

National Research Blueprint

Research & Development Working Group

January 26, 2024
Arlington, Virginia



NATIONAL
BLEEDING DISORDERS
FOUNDATION
Formerly NHF

Jill Johnsen, MD
on behalf of the R&D Working Group

Disclosures

Jill Johnsen

Biomarin (consulting)

CSL Behring (consulting)

Octapharma (consulting, research funding to University of Washington)

Takeda (consulting)

Research & Development Working Group



Tyler Buckner, MD MSc
Associate Professor of Hematology, Hematology Division, Hemophilia and Thrombosis Center
University of Colorado
School of Medicine



Veronica Flood, MD
Professor of Pediatric Hematology/Oncology/BMT
Medical College of Wisconsin



Jill Johnsen, MD
Associate Professor of Medicine; Institute for Stem Cell & Regenerative Medicine; Center for Cardiovascular Biology
University of Washington



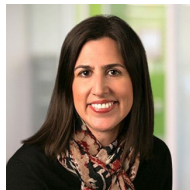
Andrew Johnson, PhD
Investigator, Population Sciences Branch
National Heart Lung and Blood Institute



Bernard Khor, MD PhD
Assistant Member, Benaroya Research Institute; Associate Medical Director, Bloodworks Northwest; Affiliate Assistant Professor, University of Washington



Donna DiMichele, MD
NRB Steering Committee
R&D WG Liaison from 11/2022-1/2024



Gallia Levy, MD PhD
Chief Medical and Product Strategy Officer
Spark Therapeutics



Glenn Pierce, MD PhD
Vice President Medical
World Federation of Hemophilia



Nick Popp, PhD
Medical Scientist Training Program, Department of Genome Sciences, University of Washington
School of Medicine
University of Washington



Jonathan Roberts, MD
Associate Medical & Research Director, Bleeding & Clotting Disorders Institute; Associate Professor of Pediatrics and Medicine
University of Illinois College of Medicine at Peoria



Erin Cirelli, Esq
Lived Experience Expert Co-Chair
R&D WG LEE Observer



Kevin Mills, PhD
Previous Chief Scientific Officer, National Hemophilia Foundation
R&D WG member and NRB Steering Committee Liaison from 7/2022- 11/2022

NRB R&D Working Group mission statement

The NRB Research and Development working group is charged with identifying *impactful and feasible Scientific Priorities* that will *improve the lives of all people living with bleeding disorders*.

These *scientific priorities will provide focal points* for the other NRB working groups to identify the essential elements from which to launch a successful and sustainable national bleeding disorder research blueprint.



NRB R&D Working Group mission statement

The NRB Research and Development working group is charged with identifying *impactful and feasible Scientific Priorities* that will *improve the lives of all people living with bleeding disorders*.

These *scientific priorities will provide focal points* for the other NRB working groups to identify the essential elements from which to launch a successful and sustainable national bleeding disorder research blueprint.

R&D Unofficial Motto
“No more one-offs!”



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

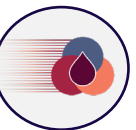
Integration in NRB



WG member breadth in:

- scientific expertise
- types of research
- career stages
- experience in different sectors

plus the research depth represented throughout the NRB



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB



Starting focus groups (LEEs)



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

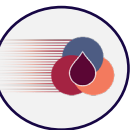
Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

Brainstorming Jamboards



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

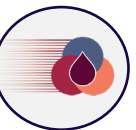
Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

Brainstorming Jamboards



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

9 Spanning Research Topics

Cross-cutting themes after brainstorming

- Lived Experiences
- Bleeding
- Female Bleeding
- Joint and Bone Health
- Lifespan
- Mechanisms of Bleeding, Non-immune Complication, and Treatment Response
- Immune Responses and Mechanisms
- Diagnostics
- Treatment



