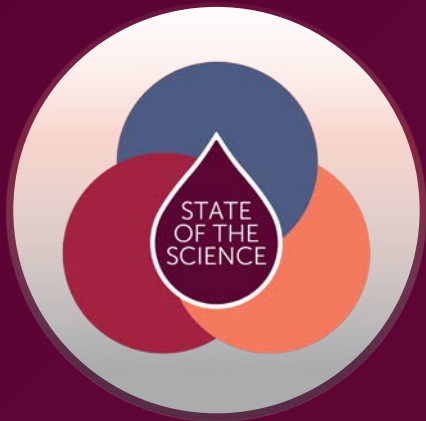




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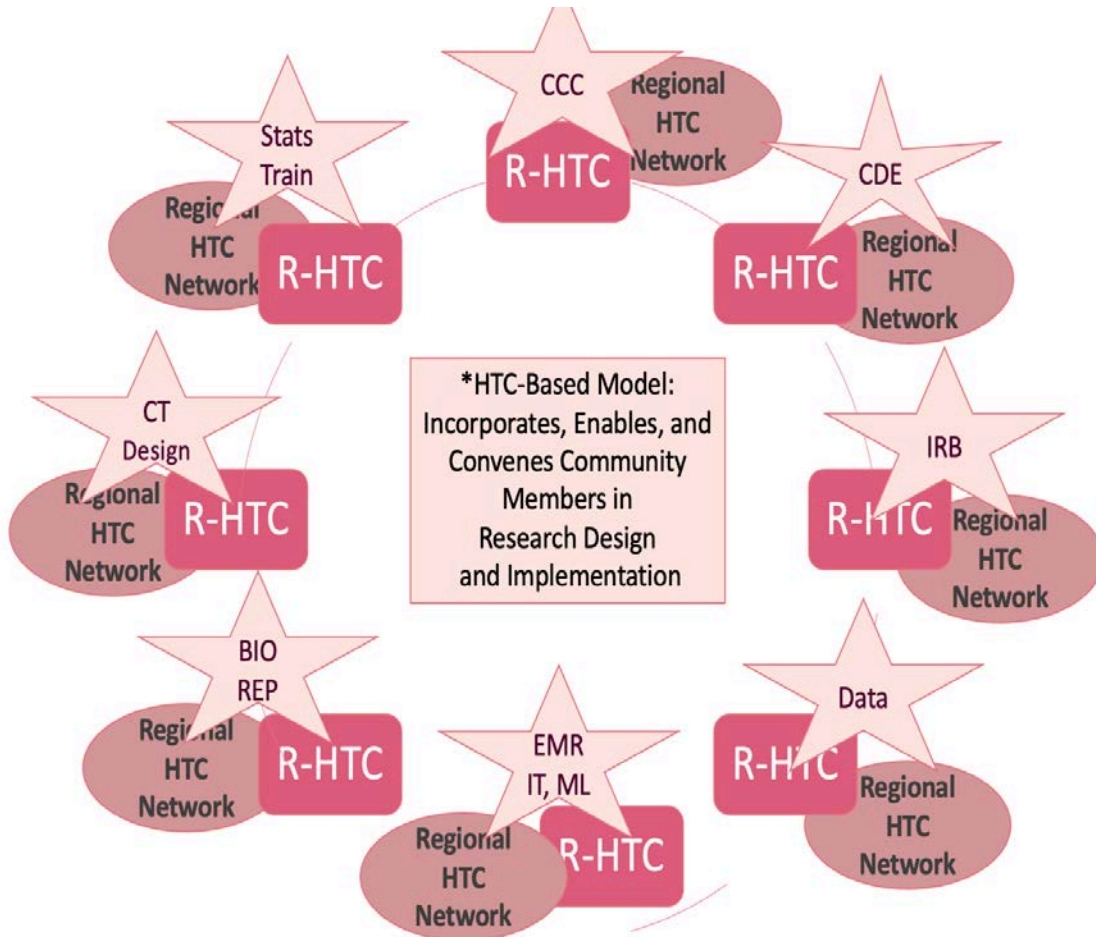
NHF State of the Science (SoS) Research Summit



