



The PAVING II Interview study: Assessing Evolving Patient Preferences Regarding Gene Therapy for Haemophilia Patients

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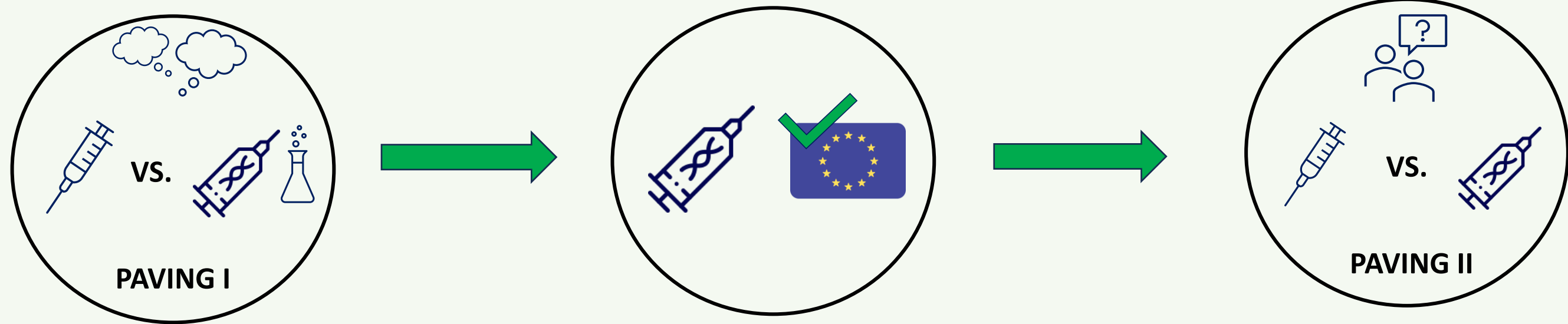
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BACKGROUND & OBJECTIVES



Aim: Assess the **evolution** of patient **preferences** regarding **gene therapy** (GT) since European Medicines Agency (**EMA**) **approval**.

METHODS

- Semi-structured **interviews** (n=14) with Belgian:
 - Haemophilia A patients (n=8)
 - Haemophilia B patients (n=4)
 - Haemophilia patients that received GT (n=2)



Ad verbatim transcription of interviews



Thematic **framework analysis** using Nvivo Software

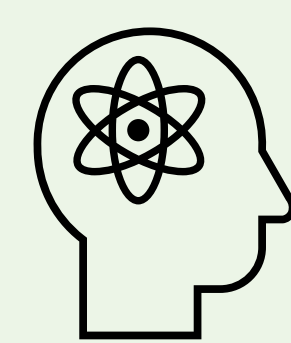
PRELIMINARY RESULTS

i) Participant characteristics

Characteristics	Percentage (%)
Age, > 40 years	50.0
Region, Flanders	85.7
Disease severity, severe	85.7
Joint damage, severe	57.1
Current treatment, prophylaxis	85.7
Treatment satisfaction, very satisfied	57.1
Bleeding frequency, < 2/year	78.6

ii) Comparing PAVING I with PAVING II

Baseline knowledge GT:



