DISEASE ATTRIBUTES MOST IMPORTANT FROM A SOCIETAL PERSPECTIVE: A CASE STUDY INVOLVING DUCHENNE MUSCULAR DYSTROPHY

SM Szabo,[1] IF Audhya,[2] DC Malone,[3] P Neumann,[4] D Feeny,[5] M Harwood,[1] KL Gooch.[2]

[1]Broadstreet HEOR, Vancouver, BC; [2]Sarepta Therapeutics, Inc., Cambridge, MA; [3]The University of Utah, Salt Lake City, UT; [4]Tufts School of Medicine, Boston, MA; [5]McMaster University, Hamilton, ON.

BACKGROUND

- Current value assessment frameworks consider elements such as health gains to the patient and net costs when assessing the cost-benefit profile of new therapies.
  - Less frequently, aspects such as lost productivity or patient adherence are considered.[1]
- There is a growing interest in more comprehensive value assessment frameworks;[2] particularly as, for some diseases, existing frameworks may fail to consider key attributes like rarity or young age of onset.
  - This may be the case for life-limiting progressive diseases like Duchenne muscular dystrophy (DMD), with a high disease burden and poor prognosis.
- If the societal perspective is taken into account in the value assessment of new therapies,[3] all disease and treatment attributes society deems important should be included.
  - Therefore, societal views on these attributes must be understood to inform decision making, and better characterize the value of new treatments.
- However, little data exist on how the general public regards different disease and treatment attributes.

OBJECTIVE

To identify the importance, from a societal perspective, of specific attributes characterizing rare pediatric disorders like DMD.

METHODS

- One-on-one qualitative interviews were conducted with members of the US general public from Seattle, San Francisco, and Dallas; recruited to reflect a varied distribution in age, sex, and number of children living at home.
- Interviews were led using a semi-structured interview guide and series of visualization props.

METHODS, CONT.

- Potentially important disease and treatment attributes for decision making were identified based on an ISPOR Special Task Force publication,[1] literature review, and feedback from eight members of the US general public.
  - These included: disease rarity; age at onset; cause (genetic vs. acquired); availability of treatments; disease severity; impact on life expectancy, mental health, activities of daily living (ADL) and health-related quality-of-life (HRQoL); and caregiver burden.

Data collection and analysis

- Participants ranked attributes on a scale of 1 (*not important)* to 10 (*extremely important*) in terms of their importance for prioritizing research and treatment, to provide a holistic value assessment
  - An anonymized disease profile that described DMD was similarly ranked
- Participants also reported their rationale for attribute importance, and provided qualitative feedback on what a 'rare disease' meant to them.
- Interview transcripts were coded in NVivo for thematic analysis.
- For each attribute, mean (min-max) rankings were calculated.
- The percentage classifying an attribute (or the DMD profile) with a rank >6 (i.e. they ranked an attribute as *very important* [rank 7 or 8] or *extremely important [*rank 9 or 10]) was calculated.

RESULTS

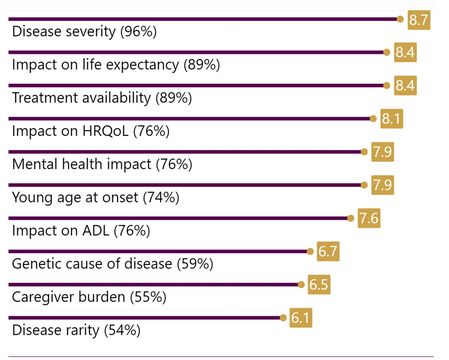
- Of the 33 participants, the mean (range) age was 49.8 (26-71) years, 48.5% were male, and 33.3% live with children (Table 1).
- Across all disease and treatment attributes considered, mean (min-max) rankings ranged from 8.7 (5-10) for disease severity, to 6.1 (2-10) for rare disease (Figure 1).

RESULTS, CONT.

Table 1. Participant characteristics

Characteristic	n	%
Highest education level		
Graduate studies	4	12.1
College/university	20	60.6
Grade or high school	9	27.3
Relationship status		
Single	12	36.4
Married/partnership	19	57.6
Divorced/other	2	6.0
# children <18 years at home		
0	22	66.7
1	6	18.2
2+	5	15.2
Household income		
Less than 25,000	2	6.1
25,000-49,999	8	24.2
50,000-99,999	8	24.2
100,000-149,999	6	18.2
150,000-199,999	4	12.1
200,000+	4	12.1
Rare disease		
Self/family member affected	8	24.2
Familiar with a rare disease	10	30.3

Figure 1. Mean importance ranking, by attribute*



*The percentage ranking an attribute as *very or extremely important* (>6) is presented in parentheses

- Estimates of how frequently individuals ranked attributes as *very or extremely important* ranged from 96.3% (disease severity) to 53.6% (disease rarity)
- For the DMD disease profile itself, the mean (min-max) ranking was 8.6 (2-10), in terms of its importance for prioritizing research and treatment
  - 87.5% of participants rated this as *very or extremely important;* citing it was the collection of individual attributes together (e.g. the combination of a life limiting severe disease, with pediatric onset) that made the DMD profile very important.
- Frequent reasons for prioritizing the DMD profile included consideration for equity, large burden, family impact, unavoidability, value of life, and timing of loss of life.
- The DMD profile was ranked at 8.6 in terms of importance, but "rare" diseases achieved the lowest mean ranking of the attributes (6.1).
  - This contrast my have occurred because most participants did not think diseases occurring as frequently as DMD (affecting an estimated 1:5,000 live male births)[4] were in fact "rare"
  - 89.5% thought a rare disease would affect <1:10,000 (i.e. ~33,000 Americans); 78.9% thought a rare disease would affect <1:100,000 (i.e. ~3,300 Americans)
- Participants did not highlight a distinction between attributes that would be important for guiding research, compared to those for guiding treatment, priorities.

DISCUSSION

- Findings from this study suggest disease and treatment attributes important from the societal perspective; in particular, attributes relevant to rare, life-limiting, genetic pediatric diseases like DMD.
  - When viewed together, the collection of attributes that characterize diseases like DMD dictate its perceived importance, more than the contribution of individual attributes.
  - Many of the attributes that members of the general public think important may 'sit outside' current value frameworks, and therefore receive no quantifiable consideration.
- Societal perspectives on the definition of disease rarity may differ from those used by healthcare decision makers.[5]
- One limitation is that these interviews were conducted just prior to the COVID-19 pandemic and it is possible that participants' views on some aspects would now differ.
- Another limitation is that the present study did not consider how factors such as geography or other potential predictors of preferences might affect results.

CONCLUSIONS

This study of a sample of the general public suggests that a multitude of characteristics may be considered in value assessment, with many of them directly applicable to diseases such as DMD. In this respect, impact on life expectancy, young age of onset and shortened lifespan were ranked highly by participants.

References

1) Lakdawalla et. al. V*alue Health.* 2018;21(2):131-139. 2) Ollendorf et. al. ICER;2017.
3) Sanders et al., *JAMA*. 2016;316(10):1093-1103. 4) Crisafulli et al., *Orphanet* 2020; 15:141.
5) FDA. Orphan Drug Act 1983.

ACKNOWLEDGEMENTS & DISCLOSURES

This study was funded by Sarepta Therapeutics, Inc. IA and KLG are employees of Sarepta Therapeutics, Inc. SMS is and MH was an employee of Broadstreet HEOR, which received funds from Sarepta for the conduct of this study. DCM, PN, DF received consultancy fees from Sarepta Therapeutics.
Contact: sszabo@broadstreetheor.com

Presented at the WMS25: Virtual Congress
September 28 – October 2, 2020