Understanding the Real-World Experience of People with Haemophilia A Receiving Turoctocog Alfa Pegol (N8-GP): Results from a Patient Experience Survey

Eichler H[1], Nagao A[2], Waller J[3], Stuber A[4]

[1]Saarland University and Saarland University Hospital, Homburg/Saar, Germany, [2]Ogikubo Hospital,Tokyo, Japan, [3]Novo Nordisk, Zurich, Switzerland, [4]Braunwald, Switzerland

Objective

To understand the real-world experience of patients with haemophilia A (HA) receiving turoctocog alfa pegol (N8-GP)

Introduction

- Prophylactic factor VIII (FVIII) replacement therapy has been the standard of care for patients with haemophilia A (HA)[1]
- Owing to their relatively short plasma half-life (10-14 hours), standard half-life recombinant FVIII products require frequent intravenous injections, placing a substantial treatment burden on patients[2]
- Turoctocog alfa pegol (N8-GP) is an extended half-life factor VIII molecule requiring fewer injections
- N8-GP has been shown in clinical trials to be an effective treatment for HA[3,4] and consequently it is indicated for use in adults and children in the U.S., Canada, Japan and Switzerland, and for hemophiliacs of ≥12 years in the EU, for routine prophylaxis, on-demand treatment including control of bleeding episodes, and perioperative management
- However, real-world data on the benefits that N8-GP provide to a patient's daily life are limited

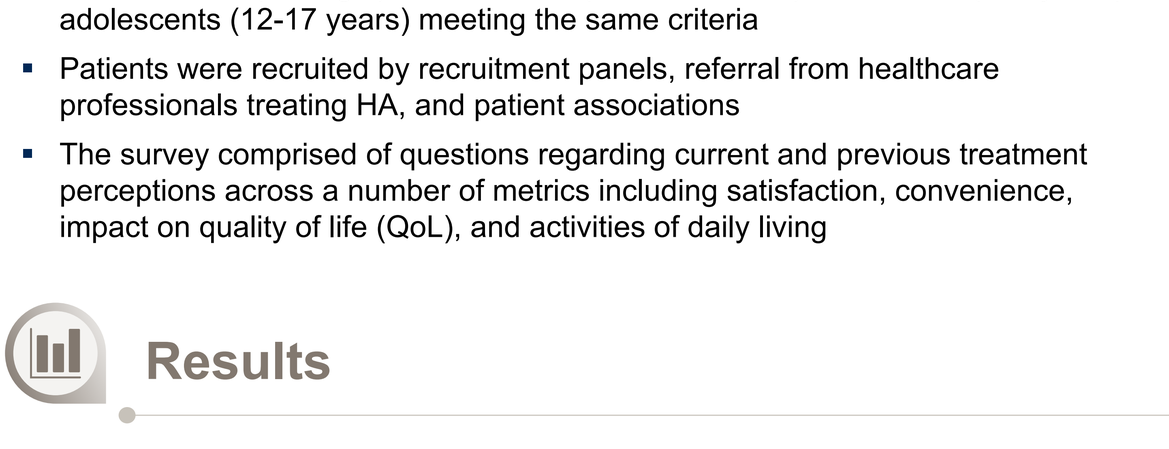
Methods

- A quantitative online survey was conducted in Germany, Italy, Portugal, Spain, the UK and the US, in patients (≥16 years) with HA and currently treated with N8-GP for >3 months (Portugal) or >6 months (other countries), and carers (≥18 years) of adolescents (12-17 years) meeting the same criteria
- Patients were recruited by recruitment panels, referral from healthcare professionals treating HA, and patient associations
- The survey comprised of questions regarding current and previous treatment perceptions across a number of metrics including satisfaction, convenience, impact on quality of life (QoL), and activities of daily living

Results

- In an interim analysis of 56 patients (98% male; mean age: 30 years; severity: 23% mild, 41% moderate,36% severe), comprising surveys from 43 patients and 13 carers, the mean duration of treatment with N8-GP was 0.8 years
- The proportion of patients reporting satisfaction with their treatment was higher with N8-GP (96%) compared to previous treatment (46%) (Figure 1)

