

Policy Working Group: Advocating with the Community

January 2024
Arlington, Virginia



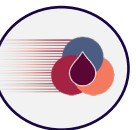
NATIONAL
BLEEDING DISORDERS
FOUNDATION
Formerly NHF

PRESENTERS :
Nathan Schaefer, MSW MCHES

Disclosures

Nathan Schaefer, MSW

None to disclose



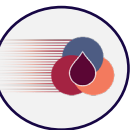


Policy Working Group Co-Chairs

- Michelle Conde, Hemophilia of Georgia
- Nathan Schaefer, NBDF
- Rich Pezzillo, NEHA

How do Public Policy & Research Intersect?

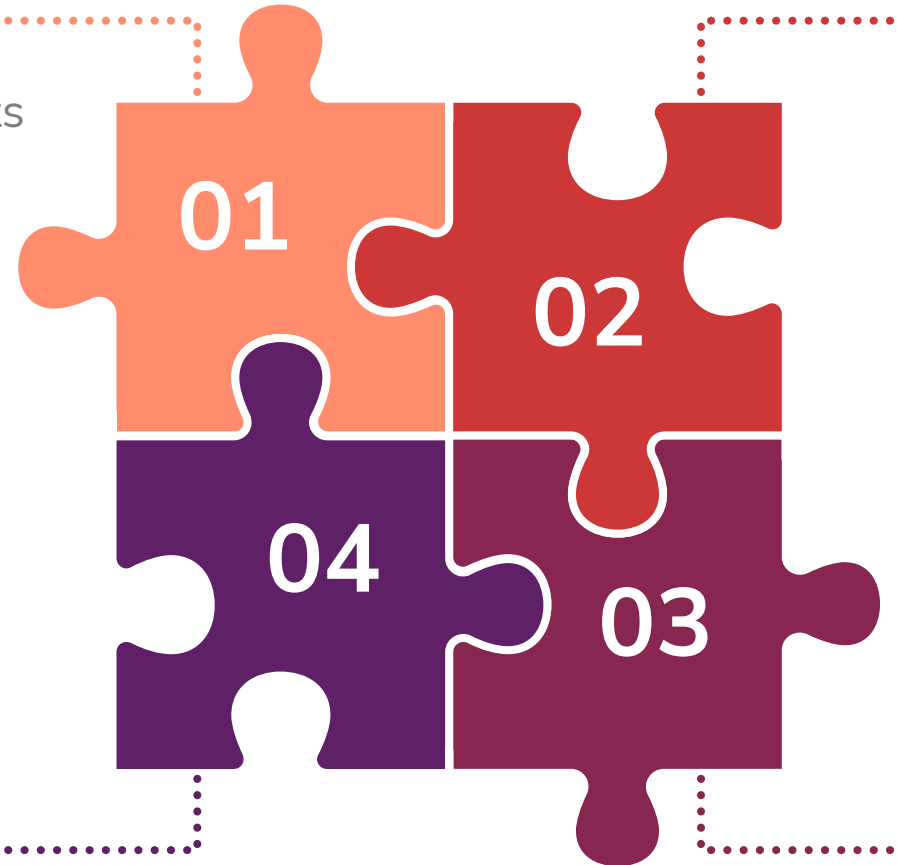
- Public policy impacts research in a number of ways:
 - Increase/decrease funding levels
 - Guide topics/areas of research
 - Impact who participates in research and the rules around participation
 - Create/fund surveillance/data collection programs
 - Both Congress and federal agencies are involved
- Which require legislation? What can be accomplished via regulation?
 - Which approach is more durable?



Critical Policy-Related Stakeholders

Lived Experience Experts (LEEs)

Provide firsthand insights into patient experiences



Policymakers

- Federal and state legislators
- Government agencies

Advocacy Groups

Influence public opinion and policymakers

Medical and Industry

- Medical providers directly involved in patient care
- Industry representatives



Agencies that Do/Could Support BD Research and Surveillance Activities

- National Institutes of Health (NIH)
- Advanced Research Projects Agency for Health (ARPA-H)
 - Current focus areas: Health Science Futures, Scalable Solutions, Proactive Health, Resilient Systems
- Centers for Disease Control and Prevention (CDC) / Division of Blood Disorders (DBD)
- The Department of Defense Congressional Directed Medical Research Programs (CDMRP)
- Patient Centered Outcomes Research Institute (PCORI)
- Congress involved in much of this policymaking and priority setting



Federal Policy Actors Affecting HTC Sustainability

HTCs being able to earn and spend revenue to support comprehensive care – and research – is key to the NRB!



