NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

NATIONAL RESEARCH BLUEPRINT

Washington, D.C.

April 8-9
2022

WELCOME BACK!

Today's Agenda

NATIONAL HEMOPHILIA FOUNDATION
for all bleeding disorders

Day 2: Saturday 4/09/2022		
7:00 - 8:30 AM	Breakfast (Salons D, E)	
8:30 - 9:30 AM	NHF, Research, and YOU!	Michelle Witkop
9:30 - 10:30 AM	Think Different: SMEs as the Catalyst for Change	Maria Santaella & Esmeralda Vazquez
10:30 - 11:00 AM	Break & Check-out	
11:00 - 12:00 PM	Community Engagement: Can You Hear Me Now?	Ilana Ostrin & Brett Spitale
12:00 - 12:30 PM	Summary of Event & Call to Action	Kevin Mills
12:30 PM	Lunch at the hotel (Salons D, E) and Departures	

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National Research Blueprint

Please scan this QR code to provide additional comments

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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NHF, Research, and YOU!

Michelle Witkop
NHF, Research Department

NHF has been funding research since 1972!

At all levels of expertise & in all disciplines, NHF is funding research across full spectrum of experience in the IBD community

Judith Graham Pool Post Doctoral Fellowship

- For the early researchers
- Basic science
- Pre-clinical research
- Awarded since 1972
- $52,000 a year for 2 years

Jeanne Marie Lusher Diversity Fellowship

- For the early researchers from diverse backgrounds
- Recruitment and retaining a diverse workforce
- Awarded since 2021
- $52,000 a year for 3 years

Career Development

- For the mid-level researchers
- Innovative research in subcellular, cellular, animal or human levels
- Awarded since 2000
- $70,000 a year for 3 years

Innovative Investigator

- For any discipline in the HTC team
- Novel technologies and advanced therapies
- Awarded since 2018
- $60,000 a year for 12-18 months

Bridge

- For the experienced researcher who applied for an NIH R01 grant or equivalent federal grant but were denied funding
- Basic, translational, patient-oriented research
- Awarded since 2018
- $125,000 for 1 year

Excellence Fellowships

$15,000

once a year

- **Nursing**
- **Social Work**
- **Physical Therapy**

Multidisciplinary advances in training, career development, and clinical care

NHF-Takeda Clinical Fellowship

$100,000/year for 2 years

Provides physicians hands-on clinical and research experience at highly regarded HTCs

Evolution of NHF Research Funding

1972

JGP
106 awarded
$9,124,000

2000

CDA
29 awardees
$6,090,000

2006

Social Work Excellence
15 awarded
$155,000

2008

Nursing Excellence
13 awarded
$135,000

2011

Physical Therapy Excellence
10 awarded
$105,000

2017

IIR
6 awarded
$360,000

NHF Research Dept. Established

2018

Bridge Award
2 awarded
$250,000

2021

JML
2 awarded
$312,000

Excellence Awards Transitioned To Research Dept.

Total NHF Funding for Research to date: $16, 531,000 = **$16.5M**

Where can SMEs access data?

Sources

- NHF website - Hemophilia.org
  - Clinical Trial Finder hemophilia.org | Community Resources | Resources Near You | Clinical Trials - redirects you to pre-filtered ClinicalTrials.gov
- CVR Participant Dashboard
- CDC Data Visualization Tool https://communitycountsdataviz.cdc.gov/blooddisorders/#!/
- HFA website

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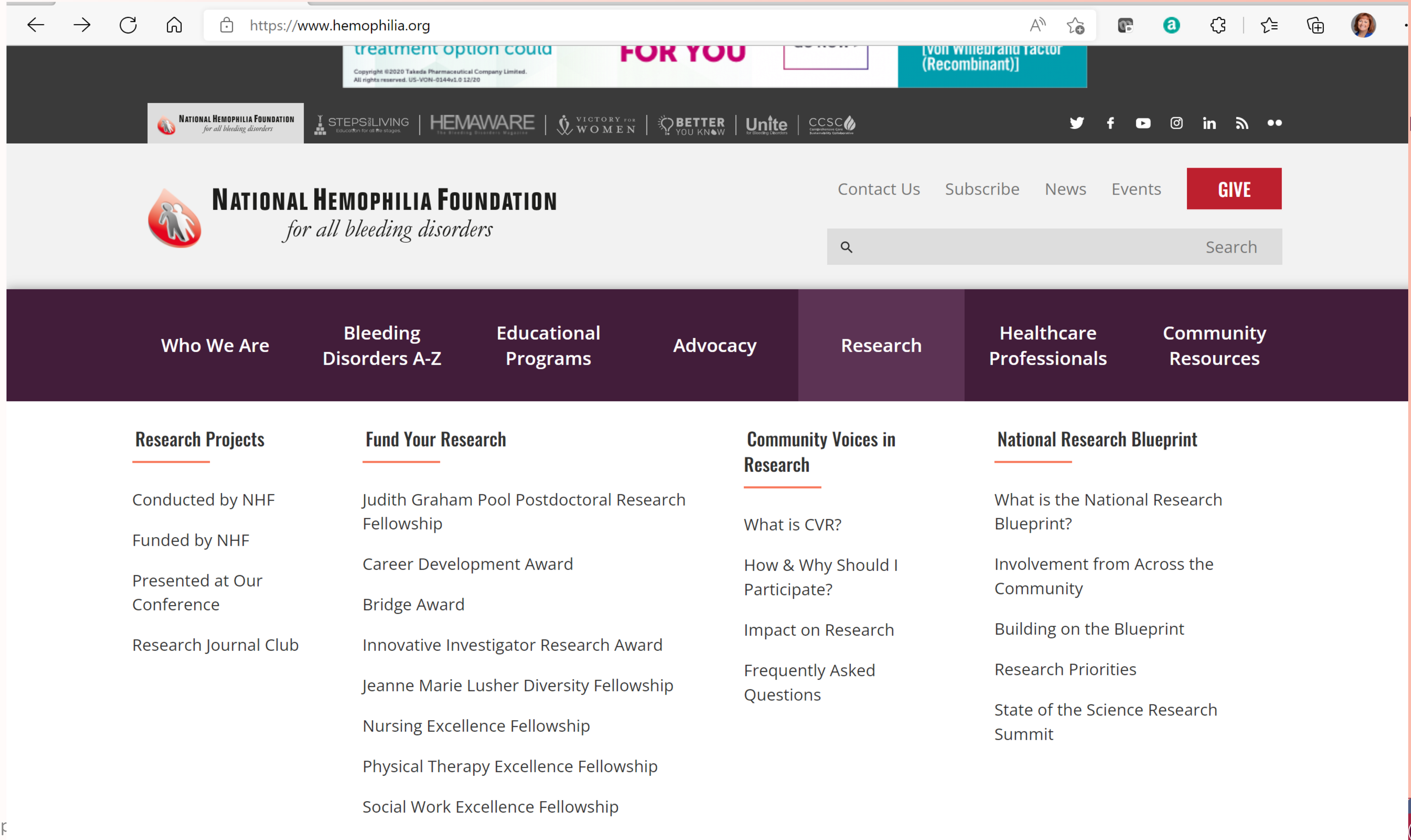
NHFs Website
www.hemophilia.org

9 April 2022



https://www.hemophilia.org

treatment option could

FOR YOU

[von Willebrand factor (Recombinant)]

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Search

Who We Are | Bleeding Disorders A-Z | Educational Programs | Advocacy | Research | Healthcare Professionals | Community Resources

Research Projects
- Conducted by NHF
- Funded by NHF
- Presented at Our Conference
- Research Journal Club

Fund Your Research
- Judith Graham Pool Postdoctoral Research Fellowship
- Career Development Award
- Bridge Award
- Innovative Investigator Research Award
- Jeanne Marie Lusher Diversity Fellowship
- Nursing Excellence Fellowship
- Physical Therapy Excellence Fellowship
- Social Work Excellence Fellowship

Community Voices in Research
- What is CVR?
- How & Why Should I Participate?
- Impact on Research
- Frequently Asked Questions

National Research Blueprint
- What is the National Research Blueprint?
- Involvement from Across the Community
- Building on the Blueprint
- Research Priorities
- State of the Science Research Summit

Clinical Trial Finder

www.Clinicaltrials.gov

Clinical Trial Finder Main

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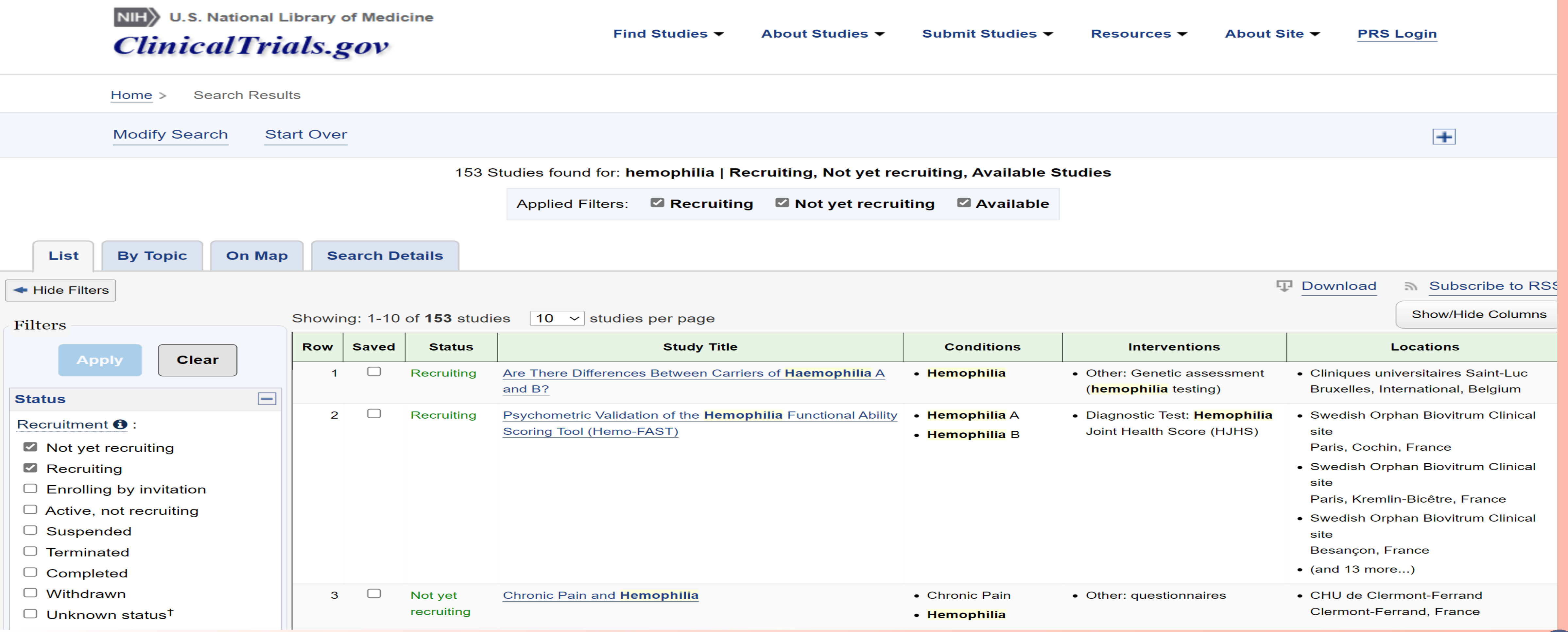

153 Studies found for: **hemophilia | Recruiting, Not yet recruiting, Available Studies**

Applied Filters: ☑ **Recruiting** ☑ **Not yet recruiting** ☑ **Available**

Status

Recruitment ℹ :