Figure 1 ♦ Satisfaction with N8-GP and previous treatment
(% patients on 1-7 scale)



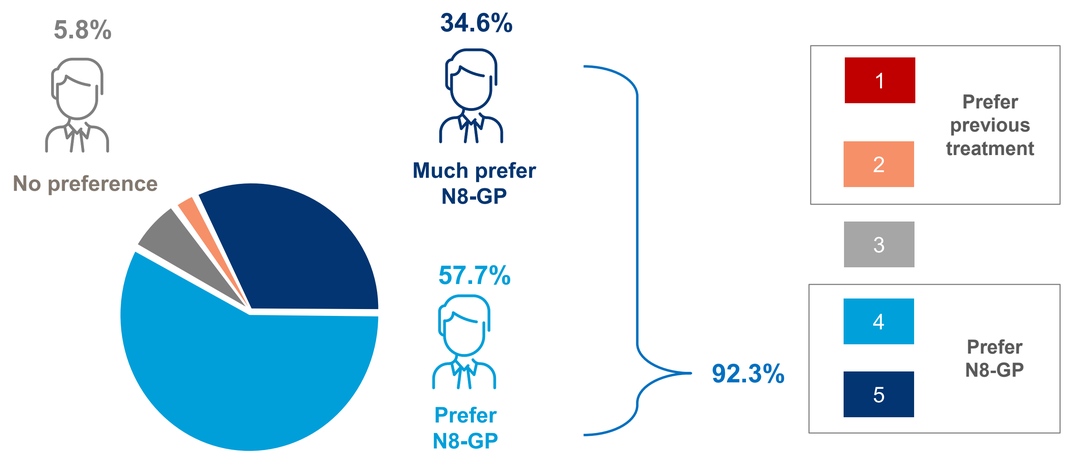
"My current treatment is still an injection, however seeing almost double the half-life out of a product that can also withstand temperatures of 103F was extremely impressive and improved my quality of treatment/life"

Adult N8-GP patient, U.S.

Results (continued)

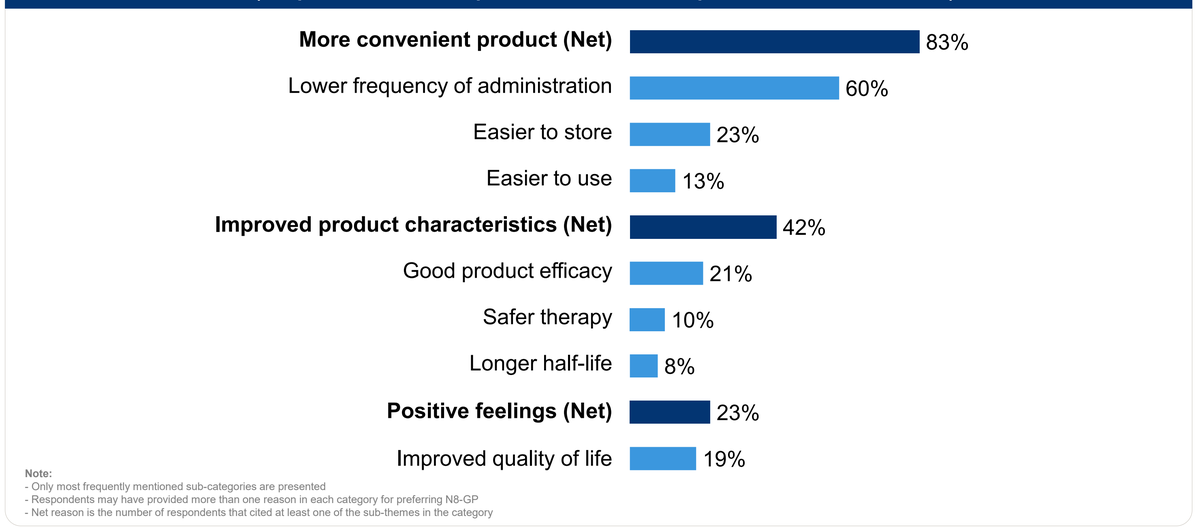
- Of the patients who previously received another treatment, 92% preferred N8-GP (Figure 2)

