

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

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Background: An ambitious initiative underway in the inherited bleeding disorder (IBD) community aims to create an actionable national research blueprint that can help accelerate research progress and address important gaps in care, particularly within rare disorders and underserved populations. Led by NHF, the effort is defined by input from across the community, including not only research leaders, but patient subject matter experts (SMEs), caregivers, allied health professionals and specialists, and industry. Two foundational principles of the blueprint are that: 1) It must deliver on key issues that most significantly impact the lives of those affected by an IBD, and 2) 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 were enlisted to participate in the development process through enrollment in 1 of 6 interdisciplinary working groups (WGs), each focusing on broad themes raised during the listening exercises (Table 1). In total, 15 individuals participated as a steering committee and 164 in the WGs, inclusive of chapter representatives, allied healthcare providers, researchers, federal partners, and other IBD research organizations. Each WG also featured experts outside the IBD community who introduced innovations from other fields and subject matter experts (SMEs) individuals affected by bleeding disorders) who provided personal perspectives on the value and potential impact of the proposed research priorities. NHF actively supported 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/Impact/Risk Assessment Tool: 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).

Each research priority or model was scored based on these areas, and the combined evaluation will determine how they are included and prioritized by each WG for ultimate inclusion in the blueprint.

Summit: Upon completion of the WG assessments, NHF brought the community together for a virtual State of the Science (SOS) Research Summit, September 12-15, 2021, during which each WG summarized their recommendations in a live, interactive discussion. During each session, panels discussed the recommendations and collected feedback from community participants, as well as from remote participation groups (RPGs) comprised of representatives from underserved segments of the population (see Table 2). NHLBI support made the inclusion of the critical input from these groups possible (Award Number R13HL158209).

Table 1. Summit Working Groups

Working Groups:	Priority Areas of Focus:
1. Research priorities for Hemophilia A and B	<input type="checkbox"/> Across the spectrum of disease <input type="checkbox"/> Understudied areas: women; non-severe disease
2. Research priorities for von Willebrand disease, platelet dysfunction, and other mucocutaneous IBDs	<input type="checkbox"/> Across genders and phenotypes <input type="checkbox"/> Diagnostics and therapeutics
3. Research priorities for ultra-rare IBDs	<input type="checkbox"/> Across genders and phenotypes <input type="checkbox"/> Diagnostics and therapeutics
4. Research priorities for health of women and girls and persons with the potential for menstruation	<input type="checkbox"/> Von Willebrand disease <input type="checkbox"/> Platelet disorders <input type="checkbox"/> Sex and gender-specific biology and IBD phenotype
5. Diversity, equity and inclusion, health services research and implementation science	<input type="checkbox"/> SOC implementation <input type="checkbox"/> DE&I health services research <input type="checkbox"/> Telehealth and delivery network development <input type="checkbox"/> Communications
6. Facilitating priority research in IBDs	<input type="checkbox"/> Infrastructure <input type="checkbox"/> Resource procurement/development <input type="checkbox"/> Workforce development

Figure 1. Feasibility Schema

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 assessment** considers the challenges of the research question, such as the risk/benefit ratio for novel strategies and any ethical considerations

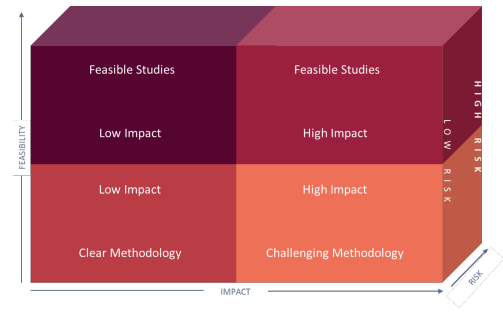


Figure 2. Questions Informing Feasibility Assessment

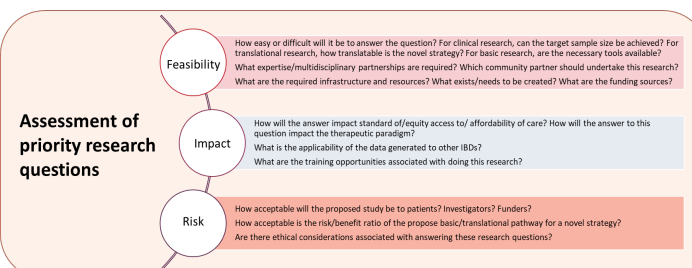


Table 2. Remote Participation Groups

Chapter/ Hemophilia Treatment Center	Representative	Priority Area	Focus	Occurred
Hemophilia of South Carolina	Suzanne Martin	Hemophilia	Black/African American	Yes, in person
New York City Hemophilia Chapter	Jeremy Griffin	Hemophilia	Aging	Yes, virtual
Lone Star Bleeding Disorders Foundation	Melissa Compton	VWD or women, girls, and persons with the potential to menstruate	Hispanic women	Yes, virtual
Virginia Hemophilia Foundation	Kelly Waters, LCSW, MSW	Women and Girls and Persons with the Potential to Menstruate	Women	Yes, virtual
Bleeding Disorders Alliance of Illinois	Bob Robinson	Ultra-rare	LBGTQ	Yes, virtual
Hemostasis and Thrombosis Center of Nevada/ Nevada, National Hemophilia Foundation	Dr. Amber Federizo Betsy VanDeusen	Hemophilia, VWD, Platelets and other Mucocutaneous IBDs, Workforce, Infrastructure	Aging, Rural/ Geographically Challenged	Cancelled
Hemophilia Foundation of Michigan	Susan Lerch	Hemophilia, Ultra-Rare	Black/African American, Women	Cancelled
Central Ohio Chapter, National Hemophilia Foundation	Wendy Perkins	Hemophilia	Amish	Cancelled