Working Group 6: R/F

Summary

SUMMARY: Facilitating Priority IBD Research

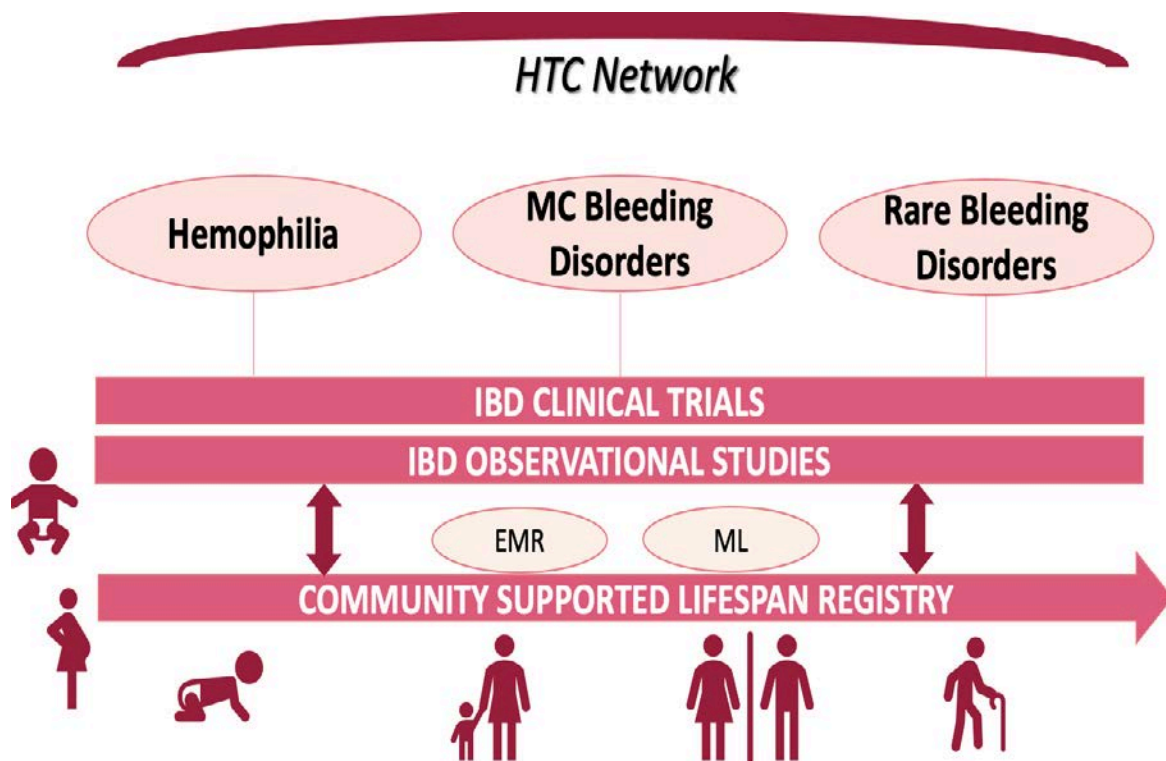


1. What is the Ideal Research Structure in which to conduct IBD Research?

- 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.



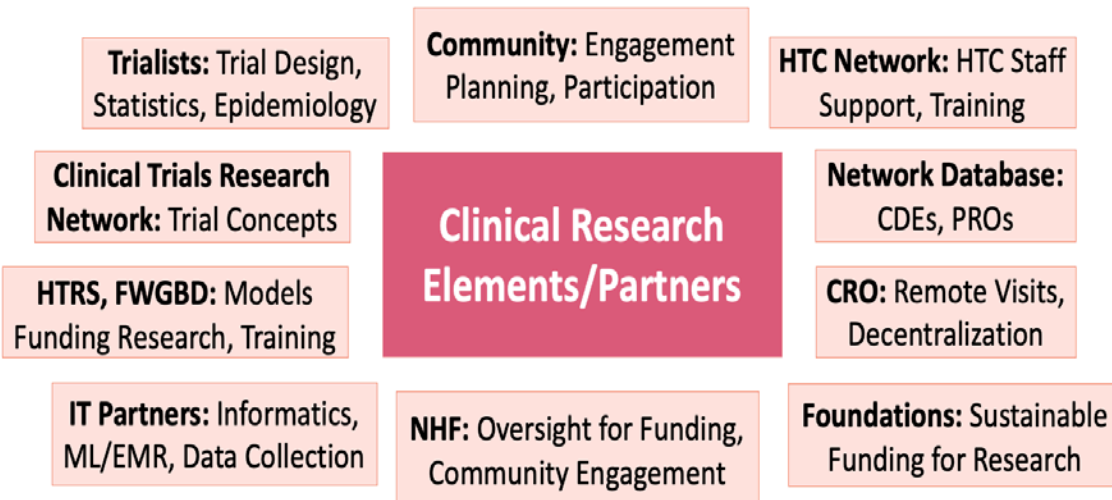
SUMMARY: Facilitating Priority IBD Research



