



Third multi-stakeholder symposium

Session 3

A transparent European
cooperation framework for the
determination of fair prices and of
sustainable healthcare budget
impacts

Speakers

- Lieven Annemans, Senior Professor of Health Economics at the Faculty of Medicine at Ghent University
- Yann Le Cam, EURORDIS Chief Executive Officer
- Angela McFarlane, Senior Market Development Director for IQVIA
- Alexander Natz, Secretary General of the European Confederation of Pharmaceutical Entrepreneurs
- Valerie Paris, OECD Secretariat
- **Health Insurance/Payer**
 - Rapporteur: Simone Boselli, EURORDIS' European and International Advocacy team
 - Moderator: Anna Bucsics, Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA)

The Current Situation

- While orphan medicinal products (OMPs) are authorized EU-wide, access and reimbursement decisions are made at the national or even regional level.
- Health Technology Assessment at the European level is the exception, not the rule.
- The legal, procedural, structural and economic frameworks for assessment, appraisal, decision-making and delivery differ widely.
- There is wide variety in access to OMPs in Europe (As is the case for many high-priced medicines).

What is Working?

- Support for rare diseases at the European level (e.g. through European Reference Networks)
- The current orphan drug regulation is a success story: more and more companies are involved in OMPs, more new medicines are being developed.
- There is an awareness of (and accommodation for) the special situation of OMPs with regard to the evidence base – even when this is not reflected in the formal procedures for reimbursement decision-making.
- Patient awareness and advocacy: awareness of the importance of patient-reported outcomes in drug development is growing.
- Awareness of the need and willingness by (many) European Member States to cooperate

What Needs to be Improved?

- Equal access across member states
- Routine Europe-wide HTA of OMPs
- Europe-wide joint price negotiations
- Acceptability of prices of newer OMPs – these are sometimes so high that OMPs are either rejected or payers feel that the current framework is being abused by companies
- Do we have as many generics for “ex-orphans” as we expected?
- Is the current orphan regulation still aligned with its original policy goals?

Questions to Discuss

What should a more cooperative framework at the European level look like?

- Can collaborative and voluntary experiences such as BeNELuxAI be scaled up and made sustainable?
- Could we negotiate a fair price at the EU level, based for example on experience such as MoCA?
- Is it possible to move to a European table of negotiation?

What is MoCA?

It is a

- voluntary
- non-legislative,
- non-regulatory and
- non-binding collaboration
- among stakeholders who are willing to work together to provide real access to a real solution for real patients with real unmet medical needs

Since 2014, MoCA has discussed 19 projects with 16 companies/consortia

MoCA discussions are possible before, during and after marketing authorisation



Mechanism of Coordinated Access
to Orphan Medicinal Products

<http://www.eurordis.org/content/moca>

The Transparent Value Framework for Multi-Stakeholder Consensus

Criterion	Lower Degree	Medium Degree	High Degree
Lack of Alternatives/Unmet Need, including non-pharmaceutical treatment options	yes, new medicine does not address unmet need	yes, but major unmet need still remains	no alternatives except best supportive care - new drug addresses major unmet need
(Relative) Effectiveness, Degree of Net Benefit (Clinical Improvement, QoL, etc. vs. side effects) relative to alternatives, including no treatment, societal impact, etc.	incremental	major	curative
Response Rate (based on best available clinically relevant criteria)	<30%	30-60%	>60%
Degree of Certainty (Documentation)	promising but not well-documented	plausible	unequivocal

New orphan medicinal products could be assessed according to how well they fulfilled the different criteria at a given point in time. This could be compared with other therapeutic alternatives and be included as one factor in pricing negotiations in Member States

How can we progress pricing negotiations at the European level?

- Are outcomes based managed entry agreements a way forward?
- Is a collaborative approach possible with this regard?