

**MULTI-STAKEHOLDER**  
**Symposium** ON IMPROVING  
**PATIENT ACCESS**  
24-25  
FEBRUARY 2016  
HOTEL LE PLAZA  
BRUSSELS  
TO RARE DISEASE  
**THERAPIES**



**Value Determination, Appraisal, Pricing & Reimbursement**

IN PARTNERSHIP WITH



A EURORDIS **RARE DISEASE DAY®** EVENT



**Conclusions Report**

## The Symposium

The **Multi-Stakeholder Symposium on Improving Patient Access to Rare Disease Therapies** aimed at addressing a crucial bottleneck in making orphan medicinal products (OMP) accessible across Europe.

A unique combination of **nearly 300 patient advocates, academics, policymakers, industry representatives, payers and HTA bodies** came together 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.

[Read the Symposium concept paper.](#)

By bringing together such a varied range of stakeholders, the Symposium created an opportunity for exchange to reach an understanding of the varied perspectives on issues and challenges surrounding patient access. Participants discussed various methods of appraisal, value determination and reimbursement and also took part in pricing simulation exercises.

Speakers included European Commissioner for Health Vytenis Andriukaitis and Members of the European Parliament Philippe De Backer and Françoise Grossetête.

## The context for developing orphan medicines

Opportunities for the translation of scientific advances into new therapies are accelerating, and it is now estimated that by 2020 30 to 50 new therapies will be approved each year. In parallel, the ambitious goal of the International Rare Diseases Research Consortium (to develop 200 new orphan medicines by 2020) is about to be reached and exceeded in 2016.

Yet this progress in science cannot be disconnected from **the issue of access**. If an innovative medicine is approved but does not reach all of the patients who need it in good time, something in the system is not right. Particularly at a time when one third of all rare disease patients in Europe today still do not have access to the approved medicines for their conditions, or when patients in smaller EU Member States may be left to wait up to 8 years from the time of market authorisation to eventually receive access.

The access conundrum can only be solved if its two main “drivers” are fully understood and recognised: the **science** on one hand, as the development of new orphan medicines needs to rest on a continuous stream of post-marketing authorisation evidence generation, strongly relying on more real-world data and patient-centric outcome measures; and the **economy** on the other hand, as a pre-requisite to patient access should be an affordable and sustainable economic model rewarding innovation and preserving the trust between payers and companies.

**Our common objective should be more, better, and cheaper treatments, accessible to patients faster. Over the two days, the Symposium set out to explore how to fulfil this goal.**

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## EURORDIS actions – paving the way for improved access to innovative medicines

In line with this Symposium, EURORDIS has been taking action to ensure that the issue of access to medicines is at the top of the agenda of European policymakers and national authorities:

- **In May 2015**, EURORDIS and the European Patients' Forum (EPF) [launched a call](#) on national pricing and reimbursement authorities to collaborate on medicines pricing and access schemes at a European level.
- **In May 2016**, EURORDIS and EPF wrote 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.
- **In June 2016**, European rare disease patients then [called](#) 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.
- **In June 2016 still**, EURORDIS contributed to the development of the EPF [“Core Principles from the Patients’ Perspective on the Value and Pricing of Innovative Medicines”](#).

Looking forward:

- EURORDIS shall continue to contribute to reflections currently under way in other settings, for example in a roundtable of experts convened by the international consultancy FIPRA, which is due to publish within the coming months consensus papers on *outcomes-based approaches to the pricing and reimbursement of innovative medicines*, and on *the use of real world data and data governance across an innovative medicine's lifecycle*.
- EURORDIS is also involved in the work of a separate multi-stakeholder working group led by health economist Lieven Annemans (University of Gent), looking at putting forward *consensus principles for the value assessment and funding of orphan medicinal products*.
- Finally, EURORDIS is continuing to work with its members to develop a new position statement on solutions to improve patient access to orphan medicinal products.

## Highlights from the opening plenary: Stakeholders' viewpoints on today's access issues

### The patient perspective

**Cees Smit (EGAN, The Netherlands)** spoke from his own experience as a haemophilia patient, highlighting how medical innovation helped in as little as 50 years of time to completely change the reality of living with this condition. However, this came with a warning not to suppress the “human factor” in any modern HTA discussion – an economic appraisal is not sufficient and must take into account aspects related to the patient's quality of life.

