NATIONAL RESEARCH BLUEPRINT

Washington, D.C.

April 8-9
2022

WELCOME

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

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!

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Brett Spitale


Kevin Mills


Michael Recht


Keri Norris


Michelle Witkop


Donna DiMichele


Esmeralda Vazquez


Maria E. Santaella

Agenda Day 1: Friday 4/8/2022

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

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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

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National Research Blueprint

Please scan this QR code to provide additional comments

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Building The National Research Blueprint: A Community Effort That Includes You!

Kevin Mills

NHF, Research Department

VISION

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.

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A Blueprint for Transformational Change

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Medicine

Education

Technology

Research

Families

Community

Policy Makers

Industry

9 April 2022

Subject Matter Experts (SME)

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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

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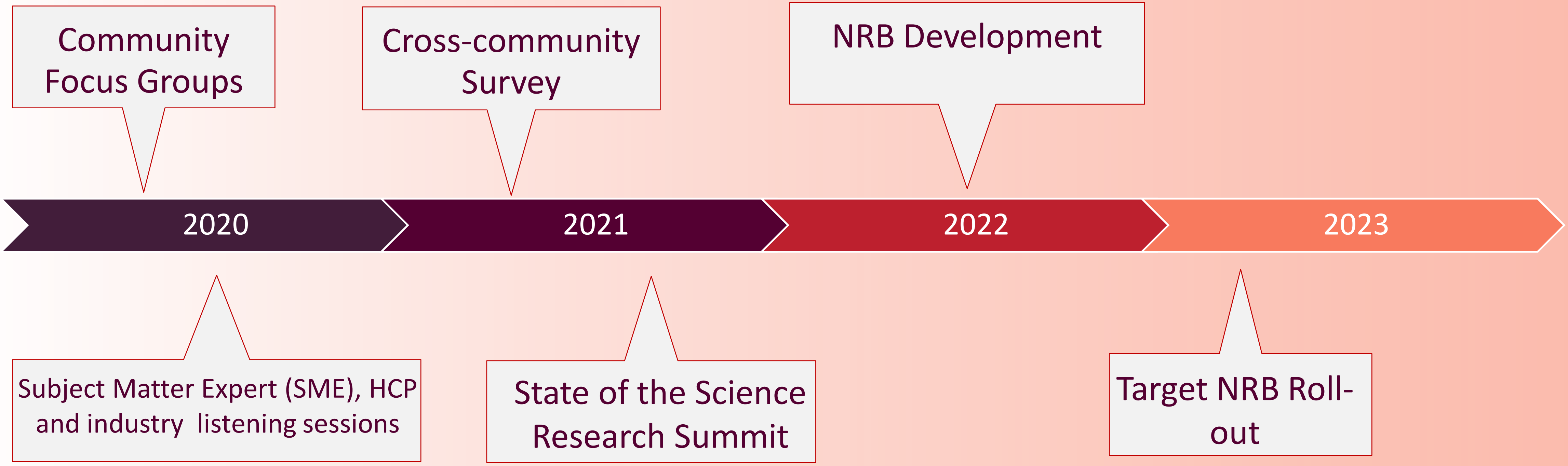

Community Focus Groups

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

The Research State of the Science

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1. Hemophilia A&B
2. VWD, Platelet Dysfunction and other Mucocutaneous IBD
3. Rare & Ultra-Rare IBD
4. Women, Girls, and People with Potential to Menstruate
5. Diversity, Equity, Inclusion, Health Services Research, Implementation Science
6. Infrastructure, Resources, and Workforce

- 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

- 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

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

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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

And now we focus on coordinated action planning

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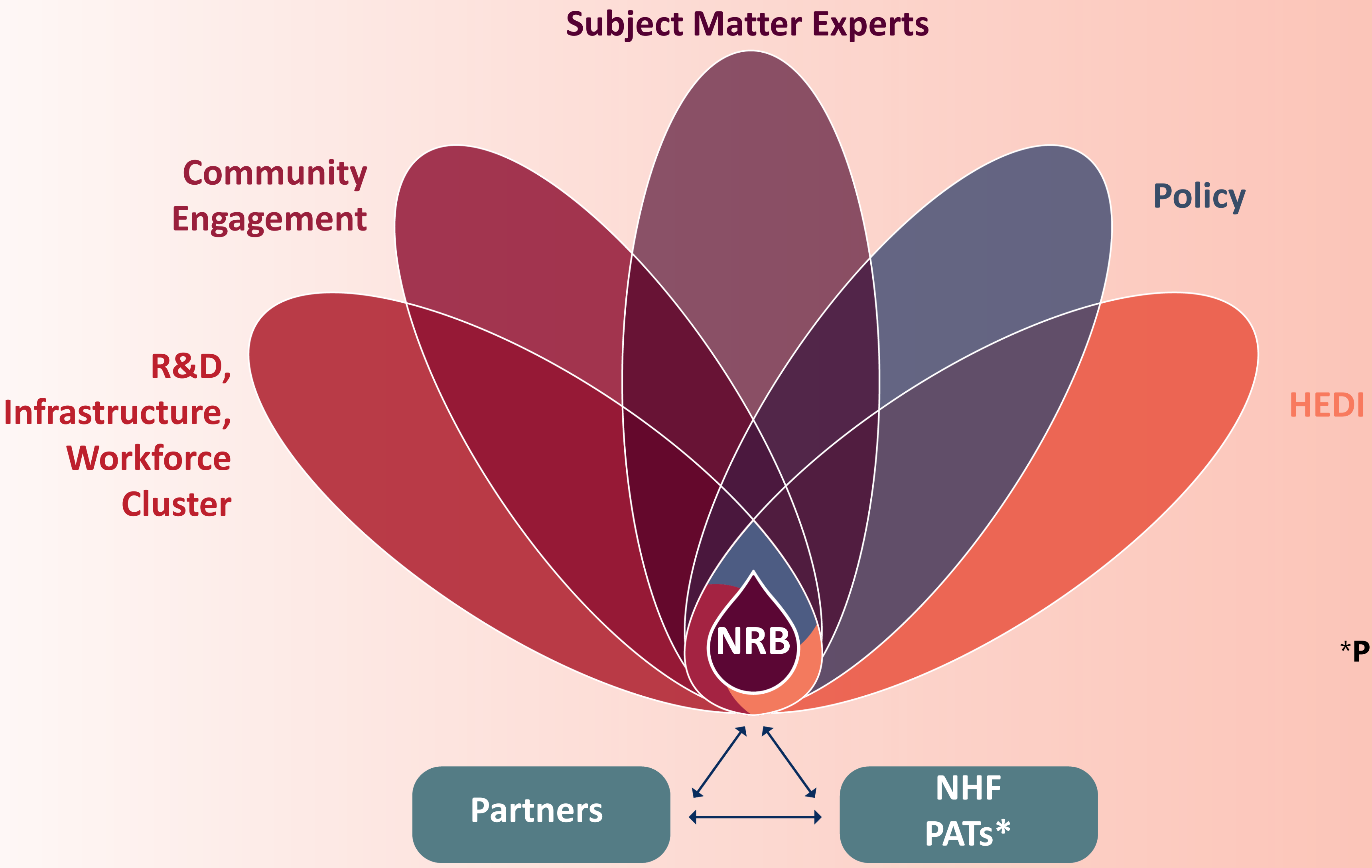

*Priority Action Teams

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

Goals for today's workshop

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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

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Research: What Is The Big Deal?

