



Patient Preferences to Assess Value in Gene Therapies (PAVING) II Study for Hemophilia Types A and B*

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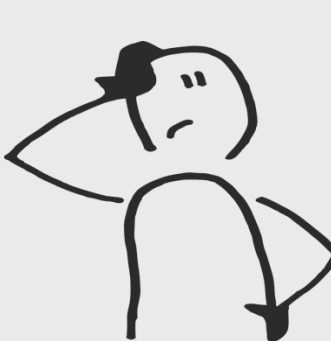
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BACKGROUND AND OBJECTIVES

Golden standard: **prophylactic factor replacement therapy (PFRT)**



+ **New gene therapies**
for hemophilia A: Roctavian®
for hemophilia B: Hemgenix® and Durevetix®



Germany, UK, Austria ✓
> HTA but uncertainties on long-term efficacy



> Patient preferences to aid in decision-making & demonstrate value

In continuation of original PAVING study: **to determine if preferences between PFRT and willingness for gene therapy changed after approval of gene therapies and treatment of certain patients.**

METHODS

Qualitative phase: semi-structured interviews

1

- 10-12 Belgian, adult patients with hemophilia (PWH) types A & B
- Open-ended questions, attribute ranking & case scenarios
- Nvivo and framework analysis

Quantitative phase: online survey & integrated educational tool

2

- Distributed among EU PWH
- Threshold technique: trade-offs between PFRT & gene therapies quantified as minimum acceptable benefit (MAB) in terms of 'Annual bleeding rate' (ABR), 'Chance to stop prophylaxis' (STOP), & 'Quality of life' (QOL)
- Analysis: interval regression models

RESULTS^x

- 1 Interviewed 20 PWH
- General **positive attitude**: very willing (40%, n=8) & willing (35%, n=7) to receive gene therapy
 - Most **important attributes**:
 - Annual bleeding rate
 - Factor levels
 - Uncertainty of long-term risks
 - Daily life impact
 - Possibility of stopping prophylaxis



- 2 117 survey entries (original PAVING, 2018)
- Substantial **preference heterogeneity**
 - MAB most influenced by time spent on educational tool
 - Most accepted **gene therapy profile** by 88% of PWH:
 - Zero bleeds/year (vs. 6 for PFRT)
 - 90% chance to stop prophylaxis
 - No impact on QOL
 - 10 years follow-up of side-effects (vs. 30 for PFRT)



PAVING II (2025):
SIMILARITIES OR DIFFERENCES ?



^x from original PAVING study: van Overbeeke et al. (2019 & 2020)

CONCLUSION

These outcomes could refine clinical or payer-initiated trials, inform managed entry agreements in decision-making and facilitate evaluations of gene therapies for hemophilia.