

# Infrastructure Working Group: *More than the framework behind NRB*

January 2024  
Arlington, Virginia



NATIONAL  
**BLEEDING DISORDERS**  
FOUNDATION  
*Formerly NHF*

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# Disclosures

## **Margaret Ragni**

M. Ragni has research funding to the University of Pittsburgh from BioMarin, Sanofi, Spark, and Takeda, and serves on Advisory Boards of BeBio, BioMarin, Hema Biologics, Sanofi, SPARK, and Takeda.

## **Moses Miles**

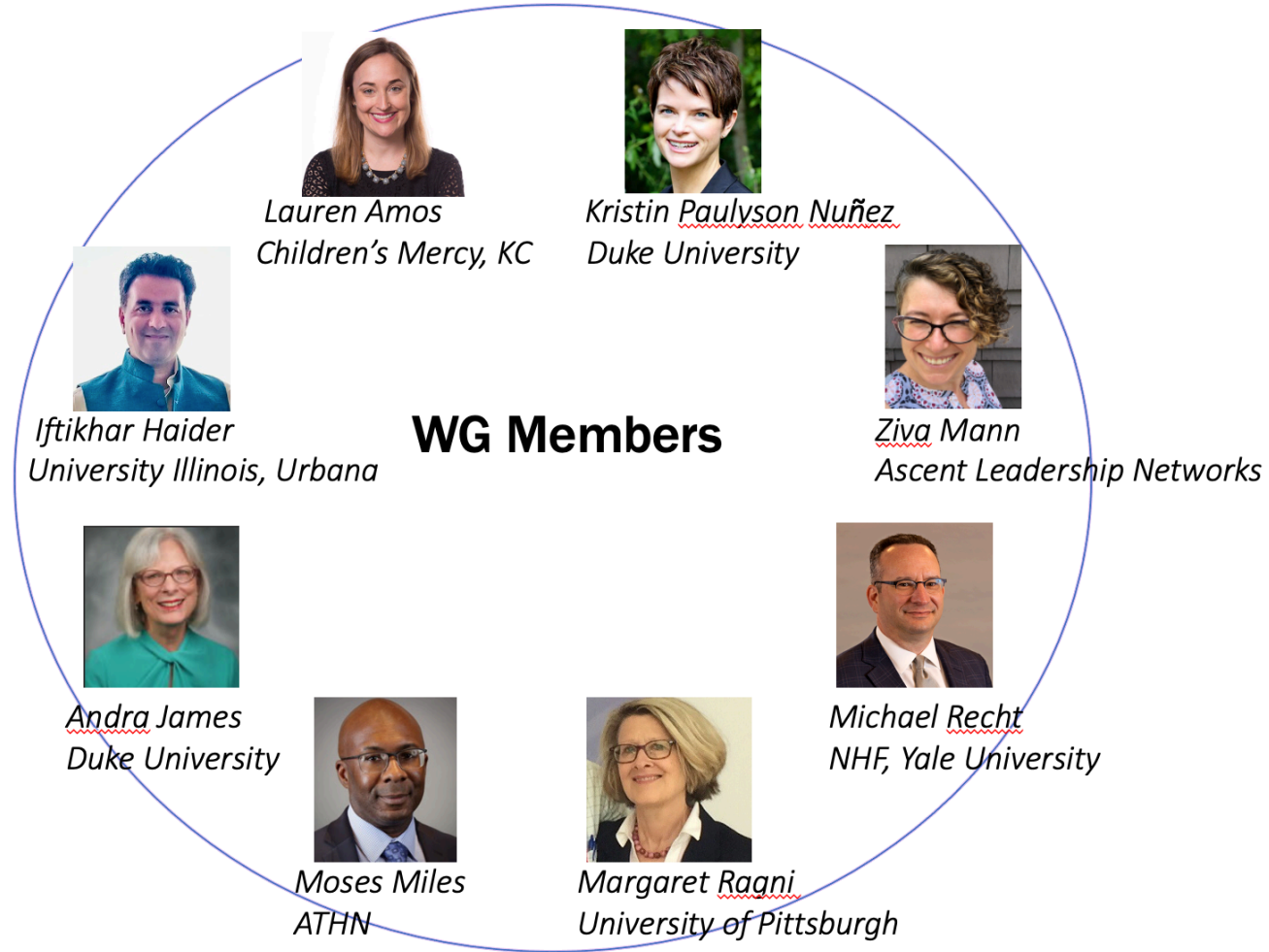
M. Miles serves as the Chief Operating Officer, American Thrombosis Hemostasis Network.