Results: NHF's SOS Research Summit attracted 887 attendees who joined 21 sessions lead by 43 speakers and totaled 2,279 unique session views. There were 5 RPGs – more are planned in the future.

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. 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 community will continue to champion the efforts defined in the blueprint.

Conclusion: This initiative presents an opportunity to catalyze impactful change in the treatment of IBDs. To ensure its success, NHF has methodically enlisted broad community involvement and driven a rigorous prioritization process in order to identify specific and actionable topics that will help guide research plans for the IBD community. This research blueprint may help shepherd advances in care that could fundamentally redefine the experience of living with IBDs.

Disclosures: **MW:** Teralmmune, Inc.: Consultancy. **LAV:** Spark: Ended employment in the past 24 months. **MR:** Takeda: Consultancy, Research Funding; uniQure: Consultancy, Research Funding; Sanofi: Consultancy; Pfizer: Consultancy; Octapharma: Consultancy; Novo Nordisk: Consultancy; Kendrion: Consultancy; HemaBiologics: Consultancy; Genentech: Consultancy; Catalyst Biosciences: Consultancy; CSL Behring: Consultancy; Foundation for Women and Girls with Blood Disorders, Partners in Bleeding Disorders: Membership on an entity's Board of Directors or advisory committees, Speakers Bureau; Bayer: Research Funding; BioMarin: Research Funding; Grifols: Research Funding; LFB: Research Funding; Novo Nordisk: Research Funding; Pfizer: Research Funding; Sanofi: Consultancy, Research Funding; Spark: Research Funding.

National Hemophilia Foundation Enlists Diverse Patient Voices to Inform a National Research Blueprint for Inherited Bleeding Disorders



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Background:

The inherited bleeding disorder (IBD) community has witnessed significant advances in care, yet important gaps persist, particularly in rare disorders and underserved populations. An initiative spearheaded by the National Hemophilia Foundation (NHF) and shaped by the patient community aims to accelerate progress through a National Research Blueprint. The blueprint is being designed to identify and guide research priorities toward those areas that most significantly impact the lives of individuals affected today and articulate clearly defined opportunities to make the greatest impact for the future.

Methods:

NHF has enlisted individuals with IBDs as subject matter experts (SMEs) to guide this initiative by elevating the most pressing issues affecting them today and informing expert discussions on actionable research priorities for the future. SME insights have been collected through listening sessions, a cross-community survey, and participation in multi-disciplinary working groups. See Table 1.

The NHF State of the Science Research Summit (SOS) in September 2021 addressed the input of the working groups and also featured patient vignettes to illustrate today's unmet needs and contextualize the research priorities identified to address them.

As part of this interactive Summit, SMEs from traditionally underrepresented patient populations were also enlisted to participate in remote participation groups (RPGs) with the goal of soliciting input to further tailor the research priorities to the needs of these populations. The RPGs were comprised of individuals with bleeding disorders or their caregivers who represented specific populations by race/ethnicity (for example African American and Hispanic individuals, etc.). Within each group, NHF included SMEs with diverse experiences based on their IBD, barriers related to access to care, gender, and sexual orientation. See Table 2.

During each live session, the moderated RPGs participated in the live summit, discussed the expert dialogue, and shared real-time perspectives and comments about how the content addressed, or not, their specific community needs. The expert SOS panel had the chance to address their comments. The commentary from these sessions will be included in the National Research Blueprint to define the research path forward for the community. The RPGs are also expected to be reconvene in Spring 2022 to review and improve upon the opportunities identified in the blueprint.

NHLBI support made the inclusion of the critical input from the RPGs possible (Award Number R13HL158209).

Table 1. Six Summit Sessions

Working Groups:	Priority Areas of Focus:
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Results:

NHF enlisted broad and diverse community support to ensure the blueprint accurately represents the opportunities to create meaningful and lasting impact for individuals with IBDs. In total, 42 patients and caregivers participated in listening sessions; 125 contributed to the community survey; 15 participated in the Summit working groups and approximately 200 were enlisted for the remote participation groups. The themes addressed during the SOS reflect the input provided by the SMEs and health professionals.

Conclusion:

Actively soliciting the patient community's views is central in our process to advance research in IBDs. By enlisting the participation of historically underserved community segments, this effort aims to address some of the most persistent and pressing issues affecting the IBD community today. Specific insights from the RPG participation in the Summit will be included in the presentation. This blueprint, which will guide the U.S. research community, could help fundamentally redefine the experience of diverse populations living with these disorders.

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