



NATIONAL
BLEEDING DISORDERS
FOUNDATION



NATIONAL
RESEARCH
BLUEPRINT

Launching a Community-Driven National Research Blueprint for Inheritable Bleeding Disorders

2024



Launching the National Research Blueprint



The National Bleeding Disorders Foundation (NBDF) is championing the National Research Blueprint (NRB), a multi-year effort driven by and for the bleeding disorders community. Together, we are working to create new ways to inform, design, prioritize, conduct, and share research in bleeding disorders through a comprehensive, coordinated, and collaborative national research strategy. Since it was launched in 2020, the NRB journey has gone through three main phases:

Phase 1: During Phase 1, NBDF started the conversation by bringing together health professionals, community leaders and most importantly, people affected by bleeding disorders – referred to as lived experience experts (or LEEs). Together we identified important issues affecting people with bleeding disorders today, and prioritized research that can help improve access to and quality of care for everyone in our community.

Phase 2 During Phase 2, a steering committee and interdisciplinary working groups defined the principles, scope, and priorities for a blueprint to address today's most pressing needs. As part of the process, they identified resource and infrastructure needs to launch and scale an integrated research pathway with opportunities for active community participation at every step. The blueprint is designed around the principle that LEEs serve as integral partners in research and closely integrates concepts of health equity, diversity and inclusion (or HEDI). So it has designed a process through which the lived experiences of those with bleeding disorders can be better understood and included as part of patient-centered research.

Phase 3: Now, in Phase 3 of the process, we are launching the first iteration of what will be an evolving NRB. Together with LEEs and community leaders, NBDF is raising funds and planning for the critical first steps for the NRB to help redefine bleeding disorders research. This can only be successful with the whole community's input – read more on how you can be a part of it!

The National Research Blueprint for inheritable bleeding disorders:

An **ambitious initiative** to build a **transformational research enterprise** through **full community engagement**



Research Approach

Rethink how we prioritize research that matters for those living with inheritable bleeding disorders



Research Process

Orient and democratize the research culture and structure to streamline and integrate efforts



Research Outcomes

Ensure that research findings are relevant, representative, inclusive, and broadly shared

WHAT'S INSIDE:

- Introducing the NRB
- Role of LEEs
- Our journey to the NRB
- What lies ahead
- Get involved!

Introducing the NRB

Now in year four of this community-driven initiative, the first version of the NRB has been shared with the community. (Read further for more details on how we arrived here!)

NRB Goals

The initial draft of the NRB aims to:

1. Delineate its governance and operational structure and the roles and responsibilities of all stakeholders,
2. Outline actionable strategies to address the most important needs today and opportunities to accelerate research to address these needs,
3. Have well defined milestones and timelines, and
4. Establish mechanisms for evaluation and improvement.



Including the patient voice in research is crucial for ensuring that studies address the real needs and concerns of those directly affected by bleeding disorders. Patients provide unique insights into the daily challenges and practicalities of living with a bleeding disorder, leading to more relevant and impactful research outcomes. Additionally, involving patients fosters a sense of ownership and empowerment, ultimately improving the trust and cooperation between researchers and the patient community."

-Erin Cirelli, LEE

Vision: Thriving in the face of an inheritable bleeding disorder begins with community-inspired research.

Mission: The National Research Blueprint serves to advance an accessible standard of care and quality of life for all who are affected by inheritable bleeding disorders:

- Through collaborative and meaningful scientific inquiry;
- Coordinated through an efficient research infrastructure;
- Undertaken by a diverse, capacitated workforce in partnership with an engaged community;
- Supported by facilitative research policy;
- Grounded in the principles of health equity, diversity, and inclusion; and
- Importantly, fully informed by lived experience experts (LEEs), who are key team members in research development and implementation.

Guiding Principles



These guiding principles define how the blueprint will be implemented to make sure it reflects the community's interests and goals.

