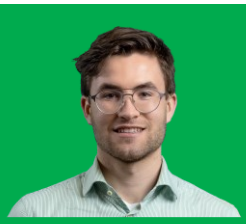

ATTRIBUTE DEVELOPMENT FOR A PATIENT PREFERENCE STUDY IN DUCHENNE MUSCULAR DYSTROPHY

Thomas Desmet, *Lauren Van Haesendonck, Liesbeth De Waele, Sam Geuens, Annelies Herman, Emma Heslop, Steven Simoens, Rosanne Janssens, Isabelle Huys*

BACKGROUND AND OBJECTIVES

Gene replacement therapy for DMD is **in phase 3 CTs.** Still a lot of uncertainties remain regarding the benefits, side effects, risks, and acceptability of patients to treatment alternatives. The aim is to:

- Study patients' **unmet needs**
- Elicit **treatment preferences** of **Duchenne patients** and **caregivers**
  - **What outcomes** are important?
  - **How important** are they?
  - What **trade-offs** are patients and caregivers willing to make?
- Measure **preference heterogeneity**

⇒ **Informing HTA and reimbursement decision-making**

METHODS

qualtrics XM

European Quantitative Survey

Preference elicitation method **Threshold technique**

Co-developed with **patients**, patient **organisations**, and **doctors**

Close interaction with advisory board

Educational

Informed by **previous qualitative research** and **literature research**

RESULTS

1 Attribute identification

- 30 characteristics from literature
- 26 characteristics from previous qualitative study
- Extra input from KOL and clinical trials

⇩

48 uniquely identified characteristics

2 Attribute selection

- **6 selection criteria** to select 6 characteristics for further attribute development
  - **29** did not fulfill all selection criteria
  - **19** fulfilled all selection criteria

3 Attribute description

- **Patient-friendly,** and easily **understandable**
- **As brief as possible**

4 Level development

- Scientifically sound levels
- Reflect **the state of the art** (clinical trials, literature, previous studies, input from KOL)

Example of a choice scenario

	A. Therapy A	**B.** Therapy B
Type of therapy	Corticosteroids	Gene therapy
Effect on life expectancy	12 years	14 years
Risk of life-threatening side effects related to the therapy	5% (5 out of 100 patients)	10% (10 out of 100 patients)
Years that ventilatory support is postponed	4 years	7 years
The number of years that you to maintain your current physical functioning	3 years	4 years
Years and number of patients in which that therapy has been studied	48 years; 20,000 patients	10 years; 100 patients

Please indicate your preferred choice:

Therapy A ○ Therapy B ◉

ABBREVIATIONS

DMD: Duchenne muscular dystrophy; CT: Clincial trial; KOL: Key opinion leader

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CONCLUSION

- This first phase revealed the **treatment outcomes** that have a **significant impact for DMD patients**.
- The **multi-stakeholder advisory board** has shown to be of great value together with **qualitative research results** to guide attributes and levels development.