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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



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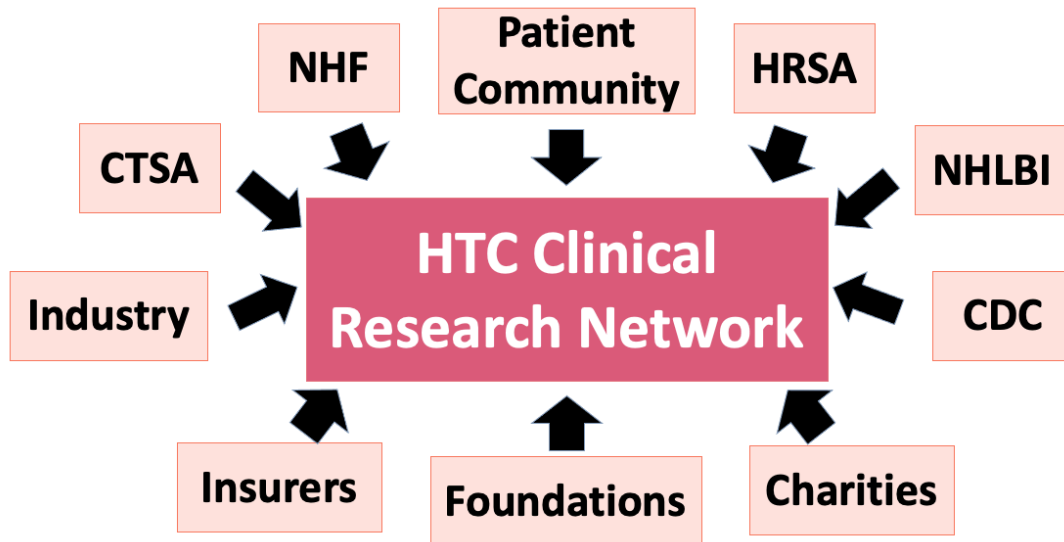


4. What Resources are required for Research Infrastructure?

- **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?

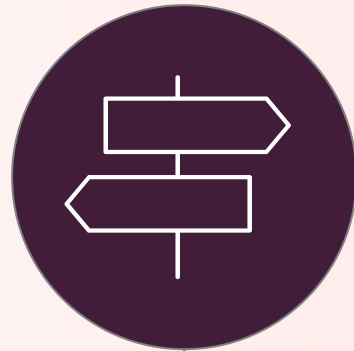
- **Patient Community:** to collaborate in research planning and execution.
- **HRSA:** to help support the HTC personnel and the HTC trial infrastructure.
- **NHLBI:** to prioritize trial funding, support ESI training, and mechanistic studies.
- **CDC:** to promote a surveillance registry to embed trials 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



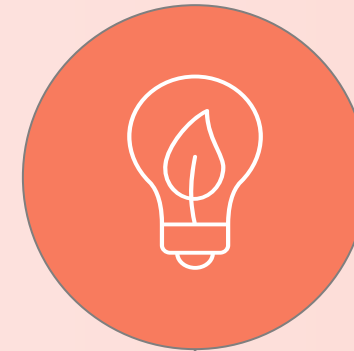
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- **Align our Focus**
Bring together the Inherited Bleeding Disorders (IBD) community in our research focus