Maria E. Santaella

NHF, Research Department

AGENDA

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- 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.

https://www.khanacademy.org/science/biology/intro-to-biology/science-of-biology/a/the-science-of-biology

Types of Research in Our Community

Basic Science

Clinical

Registries

Clinical Trials

RESEARCH

Focus Groups

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.

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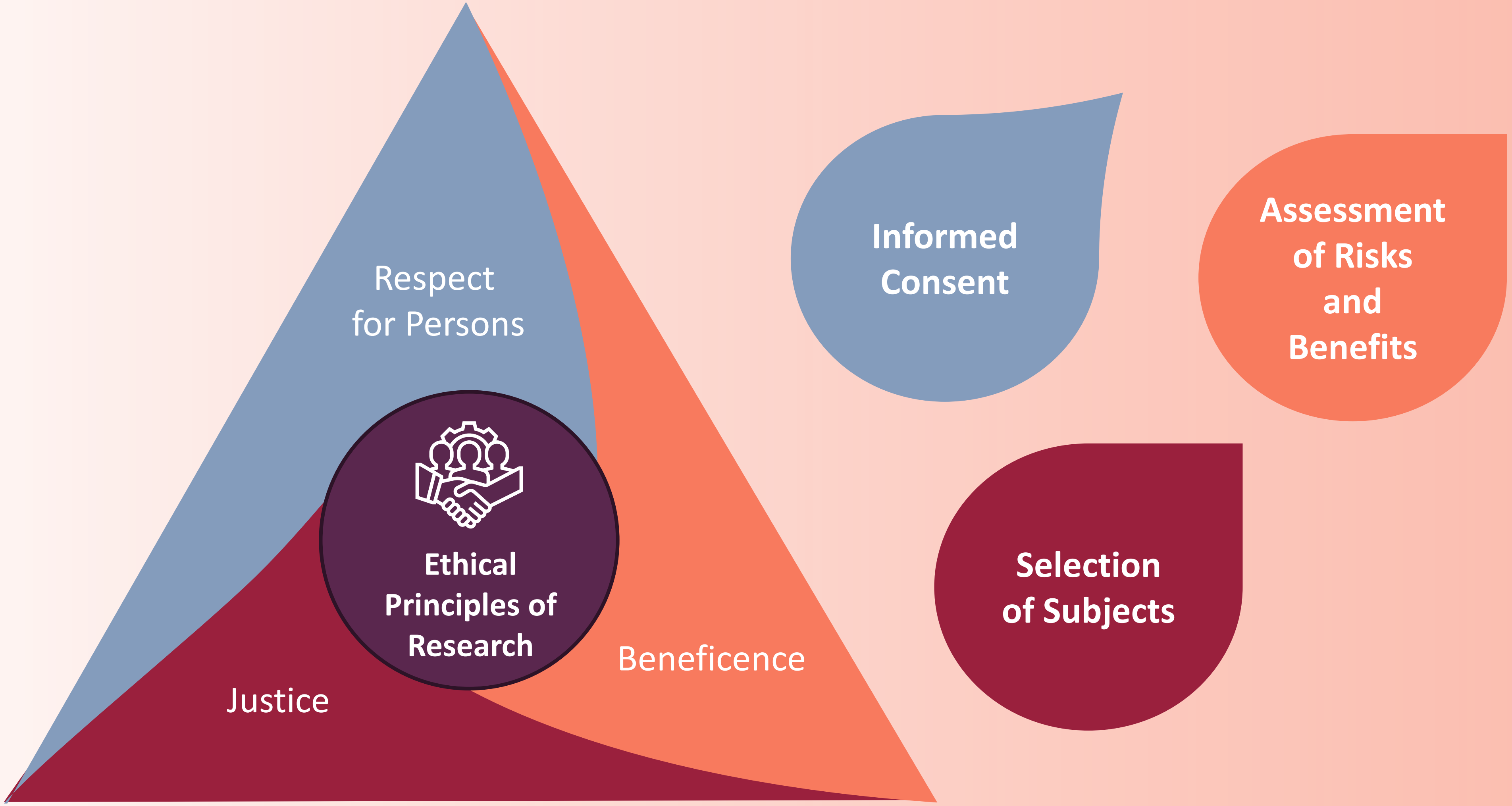

Respect for Persons

Beneficence

Justice

Ethical Principles of Research

Informed Consent

Assessment of Risks and Benefits

Selection of Subjects

National Commission for the Protection of Human Subjects of Biomedical and Behavioral Research
https://www.hhs.gov/ohrp/regulations-and-policy/belmont-report/read-the-belmont-report/index.html

Informed Consent Process

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01 The reason for the study

02 Risks and discomforts

03 Benefits

04 Alternatives to enrolling

05 Confidentiality

06 Compensation and treatment in case of an injury

07 Contacts

08 Voluntary participation

https://www.hhs.gov/ohrp/regulations-and-policy/guidance/checklists/index.html

Ensuring Safe and Ethical Research

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Health and Human Services (HHS) – specifically, Office for Human Research Protections

IRB (Institutional Review Board)

Trand Good Clinical Practice (GCP)

Food and Drug Administration (FDA), if applicable

Data and Safety Monitoring Boards

Privacy Rule

Possible Benefits of Participating in Studies

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- 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?scoid=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

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- 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?

athn
american thrombosis & hemostasis network

HTC's Multidisciplinary Team

Pharmaceutical Industry

CDC

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HFA
Hemophilia Federation of America

Chapters and YOU!

NIH National Heart, Lung, and Blood Institute

Other Researchers and Scientists

Others

Why?

IBDs are rare conditions

Research in Inheritable Bleeding Disorders (IBDs)

