WELCOME
Building the National Research Blueprint Workshop: “Nothing About us Without US”

Michelle Witkop
NHF, Research Department
On behalf of the NRB Steering Committee, THANK YOU!
<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>7:00 - 8:30 AM</td>
<td>Registration and Breakfast</td>
<td></td>
</tr>
<tr>
<td>8:30 - 9:30 AM</td>
<td>Welcome and Introductions, National Research Blueprint: A community effort that includes you!</td>
<td>Michelle Witkop &amp; Kevin Mills</td>
</tr>
<tr>
<td>9:30 - 10:30 AM</td>
<td>Research: What is The Big Deal?</td>
<td>Maria Santaella</td>
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<tr>
<td>10:30 - 11:00 AM</td>
<td>Break</td>
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<tr>
<td>11:00 - 12:00 PM</td>
<td>Equity, Inclusion, and Research: Ensuring Research Findings Include You</td>
<td>Keri Norris</td>
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<tr>
<td>12:00 - 12:30 PM</td>
<td>Group Activity</td>
<td>Entire Group</td>
</tr>
<tr>
<td>12:30 - 1:30 PM</td>
<td>Lunch (Salons D, E)</td>
<td></td>
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<tr>
<td>1:30 - 2:30 PM</td>
<td>Research from where we stand: A conversation from our perspective</td>
<td>Moderator: Michelle Witkop</td>
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<tr>
<td>2:30 - 3:30 PM</td>
<td>Let’s Design a Research Protocol TOGETHER!</td>
<td>Moderator: Donna Di Michele</td>
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<tr>
<td>3:30 - 4:00 PM</td>
<td>Break</td>
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<tr>
<td>4:00 - 5:30 PM</td>
<td>A discussion with NRB WG Chairs about charges and the role of the SME</td>
<td>WG Chairs:</td>
</tr>
<tr>
<td></td>
<td>- Infrastructure, Workforce, and Research &amp; Development (R&amp;D)</td>
<td>Infrastructure – Maggie Ragni &amp; Moses Miles</td>
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<td></td>
<td>- Health Equity, Diversity, &amp; Inclusive (HEDI)</td>
<td>Workforce – Alice Ma</td>
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<td></td>
<td>- Policy</td>
<td>R &amp; D – Jill Johnsen</td>
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<td>- SME</td>
<td>HEDI – Keri Norris &amp; Melissa Creary</td>
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<td></td>
<td>- Community Engagement</td>
<td>Policy – Nathan Schaefer, Sonji Wilkes, &amp; Deniece Chevannes</td>
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<td>SME – Esmeralda Vazquez &amp; TBD</td>
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<td></td>
<td></td>
<td>Moderators: Donna Di Michele &amp; Kevin Mills</td>
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<tr>
<td>5:30 - 6:30 PM</td>
<td>Reception followed by dinner at the hotel (Salons D, E)</td>
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Before we start...

• Virtual participants, please join in the fun. We want to hear from you too!
• Please go to www.menti.com and use the code above
• Keep your smart phone nearby
• If you encounter technical issues, try refreshing the browser
• To our virtual participants, please use zoom to ask questions
National Research Blueprint

Please scan this QR code to provide additional comments
Building The National Research Blueprint: A Community Effort That Includes You!

Kevin Mills
NHF, Research Department
A world without inheritable blood disorders ...begins with research
OUR MISSION

The National Hemophilia Foundation (NHF) is dedicated to finding cures for inheritable blood disorders and to addressing and preventing the complications of these disorders through research, education, and advocacy, enabling people and families to thrive.

NUESTRA MISIÓN

Fundación Nacional de la Hemofilia (NHF) se dedica a encontrar curas para los trastornos sanguíneos hereditarios y a abordar y prevenir las complicaciones de estos trastornos a través de la investigación, la educación y la abogacía permitiendo que las personas y familias prosperen.
A Blueprint for Transformational Change

- Medicine
- Education
- Technology
- Research
- Families
- Community
- Policy Makers
- Industry

9 April 2022
Subject Matter Experts (SME)

Who is a Subject Matter Expert?
Anyone living with an inheritable blood disorder
Patient, caregiver, family, friend, neighbor, colleague

Why?
Because nobody is more of an expert than those with the lived experience
Subject Matter Experts (SME)

“But what do I know about research...?”

You know everything you need to know about your living experience with an inheritable blood disorder.

Your expertise is critical to inform researchers on what to 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
Working groups came directly from community input.
Charges for the groups were defined by the community.
Resulting priorities were informed by community participation in each WG.
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

People Centricity
Collaboration
Health Equity, Diversity, and Inclusion
Hatching a New Research Paradigm
Elements of the Blueprint

- A clear, actionable plan that addresses unmet needs
- Delineated roles and responsibilities for all stakeholders
- Well defined milestones and timelines
- Mechanisms to measure progress and evaluate success
What we will deliver to the IBD community

In 2023, NHF will present to the Inherited Bleeding Disorders (IBD) community:
• One- and five-year strategic plans for implementation of a National Research Blueprint (NRB)
• The NRB 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**
We’ve sharpened our focus with community input

From the State of the Science WGs...

