NHF State of the Science (SoS) Research Summit

Working Group 6: R/F

Summary
SUMMARY: Facilitating Priority IBD Research

1. What is the Ideal Research Structure in which to conduct IBD Research?

- One that incorporates research into the HTC model.
- One that supports the HTC and HTC staff to facilitate and sustain research.
- One that engages a multidisciplinary group of individuals to support the network and review proposals: HTCs, foundations, and the community.
- One that is supported by a community lifespan registry.
- One that utilizes the electronic medical record and machine learning/big data.
- One that enables observational studies and clinical trials for ALL community members.
SUMMARY: Facilitating Priority IBD Research

2. What Organizational Model do we envision for research?

- A model that is supported by the Community Lifespan Registry.
- A model that utilizes the electronic medical record and machine learning (big data).
- A model that enables observational studies/ clinical trials for ALL members.
SUMMARY: Facilitating Priority IBD Research

3. Who are the Research Partners to build the IBD research infrastructure?

- **Patient Community**: to engage, plan, and participate in trials.
- **HTC staff**: to train HTC MD, NP, RN, SW to sustain research.
- **Trialists**: to design efficient, remote, decentralized trials.
- **Trials Network**: to develop trial concepts & database CDEs, PROs.
- **HTRS and FWGBD**: to advise on funding fund research, training.
- **NHF**: for oversight of funding and community engagement.
- **Foundations**: to assure sustainable funding for research.
SUMMARY: Facilitating Priority IBD Research

4. What Resources are required for Research Infrastructure?

- **Assurance of diversity, equity, inclusion**: to engage, participate, plan research.
- **Communication**: to explain ongoing research, maintain a trials roster.
- **Network Database**: to utilize common data elements, pt reported outcomes.
- **Data Sources**: to use big data, registries, repositories to define research scope.
- **Menu of Resources**: expertise in trial design, stats, IT, lab, data collection.
- **Informatics**: to use big data for case-finding, biomarkers, prediction models.
- **Biorepository**: for sample repository with privacy & patient use protection.
SUMMARY: Facilitating Priority IBD Research

5. What Funding Partners are needed for IBD Research?

- **Patient Community**: to collaborate in research planning and execution.
- **HRSA**: to help support the HTC personnel and the HTC trial infrastructure.
- **NHLBI**: to prioritize trial funding, support ESI training, and mechanistic studies.
- **CDC**: to promote a surveillance registry to embed trials and standardize assays.
- **Charities**: to help support registries, network, and community in research.
- **Foundations**: to help fund HTC network training & support community partners.
- **Insurers**: to partner to measure outcomes, natural history, EMR research.
- **Industry**: to help fund trials, HTCs, HTC research network, and post licensure trials.
- **CSA**: to leverage trials expertise, diversity, tools for trials, workforce training.
- **NHF**: to develop foundation nonprofit business models to fund research.
Fostering progress together

**Align our Focus**
Bring together the Inherited Bleeding Disorders (IBD) community in our research focus

**Define our Priorities**
Identify 4-6 research priorities and a framework that the community can advance

**Foster Progress through Collaboration**
Establish a functional platform that encourages partnerships and collaboration beyond IBD
Key milestones in our journey, each opportunities to listen and learn from our subject matter experts

- **2020**
  - Community Focus Groups
  - Subject Matter Expert (SME), HCP and industry listening sessions

- **2021**
  - Cross-community Survey
  - State of the Science Research Summit

- **2022**
  - NRB Development

- **2023**
  - Target NRB Roll-out
Blue Sky Vision

NHF will work closely with providers, patients, payers, policy-makers, and other partners to achieve our 2030 Vision

Health and Social Equity

Maximize the Value of Technology

Sustainability and Viability
Comprehensive listening sessions

98 participants over 14 sessions

- 31% individuals with IBDs
- 11% caregivers
- 44% health care professionals
- 28% leaders of NHF chapters and patient organizations
- 16 industry members representing 7 global companies

Some participants filled multiple roles

Primary topics covered:

- Experiences living with a bleeding disorder
- Most bothersome symptoms and unmet medical needs
- HCP relationships and treatment experiences; information sources
- Perceptions of registries, observational research and clinical research

- Priority areas of research and any barriers to completing research in these areas
- Ways to improve relationships between HCPs and patients
- Addressing barriers to clinical trial participation
Cross-Community Survey

- Survey distributed through NHF Chapters and FDA members to individuals and families
- Separately disseminated to HTC network providers
- Goal: Comprehensively include community voices in shaping research priorities by collecting input on emerging themes
Virtual State of the Science Research Summit

<table>
<thead>
<tr>
<th>Sept 12</th>
<th>Sept 13</th>
<th>Sept 14</th>
<th>Sept 15</th>
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<tbody>
<tr>
<td><strong>Morning Session</strong>&lt;br&gt;11 am – 2:30 pm ET&lt;br&gt;8 am – 11:30 am PT</td>
<td><strong>Research Priorities for Hemophilia A and B</strong></td>
<td><strong>Research Priorities for von Willebrand Disease, Platelet Dysfunction &amp; other mucocutaneous IBDs</strong></td>
<td><strong>Diversity, Equity &amp; Inclusion Health Services Research &amp; Implementation Science</strong></td>
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<tr>
<td><strong>Break</strong>&lt;br&gt;2:30 pm – 3 pm ET&lt;br&gt;11:30 am – 12 pm PT</td>
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<td><strong>Summary</strong></td>
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<tr>
<td><strong>Afternoon Session</strong>&lt;br&gt;3 pm – 6:30 pm ET&lt;br&gt;12 pm – 3:30 pm PT</td>
<td><strong>Research Priorities for Ultra-Rare IBDs</strong></td>
<td><strong>Research Priorities for health of women &amp; girls and persons with the potential for menstruation</strong></td>
<td><strong>Facilitating Priority Research in the IBDs Community</strong></td>
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</table>

**Missed it? Panel recordings are still available!**
hemophilia.org/events/nhf-state-of-the-science-research-summit
Clear themes and priorities have emerged from this work to date

- Understanding the pathobiology of bleeding
- Novel and improved diagnostics
- Novel and adapted therapeutics
- Impediments to care
- Bone and joint health
- Influence of sex and gender on disease
- Aging
- Immunogenicity
- Mental health
- Pain
- Research infrastructure
Thoughtful, methodical progress toward our goal

- Community Focus Groups
- Cross-community Survey
- NRB Development

2020:
- Subject Matter Expert (SME), HCP and industry listening sessions

2021:
- State of the Science Research Summit

2022:
- Target NRB Roll-out

10 March 2022
Thoughtful, methodical progress toward our goal

2020

- Community Focus Groups

2021

- Cross-community Survey
- Subject Matter Expert (SME), HCP and industry listening sessions
- State of the Science Research Summit

2022

- NRB Development

2023

- Target NRB Roll-out

WHAT do we need to accelerate

Hearing the pressing needs from the community

10 March 2022 National Research Blueprint
Thoughtful, methodical progress toward our goal

Community Focus Groups

Cross-community Survey

NRB Development

2020

2021

2022

2023

Subject Matter Expert (SME), HCP and industry listening sessions

State of the Science Research Summit

Target NRB Roll-out

Hearing the pressing needs from the community

WHAT do we need to accelerate

HOW can we accomplish these goals?

10 March 2022

National Research Blueprint
NRB: Fueling this ambitious vision together with the IBD community

A 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

National research infrastructure expansion that supports the envisioned research enterprise through collaboration and inclusion

Reinvigorated and sustainable workforce across comprehensive care and scientific disciplines that advances the health of the IBD community through the seamless integration of care and research
A world without inheritable blood disorders... starts with research.
Thank you for joining us on this journey!
How we’ll work together
Developing the NRB this year together

SOS Summit

2021
✓ Community listening sessions
✓ Focus groups
✓ Community Survey
✓ SOS Working Groups (WGs)
✓ State of the Science Research Summit

