

# NHF Builds a Community-Driven National Research Blueprint for Inherited Bleeding Disorders

Michelle Witkop, DNP, National Hemophilia Foundation; Michael Recht, MD, PhD, American Thrombosis and Hemostasis Network; Donna DiMichele, MD, Consulting, LLC; Leonard Valentino, MD, National Hemophilia Foundation



## Background:

- The inherited bleeding disorders (IBD) community has witnessed significant advances in recent years thanks to novel therapeutic advances and technologies and improved diagnostic proficiency.
- Yet important gaps persistent, particularly for those with rare disorders and underserved populations, including women with IBD.
- A new initiative led by the National Hemophilia Foundation (NHF) and shaped by the voices of the patient community is underway to address this gap.
- Our goal: to design and implement a national research blueprint that outlines actionable strategies to address the most important needs within the community and opportunities to accelerate progress through coordinated collaboration.




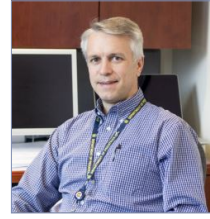



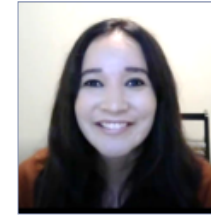


## Objectives:

- To design and implement a national research blueprint that embraces patient-centric principles and holistically addresses the priorities of the IBD community, particularly among under-represented populations.
- To inform this process through: community listening activities and solicitation of input into the critical areas that can produce dramatic improvements for the community today and in the future.

## Methods:

- Virtual community listening sessions were conducted in collaboration with the Center for Information and Study on Clinical Research Participation (CISCRP), and Tufts University Center of Study of Drug Development, with diverse groups representing adults with IBDs, caregivers, patient organizations, chapter and member organization directors, healthcare providers, and industry representatives.
- These discussions were supplemented by a cross-community survey developed by and administered through NHF's chapters and Hemophilia Federation of America (HFA) member organizations to the patient and caregiver community, and in parallel through NHF's networks to the health professional community.
- Additional insights will be gathered through NHF's community powered registry, Community Voices in Research, which convenes input from over 1300 community members to help researchers better understand how bleeding disorders affect individuals and their family members across their lifespan.
- Insights are now being distilled into common themes that represent the areas of greatest interest and need from the community. This will inform action by a newly established Steering Committee (Table 1) and

State of the Science Steering Committee (Table 1)

 <b>Leonard Valentino, MD</b> Chief Executive Officer, National Hemophilia Foundation (NHF)	 <b>Michael Recht, MD, PhD</b> Chief Science Officer, American Thrombosis and Hemostasis Network (ATHN)	 <b>Shannon Carpenter, MD</b> President, Hemostasis & Thrombosis Research Society; Children's Mercy Hospital	 <b>Steven Pipe, MD</b> Chair, NHF Medical and Scientific Advisory Council (MASAC); University of Michigan	 <b>Jill Johnson, MD</b> Chair, Scientific Committee on Hemostasis for the American Society of Hematology (ASH); University of Washington and Bloodworks Northwest
 <b>Barbara Konkle, MD</b> Program Lead, My Life Our Future (MLOF) Research Repository; Puget Sound Blood Center and Bloodworks Northwest	 <b>Sarah O'Brien, MD</b> Medical Advisory Committee, Foundation for Women & Girls with Bleeding Disorders; Nationwide Children's Hospital	 <b>Esmeralda Vasquez</b> Patient Representative	 <b>Donna DiMichele, MD</b> Consulting, LLC	 <b>Michelle Witkop, DNP</b> Head of Research, National Hemophilia Foundation

expert working groups who will convene in a State of the Science (SOS) Research Summit the week of September 13, 2021.

- These efforts will culminate in a research blueprint designed and endorsed by the entire IBD community and championed by community research leaders.

## Results:

NHF solicited broad input from the community over the course of five months: (Table 2)

- A total of 78 individuals participated in 9 listening sessions representing patient and caregiver perspectives, as well as patient organizations and chapter/member organization leads: 31% with an IBD, 5% caregivers, 51% healthcare professionals and 35% chapter leaders.
- Two sessions among health professionals reflected voices of 33 individuals from a variety of disciplines
- Two additional sessions were hosted among 16 members of industry representing seven global organizations.