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

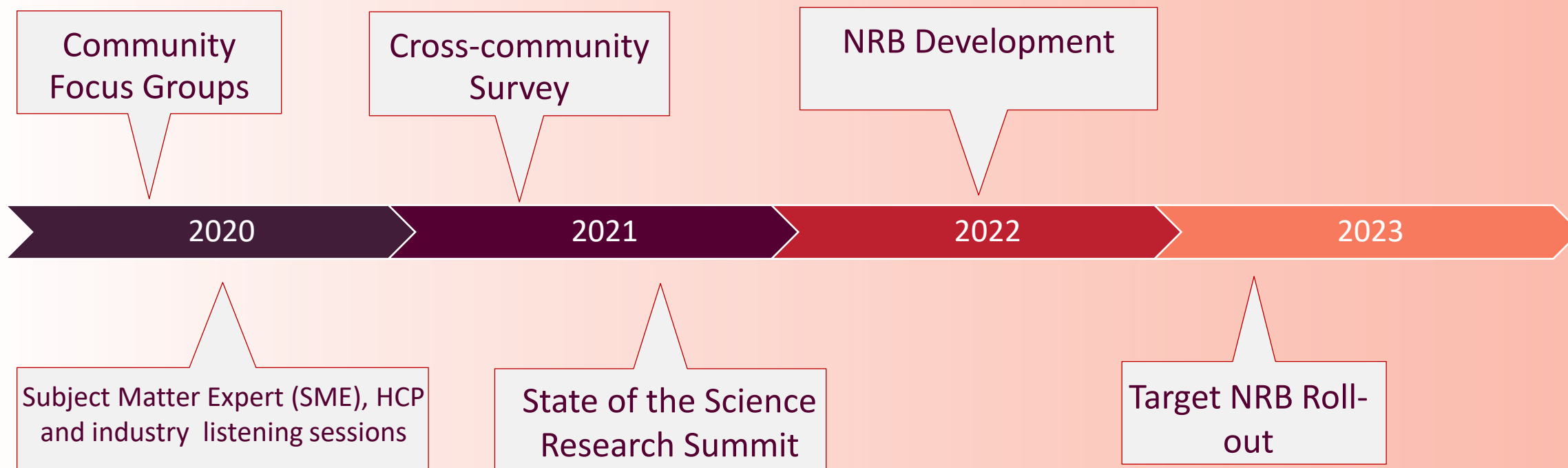


- **Foster Progress through Collaboration**
Establish a functional platform that encourages partnerships and collaboration beyond IBD





Key milestones in our journey, each opportunities to listen and learn from our subject matter experts

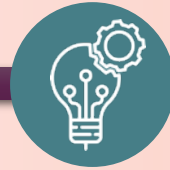


Blue Sky Vision

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



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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**



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State of the Science: Research Priorities Chapter Community Survey

English ▾

The National Hemophilia Foundation (NHF) is hosting a virtual State of the Science (SoS) Research Summit in the fall of 2021. Its goal is to identify priorities to advance research that is important to all members of the inherited bleeding disorders community -including providers, researchers, patients and their families, and all others.

Initial priorities were gathered from listening sessions held over the past several months and in collaboration with a group of chapter and member organization executive directors. Because we want to make sure that they reflect the entire community's interests and needs, we have decided to conduct a community-wide survey to get your input.







Please complete this survey and let us know which priorities are most important to you and/or if we missed any. Tell us what you think should be researched in the next 5 to 10 years to improve the lives of those affected by inherited bleeding disorders.

The survey should take you about 10 minutes to complete. You can take it on your phone but we recommend that you do it on a computer - it is a lot easier.


Thank you in advance for your participation.



Virtual State of the Science Research Summit

	Welcome • 15 mins
	SME Perspective • 2 mins
	Plenary • 45 mins
	Working Group Summary • 30 mins
	Break • 30 mins
	Panel Discussion • 90 mins • Your participation welcome!