- ☑ Not yet recruiting
- ☑ Recruiting
- ☐ Enrolling by invitation
- ☐ Active, not recruiting
- ☐ Suspended
- ☐ Terminated
- ☐ Completed
- ☐ Withdrawn
- ☐ Unknown status†

Showing: 1-10 of **153** studies 10 studies per page

Row	Saved	Status	Study Title	Conditions	Interventions	Locations
1	☐	Recruiting	Are There Differences Between Carriers of **Haemophilia A** and B?	• **Hemophilia**	• Other: Genetic assessment (**hemophilia** testing)	• Cliniques universitaires Saint-Luc Bruxelles, International, Belgium
2	☐	Recruiting	Psychometric Validation of the **Hemophilia** Functional Ability Scoring Tool (Hemo-FAST)	• **Hemophilia** A • **Hemophilia** B	• Diagnostic Test: **Hemophilia** Joint Health Score (HJHS)	• Swedish Orphan Biovitrum Clinical site Paris, Cochin, France • Swedish Orphan Biovitrum Clinical site Paris, Kremlin-Bicêtre, France • Swedish Orphan Biovitrum Clinical site Besançon, France • (and 13 more...)
3	☐	Not yet recruiting	Chronic Pain and **Hemophilia**	• Chronic Pain • **Hemophilia**	• Other: questionnaires	• CHU de Clermont-Ferrand Clermont-Ferrand, France

Clinical Trial Finder Eligibility

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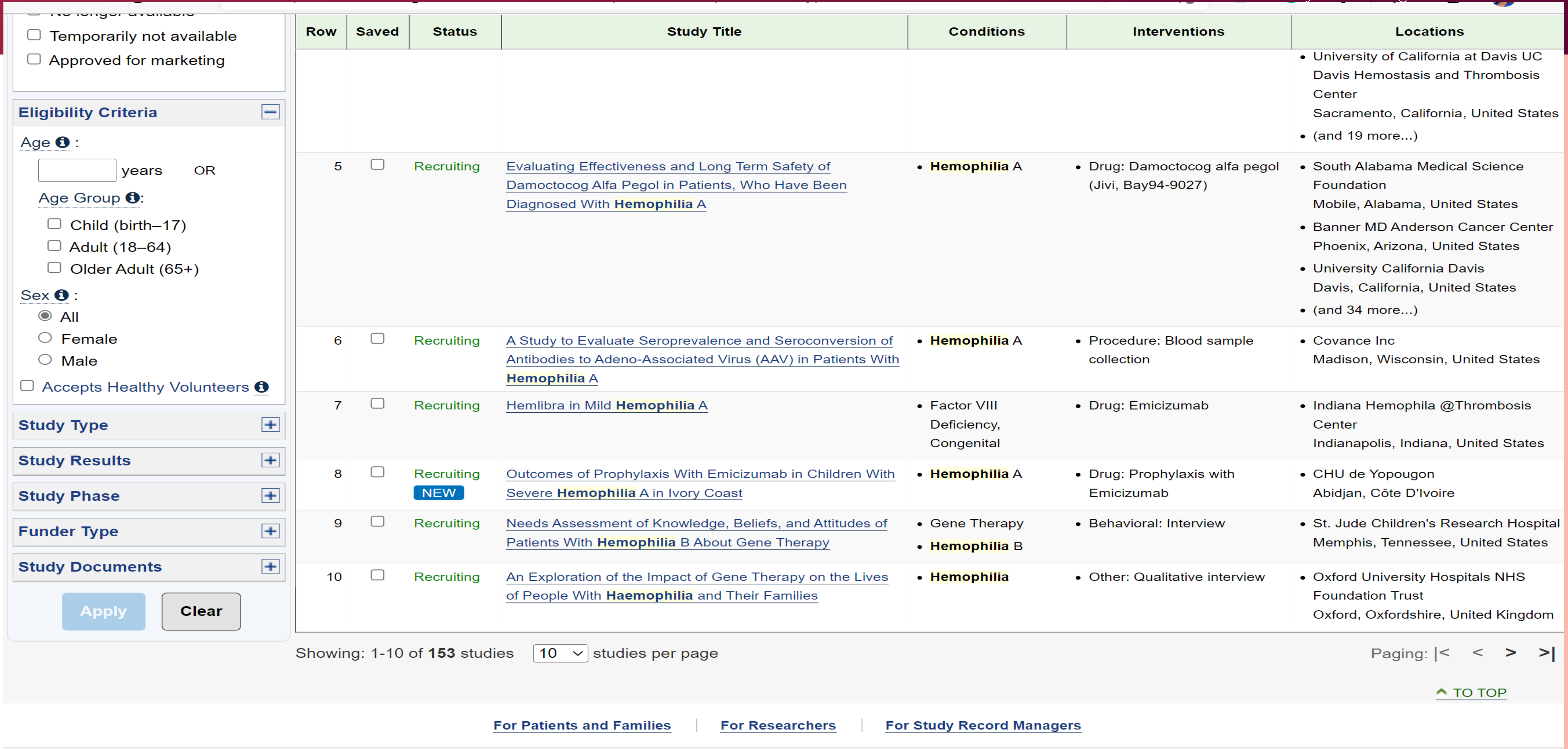

☐ Temporarily not available
☐ Approved for marketing

Eligibility Criteria

Age:
years OR
Age Group:
☐ Child (birth–17)
☐ Adult (18–64)
☐ Older Adult (65+)

Sex:
● All
○ Female
○ Male

☐ Accepts Healthy Volunteers

Study Type
Study Results
Study Phase
Funder Type
Study Documents

Row	Saved	Status	Study Title	Conditions	Interventions	Locations
						University of California at Davis UC Davis Hemostasis and Thrombosis Center Sacramento, California, United States (and 19 more...)
5	☐	Recruiting	Evaluating Effectiveness and Long Term Safety of Damoctocog Alfa Pegol in Patients, Who Have Been Diagnosed With **Hemophilia** A	**Hemophilia** A	Drug: Damoctocog alfa pegol (Jivi, Bay94-9027)	South Alabama Medical Science Foundation Mobile, Alabama, United States Banner MD Anderson Cancer Center Phoenix, Arizona, United States University California Davis Davis, California, United States (and 34 more...)
6	☐	Recruiting	A Study to Evaluate Seroprevalence and Seroconversion of Antibodies to Adeno-Associated Virus (AAV) in Patients With **Hemophilia** A	**Hemophilia** A	Procedure: Blood sample collection	Covance Inc Madison, Wisconsin, United States
7	☐	Recruiting	Hemlibra in Mild **Hemophilia** A	Factor VIII Deficiency, Congenital	Drug: Emicizumab	Indiana Hemophila @Thrombosis Center Indianapolis, Indiana, United States
8	☐	Recruiting NEW	Outcomes of Prophylaxis With Emicizumab in Children With Severe **Hemophilia** A in Ivory Coast	**Hemophilia** A	Drug: Prophylaxis with Emicizumab	CHU de Yopougon Abidjan, Côte D'Ivoire
9	☐	Recruiting	Needs Assessment of Knowledge, Beliefs, and Attitudes of Patients With **Hemophilia** B About Gene Therapy	Gene Therapy **Hemophilia** B	Behavioral: Interview	St. Jude Children's Research Hospital Memphis, Tennessee, United States
10	☐	Recruiting	An Exploration of the Impact of Gene Therapy on the Lives of People With **Haemophilia** and Their Families	**Hemophilia**	Other: Qualitative interview	Oxford University Hospitals NHS Foundation Trust Oxford, Oxfordshire, United Kingdom

Showing: 1-10 of **153** studies 10 studies per page

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Community Voices in Research (CVR)

9 April 2022

Community Voices in Research

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What is CVR?

A **community powered registry** that uses **surveys** to gather the experiences of people with inherited bleeding disorders and their family members

Information is **confidential** and will always be reported in a **grouped** manner.

Why is it being done?

CVR will help researchers understand **what it means to live with a bleeding disorder** from the community member's perspective

Improve quality of life (**QOL**) and identify research questions important to the community.

Why should I participate?

By adding your voice and experiences, you can help **shape the future of research!**

You'll get **access to**:

- Personalized Dashboard
- Educational Resources
- Virtual Advisory Panels (opportunities for compensation)

Add your experiences by joining CVR today!

For more information, including how to enroll visit:
www.hemophilia.org/cvr or scan the QR code

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CVR Dashboard

National Hemophilia Foundation
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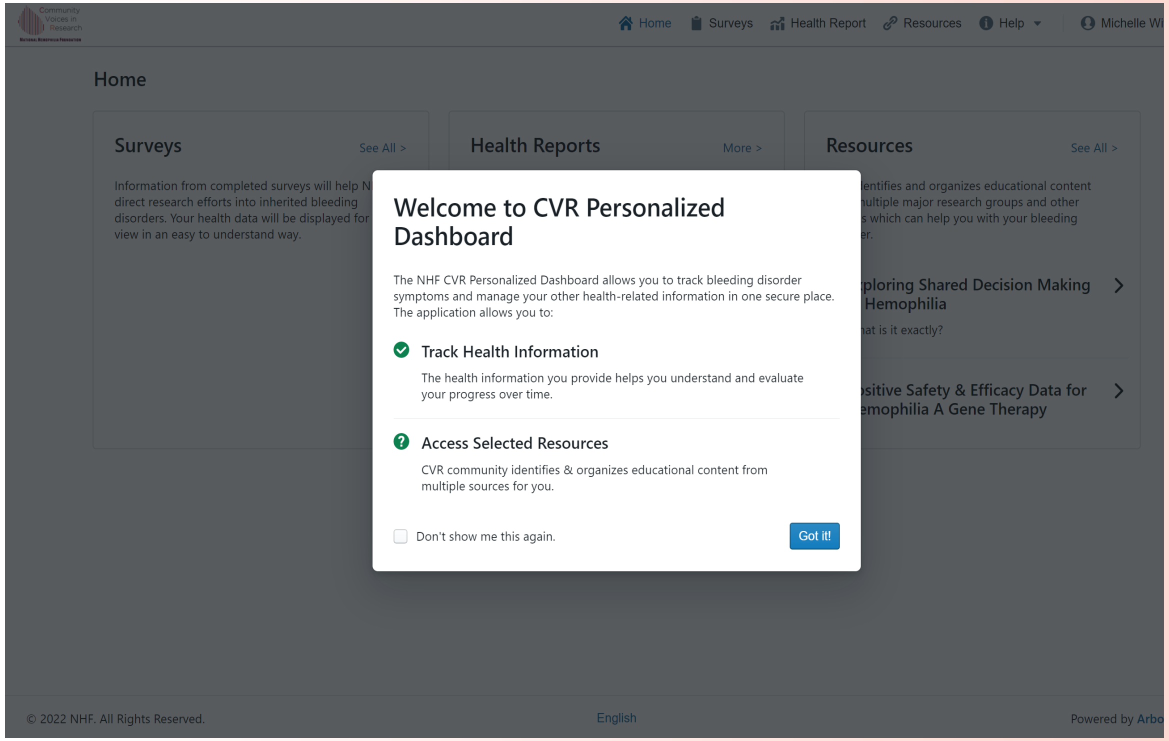
CVR Opening Screen



Home

Surveys

Information from completed surveys will help N
direct research efforts into inherited bleeding
disorders. Your health data will be displayed for
view in an easy to understand way.