1

Research values: Mutual respect for strategies and actions

- Act with boldness & flexibility to achieve innovative solutions without undue complexity.
- Collaboratively pursue incremental growth of the research enterprise with no one-off projects.
- Celebrate failures and successes as one voice, using our learnings to fuel the network.
- Globally examine and mitigate the presence of conscious and unconscious bias.

2

Authentic partnerships: Genuine collaboration

- Base partnerships on trust and respect for the dignity of all.
- Collaborate and engage with diverse multidisciplinary stakeholders, on equal footing, including those from marginalized and minoritized populations, to ensure diversity and inclusion as well as inform and guide meaningful research.

3

Respectful communication: Effective interpersonal interactions

- Engage partners with cultural humility and competence.
- Embrace uncomplicated, transparent 2-way communication through active listening.

“

This initiative is an exciting opportunity to accelerate research progress for people with bleeding disorders by bringing together all the right people, including those who are directly affected, to cooperate on urgent areas of research.”

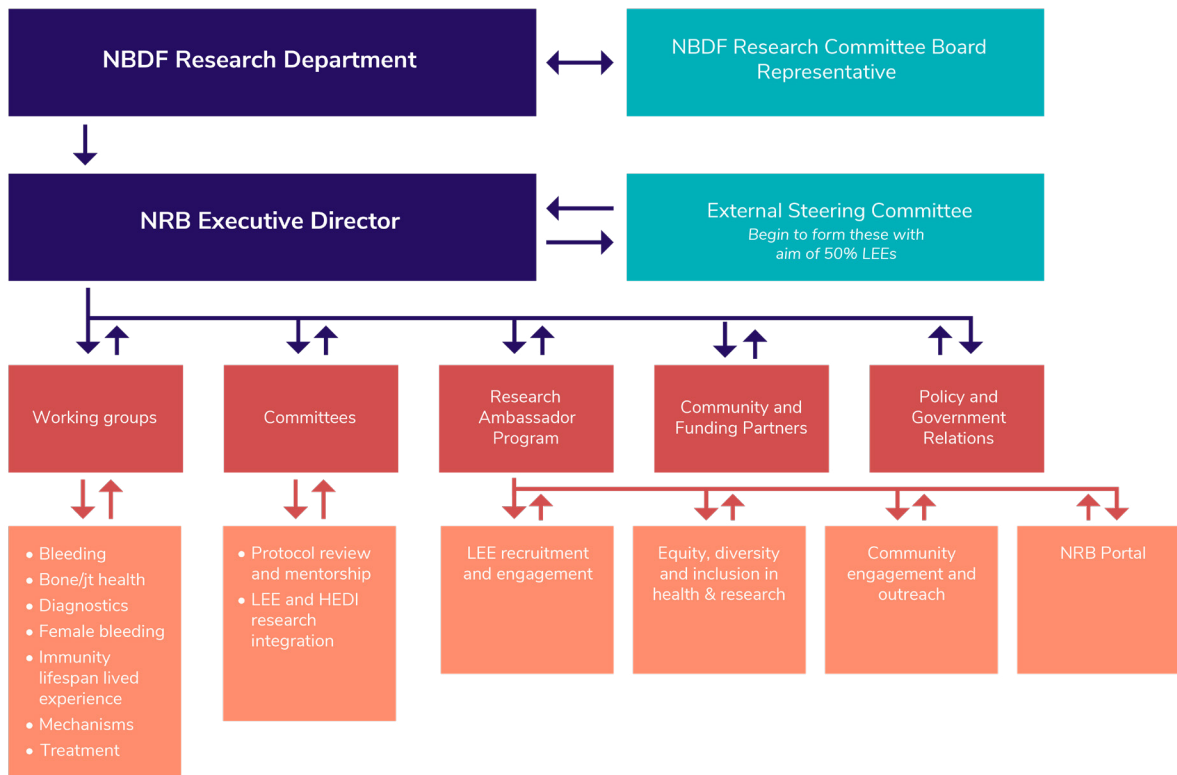
NRB Steering Committee member Maria E. Santaella, VP Research, NBDF