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

 **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



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People Centricity



Collaboration



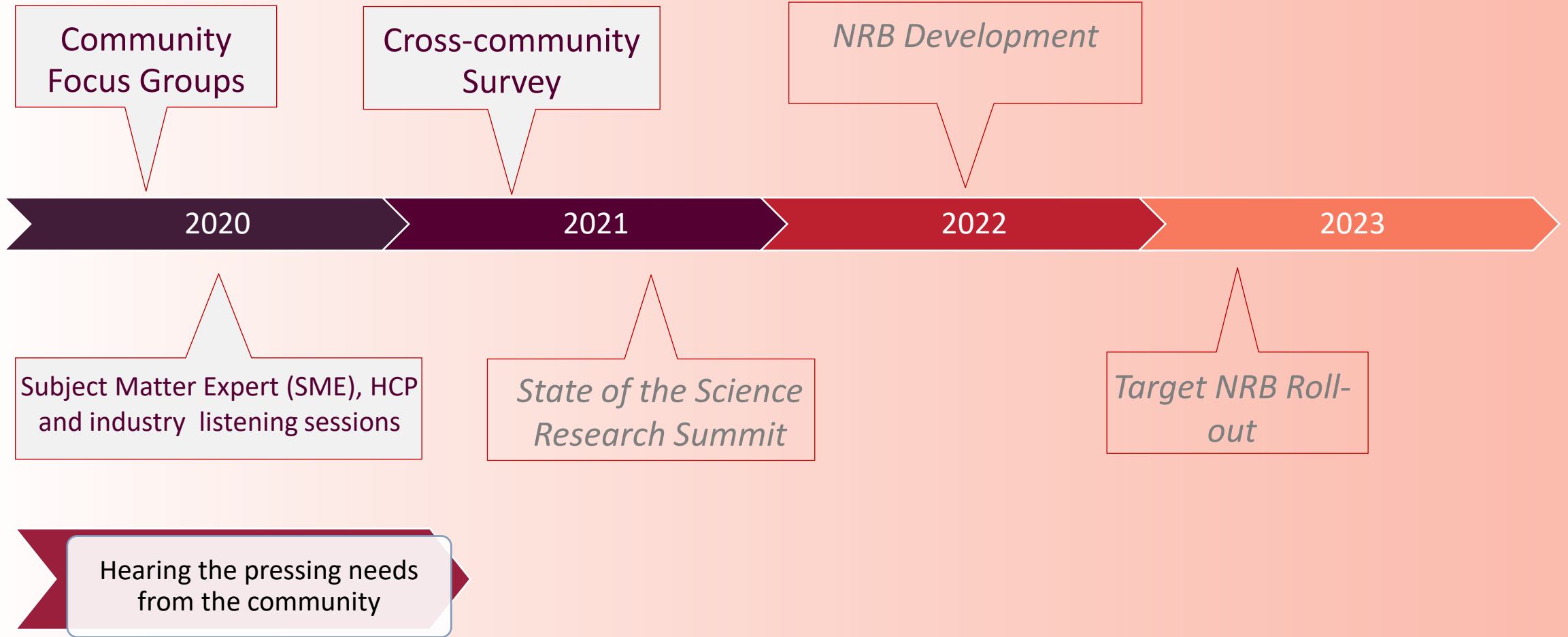
Health Equity, Diversity, and Inclusion

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

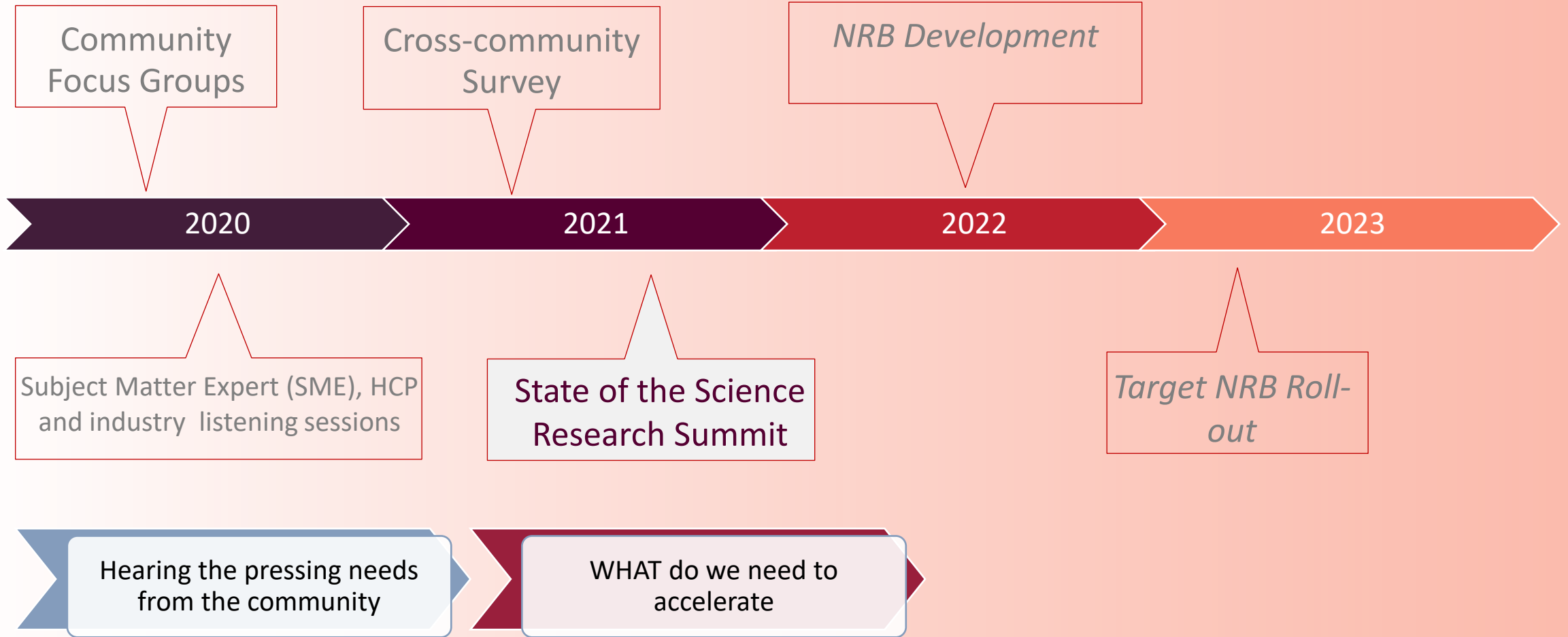
- Bone and joint health
- Influence of sex and gender on disease
- Aging
- Immunogenicity
- Mental health
- Pain
- Research infrastructure



