

SOCIETAL PERSPECTIVES ON THE IMPORTANCE OF DISEASE AND TREATMENT ATTRIBUTES: A QUALITATIVE STUDY FROM THE UNITED STATES

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

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

BACKGROUND

- All disease and treatment attributes that society deems important should be considered within value frameworks evaluating costs and benefits of new therapies.
- Assessment frameworks that seek to incorporate additional dimensions of value and take a more holistic approach to determining value have been gaining attention.^{1,2}
 - Traditional frameworks tend to focus on patient health gains and healthcare system costs.¹
 - However, for some diseases, this approach may fail to comprehensively consider additional attributes that are important for accurately assessing the full value of a new therapy.
- Little data exist on how the general public regards different disease and treatment attributes.
- A more informed understanding of the societal views of these attributes will help inform whether value frameworks should be modified accordingly.

OBJECTIVE

To investigate how the general public values the importance of disease and treatment attributes, beyond health gained by patients and overall costs to the healthcare system.

METHODS

- Potentially important attributes were identified based on: the ISPOR Special Task Force on Value Assessment,¹ a literature review, and discussions with a convenience sample of eight members of the general public.
- Attributes included: Disease rarity, age at onset, cause (genetic vs. acquired), availability of treatments, disease severity, life expectancy, mental health, impact on activities of daily living (ADL) and health-related quality-of-life (HRQoL) and caregiver burden.

METHODS, CONT.

- An interview guide and visualizations were developed and pilot tested.
- One-on-one qualitative interviews were conducted with members of the US general population from Seattle, San Francisco, and Dallas to understand their impression on the importance of these various attributes. Participants:
 - Ranked attributes on a scale of 1 (*not important*) to 10 (*very important*) in terms of importance for future research.
 - Reported on drivers of attribute importance and perceptions of relationships between attributes.
- Transcripts were coded for thematic analysis using NVivo.
- For each attribute, mean (min-max) rankings were calculated, as was the frequency of participants reporting interactions between attributes; results were displayed graphically.
- Themes that emerged in discussions across attributes were plotted and patterns reviewed.

RESULTS

- The mean (range) age of the 33 participants was 49.8 [26-71] years, 48.5% were male, and 33.3% had children <18 years living at home (Table 1).
- Of the attributes considered, disease severity (both in terms of its symptoms and burden; mean, 8.7), treatment availability (8.4), impact on life expectancy (8.4), and impact on HRQoL (8.1) were ranked most highly (Figure 1)
- Some attributes were frequently discussed in combination, or participants noted interactions between them (Figure 1).
 - Participants discussed impact on HRQoL and ADL in an interchangeable fashion, despite their receiving different importance scores in the ranking exercise.
 - Attributes frequently reported to occur in combination with others included: impact on life expectancy, impact on HRQoL/ADL, and disease severity.
- Reasons that emerged on why attributes were important included: Avoiding disability or lifetime burden, pursuit of equity, the intrinsic value of life, the ability for one to live a full life and plan for the future, impact on the family and to avoid being a burden (Figure 2).
- For those that did not highly value caregiver burden, it emerged that while they felt alleviating caregiver impact was important, they thought prioritizing treatment of the patient's underlying disease would be the optimal strategy to mitigate this.

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

Figure 1. Attribute ranking and frequency of reported relationship between attributes