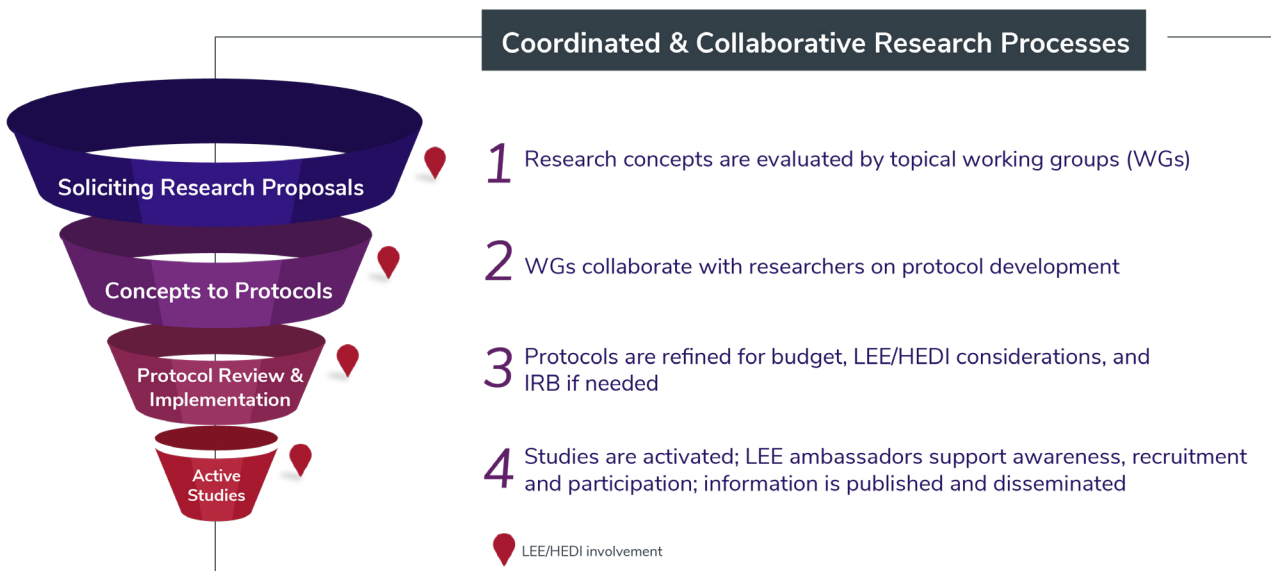
Governance and Operational Structure



The initial draft of the NRB outlines the key elements necessary to bring the journey to life. Championed by the NBDF and under the guidance of the NRB Executive Director, this initial oversight and operational structure is designed to help activate small pilot projects, which can then be enhanced and expanded over time. Community stakeholders, including LEEs, are prominent in this framework through steering committee and working group involvement.



Within this structure, participants will follow a step-wise process to pursue new research opportunities, integrating input from LEEs and HEDI priorities within each phase.



Research Priorities

The initial NRB has outlined a series of high-impact research priorities. Read more details on each of these topics and how we have prioritized these research questions.



Bleeding Disorders Research Priorities



Nine Spanning Research Topics

1. Lived Experiences
2. Bleeding
3. Female Bleeding
4. Joint and Bone Health
5. Lifespan
6. Mechanisms of Bleeding, Non-immune Complication, and Treatment Response
7. Immune Responses and Mechanisms
8. Diagnostics
9. Treatment

Bleeding Disorders Research

Main Branches:
9 Spanning Topics

Small Branches:
Scientific Areas
of Interest

Leaves:
Research Priorities

Coming soon! The NRB Steering Committee will be seeking input on these priorities. Please **connect** with the NBDF to share your thoughts and ideas.

Interested in learning more on the process details and activation of the NRB? Please visit the **National Research Blueprint** page on our website.

Engaging Lived Experience Experts (LEEs) in the NRB

The NRB is organized to enable people affected by inheritable bleeding disorders and their loved ones – recognized as lived experience experts (LEEs) – to actively participate and influence the direction of transformational research.

