

Divergent Pathways: Comparative Logistical and Perceptual Barriers to Gene Therapy Adoption in Hemophilia Versus Hemoglobinopathies in the United States

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Introduction:

Gene therapy represents a paradigm shift in hematology, offering a potentially curative, one-time treatment for inherited blood disorders. Yet despite simultaneous regulatory progress across hemophilia, sickle cell disease (SCD), and beta-thalassemia, clinical uptake remains uneven. While hemoglobinopathy programs report increasing referral momentum, adoption in hemophilia lags behind. Understanding these divergences is essential for optimizing access and implementation across indications. The aim of this study is to compare physician-reported logistical, perceptual, and systemic barriers influencing gene therapy adoption across hemophilia, sickle cell disease, and thalassemia, using cross-indication data from Spherix Global Insights (2024–2025) and expert perspectives from field hematologists.

Methods:

Mixed-method analysis integrating quantitative data from hematologists treating ≥5 patients with each condition (n=197), including referral likelihood, perceived candidate volume, and operational barriers, qualitative insights from 17 in-depth physician interviews and expert commentary, thematic synthesis of perceptual and infrastructural determinants affecting gene therapy decision-making.

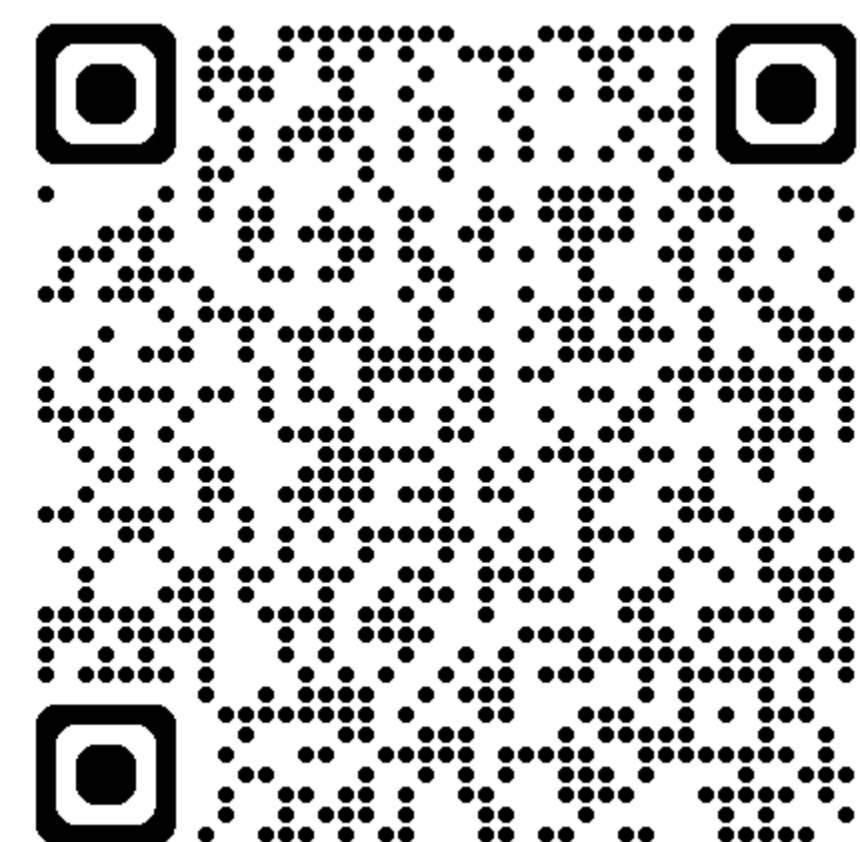


Figure 1

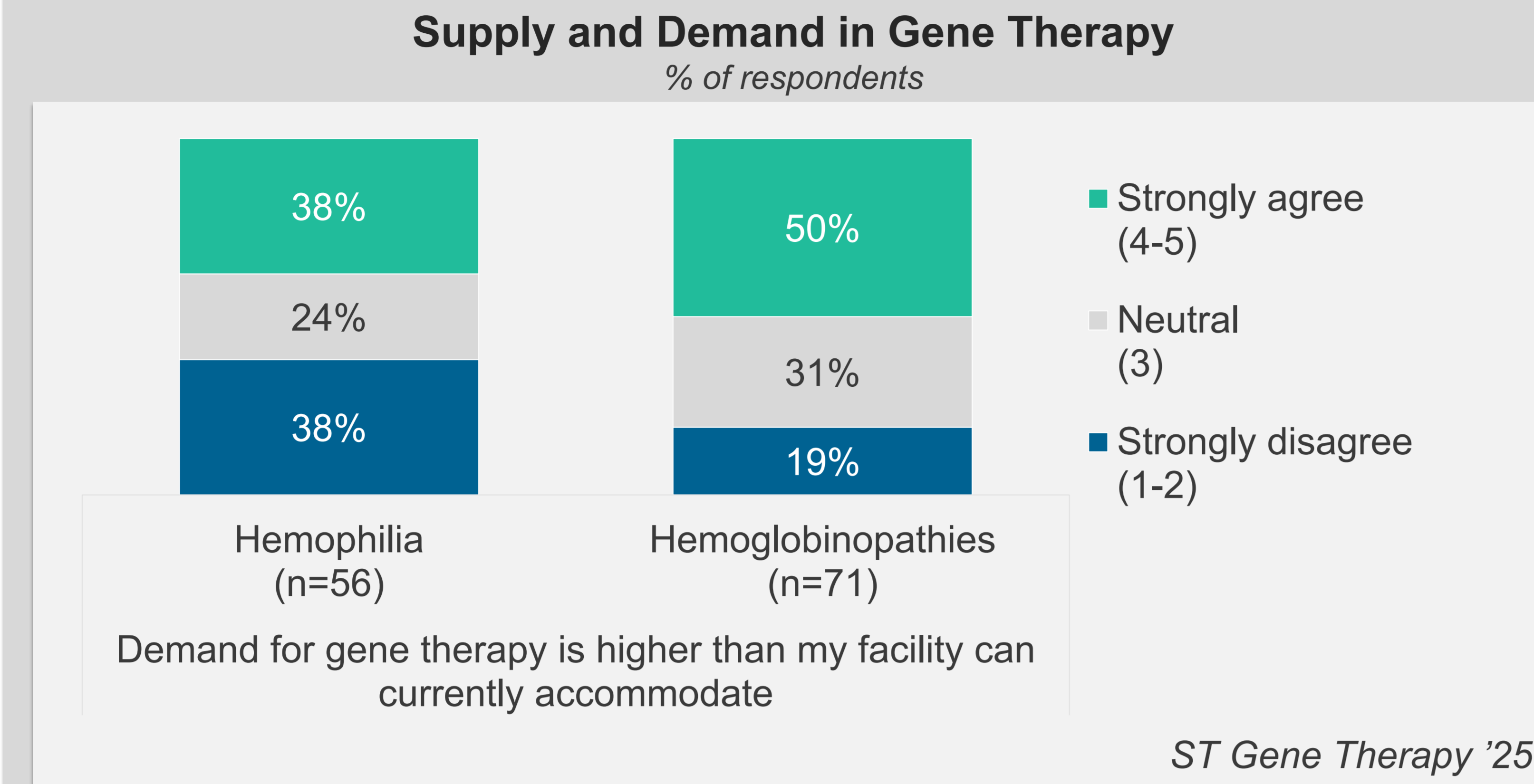
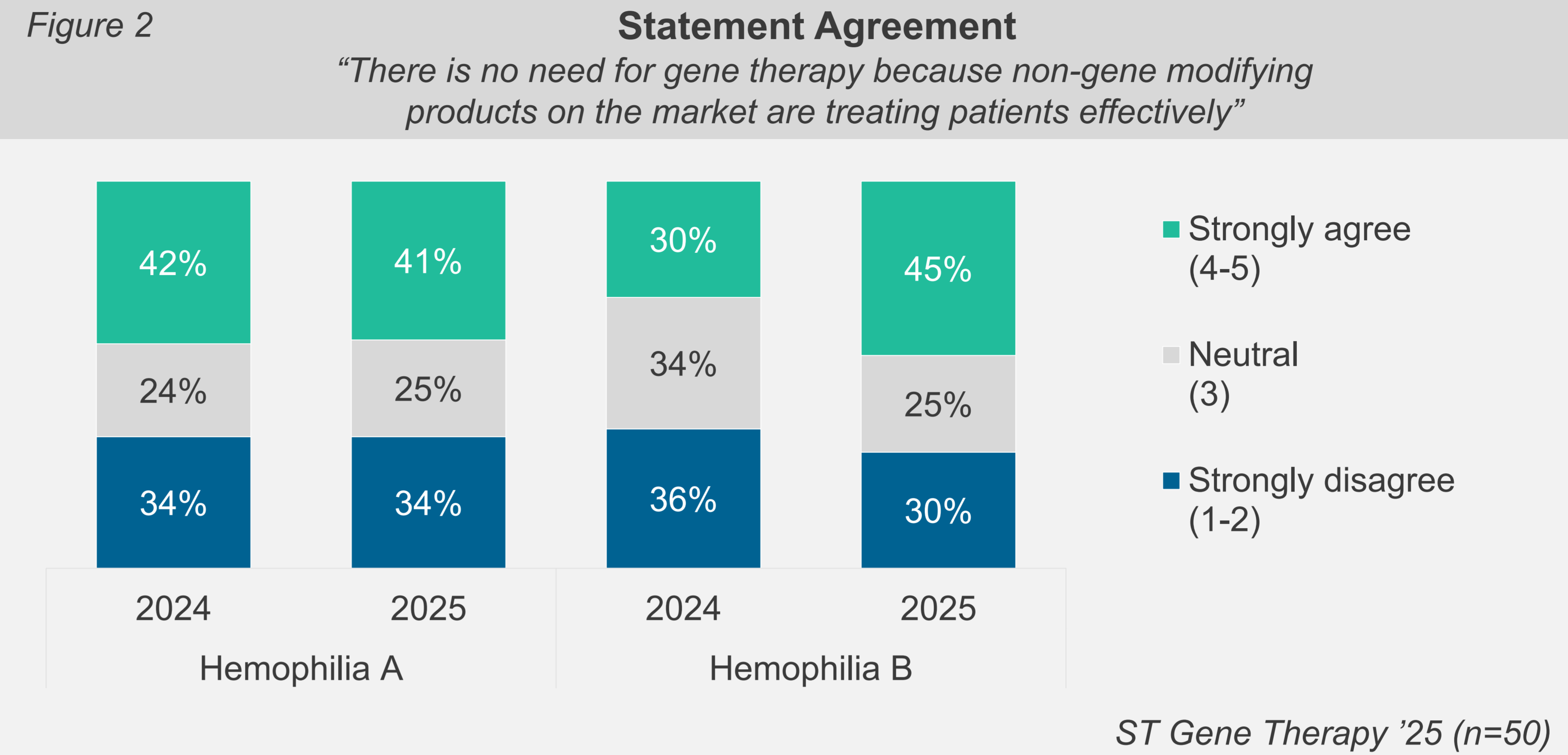


