



## Event: Improved European collaboration needed to better patients' access to rare disease therapies

**24 February 2016, Brussels, Belgium** – A unique combination of representatives from industry, patient groups, academia, health-technology-assessment bodies, regulators and payers today come together to discuss the pressing issue of how to improve patients' access to rare disease therapies across Europe. Speakers at the EURORDIS *Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies* include EU Commissioner for Health & Food Safety **Vytenis Andriukaitis** and Member of the European Parliament for Belgium **Philippe de Backer**.

**Commissioner Vytenis Andriukaitis** commented, "People with rare diseases endure years of uncertainty waiting for their disease to be diagnosed and for an appropriate treatment to be found. We must pursue our efforts in helping to ensure that each person suffering from a rare disease gets the support and treatment she or he needs. I am convinced that co-operation at EU level can make a difference. Indeed, the European Commission can help in pooling together expertise, for example through the European Reference Networks, in supporting research, in granting the authorisation to the best possible medicines and in fostering co-operation to make medicines more affordable."

A EURORDIS Rare Disease Day 2016 event, this two-day symposium has been organised to address a crucial bottleneck in making orphan medicinal products accessible across Europe:

**Yann Le Cam, Chief Executive Officer of EURORDIS**, the European Organisation for Rare Diseases, said, "There are few better examples of European co-operation and achievement than the EU Regulation on orphan medicinal products, adopted 15 years ago. Building on this momentum, more European collaboration is needed to improve access to therapies for patients."

He added, "Opportunities for the translation of scientific advances into new therapies are growing. Nevertheless, rare disease treatments are not being developed and accessed quickly enough. Scientific innovation cannot be disconnected from access to medicines. If an innovative medicine is approved but does not reach all of the patients who need it, it fails in its primary objective."

Today, European authorisation of an orphan medicine is given at **EU level**. But decisions on whether medicines should be paid for are made **nationally**. These assessments, disconnected from each other, do not produce a rational outcome. A huge lack of time, money and consistency can be overcome with a more collaborative approach at the EU level. The common objective should be more, better, cheaper treatments that reach the patient faster.

Through this symposium, EURORDIS and partners bring together relevant stakeholders to discuss the current state of play and how to shape a more effective way to address **value determination, appraisal, pricing** and **reimbursement** of orphan medicines, all with the aim of improving patients' access to rare disease therapies throughout Europe.

The symposium takes place following the 2015 EURORDIS and European Patients' Forum [call on the national authorities](#) responsible for medicines pricing and reimbursement within EU Member States to collaborate on medicines pricing at a European level.

Bringing together concrete contributions from, and by encouraging **open discussion** between, representatives from the various groups that are involved in the development of rare disease therapies, this event is the opportunity to establish a common understanding of the issues surrounding access to rare diseases therapies, an understanding that takes into account the perspectives of all stakeholders present at the event.

Following this open dialogue, the event will conclude with a set of proposals to be explored to improve patients' access to rare disease therapies.

The opening session of the symposium, including Commissioner Vytenis Andriukaitis' speech, is being web streamed live via [www.eurordis.org/rareeu2016](http://www.eurordis.org/rareeu2016). **The full event agenda is available [here](#).**

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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 more than 60 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](http://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.

## **Rare Disease Day**

Rare Disease Day was launched by [EURORDIS](#) and its Council of National Alliances in 2008. Held on the last day of February each year, it seeks to raise awareness of the impact that rare diseases have on the lives of patients and those who care for them. What began as a European event quickly became international in scope, with participants from more countries joining each year.

Since Rare Disease Day began, thousands of events have been held throughout the world, reaching hundreds of thousands of people. The political momentum resulting from the Day has also served advocacy purposes, contributing to the advancement of EU policies on rare diseases and to the creation of national plans for rare diseases in a number of EU Member States. Visit [RareDiseaseDay.org](http://RareDiseaseDay.org).

## **Press contacts**

*Eva Bearryman*  
Junior Communications Manager, EURORDIS  
Tel: +33 1 56 53 52 61  
[Eva.bearryman@eurordis.org](mailto:Eva.bearryman@eurordis.org)

*Lara Chappell*  
Communications Director, EURORDIS  
Tel: +33 1 56 53 52 60  
[lara.chappell@eurordis.org](mailto:lara.chappell@eurordis.org)