

# 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**  
**National Clinical Programme for Rare Diseases, Ireland**

# Lets make a pact to ensure Patients' sustainable access to rare disease therapies

## Pillar 4

**A continuum of evidence generation linked to healthcare budget spending**

**'The reduction of uncertainties is an essential need, not only for national healthcare systems but also – and to a not less important extent for patients and clinicians'**

## Eurordis Ambitions

- **3-5 times more RD therapies approved per year**
- **3-5 times cheaper than now by 2025**
- **The right therapeutic option for the patient, not the 'disease'**
- **Decrease inequities to access across Member States**

## Session 4 Aims:

# To address some of the challenges and barriers to approval for access and reimbursement of Orphan Medicinal Products in the post-approval phase

How can we measure the required meaningful health outcomes for patients to enable informed reimbursement decisions?

- What can be the role of Patient Centred Outcomes Measures (PCOMs) (or PROMs)?
- **Speaker:** 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?
- **Speakers:** Dr. Diego Arduci, R&D Rare Diseases Unit Head, Chiesi Pharmaceuticals, IRDiRC
- Josie Godfrey, Director Zebra Consulting ('Hercules', DMD-UK)

# IRDiRC Vision 2018 (Austin C et al, Clin Transl Sci, Jan 2018)



1. All patients with a suspected rare disease will be diagnosed within 1 year if disorder is known in the medical literature
  - Undiagnosed individuals will enter a globally coordinated diagnostic and research pipeline
2. 1000 new therapies for rare diseases will be approved
3. Methodologies will be developed to assess the impact of diagnoses and therapies on rare disease patients
  - Measure access, impact, effectiveness in real world settings

To see that these *new efficacious therapies lead to 'real benefits' for patients*

Highlights need for INFRASTRUCTURE:

- Clinical Practice Guidelines, Outcome Registries and CDEs, Regulatory Policies, Education

# What matters to Patients

## Patient Reported Outcomes

### WHO Definition of Health

**"State of complete physical, mental, and social well being, and not merely the absence of disease or infirmity."**



**"You have to learn about thousands of diseases, but I only have to focus on what's wrong with ME! Now which one of us do you think is the expert?"**

# EURORDIS Position Paper 2011

Patient Priorities for Rare Diseases Research, 2014-2020

**Most urgent priority: *Translating research into therapies***

**Barriers:**

**Lack of basic knowledge of the causes of rare diseases**

**Knowing what outcomes to study**

**Finding the rare patients**

**Determinants of success in Rare Disease Research:**

- **Patient Organisations**
- **Patient Registries**
- **International Networks-European Reference Networks**



# Path to Reimbursement and Equitable Access to OMPs



Low statistical power from small studies and few patients  
Endpoints: Surrogate Biomarkers vs. Clinical Relevance  
Unknown long term experience  
Genetic heterogeneity  
Inadequate Registries (Bouvy et al, 2017)