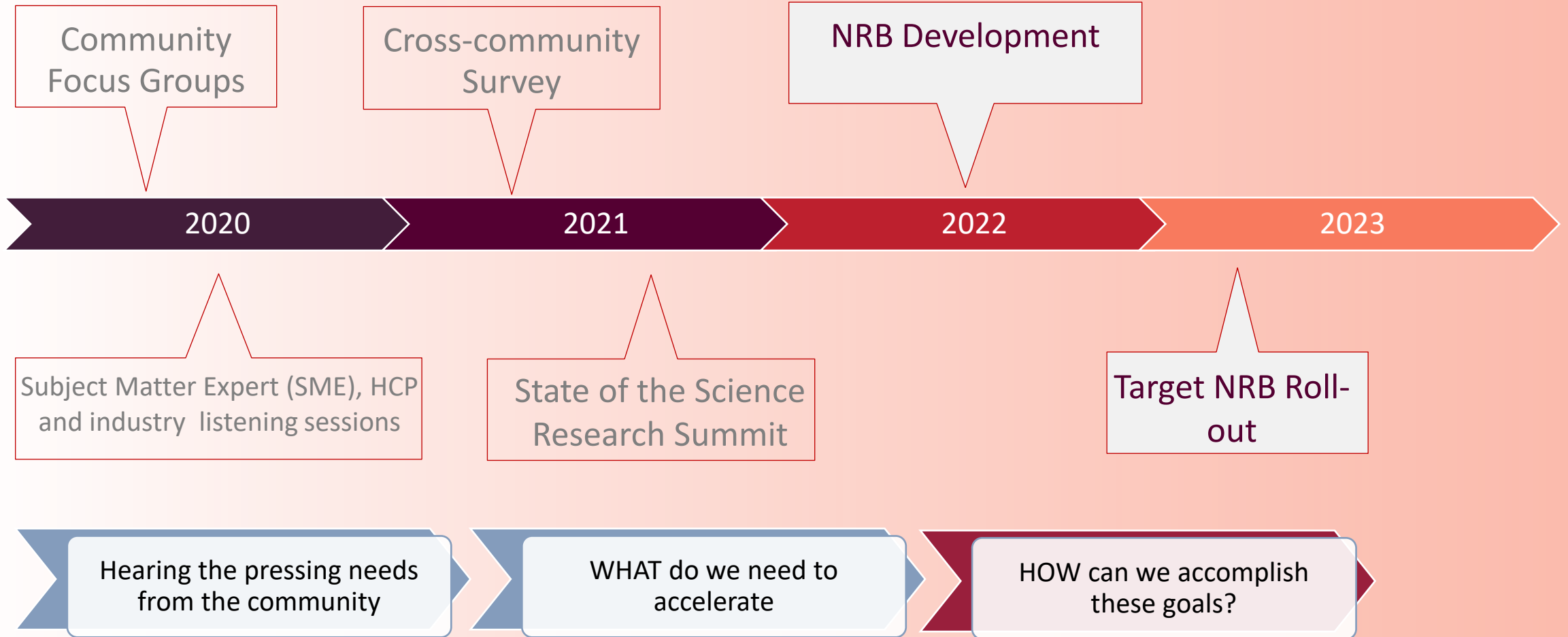
Thoughtful, methodical progress toward our goal



Thoughtful, methodical progress toward our goal



Thoughtful, methodical progress toward our goal



NRB: Fueling this ambitious vision together with the IBD community



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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





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A world
without
inheritable
blood
disorders...

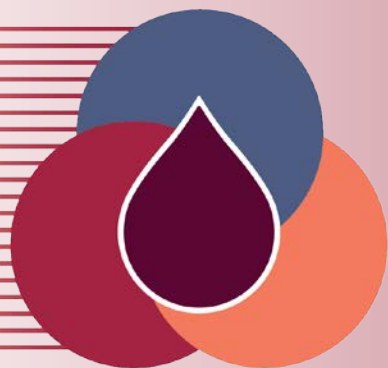
starts with
research.





