Third multi-stakeholder symposium

Session 3

A transparent European cooperation framework for the determination of fair prices and of sustainable healthcare budget impacts
The Current Situation – what are we here to reflect upon?

• 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 (HTA) 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 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?

- Is the Orphan Regulation delivering on its original policy goals?
- Equal access across EU Member States
- Routine Europe-wide HTA of OMPs...
- Europe-wide joint price negotiations...
- Acceptability of prices of newer OMPs – these are sometimes high:
  - OMPs may be rejected
  - Payers may perceive that the current framework is being “abused” by companies
- ?? What tools exist already that we might build on ??
Transparent, European cooperation frameworks?

- Multi-country collaborations:
  - BeNeLux-A-I
  - Valetta
  - Visegrad – “Fair & Affordable Pricing” (FAAP)
  - Finose
- MoCA – Mechanism of Coordinated Access
Studied cross-country collaborations

Source:
WHO Research Study on Impacts & Benefits of Cross-Border Collaboration in WHO European Region
Sabine Vogler, Fatima Suleman
WHO Regional Office for Europe
Infarmed Conference, 29-30 November 2018

Comments:
Ireland is part of two collaborations, the Beneluxa initiative and the Valetta Declaration
Lithuania is part of two collaborations, the Baltic Procurement initiative and Visegrad

Source: Data collection of the WHO Collaborating Centre for Pharmaceutical Pricing and Reimbursement Policies, Vienna, and the WHO Collaborating Centre for Pharmaceutical Policy and Regulation, Utrecht.
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 / programmes with 16 companies / consortia

MoCA discussions are possible before, during and after marketing authorisation
### The Transparent Value Framework for Multi-Stakeholder Consensus

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

- Could any of these be the starting seed for a potential voluntary round table of negotiation ...?
Panellists

- Angela McFarlane, Senior Market Development Director, IQVIA
- Yann Le Cam, Chief Executive Officer, EURORDIS
- Alexander Natz, Secretary General of the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE)
- Valérie Paris, Senior Economist, OECD

- **Rapporteur**: Simone Boselli, EURORDIS’ European and International Advocacy team

- **Moderator**: Wills Hughes-Wilson, Steering Group, Mechanism of Coordinated Access to Orphan Medicinal Products (MoCA)
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 voluntary European table of negotiation?
How can we progress pricing negotiations at the European level?

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