Health Reports

Resources

lentifies and organizes educational content
multiple major research groups and other
s which can help you with your bleeding
er.

ploring Shared Decision Making
Hemophilia

at is it exactly?

sitive Safety & Efficacy Data for
emophilia A Gene Therapy

Welcome to CVR Personalized Dashboard

The NHF CVR Personalized Dashboard allows you to track bleeding disorder symptoms and manage your other health-related information in one secure place. The application allows you to:

- ✓ **Track Health Information**

  The health information you provide helps you understand and evaluate your progress over time.

- ? **Access Selected Resources**

  CVR community identifies & organizes educational content from multiple sources for you.

☐ Don't show me this again. [Got it!]

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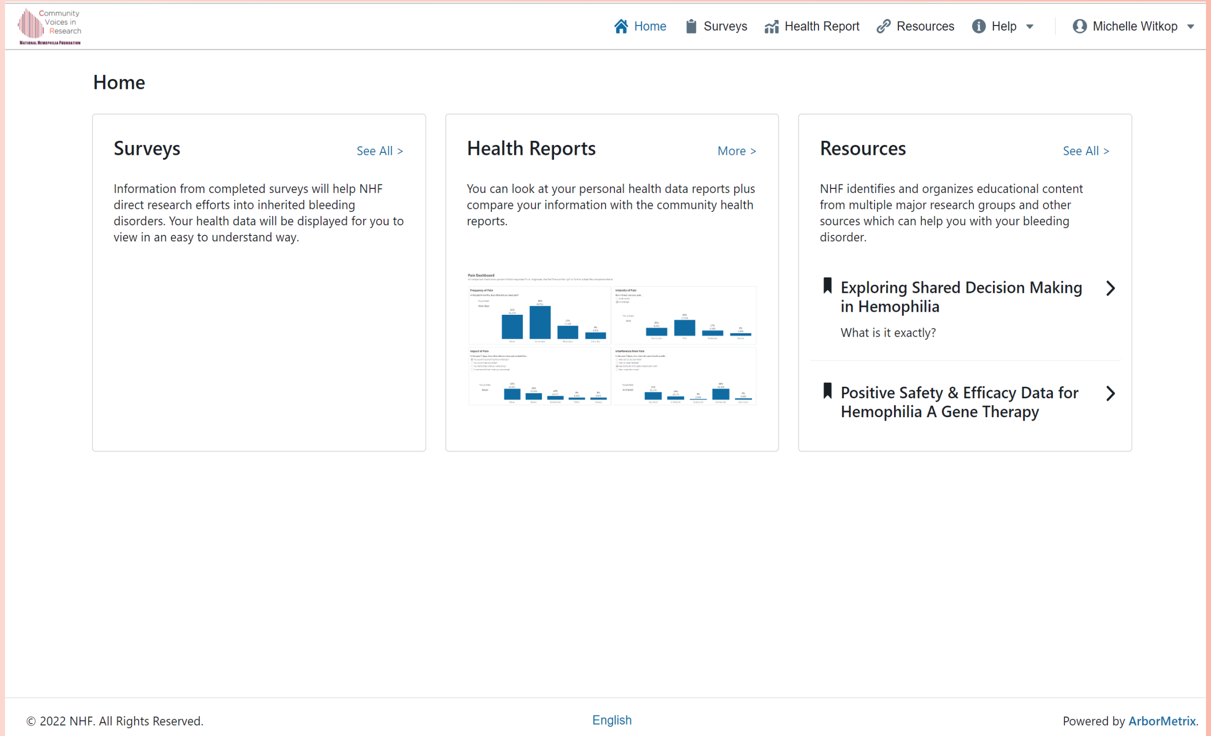
CVR Main Screen



Home

Surveys

Information from completed surveys will help NHF direct research efforts into inherited bleeding disorders. Your health data will be displayed for you to view in an easy to understand way.

Health Reports

You can look at your personal health data reports plus compare your information with the community health reports.

Resources

NHF identifies and organizes educational content from multiple major research groups and other sources which can help you with your bleeding disorder.

Exploring Shared Decision Making in Hemophilia

What is it exactly?

Positive Safety & Efficacy Data for Hemophilia A Gene Therapy

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CVR Pain Health Tile

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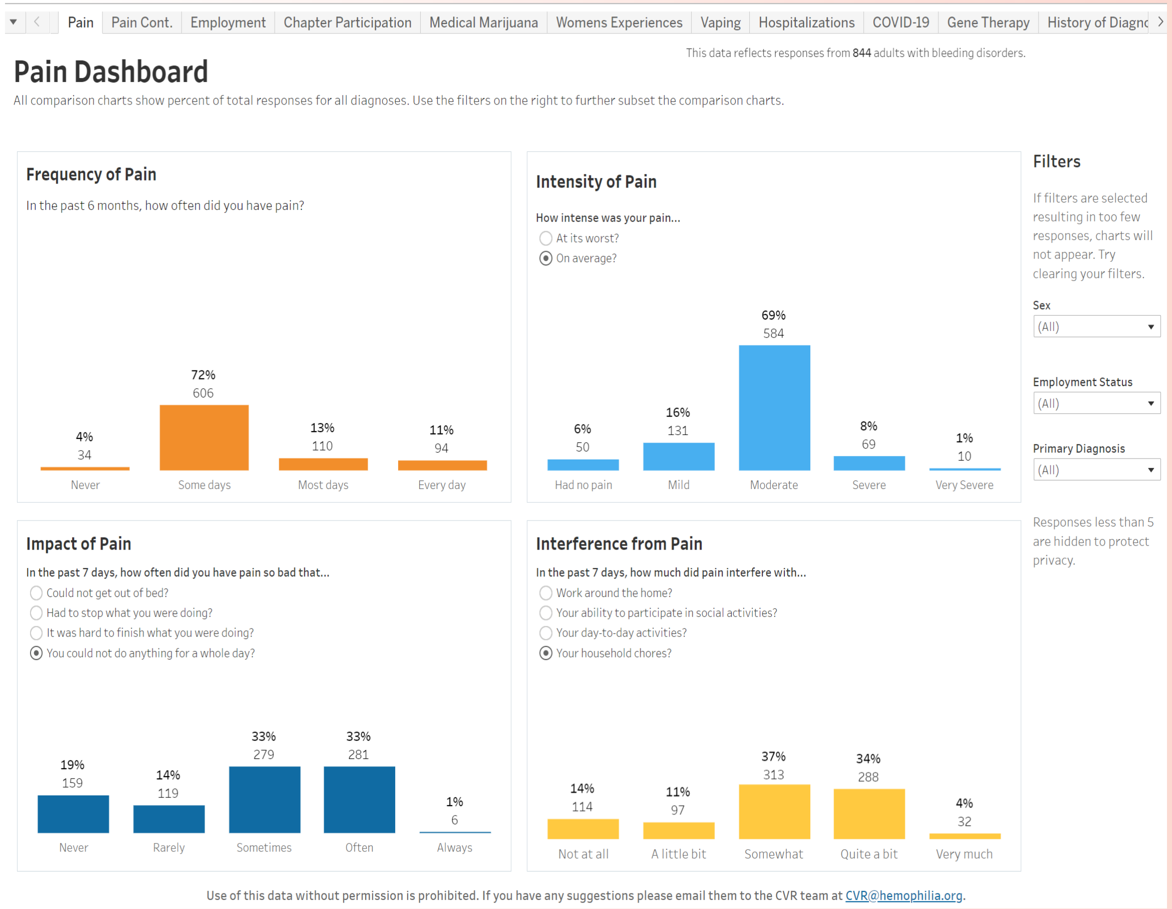


This data reflects responses from **844** adults with bleeding disorders.

Pain Dashboard

All comparison charts show percent of total responses for all diagnoses. Use the filters on the right to further subset the comparison charts.

Frequency of Pain

In the past 6 months, how often did you have pain?

Intensity of Pain

How intense was your pain...

○ At its worst?
● On average?

Filters

If filters are selected resulting in too few responses, charts will not appear. Try clearing your filters.

Sex: (All)

Employment Status: (All)

Primary Diagnosis: (All)

Responses less than 5 are hidden to protect privacy.

Impact of Pain

In the past 7 days, how often did you have pain so bad that...

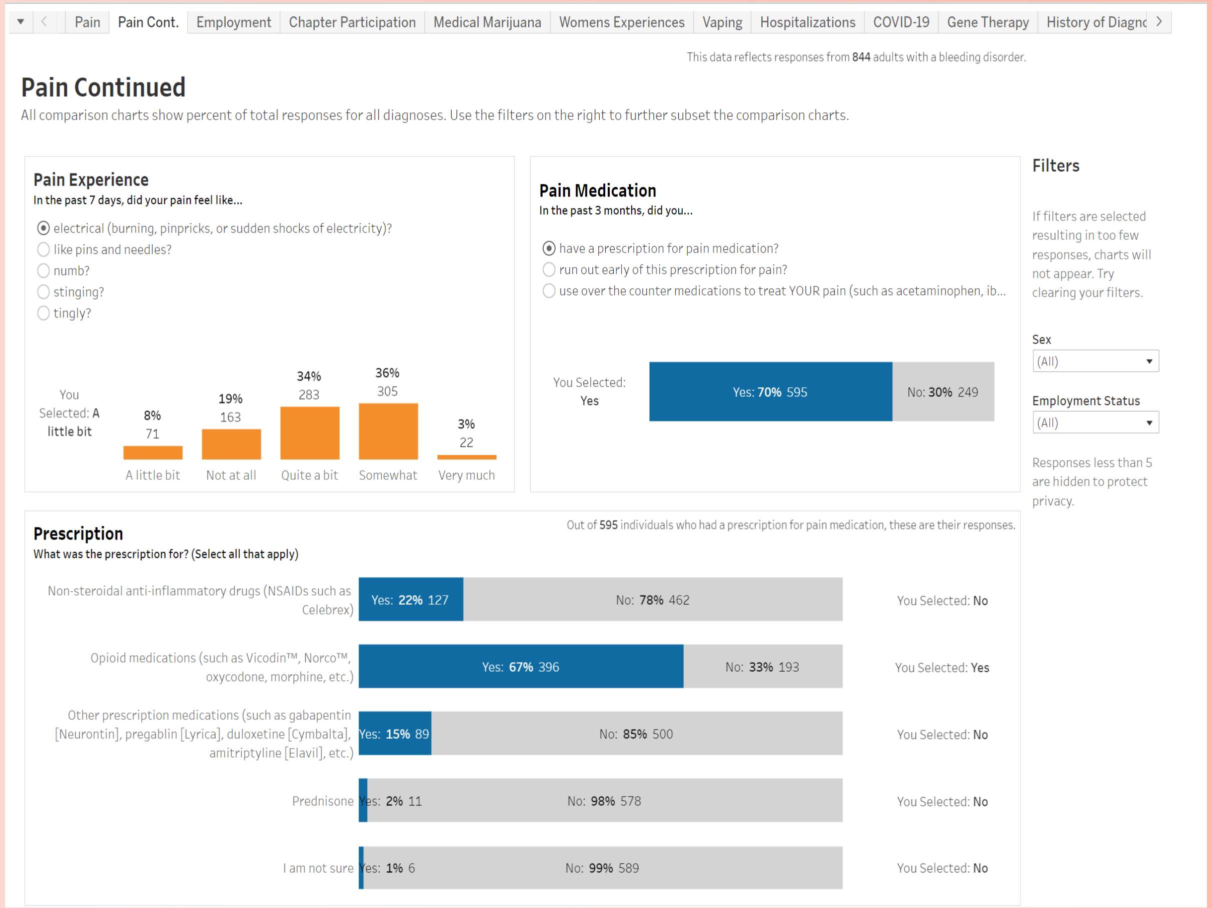
○ Could not get out of bed?
○ Had to stop what you were doing?
○ It was hard to finish what you were doing?
● You could not do anything for a whole day?