LEE Definition:

LEEs are individuals, their caregivers, and family members directly impacted by inheritable bleeding disorders.

Their diverse and personal knowledge gives them the unique ability to translate lived experiences into meaningful system change.

Based on this important perspective, LEEs should, to the best of their abilities, influence and/or drive bleeding disorders research. They should reflect a diversity as vast as those affected by them.



“
As a parent navigating health care for a child with two bleeding disorders, I understand the complexity of treating her and know that research is the only way to get her the proper care. For that reason, being involved in research as a LEE is very important to me.”

–Sammie Valadez, LEE



Interested in joining the NRB process? No research/scientific experience required

Grounding in HEDI Principles

The NRB philosophy, design and implementation process are firmly rooted in principles of health equity, diversity and inclusion. A dedicated working group is collaborating across the NRB teams to ensure these considerations are integrated, monitored and evaluated in research activities to ensure the work we develop together holistically represents our community. Considerations include:

- Workforce training
- Inclusivity in research and clinical trial participation
- Design of trials to address diversity and total affected populations
- Inclusivity elements of research policies and standards
- Community training to value health equity
- Understanding and addressing social barriers and determinants



The Central Role of Lived Experience Experts (LEEs)



LEEs have been actively involved throughout the NRB process by taking part in conversations, surveys, and working groups that have informed the blueprint. The goal is to build a comprehensive understanding of the most pressing issues they face, assess where research can be most impactful, and build a nimble, collaborative, and equitable network where they are active partners in each stage of research. This includes activities such as:

- **Helping to plan** and design study questions, processes, and goals
- **Assisting in the study** by participating, helping to recruit participants, and/or reviewing study findings
- **Communicating** the importance of the results to the community through easy-to-understand summaries and presentations

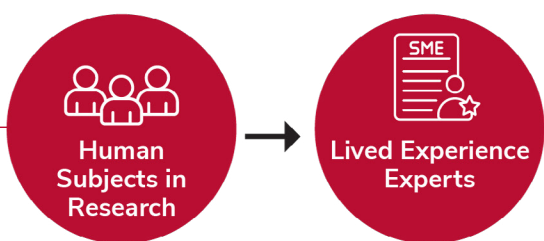
LEEs participating in the NRB will also help design research that addresses health equity and inclusivity principles. The goal is to build a more diverse research participant community that consciously includes under-represented populations, women, girls, and people who have or had the potential to menstruate (WGPPM), and individuals with rare and ultra-rare disorders.

As part of the process, the NRB is educating and training LEEs on the important aspects of research and how they can meaningfully contribute their lived experiences to support the research goals.

This active partnership of LEEs is one of the most crucial elements to the success of the NRB. If you or a loved one would like to learn more about being a research partner, please scan the QR code to contact the NRB team or email research@bleeding.org.



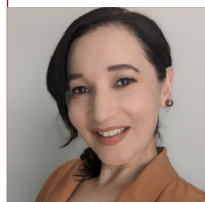
LEEs as Partners



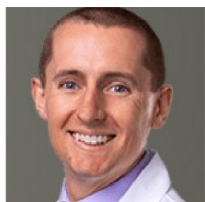
As someone living with a rare bleeding disorder, it's exciting to be involved in discussions about research that could benefit my life and healthcare outcomes. Research becomes more meaningful and relevant when the community is involved."

-Esmeralda Vazquez, LEE

Input	Consultation	Collaboration	Shared Leadership
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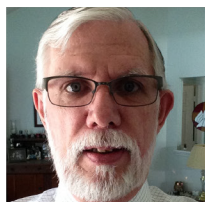
Esmeralda Vazquez



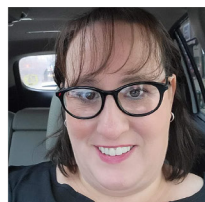
Kyle Davis



Michael Glenzer



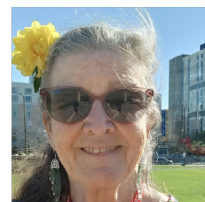
Ray Stanhope



Sammie Valadez



Erin Cirelli



Pat Deratto

If you are interested in joining this group and contributing to the advancement of science in bleeding disorders, please contact NBDF at research@bleeding.org.