*“What we need now is a new and more constructive generation of HTA. A valuable contribution to current concerns over the prices of rare disease therapies could be delivered by involving physicians and patients in the process of price negotiations. Data show that this can lead to lower prices, especially when patients take part in negotiations.”*  
(C. Smit)

**Yann Le Cam (Chief Executive Officer, EURORDIS)** called on all 28 EU Member States to support emerging collaborations like that of Belgium, the Netherlands and Luxembourg. He reminded the audience that getting a new medicine faster to the patient is not only a necessity for the said patient – it is also a must if the right real-world evidence is to be collected that will in turn demonstrate what the actual value of the medicine is and which patients should receive it in priority.

More fundamentally, **it is time to realise that prices of innovative medicines are not univocally based on their value.** Instead, the price of a medicine as set by its manufacturer often reflects past and future investment choices, portfolio decisions, anticipations of a following indication or development, etc. If we want to restore trust, greater transparency is needed, but based on a more accurate understanding of the economics behind drug development and better grounded in the reality of companies that develop them today. Similarly, what matters to payers is more than the sole price – it is also the overall budget impact, potential positive spin-off consequences in their country (e.g. new production plants, centres of expertise, etc.), or still the possibility to “bundle” other products of a manufacturer in a wider negotiation.

### The payer perspective

**Jo De Cock (INAMI/RIZIV, Belgium)** offered the view from public authorities at the Member State level, and reminded all participants that the issue is not new – it was already at the core of the work of the Belgian Presidency of the EU in 2010. The recommendations formulated then have led to substantial progress over the years, e.g. in the field of joint health technology assessments, or in the field of coordinated access to OMP through a platform like [MoCA](#) (Mechanism of Coordinated Access to orphan medicinal products).

### The industry perspective

Speaking on behalf of the pharmaceutical industry, **Andrea Chiesi (Chiesi Farmaceutici, Italy)** insisted that the principle of sustainability is essential but must be achievable for every single player in the chain. Similarly, patients should increasingly be put at the centre even in the development of new medicines, and more properly listened to when it comes to the endpoints that matter to them, which may not always coincide with what regulations foresee.

## The policymaker perspective

Speaking about the value of a European approach, **Philippe De Backer (former Member of the European Parliament for Belgium)** underlined that much positive has been achieved to date e.g. in terms of building pan-European expert networks, improving the coordination between national centres of expertise, or still opening new regulatory possibilities such as conditional approval pathways and early access schemes.

Nonetheless, much remains to be done, particularly to address the persisting fragmentation of regulatory and access frameworks at the national level, which only results in delaying patients' access to medicines that have been approved. Now, he said, is the time for a true European HTA for new, innovative medicines and for a better and more widespread use of early dialogues and real-world data in decision-making.

*"The ones who are suffering today are the patients. Their health outcomes still depend on which country they were born in – but **this is not the promise that the EU should have for them!** It is our collective job as policymakers to fix this." (P. De Backer)*

In his [keynote address](#), **European Commissioner for Health Vytenis Andriukaitis** acknowledged recent progress in the cooperation between EU Member States, and the commitment of the Dutch Presidency of the EU to tackle head-on the issue of the prices of innovative medicines to the benefit of patients first.

He insisted on the need for current incentives for the development of OMP to be applied solely with a view to encouraging the emergence of valuable therapies – not the maximisation of individual companies' profits – and indicated that the European Commission started to streamline the regulatory framework for orphan medicines by reviewing certain regulatory frameworks for OMP.

*"I welcome the EURORDIS/EPF call to create a negotiating table on pricing to allow for enhanced cooperation at the EU level between industry and Member States. **This is very timely. I am ready to part of this and to create a multi-sectorial table inviting all actors, in order to see how to progress now.**" (V. Andriukaitis)*

The Commissioner stressed how decisions on Health Technology Assessment and pricing and reimbursement decisions directly impact patient access. Therefore the European Commission supports Member States collaboration –within the limits of its competence- and furthers its engagement with them on pricing, early patient access to treatments (through the Commission's STAMP Expert Group). HTA is another example of an area where increased cooperation has the potential to reduce fragmentation, and Member States were called upon to fully support the new Joint Action starting this year. The hope is to have by 2019 a more structured network in place and greater traction for the practical use by Member States in real life of European common assessments.

In her closing keynote address on day 2 of the Symposium, **Françoise Grossetête (Member of the European Parliament for France)**, herself a pioneer of the EU legislation on OMP, offered reasons to hope. The European level is the right playing field on which the current economic and political challenges to access can be discussed and find their solution. Only by finding greater room for cooperation between Member States shall the deadlock be broken, to ensure that medical advances in the fight against rare diseases can be properly rewarded in a financially sustainable and innovation-friendly way.