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

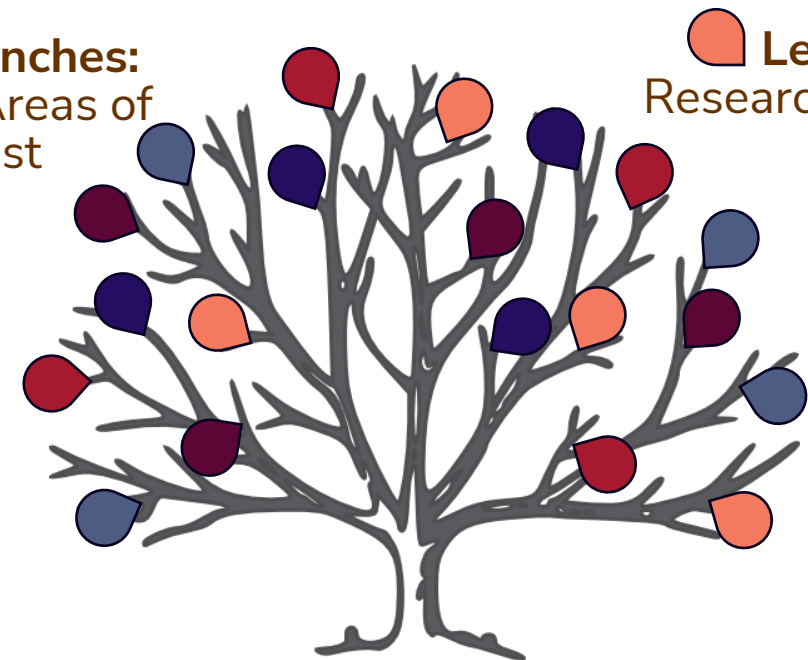
Integration in NRB

Branches and Leaves

Organization of Priorities

Small Branches:
Scientific Areas of
Interest

Leaves:
Research Priorities



Main Branches:
Spanning Topics

**Bleeding disorders
research**

Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

Research Priorities

Spanning Topic: Lived Experiences

- 6 Scientific Areas of Interest (small branches)
- 57 Research Priorities (leaves)

Lived Experiences Scientific Areas of Interest

- Understand the barriers to and challenges in accessing healthcare for all individuals living with inherited bleeding disorders
- Study interventions to improve healthcare delivery for all individuals living with inherited bleeding disorders
- Determine and improve the financial burdens of living with an inherited bleeding disorder
- How does living with a bleeding disorder impact meaningful outcomes and health-related quality of life?
- How can research efforts be designed to ensure inclusion and opportunities for meaningful involvement for all people living with bleeding disorders?
- How can we best understand and address the educational needs of people living with a bleeding disorder?

Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

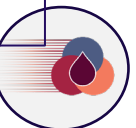
Research Priorities

Spanning Topic: Female Bleeding

- 4 Scientific Areas of Interest (small branches)
- 35 Research Priorities (leaves)

Female Bleeding Spanning Topic Scientific Areas of Interest

- Understand the biology of uterine bleeding (heavy menstrual bleeding, postpartum hemorrhage, and perimenopausal bleeding)
- Understand the biology of female hormones and their impact on inherited bleeding disorders
- Improve screening and diagnosis of females for risk for reproductive tract and other bleeding and determine approaches to improve implementation of treatment
- Determine the efficacy and safety of interventions for female reproductive tract bleeding



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

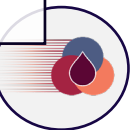
Integration in NRB

Research Priorities

Spanning Topic: Female Bleeding

- 4 Scientific Areas of Interest (small branches)
- 35 Research Priorities (leaves)

- Female Bleeding Spanning Topic
Scientific Areas of Interest
- Female bleeding disorder research priorities are also found in every other Spanning Topic - 51 additional Research Priorities**
- Understand the biology of uterine bleeding (heavy menstrual bleeding, postpartum hemorrhage, and perimenopausal bleeding)
 - Understand the impact of bleeding disorders on IBDs
 - Improve screening and diagnosis of females for risk for reproductive tract and other bleeding and determine approaches to improve implementation of treatment
 - Determine the efficacy and safety of interventions for female reproductive tract bleeding



Development of Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

The Research Priorities are published in the Program

Opportunities for broader input are planned



Integration of R&D Research Priorities

Broad scientific perspectives

LEE communication

Fluency with the Blueprint

Structured brainstorming

Spanning topics

Research Priorities

Integration in NRB

R&D principles during
harmonization and integration
with the NRB

- LEEs are central to the NRB and critical to every step in research
- HEDI principles are foundational
- Research Priorities serve the NRB (not the other way around)