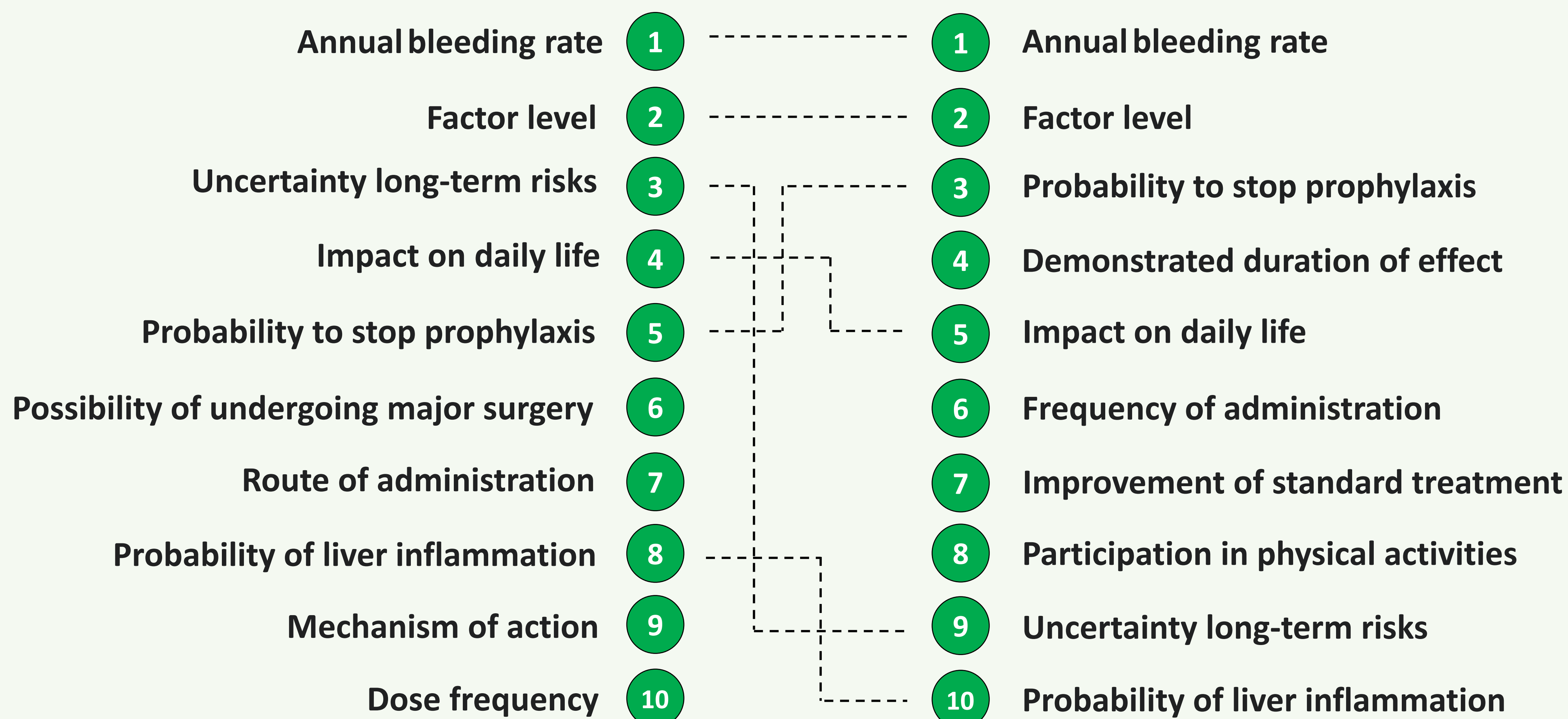
- Most participants had already **heard of GT**
- Self-reported **baseline knowledge** varied
- 42.9%** reported their knowledge as 'Reasonable'
- PAVING I:** slightly **more patients** who rated their knowledge as 'Good'

Willingness to receive GT:



- Compared to PAVING I:
 - Smaller** proportion were **very willing**
 - Larger** proportion were **willing or neutral**

iii) Treatment attributes of PAVING I vs. PAVING II



Experiences of patients who received GT

- **Improved QoL**
- **High motivation** (benefits > risks)
- **Corticosteroid treatment** (tough but manageable)
- **Newer non-factor therapies** (attractive alternatives)

ABBREVIATIONS

GT = Gene Therapy, EMA = European Medicines Agency, QoL = Quality of Life

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CONCLUSION

These preliminary findings indicate that the **main priorities** in treatment attributes **stayed the same** compared to PAVING I, but there are **some shifts** in less prioritized attributes. Overall, patients are **very satisfied** with current **standard of care**.