

Physician Perception of Patient Unmet Needs and Optimism Towards Gene Therapy: Results from a Multinational Real-World Survey


Objective

This study explores physician perceived unmet need among their consulting haemophilia patients and their attitude towards gene therapy (GTx).


Conclusions

- Physicians looking forward (LF) to offering GTx to their patients indicated a higher proportion of their patients are negatively impacted by their haemophilia, specifically mortality risk.
- Physician perception of unmet need may contribute towards optimism for prescribing GTx.
- Recognition of patients’ unmet needs may help foster productive shared-decision making, facilitating identification of appropriate GTx candidates.

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Acknowledgements:

- Data was collected by Adelphi Real World via the Haemophilia III Disease Specific Programme™, an independent survey whereby all data are the intellectual property of Adelphi Real World. Pfizer Inc. subscribed to access this data source.
- Adelphi Real World and Pfizer Inc. would like to thank the physicians that participated in this survey.
- Authors do not assert that GTx “cures” haemophilia, but rather provides functional haemostasis correction for an extended period of time

Disclosures:

- ST, VM, LW, AK, JA and SP are employees of Pfizer Inc., New York, United States of America.
- EM, NB, HS, SL ,CB and KWC are employees of Adelphi Real World, Bollington, United Kingdom.

References:

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Introduction

- Haemophilia A and B are disorders that are characterised by recurrent bleeding episodes due to dysfunctional clotting factor VIII and IX respectively.
- Although currently available factor replacement and non-factor therapies aim to reduce bleeding rates, gene therapies (GTx) offer the opportunity for a single treatment to achieve functional haemostasis in haemophilia A and B. GTx restore factor VIII and IX levels, thus reducing bleeding episodes and providing safe and durable expression while avoiding the challenges of routine clotting factor replacement therapies⁽¹⁾.
- Physician perceptions of the impact of haemophilia on their consulting patients may influence their feelings and optimism towards offering GTx as a potential treatment solution.

Methods

- Data were drawn from the Adelphi Haemophilia III Disease Specific Programme™, a cross-sectional survey with retrospective data collection of physicians and their male haemophilia A and B patients in France, Germany, Italy, Spain, and the United Kingdom. Data were collected from September 2023 to April 2024.
- The DSP™ has been previously published, validated, and proven to be consistent over time^(2,3,4,5).
- Using medical charts and their clinical judgement for their next consulting male haemophilia A and B patients, physicians provided data on attitudes towards GTx, patient demographics, patient risk of mortality, and quality of life.
- Analyses were descriptive.

Limitations

- Physicians completed surveys for their next consecutively consulting patients , meaning more frequently consulting patients and those with a more severe disease state are more likely to be captured within the DSP™.
- Participation of physicians in the study was restricted by inclusion criteria but was voluntary and influenced by their willingness to participate.
- The DSP™ uses a cross-sectional study design, and therefore does not allow causal relationships to be identified. However, identifications of associations is possible.

Results

- Overall, 134 physicians provided data for 1078 haemophilia patients. Physicians that were looking forward to offering GTx (LF) provided data for 740 (69%) patients, and those who were not looking forward to offering GTx (NLF) provided data for 338 (31%) patients.
- Physician centres or hospitals that were set up to administer gene therapy can be seen in **Figure 1**.
- Of enrolled patients, 77% had haemophilia A and 23% had haemophilia B (**Table 1**).
- Physicians LF reported 50% of their patients were suitable candidates for GTx (n=558), compared to 39% of physicians NLF (n=210).
- Physicians reported numerous rationales regarding the use of gene therapy for their suitable candidates, namely, to cure the patient's haemophilia (NLF 54%, LF 43%) (**Figure 2**) .
- Physicians LF and NLF reported that 9% and 2% of their patients requested the treatment, respectively.
- Physicians LF reported 75% of their patients had increased risk of mortality due to haemophilia, compared to 41% reported from physicians NLF (**Table 2**).
- LF and NLF physicians reported that 43% and 29% of patients experienced difficulty performing activities due to their haemophilia. Physicians LF and NLF reported 30% and 20% patients respectively had difficulty taking part in sports/hobbies due to haemophilia (**Figure 3**).