## **Ziva Mann**

Z. Mann serves on the Board of Directors, National Bleeding Disorders Foundation.



# Infrastructure Working Group Members



# NRB Infrastructure Core Elements

- 1. Guiding Principles**
- 2. Dynamic Governance**
- 3. Project Process Map**
- 4. Member Engagement**
- 5. Minimum Viable Product**

# What were we thinking??

Groundbreaking  
work

A range of  
ideas, from a  
range of sources

Deliberately  
collaborative  
environment

Learning &  
adapting  
network



# What we heard:



# What we heard:

## Groundbreaking work

- **Selection criteria** that support new ideas.
- **Cross-cutting work** (“no one-offs”).
- Projects that “**make lives better.**”
- 1<sup>st</sup> project **bringing people together** for the next.
- **Partners** to support.

## A range of ideas, from a range of sources

- **Communication, communication, communication!**
- **Mentoring, support** for ideas.
- **Cross-pollination.**
- **Active outreach:** ask what projects are meaningful.
- **Research linked to clinical care:** policy, advocacy, funding.

## Deliberately collaborative environment

- **Peer-to-peer** connections.
- **Active outreach** to voices, communities not heard.
- **Mandates for meaningful LEE engagement** at multiple levels.
- **Facilitation** and how it is going to be critical.

## Learning & adapting network

- **External accountability.**
- Making sure network is **true to the principles.**
- **Transparency.**
- Make it **easy to raise and resolve issues.**
- **Training, coaching.**

# Recommendations

- 1. Teams:** Convene, **seek partners**, use **Guiding Principles** and **Dynamic Governance** to launch teams, set tone; recruit from range of backgrounds, experiences, expertise and more, with representation on the Steering Committee.
- 2. MVP:** Gather **just what is required** to launch network, then grow, expand resources. Includes: gathering **resources**, building **support** processes, **opportunities** (orient, train, engage peers, mentor, coach, grow incrementally.)
- 3. R&D Project Portfolio:** Select **criteria to choose projects** to start, define processes, timelines, milestones. Plan HEDI, LEE-inclusive processes for selection. Recognize hand in hand fit of **research & clinical care**, to promote **advocacy, policy, and funding**.
- 4. Project Roll Out:** Develop teams, LEE peer groups, incorporate HEDI principles for each study. Define **qualities, principles, timeline, and milestones** for each protocol.
- 5. Define success:** Develop **family of metrics** (outcome, process) to evaluate projects and network success in research, collaboration, teamwork, HEDI principles, Lee integration.
- 6. Learn, adapt, improve:** Evaluate success, **recognize and address challenges** openly, using **Dynamic Governance**. Communicate transparently with network and beyond. Share learning, ideas across network, encourage cross-pollination and mutual support.



# What is Dynamic Governance ?

Trust is overarching

- Shared purpose across the organization.
- Processes foster collaboration.

Enables multiple representative voices

- Perspective, not hierarchy, drives decisions.
- Balanced representation at leadership level.

Assures work is productive, with shared purpose

- Frequent check-ins: how the group works.
- Members engaged around the work.

Enables members to define a problem together

- Inclusive process to move to decisions.
- Supports iterative design.

If disagreement, returns to the original agreement (GP)

- Promotes members to reach agreement.
- *"Can we live with this? Is it worth trying out?"*

# What is the Project Process Map?

## Project Process Map:

- A roadmap from initial point of contact.
- Supports startup, from orientation to training.
- Includes links to training, education, participation, collaboration, communication for project team.
- Provides resources, capacity building, mentoring, partnering.
- Promotes member engagement, LEE participation.
- Provides links to peers, contacts, education, training, network.



# What is Member Engagement?

## **Network Member Engagement:**

- A roadmap from initial engagement to ongoing involvement (at preferred levels).
- With training, mentoring to promote participation.
- With pathways to volunteer, join, participate in WGs, committees.
- Contributing LEE & community insights for concept development.
- Assuring HEDI representation, principles in writing, execution.

# What is Minimum Viable Product (MVP)?

## 1. Process

- Just enough of a Project.
- With just enough features.
- Usable by early consumers.

