## Media statement

# EURORDIS responds to European Commission's proposals to reform pharmaceutical legislation

***26 April, Brussels* – As part of the EU pharmaceuticals strategy, the European Commission has today published its long-anticipated proposed revisions to the EU's general legislation regulating medicines for human use.**

The Commission's proposed revisions include proposals to update the EU's Regulations on Orphan Medicinal Products and Paediatric Medicines, which are acutely important to Europe's population of patients who live with a rare disease.

Whilst the number of approved therapies has increased dramatically, they have been overly concentrated in a certain selection of medical areas, whilst other rare conditions with lower prevalence have been neglected, and many rare disease patients have not seen their major unmet medical needs adequately addressed.

Only 6% of all known rare diseases have an approved treatment option, and 69% of rare disease patients have received only symptomatic treatment for their rare condition.

Notably, legislative improvements to the Orphan Medicinal Products Regulation were a key recommendation of the landmark *Rare 2030* foresight study, which published in February 2021 and gathered the input of a large group of patients, practitioners and key opinion leaders.

Responding to the Commission's proposed legislative reforms, **Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe**, said:

> "The European Commission is proposing measures that many rare disease patients and their families will welcome, from more rapid regulatory pathways for new products to more targeted incentives for companies to develop desperately needed medicines.
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> "The proposals should provide hope to those rare disease patients who deserve longer, better and healthier lives, and whose care has failed to keep up with the fast pace of advancements in medical science and innovation.
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> "EURORDIS and our members will now call on the European Parliament and Member States to amend the proposed legislative changes for the better where needed, to ensure no EU citizen living with a rare condition is left behind.
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> "We are glad that the concept of modulation of incentives has been introduced to foster investment and research in the great majority of rare diseases that do not have any

therapeutic options, whilst maintaining a competitive level of marketing exclusivity as compared to other jurisdictions.

"Toward the goal of making our continent even more competitive, EU policymakers should consider for example awarding developers an additional year of market exclusivity for launching a product first in the European market. This would draw greater investment toward clinical trials taking place in Europe, allow treatments to reach EU citizens much faster.

"Policymakers must also realise the opportunity being presented to them to place the EU on an ambitious and more competitive footing with the US and other regions when it comes to boosting the development and accessibility of rare disease medicines. Europe should be made no less attractive to developers than the US, China or India as a place to embark upon the discovery of new, transformative, ground-breaking treatments.

"Whilst we are delighted of the inclusion of patient representatives in the Committee for Human Medicinal Products (CHMP), greater clarity is needed in the proposed legislation on the involvement of patient representatives in regulatory processes, alongside assurances that patient representatives' inclusion and advice will be mandatorily taken into account through their membership of working parties at the European Medicines Agency.

"To make the system for incentivising rare disease medicines' development as robust as possible, we need greater clarification in some of the key passages. For instance, it is not currently clear what will constitute a 'meaningful reduction in disease morbidity or mortality', or which medical conditions may be deemed 'significantly debilitating'.

"We also believe that legislative measures should be introduced that would establish a European pathway from development to access for ultra-rare diseases or those conditions that currently have no therapeutic options, for example, in the article codifying PRIME in the pharmaceutical legislation. We need a structured and seamless regulatory pathway for medicines, from early dialogue, to scientific advice, to marketing approval, to clinical assessment, to health technology assessment, and ultimately to European-level negotiation and procurement.

"In addition, reducing EU citizens' delayed access to treatments must be progressed right away through policies, investment, and structured collaborations across Member States, which fall outside the scope of this landmark piece of legislation and do not need to wait for it to pass into law. For example, extending effective approach for early access or compassionate use, as implemented in some Member States, can provide better accessibility, whilst reducing pressures on payers and sponsors when negotiating reimbursement.

"Europe has lost major ground to its global competitors over the past couple of decades. Despite a favourable regulatory and incentives framework, the market remains fragmented and there is a lack of coordinated research infrastructure, investment and access. As a community, we feel all these elements should be included in a goal-based 'European Action Plan for Rare Diseases', in order to ensure that critical pieces of legislation, such as the Pharmaceutical Package, work effectively in the building of the European Health Union."

## About EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 1,000 rare disease patient organisations from 74 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.

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