Spring NRB Workshops

Q1 2022
✓ Chairs appointed
✓ WG Chair Orientation
✓ WG Chairs attend March meeting
  • Begin workplan & workflow development
  • WG members are appointed
  • WG meetings scheduled
  • First draft of workplans and workflows established / communicated to NRB
  • Priority areas for implementation/integration are drafted/communicated to NRB
✓ April workshop
  • WGs continue work with NRB liaison to refine priority areas for implementation/integration
  • First round of cross-group meetings scheduled
  • WGs priority development plans show preliminary integration with other WG priorities

NHF’s BDC

Q2
✓ More developed and integrated priority implementation/ integration plan
  • Finalized BDC communication & feedback plan
  • Bleeding Disorder Conference in August – opportunity for community feedback
  • Second round of cross-group meetings scheduled

Q3
✓ WGs absorb BDC feedback
  • Cross-group meetings
  • Advanced plans aim for full integration across WGs
  • Teleconferences scheduled between WG Co-Chairs & NRB for progress reports/ next steps
  • Final integrated prioritized implementation plan delivered from each WG

Q4
✓ Co-development of final integrated blueprint
  • Final draft blueprint delivered for review and comment

NRB Roll-out

2023
✓ Present final NRB to the community
Thoughtful WG membership

- Distinctive Expertise: Direct, robust topic expertise based on the mandate of the WG
- Diverse Views: Thoughtfully integrated diverse perspectives to contribute to debate
- Sector Representation: Cross-community representation, including multi-disciplinary teams, government, industry, etc.
- Subject Matter Experts: Representation from those whose interests will be served by the NRB, including patients, caregivers and family
R&D, Infrastructure, Workforce Cluster

Cluster Charge

- Develop a strategic plan, in close collaboration with the SME, HEDI & Community Engagement (CE) WGs, for NHF’s one- and five-year goals for implementation of the NRB that includes:
  - Refinement of the list of the most feasible and impactful research in each of the SOS WG priority areas that will drive the development of coordinated research funding initiatives across private and public partners
  - Finalization of the elements and organization of an integrated care/research infrastructure that supports NHF’s envisioned research enterprise including processes to integrate SMEs in all stages of research development
  - Development & prioritization of an inclusive workforce development schema that will drive the development of coordinated training and skills development initiatives across private and public partners
  - Outline of a plan for community engagement in the emerging research enterprise

Working Group Chairs

- Research & Development (R&D): Jill Johnsen
- Infrastructure: Moses Miles Margaret V. Ragni
- Workforce: Alice Ma

Steering Committee Liaisons

- R&D: Kevin Mills
- Infrastructure: Michelle Witkop
- Workforce: Mike Recht
Chair
• Develop a strategic plan, in close collaboration with each of the other WGs, for NHF’s one- and five-year goals for implementation of the NRB that includes:
  • Collaboration with the Research and SME WGs to develop a schema for
    • the equitably accessible, socially just, and people centered integration of specialty and community-based care and research that steadily advances the standard of care for people with IBDs
    • Prioritization of the HEDI and social justice principles that will inform the envisioned care and research enterprise
  • Prioritization of the HEDI and social justice principles that will inform the envisioned care and research enterprise
  • Work with the Policy WG to ensure that NHF policies related to the care/research enterprise reflect the prioritized HEDI & social justice principles
  • Work with the CE WG on a communication strategy for emerging NHF policies

WG Charge

Working Group Chairs
Melissa Creary, Keri Norris

Steering Committee Liaison
• Maria Santaella
SMEs

Working Group Chairs

TBD
Esmeralda Vázquez

Steering Committee Liaison

• Maria Santaella

WG Charge

• Develop a strategic plan, in close collaboration with each of the other WGs, for NHF’s one- and five-year goals for implementation of the NRB that includes:
  • Collaboration with the Research and HEDI WGs to develop a schema for
    • SME-informed, SME-engaged, and SME-guided integration of culturally diverse & equitably accessible people-centered specialty and community-based care and research that steadily advances the standard of care for people with IBDs
    • Prioritization of SME principles and codification of fully integrated SME-participation procedures that will inform and guide the creation of an envisioned care and research enterprise that begins and remains people-centered
  • Work with the Policy WG to ensure that NHF policies related to the care/research enterprise reflect the prioritized SME principles
  • Work with the CE WG on a communication strategy for emerging NHF policies in which the SME voice is front and center
Community Engagement

