HERCULES.
DMD: HEALTH RESEARCH COLLABORATION UNITED IN LEADING EVIDENCE SYNTHESIS

DUCHENNE UK

Source Health Economics

Poster number
PRO20

PROJECT HERCULES: A paradigm shift in the development of cost-effectiveness models in rare diseases

Woodcock F[1], Mumby-Croft J[1], Ghosh S[1], Chandler F[2], Godfrey J[3], Crossley, E[4]; The Project Hercules Steering Group

[1]Source Health Economics, UK. [2]Alcmena Consulting Ltd, UK. [3]JG Zebra Consulting Ltd, UK. [4]Duchenne UK.

Background

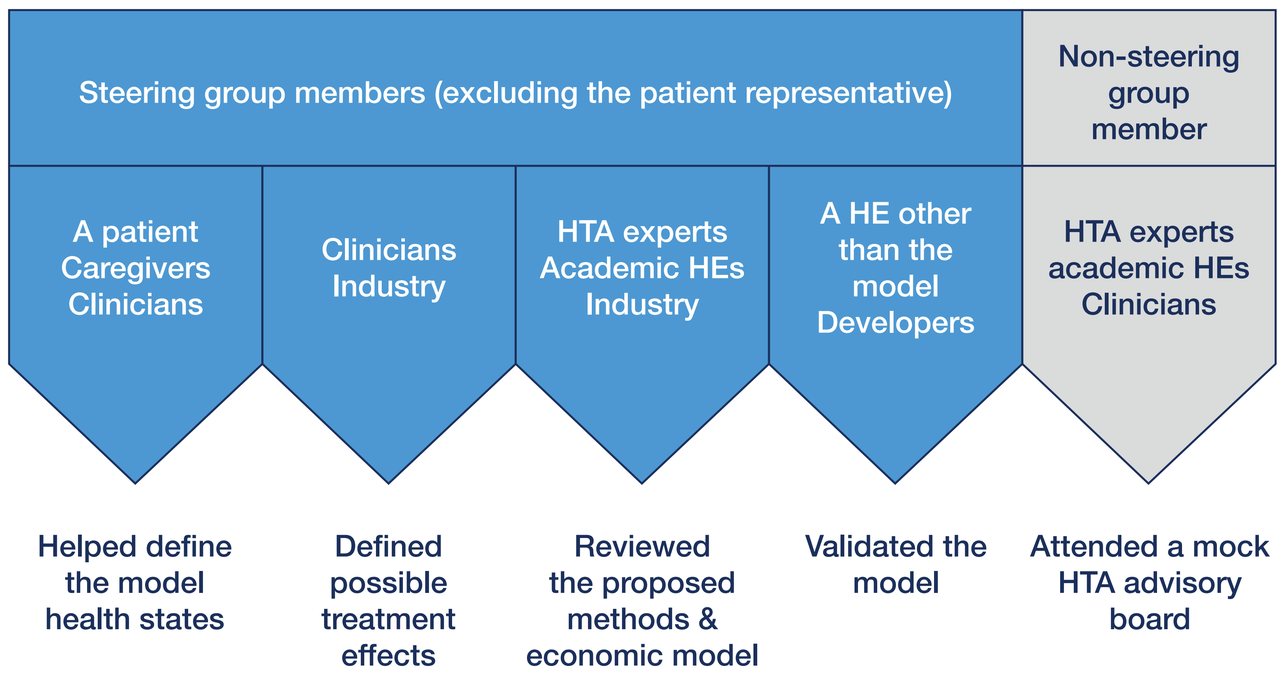
- One of the key challenges when developing cost-effectiveness models in rare diseases is a paucity of data.
- Data for natural history, health-related quality of life (HRQoL) and healthcare resource use can be lacking.
- This can lead to an oversimplification of natural history and an over-reliance on assumptions.
- This can impact the methodological quality of the economic model and increase uncertainty.

Objective

- Project HERCULES is an International collaboration between Duchenne UK and eight pharmaceutical companies.
- The collaboration set out to:
  - Develop a single, robust disease level cost-effectiveness model for use in health technology assessment (HTA) in Duchenne Muscular Dystrophy (DMD).
  - Populate this model with robust data for natural history, HRQoL and resource use.

Figure 1: Area experts involved in model development

Figure 2: Expert input during model development



Abbreviations: HE, health economist. 'Industry' partners included: Catabasis Pharmaceuticals, Pfizer, PTC Therapeutics International Ltd, Roche, Sarepta Therapeutics, Solid Biosciences LLC, Santhera Pharmaceuticals Holding AG and Wave Life Sciences USA, Inc.

Methods

- A core multi-state cohort model was developed based on clinically and economically important health states defined with input from clinicians, a patient and carers.
- Clinicians and industry partners also provided input on possible treatment effects to be included in the model.
- Data were then collected and synthesised by area experts in statistics, burden-of-disease and patient-reported outcomes (PROs) to provide a core data set.
- Transition probabilities were derived for the natural history of DMD from an analysis of multiple datasets including patient registries and the placebo-arm of published studies.
- A Delphi approach was used to identify key items of resource use, and interviews with patients, carers and clinicians provided estimates of resource use by item.
- Patients and carer interviews also provided utilities for the model.
- A de novo PRO was also developed to facilitate the future estimation of utilities in people with DMD.
- A steering group and advisory board including HTA experts, academic health economists, clinicians, carers and industry-representatives provided peer-review throughout the development process.
- Once completed, the economic model went through a rigorous external validation.

Results

- Project HERCULES has provided a disease level model for the economic evaluation of new treatments in DMD that captures the impact of disease, the benefits of treatment, and is based on a robust data set.

Conclusions:

- Project HERCULES illustrates the potential for a paradigm shift in the development of cost-effectiveness models in rare diseases where manufacturers collaborate to generate a robust core economic model and data set, and a standardised methodology is used across HTA submissions.
- Future evaluation is required to determine the ease with which the model is used and adapted by manufacturers of different interventions, and how the core model and data set is received by HTA bodies.

Acknowledgements

This project is funded by Duchenne UK, which has received funding from 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