1. Research Priorities for Hemophilia A & B
2. Research Priorities for von Willebrand Disease, Platelet Dysfunction & other mucocutaneous IBDs
3. Research Priorities for Ultra-Rare IBDs
4. Research Priorities for health of women & girls and persons with the potential for menstruation
5. Diversity, Equity & Inclusion Health Services Research & Implementation Science
6. Facilitating Priority Research in the IBDs Community

...to the National Research Blueprint WGs

1. R&D, Infrastructure, Workforce Cluster
2. HEDI
3. Subject Matter Experts
4. Community Engagement
5. Policy

9 April 2022

National Research Blueprint
And now we focus on coordinated action planning

**Priority Action Teams**
1. Ultra-Rare Disorders
2. Mental Health
3. VWD
4. Digital Health
5. Future Therapies
Goals for today’s workshop

1. Inform the community
   - Establish clarity about the plans and processes for building on the State of The Science to develop an actionable National Research Blueprint (NRB)

2. Gain broader community input
   - Solicit input from all key stakeholders into the development of the Blueprint - including different perspectives to the planning stage will result in a more comprehensive and inclusive NRB

3. Strategize our work
   - Outline the work plans for the working groups – what can we do to help them best accomplish their goals

4. Identify synergies and challenges
   - Identification of these early in the process will allow us to address them, plan for them, and ultimately save time/effort
Research: What Is The Big Deal?

Maria E. Santaella
NHF, Research Department
AGENDA

Definition of research

Different opportunities within the community

Key concepts
RESEARCH

A methodical investigation designed to produce or contribute to generalizable knowledge.

Goal depends on who is conducting it and for what purpose.
**Steps**

**Make an observation**
- The toaster won’t toast

**Ask a question**
- Why won’t my toaster work?

**Propose a hypothesis or a possible explanation**
- Maybe the outlet is not working

**Make a prediction based on the hypothesis**
- If I plug the toaster into a different outlet, then it will toast my bread

**Test the prediction**
- Plug the toaster into a different outlet and try again

**Use the results to make new hypotheses or predictions**
- It worked; my bread toasted! Prediction supported
- My bread did not toast. Prediction not supported. Maybe there is a broken wire in the toaster.
Types of Research in Our Community

- Basic Science
- Registries
- Focus Groups
- Clinical Trials
- Surveillance
- Surveys
- Interviews
Goal of Behavioral and Clinical Research:

improve the lives of persons
living with an inheritable bleeding disorder

Improve disease prevention, detection, treatment, and quality of life (QOL)

The foundation for evidence-based practice

Conducted following strict ethical principles

https://grants.nih.gov/policy/clinical-trials/definition.htm
The Belmont Report identifies basic ethical principles and guidelines that address ethical issues arising from the conduct of research with human subjects.

Ethical Principles of Research

- Respect for Persons
- Beneficence
- Justice

Informed Consent

Assessment of Risks and Benefits

Selection of Subjects

National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research
## Ensuring Safe and Ethical Research

<table>
<thead>
<tr>
<th>Health and Human Services (HHS) – specifically, Office for Human Research Protections</th>
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<tbody>
<tr>
<td>Trand Good Clinical Practice (GCP)</td>
</tr>
<tr>
<td>Data and Safety Monitoring Boards</td>
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<tr>
<td>IRB (Institutional Review Board)</td>
</tr>
<tr>
<td>Food and Drug Administration (FDA), if applicable</td>
</tr>
<tr>
<td>Privacy Rule</td>
</tr>
</tbody>
</table>
Possible Benefits of Participating in Studies

- Access the investigational treatment or therapy before it is commercially available
- Receive regular medical attention from the research team
- Receive the investigational treatment or therapy at lower to no cost
- Reimbursement for travel expenses in most cases
- Help improve treatment options for others with your condition or disorder

References:
https://www.hfalearning.org/mod/scorm/player.php?id=133&cm=38&display=popup
https://healinggenes.org/clinical-trials/
https://www.nih.gov/health-information/nih-clinical-research-trials-you/basics
Possible Drawbacks of Participating in Studies

- Possible unknown side effects of the new investigational treatment/intervention
- The investigational therapy/intervention may not work as expected
- Emotional distress
- Data breach and other potential privacy concerns
- Possible financial costs that are not reimbursed
- Possible significant time commitment
- May impact eligibility to participate in future clinical trials

References:
https://www.hfalearning.org/mod/scorm/player.php?scoid=133&cm=38&display=popup
https://healinggenes.org/clinical-trials/
https://www.nih.gov/health-information/nih-clinical-research-trials-you/basics
Who Conducts/Supports Research in Our Community?

HTC’s Multidisciplinary Team

Pharmaceutical Industry

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Hemophilia Federation of America

National Heart, Lung, and Blood Institute

Other Researchers and Scientists

Chapters and YOU!

