

Consensus On Barriers And Unmet Needs In Hemophilia Care In Brazil: A Modified Delphi Study

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BACKGROUND

Hemophilia is a rare, inherited X-linked bleeding disorder characterized by a deficiency or dysfunction of coagulation factor VIII (hemophilia A) or factor IX (hemophilia B), resulting in a lifelong tendency to spontaneous or trauma-related bleeding, particularly into joints and muscles.¹

For decades, the cornerstone of hemophilia management has been replacement therapy with plasma-derived or recombinant clotting factor concentrates, administered either on demand or as regular prophylaxis. More recently, the therapeutic landscape has expanded substantially with the introduction of extended half-life factor products, non-factor therapies and rebalancing agents targeting natural anticoagulant pathways.²

Despite therapeutic advances, hemophilia care in Brazil still relies on the prophylactic treatment with clotting factor concentrates for most patients and continues to face significant challenges related to treatment burden, access barriers, suboptimal adherence, and residual disease.^{3,4}

OBJECTIVES

To explore the perceptions and achieve consensus among Brazilian hematologists regarding unmet needs, intrinsic (individual-related) and extrinsic barriers (organization of care and current therapeutical options) to optimal care of hemophilia A and B in the context of the Unified Health System (SUS), in Brazil, as well as desired features of novel technologies.

METHODS

- A modified Delphi panel was conducted with a convenience sample of **12 hematologists** from specialized public health centers across **all geographic regions of Brazil**.
- The process comprised **three rounds conducted between October and December 2025**: two asynchronous rounds using anonymized online questionnaires followed by a structured synchronous online consensus meeting focused on unresolved items.

- The statements were developed in line with the findings of a targeted literature review conducted as a first step. Electronic searches were carried out in PubMed (via MEDLINE) and LILACS (via the Virtual Health Library), by using keywords “hemophilia”, “treatment”, “unmet needs” and “Brazil, covering articles published up to April 2025.

- The questionnaire was structured into five thematic domains: unmet needs according to hemophilia type; treatment-related aspects including adherence and residual disease burden; extrinsic and intrinsic barriers to optimal care within the SUS; and priority attributes of emerging technologies (Figure 1).

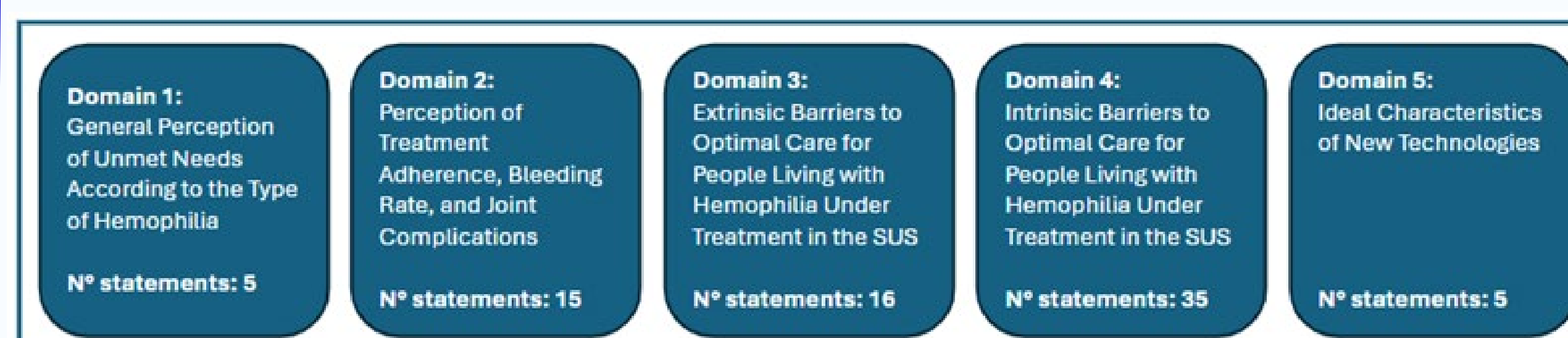


Figure 1. Domains addressed in the Delphi panel.

RESULTS

Rate of consensus

Of the **39 statements**, **23 (58,97%)** reached consensus after three rounds. **Persistent unmet needs** were identified for all clinical profiles, including **patients with and without inhibitors**. There was agreement on consolidating clinical protocols into a single national document (83.3%).

Key extrinsic barriers

- Key extrinsic barriers included long travel distances (83.3%), social vulnerability (83.3%), limited access to rehabilitation (91.7%) and acute care (83.3%), insufficient specialized centers (90%), failures in referral systems (90%), and lack of specialized emergency support (100%), scarcity of trained professionals (91.7%), poor care coordination (83.3%), and delayed adoption of new therapies (91.7%).

