



PRESS RELEASE

## **EURORDIS calls for collaborative process to improve patients' access to rare disease medicines**

**#RareEU2017**

- *EURORDIS Multi-Stakeholder Symposium brings together all stakeholders to initiate an unprecedented cooperative process to ensure patients' access to rare disease medicines*
- *New goal: a three to fivefold increase in the number of rare disease medicines approved by the European Medicines Agency per year by 2025 and those medicines available at one third to one fifth of the price.*

**22 February 2017, Brussels** – Today, at its second Multi-Stakeholder Symposium, EURORDIS-Rare Diseases Europe initiates a **collaborative process between all stakeholders** involved in developing medicines that aims to improve patients' access to rare disease medicines.

The opening session of the Multi-Stakeholder Symposium on Improving Patients' Access to Rare Disease Therapies is web streamed live via [www.eurordis.org/live](http://www.eurordis.org/live).

The event creates a unique opportunity to bring together 400 participants from all parties involved in developing medicines - patient advocates, payers, health technology assessment authorities, academics, clinicians, policymakers, investors and industry representatives - in an open environment to find ways to improve patients' access to rare disease medicines.

Even though ever-advancing science and technology now create rapidly growing opportunities to develop more innovative medicines, the rare disease community is still facing problems accessing these medicines in Europe because of their affordability.

**EURORDIS is calling for change to the system** to ensure that medicines are immediately accessible to patients, affordable and sustainable for national healthcare systems. The system should create a predictable and sustainable environment for companies and investors.

**Yann Le Cam, Chief Executive Officer, EURORDIS**, commented, "We will not take no for an answer. EURORDIS is calling for radical change. This change will not be easy but is urgently needed to translate innovation into health benefits for patients. Improving access can only be achieved by establishing a collaborative multi-stakeholder structured approach. Our goal is to leave no one behind"

He continues, "Collectively, we should aim for the goal of **a three to fivefold increase in the number of rare disease medicines approved by the European**

**Medicines Agency (EMA) per year by 2025 and those medicines available at one third to one fifth of the price.”**

Mr Le Cam added, “We need collective, multi-stakeholder negotiation that is based on the interests of all parties involved. Pharmaceutical companies, researchers and scientists, the EMA, and national competent authorities that carry out health technology or pricing assessments must all accept that there will be wins and losses for everyone involved. In the end **we must find a new deal that ultimately ensures that patients can access medicines** so that the devastating impact of rare diseases is reduced.”

After the symposium, this new collaborative process will be set in motion based on the [One-Text Process](#), used by negotiators and mediators to manage complex subjects involving numerous stakeholders who hold conflicting views.

### Goal of Symposium

This Symposium builds on the [first EURORDIS Symposium on Access](#) that took place in February 2016. **The goal is to develop a cooperative process that will lead to solutions that respect the interests of all stakeholders.** This process will enhance and sustain trust between the various stakeholder groups that play a role in improving patients’ access to rare disease therapies. It will help to build mutual understanding between each group of their respective perspectives and expectations on how to improve access.

**The ultimate goal of this cooperative process is to reach affordable, sustainable and long-lasting solutions to improve patients’ access to rare disease therapies.** There is not one simple solution, but instead numerous initiatives, some of which are already in development and in some cases are being piloted, and others, which are new but promising. Such initiatives are being discussed at today’s Symposium.

### New EURORDIS Reflection Paper on Access

EURORDIS will share a new paper “[Breaking the Access Deadlock to Leave No One Behind – a contribution on possibilities for patients' full and equitable access to rare disease therapies](#)” for the first time at the Symposium in Brussels today.

This reflection paper, still a work in progress, is EURORDIS’ first on the topic of access since the joint EURORDIS-EPF ‘[Call on Payers](#)’ in 2015. It is intended to challenge a number of existing misconceptions around rare disease medicines. It sets out how to design a new and improved approach to ensure patients’ full access to these medicines, an approach that addresses the current imbalance and restores in parallel transparency and also trust between payers and pharmaceutical companies, paving the way for common value principles and fair pricing of medicines.

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## 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.

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## 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.

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