Research by Numbers

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~330,000,000

Number of people in the US

~116,000,000

Number of people with **high blood pressure** in the US

~17,000,000

Number of people with **diabetes** in the US

~21,810

Number of people with **hemophilia A** in the US

~7,030

Number of people with **hemophilia B** in the US

~255

Number of people with **FXIII deficiency** in the US

All Inheritable Bleeding Disorders

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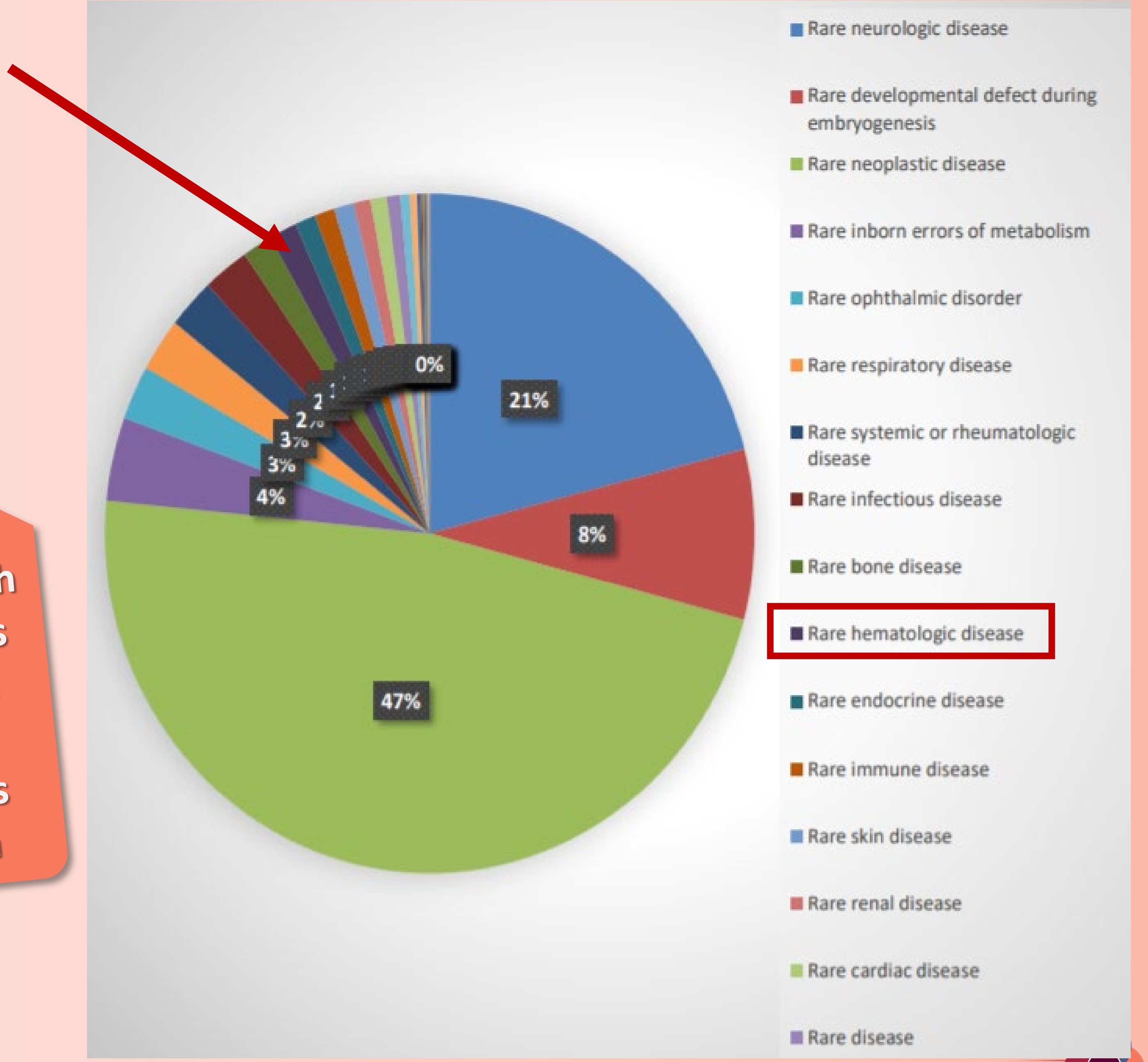
Diagnosis	Unique patients
Alpha-2 Antiplasmin deficiency	13
Bernard Soulier syndrome	83
Blood coagulation disorder without specific diagnosis	2,186
Ehlers-Danlos syndrome	429
Factor I (1)	404
Factor II (2)	123
Factor V (5)	571
Factor VII (7)	2,654
Factor VIII (8)	21,809
Factor IX (9)	7,031
Factor X (10)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

Diagnosis	Unique patients
Hermansky-Pudlak syndrome	131
PAI-1 deficiency	345
Platelet function disorder, nonspecific	3,693
Platelet release defect	55
Platelet storage pool disease	3,525
Thrombocytopenia, hereditary	920
Venous Thromboembolism (VTE)	36,535
Von Willebrand disease, type 1	21,370
Von Willebrand disease, type 1C	120
Von Willebrand disease, type 2A	1,140
Von Willebrand disease, type 2B	646
Von Willebrand disease, type 2M	620
Von Willebrand disease, type 2N	193
Von Willebrand disease, type 2, type unknown	482
Von Willebrand disease, type 3	472
Von Willebrand disease, type other	343
Von Willebrand disease, unknown	2,269

IBDs are rare conditions

IBD research constitutes <2% of all rare disorders research



The International Rare Disease Research Consortium, 2018

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IBDs are rare conditions

IBD research constitutes <2% of all rare disorders research

Significant progress requires all of us

Research in Inheritable Bleeding Disorders (IBDs)

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https://www.reddit.com/r/mildlyinteresting/comments/9naf24/this_largegrain_sand_on_the_beach/

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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

Research in Inheritable Bleeding Disorders (IBDs)

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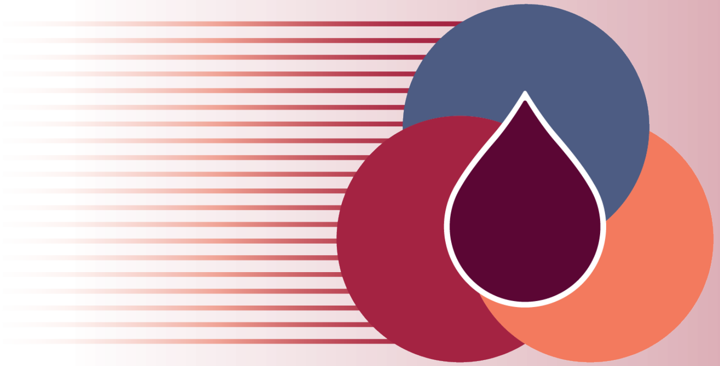

Equity, Inclusion, and Research: Ensuring Research Findings Include You

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

Dennis, R.M. (2004). "Racism". In Kuper, A.; Kuper, J. (eds.). The Social Science Encyclopedia, Volume 2 (3rd ed.). London; New York: Routledge.

Definitions

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- **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"

Calliste, A.M.; Dei, G.J.S. Power, Knowledge and Anti-Racism Education: A Critical Reader; Fernwood: Halifax, NS, Canada, 2000;
188p.

Historical Overview of Medicine in the United States

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- 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
J Natl Med Assoc. 2001 Mar; 93(3 Suppl): 11S–34S.

Recognition of Racism in Medicine & Science

AAFP / About Us / Policies / All Policies / Institutional Racism in the Health Care System

Institutional Racism in the Health Care System

The American Academy of Family Physicians (AAFP) recognizes that racism is a system that categorizes people based on race, color, ethnicity a
allocate societal goods and resources in a way that unfairly disadvantages some, while without merit, rewards others. As a system, racism has b
way that permits the establishment of patterns, procedures, practices and policies within organizations that consistently penalizes and exploits p
race, color, culture or ethnic origin. The system of racism affects the attitudes, beliefs and behaviors of one individual towards another (personally
individuals perceive themselves (internalized).

The AAFP also recognizes the impact of racism within the U.S. health care delivery system, which has historically engaged in the systematic seg
discrimination of patients based on race and ethnicity, the effects of which persist to this day. Hospitals and clinics, which were once designated
minorities, continue to experience significant financial constraints and are often under-resourced and improperly staffed. These issues result in i
quality of health care and are major contributors to racial and ethnic health disparities. While segregation and discrimination based on race and e
today, some organizations continue to discriminate based on insurance status, which also disproportionately impacts non-white populations.