## 2. Iterative Design

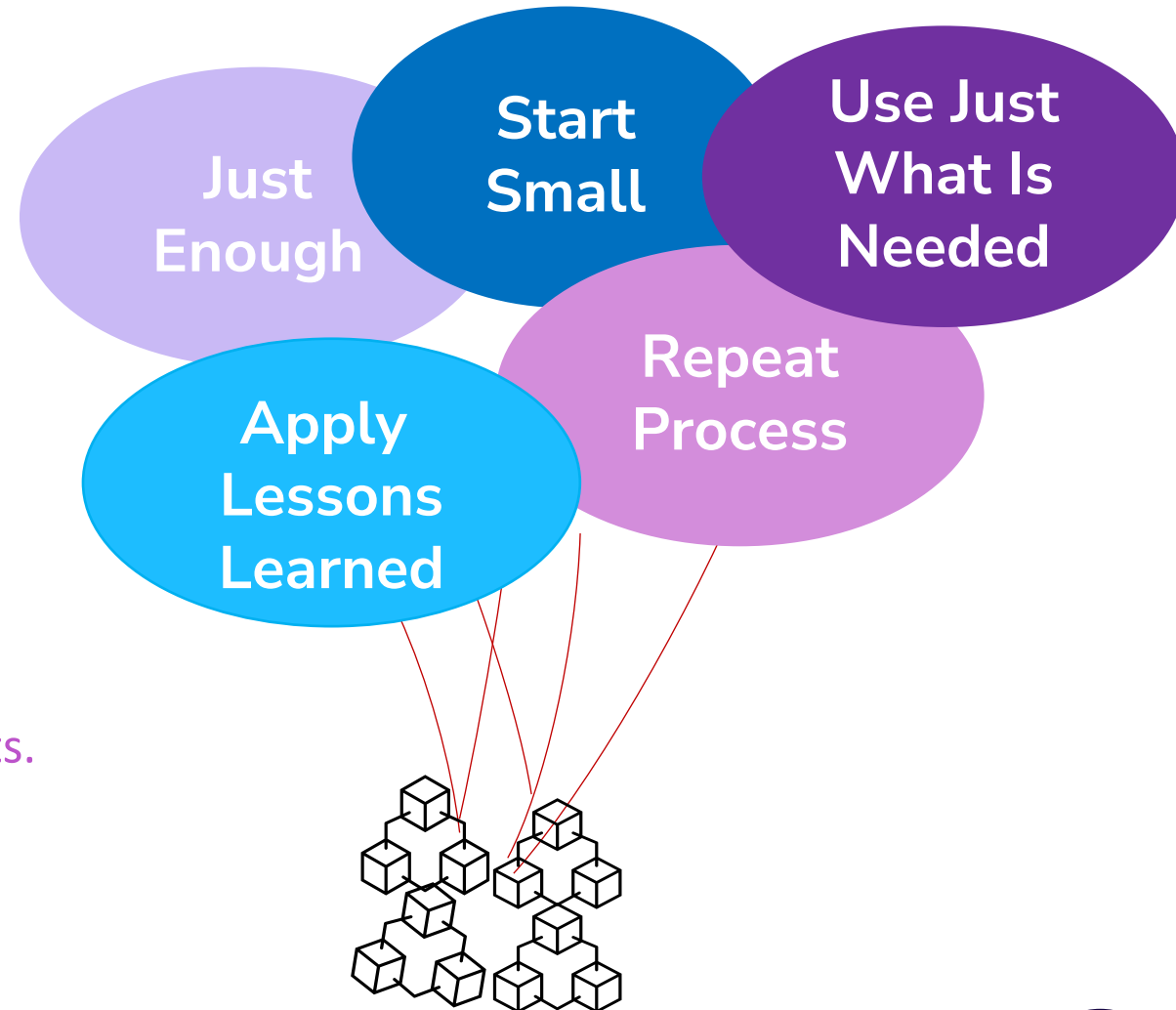
- Start small, evaluate.
- Apply lessons learned.
- Repeat the process.