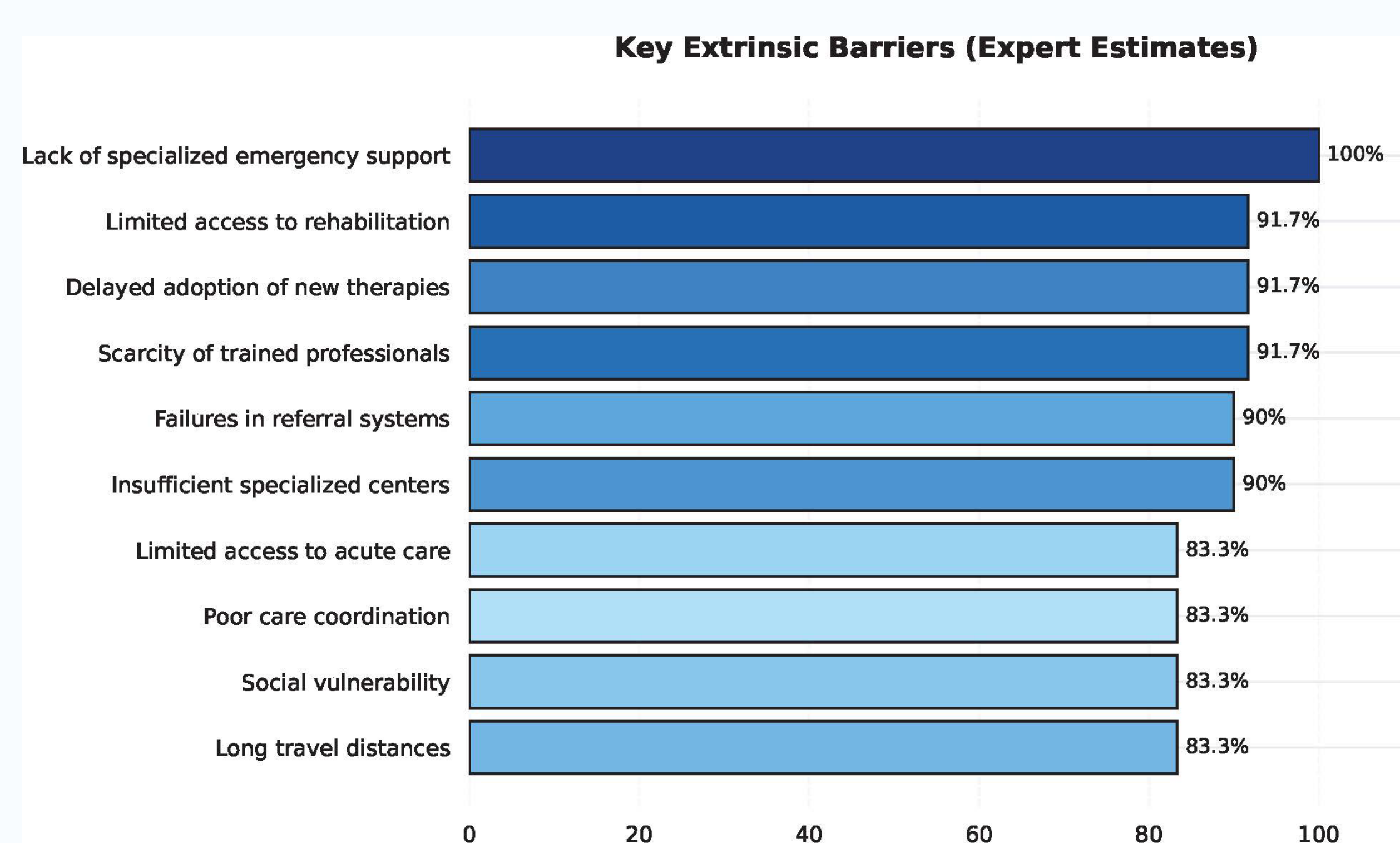


Figure 2. Key Extrinsic Barriers to Hemophilia Care (% Agreement)

Key intrinsic barriers

Intrinsic barriers included venous access difficulties in children (100%), lack of social support, infusion-related discomfort, mental health issues (80–91.7%), challenges in transitioning to self-infusion (91.7%), and functional limitations in older adults (83.3%). (Figure 3)

