Third multi-stakeholder symposium

Session 3

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

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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

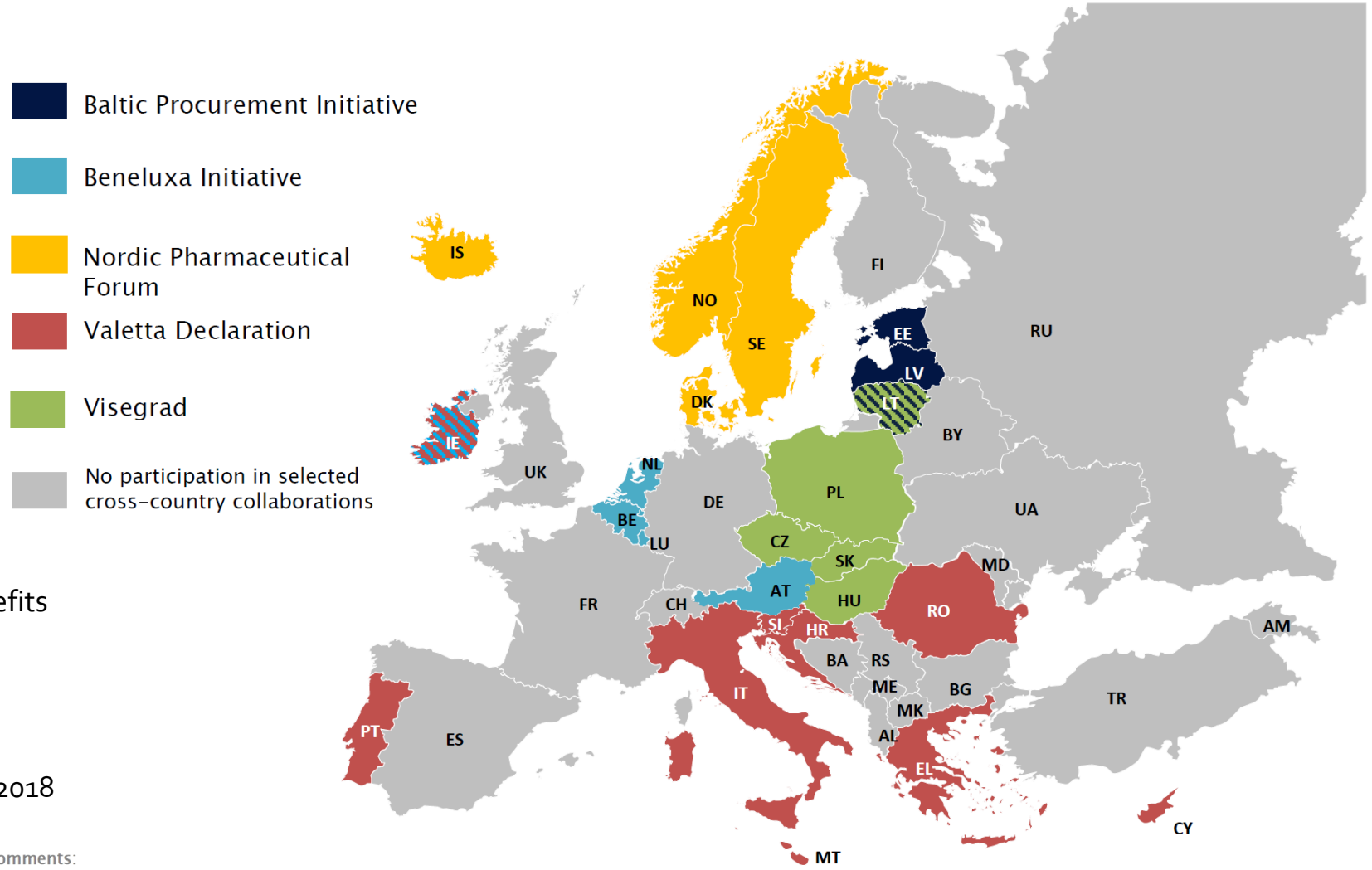
What Needs to be Improved?

- 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?

http://www.eurordis.org/content/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

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

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

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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?