

# QUALITATIVE RESEARCH EVALUATING PATIENT PREFERENCE FOR HAEMOPHILIA THERAPY

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# DISCLOSURE FOR **WOLFGANG MIESBACH**

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Grant / Research Support	Bayer, BioMarin Pharmaceutical Inc., Biotest, CSL Behring, Chugai, Freeline, LFB, Novo Nordisk, Octapharma, Pfizer, Roche, Sanofi, Takeda/Shire, and uniQure
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Other	No relevant conflicts of interest to declare

Presentation includes discussion of the following off-label use of a drug or medical device:

<N/A>

## INTRODUCTION AND OBJECTIVES

- In the changing haemophilia treatment landscape, it is critical to understand which impacts and outcomes of treatment are important to people with haemophilia
- Qualitative research is the first step in understanding individual treatment preference drivers and risk tolerance for new gene therapies among people with haemophilia
- Here, we present results from concept elicitation and ranking of attributes in development of a discrete choice experiment designed to identify the key drivers of individual preference when selecting one haemophilia therapy over another

## METHODS

- Adult participants with severe haemophilia A (FVIII  $\leq 1$  IU/dL) were recruited via the National Hemophilia Foundation's community-powered registry in the US
  - Participants in gene therapy clinical trials were excluded
- Semi-structured, 60-minute concept elicitation telephone interviews were conducted with participants to collect treatment preferences
  - A combination of thematic and content analysis was used to identify themes and concepts that emerged from audio transcripts<sup>1</sup>
- Participants rated 15 predetermined treatment attributes on a 4-point scale from "not important (1)" to "very important (4)" and ranked attributes from most "important (1)" to "least important (15)"
- Data were analysed with descriptive statistics and mean (SD) ratings and rankings were calculated
  - Mean rankings were themselves ranked to provide final attribute rankings for the sample

## RESULTS

### Participant demographics and baseline characteristics

- Concept elicitation interviews were conducted with 20 participants with severe haemophilia A in the US

	N = 20
Age at enrolment, median (range), years	34.5 (20–57)
Race/ethnicity, n (%)	
White	14 (70.0)
Black	1 (5.0)
Asian	1 (5.0)
Hispanic	1 (5.0)
Other	3 (15.0)
Male sex, n (%)	20 (100)
Number of target joints, n (%)	
0	5 (25.0)
1	2 (10.0)
$\geq 2$	13 (65.0)
Other comorbidities, n (%)	
Arthritis	9 (45.0)
Depression/anxiety	4 (20.0)
Hepatitis B	1 (5.0)
Hepatitis C	5 (25.0)
Hypertension	4 (20.0)
Other	4 (20.0)
None	6 (30.0)

FVIII, factor VIII; HIV, human immunodeficiency virus; SD, standard deviation.

1. Joffe H, Yardley L. Content and thematic analysis. In: D. Marks and L. Yardley (eds) Research Methods for Clinical and Health Psychology. London: Sage. 2004: 56–68

## TREATMENT CHARACTERISTICS – THE ‘IDEAL’ TREATMENT

- Overall, 40.0% of participants spontaneously mentioned “reduction in bleeds” as an ideal treatment characteristic, increasing to 100% on probing

N = 20	Reduction in bleeds	Physical activities ability	Treatment frequency/ duration	Increase FVIII levels	Reduction in joint bleeds <sup>a</sup>	Mode of administration	Reduction in pain/joint pain	Reverse joint damage	Ability to have surgeries; safety of treatment; cure disease; avoid side effects; improve QOL <sup>b</sup>
Spontaneous, n (%)	8 (40.0)	1 (5.0)	7 (35.0)	2 (10.0)	1 (5.0)	6 (30.0)	4 (20.0)	2 (10.0)	1 (5.0)
Probed, n (%)	12 (60.0)	18 (90.0)	11 (55.0)	15 (75.0)	15 (75.0)	0	0	0	0
Total <sup>c</sup> , n (%)	20 (100.0)	19 (95.0)	18 (90.0)	17 (85.0)	16 (80.0)	6 (30.0)	4 (20.0)	2 (10.0)	1 (5.0)

<sup>a</sup>Some participants did not see the difference between bleeds and joint bleeds and considered them the same. <sup>b</sup>Participants mentioned each of these treatment characteristics spontaneously. <sup>c</sup>Not all treatment characteristics were probed for when discussing the “ideal” treatment.

