Measuring what matters to patients

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Disclaimer

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‘Uncertainty’ and healthcare decision-makers…

Morel et al. Orphanet Journal of Rare Diseases 2013, 8:198
How does observed treatment effect translate into patient benefit?

COMPOSITE ENDPOINTS

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

- SELENA-SLEDIA (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-SLEDIA 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

Improvement in biology measures

Reduction in organ size

‘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

How patients: feel, function, survive.
IRDiRC: Patient-Centred Outcomes Measures in RD: ‘a necessity’

DMD Upper Limb PROM
Myasthenia Gravis PROM

Morel et al. Orphanet J Rare Dis. 2017 Nov 2;12(1):171
Myasthenia gravis: a UCB example

Less affected patients

More affected patients

Change

Low impairment

Physical Fatigue

Respiratory function

Fatigability

Ocular

Bulbar function

Severe impairment
‘On track’ to Patient-Centered Outcome Measurement
Five core concepts around PCOMs

EURORDIS Community Advisory Boards

Joint scientific advice

EJP – WP 20
‘validation, use and development of innovative methodologies for clinical studies in rare diseases’
THANK YOU