The AAFP opposes all forms of institutional racism and supports family physicians to actively work to dismantle racist and discriminatory practic
organizations and communities.

https://www.aafp.org/about/policies/all/institutional-racism.html

AMA adopts guidelines that confront systemic racism in medicine

JUN 15, 2021

CHICAGO — The nation's physicians and medical students today continued to acknowledge the realities of structural racism in medicine by adding to policy that informs the American Medical Association's ambitious work to dismantle racist policies and practices across all of health care.

Members of the AMA's House of Delegates representing their peers from all corners of medicine voted to adopt guidelines addressing systemic racism in medicine, including discrimination, bias and abuse, including expressions of prejudice known as microaggressions. The AMA will recommend that health care organizations and systems use the new guidelines to establish institutional policies that promote positive cultural change and ensure a safe, discrimination-free work environment.

"Systemic racism in medicine is the most serious barrier to the advancement of health equity and appropriate medical care," said AMA Board Member Willarda V. Edwards, M.D., M.B.A. "Today's actions by the House of Delegates will inform the AMA's active work to proactively identify, prevent, and eliminate racism and will help the AMA guide health care organizations in efforts to adopt workplace policies that promote positive cultural transformation and address the root cause of racial health inequities."

https://www.ama-assn.org/press-center/press-releases/ama-adopts-guidelines-confront-systemic-racism-medicine

Analysis: Declarations of Racism as a Public Health Crisis

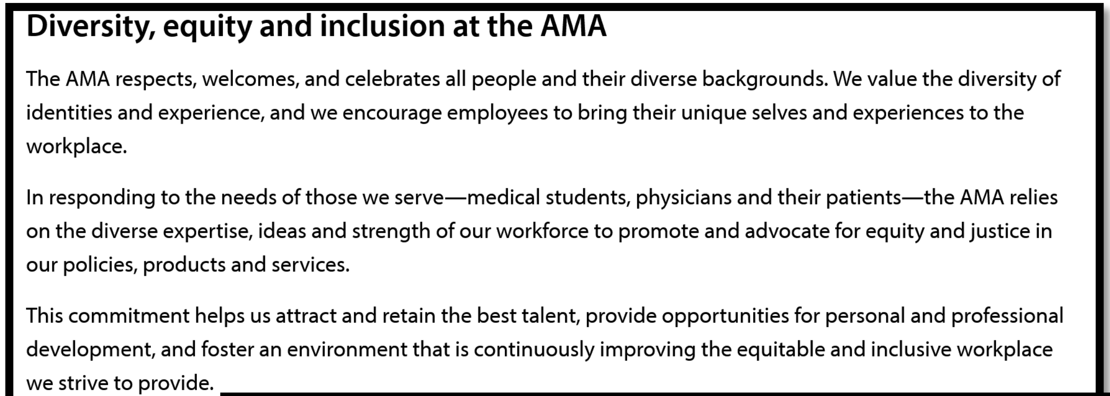
In 2018, Milwaukee County, Wisconsin, became the first community to declare racism a public health crisis. Many communities have since done so, with the pace of adoption accelerating after the police killing of George Floyd and the protests for justice that followed, with nearly 200 declarations passing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

DEI statements – AMA, ASH, NASW



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.

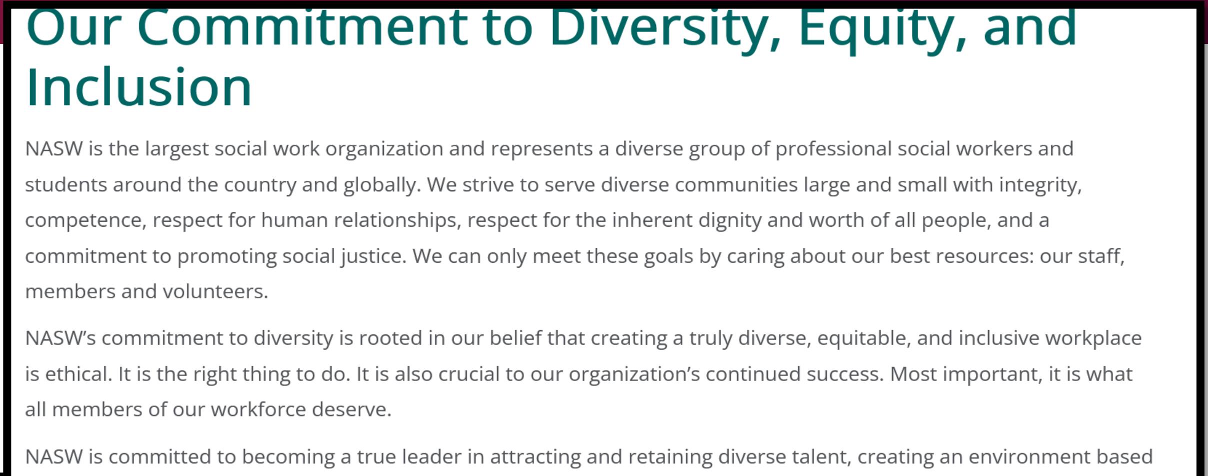
This commitment helps us attract and retain the best talent, provide opportunities for personal and professional development, and foster an environment that is continuously improving the equitable and inclusive workplace we strive to provide.

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 combatting 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

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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