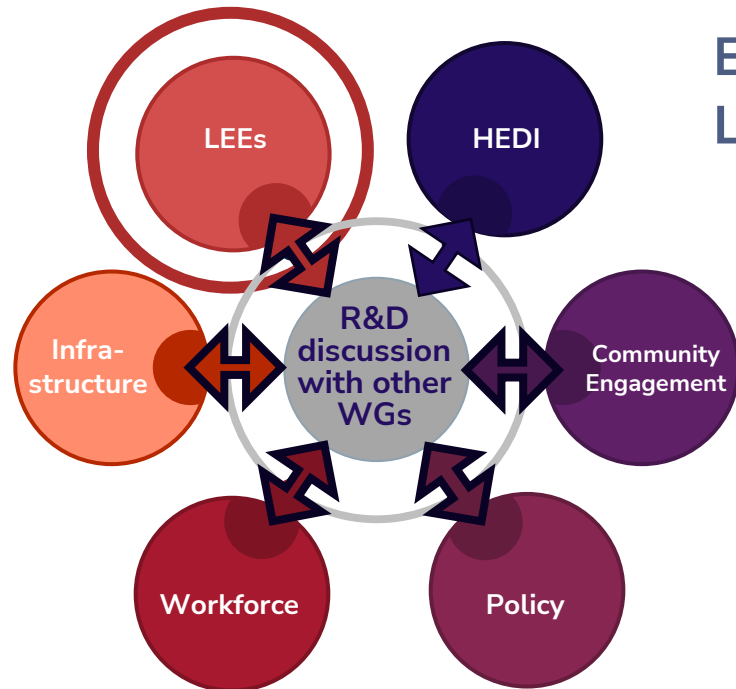
Integration and harmonization of R&D within the Blueprint

R&D Dyad meetings: R&D met in pairs with every other working group
Co-developed matrices to aid harmonization



Integration and harmonization of R&D within the Blueprint

R&D Dyad meetings: R&D met in pairs with every other working group
Co-developed matrices to aid harmonization



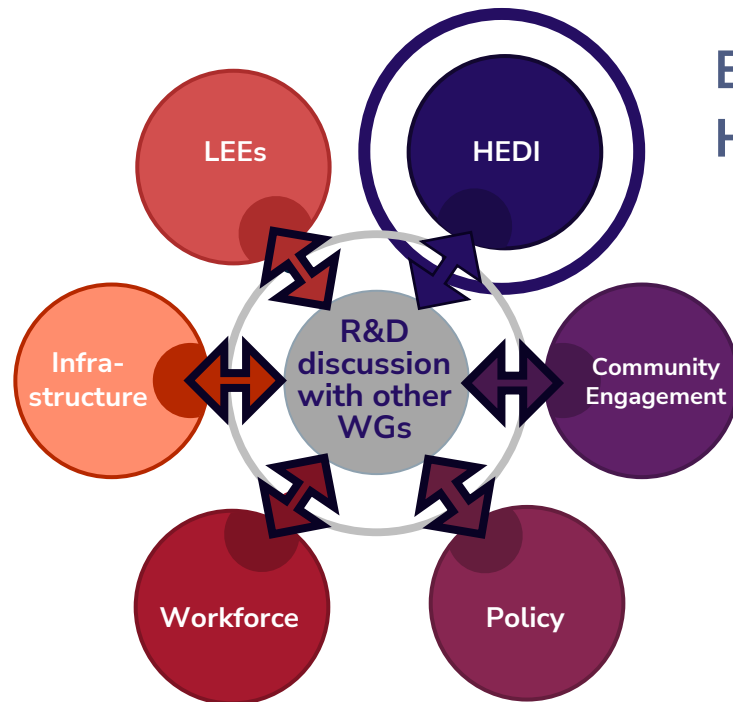
Examples of points made during the R&D and Lived Experience Expert (LEE) dyad

- LEEs are at the center of the NRB's research enterprise
- The R&D agenda will be continuously refined in the future based on LEEs who will be driving the agenda, community input, and the capacity of the NRB
- LEEs should be fully integrated in research from the beginning (or the earliest timepoint for ongoing projects), through design and implementation, to dissemination
- There should be transparency and regular research updates for LEES and the full community



Integration and harmonization of R&D within the Blueprint

R&D Dyad meetings: R&D met in pairs with every other working group
Co-developed matrices to aid harmonization