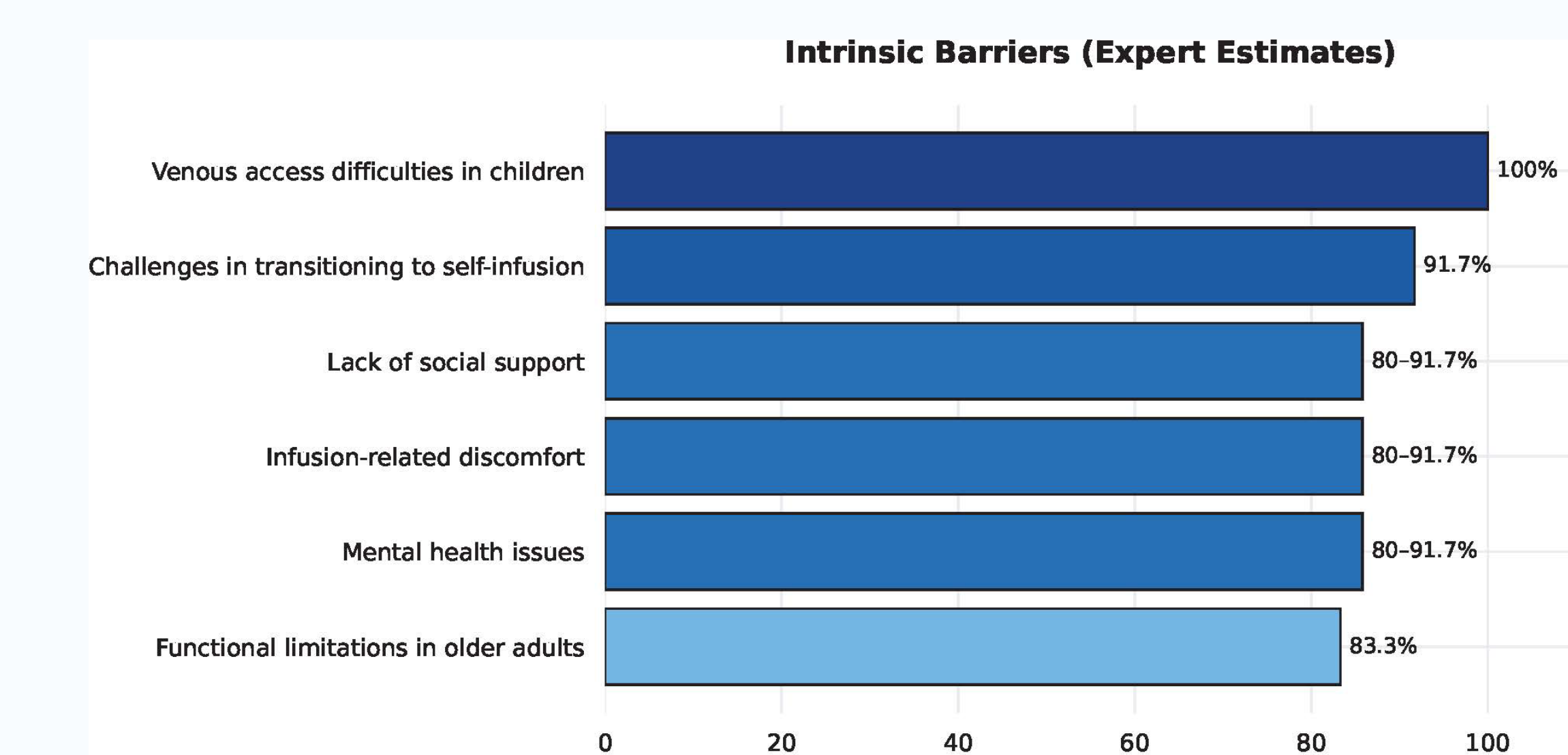


Figure 3. Key Intrinsic Barriers to Hemophilia Care (% Agreement)

Desired features of new technologies

Desired features of new technologies included **subcutaneous administration (100%)**, **reduced dosing frequency (100%)**, and **no need for laboratory monitoring (83.3%)**. Conclusion: High consensus among specialists highlights priority barriers and unmet needs in hemophilia care in Brazil, as well as attributes of innovative therapies to guide health policy.

DISCUSSION

Our findings indicate that hemophilia care in Brazil remains constrained by a combination of systemic limitations and treatment-related burden experienced by patients throughout their lives.

Experts consistently highlighted extrinsic barriers related to access, organization of services, and workforce preparedness, suggesting that even within a universal health system such as the SUS, continuity and quality of care are unevenly distributed.

At the health system level, the lack of integration between specialized hemophilia centers and general health services emerged as a critical weakness.

In parallel, intrinsic patient-level barriers related to treatment burden were identified as persistent obstacles to optimal outcomes. Frequent intravenous administration, difficulties with venous access, and the cumulative demands of lifelong prophylaxis were viewed as key factors negatively affecting adherence, particularly during adolescence and adulthood. These challenges are consistent with a life-course perspective, in which treatment demands interact with changing social, psychological, and occupational circumstances. Reduced adherence over time may partially explain preventable bleeding episodes and progressive morbidity, even when effective therapies are available.

Experts' consensus around desired characteristics of new treatment options, including easier administration and reduced dependence on venous access, reflects a recognition that technological innovation can play a role in mitigating these interconnected challenges.

CONCLUSION

This expert consensus highlights persistent unmet needs and major structural and individual barriers to optimal hemophilia care in Brazil. The results support health system reorganization, improved care integration, and prioritization of innovative therapies that reduce treatment burden and may improve adherence and outcomes within the SUS.

DISCLOSURES

This study was sponsored by Pfizer, which commissioned IQVIA for its execution.

JL, FI, and BW are employees of IQVIA, which received funding from Pfizer for the conduct of this study. DP, MA, AKN, RFA, and AD are current employees of Pfizer.

Ethical approval was not required for this study. All participants provided electronic informed consent prior to their inclusion in the study.

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