Value Determination, Appraisal, Pricing & Reimbursement

CONCEPT PAPER

Setting the context

There are few better examples of European co-operation and achievement than the EU Regulation on orphan medicinal products, adopted 15 years ago.

Tangible benefits have been delivered to patients and there has been international investment in uncharted scientific and medical areas. This has triggered innovation in life sciences and the creation of high-skill jobs, while addressing public health needs.

Building on this momentum, more European collaboration is needed to improve access to therapies for patients. The EU has granted 116 rare disease therapies with marketing authorisation and 1,605 orphan products in development for diseases that have no effective treatment.

EURORDIS estimates that a third of patients have no access to the necessary orphan medicine; another third have access only after waiting years, as medicines are introduced first in main markets and later in others. More recently, some important medicines are not being made available because they are perceived to be too highly priced in comparison to the determined value.

Why this symposium now?

A EURORDIS Rare Disease Day 2016 event, the **Multi-stakeholder Symposium on Improving Patient Access to Rare Disease Therapies** has been organised to address a crucial bottleneck in making orphan medicinal products accessible across Europe.

The International Rare Diseases Research Consortium, launched in 2011, set the objective of delivering 200 new rare disease therapies by 2020 (compared to 2010). This will be met by 2016. 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 the patients who need it, it fails in its primary objective.

To address the access challenge, two drivers need to be recognised by all stakeholders: the scientific and the economic. All parties need to recognise that the scientific development of rare disease therapies is a continuum of evidence generation all along the life cycle of the medicine, particularly in the years following marketing authorisation. This continuum should be inclusive of more real-world data and more patient-centred outcomes measures. This will help to treat the right patients with the right protocol, regimen and dose, at the right time, ensuring better medicines are available.

All parties also need to recognise that patient access requires both an affordable and sustainable economic model rewarding innovation, one that increases trust between payers and industry, and to some extent all other parties.

Member States' decision-making processes to provide access to medicines should be based first on therapeutic value and second on value for money. For rare diseases, **this is only possible if these processes are supported by European collaboration mechanisms** that engage all those involved along the life cycle of medicines.

Today, European authorisation of an orphan medicine is given at **EU level**. One of the main incentives of the EU Regulation is market exclusivity. But the European market does not truly exist, it is fragmented and 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**.

Decisions on reimbursement of a medicine should remain with national authorities, but there should be pan-European co-operation. **Patients are calling for a smarter Europe**; we urgently need a seamless approach that can bridge the gap between EU regulation and fragmented health technology assessment, local pricing and reimbursement decisions.

In 2015, EURORDIS and the European Patients' Forum (EPF) launched a call on the national authorities responsible for medicines pricing and reimbursement within EU Member States to collaborate on medicines pricing at a European level, all with the aim of improving patient access to medicines.

Please find the full *Call to Payers* document here.

Two issues need to be addressed as priority. Firstly, there is growing scepticism from national competent authorities (HTA and payers) on the value of some orphan medicines. This is because of the high level of uncertainties surrounding an orphan medicine at the time of marketing authorisation, and also because the value of the medicine is sometimes still not proven after years on the market. The second issue to be addressed is that of medicines that are perceived to be too highly priced per patient for the assessed value and/ or the high impact of orphan medicines on health budgets.

Issues preventing patients' access to rare disease therapies can only be addressed if we recognise the two drivers: science *and* economy. If all stakeholders involved join forces to ensure a structured EU approach to access, the EU can become the best place in the world to develop rare disease therapies.

Our common objective should be **more, better, cheaper treatments accessible to patients faster.**

Key mechanisms supported by the EU Commission and Member States, such as the EMA's parallel scientific advice with health-technology-assessment bodies, the Shaping European Early Dialogues for health technologies project (SEED) and EUnetHTA early dialogues, should be strengthened to help overcome specific issues surrounding rare disease therapies and to favour evidence generation all along the life cycle of a product development. This will in turn give more predictability to the entire process of medicines development.