Our Journey to the NRB

The NRB journey has been informed by conversations across the community including LEEs. Starting with broad, “what if” kinds of questions, we have steadily worked toward a blueprint that will help us create stronger research networks and take greater leaps to improve the lives of people with bleeding disorders.

Considering the Possibilities: 2020/2021 Community Dialogue

The concept of a national research blueprint was first identified through a community listening and learning process called Blue-Sky Project: Envisioning NBDF in 2030. It was conducted in collaboration with the Center for Information and Study on Clinical Research Participation (CISCRP), and Tufts University Center of Study of Drug Development, and led by then NHF CEO Leonard Valentino. We asked...

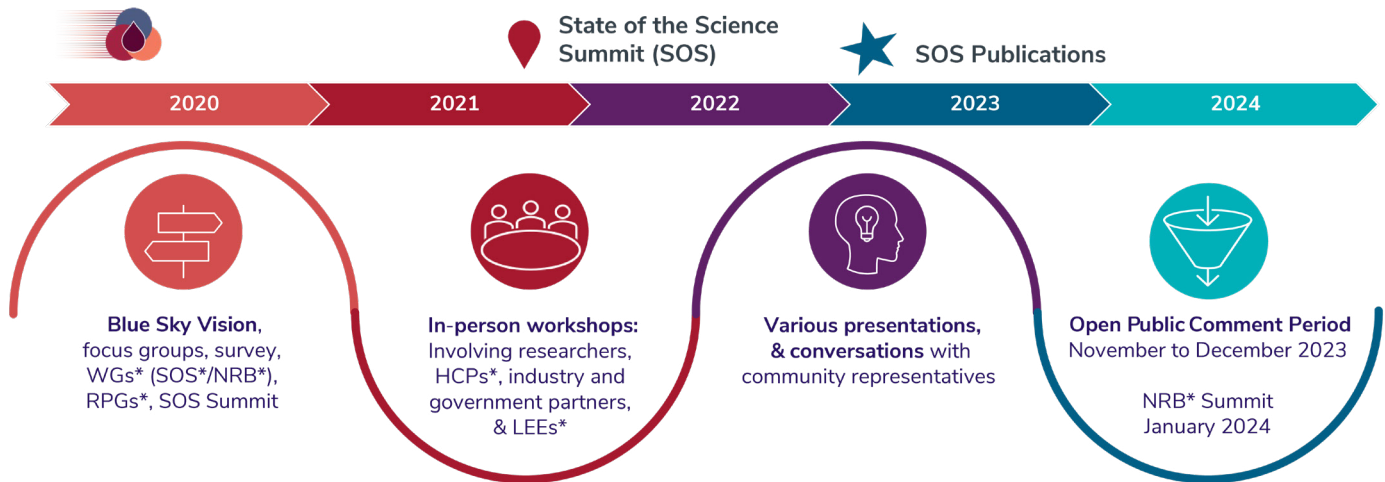
“What matters most to the future of care in bleeding disorders?”

“What does our community need from research to achieve our goals?”

“If not constrained by time or resources, what is possible?”

