million people living with a rare disease as an opportunity to recognise and review the access to treatments landscape as a whole. Unfortunately, with the emphasis mainly on incentives for the time being, this falls short of us being able to make a real difference in how people with rare diseases access treatments in Europe.

It remains to be seen how some very promising initiatives (e.g. on parallel scientific advice, on innovative procurement mechanisms, repurposing of medicines and knowledge sharing to improve transparency, availability and affordability) are effectively implemented to make the much needed change.

Results from a EURORDIS survey of 7,500 rare disease patients and family members revealed that only 5% of respondents already accessed a centrally approved curative or transformative treatment. We hear regularly of cases when a patient's access to an approved treatment is obstructed because of the fragmented European market. And most of the 6,000+ rare diseases still do not have a treatment, resulting in a high unmet need. This is why urgent action is needed

The EU Regulation on orphan medicinal products has helped transform the lives of many people, with 190 new orphan treatments approved and 2,338 orphan designations. As it is reviewed, EURORDIS urges the Commission **to uphold the principle established by the Regulation that people with rare diseases deserve access to the same quality treatment as the general population.**

In proposing legislation that will shape the way we do things for the next twenty years, Europe must confront what works and what does not work well now. Whilst we appreciated the

assessment and some of the options set forth by the Inception Impact Assessment on Orphan Medicinal Products and Paediatric Medicines Regulations, we are missing a clear link with the proposed initiatives provided in the Pharmaceutical Strategy, notably on cooperation on access and price where Europe can have a real added value. This rings true also for the Inception Impact Assessment on Paediatric Medicines and Orphan Medicinal Products Regulations: whilst we share much of the analysis of the situation and some of the solutions provided, the options outlined provide only a partial solution to the existing challenges, and we regret the lack of specific link between incentives and access.

In our 2018 paper Breaking the Access Deadlock to Leave No One Behind we set forth a structured approach to market access to therapies for rare diseases, and we are pleased to see many recommendations being taken up, ranging from supporting innovative clinical trials design to enabling cross border exchange of data.

However, we urge policymakers to pay close attention to the potential of setting up a voluntary mechanism for joint negotiations for rare and complex disorders, particularly with the scientific promises represented by advanced therapies, supported by a European financial instrument to support data collection post authorisation, to enable Member States to put in place flexible and innovative financial arrangements to provide access for those in desperate needs of access.

EURORDIS and its community stands ready to provide input on the current challenges devise a cutting edge framework enhancing the role of Europe as a global innovator leader and enabling solutions that are truly patient-centred and patient-driven.

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About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 930 rare disease patient organisations from 73 countries that work together to improve the lives of the 30 million people living with a rare disease in Europe. By connecting patients, families and patient groups, as well as by bringing together all stakeholders and mobilising the rare disease community, EURORDIS strengthens the patient voice and shapes research, policies and patient services.

About rare diseases

The European Union considers a disease as rare when it affects less than 1 in 2,000 citizens. Over 6,000 different rare diseases have been identified to date affecting an estimated 30 million people in Europe and 300 million worldwide. 72% of rare diseases are genetic whilst others are the result of infections (bacterial or viral), allergies and environmental causes, or are degenerative and proliferative. 70% of those genetic rare diseases start in childhood.

Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offerings inadequate and research limited. Despite their great overall number, rare disease patients are the orphans of health systems, often denied diagnosis, treatment and the benefits of research.