Interference from Pain

In the past 7 days, how much did pain interfere with...

○ Work around the home?
○ Your ability to participate in social activities?
○ Your day-to-day activities?
● Your household chores?

Use of this data without permission is prohibited. If you have any suggestions please email them to the CVR team at CVR@hemophilia.org.



This data reflects responses from **844** adults with a bleeding disorder.

Pain Continued

All comparison charts show percent of total responses for all diagnoses. Use the filters on the right to further subset the comparison charts.

Pain Experience

In the past 7 days, did your pain feel like...

● electrical (burning, pinpricks, or sudden shocks of electricity)?
○ like pins and needles?
○ numb?
○ stinging?
○ tingly?

You Selected: A little bit

Pain Medication

In the past 3 months, did you...

● have a prescription for pain medication?
○ run out early of this prescription for pain?
○ use over the counter medications to treat YOUR pain (such as acetaminophen, ib...

You Selected: Yes

Filters

If filters are selected resulting in too few responses, charts will not appear. Try clearing your filters.

Sex: (All)

Employment Status: (All)

Responses less than 5 are hidden to protect privacy.

Prescription

What was the prescription for? (Select all that apply)

Out of **595** individuals who had a prescription for pain medication, these are their responses.

	Yes	No	
Non-steroidal anti-inflammatory drugs (NSAIDs such as Celebrex)	Yes: 22% 127	No: 78% 462	You Selected: No
Opioid medications (such as Vicodin™, Norco™, oxycodone, morphine, etc.)	Yes: 67% 396	No: 33% 193	You Selected: Yes
Other prescription medications (such as gabapentin [Neurontin], pregablin [Lyrica], duloxetine [Cymbalta], amitriptyline [Elavil], etc.)	Yes: 15% 89	No: 85% 500	You Selected: No
Prednisone	Yes: 2% 11	No: 98% 578	You Selected: No
I am not sure	Yes: 1% 6	No: 99% 589	You Selected: No

CVR Employment Health Tile

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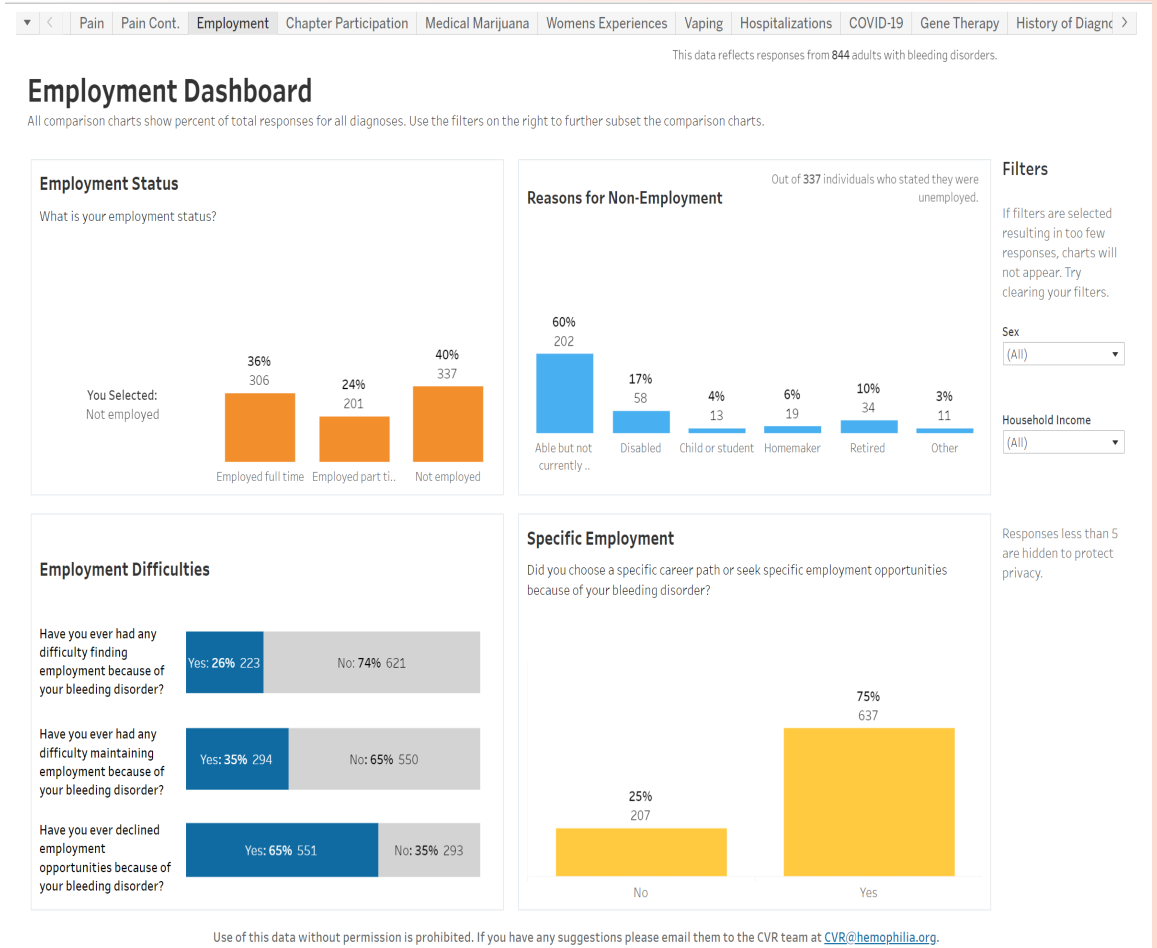

This data reflects responses from **844** adults with bleeding disorders.

Employment Dashboard

All comparison charts show percent of total responses for all diagnoses. Use the filters on the right to further subset the comparison charts.

Employment Status

What is your employment status?

You Selected:
Not employed

Reasons for Non-Employment

Out of **337** individuals who stated they were unemployed.

Filters

If filters are selected resulting in too few responses, charts will not appear. Try clearing your filters.

Sex: (All)

Household Income: (All)

Responses less than 5 are hidden to protect privacy.

Employment Difficulties

Have you ever had any difficulty finding employment because of your bleeding disorder?

Have you ever had any difficulty maintaining employment because of your bleeding disorder?

Have you ever declined employment opportunities because of your bleeding disorder?

Specific Employment

Did you choose a specific career path or seek specific employment opportunities because of your bleeding disorder?

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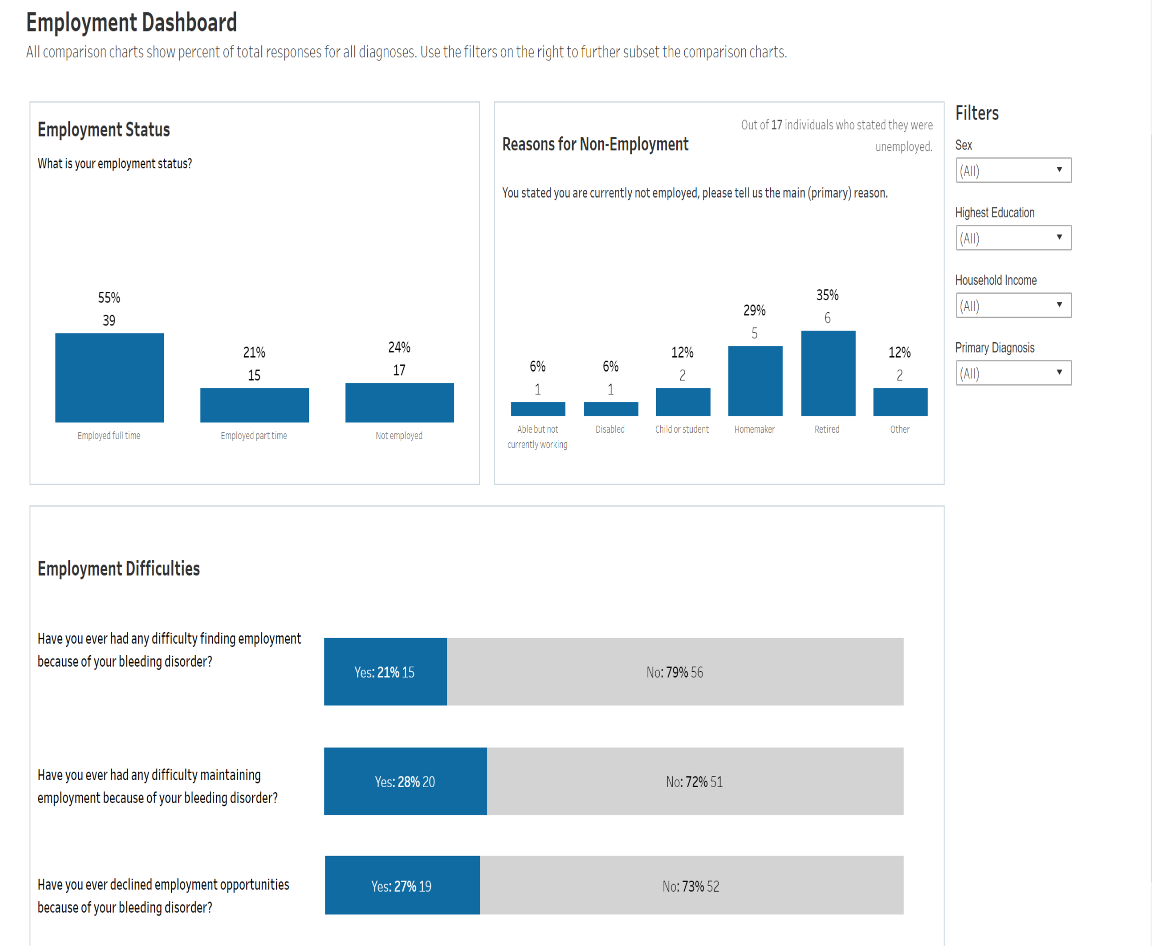

Employment Dashboard

All comparison charts show percent of total responses for all diagnoses. Use the filters on the right to further subset the comparison charts.

Employment Status

What is your employment status?

Reasons for Non-Employment

Out of **17** individuals who stated they were unemployed.

You stated you are currently not employed, please tell us the main (primary) reason.

Filters

Sex: (All)

Highest Education: (All)

Household Income: (All)

Primary Diagnosis: (All)

Employment Difficulties

Have you ever had any difficulty finding employment because of your bleeding disorder?

Have you ever had any difficulty maintaining employment because of your bleeding disorder?

Have you ever declined employment opportunities because of your bleeding disorder?