Throughout this process, diverse and important considerations have been distilled into key themes, which will be addressed by multidisciplinary working groups: (Table 3)

- Patients and families cited opportunities for improvements in comprehensive care: mental health support, greater healthcare community awareness of IBDs, improved tools for diagnosis and supportive care outside of the HTC settings, particularly for underserved segments of the community.
- Emerging trends illustrate the need for mechanistic, translational, clinical/implementation, and health services research that addresses the diagnostic, therapeutic, and QOL needs across all IBDs, as well as the disparate access to care that particularly affects under-represented IBD populations that include women and rare bleeding disorders, groups that often intersect.
- Broadly, community members have cited the need for greater infrastructure, resource planning and workforce development to foster these research priorities.
- The planned September 2021 State of the Science Summit will serve to consolidate these themes into actionable, prioritized focus areas that can guide IBD community research and encourage more collaborative efforts to accelerate progress.

Table 2

	Patient and Caregivers	Chapter Leads
	<b>n=28 Total Participants</b>	<b>n=27 Total Participants</b>
<b>Role</b>	93% Patients   7% Caregivers	19% Patients   33% Caregivers
<b>Gender</b>	32% Males   68% Females	34% Male   66% Female
<b>Age</b>	46% Under 40   54% 40 and Over	30% Under40   70% 40 and Over
<b>Ethnicity</b>	18% Hispanic   82% Non-Hispanic	11% Hispanic   89% non-Hispanic
<b>Race</b>	21% Black   57% White   7% Asian   14% Other	92% White   4% Black   4% Middle Eastern
<b>Clinical Trial Experience</b>	18% CT Experience	
<b>HCPs n=27 Total Participants</b>		
Key Opinion Leaders: n=5, Nurses: n=3, Social Workers: n=3, Physical Therapists: n=2, Pharmacist: n=1, Advanced Practice Providers: n=7, Physicians: n=9, PhD: n=1, Genetic counsellors: n=1		
<b>+ 16 industry members representing 7 global innovators</b>		

Bleeding Disorders represented: Hemophilia A, Hemophilia B, Von Willebrand Disease, Factor X, Factor XI, Factor XIII, Factor VII, Platelet Dysfunction, Glanzmann's Disease

Table 3

Working Group Scope	Focus Areas
1. Research Priorities for Hemophilia A & B	<ul style="list-style-type: none"> <li>Across the spectrum of research</li> <li>Understudied areas in women and phenotype</li> </ul>
2. Research Priorities for von Willebrand Disease, Platelet Dysfunction and other Mucocutaneous Inherited Blood Disorders	<ul style="list-style-type: none"> <li>Across genders and phenotypes</li> <li>Diagnostics and Therapeutics</li> </ul>
3. Research Priorities for Ultra-Rare Bleeding Disorders	<ul style="list-style-type: none"> <li>Across genders and phenotypes</li> <li>Diagnostics and Therapeutics</li> </ul>
4. Research Priorities for Health of Women & Girls and those with the Potential for Menstruation	<ul style="list-style-type: none"> <li>Across genders and phenotypes</li> <li>Diagnostics and Therapeutic</li> <li>Female biology and phenotype</li> </ul>
5. Facilitating Priority Research in the Bleeding Disorders Community	<ul style="list-style-type: none"> <li>Infrastructure</li> <li>Resource Procurement/ Development</li> <li>Workforce Development</li> </ul>
6. Diversity, Equity & Inclusion Health Services Research & Implementation Science	<ul style="list-style-type: none"> <li>SOC Implementation</li> <li>DE&amp;I health services research</li> <li>Telehealth and delivery network development</li> <li>Communications</li> </ul>

## Conclusions:

- Actively soliciting the community's views is central in our process to advance research in IBDs. At the end of the SOS process, with input from patients and families, we will have a blueprint that will ring true to the voices heard during this planning process.
- Our ultimate goal through this effort is to bring forward those areas that can create the greatest impact for individuals with bleeding disorders and their families. This blueprint will help to guide community research that could fundamentally redefine the experience of living with these disorders.