Figure 2



Results:

Across conditions, 83% of hematologists agreed that the efficacy of gene therapy outweighs long-term risks. However, logistical and perceptual divides emerged. In hemoglobinopathies (SCD, thalassemia), adoption constrained primarily by systemic logistics—prior authorization, coordination of services, and payer readiness (Fig. 4). 82% of surveyed hematologists cited institutional capacity as limiting uptake (Figs. 2,5). Patient motivation, caregiver engagement, and established transplant infrastructures facilitate readiness despite administrative bottlenecks. In hemophilia, barriers skew toward psychological and clinical inertia. Nearly half (45%) of physicians reported diminished urgency due to effective non-gene-modifying therapies (Fig. 1). Logistical complexity (immunosuppression, monitoring) and institutional hesitancy persist, with 43% citing staff burden as a deterrent to referrals (Figs. 3,4). Cost was a dominant constraint across all indications, perceived as more prohibitive in hemophilia due to single-patient contracting and center certification demands (Figs. 6,7).

Figure 4

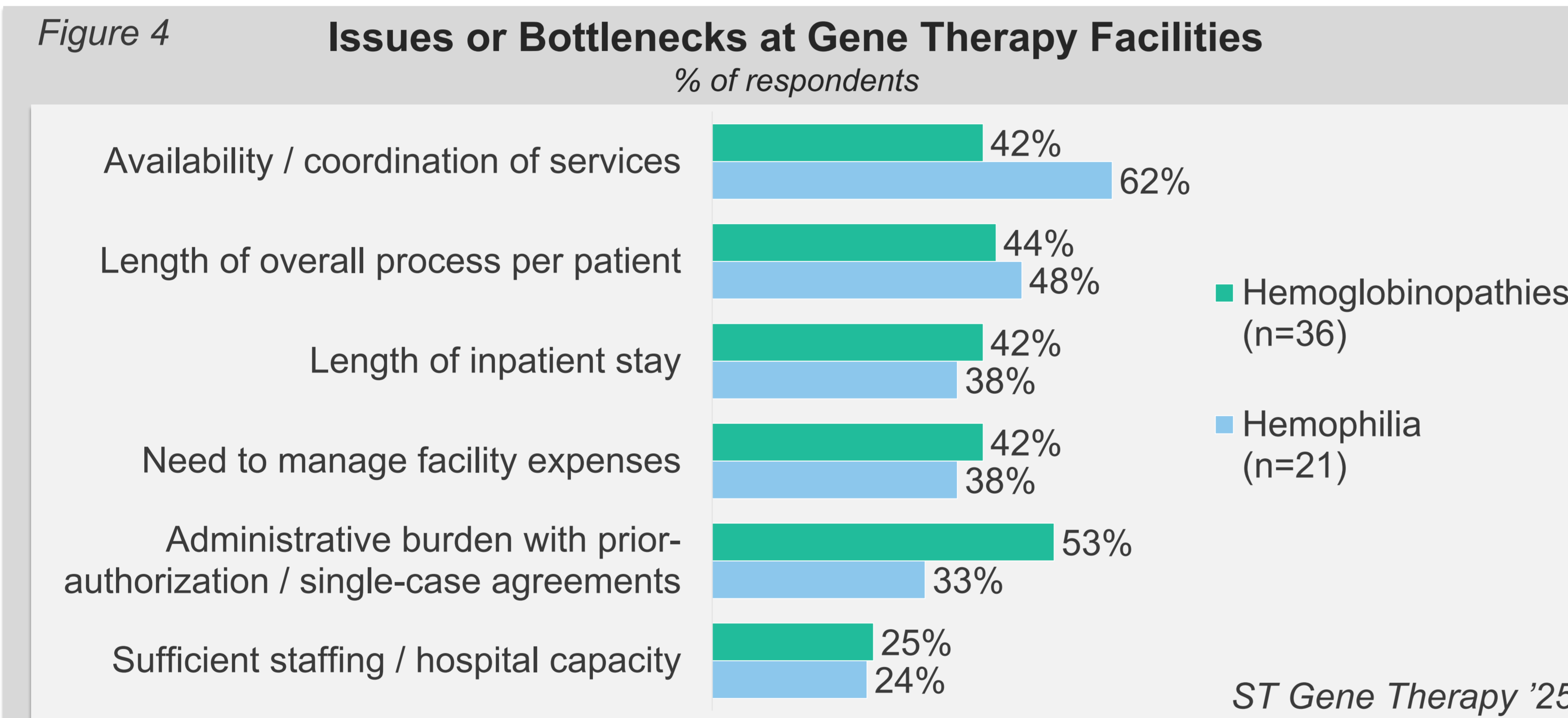
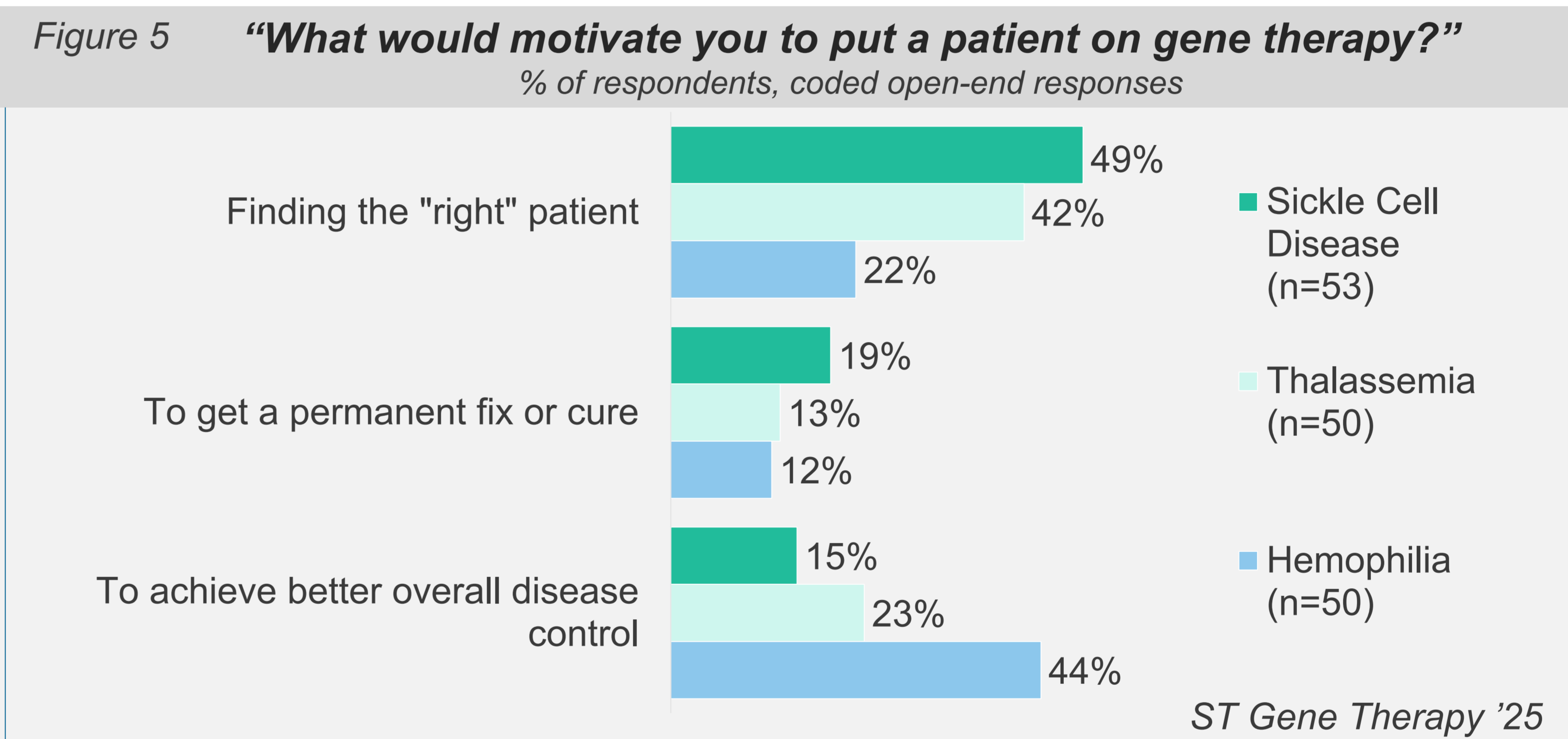


