

# ELICITING PATIENT EXPERIENCE FOR VALUE ASSESSMENT IN A CASE STUDY OF SPINAL MUSCULAR ATROPHY

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## INTRODUCTION

As we enter the era of patient centric healthcare and decision making, conventional value assessment mostly based on regulatory anchored data can still fall short when capturing the patient experience. A number of PEx values have been identified in the literature already, however it is not clearly defined which additional patient centric value elements (PEx) should be considered [Inotai et al., 2021], especially for patients living with a rare disease, where trial evidence may be limited. Although patient preferences are essential to inform value assessment in a holistic way [FDA, 2018], their involvement can often be hindered by challenges including information asymmetry, difficulties with recruitment in rare diseases, and methodological/measurement challenges (e.g. limited ability to operate smart devices) that can limit participation due to their condition. Moreover, multiple aspects, including nature of the disease, may have an impact on PEx preferences. Weights to value such elements have not yet been described in value assessment frameworks. Spinal Muscular Atrophy (SMA) Type II-III., autosomal recessive neuromuscular, rare disease [D' Amico et al., 2011], as a case study demonstrates the complexity of different perspectives of patients, when selecting a treatment or novel treatment for a rare disease.

**OBJECTIVES** This research aims to compare the importance of conventional value vs. additional PEx, from young adult SMA patients' perspective and reveal which are the most important elements of the patient experience to be considered in value assessment.

**METHODS** Patients were recruited through Children's Hospital Colorado between November/December 2021. Altogether 9 SMA type II-III young adults were included (6 male and 3 female). All patients participated voluntarily and gave consent before entering the research. A two-day online workshop applied a combined quantitative (coin allocation) and qualitative (moderated group discussion) approach to explore the importance of conventional/clinical trial endpoints (e.g. standardized motor scale outcomes) that are utilized in the approval of SMA treatments (referred to as conventional value elements) as well as additional PEx [McQueen et al., 2023]. During the first day's session the concepts and methodology were explained and patients were given the opportunity to familiarize themselves with the various value elements, ask questions, and provide feedback and comments. During the second day, a series of coin allocation and ranking exercises were carried out (quantitative part). First, participants were asked to prioritize between clinical trial endpoints and PEx by allotting 10 coins between the two categories. Secondly, they were asked to select the top three PEx they would consider when selecting a disease-modifying treatment for SMA, and again allocate 10 coins amongst them. Finally, a group discussion was facilitated to reveal the thought process behind decisions made in the coin allocation exercises (qualitative part). To break down and highlight patients' thought processes when considering conventional vs. PEx elements they were asked to elaborate on their everyday experiences regarding the accuracy of measures capturing their health status and improvement. The discussion was qualitatively analysed based on the workshop transcript. Within an interpretative approach: content analysis and text-mining tools (Voyant software and narrative analysis) were applied.

## RESULTS

**Quantitative results** Nine participants allotted 41.25% of their coins to conventional endpoints and 58.75% for PEx. Despite being drawn towards the patient centric approach, the participants acknowledged that "today's revolutionary disease-modifying treatments couldn't have been developed without old endpoints".

The top three ranked PEx were: 1) Ability to reach personal milestones taking up 46.25% of overall coins; 2) Patient's financial burden; and 3) Value of Hope/Balance timing of risks and benefits with 12.5% of overall coins each, respectively.

TOP 3 PEX	PATIENTS QUOTES
1. Ability to reach personal milestones 46.25 %	"Since beginning treatment I've had a mental and emotional shift rather than just trying to get through the day, I have allowed myself to think about the future."  "I always just wanted to die a little slower, that's the goal for everybody. Now I can really put more effort into myself."
2. Patient's financial burden 12.50 %	"I struggled with the financial aspect, because of insurance. Here I am receiving millions of dollars of <i>x drug</i> in my body and I haven't paid a penny for that. I can weigh the non-medical cost and if I do have to pay out of pocket expenses, but you take away insurance and none of this becomes possible at all."  "I have multiple insurances to cover everything, but then also talk about depth and out of pocket. On the flipside, if I am stronger, I am healthier, how does that start to reduce my medical costs?"
2. Value of Hope/Balance timing of Risks and Benefits 12.50 %	"I get the personal milestones, but I think hope and the bouncing of that risk you know, I think it's big to have that mental gain."  "I'm kind of like a realistic pessimist, I always had hope, that there will be a treatment for me. If we do find a cure in my lifetime it's not really gonna affect me much, cos the damage has been done by the SMA already."

Table 1. Top ranked PEx elements paired up with quotes from the discussion

**Qualitative results** Meaningful aspects of value assessment for patients articulated during group discussion:

- conventional endpoints are not sensitive enough to capture the subtle, but valuable changes in quality of life from patients' perspective.
- Without conventional measures there is no drug development. Backing up 40% of their allocation, participants articulated their understanding towards the use of currently used indispensable benchmarks offered by conventional endpoints.
- Participants allotted coins differently in the present with newer treatments available, then they would have back at the time of their initial diagnosis.
- The struggle with insurance and approval of treatments are at the forefront of patients' everyday lives and impacts their quality of life on several levels.
- As an additional outcome of the research itself, participants expressed feeling empowered and more hopeful towards a changing narrative in society.

*"It was so exciting for me to hear that this narrative is even happening. I think we would probably all agree, that this is what we've been frustrated with for a long time. I'm so grateful to have these treatments, but the conversation around what the treatments are doing hasn't always hit the mark. I just felt so excited for the research. We're starting to dive into what really matters to us!"*

**DISCUSSION** Our pragmatic approach proved to be effective in channelling information coming directly from patients into value assessment without putting too much cognitive burden on the participants. At the same time, it resulted in meaningful patient engagement through recognition and partnership during the process. The extension of quantitative methods with an additional qualitative pillar, where patients get to reflect and discuss their thoughts and experiences has clear added value.

Another insight from this work, is that preferences of PEx may alter in accordance with the impact that a disease might have on an individual and correspondingly what treatments are available at the time. In similar previous work conducted by the authors, consisting of eight Neuromyelitis optica spectrum disorder (NMOSD) patients, participants ranked PEx elements as followed: 'Uncertainty about long-term benefits and safety', 'Patient experience related to treatment regimen' and 'Patient's financial burden', with a respective weight of 25%, 19.2% and 14.4% (out of total 100%) indicating that preferences of PEx criteria are likely to be disease specific. [McQueen et al. 2023] Different preferences of patients with distinct disease groups are likely to be driven by several factors e.g. the nature and progress of the disease, time passed since receiving diagnosis, age and life cycle of patients. Currently, there appears to be a strong emphasis on personal milestones and value of hope for SMA patients. This may be the result of developmental shifts (physical, mental and emotional) made possible by recent drug developments, and patients project this tendency into the future too.

Limitations of this study include a small sample size, which also results in a smaller text corpus (transcript of group discussion contains only 4,727 total words and 939 unique word forms), which did not allow for an in-depth content analysis.

**CONCLUSIONS** Our findings may contribute to improved disease outcomes by emphasizing the importance of including PEx not only in value assessment but also at the regulatory phase of development. Future research should work prioritize additional societal value elements in addition to conventional and PEx, to address remaining gaps in value assessment.

## REFERENCES

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