Current Policy Barriers to Hematology Research

Limited funding:

- Identify challenges in obtaining adequate research funds
- Identify ways to leverage existing funding opportunities and seek out new opportunities
- Advocate for increased funding to overcome barriers

Regulatory complexities:

- Examine hurdles in regulatory processes affecting study enrollment
- Propose solutions to streamline and improve regulatory requirements

Competition and access issues:

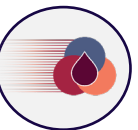
- Advocate for policies that ensure fair competition AND increase diversity and inclusion in clinical trials
- Address the zero-sum game of research funding



Clinical Trials Access & Diversity



- Congress and the Administration have prioritized increasing diversity of and access to clinical trials.
 - Consolidated Appropriations Act, 2022, included a number of provisions related to diversity of clinical trials.
 - FDA Draft Guidance on Decentralized Clinical Trials → a clinical trial where some or all of the trial-related activities occur at locations other than traditional clinical trial sites.
 - NIH Clinical Trials Diversity Act
- Impact on the bleeding disorder community?
 - Expand diversity in clinical trials to be more inclusive of all those impacted by bleeding disorders, across different races, ethnicities, gender, geographic areas, etc.



Clinical Trials Access & Diversity: WGPPM

- Women, girls, and people with the potential to menstruate (WGPPM) have been excluded from hemophilia-related clinical trials, including those for the two gene therapy products, despite their diagnosis of hemophilia.
- NBDF and others are working to make the bleeding disorders community more inclusive of WGPPM, including exploring ways in which to better involve WGPPM in research and clinical trials for bleeding disorder treatments.

**Improving
Outcomes for Women,
Girls, and People Who
Have or Had the
Potential to
Menstruate (WGPPM):
A Roadmap for Moving
Forward**



Author: Mary Hicks
Editors and Reviewers: Kate Nammacher, Jasmine Pauldurai, Anne-Marie Rodriguez de Killeen, Dawn Rotellini, Natalia Winberry, Lena Volland
November 2023

Role of Public Policy to Support National Research Blueprint

Policies
facilitating
research
funding and
prioritization of
NRB
topics/areas

Collaboration
incentives for
researchers,
LEEs, and
medical
providers

Accelerating
research
progress
aligned with the
National
Research
Blueprint (NRB)
goals

Specific Policy Ideas To Support the NRB



Research & Development (R&D)

- Appropriations strategy to support funding for specific projects (funding/report language)
- Advocacy with Congress / FDA related to regulatory pathways and guidance for research in BDs

Infrastructure (IFS)

- Advocate for federal funding for bleeding disorders research
- Support policies to facilitate HTC's using program income to support research
- Incorporate policy updates / calls to action in regional meetings

Lived Experience Expert (LEE)

- Provide training and resources to LEEs on how to engage with policymakers to garner support for research funding - Network of Research Champions?
- Policy work to support WGPPM



Specific Policy Ideas To Support the NRB



Community Engagement (CE)

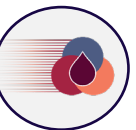
- Advocacy to support translation of research into clinical care at HTC
- Add LEEs and researchers to CCSC to build relationships with payers

Workforce (WF)

- Increase policymaker support for HTC model generally and use of 340B program income to support NRB activities
- Continue work on HTC sustainability more generally

Health, Equity, Diversity & Inclusion (HEDI)

- Advocate for legislation and regulation that will increase diversity of research participants
- Advocate for policy change to address social determinants of health (SDOH)
- Increase inclusion in NBDF advocacy activities



Steps for Policy Change/Implementation



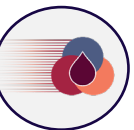
Resources for Effective Policy Advocacy

Funding, staff, policy expertise

- Ensuring financial resources for advocacy campaigns
- Allocating staff and expertise for effective policy engagement
- Collaborating with policy experts and decision-makers
- Trained advocates including LEEs, researchers, HTC staff, chapters, regional leaders, etc

Compelling stories, data, and broad coalition support

- Sharing personal stories to humanize the impact of policy changes
- Presenting data to support the need for policy adjustments
- Building broad coalitions for increased advocacy strength



Optimal Process for Evaluating Policies



Engagement with stakeholders

- Involving LEEs, medical providers, researchers, advocates, policymakers, and industry
- Gathering diverse perspectives for comprehensive policy evaluation

Thorough evidence review

- Analyzing research findings, patient experiences, and industry insights
- Ensuring evidence-based policymaking and evaluation

Recognition of policy opportunities

- Identifying key points in the policymaking process for effective engagement
- Seizing opportunities for policy-related changes in funding and regulations





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