CVR Educational Resources

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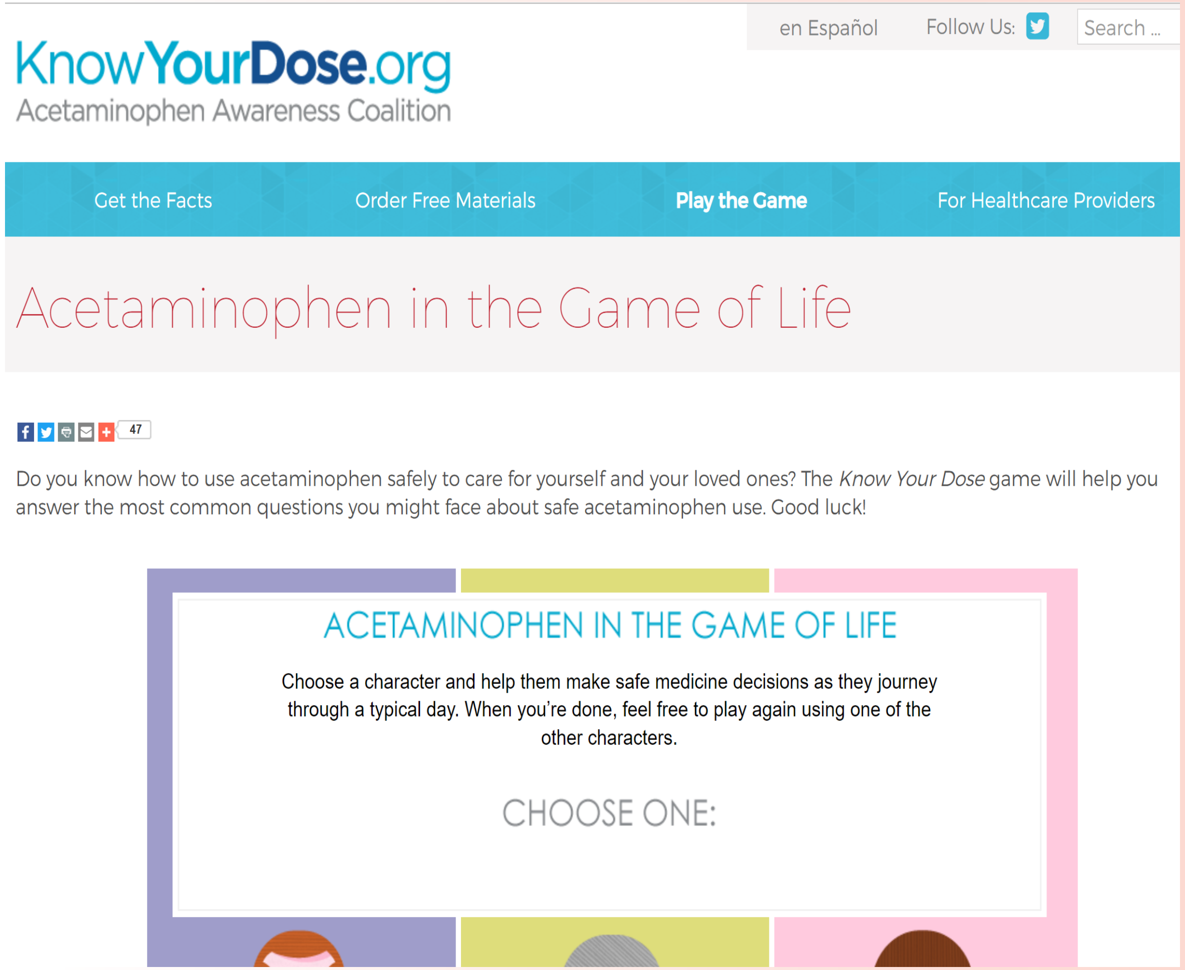
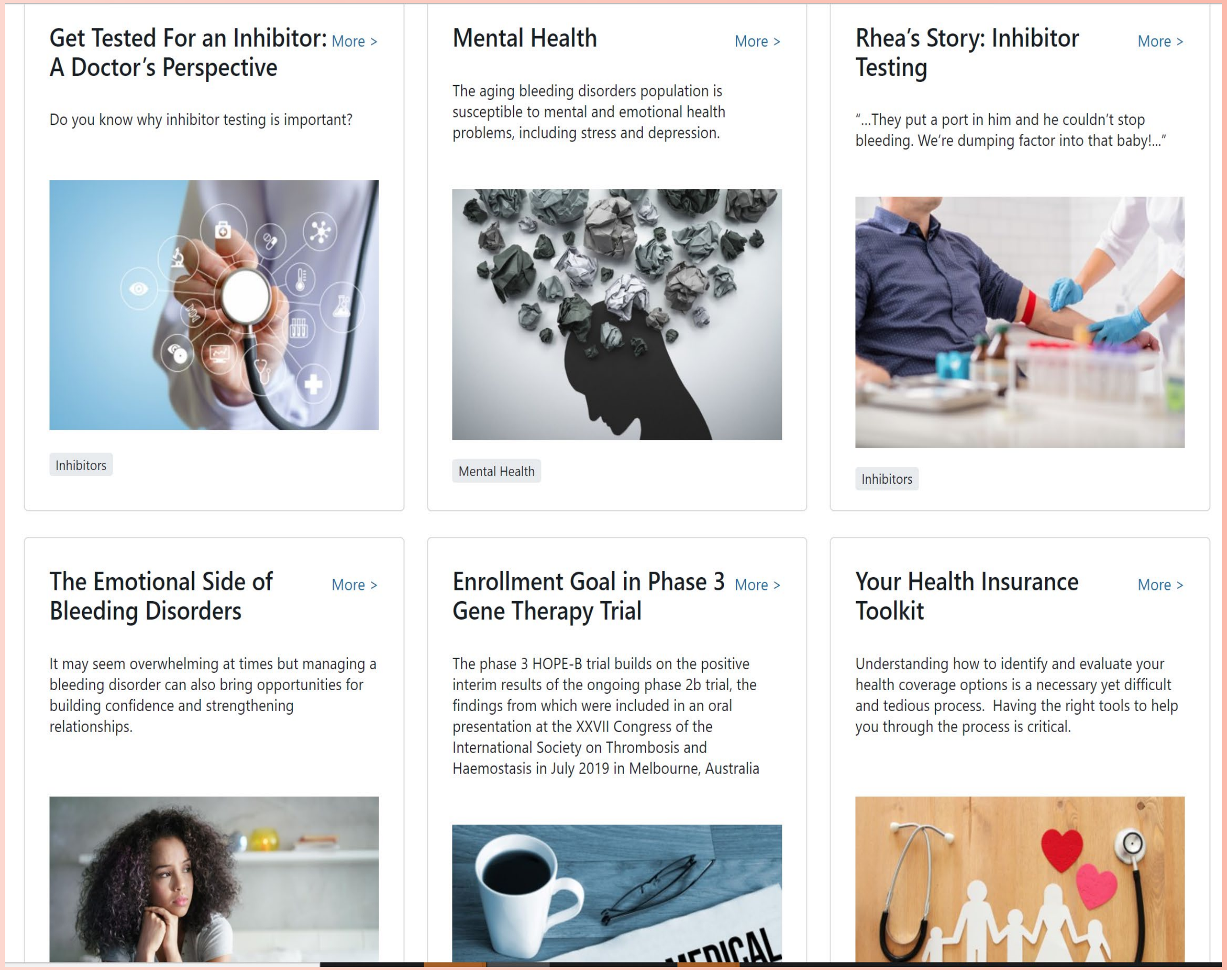


Acetaminophen in the Game of Life

Do you know how to use acetaminophen safely to care for yourself and your loved ones? The *Know Your Dose* game will help you answer the most common questions you might face about safe acetaminophen use. Good luck!

ACETAMINOPHEN IN THE GAME OF LIFE

Choose a character and help them make safe medicine decisions as they journey through a typical day. When you're done, feel free to play again using one of the other characters.



Get Tested For an Inhibitor: A Doctor's Perspective

Do you know why inhibitor testing is important?

Mental Health

The aging bleeding disorders population is susceptible to mental and emotional health problems, including stress and depression.

Rhea's Story: Inhibitor Testing

"...They put a port in him and he couldn't stop bleeding. We're dumping factor into that baby!..."

The Emotional Side of Bleeding Disorders

It may seem overwhelming at times but managing a bleeding disorder can also bring opportunities for building confidence and strengthening relationships.

Enrollment Goal in Phase 3 Gene Therapy Trial

The phase 3 HOPE-B trial builds on the positive interim results of the ongoing phase 2b trial, the findings from which were included in an oral presentation at the XXVII Congress of the International Society on Thrombosis and Haemostasis in July 2019 in Melbourne, Australia

Your Health Insurance Toolkit

Understanding how to identify and evaluate your health coverage options is a necessary yet difficult and tedious process. Having the right tools to help you through the process is critical.

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CDC Data Visualization Tool

9 April 2022

CDC Data Visualization Tool

https://communitycountsdataviz.cdc.gov/blooddisorders/#!/

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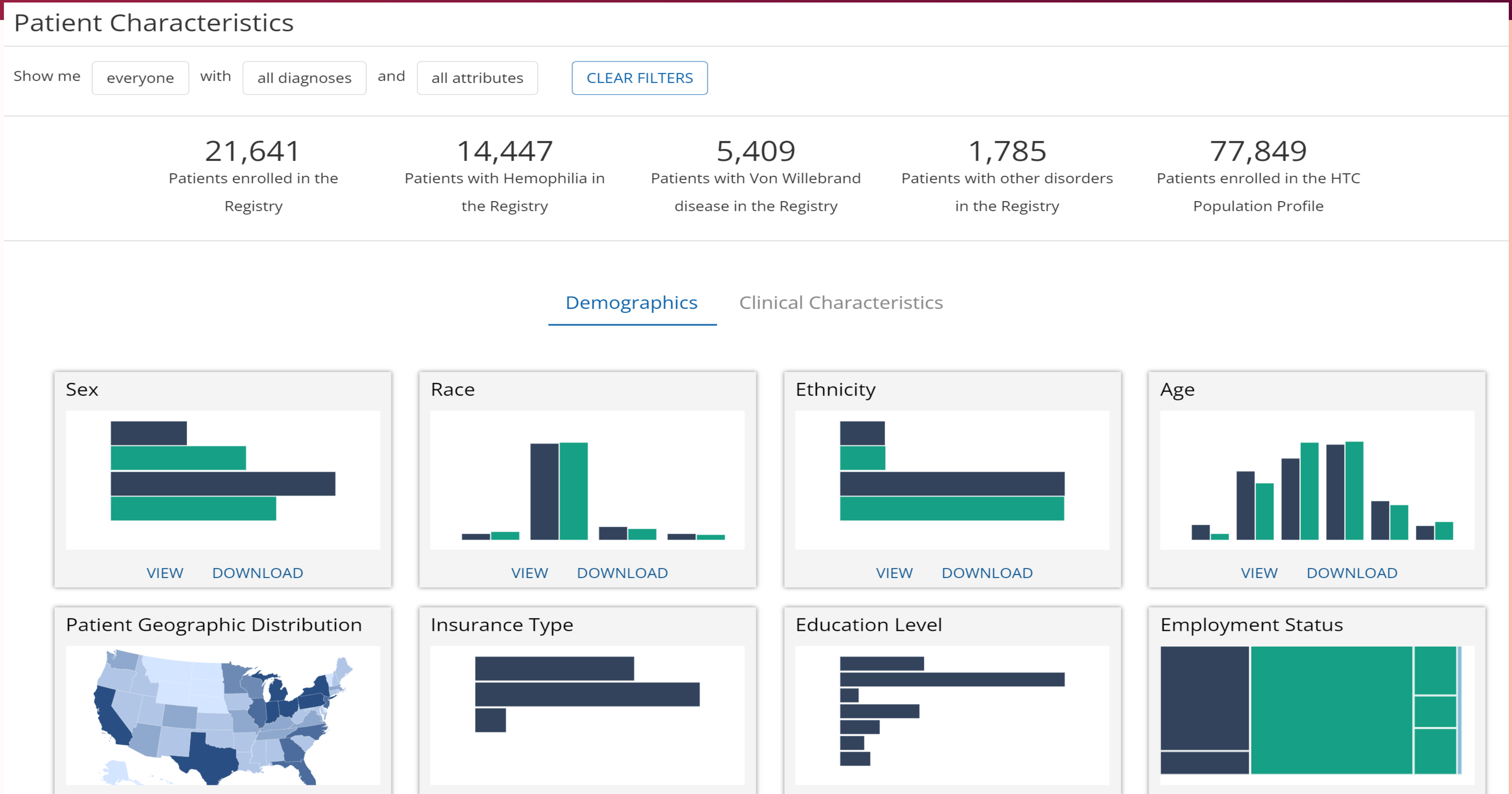