## 3. Meaning for NRB

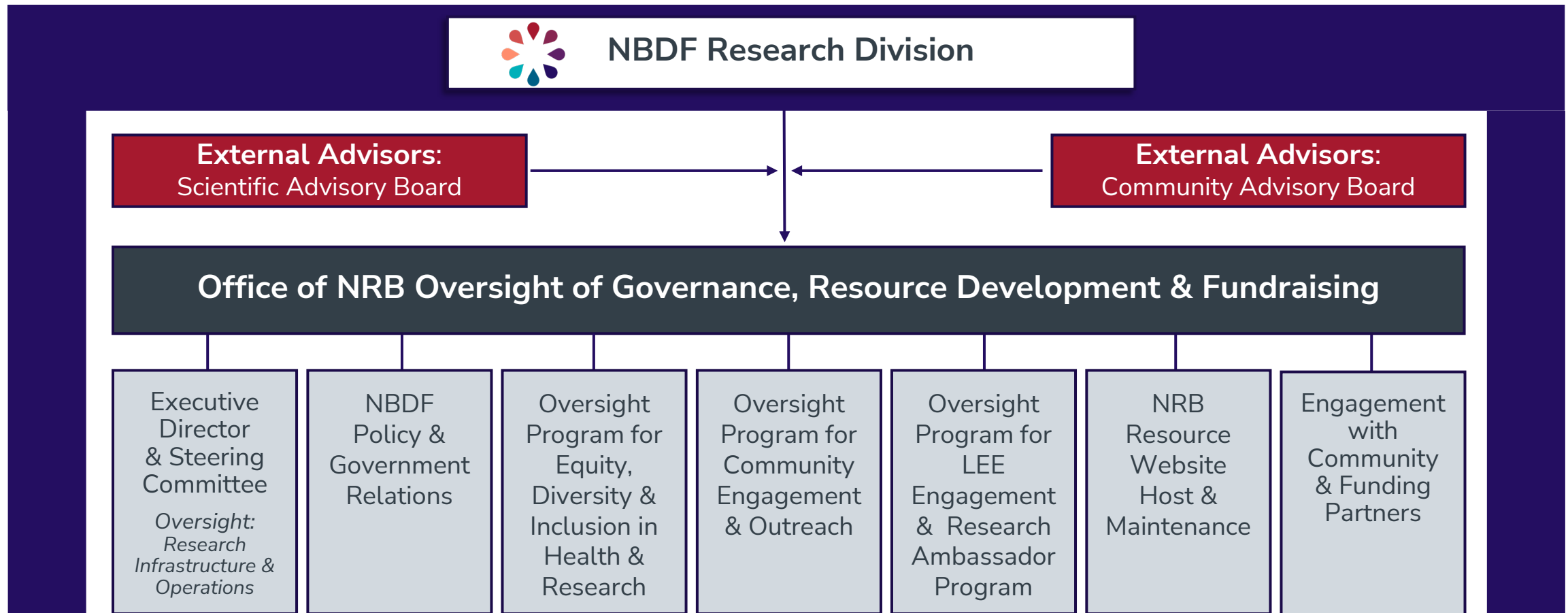
- Identify a project or projects.
- Identify what is core to those projects.

## 4. Action Step

- Implement just what is needed.



# What is the Organizational Structure?



\*LEE: Lived Experience Expert  
\*HEDI: Health Equity Diversity and Inclusion

# How are WGs, Committees Coordinated?

## Coordinated Operations

Executive Director & Steering Committee  
*Dynamic Governance Model for Oversight of Research Infrastructure & Operations*

### 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

## How will NRB Facilitate Research?

- LEE:** Access, representation, engagement, training, participation, providing input to research priorities, with oversight program to promote trust.
- HEDI:** HEDI representation on every WG, committee; HEDI principles incorporated into every protocol.
- CE:** Public input, website to engage, orient, educate, with oversight program, to promote and trust through dynamic governance.
- WF:** Community-wide training, career development, mentoring, with metrics to measure success, built with culture and support.
- R&D:** Priorities based on consensus, community, with incremental growth per MVP.
- Policy:** Research & clinical care linked, for advocacy, policy, funding.

# How will NRB Promote R&D Priorities?

## R&D Priorities

1. **Projects executed** by NRB will be based on R&D priorities.
2. **Consensus building** will promote community-based projects.
3. **Scientific WGs & charge** will propose concepts by research area.
4. **Research objectives** will align with the Strategic Plan.
5. **Research priorities** will be promoted incrementally, per MVP.
6. **Research priorities** will inform funding, MVP to full capacity.



