Executive Summary

2nd Multi-Stakeholder Symposium Proceedings

The Symposium

The goal of the Symposium was to bring together stakeholders playing a key role in getting medicines and therapies to rare disease patients, and to initiate a dialogue and cooperative process that respects the interests of all parties and that will lead to solutions for improving patients' access to rare disease therapies.

This 2nd edition of the Symposium culminated with the formation of a new multi-stakeholder group that will a draft a 'One-Text' Plan of Action for all parties to collaborate on access to rare disease therapies.

The method for collaboration

The 'One-Text' Process is a method developed by PrimeMover Associates and is a way in which negotiators and mediators manage complex subjects with numerous stakeholders who hold conflicting views and exercise different levels of authority through the use the following 'Seven Elements': interests, options, commitment, legitimacy, communication, relationship and alternatives.

The process always begins by the clarification of interests and is followed by the exploration of the maximum numbers of possible options. The notion of fairness/legitimacy is consistently reflected upon throughout the process.

Following the Symposium, the designated committee of drafters now has the task of elaborating a first One-Text with all the options discussed and present it for consultation to a group of commentators following a continuous cycle of redrafting & consultation until the draft cannot be improved further and a 'yes/no' choice needs to be made.

Interests of pharmaceutical companies

- Predictability
- Price
- Keeping innovation moving forward
- Creating value for patients
- Partnering with patient groups
- Innovative reimbursement and regulatory solutions
- Better education of both investors and the public

Interests of payer and HTA bodies

- Robust evidence of medical benefit
- Early dialogue between the industry and the payer/HTA body
- Patient engagement
- More visibility on price-setting
- Sustainability of health care system
- Meeting high unmet medical need

Existing collaboration initiatives & success factors

A number of existing initiatives were presented during the Symposium:

- PRIME
- Adaptive Pathways.
- Patient engagement in clinical trial design
- EUnetHTA
- Mechanisms of Coordinated Access to Orphan Medicinal Products (MoCA)

The success factors identified included:

- Urgency to have new products approved as basis of initiative
- Patient involvement in early dialogue
- Patient-safe harbours
- Involvement of patients in decision-making within the healthcare system.
- Providing training and support for patient engagement.
- Multi-criteria decisions analysis being used.
- Common HTA methodology across EU.
- Moving from value-based pricing to value-informed pricing.

Three themes for improving patient access to rare disease therapies

The second day of the Symposium focused on exploring in more depth *how* collaboration can be achieved. The structure of discussions followed three broad themes: Quality Data Generation; Value for Money across Europe; and Outcomes. During different breakout sessions, initiatives that have already been pilot-tested or that have been conceptualised were discussed. Challenges and opportunities were explored for each theme.

Quality Data Generation

The breakout sessions for this theme explored both registries and the European Reference Networks (ERNs).

Challenges

- Time
- Expert data management
- High-quality data
- Fragmentation
- Interoperability challenges
- Data validation
- Data ownership/custodianship
- Budget constraints and long-term sustainability

Opportunities

- Patient engagement as trust-builders and catalysers of collaboration
- Training of patients and professionals on FAIR data-entry
- Cooperation at EU and international level
- Reducing time to diagnosis
- ERNs having an inherent mark of quality
- Engagement with industry for the generation of post-authorisation data and for quicker access.

Value for money across Europe

The first breakout session for this theme explored the Recommendations from the European Working Group for Value Assessment and Funding Processes in Rare Diseases (ORPH-VAL) which acknowledge that value of Orphan Medicinal Products (OMPs) can be measured at the patient, healthcare system and societal level, and a series of principles can help improve consistency of assessments. The 2nd breakout session for this theme looked into the proposals for coordination of HTA across Europe, concretely EUnetHTA, which consider cooperation at the scientific & technical level

and at the strategic level between Member States.

Challenges

- Valid evidence gathering for OMP assessment.
- Fragmentation: between and within countries (in assessment criteria and societal preferences).
- Differences in the level of patient engagement.
- Low uptake of current proposals for alignment and cooperation.
- Sustainability: lack of funding and technical implications of support by industry.

Opportunities

- Trust-building and will created by cooperation.
- Pooling of high-quality evidence
- Early and continuous dialogue on acceptable evidence.
- A broader definition of value can help overcome evidence gathering issues.
- A common base of relevant elements of product value that still allows for different national prioritisation can increase uptake.
- More structured patient involvement for increasing real-world patient experience.

Outcomes

The first breakout session for this theme explored innovative performance-based outcomes agreements, looking in particular at Managed Entry Agreements (MEAs), agreements between the manufacturer and the payer, where each stakeholder agrees upon a certain degree of risk sharing and how to assess the added-value of a new product. The second breakout session consisted of a debate on the potential for collaboration among payers and companies. The main focus of the discussion turned towards the commonly called 'BeneluxA' initiative, a joint negotiation project by Belgium, The Netherlands, Luxembourg and Austria.

Challenges

- Defining patient-relevant outcomes: patient-reported outcomes or measures of the quality of life?
- Choosing surrogate end-points.
- Timing: selecting a timeline for agreement between payers and industry is challenging.
- Data collection and sharing: issues of data capture, quality, validation & interoperability.
- Ethical balance between data collection and burden on patients' everyday life.
- Fragmentation: differences in national regulations and regionalisation.

Opportunities

- Openness and trust built through cooperation allows for flexibility.
- Patient engagement on definition of relevant outcomes.
- Combine the opportunities offered by various initiatives (ERNs & EUnetHTA) into an integrated system.
- Creation of a common ecosystem for industry.

This last theme raised a great number of points of contention that are left as open questions in this report:

- Sustainability of health system.
- The complexity of the environment pharma companies work in
- Early agreement of level of evidence gathering.
- Elucidating societal preferences.
- Differences in countries' ability to pay.
- Transparency of pricing.
- Harmonising price.
- Incentives.
- Price-cutting.

Conclusions & opportunities

Challenges

- **Time:** different timelines of all stakeholders.
- Long-term **sustainability**: of health systems and of collaboration initiatives.
- Ensuring **high-quality and patient-relevant data**
- **Fragmentation:** in national/regional health and innovation policies and in standards of care and research
- **Duplication:** of processes and of collaboration initiatives
- Differences in **patient engagement:** from country to country and between initiatives
- **Low Member State uptake** in collaboration initiatives
- **Ethical balance:** in conflict of interests, data collection and data sharing
- Need of a **new ecosystem for payer-industry relations**

Opportunities

- **Patients and patient groups** can act as **catalysers of trust and collaborators**
- Training for patients and professionals on **FAIR (Findable, Accessible, Interoperable and Reusable) data-entry**
- **ERNs** offer an unprecedented chance to **reduce time to diagnosis**
- Creation of **Pan-European registries**
- Consolidating a **common base of elements of product value**
- **HTA collaboration** can avoid duplication and scale up evidence
- Exploring existing intiatives (i.e. ERNs & EUnetHTA) as part of **integrated system**
- Move forward with **public-private partnerships**