Eurordis Symposium: *Let's make a pact to ensure Patients’ sustainable access to rare disease therapies*, February 13-14, 2019

**BREAKOUT SESSION 4**

**Moderator:** Prof. Eileen Treacy, MD, FRCPI, FRCPC, FCCMG

**Speakers:**

Dr. Thomas Morel, Director Patient-Centred Outcomes Research & Policy, UCB

*How to involve multiple stakeholders in data sharing and ways to facilitate this collaboration?*

*What are the realistic expectations of risk-sharing reimbursement models?*

Dr. Diego Ardigo, R&D Rare Diseases Unit Head, Chiesi Pharmaceuticals, IRDiRC

*A continuum approach to evidence generation in post-approval phase linked to healthcare budget spending*

Josie Godfrey, Director, Zebra Consulting

*Duchenne Muscular Dystrophy: Project HERCULES*
BREAKOUT SESSION 4

• Uncertainty and healthcare decision-makers
• How does observed treatment effect translate into patient benefit?
• A need for ‘interpretable’ and ‘unequivocal’ measure of patient benefit
• IRDiRC: Patient-Centred Outcomes Measures in RD: ‘a necessity’
  • **A case study**: Myasthenia Gravis

How to achieve truly Patient-Centered Outcome Measurement

Five core concepts around PCOMs:

*Collaboration, Alignment, Communication, Innovation, Integration.*
BREAKOUT SESSION 4

• Involve patients from the start in defining the health outcomes that matter and ought to measured.

• Assistance from EU funds to support infrastructure to measure PCOMs gathering and measurement.

• Multi stakeholder consultation process: bringing together EMA, HTA, Payers, ERNs.

• What matters to patients are holistic outcomes.

• Why PROs currently are not integrated or poorly considered and valued within HTAs?
BREAKOUT SESSION 4

SUSTAINABILITY : Technical & Economic

Needs for the pathway of development of an RD product compared to traditional indications.

- **Continuous evidence generation** (Registries integrated with Healthcare)
- **Efficient use and available tools** (IRDIRC tool box)
- **Common definition** of value based on common needs. PROMs on thematic areas of commonalities – a cross sectional approach needed.
- Ways to determine the value and manage **“value uncertainty”**
- Paying for **Real performance**
- Dedicate **funds to continue risk sharing** during evidence generation phase.
BREAKOUT SESSION 4

Data sharing platform – how can this be organised by a multidisciplinary approach.

• Data **owned by patients** and safeguarded into a single platform.

• Different rules for accessing data by various stakeholders according to purpose and needs? EMA and ERN roles to be promoted.

• Crucial to ensure data quality – GCP compliance, auditable, integrated with PhV system.
Duchenne Muscular Dystrophy: Project HERCULES

A successful model of integration of academic centres, patients, multiple industry partners to share and build data and improve outcomes.

Interaction between timelines for Translarna and for DMD disease progression.

Unique multi-stakeholder collaboration – Achievements:

- To allow pharmaceutical companies, charities, academics, patient organisations and experts to work together to build the evidence base for DMD HTAs.
- To generate, align and share high quality disease-level evidence across an entire condition TOWARDS more transparent and consistent reimbursement decisions.

- The Project Hercules Steering Group description.
- What were the HERCULES Deliverables
- Key Project Hercules events/milestones 2019