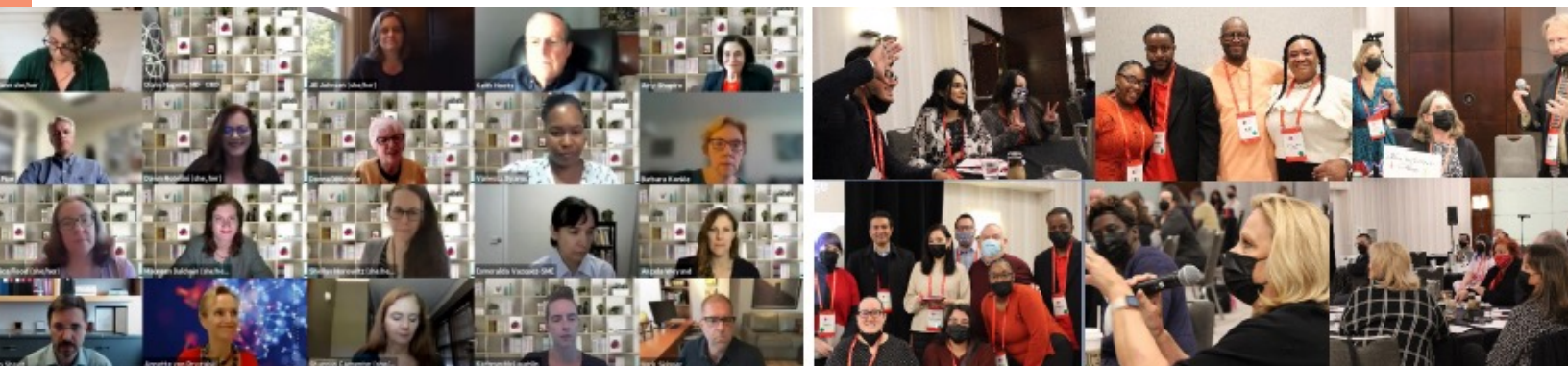
“What specific areas will create the greatest impact for those who need it most?”

Our Journey Together



*WG = Working groups
*SOS = State of the Science Research Summit
*NRB = National Research Blueprint

*RPGs = Remote participation groups
*HCPs = Health care providers
*LEE = Lived experience expert



State of the Science, September 2021



State of the Science Summit (SOS)

2020

2021



A virtual State of the Science (SOS) Research Summit was convened in September 2021 with input from community listening sessions, focus groups, surveys and working groups, all of which represented the voices of hundreds of individuals from across the community. During this Summit, each of the six diverse interdisciplinary SOS working groups presented and ranked priority research questions in key areas of unmet need, collecting comments and questions from community representatives to uncover breakthrough challenges and opportunities. In total, more than 2,000 people presented, participated, or viewed the summit, which helped to shape the next phase of the journey.

We are deeply grateful to the many people who prepared, presented, and took part in the summit. Session recordings are still available [online!](#)

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

GLOBAL THEMES


People Centricity


Collaboration

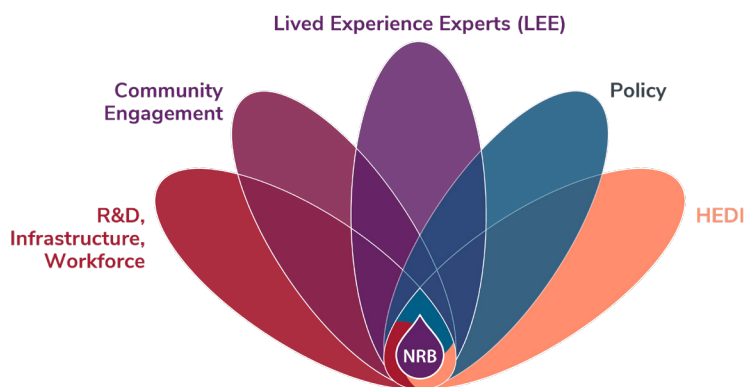

Health Equity,
Diversity & Inclusion

RESEARCH THEMES

- Understanding the pathobiology of bleeding
- Novel and improved diagnostics
- Novel and adapted therapeutics
- Impediments to care

RESEARCH PRIORITIES

- Bone and joint health
- Influence of sex and gender on disease
- Aging
- Immunogenicity
- Mental health
- Pain
- Research infrastructure



Publication of Initial NRB Recommendations



Publication of Initial NRB Recommendations

Following more than a year of design, evaluation, and community input, a series of manuscripts were published in a March 2023 supplement of the journal Expert Review of Hematology describing each working group's initial recommendations on how to activate the NRB process.

Importantly, the papers described the efforts of each working group to translate important ideas and needs into tangible, feasible, and prescriptive guidance on how to improve research for the future.