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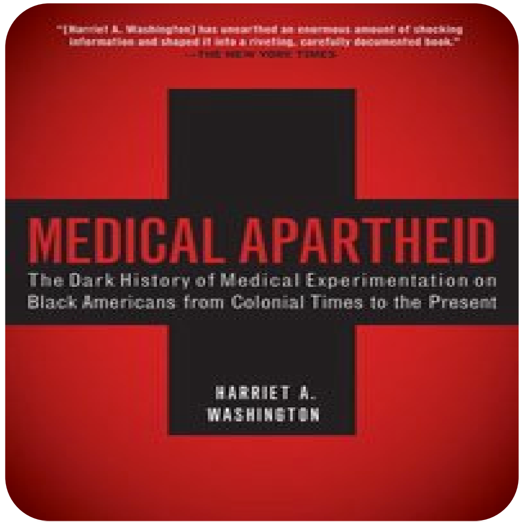
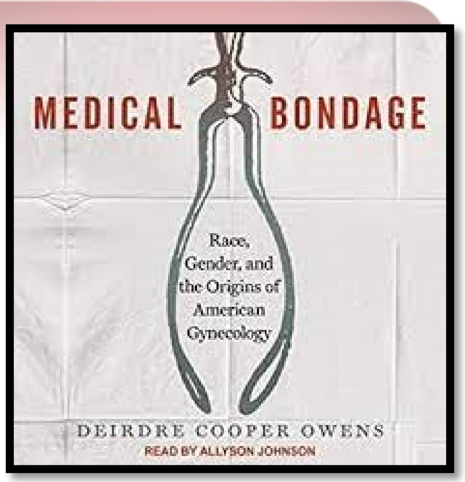
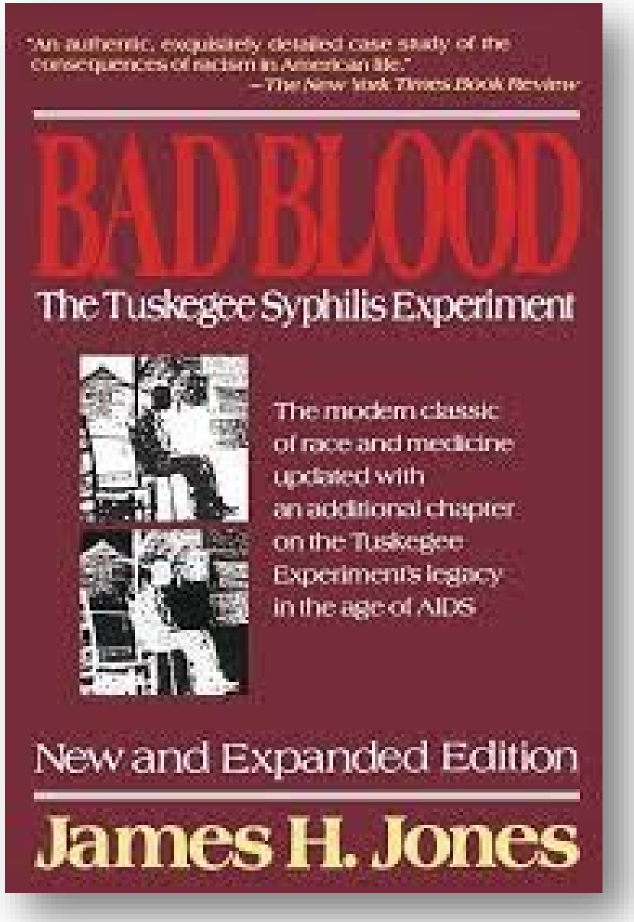
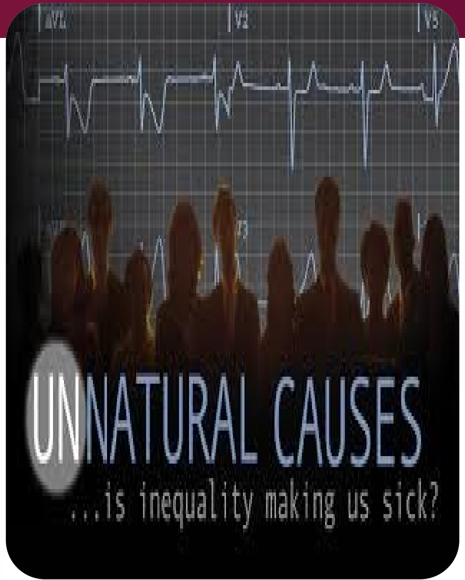
- Gender bias in hemophilia
- Tension in the healthcare system for women with hemophilia
- Dr. Marion Sims performs gynecological exams on slaves WITHOUT anesthesia (continued teaching in medical schools)
- Early radiation experiments on Black children in the late 1920s
- Darwinism is taught promoting 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

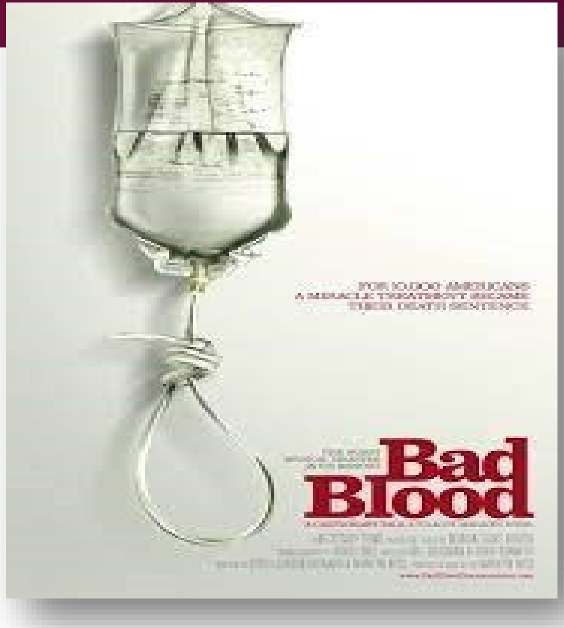
Books and films that document the history of prejudice in medicine

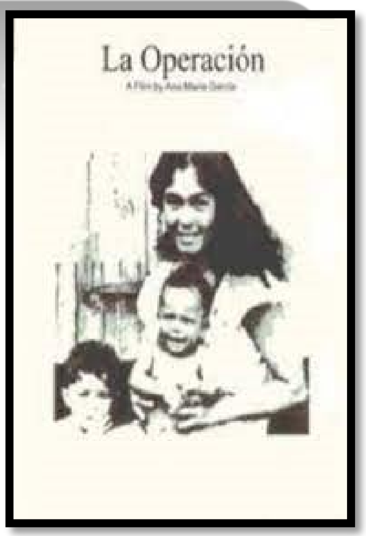


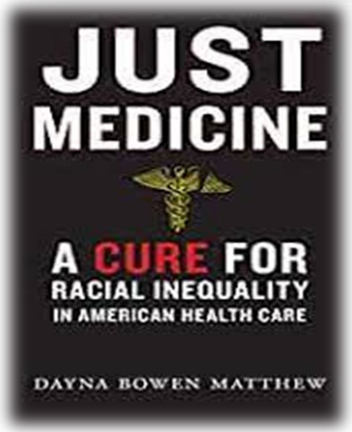












What does the research indicate?

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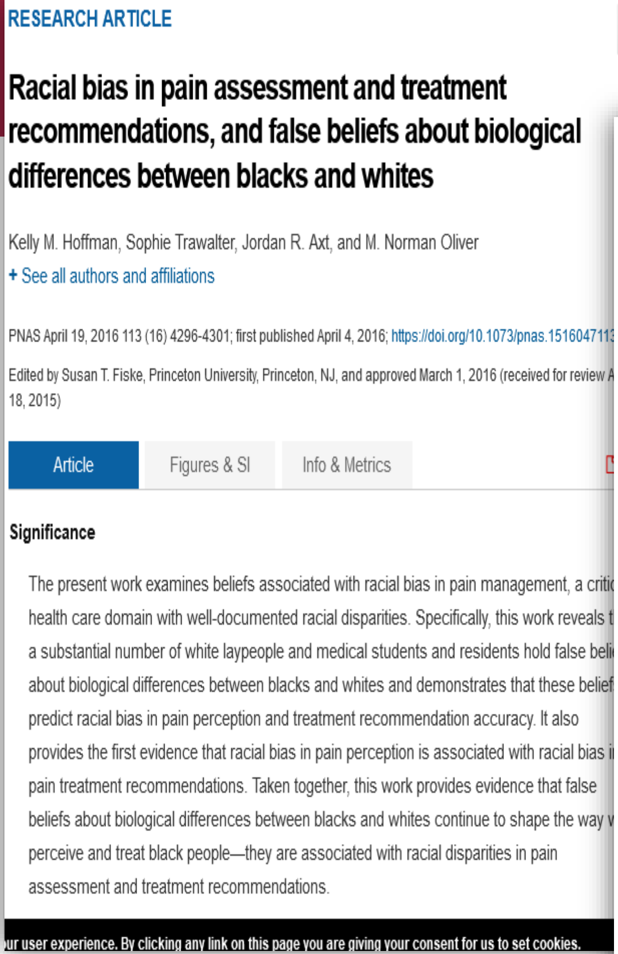

RESEARCH ARTICLE

Racial bias in pain assessment and treatment recommendations, and false beliefs about biological differences between blacks and whites

Kelly M. Hoffman, Sophie Trawalter, Jordan R. Axt, and M. Norman Oliver

+ See all authors and affiliations

PNAS April 19, 2016 113 (16) 4296-4301; first published April 4, 2016; https://doi.org/10.1073/pnas.151604711

Edited by Susan T. Fiske, Princeton University, Princeton, NJ, and approved March 1, 2016 (received for review A
18, 2015)

Article | Figures & SI | Info & Metrics

Significance

The present work examines beliefs associated with racial bias in pain management, a critic
health care domain with well-documented racial disparities. Specifically, this work reveals t
a substantial number of white laypeople and medical students and residents hold false beli
about biological differences between blacks and whites and demonstrates that these belief
predict racial bias in pain perception and treatment recommendation accuracy. It also
provides the first evidence that racial bias in pain perception is associated with racial bias i
pain treatment recommendations. Taken together, this work provides evidence that false
beliefs about biological differences between blacks and whites continue to shape the way v
perceive and treat black people—they are associated with racial disparities in pain
assessment and treatment recommendations.