## The way forward: steps towards breaking the deadlock

In focusing on how to shape a more effective way to address value determination, appraisal, pricing and reimbursement of orphan medicines, the following points were discussed at the Symposium:

### How to determine value?

- **Social values and preferences could, and should, be much better integrated** into decisions on the reimbursement of and access to orphan medicines, although such preferences and their resulting trade-offs can vary from one country or constituency to another. Pilot projects currently under way (e.g. in Switzerland) may deliver useful insights in upcoming months.
- A common understanding of which factors must be unequivocally considered to determine the value of orphan medicines should be developed at the EU level. Rarity in itself does not suffice to demonstrate the value of a new medicine. The cost of treating patients should be more consistently compared to the cost of not treating them. Major principles such as equitability of access or equal quality of care should not be discarded in any decision on value. Patient involvement is essential and should intervene as far upstream in the process as possible.
- The mix of high uncertainty at marketing authorisation, lack of knowledge about the medicine/disease, and fragmentation of Member States' systems leads to an overly resource-intensive situation for healthcare systems and authorities – with patients often left unsure whether a product will be reimbursed or not. A system under which immediate access could be granted from the time of marketing authorisation, even based on forecasts of budget impact, could be welcome. Such a system needs to be backed up with a commitment to monitor how a given medicine performs in real-world practice, so as to build sufficient data and start reducing the original uncertainty.
- Quality tools, models and frameworks exist that can support the determination of value, but what matters is the political will to adhere to these recommendations.

### From value to appraisal

**Prof. Panos Kanavos (London School of Economics, UK)** compared how 3 national HTA agencies (Scotland's SMC, the Netherlands' ZIN, and the UK's NICE) assess advanced therapies and OMP, and searched for what could be done in a more harmonised way:

- There is a need for a better understanding of when innovative therapies must be prescribed and reimbursed, and which patient populations can really benefit from them.
- Scarcity of evidence at initial review is not seen as an insurmountable obstacle to appraisal.
- Existing frameworks enable patients, clinicians and other experts (e.g. ethicists) to take part in the decision-making, and helps capture information that could otherwise not slip through more "regular" HTA analysis.
- Patient-reported outcomes measures (PROMs) are very important and companies are encouraged to develop them much more from clinical trials onwards.
- European collaboration is not an option – it will increasingly be a requisite, as doing 28 times the same thing is neither feasible nor sustainable.

*"We work hard to find specialists across England to better understand the disease, the pathway and where the product fits in it. **Patients' participation allows us to bring the disease to life, so that a stronger real-world component is added to the overall picture.**"*  
(S. Upadhyaya, NICE, UK)

## From appraisal to pricing

For this last part of the symposium, **Charles Barker (CMI Concord Group)** introduced participants to good practices of collaborative negotiations. The breakout sessions that followed were all built around an interactive simulation exercise on the pricing of a new pharmaceutical product. The aim was to open the eyes of the participants to the factors that companies take into account when setting the price of a new medicine, and those that condition payers' pricing and reimbursement decisions.

Some of the highlights from the overall feedback received from the participants:

- The perception of the value of a medicine is not a given valid at all times. It does evolve sometimes even radically as more information (particularly patient data) becomes available and as the decision becomes more complex.
- Such processes are not easy, and value can be appraised in very divergent manners unless a founding principle (e.g. patient access as the ultimate goal) is used as a benchmark.
- Health budgets are tight and a reasonable limit must be put on prices, otherwise access (incl. to other components of healthcare) may have to be restricted or even halted.
- It is reasonable for a payer to seek information on how a company wants to develop a new medicine and to expect fair transparency in return.

## Concluding thoughts

The closing panel, moderated by **Ri de Ridder (INAMI/RIZIV, Belgium)**, agreed that **the time for action is now, and there is clearly a political will to build a new system for tomorrow.**

The symposium helped to start identifying concrete, practical ideas and solutions towards a more sustainable future. Pricing decisions must be taken with a holistic, long-term mind-set as they inevitably impact not only the present, but also the decisions that all stakeholders will make tomorrow. Incentives for future innovation must not be forgotten.

All throughout the symposium, a number of ideas were repeatedly discussed amongst participants, ranging from adaptive pathways (or how the value of a new, transformational medicine can be demonstrated over time) to the notion of a pan-European fund to collectively "subsidise" the introduction to the market of new, potentially expensive health technologies with a high level of uncertainty.

The 'new model' emerging at the end of the Symposium could tentatively rely on: (1) a more generalised practice of early dialogue and (2) an earlier market entry, based on a lower entry price and a defined period of time during which post-marketing authorisation evidence generation could help to reduce the uncertainty commonly associated with many new orphan medicines.



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