PROJECT HERCULES: A MODIFIED DELPHI STUDY TO INFORM A BURDEN-OF-ILLNESS STUDY IN DUCHENNE MUSCULAR DYSTROPHY

Introduction

- Duchenne muscular dystrophy (DMD) is a rare disease affecting 15.9-19.5 in 100,000 births in the United Kingdom (UK) (1).
- DMD causes progressive damage and degeneration to muscle tissue and creates a variety of problems including muscular weakness, respiratory impairment and a loss of ambulation (2).
- By mid-teens people with DMD will normally have lost ambulation and are wheelchair bound. Severe respiratory and cardiac problems develop in their twenties and are usually the cause of death (2).
- There is no cure for DMD and treatment options focus on alleviation of symptoms and management of complications. There is an urgent need for therapies which can alter the fundamental course of DMD (2).
- Corticosteroids are the standard of care but are associated with a number of side effects (3,4).
- There is a need for a comprehensive burden of illness (BOI) capturing the impact, epidemiology, costs and treatment associated with DMD across all stages.
- Given the extensive impact of DMD, a review of all aspects, from definitions to prioritisation of questions needs to be undertaken. Findings inform our BOI study design to effectively capture the experience by people with DMD and caregivers.

Methods

- A modified Delphi process was implemented to inform the generation of research items and questions.
- Invitations to participate in the expert panel (EP) were sent to members of Project HERCULES to include perspectives from multiple stakeholders including:
  - Physicians
  - Parents / Caregivers / Patient advocates
  - Health regulators
  - Industry representatives and health economists.
- The Delphi panel consisted of two rounds, with participants reviewing anonymised responses in the second round from the previous, as shown in Figure 1.

Results

- Members of the steering committee completed the questionnaire by rating the importance of each research item in capturing the BOI of DMD in the UK study in regard to an HTA submission.
- A 5-point Likert scale was used with answers representing level of agreement of capturing the item in the DMD BOI study, with 1 representing strongly disagree, 2 representing disagree, 3 representing a neutral view, 4 representing agree and 5 representing strongly agree (Figure 2).
- After the second round, median ratings in all categories were captured alongside the range of responses and any comments which had been made by participants.

Conclusions

- This study has collated the views of a variety of perspectives to inform the development of a relevant set of core research items.
- The implementation of these items into the subsequent DMD BOI study will help to effectively describe and quantify the impact of DMD on people with DMD and caregivers.

References


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