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Review > Pain Med. 2012 Feb;13(2):150-74. doi: 10.1111/j.1526-4637.2011.01310.x.

Epub 2012 Jan 13.

Time to take stock: a meta-analysis and systematic
review of analgesic treatment disparities for pain in
the United States

Salimah H Meghani [1], Eeeseung Byun, Rollin M Gallagher

Affiliations + expand

PMID: 22239747 DOI: 10.1111/j.1526-4637.2011.01310.x

Abstract

Background: The recent Institute of Medicine Report assessing the state of pain care in the United
States acknowledged the lack of consistent data to describe the nature and magnitude of unrelieve
pain and identify subpopulations with disproportionate burdens.

Objectives: We synthesized 20 years of cumulative evidence on racial/ethnic disparities in analgesi
treatment for pain in the United States. Evidence was examined for the 1) magnitude of associatior
between race/ethnicity and analgesic treatment; 2) subgroups at an increased risk; and 3) the effec
moderators (pain type, setting, study quality, and data collection period) on this association.

Methods: United States studies with at least one explicit aim or analysis comparing analgesic
treatment for pain between Whites and a minority group were included (SciVerse Scopus database,
1989-2011).

Results: Blacks/African Americans experienced both a higher number and magnitude of disparities
than any other group in the analyses. Opioid treatment disparities were ameliorated for
Hispanics/Latinos for "traumatic/surgical" pain (P = 0.293) but remained for "non-
traumatic/nonsurgical" pain (odds ratio [OR] = 0.70, 95% confidence interval [CI] = 0.64-0.77, P =
0.000). For Blacks/African Americans, opioid prescription disparities were present for both types of
pain and were starker for "non-traumatic/nonsurgical" pain (OR = 0.66, 95% CI = 0.59-0.75, P =
0.000). In subanalyses, opioid treatment disparities for Blacks/African Americans remained consiste
across pain types, settings, study quality, and data collection periods.

Conclusion: Our study quantifies the magnitude of analgesic treatment disparities in subgroups of
minorities. The size of the difference was sufficiently large to raise not only normative but quality a
safety concerns. The treatment gap does not appear to be closing with time or existing policy
initiatives. A concerted strategy is needed to reduce pain care disparities within the larger quality o
care initiatives.

Wiley Periodicals, Inc.

PLoS One. 2020; 15(2): e0229099. PMCID: PMC7032703
Published online 2020 Feb 20. doi: 10.1371/journal.pone.0229099 PMID: 32078655

Healthcare provider perspectives on inequities in access to care for patients with inherited bleeding disorders

Sumedha Arya, Conceptualization, Data curation, Formal analysis, Funding acquisition, Investigation, Methodology, Project administration, Resources, Visualization, Writing – original draft, Writing – review & editing,[1] Pamela Wilton, Methodology, Resources, Writing – review & editing,[2] David Page, Methodology, Resources, Writing – review & editing,[2] Laurence Boma-Fischer, Methodology, Resources, Writing – review & editing,[3,4] Georgina Floros, Methodology, Resources, Writing – review & editing,[4,5] Katie N. Dainty, Conceptualization, Methodology, Writing – review & editing,[6,7] Rochelle Winikoff, Conceptualization, Methodology, Writing – review & editing,[8] and Michelle Sholzberg, Conceptualization, Funding acquisition, Investigation, Methodology, Project administration, Resources, Supervision, Validation, Writing – review & editing[9,10,*]

Janhavi Ajit Vaingankar, Editor

- Author information - Article notes - Copyright and License information Disclaimer

Associated Data

- Supplementary Materials
- Data Availability Statement

Abstract

Introduction

The ways in which social determinants of he
unclear. The objective of this study was to u
care and diagnostic delay amongst this patie

Methods

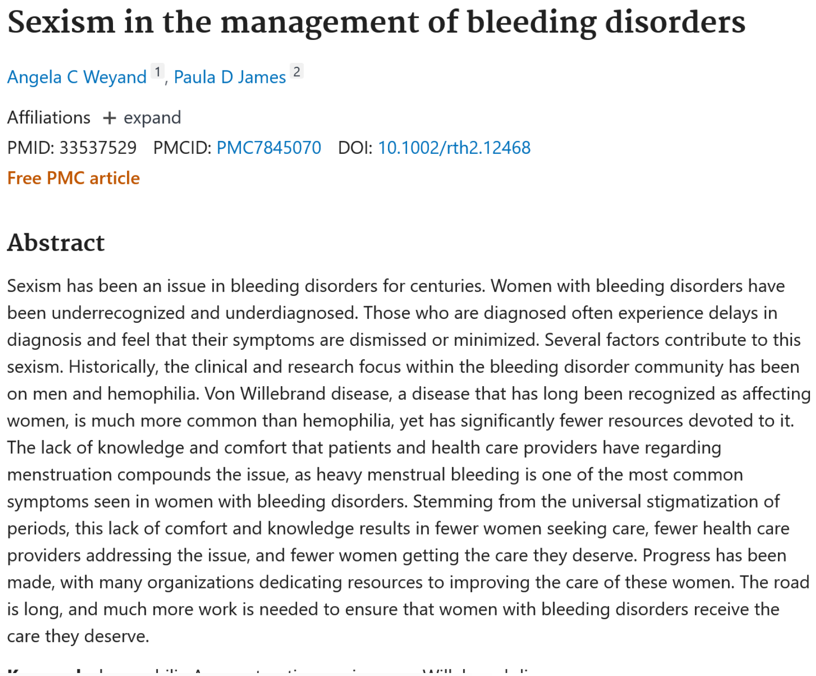
A healthcare provider survey comprising 24
disseminated online with recruitment to all r
Canada (N = 73), members of the Canadian
members of the Canadian Physiotherapists i

Results

There were 70 respondents in total, for a tota
delays for patients with mild symptomatolog
bleeding as their only or primary symptom (
35). Fewer respondents felt that factors such
influenced access to care, particularly as con

Discussion

We found that healthcare providers identified
uterine bleeding, and residence in rural locat
factors warrant further study, and will be inv
survey and ongoing in-depth qualitative pati



Sexism in the management of bleeding disorders

Angela C Weyand [1], Paula D James [2]

Affiliations + expand

PMID: 33537529 PMCID: PMC7845070 DOI: 10.1002/rth2.12468

Free PMC article

Abstract

Sexism has been an issue in bleeding disorders for centuries. Women with bleeding disorders have been underrecognized and underdiagnosed. Those who are diagnosed often experience delays in diagnosis and feel that their symptoms are dismissed or minimized. Several factors contribute to this sexism. Historically, the clinical and research focus within the bleeding disorder community has been on men and hemophilia. Von Willebrand disease, a disease that has long been recognized as affecting women, is much more common than hemophilia, yet has significantly fewer resources devoted to it. The lack of knowledge and comfort that patients and health care providers have regarding menstruation compounds the issue, as heavy menstrual bleeding is one of the most common symptoms seen in women with bleeding disorders. Stemming from the universal stigmatization of periods, this lack of comfort and knowledge results in fewer women seeking care, fewer health care providers addressing the issue, and fewer women getting the care they deserve. Progress has been made, with many organizations dedicating resources to improving the care of these women. The road is long, and much more work is needed to ensure that women with bleeding disorders receive the care they deserve.