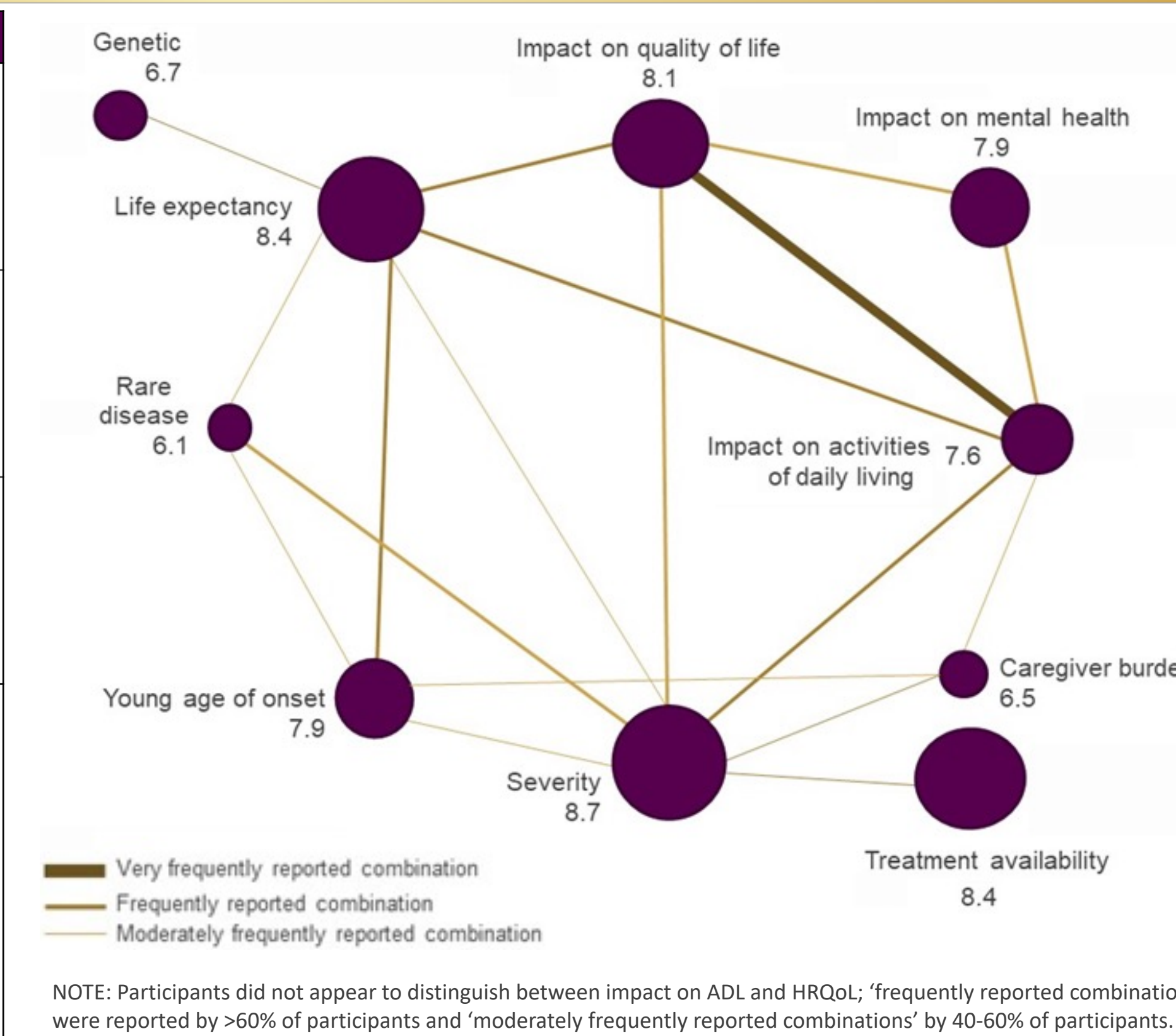
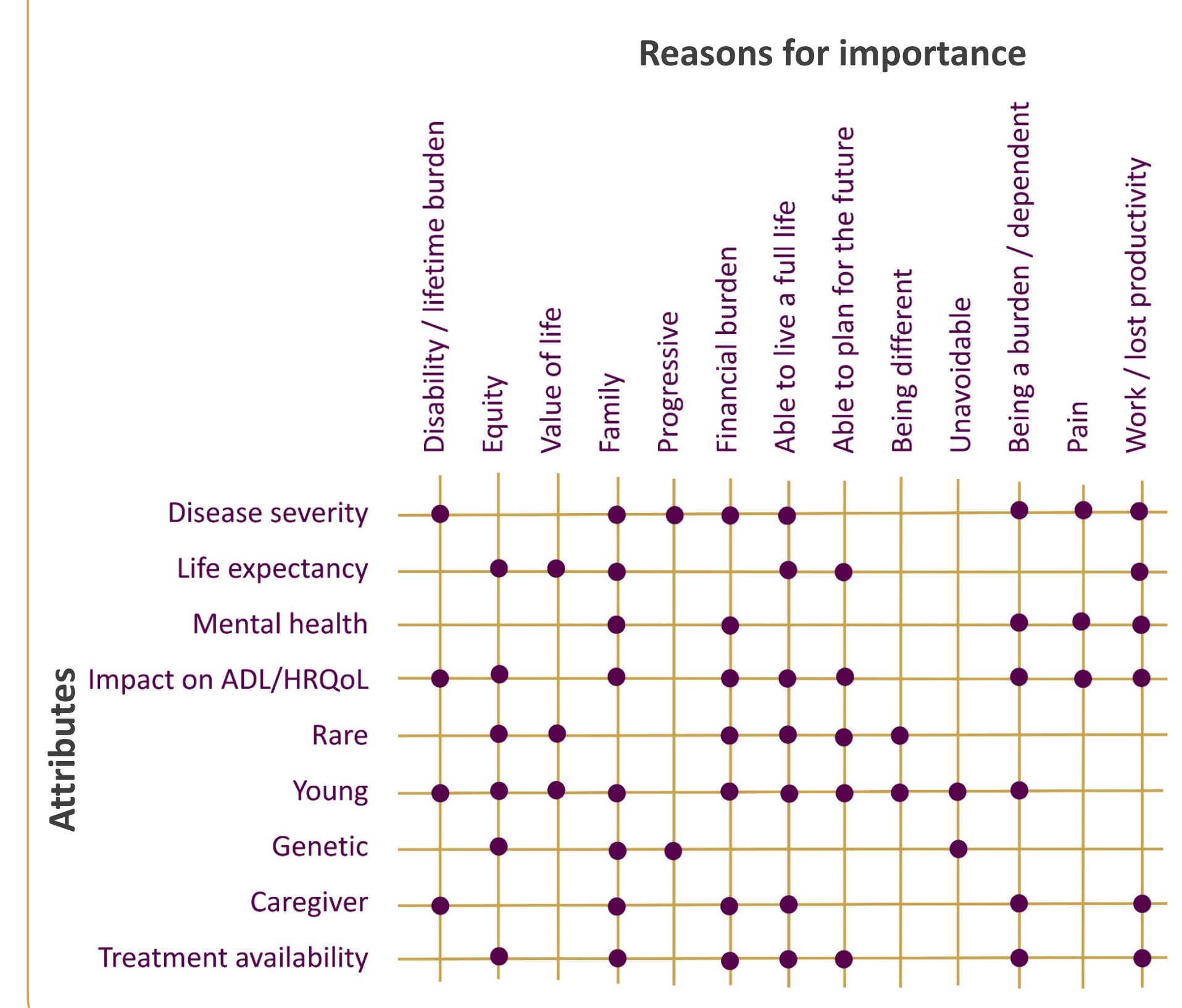