Patient Characteristics

Show me everyone with all diagnoses and all attributes CLEAR FILTERS

21,641	14,447	5,409	1,785	77,849
Patients enrolled in the Registry	Patients with Hemophilia in the Registry	Patients with Von Willebrand disease in the Registry	Patients with other disorders in the Registry	Patients enrolled in the HTC Population Profile

Demographics | Clinical Characteristics

CDC Data Visualization Tool

https://communitycountsdataviz.cdc.gov/blooddisorders/#!/

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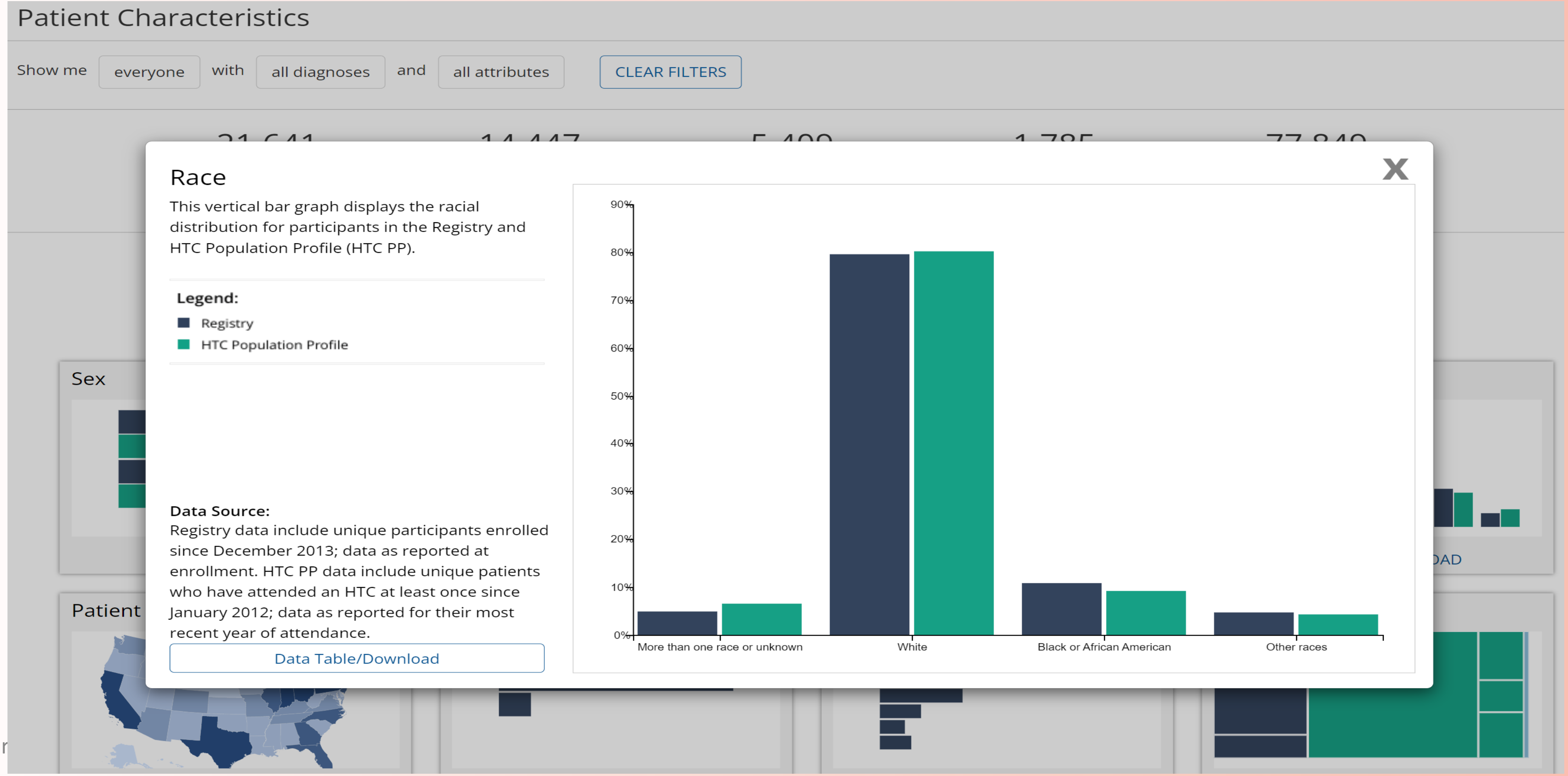

Patient Characteristics

Show me everyone with all diagnoses and all attributes CLEAR FILTERS

Race

This vertical bar graph displays the racial distribution for participants in the Registry and HTC Population Profile (HTC PP).

Legend:

- 🟦 Registry
- 🟩 HTC Population Profile

Data Source:
Registry data include unique participants enrolled since December 2013; data as reported at enrollment. HTC PP data include unique patients who have attended an HTC at least once since January 2012; data as reported for their most recent year of attendance.

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HFA Website

9 April 2022

HFA Community Based Research Network

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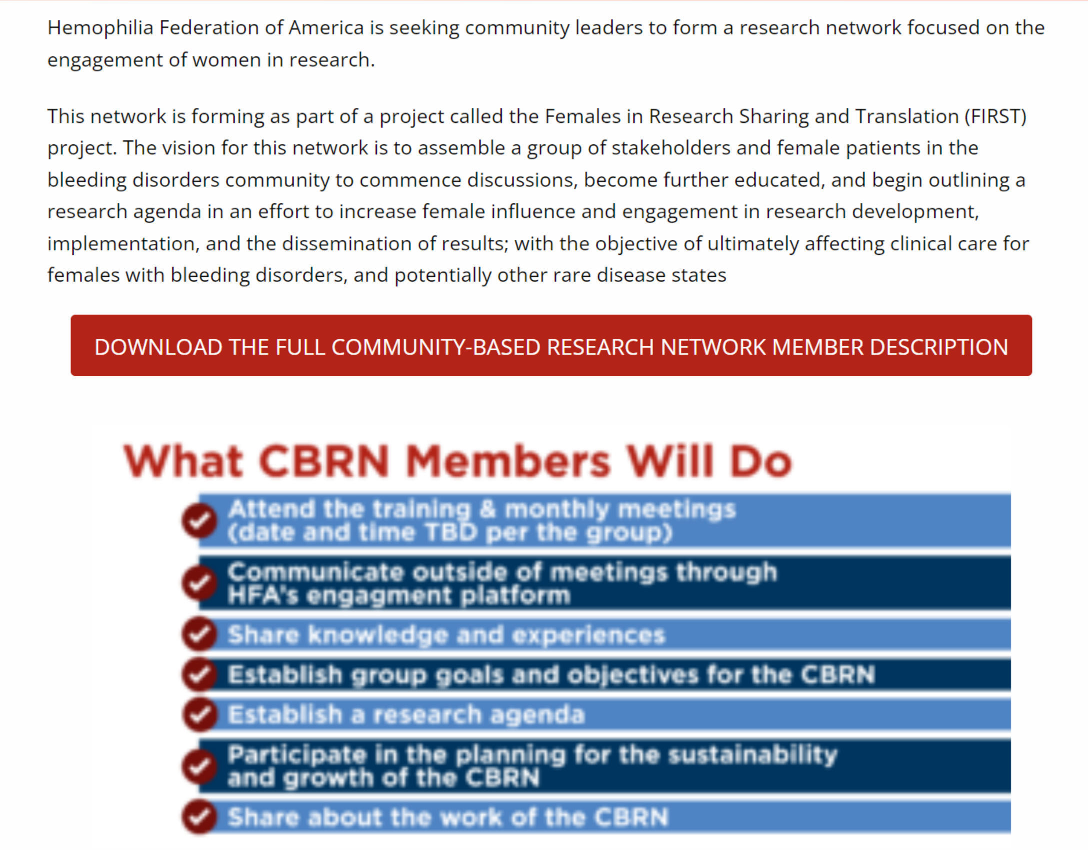
COMMUNITY BASED RESEARCH NETWORK (CBRN)

Hemophilia Federation of America is seeking community leaders to form a research network focused on the engagement of women in research.

This network is forming as part of a project called the Females in Research Sharing and Translation (FIRST) project. The vision for this network is to assemble a group of stakeholders and female patients in the bleeding disorders community to commence discussions, become further educated, and begin outlining a research agenda in an effort to increase female influence and engagement in research development, implementation, and the dissemination of results; with the objective of ultimately affecting clinical care for females with bleeding disorders, and potentially other rare disease states

DOWNLOAD THE FULL COMMUNITY-BASED RESEARCH NETWORK MEMBER DESCRIPTION



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DOWNLOAD THE FULL COMMUNITY-BASED RESEARCH NETWORK MEMBER DESCRIPTION

What CBRN Members Will Do

- ✓ Attend the training & monthly meetings (date and time TBD per the group)
- ✓ Communicate outside of meetings through HFA's engagment platform
- ✓ Share knowledge and experiences
- ✓ Establish group goals and objectives for the CBRN
- ✓ Establish a research agenda
- ✓ Participate in the planning for the sustainability and growth of the CBRN
- ✓ Share about the work of the CBRN

HFA FIRST & Wired

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GETTING WIRED WITH RESEARCH DURING A PANDEMIC

September 28, 2021

By Whitney Armijo, Research Project Coordinator

In the past two years, two Hemophilia Federation of America (HFA) projects have aimed to empower women in the bleeding disorders community to take part in research and advocate for their own health.

In 2019, the HFA research team created Females In Research Sharing and Translation (FIRST) to gather more details about how women in the bleeding disorders community feel about research, what prevents them from participating in research, and what researchers can do to encourage more women to participate in research. FIRST was funded by the Patient-Centered Outcomes Research Institute (PCORI).

FIRST included four in-person focus groups of women with bleeding disorders or symptoms of bleeding disorders. A fifth focus group was done over Zoom and included women from across the country. One of the themes observed in these conversations was that women do not participate in bleeding disorders research because they simply are not aware of any research opportunities that include women.

After the focus groups were conducted in late 2019 and early 2020, the next step in the FIRST project was to hold an in-person training to provide information about bleeding disorders and about patient-centered outcomes research to women. The aim was to empower women to engage in research in the future.

However, because the COVID-19 pandemic prevented any large gatherings in 2020, HFA applied for and received funding from PCORI to promote virtual engagement. This funding was used to create a virtual, web-based academy where women could safely engage with each other and receive education on bleeding disorder topics.

The academy—called Women In Research Engaged while Distanced (WIRED)—was an educational platform created by HFA and tailored for women with bleeding disorders. It ran from October 26 to November 24, 2020.

WIRED Academy

In designing WIRED, the research team used what we learned from the FIRST focus groups to encourage as many women as possible to attend. To facilitate that, the program was designed to be mostly self-paced, allowing women to engage whenever their busy schedules allowed. In addition, live meetings were scheduled on weekday evenings, monetary compensation was offered, and women with all forms of bleeding disorders were included, all as requested by women during the FIRST focus groups.

Through The Patient-Centered Outcomes Research Institute (PCORI) funded engagement project, the PRIDE (Patient-centered Research for Innovation, Development, and Education) project, HFA identified several underserved groups related to engagement in research, including females. Females are defined as: patients with a diagnosis or undiagnosed symptomatic carrier, and/or caregiver of someone with a bleeding disorder.

Based on the results of the PRIDE project, HFA developed the FIRST project, or Females in Research Sharing and Translation. The goal of this project was to help facilitate and engage females to become integral members of the research process. Using a strategy known as peer modeling, which educates and strengthens the relationships between stakeholders, HFA identified a cohort of females from which focus groups were created. The main focus of these discussions was to identify facilitators and barriers to participation in research by women with bleeding disorders.