Others
Why?
Research in Inheritable Bleeding Disorders (IBDs)
Number of people in the US: ~330,000,000
Number of people with high blood pressure in the US: ~116,000,000
Number of people with diabetes in the US: ~17,000,000
Number of people with hemophilia A in the US: ~21,810
Number of people with hemophilia B in the US: ~7,030
Number of people with FXIII deficiency in the US: ~255
## All Inheritable Bleeding Disorders

<table>
<thead>
<tr>
<th>Diagnosis</th>
<th>Unique patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alpha-2 Antiplasmin deficiency</td>
<td>13</td>
</tr>
<tr>
<td>Bernard Soulier syndrome</td>
<td>83</td>
</tr>
<tr>
<td>Blood coagulation disorder without specific diagnosis</td>
<td>2,186</td>
</tr>
<tr>
<td>Ehlers-Danlos syndrome</td>
<td>429</td>
</tr>
<tr>
<td>Factor I (1)</td>
<td>404</td>
</tr>
<tr>
<td>Factor II (2)</td>
<td>123</td>
</tr>
<tr>
<td>Factor V (5)</td>
<td>571</td>
</tr>
<tr>
<td>Factor Vll (7)</td>
<td>2,654</td>
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<tr>
<td>Factor VIII (8)</td>
<td>21,809</td>
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<tr>
<td>Factor IX (9)</td>
<td>7,031</td>
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<tr>
<td>Factor X (10)</td>
<td>284</td>
</tr>
<tr>
<td>Factor XI (11)</td>
<td>1,579</td>
</tr>
<tr>
<td>Factor XIII (13)</td>
<td>255</td>
</tr>
<tr>
<td>Factors V (5) &amp; VIII (8), combined</td>
<td>22</td>
</tr>
<tr>
<td>Glanzmann thrombasthenia</td>
<td>311</td>
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<tr>
<td>Gray platelet syndrome</td>
<td>19</td>
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</tbody>
</table>

<table>
<thead>
<tr>
<th>Diagnosis</th>
<th>Unique patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hermansky-Pudlak syndrome</td>
<td>131</td>
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<tr>
<td>PAI-1 deficiency</td>
<td>345</td>
</tr>
<tr>
<td>Platelet function disorder, nonspecific</td>
<td>3,693</td>
</tr>
<tr>
<td>Platelet release defect</td>
<td>55</td>
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<tr>
<td>Platelet storage pool disease</td>
<td>3,525</td>
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<tr>
<td>Thrombocytopenia, hereditary</td>
<td>920</td>
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<tr>
<td>Venous Thromboembolism (VTE)</td>
<td>36,535</td>
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<tr>
<td>Von Willebrand disease, type 1</td>
<td>21,370</td>
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<td>Von Willebrand disease, type 1C</td>
<td>120</td>
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<td>Von Willebrand disease, type 2A</td>
<td>1,140</td>
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<tr>
<td>Von Willebrand disease, type 2B</td>
<td>646</td>
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<td>Von Willebrand disease, type 2M</td>
<td>620</td>
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<tr>
<td>Von Willebrand disease, type 2N</td>
<td>193</td>
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<tr>
<td>Von Willebrand disease, type 2, type unknown</td>
<td>482</td>
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<tr>
<td>Von Willebrand disease, type 3</td>
<td>472</td>
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<tr>
<td>Von Willebrand disease, type other</td>
<td>343</td>
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<tr>
<td>Von Willebrand disease, unknown</td>
<td>2,269</td>
</tr>
</tbody>
</table>
Research in Inheritable Bleeding Disorders (IBDs)

IBDs are rare conditions

IBD research constitutes <2% of all rare disorders research

Significant progress requires all of us
https://www.reddit.com/r/mildlyinteresting/comments/9naf24/this_largegrain_sand_on_the_beach/
Because we want findings to be relevant and generalizable

Research in Inheritable Bleeding Disorders (IBDs)

IBDs are rare conditions

IBD research constitutes <2% of all rare disorders research

Significant progress requires all of us

Because we want findings to be relevant and generalizable
Equity, Inclusion, and Research: Ensuring Research Findings Include You

Melissa Cleary & Keri Norris
ATHN & NHF, HEDI Department
Welcome!

Keri Norris, Co-Chair
NHF
Overview

- Define Racism
- Define Antiracism
- Overview of historical racism in medicine and current mistrust
- Possible solutions including Community Based Participatory Research (CBPR)
- Community Based Participatory Research & Steps
- CBPR Challenges
Definitions

• **Racism** – Defined as “prejudice, discrimination, or antagonism directed against a person or people on the basis of their membership in a particular racial or ethnic group, typically one that is a minority or marginalized”
  
• Racism can be imbedded into a system known as systemic racism and be so steeped into the system that it is seemingly invisible to the unconscious person

Definitions

- **Antiracism** – does not mean “not racist” because that implies that no course of action accompanies the statement; antiracism requires action

- Antiracism is “action-oriented, educational and/or political strategy for systemic and political change that addresses issues of racism and interlocking systems of social oppression”

Historical Overview of Medicine in the United States

- Throughout the history of medicine and science racism was taught in medical education and science.
- Medical doctors, philosophers, and scientist helped to continue perpetuating the stereotypes over time.
- Theories and stereotypes led to many unethical practices in medicine and public health, that continue today.
- Beliefs about pain, drug seeking, believability of the patient, and literacy are all attributed to past adoptions of stereotypes by the medical and scientific fields.