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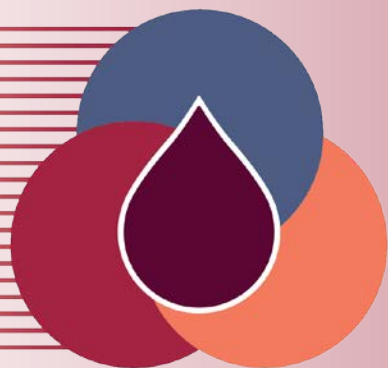


Thank you for joining us on this journey!



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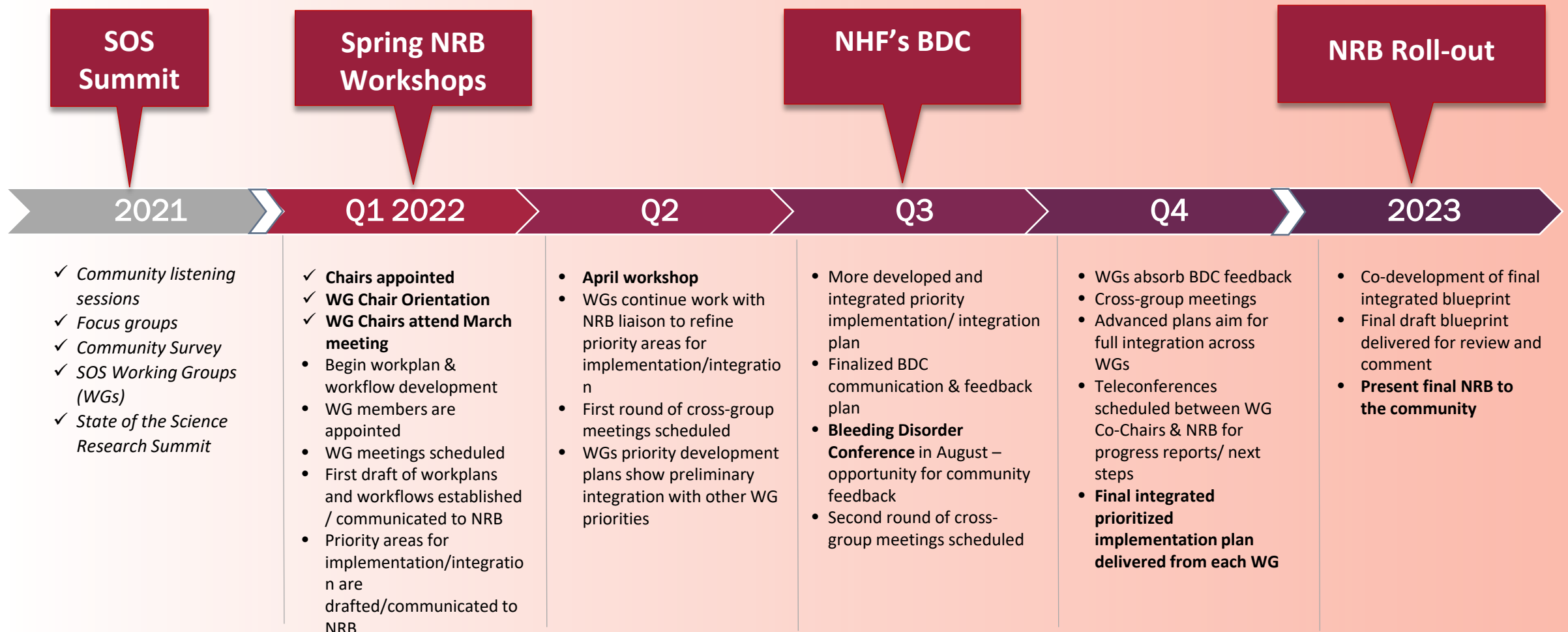


How we'll work together

Developing the NRB this year together



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Thoughtful WG membership



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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



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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



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



Melissa Creary



Keri Norris



Steering Committee Liaison

- Maria Santaella



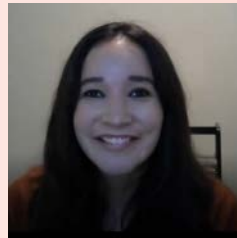
WG Charge

- 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 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
 - 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





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



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Working Group Chairs

Looking for a volunteer

Looking for a volunteer



Steering Committee Liaison

- Brett Spitale



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 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