Publications represent the foundations of a clear and actionable NRB

- **Methodical processes** taken to ensure the recommendations are well grounded in clear unmet needs and under-addressed challenges
- Opportunities thoroughly **assessed for feasibility, risk and impact**
- Conclusions of each working group offer **defined, specific steps to make progress** in each focus area

Most importantly, they reflect the interests and desires of our community

- Combined conclusions reflect months and years of **community dialogue and debate**
- Grounded in social **justice principles, diversity, equity and inclusion**
- Deep **integration of lived experience experts (LEEs)** through the process, supporting more realistic applicability of research priorities that will address care for excluded populations

Foreword: The National Hemophilia Foundation's state of the science research summit: the foundation of a national research blueprint for inheritable bleeding disorders

Working Group 1: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Research priorities to transform the care of people with **hemophilia**

Working Group 2: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Research priorities for **mucoctaneous bleeding disorders**

Working Group 3: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Research priorities for **ultra-rare inheritable bleeding disorders**

Working Group 4: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Research to advance the health of **women and girls and people with inheritable bleeding disorders with the potential to menstruate**

Working Group 5: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Research priorities in **health services; diversity, equity, and inclusion; and implementation science**

Working Group 6: Building the foundation for a community-generated national research blueprint for inheritable bleeding disorders: Facilitating research through **infrastructure, workforce, resources, and funding**

Lived Experience Expert Editorial: Lived Experience Experts: A name created by us for us

International Perspective: Soliciting international perspectives on an American national research agenda for inheritable bleeding disorders

The guidance provided in these papers formed the basis of the National Research Blueprint and the NRB Steering Committee extends sincere thanks to everyone who contributed to this important work.

Defining our Priorities: NRB Blueprint

The inputs from the SOS Summit helped to define the scope of the blueprint:

1. **A patient-centric, multidisciplinary research enterprise** that is centered in a network of both specialty and community-based care
2. **National research infrastructure** expansion that supports research through facilitation collaboration and inclusion
3. **Reinvigorated and sustainable workforce** across comprehensive care and scientific disciplines

To be successful, the design and implementation of these elements must be:

- Activated and driven by an educated, informed and engaged community of LEEs, researchers, and health care providers, and other non-profit, federal, and industry partners;
- Grounded in sound principles of equitable and inclusive access to care and research;
- And fueled by strong research advocacy, communications, funding, and policy progress.

With the concept and scope well defined, NRB working groups were set up to consider how to build and implement an NRB that can effectively achieve the goals defined by the community. Special thanks to the leaders who graciously championed each working group.

As the working groups progressed through 2022, two immersive workshops were conducted with representatives from across the community to discuss how to translate broad themes into a clear, defined research roadmap. Participants assessed research questions and prioritized them based on a set of criteria to help make the greatest impact on care:

- **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 apply 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.



Organizing Research Themes Into Priorities

Following the initial assessments and characterization completed by the SOS Working Groups, the NRB Research and Development group then evaluated this diverse group of research questions, reviewed them for gap areas, and translated the list into broad topics while identifying areas of shared research interest across disorders and genders. This important work has defined the nine research priorities now established in the first iteration of the NRB.

2024 NRB Summit



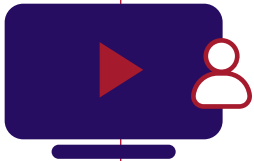
2023

2024

With the combined input from community listening, the SOS summit, manuscripts, and workshops, the NRB working groups presented a series of recommendations for an integrated research enterprise during a community summit in January 2024. The conversation included moderated panels and Q&As to discuss the defined principles of the NRB and the necessary elements as implementation begins.

Recordings of the presentations by each working groups are available at the links below:

- [Research and Development Working Group](#)
- [Infrastructure Working Group](#)
- [Workforce Working Group](#)
- [Community Engagement Working Group](#)
- [Lived Experience Expert Working Group](#)
- [Policy Working Group](#)
- [Health Equity, Diversity, and Inclusion Working Group](#)



“We want to demonstrate that the idea of the NRB is feasible.”