Race, medicine, and health care in the United States: a historical survey.
W. M. Byrd, L. A. Clayton
Recognition of Racism in Medicine & Science

Institutional Racism in the Health Care System

The National Academy of Family Physicians (AAFP) recognizes that racism is a system that can be systemic, societal, and individual, affecting all aspects of health care delivery. The American Medical Association (AMA) recently adopted guidelines that confront systemic racism in medicine, which aims to address and combat systemic racism in the medical profession.

AMA adopts guidelines that confront systemic racism in medicine

JUN 15, 2021

AMA’s House of Delegates adopted new guidelines that confront systemic racism in medicine. These guidelines aim to address structural racism and promote equity in health care, focusing on eliminating bias and discrimination based on race and ethnicity.


Analysis: Declarations of Racism as a Public Health Crisis

In 2018, Milwaukee County, Wisconsin, became the first county to declare racism a public health crisis. Many communities have since done so, with the pace of adoption accelerating after the police killing of George Floyd and the protests for justice that followed, with nearly 200 declarations passing since May 2020.

As of August 2021, 209 declarations of racism as a public health crisis have passed in 37 states. These declarations were adopted by city/town councils, county boards, governor/mayoral statements, education boards (e.g., school boards), and health associations or public health departments.

While resolutions and formal statements themselves are not necessarily legally enforceable, they are an important first step in calling attention to racism and shifting the narrative in a way that can drive changes to policies, laws, and resource allocation. These resolutions create the opportunity for strategic action to eliminate racist policies and practices and adopt those that advance racial equity.

https://www.apha.org/-/media/Files/PDF/topics/racism/Racism_Declarations_Analysis.ashx

Additional resources:

- https://www.aafp.org/about/policies/all/institutional-racism.html
- https://www.apha.org/-/media/Files/PDF/topics/racism/Racism_Declarations_Analysis.ashx
Diversity, equity and inclusion at the AMA

The AMA respects, welcomes, and celebrates all people and their diverse backgrounds. We value the diversity of identities and experience, and we encourage employees to bring their unique selves and experiences to the workplace.

In responding to the needs of those we serve—medical students, physicians and their patients—the AMA relies on the diverse expertise, ideas and strength of our workforce to promote and advocate for equity and justice in our policies, products and services.

This commitment helps us attract and retain the best talent, provide opportunities for personal and professional development, and foster an environment that is continuously improving the equitable and inclusive workplace we strive to provide.

ASH Statement on Diversity, Equity, and Inclusion

The American Society of Hematology (ASH) is committed to building and nurturing a global hematology community and workforce inclusive of diverse perspectives, talents, and experiences as it works toward one collective goal: helping hematologists conquer blood diseases worldwide. ASH stands in solidarity with our members and patients in communities that are targets of discrimination, harassment, and violence, and remains committed to combating all forms of bigotry. The four-pronged approach to fulfilling the Society’s commitment to diversity and inclusion is:

1. Inspiring, recruiting, and supporting researchers and clinicians from diverse backgrounds to pursue and succeed in careers in hematology and related fields.
2. Involving people with diverse perspectives, talents, and experiences in leadership, volunteer, and staff positions.
3. Advocating for policies and supporting programs that aim to eliminate health disparities in the care of hematology patients.
4. Providing resources and education to help build awareness of and seek to dismantle systemic racism and other forms of discrimination affecting our members and patients.

ASH welcomes and encourages engagement and participation of individuals in the Society regardless of their race, ethnicity, religion, age, sexual orientation, gender identity or expression, ability, national origin, or other attributes.

Our Commitment to Diversity, Equity, and Inclusion

NASW is the largest social work organization and represents a diverse group of professional social workers and students around the country and globally. We strive to serve diverse communities large and small with integrity, competence, respect for human relationships, respect for the inherent dignity and worth of all people, and a commitment to promoting social justice. We can only meet these goals by caring about our best resources: our staff, members and volunteers.

NASW's commitment to diversity is rooted in our belief that creating a truly diverse, equitable, and inclusive workplace is ethical. It is the right thing to do. It is also crucial to our organization's continued success. Most important, it is what all members of our workforce deserve.