Figure 2. Patterns in reasons why different attributes were considered important for research and treatment



DISCUSSION

- Attributes including disease severity, impact on life expectancy and HRQoL, and treatment availability were all highly ranked by members of the general public in terms of their importance for guiding research into diseases with these attributes.
- Numerous reasons that attributes were considered important were highlighted; e.g. the burden they placed on the patient or family, value of life, and need for equity.
- Comparing reasons for importance across attributes revealed that, in general, the attributes considered most important, shared numerous reasons for importance (e.g. impact on family was raised for all of the most highly ranked attributes).
- Limitations include that the interviews were conducted just prior to the COVID-19 pandemic and it is possible that participants' views may now differ; and
- As the present study did not consider how factors such as geography or other potential predictors of preferences might affect results, this represents an avenue for further research.

CONCLUSIONS

Findings from this study suggest attributes like disease severity, impact on life expectancy and HRQoL, and treatment availability are important to members of the US general public; these may be useful to more explicitly consider within evolving frameworks for assessing costs and benefits of new therapies.

REFERENCES

- Lakdawalla et. al. *Value Health*. 2018;21(2):131-139.
- Ollendorf et al., *ICER* 2017.

ACKNOWLEDGEMENTS & DISCLOSURES

This study was funded by Sarepta Therapeutics, Inc. IA and KLG are employees of Sarepta Therapeutics, Inc. SMS and MH at the time of study conduct are/were employees of Broadstreet HEOR, which received funds from Sarepta for the conduct of this study. DCM, PN, DF received consultancy fees from Sarepta Therapeutics
Contact: iaudhya@sarepta.com