National Hemophilia Foundation Convenes Diverse Community Voices to Define an Actionable National Research Blueprint for Inherited Bleeding Disorders

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**Abstract**

**Background:** An ambitious initiative underway in the inherited bleeding disorder (IBD) community aims to create a national research blueprint that can help accelerate research progress and address important gaps in care, particularly within rare disorders and underserved populations. Led by the National Hemophilia Foundation (NHF), the effort is defined by input from across the community, including research leaders, patient subject matter experts (SMEs), caregivers, allied health professionals and specialists, and industry. Two foundational principles of the blueprint are that a) it must deliver on key issues that most significantly impact the lives of those affected by an IBD, and b) the priorities defined are relevant and actionable in order to provoke real and lasting changes in the care paradigm.

**Methods:** To ensure the blueprint accurately reflects the most pressing needs from across the community, NHF has enlisted the support of diverse segments of the population throughout the process.

**Listening:** NHF coordinated a comprehensive, community-wide listening exercise, including focus groups, virtual listening sessions, and consumer and professional surveys, to collect insights that have shaped and guided the blueprint development.
**Engagement:** Representatives from across the IBD community have been enlisted to participate in the development process through enrollment in one of six interdisciplinary working groups (WGs), each focusing on broad themes raised during the listening exercises. (Table 1) In total, 164 individuals are participating in the WGs, including chapter representatives, allied healthcare providers, researchers, federal partners and other IBD organizations. Each WG also features experts outside the IBD community who can introduce innovations from other fields. Finally, each WG includes the participation of subject matter experts (SMEs), individuals affected by bleeding disorders who provide personal perspectives on the value and potential impact of the proposed research priorities. NHF is actively supporting these groups with regular engagement, guidance, and recommendations while encouraging robust dialogue to distill critical priority research areas.

To ensure the blueprint is well defined and actionable, NHF has devised a rigorous development and refinement process.

**Feasibility Assessment:** Together with expert advisers, NHF has defined a set of feasibility criteria to help the WGs address potential opportunities based on three key areas: (see Figures 1 and 2)

- Feasibility assesses the difficulty in answering the proposed question, including required expertise, infrastructure, and resources.
- Impact estimates the change we can foster through the priority.
- And risk considers the challenges of the research question, such as the risk/benefit ratio for novel strategies and any ethical considerations.

Each research priority or model is scored based on these areas, and the combined evaluation will determine how they are included and prioritized in the blueprint.

**Summit:** Upon completion of the WG assessments, NHF will bring the community together for a State of the Science (SOS) Research Summit, September 12-15, 2021, during which each WG will summarize their recommendations for live, interactive discussion. During each session, panels will discuss the recommendations and collect feedback from community participants, as well as from remote participation groups comprised of representatives from underserved segments of the population.

**Results:** The discussions from the working groups and Research Summit will be consolidated into a series of manuscripts and published as a community-driven national research blueprint in mid-2022. The voices of individuals affected by IBDs have been the central driver in this process, from the listening activities and WGs to the planned SOS, and the community will continue to champion the efforts defined in the blueprint.

**Conclusions:** This initiative represents an opportunity to catalyze impactful change in the treatment of IBDs. To ensure its success, NHF has methodically enlisted broad community involvement and...
guide research plans for the IBD community. Our hope is that this blueprint will help shepherd advances in care that could fundamentally redefine the experience of living with these disorders.

**Figure 1**

<table>
<thead>
<tr>
<th>Working Groups:</th>
<th>Priority Areas of Focus:</th>
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| 1. Research priorities for Hemophilia A & B | • Across the spectrum of disease  
• Understudied areas including women |
| 2. Research priorities for von Willebrand Disease, platelet dysfunction and other mucocutaneous inherited bleeding disorders | • Across genders and phenotypes  
• Diagnostics and therapeutics |
| 3. Research Priorities for ultra-rare bleeding disorders | • Across genders and phenotypes  
• Diagnostics and Therapeutics |
| 4. Research priorities for health of women & girls and persons with the potential for menstruation | • Von Willebrand Disease  
• Platelet Disorders  
• Sex and gender-specific biology and IBD phenotype |
| 5. Diversity, equity & inclusion health services research & implementation science | • SOC Implementation  
• DE&I health services research  
• Telehealth and delivery network development  
• Communications |
| 6. Facilitating priority research in the IBDs community | • Infrastructure  
• Resource Procurement/ Development  
• Workforce Development |

**Figure 2: Questions Informing Feasibility Assessment**

- How easy or difficult will it be to answer the question? For clinical research, can the target sample size be achieved? For translational research, how translatable is the novel strategy? For basic research, are the necessary tools available?
- What expertise/multidisciplinary partnerships are required? Which community partner should undertake the research?
- What are the required infrastructure and resources? What exists/needs to be created? What are the funding sources?
- How will the answer impact standard of/quality of care? How will the answer impact the therapeutic paradigm?
- What is the applicability of the data generated in other IBDs?
- What are the training opportunities associated with doing this research?
- How acceptable will the proposed study be to patients, investigators, funders?
- How acceptable is the risk/benefit ratio of the proposed basic/translational pathway for a novel strategy?
- Are there ethical considerations associated with answering these research questions?
### Disclosures

**Witkop:** Teralimmune, Inc.: Consultancy. **Recht:** Sanofi: Consultancy; Octapharma: Consultancy; Novo Nordisk: Consultancy; Foundation for Women and Girls with Blood Disorders, Partners in Bleeding Disorders: Speakers Bureau; American Thrombosis and Hemostasis Network: Current Employment; Oregon Health & Science University: Current Employment; Genentech: Consultancy; Hema Biologics: Consultancy; CSL Behring: Consultancy; Takeda: Consultancy; uniQure: Consultancy; Catalyst Biosciences: Consultancy; Pfizer: Consultancy; Kedrion: Consultancy. **Valentino:** Spark: Ended employment in the past 24 months.

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Potential Articles of Interest

National Hemophilia Foundation Enlists Diverse Patient Voices to Inform a National Research Blueprint for Inherited Bleeding Disorders
Maria E Santaella et al., Blood

Pain in the Bleeding Disorders Community: Patient and Caregiver Perspectives
Buckner, Blood, 2016

Novel Approach to and Results of Genetic Analysis of 3000 Hemophilia Patients Enrolled in the MyLifeOurFuture Initiative
Johnsen et al., Blood, 2016

Experts join forces to create first-of-its-kind Blueprint for Youth Suicide Prevention
May Lau et al., AAP News, 2022

Reducing Harm, Supporting Recovery: a partnership and evidence-informed approach to developing the new Irish health led, National Drug Strategy
Catherine Comiskey, Harm Reduction Journal, 2020

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