Figure 5



Conclusions:

While hemoglobinopathy gene therapy adoption is slowed by logistical hurdles, hemophilia faces deeper perceptual and operational resistance—stemming from therapeutic alternatives, infrastructure immaturity, and physician caution. Realizing equitable access requires disease-specific strategies: for hemophilia, focus needs to center on institutional enablement with re-education. The forthcoming CMS Cell and Gene Therapy Access Model may alleviate economic barriers but will not alone bridge the perceptual divide between these patient communities.

Figure 6

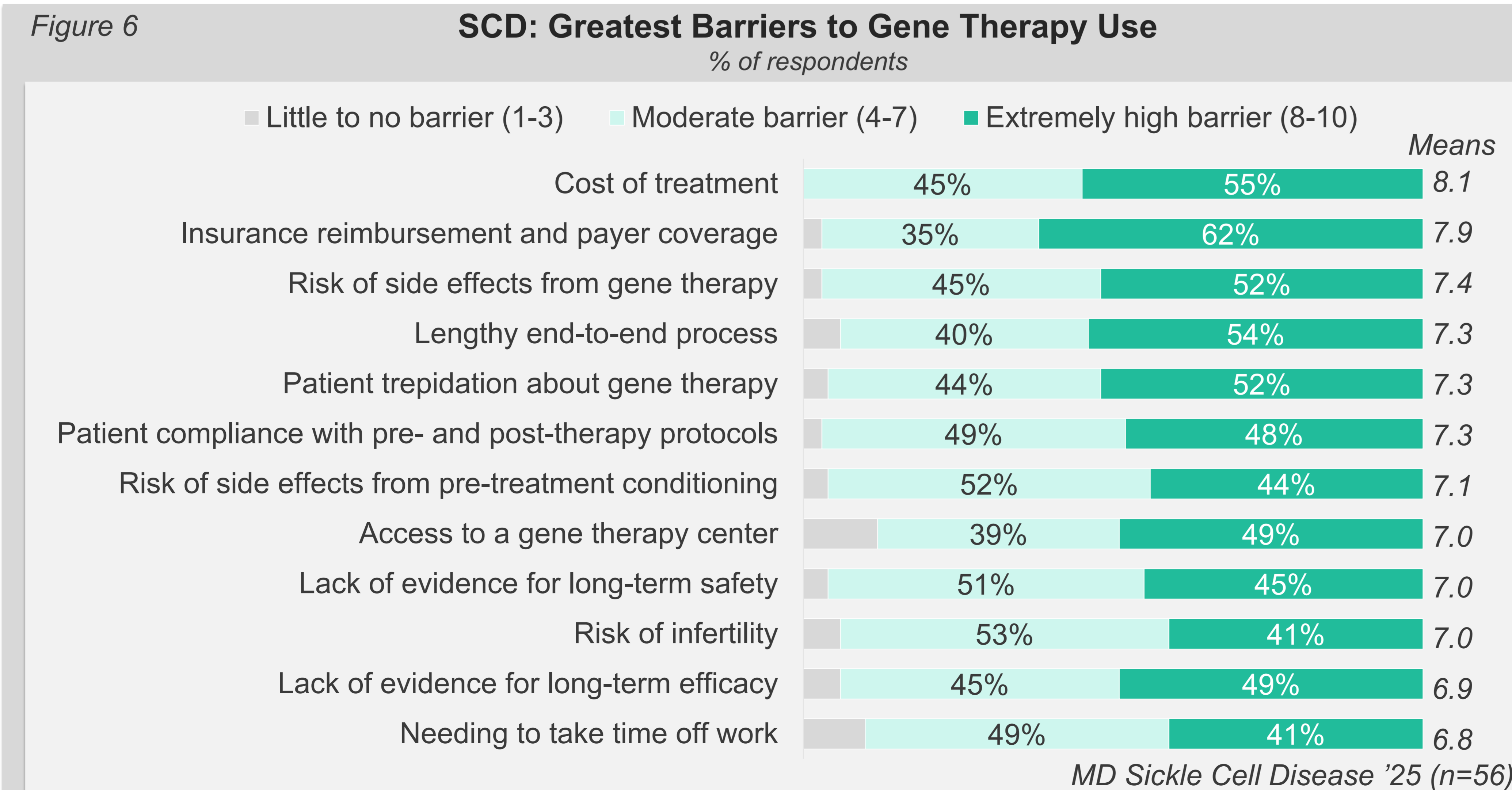
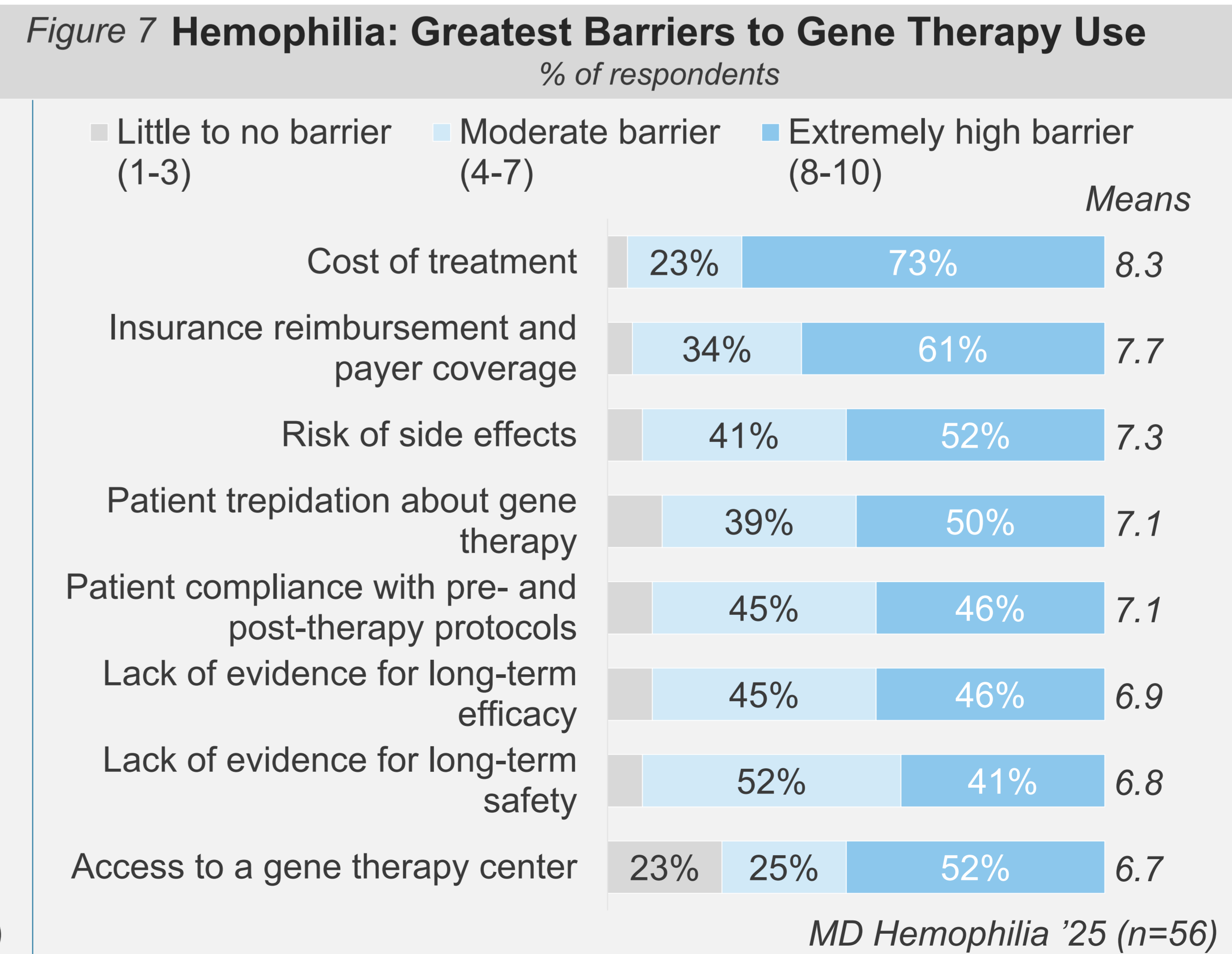


Figure 7



Disclosures:

Spherix Global Insights is an independent market intelligence firm and has received no industry funding to conduct and report on this study.

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