



# A world without bleeding disorders begins with research:

Progress Update  
on NHF's National  
Research Blueprint



**NATIONAL HEMOPHILIA FOUNDATION**  
*for all bleeding disorders*

# A world without bleeding disorders begins with research.

The National Hemophilia Foundation (NHF) has taken the lead on a community-driven journey to shape the future of research for inheritable bleeding disorders (IBDs). Our goal is to establish a clear understanding of the most pressing issues challenging people and families with IBDs. This broad effort aims to bring the experiences of people who live with bleeding disorders to the forefront of transformational research and examine where research can have the most community impact.

The following progress update is intended to share with you our accomplishments with the State of the Science Research Summit (SOS) and plans to bring the National Research Blueprint (NRB) to life. We'll review with you the highlights of the many forums for community input to date, the robust dialogue and debate we've fostered on key research priorities, and our current focus and future plans.

## WHAT'S INSIDE:

- How Far We've Come Together
- Highlights from the State of the Science (SOS) Research Summit in September 2021
- Kicking Off the National Research Blueprint (NRB) in 2022
- What Lies Ahead
- Our Ask to You

## NRB: Fueling an Ambitious Vision

In 2023, NHF will present to the Inheritable Bleeding Disorders (IBD) community a National Research Blueprint (NRB) that will define priorities for:



**A patient-centric multidisciplinary research enterprise**



**National research infrastructure expansion**



**Reinvigorated and sustainable workforce**

All of this will steadily advance standards of care for people with IBDs through:

- Impactful basic, translational, clinical, health outcomes, and implementation research
- As well as meaningful advocacy efforts and productive community education and engagement



# How Far We've Come Together

A community-led effort from the beginning

We started by listening to and learning from the community. This collective input has informed our journey together.

## 2020

Community focus groups:  
"What if...?"



Listening sessions with subject matter experts (SMEs), including patients, caregivers and families, health care professionals and industry:

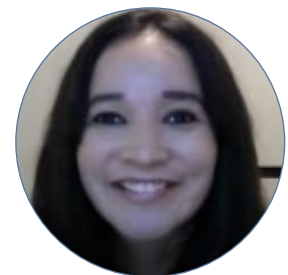
"Where can we make the greatest impact?"

## 2021

Cross-community survey:  
"How should we prioritize the themes we're hearing?"



National Institutes of Health (NIH)-funded efforts to include diverse voices in the research process, fostering engagement



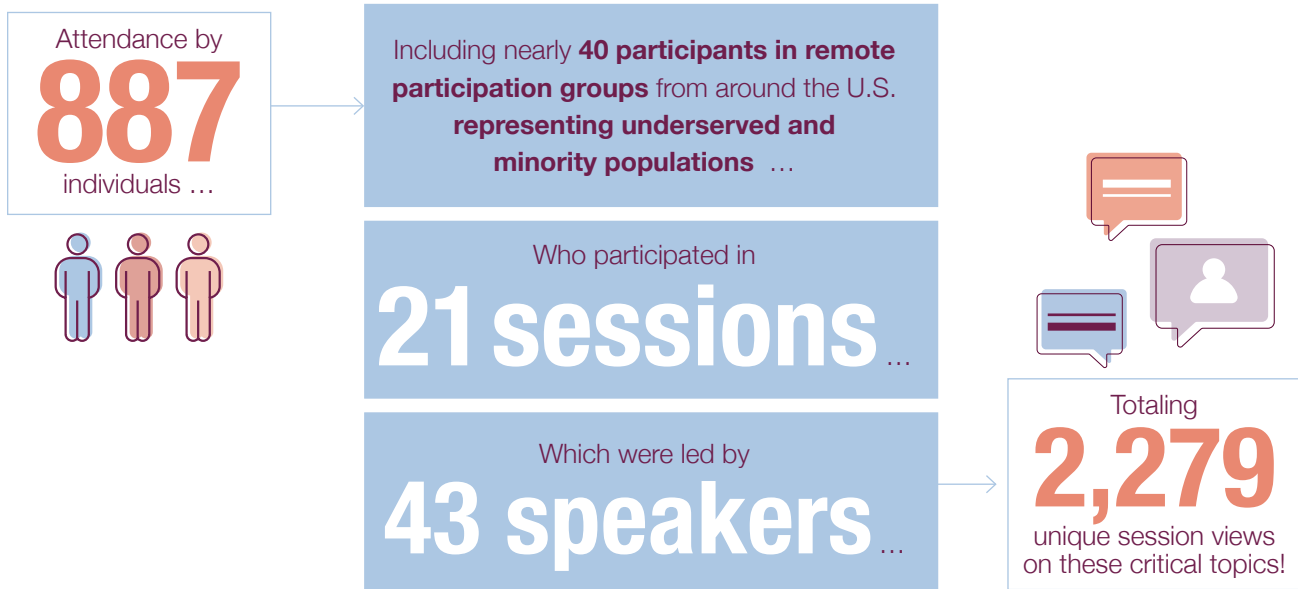
*"As someone living with a rare bleeding disorder, it's exciting to be involved in discussions about research that could benefit my life."*