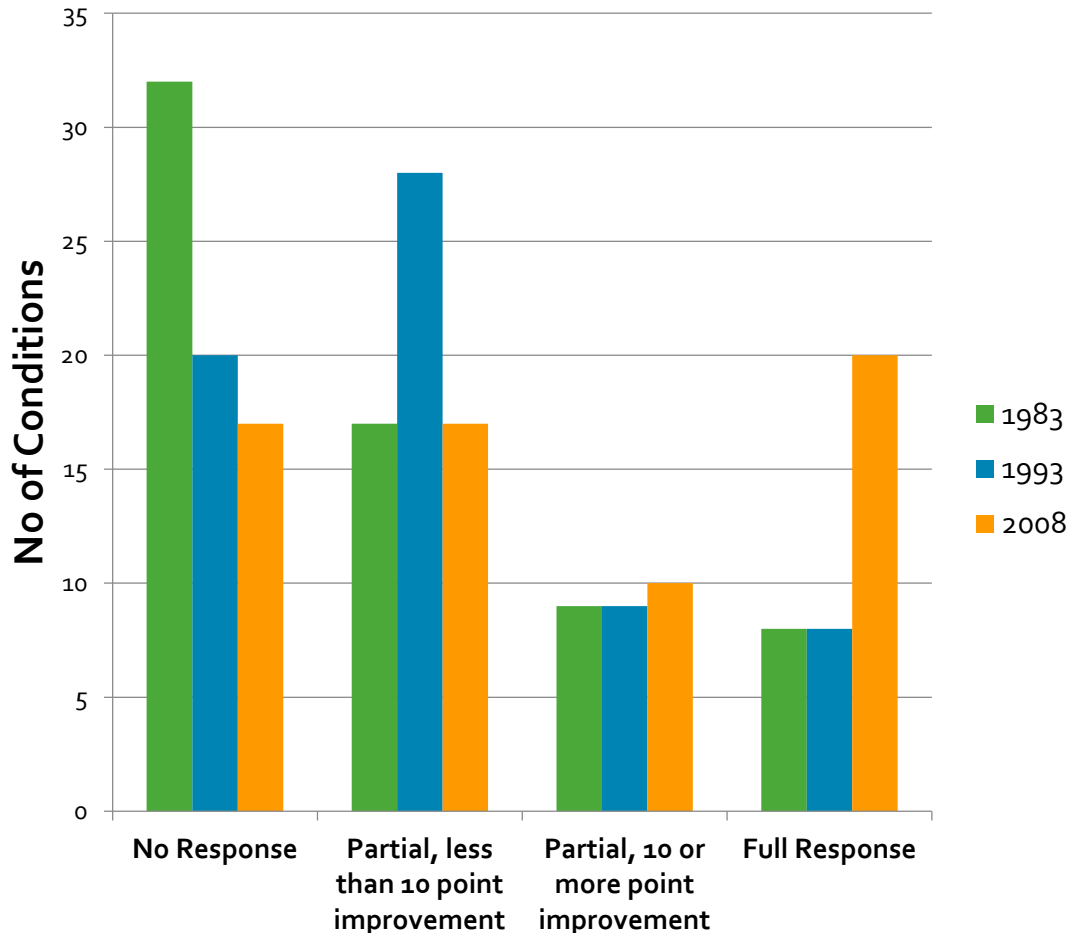
Research required to generate evidence and compare effectiveness  
IRDiRC 'Building Blocks'  
RD professionals and patient participation in OMP assessment



Outcome Registries, PROs and Core Datasets (CDEs)  
Risk-sharing schemes during data collection  
EU-wide discussions on the balance of long-term effectiveness vs price

# 25 year Response to Treatment of Rare IMDs

Campeau et al 2008; Treacy et al 1999;  
Treacy et al 1995; Hayes et al 1985



- Effect on biological selection
- Longevity
- Reproductive capability
- Effect on development
- Somatic growth
- Intellectual development
- Effect on social adaptation
- Learning handicap
- Work handicap
- Cosmetic impairment



# Progress to date-(Eurordis 2018 Position Paper)

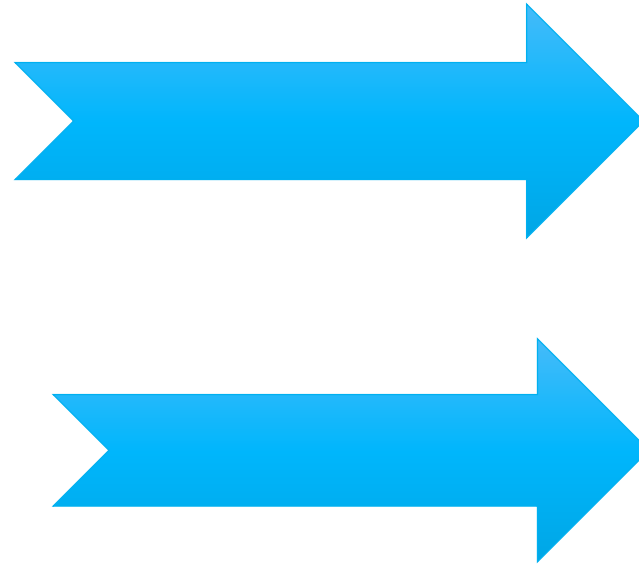
## Challenges

- Long-term sustainability of national health funding systems-cost of developing OMPs
- Differences in patient engagement and systems across MS
- Small patient numbers, statistical power of studies - genetic heterogeneity
- Accessing and sharing 'fit for purpose data', Conflict of Interest, IP
- Need for new ecosystem for payer-industry relations

## Opportunities

- European Joint Programme on Rare Diseases and 'FAIR'
- Involvement of Patient Advocacy Groups to develop patient orientated evidence (PROMs,PCOMs)
- European Reference Networks-opportunity to create outcome registries (e.g. ERKNet, Haemophilia, Cystic Fibrosis, CTSR, GalNet)
- 'STAMP', EMA Registry initiative and cross-European cooperation for value assessments
- IRDiRC Toolbox
- Public-Private partnerships

**Thank  
you for  
your  
attention**



**Breakout  
Session 4**