Title: Advancing Hemophilia Care: Insights from U.S. Hematologists on Novel Therapies and Clinical Practice Trends

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Background/Introduction:
Hemophilia A and B are X-linked recessive bleeding disorders resulting from deficient or absent clotting factors VIII and IX, respectively. Recent therapeutic advancements are moving beyond traditional factor replacement toward innovative approaches, including gene therapies and non-factor agents such as fitusiran and Mim8. This study reports survey data on U.S. hematologists' current clinical practices and evolving expectations for the treatment of hemophilia A and B.

Methods: Data were collected across four U.S. studies: (1) an online survey of 50 hematologists (July 16–August 12, 2024); (2) eight qualitative interviews with a subset of survey participants (August 2024); (3) an online survey of 31 hematologists (December 3–7, 2024); and (4) an online survey of 46 hematologists (March 4–10, 2025). All data were aggregated and analyzed by the Spherix analytics team.

Results: Nearly all respondents cited a moderate to high unmet need for new treatment options, with 60% identifying an extremely high unmet need for hemophilia A and 68% for hemophilia B. Fifty-six percent reported that hemophilia remains extremely challenging to manage, noting that 34% of their patients are not optimally controlled with current therapies. Key management challenges include selecting the most appropriate treatment (30%), ensuring patient adherence (28%), and securing insurance coverage (20%), particularly for newer therapies.

Forty-five percent of hematologists reported modifying their approach to hemophilia A management within the past year, primarily through increased adoption of emicizumab and efanesoctocog alfa, both associated with high physician satisfaction. In contrast, only 29% had changed their management of hemophilia B, and reported satisfaction with both standard and extended half-life factor products was lower. While 86% of physicians believe genetic therapies will significantly alter the treatment landscape, few have yet prescribed gene therapy.

Physicians expressed a desire for new therapies offering sustained efficacy, substantial reductions in annual bleed rates, and meaningful improvements in pain and joint damage. On average, they expect new agents to achieve a 50% reduction in uncontrolled or joint bleeds.

For hemophilia A, the highest prescribing interest was in fitusiran (24%) and Mim8 (22%), followed by marstacimab (14%) and concizumab (10%). Among hemophilia B treatments, interest in fitusiran (54%) slightly exceeded marstacimab (46%). Respondents estimated that 46% of their hemophilia A and 47% of their hemophilia B patients could be candidates for fitusiran, with 57% intending to prescribe it within three

months of its approval. Early interest is driven by its applicability to patients with and without inhibitors.

Conclusion: Despite ongoing management challenges, newer treatment options promise improved efficacy and administration, with physicians anticipating broad patient eligibility and rapid adoption to enhance care quality.