– Esmeralda Vazquez, Steering Committee patient representative



# The State of the Science Research Summit (SOS)

The State of the Science Research Summit that convened virtually in September 2021 provided an incredible opportunity for robust dialogue and debate on how to prioritize research opportunities for real patient impact. The event accelerated progress toward our mission with:



**Key Outcome:** The SOS discussions helped us identify key themes and areas of focus:

GLOBAL THEMES	RESEARCH THEMES	RESEARCH PRIORITIES
 <b>People Centricity</b>  <b>Collaboration</b>  <b>Health Equity, Diversity &amp; Inclusion</b>	<ul style="list-style-type: none"> <li>• Understanding the pathobiology of bleeding</li> <li>• Novel and improved diagnostics</li> <li>• Novel and adapted therapeutics</li> <li>• Impediments to care</li> </ul>	<ul style="list-style-type: none"> <li>• Bone and joint health</li> <li>• Influence of sex and gender on disease</li> <li>• Aging</li> <li>• Immunogenicity</li> <li>• Mental health</li> <li>• Pain</li> <li>• Research infrastructure</li> </ul>



We are grateful to everyone who presented, participated, or listened to the event. If you missed it, the session recordings are still available! [Click here for more](#). The themes identified in our discussions together have outlined a clear path forward toward the kick-off of the development of a National Research Blueprint (NRB).



# Kicking off the National Research Blueprint (NRB) in 2022

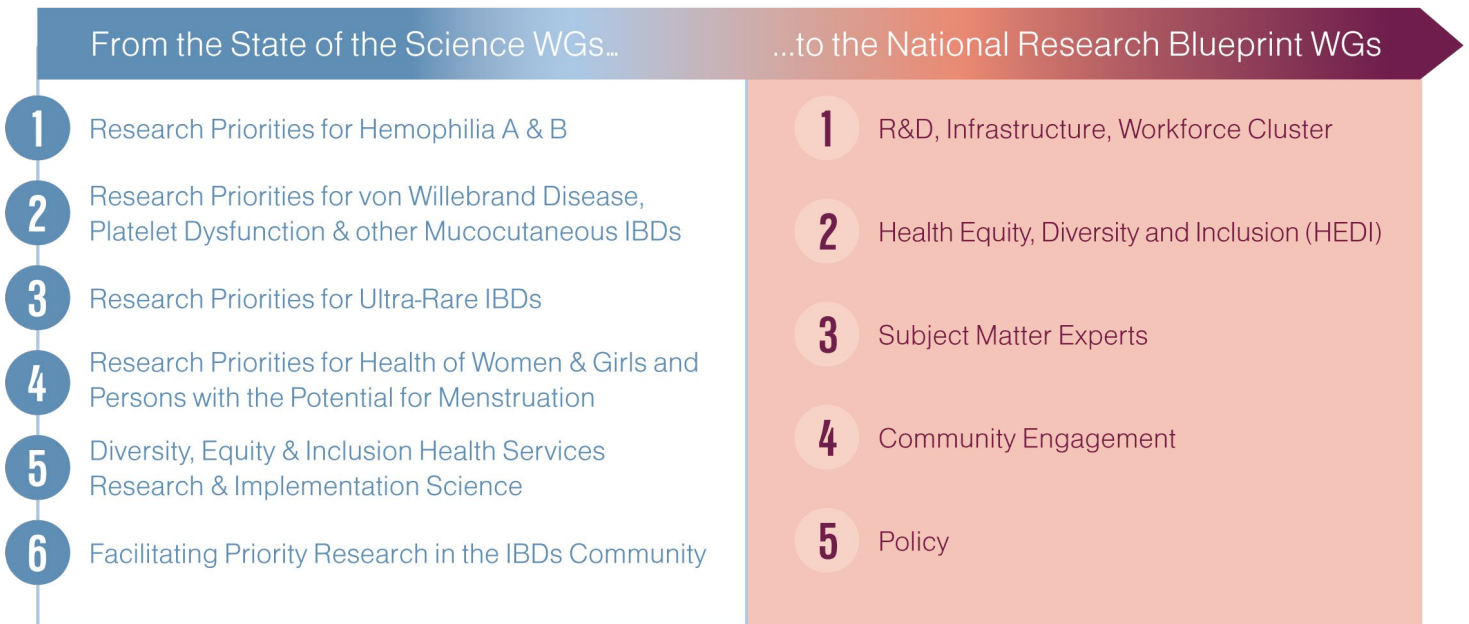
The SOS was *just the beginning* for this important initiative. Since September, our teams have been hard at work designing a process to build a NRB that encapsulates the priorities dictated by the community and provides a clear roadmap on how to activate these priorities while fostering a productive, inclusive research culture across the bleeding disorder community. The NRB will be purposefully designed to advance research across three critical areas:

**1. Multidisciplinary research enterprise** that is centered in a network of both specialty and community-based care; driven by patients/families/caregivers as the subject matter experts (SMEs); embedded in

the principles of social justice and sustainability; and that steadily advances the standard of care for people with IBDs through impactful basic, translational, clinical, health outcomes, and implementation research as well as advocacy and education

**2. National research infrastructure expansion** that supports the envisioned research enterprise through facilitation collaboration and inclusion

**3. Reinvigorated and sustainable workforce** across comprehensive care and scientific disciplines that incorporates the SME perspective to advance health through the seamless integration of care and research as well as active community engagement to foster a research culture



# Building the Blueprint: March Workshop

So far in 2022, we have established a Steering Committee to guide our NRB efforts and named chairs for each of our working groups. And with a framework ready, we've begun to convene to develop workplans, membership, and templates to build efficient implementation and integration approaches that effectively address each of the priorities that the community has helped us define.

During the first NRB workshop, hosted March 10, 2022 in Washington, D.C., our steering committee and working group chairs led a discussion with attendees to clarify how we can move from broad research themes to a clear, defined roadmap toward research progress.

More than 70 people attended the workshop, including health professionals, government and industry representatives. The participants engaged in thoughtful dialogue, offered diverse perspectives on the challenges of the patient experience today and our aspirational goals to make a lasting difference for individuals with bleeding disorders.

## Workshop Goals:

- 1. Inform the community:** Establish clarity about the plans and processes for building on the State of the Science to develop an actionable National Research Blueprint
- 2. Gain broader community input:** Solicit input from all key stakeholders into the development of the Blueprint - Include different perspectives to the planning stage will result in a more comprehensive and inclusive Blueprint
- 3. Strategize our work:** Outline the work plans for the working groups – what can we do to help them best accomplish their goals
- 4. Identify synergies and challenges:** Identification of these early in the process will allow us to address them, plan for them, and ultimately save time/effort

## NRB Steering Committee



Brett Spitale



Kevin Mills



Michael Recht



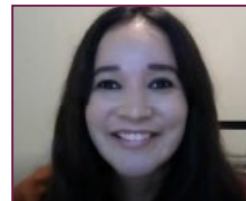
Keri Norris



Michelle Witkop



Donna DiMichele



Esmeralda Vazquez



Maria E. Santaella

Working Group	Chair(s)
R&D, Infrastructure, Workforce Cluster	Jill Johnsen Moses Miles Margaret Ragni Ziva Mann Amy Shapiro Lynn Malec
Health Equity Diversity and Inclusion (HEDI)	Keri Norris
Subject Matter Experts	Esmeralda Vazquez Erin Cirelli Sammie Valadez
Community Engagement	Patrick Lynch Jeremy Griffin Shannon Carpenter
Policy	Rich Pezzillo Michelle Conte Nathan Schaefer

## Their Charge

Within and across working groups, identify the essential elements of a platform from which to launch a successful and sustainable national IBD research network





# Prioritizing Research Questions

To inform the development of these critical strategic plans, participants in our workshop reviewed summaries of the findings from each of the SOS working groups. These initial efforts evaluated a wide range of potential research questions on the themes identified by the community, prioritizing based on a defined set of feasibility criteria to help us better understand where we can make the most difference together. The criteria evaluated three parameters:

- **Feasibility**, which assesses the difficulty in answering the proposed question, including required expertise, infrastructure, and resources.
- **Impact**, estimating the change we can foster through the priority. Will the answer impact standards of care or access to care? Will it change the therapeutic paradigm, and could it be applicable to other areas?
- **Risk**, which considers the challenges of the research question, such as the risk/benefit ratio for novel strategies and any ethical considerations.