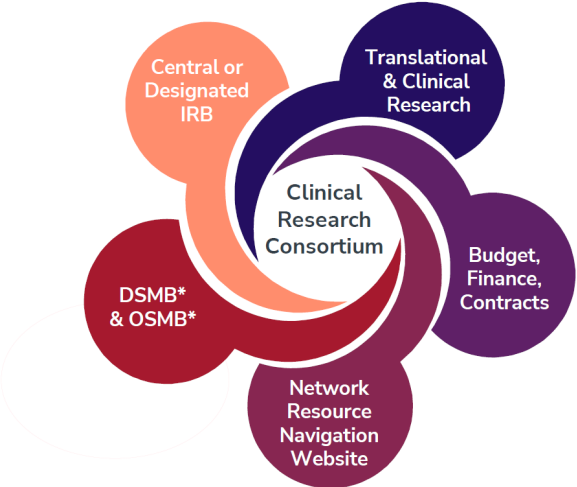


Examples of points made during the R&D and Health Equity, Diversity & Inclusion (HEDI) dyad

- HEDI principles are essential to all research
- Need to address access/equity gaps that impact representation in research (for both participants and researchers)
- Need inclusive, representative, accessible, sharing study designs
- All researchers should have HEDI training
- HEDI champions will be present throughout the NRB
- Representative LEE groups are needed to ensure goals and outcomes are meaningful and understandable to all
- Research Priorities should address critical HEDI data gaps



R&D contributions in the National Research Blueprint schema

| Bleeding Disorders Research Working Groups | Bleeding Disorders Research Infrastructure/Operations | Cross-Cutting Committees |
|---|--|--|
| <ul style="list-style-type: none"> • Bleeding • Bone & Joint Health • Diagnostics • Female Bleeding • Immunity • Lifespan • Lived Experience • Mechanisms of Disease • Treatment | <p>Clinical Research Consortium (CRC) (hub & spoke extending into the community)</p>  <p>*DSMB Data and Safety Monitoring Board *OSMB Observational Study Monitoring Board</p> | <ul style="list-style-type: none"> • Workforce & Leadership Dev. • Mentorship / Training Resources Dev. • LEE* & HEDI Research Integration • Protocol & Protocol Resource Dev. • CT Design, Epidemiology & Biostats & Resource Dev. • Data & Biospecimens & Resource Dev. • Genetics, Omics & Bioinformatics • Protocol Review |



R&D contributions in the National Research Blueprint schema

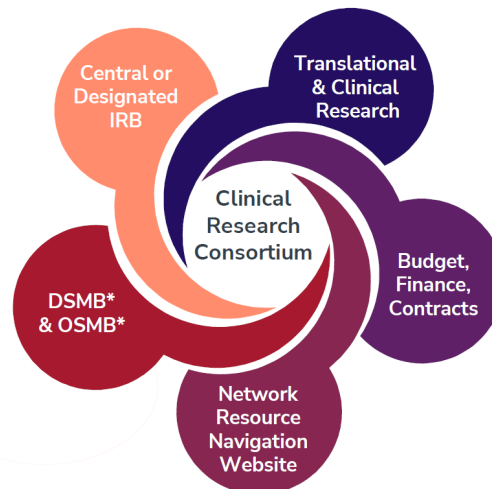
Bleeding Disorders Research Working Groups

- Bleeding
- Bone & Joint Health
- Diagnostics
- Female Bleeding
- Immunity
- Lifespan
- Lived Experience
- Mechanisms of Disease
- Treatment

R&D Spanning Topics reflected in the structure of the Bleeding Disorders Research Working Groups

Bleeding Disorders Research Infrastructure/Operations

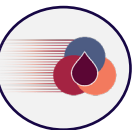
Clinical Research Consortium (CRC) (hub & spoke extending into the community)



*DSMB Data and Safety Monitoring Board
*OSMB Observational Study Monitoring Board

Cross-Cutting Committees

- Workforce & Leadership Dev.
- Mentorship / Training Resources Dev.
- LEE* & HEDI Research Integration
- Protocol & Protocol Resource Dev.
- CT Design, Epidemiology & Biostats & Resource Dev.
- Data & Biospecimens & Resource Dev.
- Genetics, Omics & Bioinformatics
- Protocol Review



