

# Measuring what matters to patients

Thomas Morel

**Eurordis symposium**  
February 2019



Inspired by **patients.**  
Driven by **science.**



**IRDiRC**  
INTERNATIONAL  
RARE DISEASES RESEARCH  
CONSORTIUM

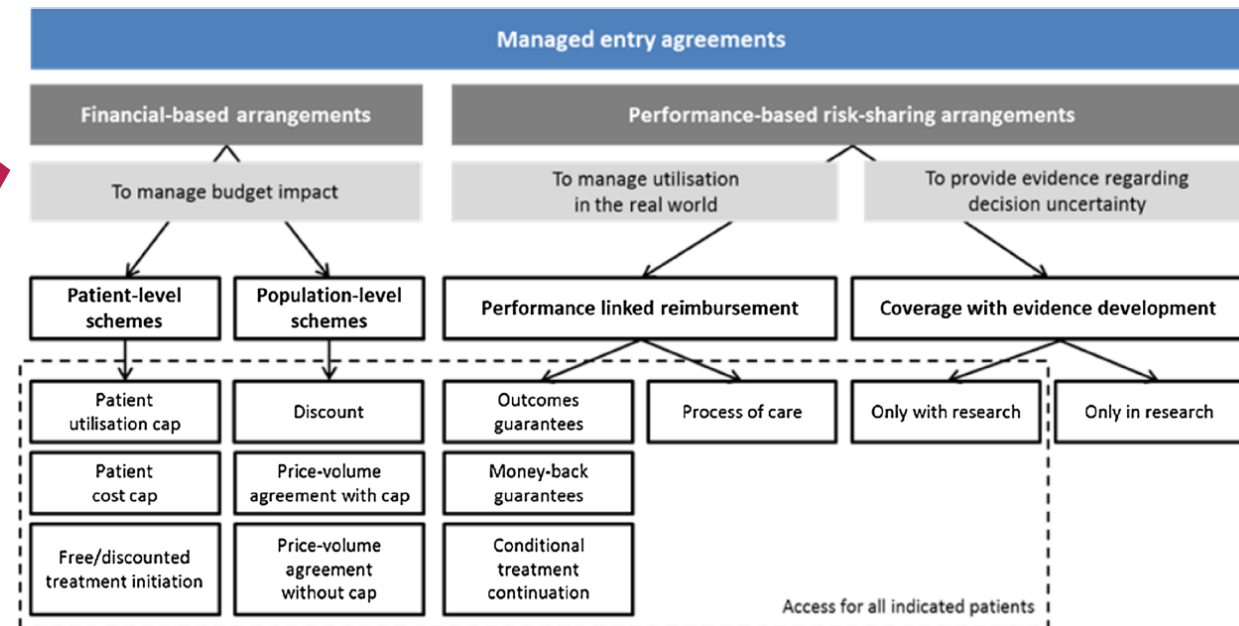
**KU LEUVEN**

# Disclaimer

An employee of **UCB Biopharma**, Belgium

Also staff member at **KU Leuven**; individual contributor to IRDiRC and EJP RD

# ‘Uncertainty’ and healthcare decision-makers...



# How does observed treatment effect translate into patient benefit?

## COMPOSITE ENDPOINTS

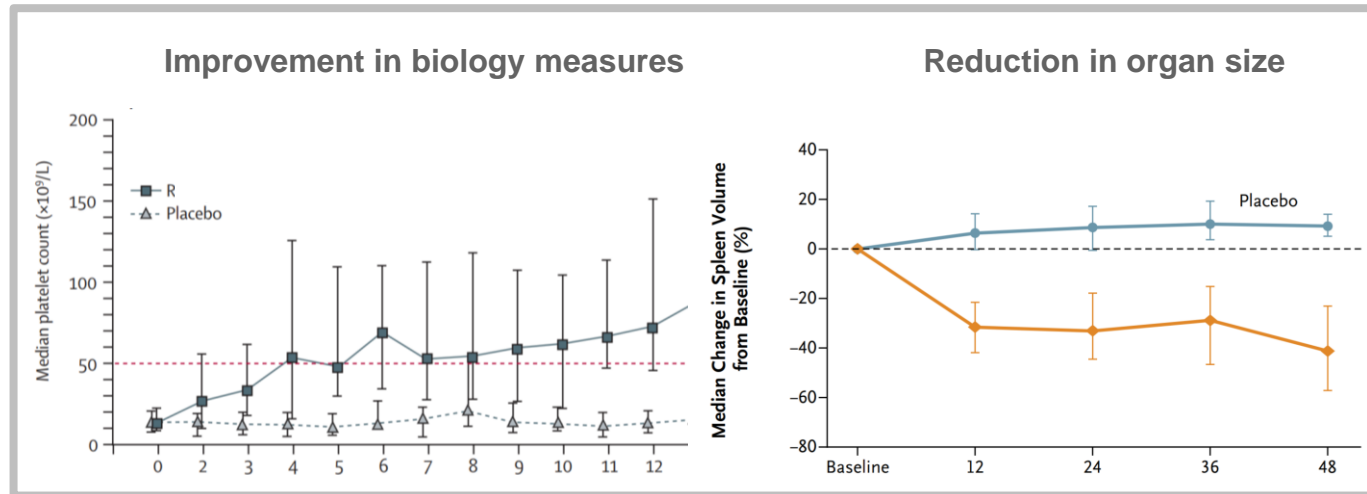
The **SLE Responder Index (SRI)** is composed of the following scores:

