Eurordis Symposium: *Lets 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

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- 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 trully Patient-Centered Outcome Measurement

Five core concepts around PCOMs :

Collaboration, Alignment, Communication, Innovation, Integration.

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- 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 ?

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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.

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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.

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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