R&D contributions in the National Research Blueprint schema

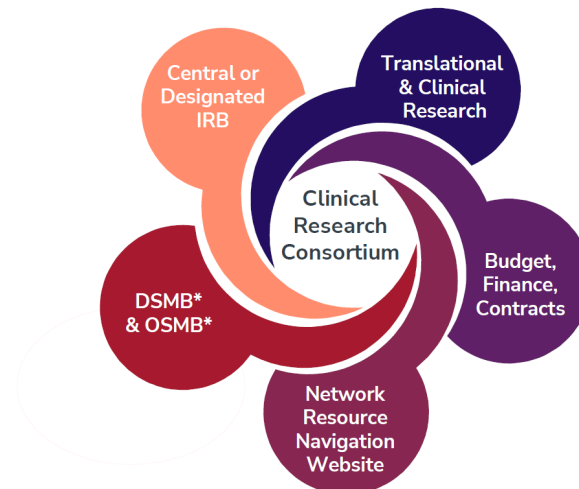
Bleeding Disorders Research Working Groups

- Bleeding
- Bone & Joint Health
- Diagnostics
- Female Bleeding
- Immunity
- Lifespan
- Lived Experience
- Mechanisms of Disease
- Treatment

Bleeding Disorders Research Infrastructure/Operations

Clinical Research Consortium (CRC)

(hub & spoke extending into the community)



*DSMB Data and Safety Monitoring Board
*OSMB Observational Study Monitoring Board

Cross-Cutting Committees

- Workforce & Leadership Dev.
- Mentorship / Training Resources Dev.
- * LEE* & HEDI Research Integration
- * Protocol & Protocol Resource Dev.
- * CT Design, Epidemiology & Biostats & Resource Dev.
- * Data & Biospecimens & Resource Dev.
- * Genetics, Omics & Bioinformatics
- * Protocol Review

R&D contributions relevant to all Cross-Cutting Committee topics (particularly those with *asterisks)



R&D contributions in the National Research Blueprint schema

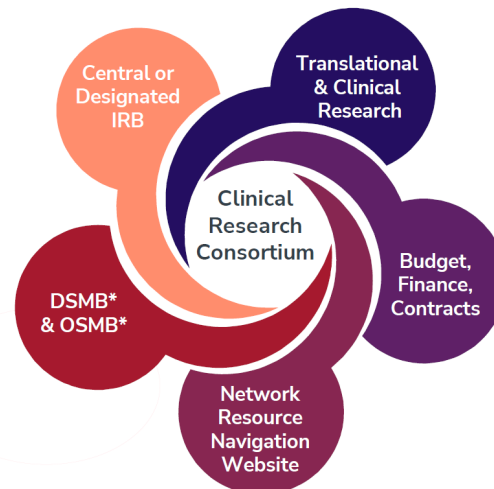
Bleeding Disorders Research Working Groups

- Bleeding
- Bone & Joint Health
- Diagnostics
- Female Bleeding
- Immunity
- Lifespan
- Lived Experience
- Mechanisms of Disease
- Treatment

Bleeding Disorders Research Infrastructure/Operations

Clinical Research Consortium (CRC)

(hub & spoke extending into the community)



Cross-Cutting Committees

- Workforce & Leadership Dev.
- Mentorship / Training Resources Dev.
- * LEE* & HEDI Research Integration
- * Protocol & Protocol Resource Dev.
- * CT Design, Epidemiology & Biostats & Resource Dev.
- * Data & Biospecimens & Resource Dev.
- * Genetics, Omics & Bioinformatics
- * Protocol Review

R&D Research Priorities and feasibility assessments will be a resource for the CRC



R&D Summary of Development of NRB Research Priorities

Built on the **foundation of the 2021 State-of-the-Science (SOS)**






- Developed priorities in 9 Spanning Topics

- 4-6 Scientific Areas per Topic
- Multiple **Research Priorities** were coalesced within each Scientific Area
- Every priority can be **studied in multiple ways**
- Some themes appear in **more than one priority or topic**



- Assessed Feasibility:

- **Scientific** (feasible or not)
- **Time** (1-2 yrs, < 5 yrs, ≥ 5 yrs)   



- Revised and Integrated into the NRB

- Dedicated input and meetings with **LEE** and **HEDI** leaders
- Input from **all working groups** (including LEE, HEDI) – dyads, other meetings



Q | A

Thank you!



NATIONAL
BLEEDING DISORDERS
FOUNDATION
Formerly NHF