- SELENA-SLEDAI (Safety of Estrogens / Disease Activity Index);
- BILAG (British Isles Lupus Assessment Group);
- PGA (physician global assessment),

SRI response is defined by the following:

- A 4-point or greater reduction in the SELENA-SLEDAI score;
- No new BILAG A or no more than 1 new BILAG B domain score;
- No deterioration from baseline in the PGA by 0.3 or more points.

## SURROGATE ENDPOINTS



?

*'interpretable'*  
patient benefit ?

# A need of '*interpretable*' and '*unequivocal*' measure of patient benefit

## CLINICAL OUTCOMES ASSESSMENTS

*'the **clinical relevance of a response** [in this composite endpoint] is difficult to determine'*

*'the evidence did not support the achievement of outcomes known to be **clinically relevant to patients**'*

*'the use of [this surrogate endpoint] is **debatable**'*

*'there is a **lack of correlation** with clinical outcomes that may be more relevant'*

*'all too often what matters most to patients is poorly captured in the available clinical trial data'*

U.S. Institute for Clinical and Economic Review

?

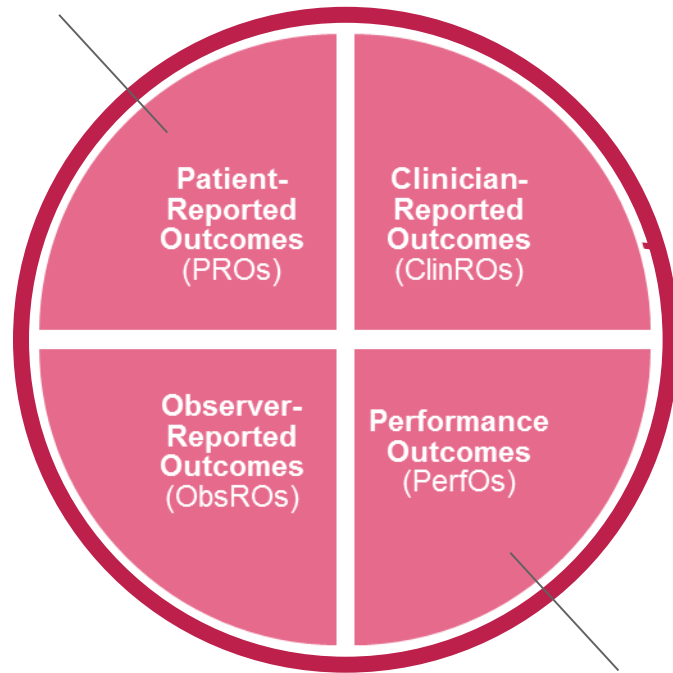
*'interpretable'*  
patient benefit



How patients:  
**feel,**  
**function,**  
**survive.**

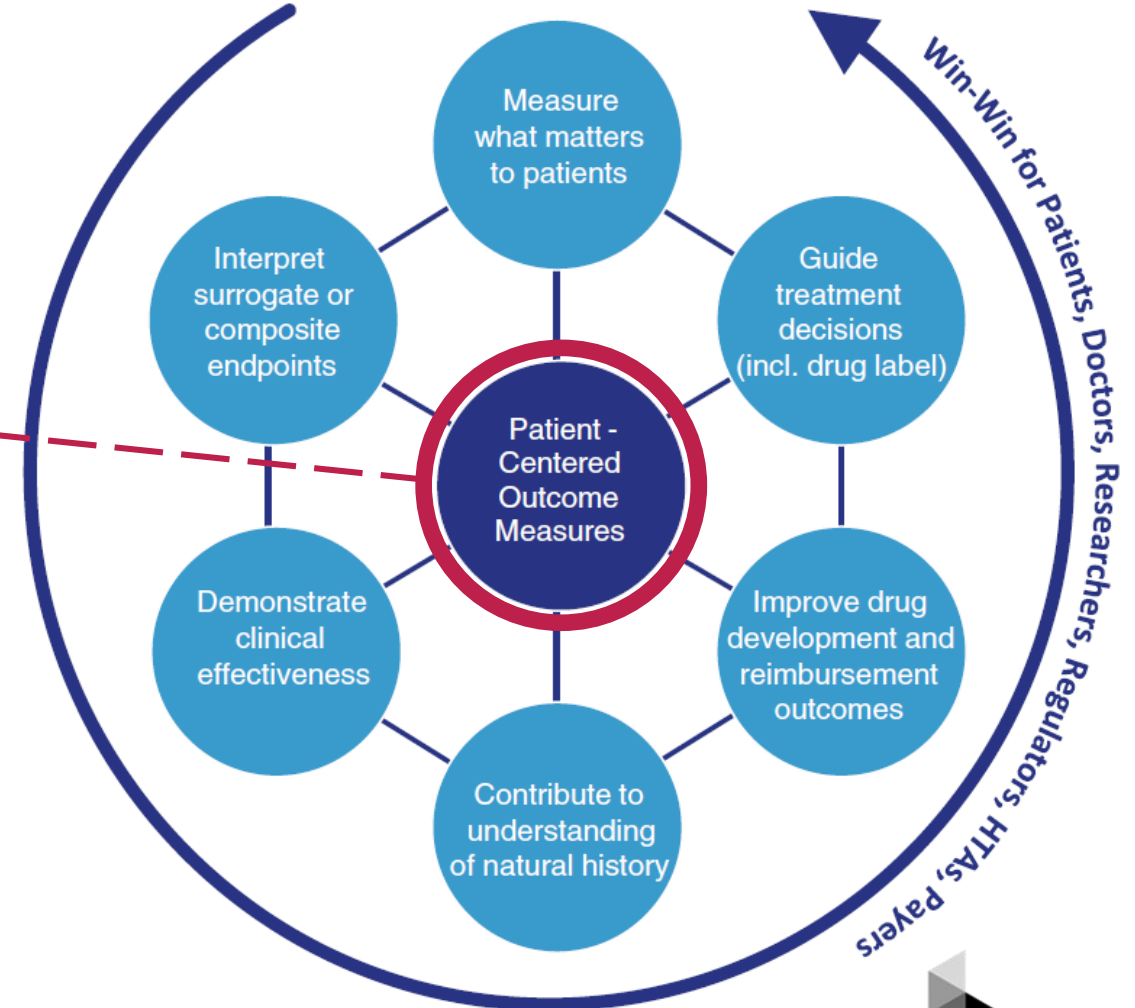
# IRDiRC: Patient-Centred Outcomes Measures in RD: '*a necessity*'