Solutions with patients at the center

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

B A Israel [1], A J Schulz, E A Parker, A B Becker

Affiliations + expand

PMID: 9611617 DOI: 10.1146/annurev.publhealth.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.

› J Natl Med Assoc. 2008 Nov;100(11):1275-85. doi: 10.1016/s0027-9684(15)31505-4.

Patient centeredness, cultural competence and healthcare quality

Somnath Saha [1], Mary Catherine Beach, Lisa A Cooper

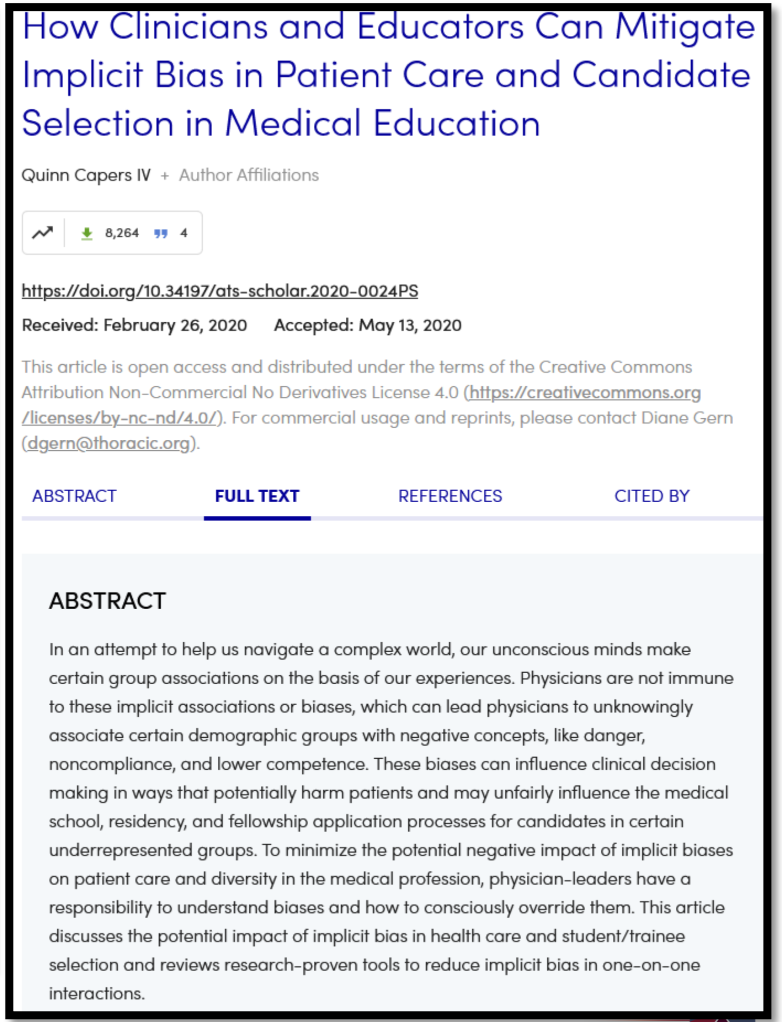
Affiliations + expand

PMID: 19024223 PMCID: PMC2824588 DOI: 10.1016/s0027-9684(15)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 Capers IV + Author Affiliations

8,264 4

https://doi.org/10.34197/ats-scholar.2020-0024PS

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ABSTRACT

In an attempt to help us navigate a complex world, our unconscious minds make certain group associations on the basis of our experiences. 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 health care and student/trainee selection and reviews research-proven tools to reduce implicit bias in one-on-one interactions.

Saha,S., Beach, M.C., & Cooper, L.A. (2008). J Natl Med Assoc, 100(11), 1275-1285.

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)

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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.

Minkler, Meredith. "Ethical challenges for the "outside" researcher in community-based participatory research." *Health Education & Behavior* 31, no. 6 (2004): 684-697.

Community Based Participatory Research (CBPR)

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- 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.

Minkler, Meredith. "Ethical challenges for the "outside" researcher in community-based participatory research." *Health Education & Behavior* 31, no. 6 (2004): 684-697.

Community Based Participatory Research (CBPR)

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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.

Minkler, Meredith. "Ethical challenges for the “outside” researcher in community-based participatory research." *Health Education & Behavior* 31, no. 6 (2004): 684-697.

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.

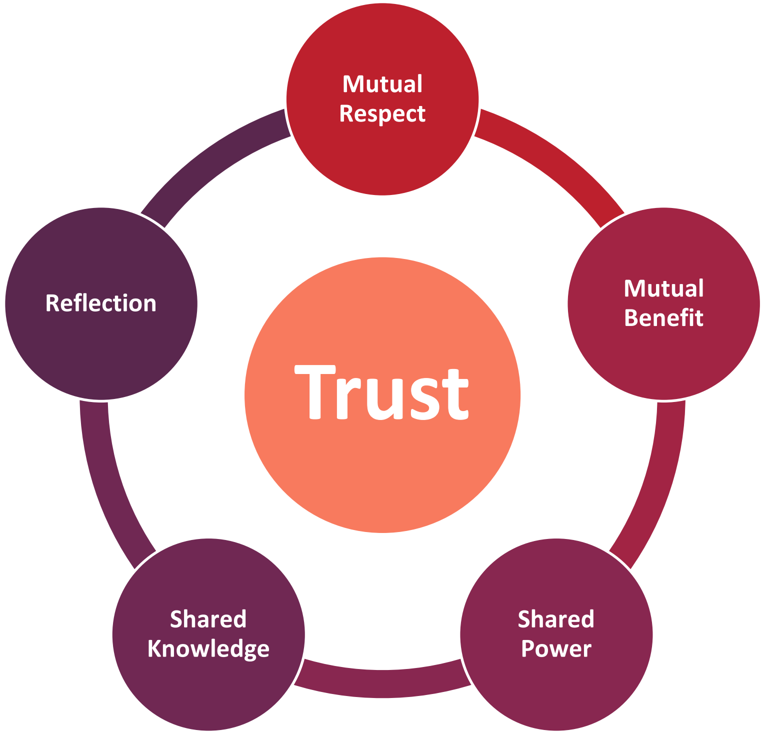
- CBPR emphasizes the dissemination of research findings to all partners and communities involved and in ways that are understandable, respectful, and useful
- All partners must engage in the broader dissemination of results, for example as coauthors of publications and co-presenters at meetings and 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



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Research from where we stand: A conversation from our perspective

Michelle Witkop

NHF, Research Department

The Conversation

Erin

The Fundraiser Experience

Ryan

The Personal Experience

Liz & Miguel

The Parent's Experience

Esmeralda

The SME Experience

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?