Working Group	Key Areas of Focus	Research priorities identified (examples, not inclusive)
WG1: Hemophilia A & B	7 sub-working groups: Pain / bone health; Inhibitors; Diagnostics; Gene therapy; Transition; Disparities / literacy; cardiovascular disease	<ul style="list-style-type: none"> <li>• Optimal rehabilitation protocols after joint bleeds</li> <li>• Prediction of inhibitor development</li> <li>• Development of home patient self-imaging techniques</li> </ul>
WG2: von Willebrand disease (VWD), Platelets, Mucocutaneous IBDs	8 key topics discussed: VWD, Inherited platelet defects (IPD), Ehlers-Danlos syndrome (EDS), Hereditary Hemorrhagic Telangiectasias (HHT), Bleeding of Undetermined Cause (BUC), novel therapeutics, aging, biology of mucocutaneous bleeding (MB)	<ul style="list-style-type: none"> <li>• Improving understanding of basic biology</li> <li>• Aging-related concerns</li> <li>• Diagnostic testing and definitions</li> <li>• Creative use of novel therapies</li> </ul>
WG3: Ultra-rare Disorders	Diagnostics, Systems Biology, Mechanistic Science; Clinical, Data Collection, Research Infrastructure; Regulatory Processes For Novel Therapeutics & Required Data Collection	<ul style="list-style-type: none"> <li>• Proper diagnostic testing</li> <li>• Identification of disease modifiers</li> <li>• Centralized data collection capabilities</li> <li>• Regulatory pathways</li> <li>• Incentivizing product development</li> </ul>
WG4: Women, Girls, and Persons Who Have or Have Had the Potential to Menstruate	Sex/gender lifespan biology; PROs and QOL; Pregnancy and post-partum; Uterine bleeding; Bone and joint health; Healthcare delivery; Foundational research gaps	<ul style="list-style-type: none"> <li>• Phenotype prevalence insights</li> <li>• Improving norms and standards</li> <li>• Patient-centered outcomes</li> <li>• Bleeding measurement</li> </ul>
WG5: DE&I, HSR, Implementation Science	Health services research; Diversity equity and inclusion; Implementation science	<ul style="list-style-type: none"> <li>• Access to quality care and services</li> <li>• Inclusion of QOL outcomes</li> <li>• Addressing barriers to access for marginalized and minority populations</li> <li>• Incorporating implementation outcomes into clinical studies</li> </ul>
WG6: Workforce	Workforce, skillsets, recruitment, mentorship, retention	<ul style="list-style-type: none"> <li>• Improved utilization of 340b funding and other resources</li> <li>• Expanding training program eligibility</li> <li>• Broadening recognition of hematology field and structure</li> </ul>
WG6: Resources and Funding	Resources required for ideal research infrastructure and necessary funding	<ul style="list-style-type: none"> <li>• Resource toolbox including network databases, biorepositories, informatics tools and data sources</li> <li>• Access to specialized research expertise and communication to educate and engage the community</li> <li>• Broad and diverse funding partners including government, non-profit, and industry</li> </ul>
WG6: Infrastructure	Infrastructure; organizational models; research partners	<ul style="list-style-type: none"> <li>• Centralization of research hubs for certain services</li> <li>• Provisions for educational development / training</li> </ul>

**Note:** NHF will make available the detailed priorities from each working group as part of forthcoming manuscripts to be published in 2023.