Potential characteristics	Treatment characteristic rating task		Treatment characteristic ranking task	
	Mean (SD) <sup>a</sup>	Overall rating (N = 20)	Mean (SD) <sup>b</sup>	Overall ranking (N = 20)
Annualised bleeds	3.87 (0.52)	Very important	3.50 (2.31)	1 <sup>c</sup>
HRQOL impact	3.78 (0.73)	Very important	3.50 (2.91)	1 <sup>c</sup>
Annualised joint bleeds	3.93 (0.26)	Very important	4.25 (1.89)	2
Risk of long-term side effects	3.86 (0.36)	Very important	4.45 (2.76)	3
Long-term side effects related to integration	3.79 (0.42)	Very important	5.75 (3.29)	4
Predictability of treatment	3.33 (0.97)	Important	7.90 (3.82)	5
FVIII levels	3.33 (0.97)	Important	8.25 (4.46)	6 <sup>b</sup>
Length/duration of treatment	2.89 (0.88)	Important	8.25 (3.57)	6 <sup>b</sup>
Risk of short-term side effects	2.82 (0.88)	Important	9.05 (2.93)	7
Steroid use	2.82 (1.19)	Important	9.25 (2.23)	8
Shedding/double barrier contraception	2.95 (1.27)	Important	9.65 (3.48)	9
Potential to redose	2.76 (0.97)	Important	10.15 (2.64)	10
Treatment mode and frequency	2.39 (1.09)	Somewhat important	10.25 (3.92)	11
Additional doctor visits	2.26 (0.93)	Somewhat important	11.7 (3.10)	12
Alcohol abstinence	1.50 (0.99)	Somewhat important	14.1 (1.74)	13

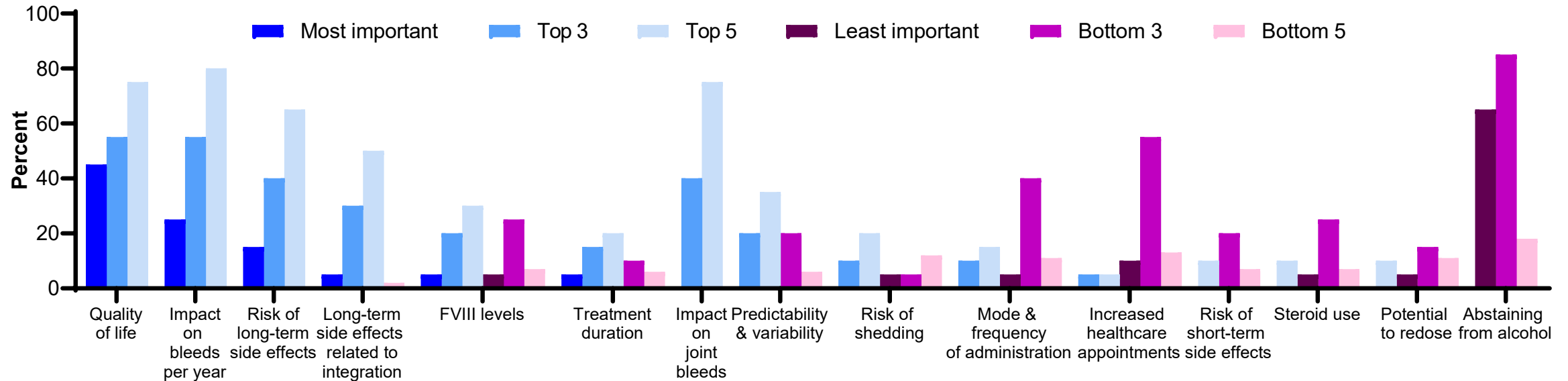
<sup>a</sup>Attributes were rated from 1 (least important) to 4 (most important). <sup>b</sup>Attributes were ranked from 1 (most important) to 15 (least important). <sup>c</sup>The average ranks led to an overall tied ranking for these attributes.

FVIII, factor VIII; HRQOL, health-related QOL; QOL, quality of life; SD, standard deviation.

## RATING & RANKING

- All characteristics were considered at least somewhat important
- Annualised bleeds and HRQOL were both rated as “very important” and overall tied for first place in the ranking
- The least important attributes by both rating and ranking were additional doctor visits and alcohol abstinence

## MODAL RANKING OF ATTRIBUTES



## CONCLUSIONS

- Overall, participants most frequently spontaneously mentioned that the “ideal treatment” would reduce the number of bleeds, have longer treatment duration, and would have an improved mode of administration (eg, pill)
  - When probed, participants deemed the ability to partake in physical activities, increased treatment duration, and increased FVIII levels as the most ideal treatment characteristics
- Participants ranked a reduction in annualised bleeds and an improvement in HRQOL as the most important treatment characteristics, followed by reduction in annualised joint bleeds and risk of long-term side effects
  - These attributes were also rated as “very important,” as were long-term side effects rated to integration
  - Additional doctor visits and alcohol abstinence were ranked as the least important treatment characteristics

FVIII, factor VIII; HRQOL, health-related quality of life.