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Let’s Design a Protocol TOGETHER!

Donna Di Michele
Consultant

Clinical Trial Phases

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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

FDA

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!

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for all bleeding disorders

- 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!

National Hemophilia Foundation
for all bleeding disorders

- ❖ 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

Let’s Get Started!

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Study Question: *Is the study studying the treatment effect that is the most useful to the IBD community? Is it addressing the safety concerns?*

Study Eligibility: *Who will the study allow to participate?*

Recruitment: *How do we give all eligible participants across all bleeding disorders a chance to participate?*

Study Design: *How can we design a study that will get all the information we need w/o being overly burdensome on participants?*

Dissemination: *How should participants be informed about the interim and final results of the study?*

All Inheritable Bleeding Disorders

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

Diagnosis	Unique patients
Hermansky-Pudlak syndrome	131
PAI-1 deficiency	345
Platelet function disorder, nonspecific	3,693
Platelet release defect	55
Platelet storage pool disease	3,525
Thrombocytopenia, hereditary	920
Venous Thromboembolism (VTE)	36,535
Von Willebrand disease, type 1	21,370
Von Willebrand disease, type 1C	120
Von Willebrand disease, type 2A	1,140
Von Willebrand disease, type 2B	646
Von Willebrand disease, type 2M	620
Von Willebrand disease, type 2N	193
Von Willebrand disease, type 2, type unknown	482
Von Willebrand disease, type 3	472
Von Willebrand disease, type other	343
Von Willebrand disease, unknown	2,269

Study Question

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

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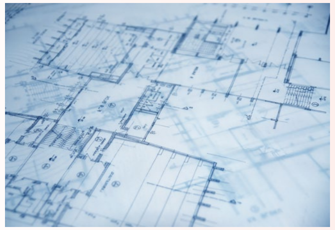
To be eligible for this study, participants would need to be:

☐ 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

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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

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NRB Working Group Chairs & YOU

Moderator: Donna Di Michele
Consultant

NRB Working Groups and their charges

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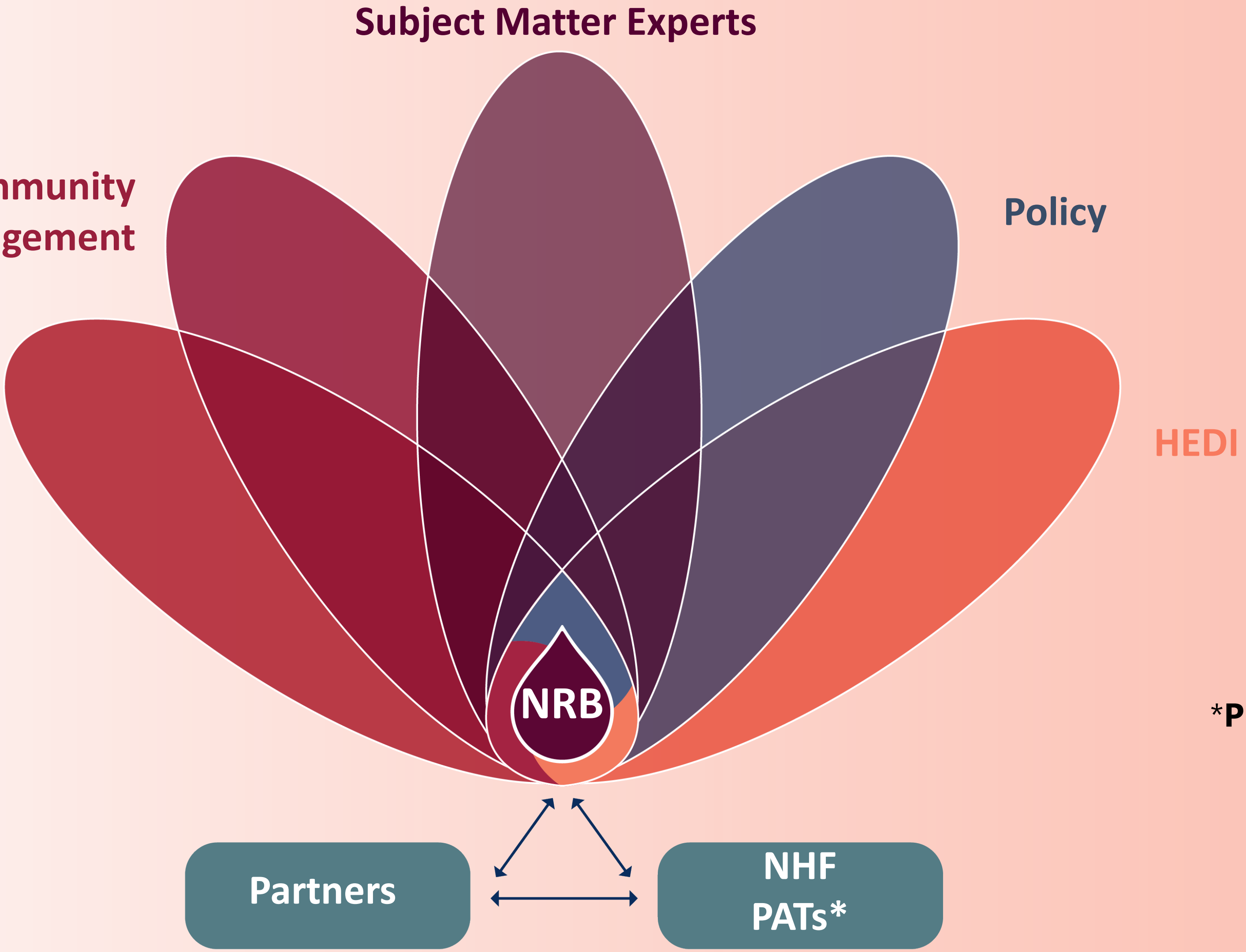

*__Priority Action Teams__
1. Ultra-Rare Disorders
2. Mental Health
3. VWD
4. Digital Health
5. Future Therapies

R&D, **Infrastructure**, Workforce Cluster

Moses Miles
Maggie Ragni

about us

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Moses Miles

Maggie Ragni

Ziva Mann, SME

Our goal:

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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

People

Processes

Tools

Structures

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?

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Tell us!

Maggie Ragni
ragni@pitt.edu

Ziva Mann
ziva.mann.consulting@gmail.com

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R&D, Infrastructure, **Workforce** Cluster

R&D Jill Johnsen
Workforce Alice Ma

R&D, Infrastructure, Workforce Cluster

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Working Group Chairs

Research & Development (R&D)

Jill Johnsen

Infrastructure

Moses Miles

Margaret 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

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Health Equity, Diversity, and Inclusion Working Group

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

HEDI

National Hemophilia Foundation
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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

Policy Working Group

Deniece Chevannes, Nathan Schaefer, and Sonji Wilkes

Policy

National Hemophilia Foundation
for all bleeding disorders

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

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Subject Matter Experts (SME) Working Group

Erin Cirelli and Esmeralda Vázquez

SMEs

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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

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Community Engagement Working Group

Community Engagement

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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

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THANK YOU!