Figure 2 ♦ Preference for N8-GP vs. previous treatment
(% patients with a previous treatment)



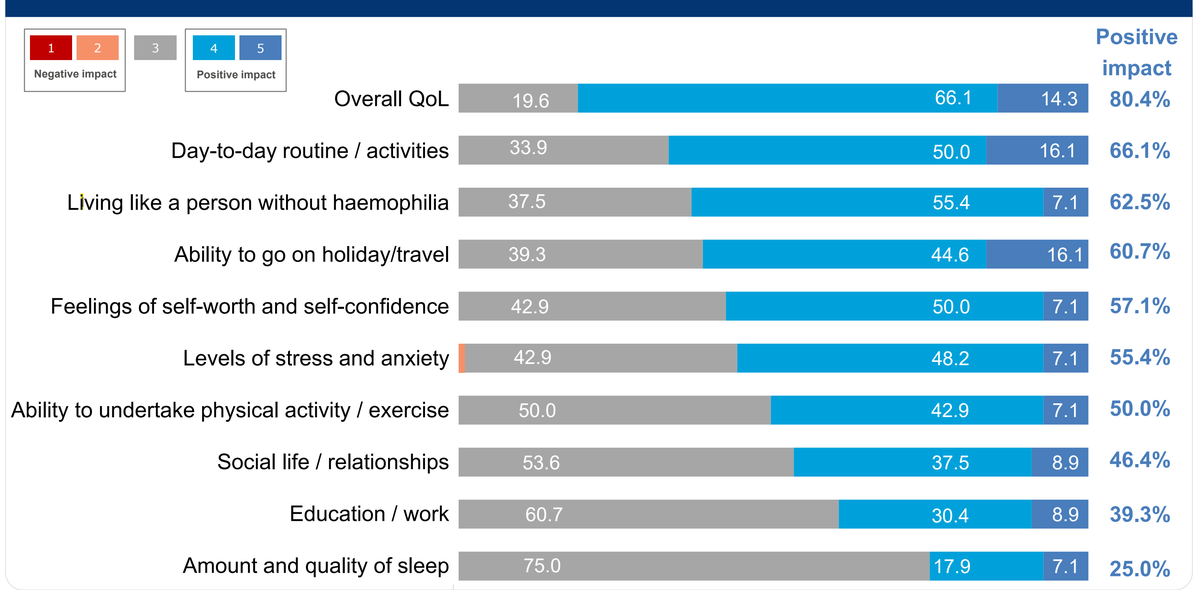
- Unpromoted reasons for preferring N8-GP included more convenient treatment (lower frequency of administration, easier to store and use), improved treatment characteristics (efficacious, good safety profile and longer half-life) and improved QoL (Figure 3)

Figure 3 ♦ Reason(s) for preferring N8-GP
(% patients who prefer N8-GP to previous treatment)



- Patients reported that N8-GP had a positive impact on many aspects of their lives, including overall QoL, day-to-day routine and independence to live like a person without HA (80%, 66% and 63% of patients respectively) (Figure 4)

Figure 4 ♦ Patient-reported impact of N8-GP on aspects of daily life
(% patients on 5 point scale)



"[N8-GP] made it much safer for me to do jobs, e.g. handyman tasks and/or repairs in the home or garden. Minor injuries (e.g. rose thorns) no longer lead to panic like before"

Adult N8-GP patient, Germany

- Although N8-GP has reduced the number of injections and provided self-reported benefits to daily life for many patients with HA, some unmet needs in patients' key hopes still exist
- Most patients still perceive their QoL to be less than an average person's and report experiencing emotional burden, presenting an opportunity for further improvement in treatment options

Conclusions

The advantages of lower frequency of injections, ease of storage, strong efficacy and improved QoL offered by N8-GP drive patient satisfaction and preference for it over previous treatment options among patients with HA

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CONFLICT OF INTEREST DISCLOSURE

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