Press release

EURORDIS-Rare Diseases Europe publishes a Joint Statement with EFPIA on proposals to improve patient access to medicines

15 June, Brussels **– Today marks the publication of the EFPIA-EURORDIS Joint Statement on Patient Access to Medicines for Rare Diseases. The statement is based on an 18-month structured dialogue between the two organisations focused on identifying proposals that can improve access to orphan medicines across Europe. At the same time, the statement recognises areas where the two organisations have different perspectives.**

This is the first time that EURORDIS-Rare Diseases Europe, as a patient organisation, has come together with the pharmaceutical industry's European organisation in such a structured dialogue. Discussions focused on how to improve patient access to orphan medicinal products, which remains inequitable across countries and routinely delayed. The issue of access was addressed from a multi-faceted perspective, based on the root causes identified by the two organisations. This common effort between two key stakeholders on rare disease therapies is particularly relevant in the context of the upcoming revisions of the general EU General Pharmaceutical Legislation, including the Regulation on Orphan Medicinal Products, and the EU Regulation on Paediatric Use of Medicines, later this year.

EURORDIS believes that the three sets of proposals put forward - increased equity of access for patients and solidarity between Member states, improved HTA and Pricing and Reimbursement (P&R) processes, and accelerated needs-led innovation - will bring about tangible change in access to medicines for people living with a rare disease.

EURORDIS has promoted Differential Pricing as a route for better access and more equity across Europe in a constant effort since 2012. A proposal on Equity-Based Tier Pricing has been a hallmark of EURORDIS' Position "Breaking the Access Deadlock" issued in 2018. EFPIA jointly agreeing to the conceptual framework reflects progress and advances in finding solutions at the European level for rare disease therapies.

The proposal to reinforce European collaboration on post-launch Real-World Evidence came from the shared views on the obstacles caused by evidential uncertainties at the time of marketing authorisation for value assessment, and its impact on a well-informed discussion on pricing & reimbursement, recognising that the solution comes from a continuum of evidence generation.

In addition, EFPIA's commitment to "a Moonshot to develop science for rare diseases", is welcome given how much there is still to learn about rare diseases, especially in underserved areas. Funding in Public-Private Partnerships is a critical success factor in both research and healthcare, for clinical research before and after marketing authorisation.

> *"Often European and national policies cannot progress, because the analysis of the issues is unclear, the solutions are not identified, or there is no consensus among the stakeholders. Here, in rare disease therapies, there is a wide agreement on the issues of access and their root causes, there are concrete solutions designed over the years through robust work embedded in real-life experience and multi-stakeholder dialogues, and now there are clear common positions amongst stakeholders. This Joint Statement represents the progress that would not happen without taking ourselves out of our comfort zone in such discussions. We hope it comes at the right time to help align the will of policy makers and stakeholders for positive change."*
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> – Yann Le Cam, Chief Executive Officer of EURORDIS-Rare Diseases Europe

Speaking about the publication of the statement, EFPIA Director General, Nathalie Moll said: *"EFPIA and EURORDIS share the goal of faster, more equitable access to medicines for patients living with rare disease across Europe. Both the structured dialogue and today's Joint Statement are a proactive effort to find solutions to access issues together. We did*

not agree on all points but found enough common ground to bring forward a series of concrete proposals that we believe can make a real and tangible difference to Europeans living with rare disease."

Despite reaching agreement on certain initiatives, there remained areas where the two bodies favoured different approaches, such as on EURORDIS' proposal for an EU Fund for Real World Evidence, and on the scope or methodologies of European joint procurement. On the issues of medicines prices and budget impact, which remains contentious in facilitating patients' access to treatment, the positions remain different. While it was not covered in the statement - and we are calling on industry to be more ambitious and flexible in their approach - this should not undermine the value of consensus reached on the other solutions.

EURORDIS and EFPIA invite other stakeholders to consider the proposals and to continue the debate, on the proposals discussed and beyond. Both organisations encourage the dialogue to be formalised in policy commitments as EU Regulations on Orphan Medicinal Products, on Paediatric Use of Medicines and the Pharmaceutical Package more broadly are reviewed at the end of 2022.

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of 1000 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. Follow @eurordis or see the EURORDIS Facebook page. For more information, visit eurordis.org.

EFPIA-EURORDIS Joint Statement

This statement is the result of 6 meetings between late 2020 and December 2021. On the side of EURORDIS, staff and volunteers took part in the dialogue, with additional consultations with EURORDIS' wider membership at specific points. The structured dialogue and resulting statement were prepared by Dolon, the knowledge partner.

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