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DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

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PRO130

PROJECT HERCULES: *CONSTRUCTION OF A NEW PREFERENCE-BASED MEASURE OF QUALITY OF LIFE FOR DUCHENNE MUSCULAR DYSTROPHY (DMD)*

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Objectives

Duchenne muscular dystrophy (DMD) is a rare progressive life-limiting paediatric neuromuscular disease. There is no known cure, so interventions focus on slowing progression and improving quality of life (QoL). Evidence suggests that existing preference-based measures (PBMs) may be inadequate for assessing QoL in DMD. **Project HERCULES** is developing a new PBM in DMD.

Methods

Here we describe the results of stages 1 and 2a of a 3-stage PBM development process. In Stage 1, we undertook **18 semi-structured interviews** with people with DMD of varying ages. We used framework analysis to identify themes and an initial descriptive system. In Stage 2a, **cognitive debriefing** was done with 10 patients, 10 parents, and 8 clinicians to refine a draft questionnaire.

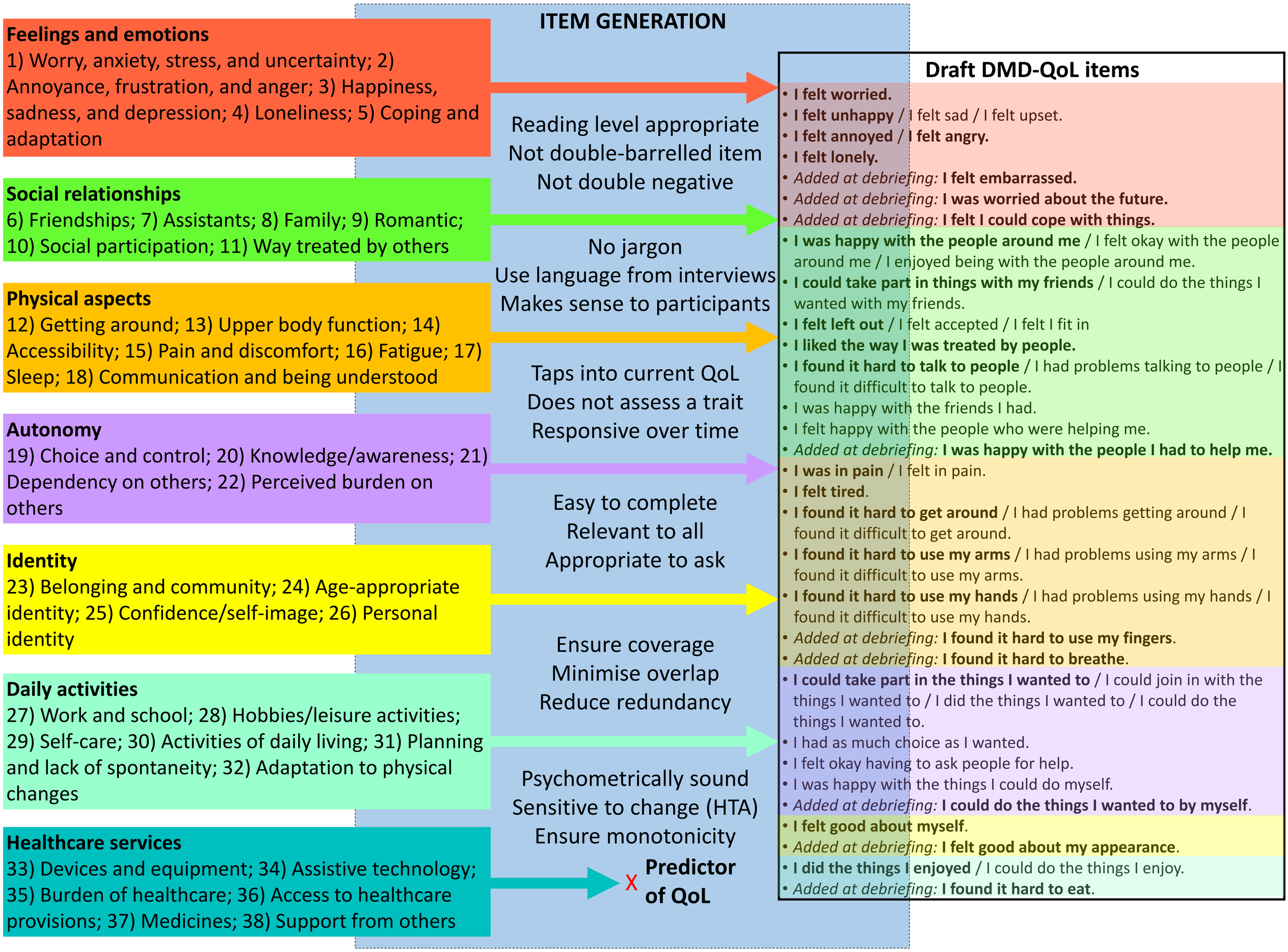

Figure 1. Mapping qualitative themes onto items using principles of item generation. 43 items went to cognitive debriefing. Bold items chosen after cognitive debriefing for 27-item draft measure.

Results and Conclusions

Seven QoL domains were identified as important in Stage 1. A draft 43-item questionnaire was developed. In Stage 2a, the draft questionnaire was refined following cognitive debriefing. This included an assessment of content validity, item wording, response options, and instructions. This resulted in a **27-item draft measure** to be tested in a national psychometric survey (see **Figure 1**). The draft questionnaire has high content validity. Key challenges included a sensitive subject matter and differing views as to which items should be included in the new PBM.

Acknowledgements

- Project HERCULES is an international multi-stakeholder collaboration led by Duchenne UK that is developing disease-level tools and evidence to support HTA and access decisions for new treatments for Duchenne Muscular Dystrophy.
- HERCULES is funded by Duchenne UK, Catabasis Pharmaceuticals Inc, Pfizer Inc, PTC Therapeutics, Roche, Sarepta Therapeutics Inc, Solid Biosciences, Santhera Pharmaceuticals Holding AG, Wave Lifesciences USA Inc.

Presented at ISPOR Europe 2019, Copenhagen, 2nd-6th November 2019