NASW is committed to becoming a true leader in attracting and retaining diverse talent, creating an environment based on policies and practices that are just and to fostering a true sense of inclusion and belonging. Our goal is to be the example for other associations.
Two example case studies

**Tuskegee Syphilis Study**
- 1932 Government studies of untreated syphilis in the male negro (n=600)
- No informed consent, being treated for bad blood
- 1940 Concerns about study raised but ignored by CDC
- 1947 penicillin becomes the treatment (ignored by study PIs)
- 1972 study ends, condemned
- $10MM class action suit
- 1997 President Clinton apologizes
- 2001 est President Council on bioethics
- CDC funds Tuskegee Center for Bioethics

**Contraception in Puerto Rico**
- 1873 Comstock Laws prevent women from reproductive rights
- 1916 Margaret Sanger opens first Planned Parenthood in Brooklyn
- 1954 largest clinical birth control trial (n=1500) in Rio Piedras, PR after Boston trial had severe side effects
- 1960 first birth control pill approved by FDA
- 1962 six women died and 26 had severe clots
- 1982 Ana Maria Garcia debuts 40 min documentary, La Operacion
Additional examples

- Gender bias in hemophilia
- Tension in the healthcare system for women with hemophilia
- Dr. Marion Sims performs gynecological exams on slaves WITHOUT anesthesia (continued teaching in medical schools)
- Early radiation experiments on Black children in the late 1920s
- Darwinism is taught promoting racism and inferiority of others who are not white
- Josef Mengele experiments at Auschwitz concentration camp
- STD study in Guatemalan men performed by US government
- Leads to disparities in care, disparities in health outcomes, barriers to access, strained patient-provider relationships, late diagnosis, lack of participation in clinical trials, mistrust of the healthcare system
Books and films that document the history of prejudice in medicine
The research indicates that racial bias in pain assessment and treatment recommendations, and false beliefs about biological differences between blacks and whites contribute to disparities in pain treatment and perception. The research also highlights the importance of considering race and ethnicity in pain management. It underscores the need for equitable treatment practices and the development of strategies to address these disparities.
Solutions with patients at the center

Review of community-based research: assessing partnership approaches to improve public health

B A Israel 1 , A J Schultz; E A Parker, A B Becker

Affiliations + expand

PMID: 9611617 DOI: 10.1146/annurev.pubhealth.19.1.173

Abstract

Community-based research in public health focuses on social, structural, and physical environmental inequities through active involvement of community members, organizational representatives, and researchers in all aspects of the research process. Partners contribute their expertise to enhance understanding of a given phenomenon and to integrate the knowledge gained with action to benefit the community involved. This review provides a synthesis of key principles of community-based research, examines its place within the context of different scientific paradigms, discusses rationales for its use, and explores major challenges and facilitating factors and their implications for conducting effective community-based research aimed at improving the public's health.

Patient centeredness, cultural competence and healthcare quality

Sonnath Saha 1 , Mary Catherine Beach, Lisa A Cooper

Affiliations + expand

PMID: 19024223 PMCID: PMC2824588 DOI: 10.1016/j.0027-9684(13)31505-4

Free PMC article

Abstract

Cultural competence and patient centeredness are approaches to improving healthcare quality that have been promoted extensively in recent years. In this paper, we explore the historical evolution of both cultural competence and patient centeredness. In doing so, we demonstrate that early conceptual models of cultural competence and patient centeredness focused on how healthcare providers and patients might interact at the interpersonal level and that later conceptual models were expanded to consider how patients might be treated by the healthcare system as a whole. We then compare conceptual models for both cultural competence and patient centeredness at both the interpersonal and healthcare system levels to demonstrate similarities and differences. We conclude that, although the concepts have had different histories and foci, many of the core features of cultural competence and patient centeredness are the same. Each approach holds promise for improving the quality of healthcare for individual patients, communities and populations.

How Clinicians and Educators Can Mitigate Implicit Bias in Patient Care and Candidate Selection in Medical Education

Quinn Cooper IV + Author Affiliations

https://doi.org/10.3418/jets-scholhr-2020-0024PS

Received: February 26, 2020 Accepted: May 13, 2020

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ABSTRACT FULL TEXT REFERENCES CITED BY

ABSTRACT

In an attempt to help us navigate a complex world, our unconscious minds make certain group associations on the basis of their experience. Physicians are not immune to these implicit associations or biases, which can lead physicians to unknowingly associate certain demographic groups with negative concepts, like danger, noncompliance, and lower competence. These biases can influence clinical decision making in ways that potentially harm patients and may unfairly influence the medical school, residency, and fellowship application processes for candidates in certain underrepresented groups. To minimize the potential negative impact of implicit biases on patient care and diversity in the medical profession, physician leaders have a responsibility to understand biases and how to consciously override them. This article discusses the potential impact of implicit bias in healthcare and student/trainee selection and reviews research-proven tools to reduce implicit bias in one-on-one interactions.
DEI as a means of addressing historical mistrust

• The lack of diversity and inclusion in clinical trials is evident
• In order to make effective and efficient change to health outcomes it is imperative that we see:
  • Researchers knowledgeable about culture, educated on challenges, and responsible in their science
  • Communities at the table shaping research, asking questions, and participating in clinical trials (CBPR)
  • Industry efforts to partner with communities and those who historically have been left out
Community Based Participatory Research (CBPR)

A collaborative process that equitably involves all partners in the research process and recognizes the unique strengths that each brings. CBPR begins with a research topic of importance to the community with the aim of combining knowledge and action for social change.

9 April 2022

• CBPR is not a method *per se* but an orientation to research that may employ any number of qualitative and quantitative methodologies.

• Explicit throughout the CBPR process are the *deconstruction of power and the democratization of knowledge.*

❓ Can true CBPR take place when the research question itself comes from an outsider to the community?