Chair

- Develop a strategic plan, in close collaboration with each of the other WGs, for NHF’s one- and five-year goals for implementation of the NRB that fully integrates:
  - Work with the Research/Workforce Cluster WG on a plan for community engagement in the emerging research enterprise
  - Work with the HEDI WG to develop a community engagement policy for emerging NHF policies that prioritize HEDI principles
  - Work with the SME WG to develop a community engagement policy for emerging NHF policies in which the SME voice is front and center
  - Develop a fully integrated community engagement policy and plan (research acculturation) across all channels that starts community messaging before an implementation plan is finalized to evolve the concept of what it means to do high quality research in a rare disease community and the requirement for participation at all levels

Working Group Chairs

Looking for a volunteer

WG Charge

- Brett Spitale

Steering Committee Liaison

Looking for a volunteer

Looking for a volunteer

Steering Committee Liaison

- Brett Spitale
• Develop a strategic plan, in close collaboration with each of the other WGs, for NHF’s one- and five-year goals for implementation of the NRB that fully integrates:
  • IBD community-informed NHF policies that govern an emerging research enterprise that is centered in patient care, informed and guided by the patient voice, equitably accessible and socially just
  • Emerging NHF policies that integrate & prioritize HEDI principles across the organization
  • Emerging NHF organizational policies to ensure the SME voice is front and center
  • Influence broader public research policies with the wider community through engagement, education, and advocacy

Deniece Chevannes  Sonji Wilkes  Nathan Schaefer

Steering Committee Liaison

• Michelle Witkop
Let's come back to the ASK

10 March 2022
What are we asking the NRB WG Chairs to deliver to the IBD community??

By early 2023, the NRB WG Chairs will have completed:
• One- and five-year strategic plans for implementation of a National Research Blueprint (NRB)
• This Blueprint will define priorities for:

A 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
To be successful, the NRB must have:

1. A clear, actionable plan
2. Delineated roles and responsibilities of key stakeholders
3. Well defined milestones and timelines
4. Mechanisms for measurement and progress
Critical elements of the NRB

1. What do you see as the most critical elements of this strategic plan?
2. Based on what we’ve covered so far, are those elements clear?
3. How do we prioritize them?
4. How do we implement them?
5. What is missing?
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<thead>
<tr>
<th>Time</th>
<th>Activity</th>
<th>Moderator(s)</th>
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<tbody>
<tr>
<td>1:30-pm – 1:45 pm</td>
<td>Welcome Back – Instructions for Afternoon</td>
<td>Dr. Kevin Mills</td>
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<tr>
<td>1:45 pm - 2:45 pm</td>
<td><strong>Discussion Group One:</strong> Charges for R&amp;D, Infrastructure, Workforce</td>
<td>Moderators: Drs. Kevin Mills/ Mike Recht</td>
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<td>Charge for Contributions from Subject Matter Experts</td>
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<td></td>
<td>Charge for Contributions from HEDI</td>
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<tr>
<td>2:45 pm - 3:15 pm</td>
<td>Break</td>
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<tr>
<td>3:15 pm - 4:15 pm</td>
<td><strong>Discussion Group Two:</strong> Charges for HEDI, Policy, Community Engagement</td>
<td>Moderators: Dr. Kevin Mills/ Donna DiMichele</td>
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<td>Charge for Contributions from Subject Matter Experts</td>
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<td>4:15 pm</td>
<td>General Audience Released until Reception at 5:00 PM in Lower Level</td>
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<td>Followed by Dinner</td>
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<tr>
<td>4:15 pm - 5:00 pm</td>
<td>NRB WG Chairs Meet to Debrief</td>
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Break for Lunch