DMD Upper Limb PROM  
Myasthenia Gravis PROM

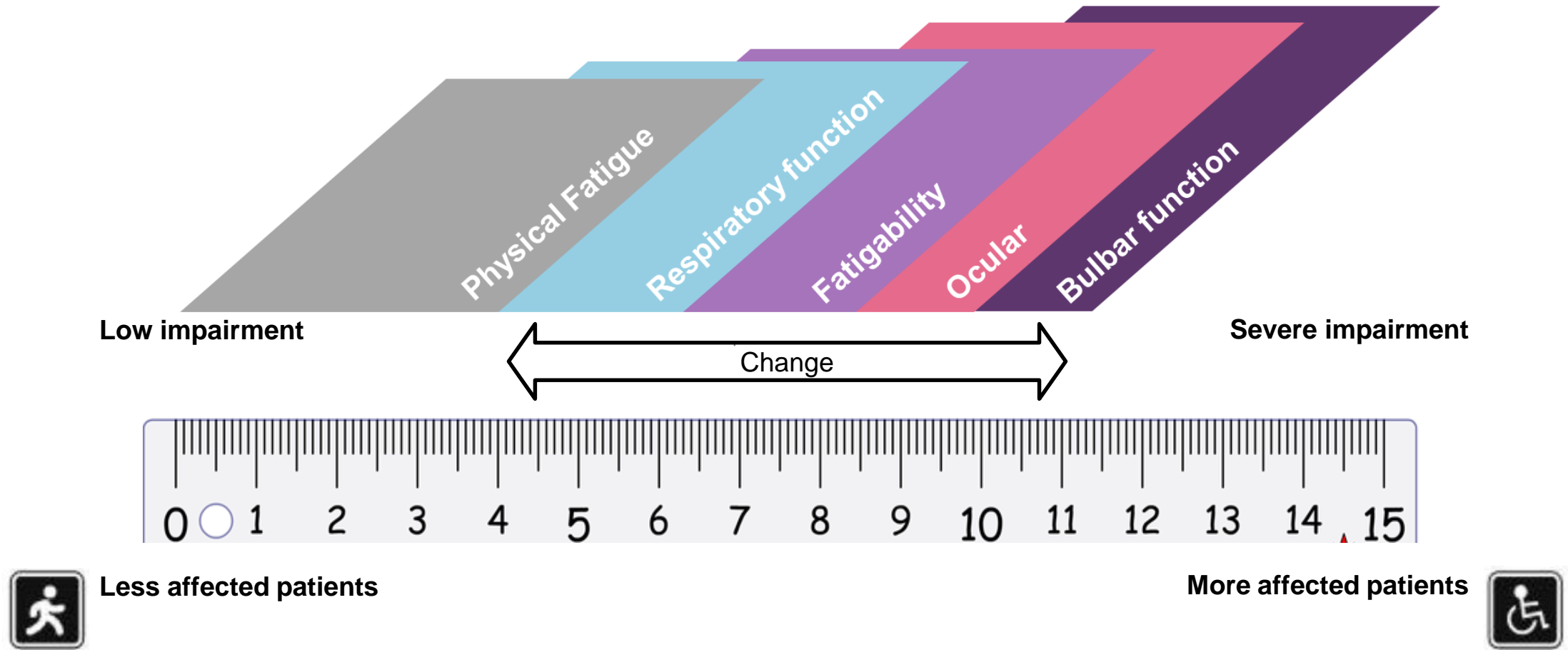


DMD Performance of the Upper Limb