“We have big goals...”

“We understand this is going to be an evolution.”

NRB Summit Proceedings

Stay tuned for an executive summary from the 2024 Summit.

The final recommendations for the NRB presented by the working groups will be published in a series of manuscripts in 2025.

What Lies Ahead

With the initial concept of the blueprint now established, the third phase of the NRB has begun in 2024. With a new Steering Committee, the NRB focuses on fundraising, awareness, and infrastructure priorities to begin piloting research programs based on the defined research priorities.

The process to build and expand the impact of an NRB will be iterative. This means we will test the effort with

research partners, and then listen, learn, and adapt to strengthen the network and improve its impact for everyone involved.

This is why community collaboration is so important! The NRB’s success depends on participation across the community so we can make a real difference in the future of care for bleeding disorders. Join us on this next chapter of the NRB!

How you can get involved

What will this mean for our community?

“

No more one-off research projects. Having LEE's input, guidance, and insight at every level of the NRB will make research in the Bleeding Disorders Community better and more applicable to all.

- Ray Stanhope, LEE

Faster progress in truly unmet needs

Novel insights from integrated data

Direction from **LEE voices**

Mechanisms to **build quickly** on learnings

Acceleration from discovery to delivery

Efficient **resource** use to do more faster

And **more** that we will learn as we go...

The NRB represents an opportunity to redefine the treatment of bleeding disorders. This important initiative has only been possible thanks to the incredible contributions of so many community members and leaders since 2020. Never before have we had so much engagement from every corner of our community, with clear appreciation for the opportunity ahead of us to make a real difference for the next generation.

Now, as we begin to put this community-driven idea into practice, we need your participation! Our first steps in launching the NRB pilot are being led by enthusiastic volunteers from across the community who are lending personal experience or professional expertise to make this a success.

“

As a person living with and the parent of children who are now young adults with a rare bleeding disorder, I can bring a perspective that is only seen from the inside. This insight is so beneficial for researchers to consider ensuring the most inclusive and impactful improvements to the treatment of this rare disorder.”

- Pat Deratto, LEE



How you can get involved

Here's how you can join!

1. Join the NRB Pilot

- If you are a person affected by a bleeding disorder, consider participating as a Lived Experience Expert (LEE)! If you are connected to the bleeding disorders community as a researcher, health professional, government partner or industry member, consider joining to share your expertise. The NRB will benefit from your participation through a variety of committees, working groups, and advisory boards, and welcomes everyone to join! Scan the QR code to sign-up or contact **NBDF** for more information.



2. Participate in Other Research Opportunities

- If you haven't already, consider joining **Community Voices in Research (CVR)**. This initiative aims to better understand the personal experience of living with a bleeding disorder and provide critical data to help identify the community's most pressing needs. Participants get access to a Personalized Dashboard connecting them to other opportunities to participate in research, among other things. Scan the QR code to join!
- Participate in Virtual Advisory Panels and other research projects. For more information, contact **NBDF's Research Department**



3. Encourage Others to Join

- Spread the word! Talk with your friends, family and colleagues about the NRB and encourage them to sign up to take part in the process. Sign up for more information [here](#).

4. Stay Connected with NRB Progress

- Learn more about the NRB during NBDF's annual **Bleeding Disorders Conference** in Atlanta scheduled for September 2024.
- Follow our journey:
 - Check the National Research Blueprint page [online](#)
 - Sign up for [email communications](#)
 - Follow us online on [Facebook](#), [X/Twitter](#) and [LinkedIn!](#)

We will continue to share updates as we progress. You can check the National Research Blueprint ([link](#)), sign up for [email communications](#) or follow us online on [Facebook](#), [X/Twitter](#) and [LinkedIn!](#) Thank you for [joining](#) us on this journey.

“



As a person living with a bleeding disorder who works in medicine, I embrace the opportunity to guide research that will impact patients like me. LEEs provide the human element to research and ensure research goals align with the goals of our community.”

- Kyle Davis, MD, LEE