# How will NRB Facilitate LEE Participation?

## LEE Participation

1. **Portal for participant entry, engagement.**
2. **Ambassador Program** to direct to NRB opportunities, including training, education, recruitment, and participation.
3. **LEE and HEDI oversight programs** will be provided.
4. **LEEs will have access** to education, protocol training, and participation.

# How will NRB Incorporate HEDI Principles?

## HEDI Principles

1. **HEDI representatives** will oversee and facilitate tailored research training on HEDI principles.
2. **HEDI principles** will be included in every committee, WG and monitored as part of Network operation.
3. **HEDI representation** will be part of each WG & committee.
4. **HEDI principles** will be included on every protocol.

# How will NRB Implement Workforce Training?

## Training Implementation

1. **Community-wide research** education & training: LEE, CE, HTC.
2. **Career development** across NRB: mentors, mentees.
3. **Research & clinical care**, hand in hand, by clinical team.
4. **Metrics to measure success** in training, learning.
5. **Training rolled out for community**, HTC, by protocol.
6. **Continuing education** for community, HTC, by protocol.
7. **Culture and support incorporated** into training.

# How will NRB Train, Execute, Communicate?

## NRB Website:\*

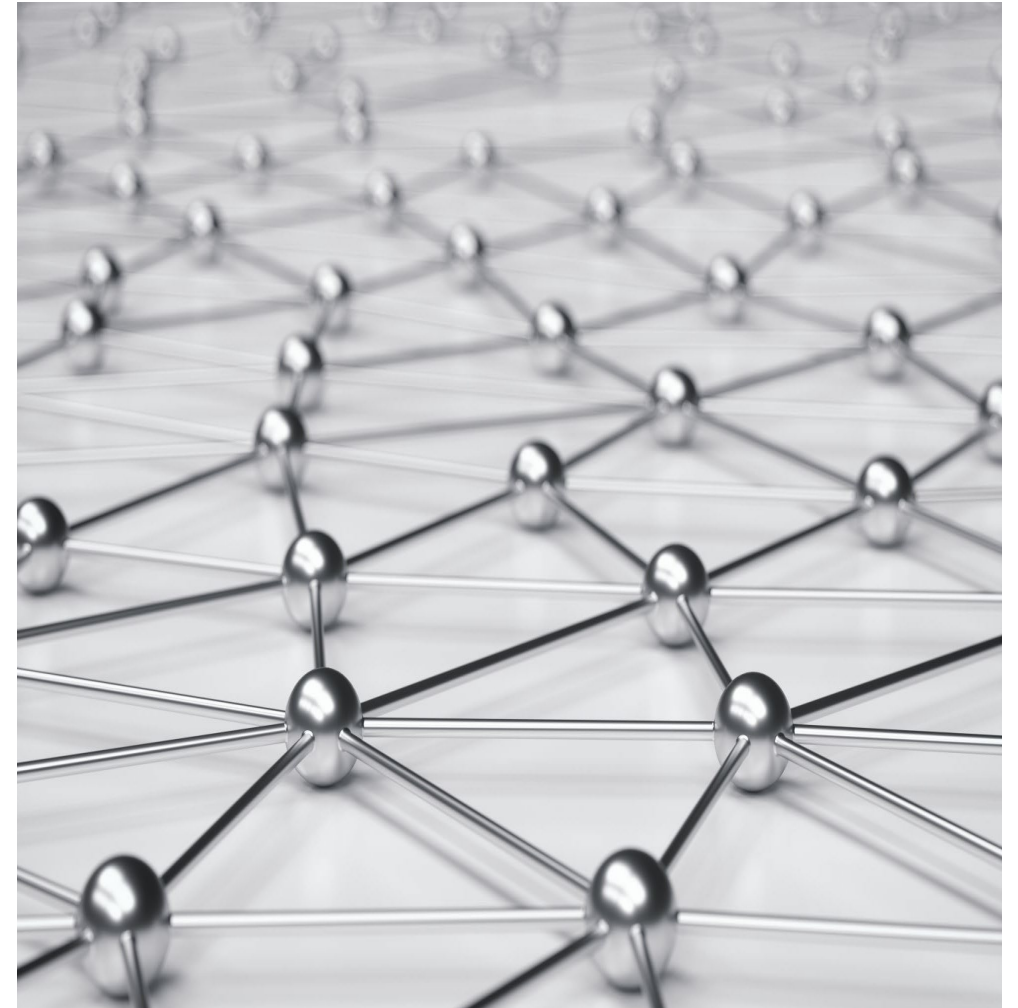
- **Links:** Guidance: design, propose, run a trial.  
Onboarding: site, subject enrollment.  
Participation: Ambassador program.  
Education: training, volunteering.  
Data: collection, management, analysis.  
Resources: statistical, scientific, design.