# Building the Blueprint: April workshop



Building on the productive dialogue at the March workshop, we reconvened for a special workshop on April 8-9, 2022, again in Washington, D.C. The session, comprised of 79 attendees in total, allowed us to connect directly with 39 subject matter experts (SMEs) – patients and their families. The session accomplished the following:

- ✓ Explained to SMEs our goals and key areas of focus for the NRB and WHY
- ✓ Gathered input on the importance of research and the role of active community involvement in research
- ✓ Collected SME input about our research priorities to inform the working group planning efforts

*"I am a patient with hemophilia who went into medicine and became a pediatric hematologist to care for other children with bleeding disorders. I recognize the impact that research has had on my life and the potential to improve the future lives of the people in our community. I am eager to help shape the direction of future research for patients with bleeding disorders like myself."*

– Kyle Davis, Participant

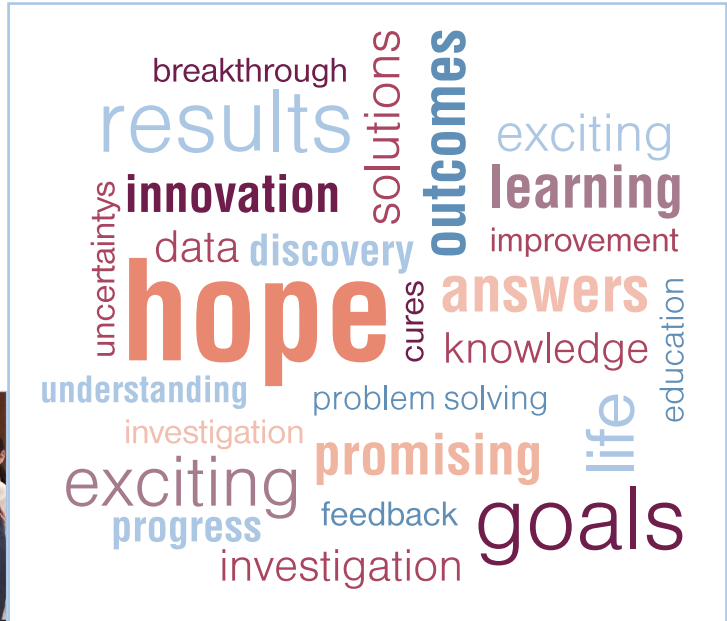
Agenda Day 1: Friday, April 8, 2022	
8:30 - 9:30 AM	Welcome and Introductions National Research Blueprint: A community effort that includes you!
9:30 - 10:30 AM	Research: What is The Big Deal?
11:00 - 12:00 PM	Equity, Inclusion, and Research: Ensuring Research Findings Include You
12:00 - 12:30 PM	Group Activity
1:30 - 2:30 PM	Research from where we stand: A conversation from our perspective
2:30 - 3:30 PM	Let's Design a Research Protocol TOGETHER!
4:00 - 5:30 PM	A discussion with NRB WG Chairs about charges and the role of the SME <ul style="list-style-type: none"> <li>• Infrastructure, Workforce, and Research &amp; Development (R&amp;D)</li> <li>• Health Equity, Diversity, &amp; Inclusion (HEDI)</li> <li>• Policy</li> <li>• SME</li> <li>• Community Engagement</li> </ul>
Day 2: Saturday, April 9, 2022	
8:30 - 9:30 AM	NHF, Research, and YOU!
9:30 - 10:30 AM	Think Different: SMEs as the Catalyst for Change
11:00 - 12:00 PM	Community Engagement: Can You Hear Me Now?
12:00 - 12:30 PM	Summary of Event & Call to Action



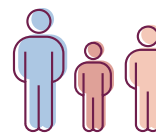
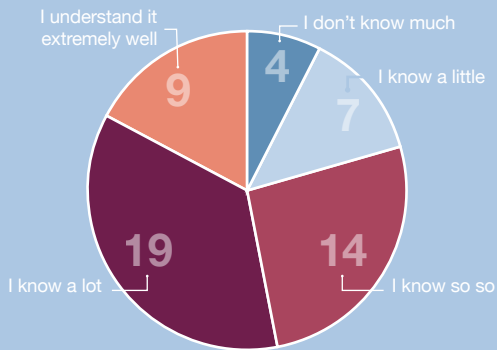


# What we learned from SMEs about research...

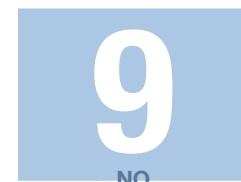
What comes to mind when you hear the word, "research?"



How would you rate your understanding of research?



Have you or a loved one participated in a clinical trial?



This is why research is central to our efforts. **Everyone plays a role in contributing to a research culture to improve the care of IBDs and work toward a cure.** Even if not participating in a clinical trial, families can raise questions about research and new ideas and actively collaborate with their providers.



