PRESS RELEASE

European rare disease patients call for increased European collaboration on medicine pricing to improve access

Paris, 1 June - EURORDIS-Rare Diseases Europe and its member organisations are calling for more collaboration between all relevant national and European authorities and the pharmaceutical industry to streamline the medicine pricing process **so that patients can access the medicines they need**.

At the recently held EURORDIS Membership Meeting, exasperated rare disease patient representatives expressed their frustration concerning the current situation around access to medicines; **an increasing number of cases are being reported across Europe of medicines not being reimbursed following marketing authorisation or even of reimbursement decisions being reversed.**

European authorisation of an orphan medicine is granted at the EU level but decisions on its pricing and reimbursement are made nationally. This leads to unwanted fragmentation and in turn often irrational pricing decisions.

Increasingly, new and innovative medicines approved for use in Europe are considered to be priced so highly in comparison to their perceived value that they are not reimbursed by national healthcare systems, **and therefore not available to the patients that need them**.

The value of a medicine- A call to look at solutions beyond the problem

Due to the technological complexity of orphan medicines, and also to the heterogeneity and scarcity of rare disease patient populations, these medicines often reach the milestone moment of marketing authorisation **without offering all of the necessary evidence** that demonstrates the value they deliver for patients and healthcare systems. As a result, in a time of constrained budgets, national authorities are becoming increasingly reluctant to reimburse orphan medicines because of that very uncertainty over their efficacy or effectiveness in the long run.

The rare disease patient community is therefore calling on all parties to recognise that **the real added value of an orphan medicine** can only be demonstrated through the continuous generation of real-life evidence in the years *following* marketing authorisation and all along the life cycle of the medicine.

Greater efforts are needed from **the pharmaceutical industry to openly justify that the prices assigned to their medicines** reflect the true cost of innovation and a commitment to long term sustainable development of new medicines rather than a short term, quick return in pursuit of maximum profit.

With that in mind, **EURORDIS and members call on all parties to take action and collaborate constructively to ensure this new European approach becomes a reality.**

Yann Le Cam, Chief Executive Officer, EURORDIS, commented: "Our members have spoken. Enough is enough. Scientific innovation cannot be disconnected from access to medicines. If an innovative medicine is approved but does not reach all the patients who need it, it fails in its primary objective. Scientific advances *are* being translated into new medicines, but it is a catastrophic failure when these medicines do not reach the patient because of a failing business model."

He added, "Rare disease patients and their families are growing increasingly angry as a result of this unethical situation, one that is detrimental to the lives of people living with a rare disease and also to industry's readiness to develop new innovative medicines. There is a high level of mistrust between payers and industry; patients should not be paying the price for this lack of collaboration and dialogue."

The smart move- joining forces

EURORDIS and its members want payers and industry leaders to react more quickly to address these issues by engaging in European collaboration on pricing, funding and patient access. In May 2015, EURORDIS and the European Patients' Forum (EPF) launched a call on the national authorities to collaborate on medicines pricing and access schemes at a European level.

A year later, EURORDIS and EPF have recently written to national authorities in Belgium, the Netherlands and Luxembourg to call upon them to extend their agreement for joint negotiation of orphan medicines pricing to other Member States expressing an interest.

Next steps

EURORDIS continues to work closely with EPF on a new position statement on access, value, pricing and sustainability of medicines (June publication).

EURORDIS is also contributing to reflections currently under way in other settings, for example in a working group run by international consultancy FIPRA, which will publish a paper on outcomes-based value and funding in the near future.

Together with a working group on value led by health economist Lieven Annemans, EURORDIS is also developing Principles for Value Assessment and Funding Processes for Rare Diseases.

Finally, EURORDIS is working with its members to develop a new call for action proposing a detailed structured approach to improve patient access.

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EURORDIS

EURORDIS, the European Organisation for Rare Diseases, is a non-governmental patient-driven alliance of patient organisations representing over 700 rare disease patient organisations in 63 countries. EURORDIS represents the voice of an estimated 30 million people living with a rare disease in Europe. Follow @eurordis or see the EURORDIS Facebook page. For more information visit: www.eurordis.org

Rare Diseases

The European Union considers a disease as rare when it affects fewer than 1 in 2,000 citizens. Over 6000 different rare diseases have been identified to date, affecting over 60 million people in Europe and the USA alone. Due to the low prevalence of each disease, medical expertise is rare, knowledge is scarce, care offering 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.