

National Hemophilia Foundation Champions a National Research Blueprint Defining a Community-Coordinated Research Ecosystem to Accelerate Progress in Inherited Bleeding Disorders



NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

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BACKGROUND

- The recent advent of novel technologies, therapeutics and genetic medicine, presents a powerful opportunity to accelerate inherited bleeding disorders (BDs) research
- A comprehensive, cross-community initiative spearheaded by the National Hemophilia Foundation (NHF) aims to deliver a National Research Blueprint (NRB) to foster accessible and high-impact research for people with BDs
- As part of the State of the Science (SOS) Research Summit in September 2021, inter-disciplinary and multi-stakeholder groups made considerable progress in identifying research priorities that most urgently require attention and opportunities to make the greatest impact for the future (Valentino, Haemophilia, 2022.) (See Table 1)
- NRB working groups (WGs) have been established and charged with working collaboratively to define the necessary components to launch, sustain, and expand the blueprint for a community-focused research network to accomplish these goals (See Figure 1)

Figure 1. National Research Blueprint (NRB) Working Groups



METHODS

- Based on the insights generated during the SOS, NHF has now convened seven multi-disciplinary NRB WGs, each charged with addressing critical questions in the formation of this network (See Table 2)
- The NRB will define critical components of a collaborative BD national research network, considering: the stakeholders to be involved, the resources necessary, and the processes and the systems required for productive research
- Given the rare nature of most BDs, this network must be not only sustainable, flexible and adaptable, but also uniquely accessible and partnership-oriented to effectively engage communities locally, built upon the models in place today through local and regional Hemophilia Treatment Centers, and coordinated through centralized research mechanisms.

Table 1. Key themes identified from the SOS, September 2021
Key Outcome: The SOS discussions helped us identify key themes and areas of focus

Global Themes	Research Themes	Research Priorities
<ul style="list-style-type: none"> • People Centricity • Collaboration • Health Equity, Diversity, and Inclusion 	<ul style="list-style-type: none"> • Understanding the pathobiology of bleeding • Novel and improved diagnostics • Novel and adapted therapeutics • Impediments to care 	<ul style="list-style-type: none"> • Bone and joint health • Influence of sex and gender on disease • Aging • Immunogenicity • Mental health • Pain • Research infrastructure

Table 2. NRB WGs	Critical Questions to be Addressed
Research and Development (R&D)	What basic, translational, clinical, implementation and outcomes research should the national BD research network either initiate or prioritize for development?
Infrastructure	What infrastructure elements must be prioritized to support research and workforce development activities? How will the infrastructure be sustainable?
Workforce	What are the strengths and needs for skill development to be successful and how can this network support career development?
Community Engagement	How can we help establish an active research culture across the community? What are the best communication, education and acculturation strategies to facilitate enthusiastic participation?
Lived Experience Experts (LEEs, People affected by BDs)	How can an effective research culture be fostered among patients, caregivers and family members and how can we ensure sustainable LEE input at every level to inform research strategy?
Policy	What are the essential elements of or necessary changes in research policy to facilitate research priorities, infrastructure and workforce development?
Health Equity, Diversity, and Inclusion (HEDI)	What health equity, diversity, and inclusion (HEDI) principles must be embedded in the network and how should they be integrated and employed over time?

RESULTS

Researchers, clinicians, LEEs, caregivers, non-profit and government leaders, and industry are contributing to the NRB network implementation plan. By 2023, NHF aims to deliver the blueprint to establish a community-integrated research enterprise that will not only help to coordinate research efforts in the areas that matter most for patients, but also foster a greater culture of research and inclusivity across the community to continually fuel progress.

CONCLUSIONS

The ultimate ambition of the NRB is to prioritize and accelerate research in partnership with a diverse and fully engaged patient community. The NRB efforts will deliver a collaborative research infrastructure and workforce capable of conducting this research expertly, equitably, efficiently and expeditiously, supported by the right research policies and the right processes to both communicate research results and translate them into a progressively evidence-based and universally accessible standard of care. This NRB effort, driven by, for, and with the BD community, has potential to redefine the BD landscape for the benefit of everyone.