



[Press release]

Rare disease community calls for radical change to improve patients' access to medicines

6 December 2017, Brussels – EURORDIS-Rare Diseases Europe and its over 700 member patient organisations today launch a new position paper calling for urgent, radical change to ensure patients' full and fast access to rare disease therapies in Europe.

Read the full position paper at eurordis.org/accesspaper

The position paper sets out a new four-pillar approach to tackling the challenges that prevent patients' access to care and medicines, as well as the **ambition to have 3 to 5 times more new rare disease therapies approved per year, 3 to 5 times cheaper than today by 2025.**

Today's science and technology create unprecedented opportunities to address the unmet medical needs of people living with a rare disease. Before this potential can be translated into actual health benefits, **issues around access are creating a deadlock.**

The majority of people living with a rare disease have delayed or no access to the medicine they need, or no medicine even exists. If a therapy is approved for market but does not reach those who need it, it has failed in its primary purpose.

In the paper, EURORDIS calls for a new model based on a collective conversation involving all stakeholders (patients, the pharmaceutical industry, national competent authorities, national health ministries, researchers, scientists and regulators).

Yann Le Cam, Chief Executive Officer at EURORDIS, commented, *"When it comes to access, we refuse to take no for an answer. We will not cave under the weaknesses of the current model. We, rare disease patients, are not the problem: we are part of the solution".*

"We need to close the gap between innovation and access. All stakeholders have an urgent collective responsibility to shape a new approach that will accelerate the translation of major scientific advancements into new therapies. We believe that a new ecosystem is possible, a framework based on a global approach to innovation for unmet medical needs and on sustainability for healthcare systems as well as financial attractiveness to industry and investors."

"We call on national competent authorities for pricing and pharmaceutical manufacturers to indicate where they stand on the approach set out in this paper – what they are willing to support and what they are more reluctant to accept."

The new position paper sets out a [four-pillar approach](#) that encompasses:

1. A new blueprint to cut costs and fast-track R&D ([read more](#));
2. Early dialogue and cooperation between healthcare systems on the determination of value of a medicine and on patient access ([read more](#));

3. A transparent European cooperation framework between national healthcare systems for the determination of fair prices and of sustainable healthcare budget impacts ([read more](#)); and
4. A continuum approach to evidence generation linked to healthcare budget spending ([read more](#)).

The paper also dispels misconceptions around the pricing of orphan medicines and sets out the facts: that **orphan status of a produced in Europe is not easy to obtain** or maintain; that according to research out of 70+ orphan medicines approved by the EMA up to April 2014, **24% of them had an annual cost inferior to £10,000 (€11,000)**, and only 18% of them an annual cost superior to £100,000 (€111,000)ⁱ; and that evidence demonstrates that **orphan medicines continue to represent overall an extremely small fraction of the pharmaceutical budgets** of EU Member States (i.e. around 5% of the total pharmaceutical expenditure on average for EU Member States)ⁱⁱ.

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About this EURORDIS Position Paper

The paper 'Breaking the Access Deadlock to Leave No One Behind' is a contribution from EURORDIS and its members and follows a reflection process initiated at the EURORDIS Symposium on "Improving Patient Access to Rare Disease Therapies" (February 2017). It offers a synthesis of their analysis, reflections and perspectives on the issue of access to rare disease therapies. [Read the full paper](#).

EURORDIS-Rare Diseases Europe

EURORDIS-Rare Diseases Europe is a unique, non-profit alliance of over 700 rare disease patient organisations from more than 60 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 www.eurordis.org.

Rare diseases

The European Union considers a disease as rare when it affects less 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.

Press contact

Eva Bearryman, Communications Manager, EURORDIS-Rare Diseases Europe
eva.bearryman@eurordis.org +33 1 56 53 52 61

ⁱ "Effectiveness, safety and costs of orphan drugs: an evidence-based review" Onakpoya IJ, Spencer EA, Thompson MJ and Heneghan CJ. *BMJ Open*. 2015; 5(6): e007199. Published online 2015 Jun 24. doi: 10.1136/bmjopen-2014-007199, accessible here: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4480037/pdf/bmjopen-2014-007199.pdf>

ⁱⁱ Orphanet Journal of Rare Diseases. 2011 Sep 27;6:62. DOI: 10.1186/1750-1172-6-62. "Estimating the budget impact of orphan medicines in Europe: 2010 - 2020." Schey C, Milanova T, Hutchings A. <https://ojrd.biomedcentral.com/articles/10.1186/1750-1172-6-62>