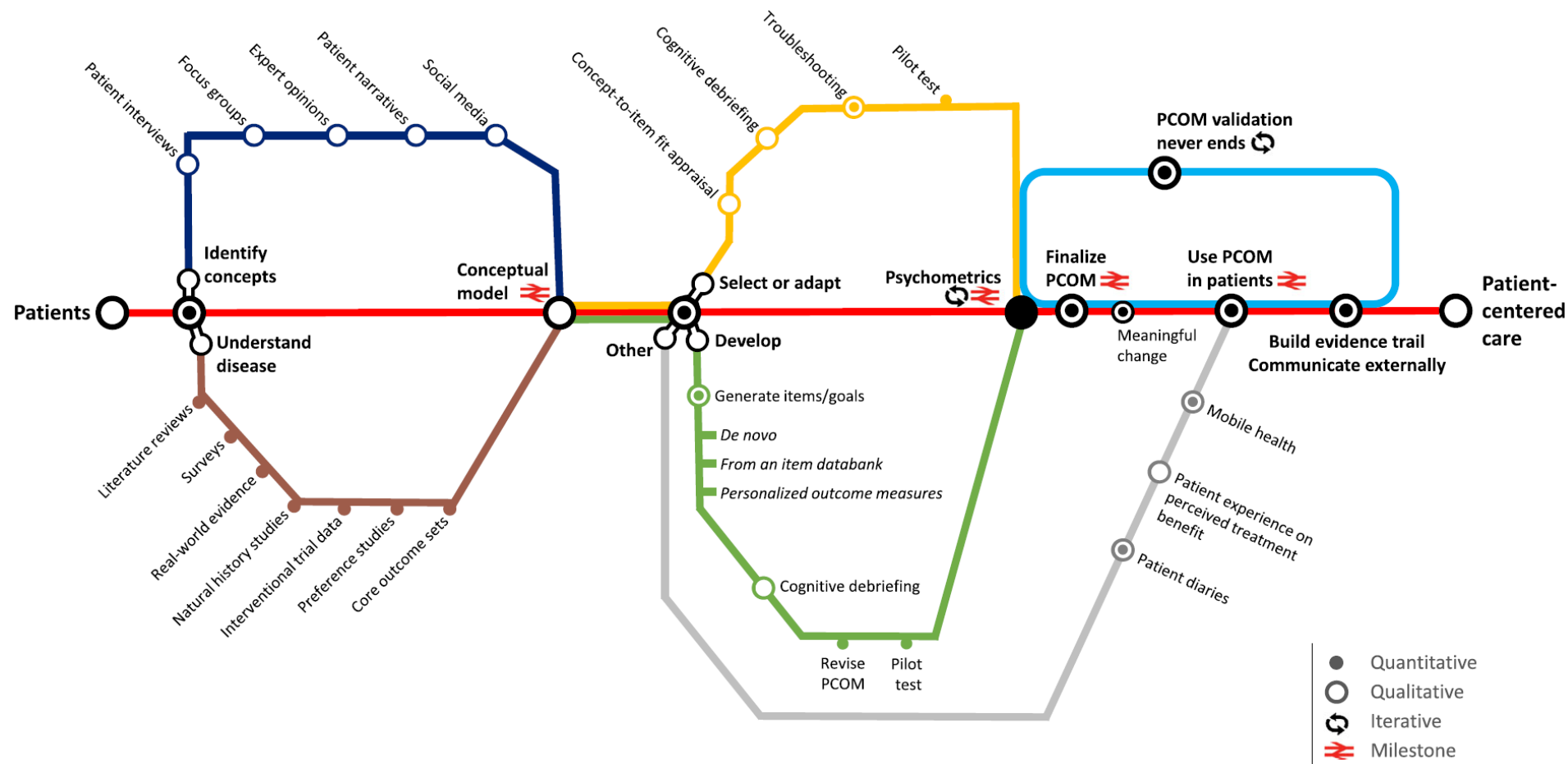
A more meaningful and interpretable  
measure of patient benefit