• YES, but there is critical need for skills in community organizing and group dynamics if the outsider is to be successful in helping shift control to the community.
Community-based Participatory Research

- is participatory
- is cooperative, engaging community members and researchers in a joint process to which each contributes equally
- a co-learning process
- it involves systems development and local capacity building
- it is an empowering process through which participants can increase control of their lives
- it achieves a balance between research and action.
Many Approaches...

...but guided by NINE principles

1. CBPR acknowledges community as a unit of identity.
   - Community as a unit of identity is defined by a sense of identification with and emotional connection to others through common symbol systems, values, and norms; shared interests; and commitments to meeting mutual needs.
   - CBPR partnerships seek to work with existing communities of identity, and/or to enhance a sense of community through the collaborative process.
   - There also may be situations where communities of identity may benefit from involving individuals and groups from outside the community of identity who bring additional needed skills and resources.
2. CBPR builds on strengths and resources within the community.

• CBPR recognizes and builds on the strengths, resources, and assets that exist within communities of identity, such as individual skills, social networks, and organizations, in order to address identified concerns
3. CBPR facilitates a collaborative, equitable partnership in all phases of research, involving an empowering and power-sharing process that attends to social inequalities.

- All partners participate in and share decision making and control over all stages of the research process, such as defining the problem, collecting and interpreting data, disseminating findings, and applying the results to address community issues.

- Researchers involved in CBPR recognize the inequalities that exist between themselves and community partners and attempt to address these inequalities through developing relationships based on trust and mutual respect and by creating an empowering process that involves open communication and sharing information, decision-making power, and resources.
4. CBPR fosters co-learning and capacity building among all partners.

• CBPR is a co-learning process that fosters the reciprocal exchange of skills, knowledge, and capacity among all partners involved, recognizing that all parties bring diverse skills and expertise and different perspectives and experiences to the partnership process.
5. CBPR integrates and achieves a balance between knowledge generation and intervention for the mutual benefit of all partners.

- CBPR aims to contribute to science while also integrating and balancing the knowledge gained with interventions and policies that address the concerns of the communities involved
- CBPR projects will have a commitment to the translation of research findings into action strategies that will benefit the community
6. CBPR focuses on the local relevance of public health problems and on ecological perspectives that attend to the multiple determinants of health.

• CBPR addresses public health concerns that are of local relevance to the communities involved, and it emphasizes an ecological approach to health that pays attention to individuals, their immediate context, and the larger communal and societal contexts.

• CBPR efforts consider the multiple determinants of health and disease, including biomedical, social, economic, cultural, and physical environmental factors, and necessitate an interdisciplinary team of researchers and community partners.
7. CBPR involves systems development using a cyclical and iterative process

- CBPR recognizes partnership as a system which draws on the competencies of each partner to engage in a cyclical, iterative process that includes all the stages of the research process.
- These include: community assessment, problem definition, research design, data collection and analysis, data interpretation, dissemination, determination of intervention and policy strategies, and action taking, as appropriate.
8. CBPR disseminates results to all partners and involves them in the wider dissemination of results.

- CBPR emphasizes the dissemination of research findings to all partners and communities involved and in ways that are understandable, respectful, and useful

- All partners must engage in the broader dissemination of results, for example as coauthors of publications and co-presenters at meetings and conferences
9. CBPR involves a long-term process and commitment to sustainability.

• CBPR involves a long-term process and commitment to sustainability in order to establish and maintain the trust necessary to successfully carry out CBPR endeavors, and to achieve the aims of addressing multiple determinants of health.

• This long-term commitment frequently extends beyond a single research project.
Challenges

• Establishing trust → Building equitable partnerships
• Sharing power → Power dynamics and other sources of insider-outsider tension
• Aligning objectives and expectations
• Differential reward structures for partners in CBPR
• Racism and other –isms (root causes of inequities)
• Community representation
• Assuring participation
• Communication
• Action

Citations


• Capers, Q IV. (2020). How Clinicians and Educators Can Mitigate Implicit Bias in Patient Care and Candidate Selection in Medical Education. ATS Scholar, 1(3), 211-217.


Citations


Citations


Research from where we stand: A conversation from our perspective

Michelle Witkop
NHF, Research Department
1. Describe your experience
   • What did it entail?
   • How did you hear about it?
   • Why were you interested?
   • How did you decide to participate?
2. What were the barriers to participating?
3. What were the benefits of participating?
4. Looking back, how do you feel about the experience?
Let’s Design a Protocol TOGETHER!

Donna Di Michele
Consultant
Clinical Trial Phases

Pre-clinical
Done in the lab usually using animal models
Goal: accurately test the desired effect of a drug to determine effectiveness, safety, and toxicities (predict side effects)

Phase 1
Done in a small group of people for the first time—sometimes in healthy volunteers
Goal: determine safety, dose, and identify side effects

Phase 2
Done in large groups of people affected by the disease/condition being studied
Goal: determine effectiveness and further study safety

Phase 3
Larger groups of people affected by the disease/condition being studied
Goal: confirm effectiveness, monitor side effects, compare it to standard or similar treatment

Phase 4
Post-marketing analysis
Goal: follow up on side effects, efficacy, use, cost

8+ years or more

Reference:
What Are the Different Types of Clinical Research? | FDA
Let’s Help Design a Trial Protocol Together!