# What Lies Ahead

We've made tremendous progress together on this journey to date. But so much lies ahead. Together with our working group partners and advisors, we are taking a methodical approach from these broad concepts toward the delivery of a robust, actionable blueprint.

What happens next:

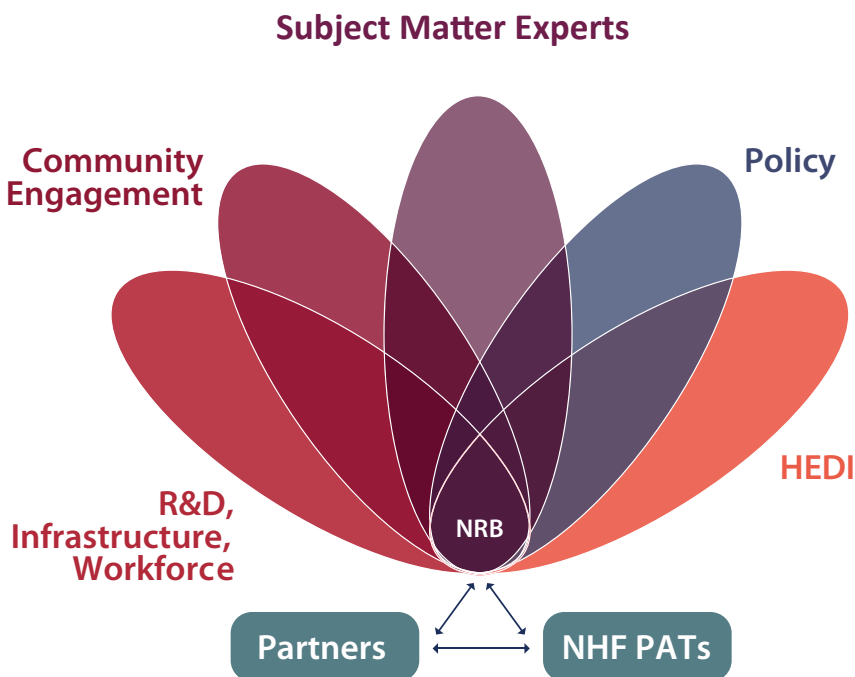
1. Working groups will be **collaborating over the coming months** in working sessions and discussions with the other groups to outline the critical elements of a platform to build a successful and sustainable national IBD research network
2. We'll be looking for more **community input during a special session at the Bleeding Disorders Conference (BDC) in Houston, TX, on August 26 at 1:30 PM CST.** Stay tuned for more on that!
3. The groups will have integrated, **prioritized plans delivered by the end early in 2023**, which will be organized into a final set of recommendations and compiled Blueprint

## What will be covered in these plans?

The key to the success of the NRB is to provide specific, actionable steps toward progress in the key areas we've identified together. The real task for our working groups will be to outline these steps with enough detail so we can guide community activities – either ongoing or planned. **Each strategic document will outline the following:**

1. Specific actions with quantifiable steps;
2. Key stakeholders in the process and their responsibilities;
3. Measurable milestones and timelines for each activity; and
4. Mechanisms by which we can measure our progress.

## National Research Blueprint Working Groups



# The NRB Roll-Out: Initial Plans

**We're looking ahead!** Once we have a clear set of recommendations from our working groups, we'll prepare the full National Research Blueprint in 2023.

- We expect to convene the community again for an **NRB-focused Summit**, during which we'll review and discuss strategies to activate the strategic plan
- **Manuscripts from each working group will be developed** explaining the methodology, dialogue and concluding recommendations; this will include considerations on how the initiatives could be applied to communities around the world
- And as part of the NRB communications activities, we'll work together to **foster a strengthened research culture across the community**, which will be central to our collective success



## Our Ask to You

We can only make a real, lasting impact if we all do this together. This NRB is truly a collaborative effort involving the entire community. Learn more about what you can do to join:

- Follow our progress online by visiting the [National Research Blueprint](#) section of our website
- Join [Community Voices in Research \(CVR\)](#) via QR code
- Sign up to get communications from us! [Subscribe for Email Updates](#) (select all that apply but especially research)
- Join us for a special [Bleeding Disorders Conference](#) (BDC session called *You Can Impact the Future of the Inherited Bleeding Disorders Community: The National Research Blueprint*)



We look forward to sharing more updates soon; thank you for joining us on this journey! We encourage you to contact [research@hemophilia.org](mailto:research@hemophilia.org) with any questions or comments.