Deniece Chevannes



Sonji Wilkes



Nathan Schaefer



Steering Committee Liaison

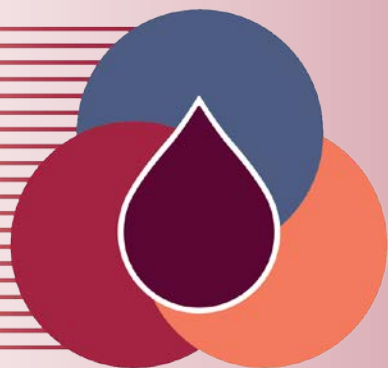
- Michelle Witkop



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 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





Lets 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:



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:

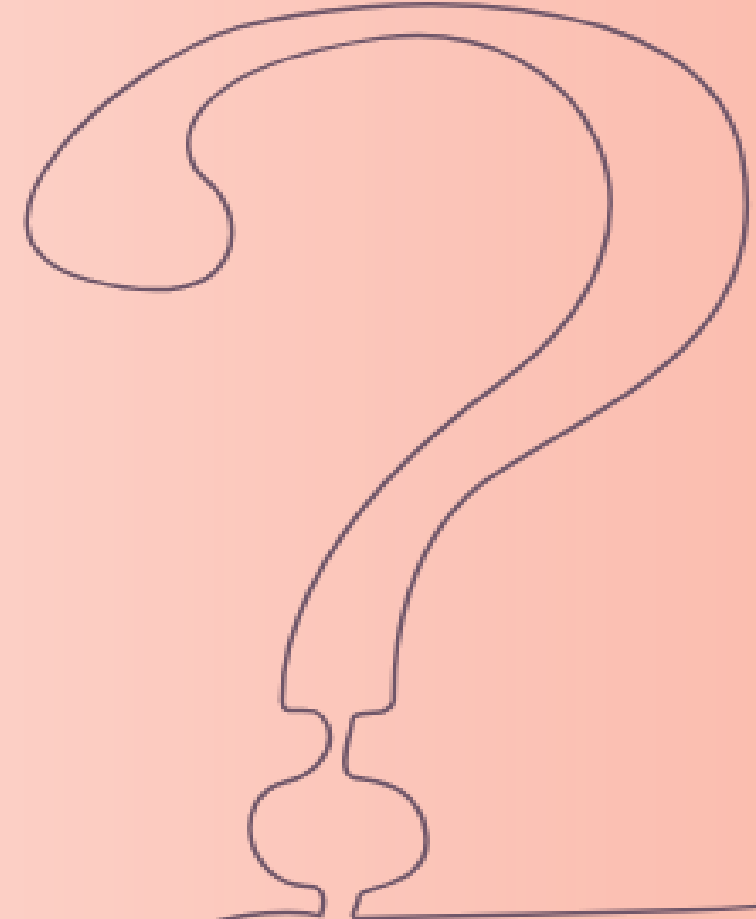


Critical elements of the NRB



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- 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?



Afternoon Agenda



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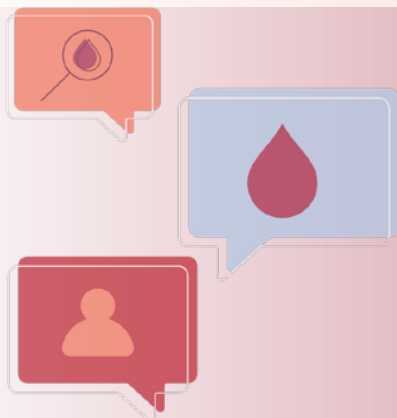
1:30-pm – 1:45 pm	Welcome Back – Instructions for Afternoon	Dr. Kevin Mills
1:45 pm - 2:45 pm	<i>Discussion Group One:</i> Charges for R&D, Infrastructure, Workforce Charge for Contributions from Subject Matter Experts Charge for Contributions from HEDI	Moderators: Drs. Kevin Mills/ Mike Recht
2:45 pm - 3:15 pm	Break	
3:15 pm - 4:15 pm	<i>Discussion Group Two:</i> Charges for HEDI, Policy, Community Engagement Charge for Contributions from Subject Matter Experts	Moderators: Dr. Kevin Mills/ Donna DiMichele
4:15 pm	General Audience Released until Reception at 5:00 PM in Lower Level Followed by Dinner	
4:15 pm - 5:00 pm	NRB WG Chairs Meet to Debrief	





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Break for Lunch