Symposium organisation & objectives

Through this symposium, EURORDIS and partners are bringing together relevant stakeholders (industry, HTA bodies, regulators, payers and patient advocates) 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 overarching aim of this symposium is to gain common understanding and agreement on the determinants of value and of the assessment and appraisal methods for orphan medicinal products by:

- Bringing all participants to a common understanding of issues/challenges of access to rare disease therapies
- Encouraging open discussion on the day between the various participating stakeholders to exchange on how they each appraise, determine value, price and reimburse
- Reaching an understanding of the varied perspectives of all participating stakeholder groups on issues/ challenges surrounding patient access
- Establishing how these challenges should be addressed and if/how different approaches of these various stakeholders may need to be adapted to better serve the end goal of improving patient access to rare disease therapies
- Sharing existing and new pricing, reimbursement and access processes to recognise which ones have larger potential
- Hearing how HTA agencies assess advanced therapies and medicines for rare diseases
- Understanding regulatory initiatives designed to address the gap between authorisation and access
- Being challenged to evaluate and price orphan medicinal products from different stakeholders' perspectives (via on-site simulation exercises)
- In view of a second symposium in 2017, agreeing on a structured approach and a set of solutions to explore.

The symposium is divided into 4 sessions (*see attached agenda for detail of speakers*):

1. **Value determination (including breakout sessions)**
2. **From value to appraisal**
3. **From appraisal to pricing: simulation exercises in breakout sessions**
4. **Pricing & reimbursement, conclusions and next steps**

A Programme Committee made up of representatives of industry, patient representatives, concerned EU entities, national payers and EURORDIS has been working since August 2015 to organise this symposium.

Programme Committee members:

- Lieven Annemans, Professor of Health Economics, Gent University, Belgium
- Ri De Ridder, Director-General of Healthcare, RIZIV-INAMI, Belgium
- Karen Facey, Evidence Based Health Policy Consultant, Glasgow University, UK
- Ruediger Gatermann, Director, Healthcare Policy and External Affairs Europe, CSL Behring, Germany
- Josie Godfrey, Public Policy and External Affairs, Sobi - Swedish Orphan Biovitrum AB, UK
- Laura Gutierrez, Senior Director European Government Relations and Public Policy, Celgene, Belgium
- Adam Heathfield, Co-chair EFPIA - EuropaBio Task Force, Senior Director, International Policy, Pfizer, UK

- Christian Hill, Managing Director, Map Biopharma, UK
- Virginie Hivert, Therapeutic Development Director, EURORDIS, France
- Yann Le Cam, Chief Executive Officer, EURORDIS, Europe
- Vinciane Pirard, Co-chair EFPIA - EuropaBio Task Force, Director Public Affairs, Genzyme Europe BV, Belgium

Advisors to the Programme Committee:

- François Houÿez, Treatment Information and Access Director / Health Policy Advisor, EURORDIS, France
- Kristina Larsson, Head of Office – Orphan Medicinal Products, European Medicines Agency (EMA), UK
- Solange Rohou, ADAPT SMART consortium (EMA), Director Regulatory Affairs, AstraZeneca, France
- Ad Schuurman, Head of the Business Contact Centre & International Affairs, National Health Care Institute (former CVZ), The Netherlands
- Cees Smit, Patient advocate, European Genetic Alliances Network (EGAN), The Netherlands
- Chris Sotirelis, Patient advocate, UK Thalassaemia Society, UK

The opening session of the symposium, including a speech given by Vytenis Andriukaitis, European Commissioner for Health & Food Safety, is being web streamed live via www.eurordis.org/rareeu2016.

Registered participants were offered the chance to prepare for the symposium by participating in this webinar: An Introduction to HTA

Symposium outcomes

- In view of a second symposium in 2017, agreeing on a structured approach forward and a set of solutions to explore
- These suggested solutions will be brought together in an article to be submitted for publication the HTAi Society's journal and other scientific journals, as well as the Orphanet Journal of Rare Diseases.