As HFA had anticipated, the key findings from these discussions confirmed that there is a lack of inclusion of woman in any research related to bleeding disorders. Most women had either never been invited to participate in a clinical study, felt they weren't eligible for studies, that there weren't studies specifically for women with bleeding disorders or had conflicts around time constraints due to work and family obligations.

What can you use this information for?

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- Understand your disorder
- Understand where the community stands on different topics
- Being an informed consumer
- Shared decision-making with your provider
- Chapters – grant opportunities
- Share information with legislators during Legislative Days

Some of the Ways NHF Amplifies Your Voice

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- Gathering Voices - https://gather.video/sAqs
- Virtual Advisory Panels (VAPs)
- National Research Blueprint
- Community Voices in Research

NHF needs your help

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- NHF website redesign
- CDC Data Visualization Tool in collaboration with ATHN
- CVR Stakeholder Committee (after transition to new platform vendor)
- THSNA / NHF Partnership - https://gather.video/sAqs

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Think Different: SMEs as the Catalyst for Change

Esmeralda Vazquez & Maria Santaella
SME & NHF, Research Department

14 September 2021

What does being an SME mean to me

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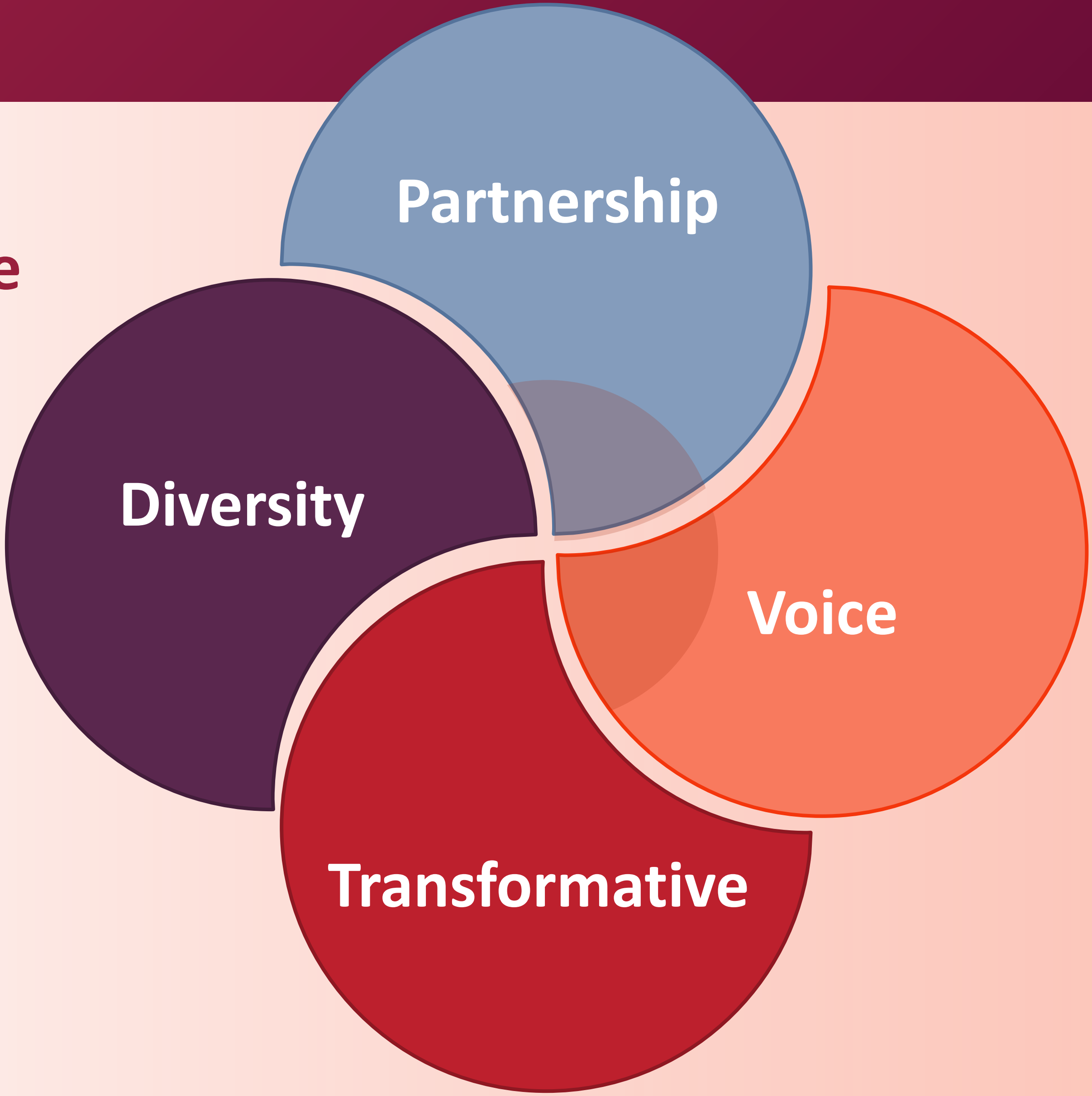
Our lived experience cannot be taught!


Partnership

Diversity

Voice

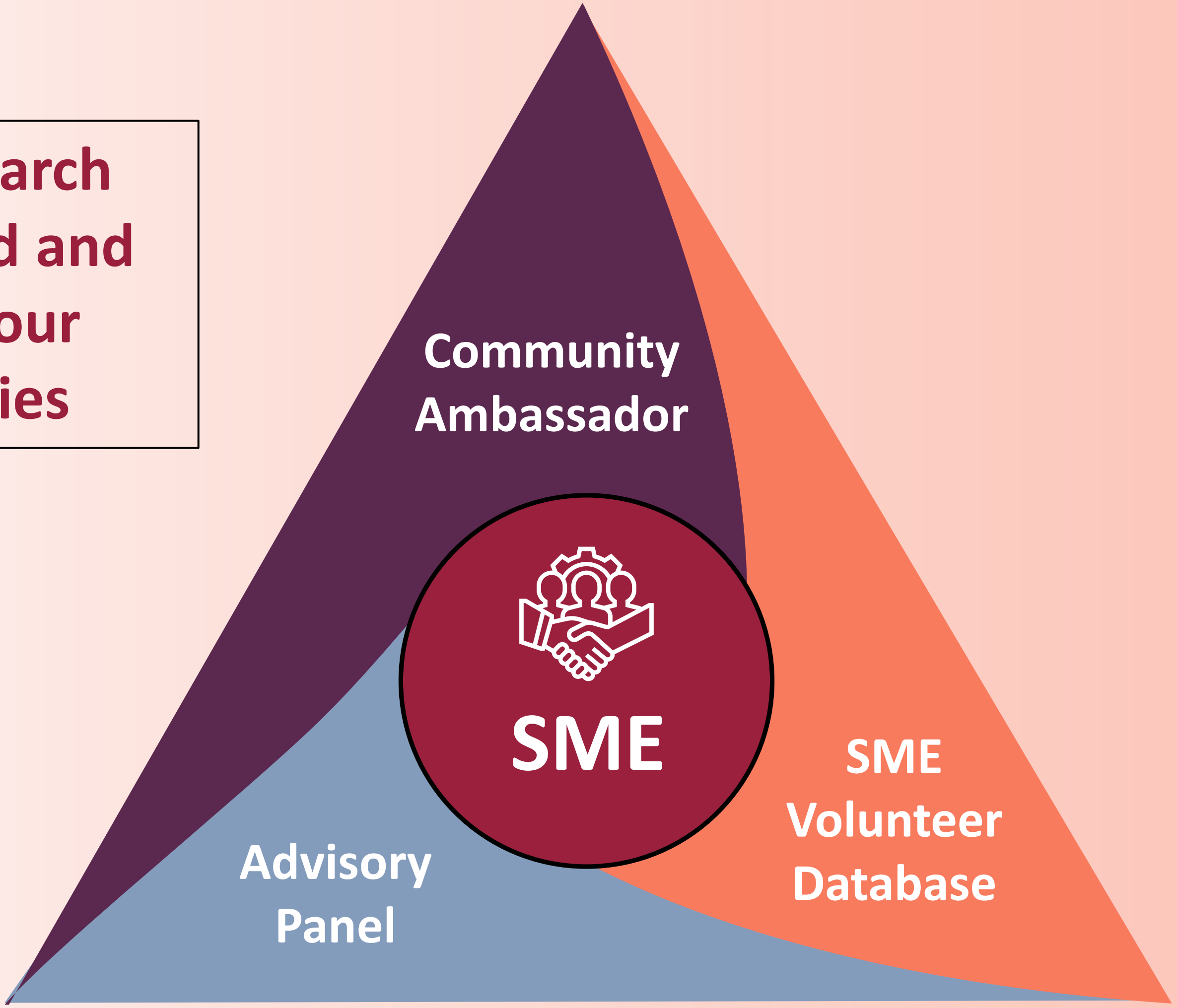
Transformative

Community Based Participatory Research in MY Community (CBPR)

Developing Community Partnerships

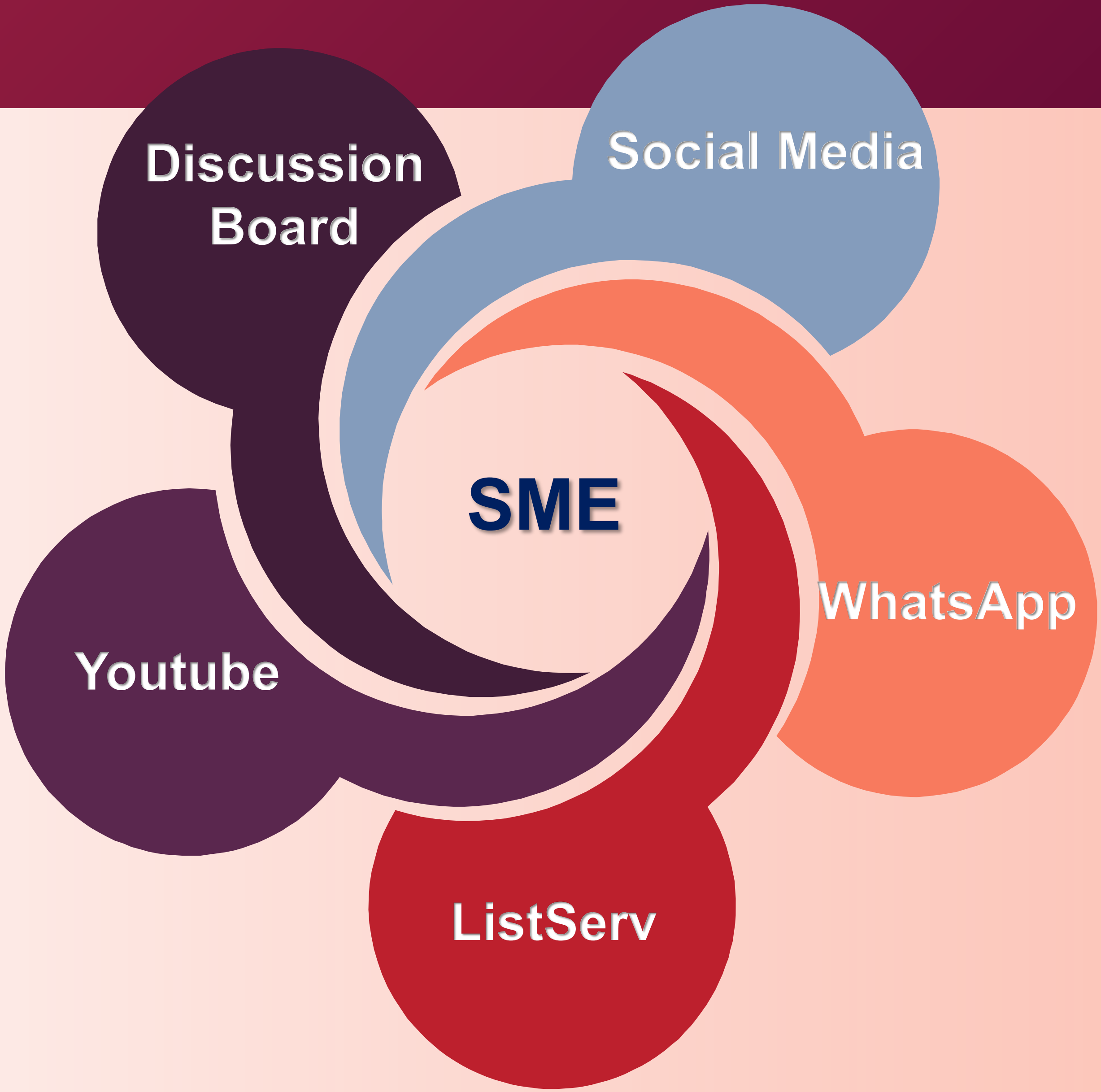
These are some of my ideas

Ensuring that research is patient-centered and representative of our diverse communities



Digital Community Engagement

A place to be heard!