# Myasthenia gravis: a UCB example



# 'On track' to Patient-Centered Outcome Measurement

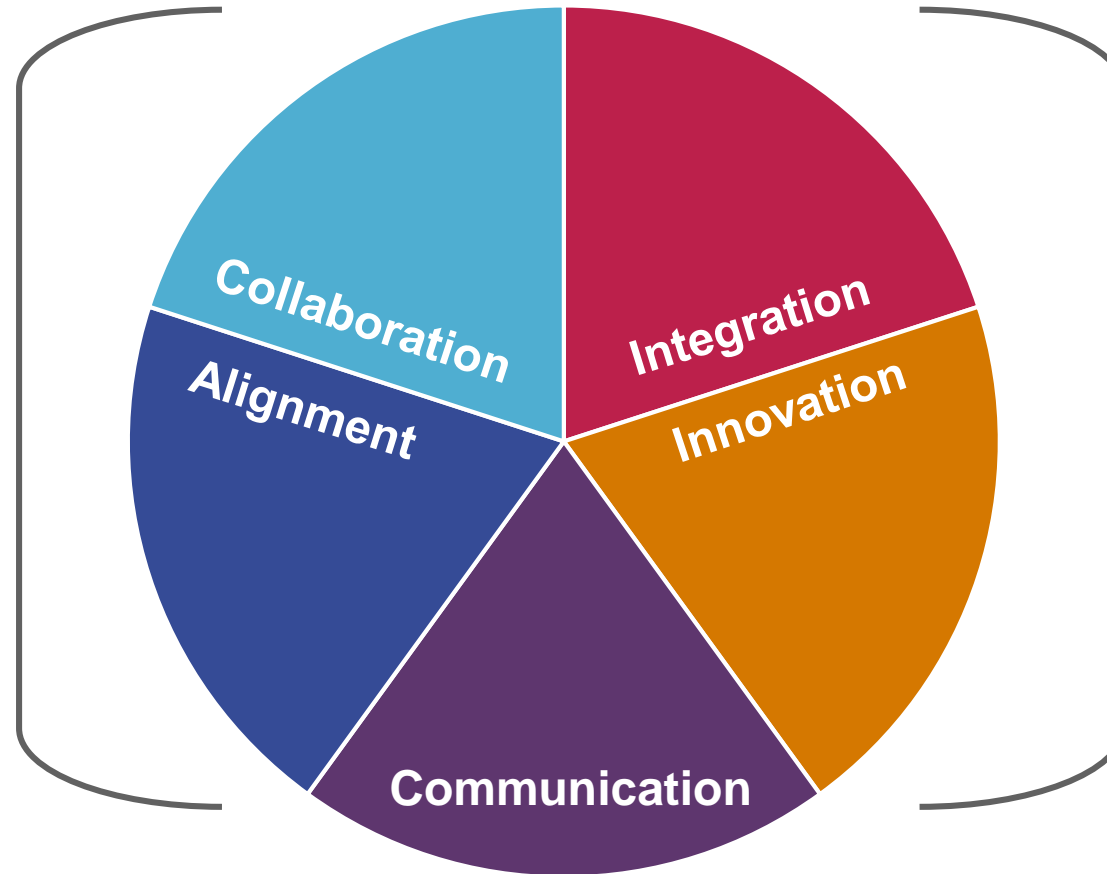




# Five core concepts around PCOMs

EURORDIS  
Community Advisory Boards

Joint scientific advice



European  
Reference  
Networks

EJP – WP 20

‘validation, use and  
development of innovative  
methodologies for  
clinical studies in rare diseases’

# THANK YOU