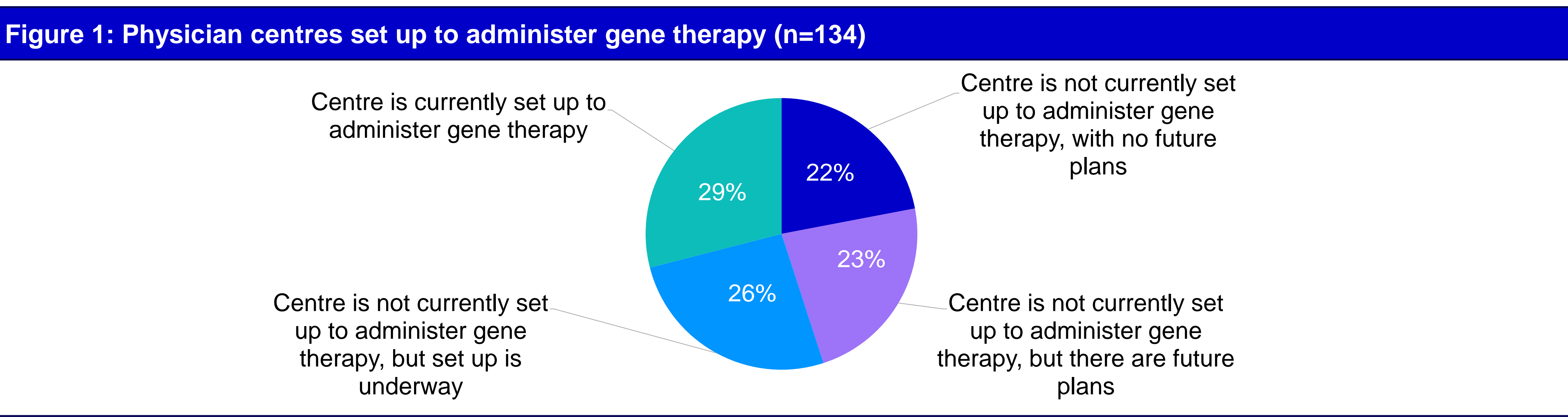


Table 1: Patient demographics			
	Overall n=1078	LF n=740	NLF n=338
Patient age			
Mean (SD)	27.91 (15.53)	28.26 (14.58)	27.15 (17.43)
Ethnic Origin, n (%)			
White	796 (94%)	552 (93%)	244 (96%)
Patient BMI			
Mean (SD)	23.62 (4.27)	23.71 (3.85)	23.43 (5.07)
Haemophilia type			
Haemophilia A	835 (77%)	583 (79%)	252 (75%)
Haemophilia B	243 (23%)	157 (21%)	86 (25%)
Inhibitor Status, n (%)			
Never had inhibitors	936 (87%)	642 (87%)	294 (87%)
Previous inhibitors	86 (8%)	53 (7%)	33 (10%)
Current inhibitors	56 (5%)	45 (6%)	11 (3%)

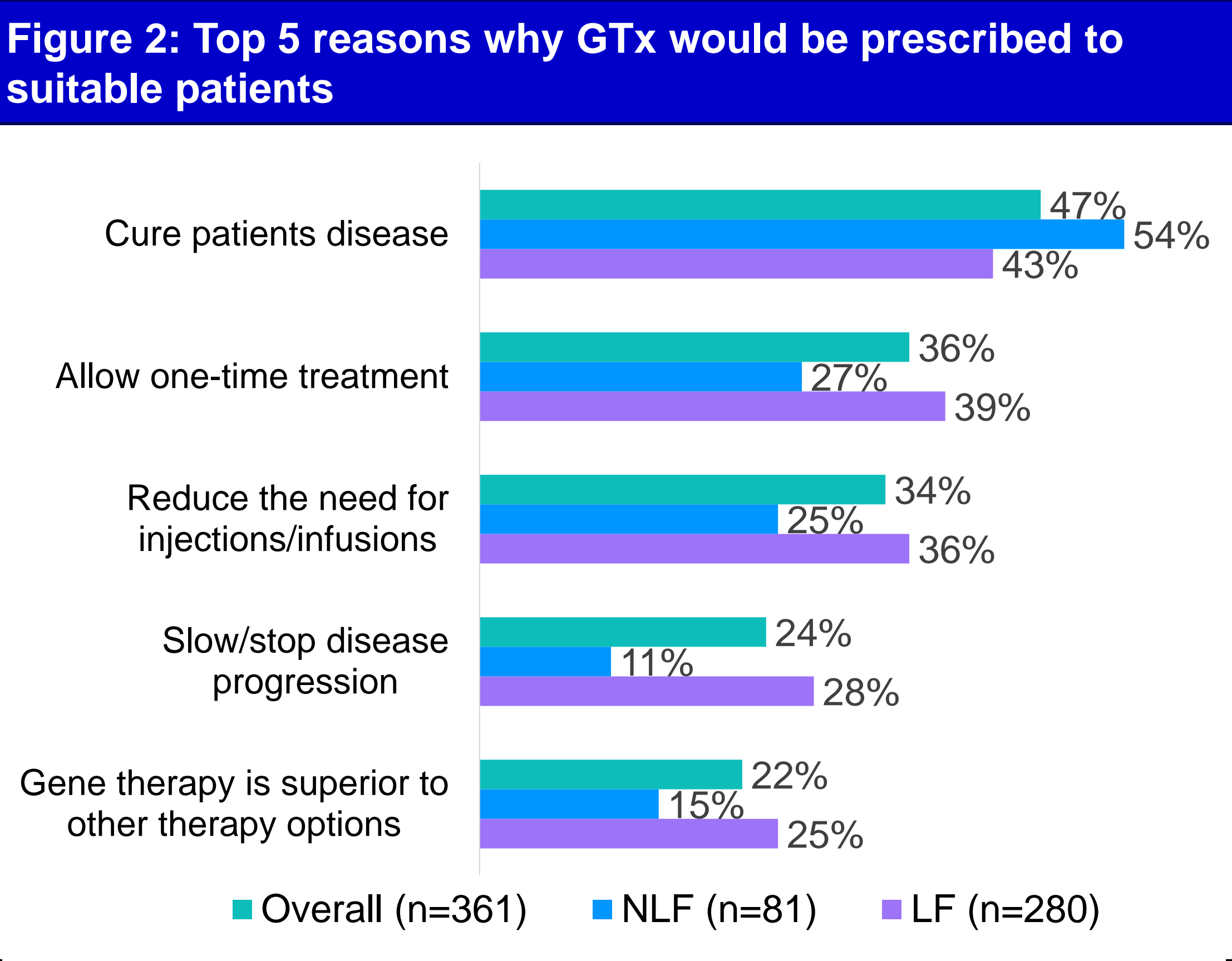


Table 2: Patient mortality risk			
	Overall n= 1078	LF n= 740	NLF n=338
A little/slightly, n (%)	474 (44%)	373 (50%)	101 (30%)
Moderately, n (%)	195 (18%)	164 (22%)	31 (9%)
Significantly, n (%)	26 (2%)	20 (3%)	6 (2%)
Not at all, n (%)	383 (36%)	183 (25%)	200 (59%)

