

Fleur Chandler

- Health Economist, Clinical Research and Market Access Specialist
- Previously
 - Head of Market Access UK Ireland Sanofi
 - Head of Global Health Outcomes (Respiratory) GSK
- Parent of young adult with Duchenne Muscular Dystrophy
- Parent of child in clinical trial
- Parent Advisory Board Duchenne UK
- Chair of Project HERCULES
- Co developed DMD-QOL
- Stock holder in Sanofi, Haleon and GSK

Holistic view



DEVELOPMENT



IMPLEMENTATION



RESPONDING



ANALYSING



PRESENTING



DECISION MAKING

Barriers and Challenges in Paediatric PRO

Invention

Innovation

Instrument

Interpolation

Implementation

Interpretation

Inference

Impact

Invention and Innovation of Instruments

- Creation of instruments – very challenging space
- Who is it for?
- Whose value sets?
- Parent or child or population preferences
- Age: infant, child, adolescent, teen, young adult
- Chronic, progressive or acute
- Rare or common
- Severe and or life-limiting
- Cognitive or developmental challenges
- Ethics
- Burden

Interpolation and Implementation

What could be done better?

- Guidance from decision makers
- Clinical trial or RWE setting
- Long term data collection
- Reassurance on interpretation
- Understand patients and parents

- Paediatric trials very different to adult trials
- Licence: adult extension, paediatric origin or paediatric
- PIP programmes post adult programmes
- Early decision making on trial design
- PRO inclusion
 - Who – Patient or Parent
 - What – Purpose of collection
 - Why – decision-makers
 - Where – clinical trial or real world
 - When – Time points essential
- Setting
- Practicalities
- Ethics

Interpretation and Inference

- Paediatric evidence subjective
- Analysts and Decision Makers must consider
 - Setting
 - Respondent
 - Timing
 - Age
 - Severity
 - Repetition
 - Disease progression
 - Fatigue
 - Response bias
- Needs interpretation with caution and high degree of flexibility before inferring results into decisions

Impact

- PROs a vital part of the decision making for access to medicines for paediatrics – particularly in cost-effectiveness markets
- Can lead to life impacting decisions – in paediatric life-limiting progressive diseases*
- Vitally important we get it right