## Dashboard:

- **Tracking:** Projects: from concept to completion.  
Timeline for protocol development.  
Communication: for community, partners.

## Development:

- **Support:** Service: IT, design, update, maintenance.



\*Modeled after COG, NCI CETP Programs

# How will NRB **Research Impact Policy**?

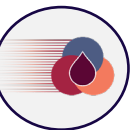
## **NRB & Policy**

1. **Patient care & research** will be linked for advocacy, policy, funding.
2. **Foundational, governmental, partner support** will be sought for data collection based on linked care & research.
3. **Foundational, governmental, partner representatives** will be invited to join NRB committees.
4. **RFAs for funding** (NIH, foundations) will be proposed.
5. **Linkage with national research** projects and collaboration with partners.

# Q | A



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# How will NRB goals, plans be supported ?

## **IFS Support:**

- Training:** Facilitate inclusive research training for all: LEE, CE, HTC, PI, staff.  
Career training across NRB for mentors, mentees.  
General training for project-specific research.
- Culture:** Support, incorporate values into training.
- HEDI:** Include HEDI reps on committees, HEDI principles in training.
- LEE:** Mandates for involvement on a range of levels, roles.
- Expertise:** Build resources from 1<sup>st</sup> project, incrementally.
- Research:** Link research & clinical care: use in advocacy, policy, funding.
- Metrics:** Monitor success, sustain excellence in training, learning, project teams.
- Trust:** Build trust among members, communicate and share with potential partners.

# How will the Network Operate?

## **Network Operation:**

- Lean organization:** Uses existing strengths, capabilities to start, then build out.
- Development:** Incremental (MVP) using existing resources, then expanding.
- Initial milestone:** Success and completion of 1<sup>st</sup> study.
- Minimal startup:** IRB, lab, data management.
- Data collection:** Registry, observational to start.
- Future growth:** Gradual scientific, statistical complexity.
- Project success:** Promotes resources, funds for next study.



# What will the Network **Require for Launch?**

## **Network Launch:**

- Implementation:** Establish roles, committees, processes.
- Processes:** Build culture, supports, teams.
- Communication:** Engage internal/ external outreach, establish website.
- Project Portfolio:** Prioritize, set selection criteria.
- Metric Portfolio:** Evaluate outcomes, day-to-day function, how the network is doing, what it's doing, how research team & committees function: are they inclusive? do they use dynamic governance?
- Sustainability:** Grow strength, capacity, community.
- Collaboration:** Partner with organizations/ foundations/ funders.
- Accountability:** Adapt, learn from mistakes, build trust.

# With which **Projects** should we Start ?

## **Potential Projects\*** - *framing, for consideration, not defined*

- #1: Registry:** Set up data collection, share: LEE, HTC, CE.
- #2: Observational study:** Use existing data to plan future studies.
- #3: Case-control study:** Use existing data, PRO outcomes in future studies.
- #4: IBD-priority Qs:** Identify and study by gender, race, age.
- #5: Gaps in care:** Assess care in IBD groups to develop concepts.
- #6: Prevalence & risk:** to study IBD outcomes, HTC vs. non-HTC.
- #7: Current practice:** Survey current practice to to plan novel RCTs.

\*Partnering with R&D, working with LEEs, community, engaging science partners. RCT=randomized clinical trial.

# How will Network Research grow, expand ?

**MVP:** *Start with existing resources, expand IFS incrementally*

- **LEE peer groups:** Set up at 1<sup>st</sup> study, build out future studies.
- **Ambassador role:** Train for 1<sup>st</sup> and future studies.
- **Expansion:** Grow iteratively, develop new ideas, partnerships.
- **Data collection:** Expand, improve efficiency, accessibility.
- **Care & research:** Link care & research at HTC, in advocacy, policy, funding.
- **Collaboration:** Partner with scientists, national projects.
- **HRSA Funding:** Seek 340B support, invite HRSA to partner in NRB.
- **RFAs, Grants:** Secure funding for 1<sup>st</sup>, future projects.