- A company has discovered a new oral drug that appears to be able to prevent bleeding in all persons with a bleeding disorder, regardless of the underlying factor deficiency.

- The new drug has already been studied in animals and in some people (in Phase I and 2 clinical trials) where it has so far been shown not to have severe side effects and looks like it may prevent all but very mild bleeding in adults with different inherited bleeding disorders (IBDs).

- Now the FDA is asking the company to do a clinical trial in a larger number of adults with many different IBDs to be sure that this new possible treatment for bleeding is really safe and effective enough to get licensed for all IBDs (Phase 3 trial).

- Your doctor is part of a team of HTC Directors who are ‘study investigators’ – hemophilia doctors who believe that this oral drug holds the potential to really improve the lives of their IBD patients, so they agree to help the company design the Phase 3 clinical trial and to enroll their patients who agree to help test this drug.
Let’s Help Design a Trial Protocol Together!

- But, thanks to the NHF, your doctor is now smart enough to realize that advice from you, the subject matter expert in your own disorder, is critical to designing a worthwhile study and asks for your input into the following:

  ✓ If the trial is studying the effect of the treatment in a way that is useful to those who are living with IBDs and who will end up using the new therapy
  ✓ How the trial can reassure persons living with IBDs that the drug is safe enough to use
  ✓ How the investigators can best ensure that the design of the trial is not too burdensome on those who agree to test the new therapy
  ✓ How investigators fairly decide who can participate, and widely communicate the trial to the bleeding disorders community, in a way that ensures that all who would like to participate get the chance to do so
  ✓ How would participants prefer to be informed about how the trial is going and the final results of the study
<table>
<thead>
<tr>
<th>Section</th>
<th>Question</th>
</tr>
</thead>
<tbody>
<tr>
<td>Study Question</td>
<td>Is the study studying the treatment effect that is the most useful to the IBD community? Is it addressing the safety concerns?</td>
</tr>
<tr>
<td>Study Eligibility</td>
<td>Who will the study allow to participate?</td>
</tr>
<tr>
<td>Recruitment</td>
<td>How do we give all eligible participants across all bleeding disorders a chance to participate?</td>
</tr>
<tr>
<td>Study Design</td>
<td>How can we design a study that will get all the information we need w/o being overly burdensome on participants?</td>
</tr>
<tr>
<td>Dissemination</td>
<td>How should participants be informed about the interim and final results of the study?</td>
</tr>
</tbody>
</table>
# All Inheritable Bleeding Disorders

<table>
<thead>
<tr>
<th>Diagnosis</th>
<th>Unique patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Alpha-2 Antiplasmin deficiency</td>
<td>13</td>
</tr>
<tr>
<td>Bernard Soulier syndrome</td>
<td>83</td>
</tr>
<tr>
<td>Blood coagulation disorder without specific diagnosis</td>
<td>2,186</td>
</tr>
<tr>
<td>Ehlers-Danlos syndrome</td>
<td>429</td>
</tr>
<tr>
<td>Factor I (1)</td>
<td>404</td>
</tr>
<tr>
<td>Factor II (2)</td>
<td>123</td>
</tr>
<tr>
<td>Factor V (5)</td>
<td>571</td>
</tr>
<tr>
<td>Factor VII (7)</td>
<td>2,654</td>
</tr>
<tr>
<td>Factor VIII (8)</td>
<td>21,809</td>
</tr>
<tr>
<td>Factor IX (9)</td>
<td>7,031</td>
</tr>
<tr>
<td>Factor X (10)</td>
<td>284</td>
</tr>
<tr>
<td>Factor XI (11)</td>
<td>1,579</td>
</tr>
<tr>
<td>Factor XIII (13)</td>
<td>255</td>
</tr>
<tr>
<td>Factors V (5) &amp; VIII (8), combined</td>
<td>22</td>
</tr>
<tr>
<td>Glanzmann thrombasthenia</td>
<td>311</td>
</tr>
<tr>
<td>Gray platelet syndrome</td>
<td>19</td>
</tr>
<tr>
<td>Hermansky-Pudlak syndrome</td>
<td>131</td>
</tr>
<tr>
<td>PAI-1 deficiency</td>
<td>345</td>
</tr>
<tr>
<td>Platelet function disorder, nonspecific</td>
<td>3,693</td>
</tr>
<tr>
<td>Platelet release defect</td>
<td>55</td>
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<tr>
<td>Platelet storage pool disease</td>
<td>3,525</td>
</tr>
<tr>
<td>Thrombocytopenia, hereditary</td>
<td>920</td>
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<tr>
<td>Venous Thromboembolism (VTE)</td>
<td>36,535</td>
</tr>
<tr>
<td>Von Willebrand disease, type 1</td>
<td>21,370</td>
</tr>
<tr>
<td>Von Willebrand disease, type 1C</td>
<td>120</td>
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<tr>
<td>Von Willebrand disease, type 2A</td>
<td>1,140</td>
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<tr>
<td>Von Willebrand disease, type 2B</td>
<td>646</td>
</tr>
<tr>
<td>Von Willebrand disease, type 2M</td>
<td>620</td>
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<tr>
<td>Von Willebrand disease, type 2N</td>
<td>193</td>
</tr>
<tr>
<td>Von Willebrand disease, type 2, type unknown</td>
<td>482</td>
</tr>
<tr>
<td>Von Willebrand disease, type 3</td>
<td>472</td>
</tr>
<tr>
<td>Von Willebrand disease, type other</td>
<td>343</td>
</tr>
<tr>
<td>Von Willebrand disease, unknown</td>
<td>2,269</td>
</tr>
</tbody>
</table>
This trial will study the following question about treatment effectiveness:

Will this new drug be just as effective as the IBD participant’s current treatment in preventing a major bleed* when it is taken orally 3 times a day every day for the length of the trial?

* Major bleed is defined as one that would need treatment if it were to occur
Study Eligibility

To be eligible for this study, participants would need to be:

- Between 18 and 50 yrs. of age
- Of any sex or gender
- Not pregnant and willing to prevent pregnancy for the trial duration
- Diagnosed with an IBD of any type
- On prophylaxis with a treatment product that is IBD-specific
Study Recruitment Strategy

*The investigators plan to inform the community about this study by:*

- Speaking to eligible IBD patients at the time of their HTC visits
- Posting information about the study on the NHF and HFA websites
Design of the Clinical Trial

Clinical trial participants will be asked to:

✓ Enroll in the trial through their HTC

✓ Keep a paper diary of their current prophylactic treatment product and schedule, and the number of new bleeds over a 6-month period before the start on the experimental treatment

✓ Continue the paper diary on both new bleeding episodes and any possible side effects while on the novel oral treatment 3x /day for the following 6 months

✓ Come in to the HTC once /month for an examination and study-mandated lab work for duration of the study
Disseminating Study Information

Investigators don’t have any good ideas about how to do this, so they are asking for your suggestions on how they can continue to inform participants about interim and final results of the study.
NRB Working Group Chairs & YOU

Moderator: Donna Di Michele
Consultant
NRB Working Groups and their charges

NRB Working Groups:
- Partners NHF
- PATs*
- R&D, Infrastructure, Workforce Cluster
- Community Engagement
- Policy
- HEDI

Subject Matter Experts

Priority Action Teams:
1. Ultra-Rare Disorders
2. Mental Health
3. VWD
4. Digital Health
5. Future Therapies

*Priority Action Teams
R&D, **Infrastructure**, Workforce Cluster

Moses Miles
Maggie Ragni
about us

Moses Miles

Maggie Ragni

Ziva Mann, SME
Our goal:

figure out how to support

- people and families living with inherited bleeding disorders
- people providing care
- researchers

to study the things that matter (research), in ways that are accessible and respectful to all.
Infrastructure: pieces of a puzzle

Our goal: figure out how to support

- people and families living with inherited bleeding disorders
- people providing care
- researchers
to study the things that matter (research), in ways that are accessible and respectful to all.
What fits you?

Participation
  providing information, blood

Consultation
  be asked about a topic, how to do a research project

Partnership
  be part of a team planning research; what to study, how, results

Shared Leadership
  join a central group helping multiple research studies

Where would you want to be on this CONTINUUM spectrum?
What fits you?

Participation: providing information, blood

Consultation: be asked about a topic, how to do a research project

Partnership: be part of a team planning research; what to study, how, results

Shared Leadership: join a central group helping multiple research studies

What might you need to be a part of research at that level?
Can you help us think about our puzzle?

Tell us!

Maggie Ragni
ragni@pitt.edu

Ziva Mann
ziva.mann.consulting@gmail.com
R&D, Infrastructure, Workforce Cluster

R&D Jill Johnsen
Workforce Alice Ma
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
Health Equity, Diversity, and Inclusion Working Group

Melissa Creary, PhD, MPH
Keri Norris, PhD, JM, MPH, MCHES
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
  • 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
Policy

Working Group Chairs

Deniece Chevannes  Nathan Schaefer  Sonji Wilkes

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
Subject Matter Experts (SME) Working Group

Erin Cirelli and Esmeralda Vázquez
SMEs

Working Group Chairs

Erin Cirelli
Esmeralda Vázquez

Steering Committee Liaison

Maria Santaella

WG Charge

• Develop a 1- and 5-year strategic plan for the implementation of the NRB that includes:

  • Collaborate with the Research and HEDI WGs to develop a schema for
    • SME-informed, SME-engaged, and SME-guided integration of diverse people into community-based care and research to improve the lives of people living with IBDs
    • Prioritizing SME principles and participation procedures to inform the research enterprise that begins and remains people-centered
  
  • Work with the Policy WG to ensure that policies reflect the prioritized SME principles
  • Work with the Community Engagement WG on a communication strategy
Community Engagement Working Group
Community Engagement

Working Group Chairs

To be determined

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
THANK YOU!