What are your ideas?

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We would love to hear from you!

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Community Engagement: Can you hear me know?

Ilana Ostrin & Brett Spitale

NHF, Communications Department

Welcome!

Brett Spitale

VP, Advancement

NHF

Ilana Ostrin

Senior Director, PR & Communication

NHF

Before we begin …

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Follow NHF on social media!

@nhf_hemophilia

@nhf_hemophilia

Youtube.com/NHFVideo

National Hemophilia Foundation

Community Engagement 101

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Community informed

=

Community as advisors

Community involvement

=

Community as collaborators

Community direction

=

Community as vocal leaders

GREATER COMMUNITY ENGAGEMENT

Community Engagement 101

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for all bleeding disorders

Community informed

=

Community involvement

=

Community direction

=

Your involvement is key!!!

Recruitment & Engagement

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You can help!

Where your audience engages (online vs. in-person, etc.)

Research goals

Audience (age, demographics, disease state, location, etc.)

Budget & capabilities (traditional outreach vs. digital)

Recruitment & Engagement (Cont'd)

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You might find studies that are right for you via these ways

Digital

- Web advertising
- Email newsletters, etc.

Social

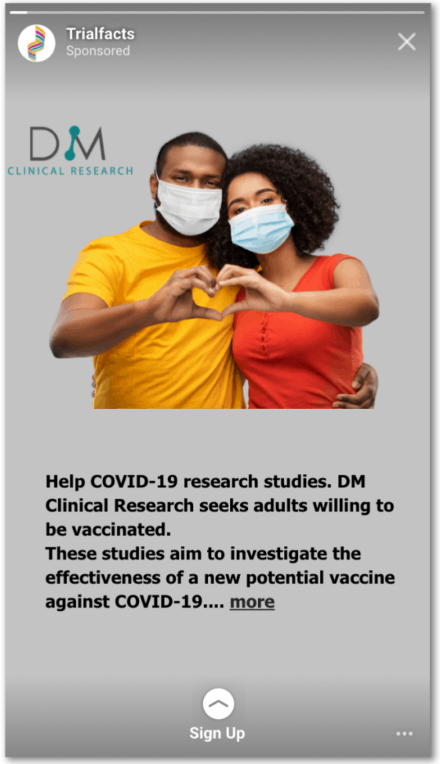
- Paid social media advertising (boosted, influencer, etc.)
- Organic social

Traditional

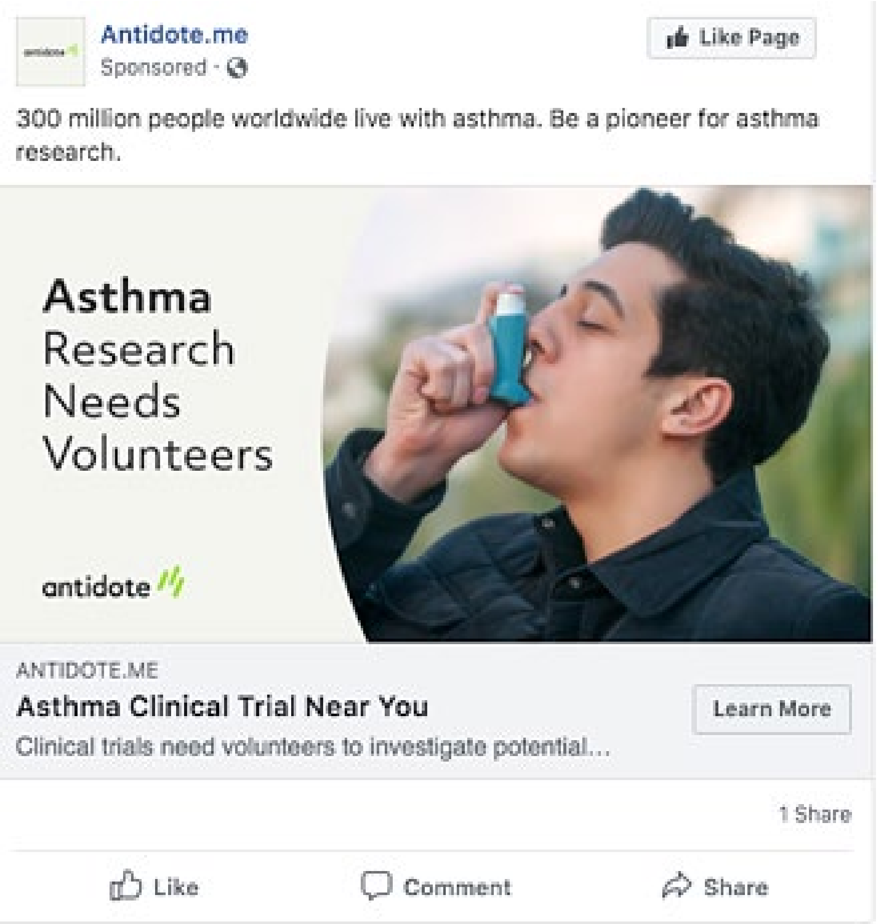
- In-person events (community health fairs, etc.)
- Print materials (direct mail, brochures, etc.)
- Radio/TV/print publication advertising

Social Media as a Recruitment Tool

Recruitment & Engagement (Cont'd)



Help COVID-19 research studies. DM Clinical Research seeks adults willing to be vaccinated.
These studies aim to investigate the effectiveness of a new potential vaccine against COVID-19.... more



300 million people worldwide live with asthma. Be a pioneer for asthma research.

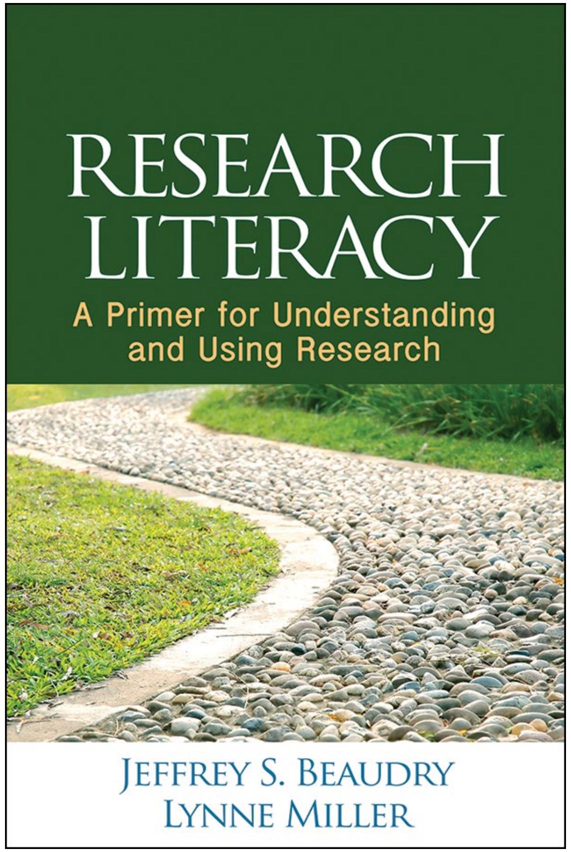
ANTIDOTE.ME
Asthma Clinical Trial Near You
Clinical trials need volunteers to investigate potential...

Be an active member of the Parkinson's community without having to leave home.

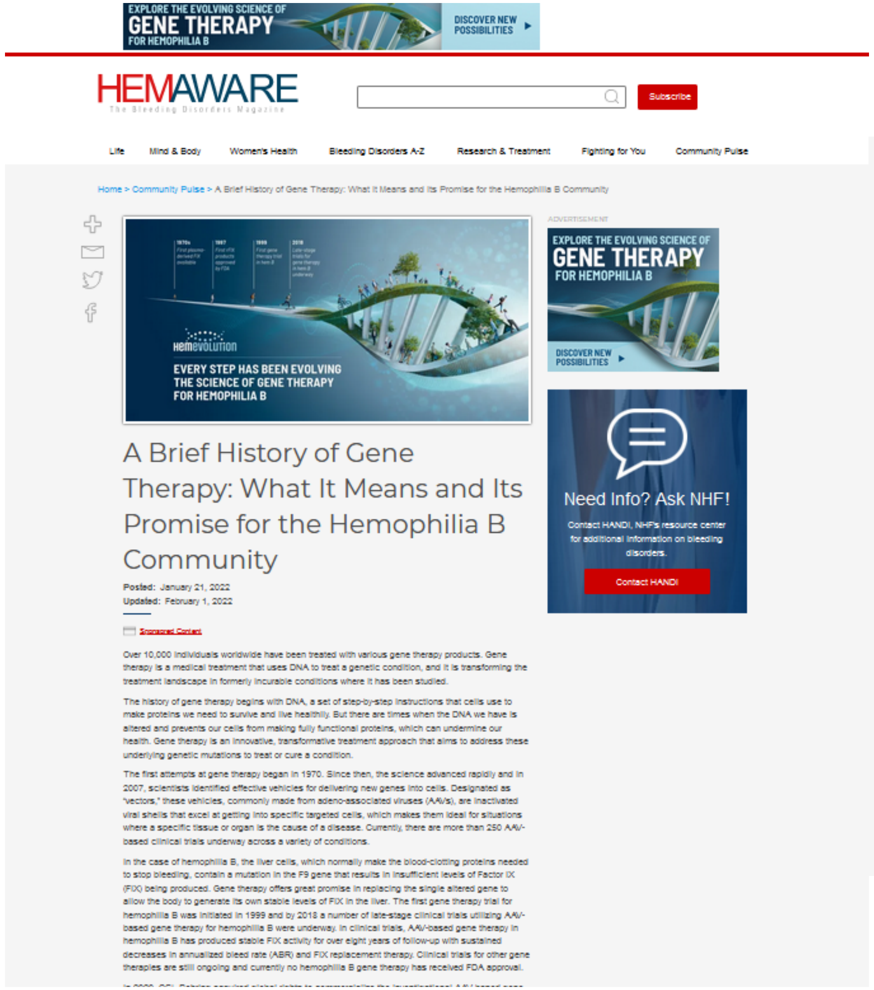
Participate in Fox Insight
Fox Insight is an online clinical study composed of questionnaires you can complete from your computer, tablet or smartphone four times a year to help advance research.
FOXINSIGHT.MICHAELJFOX.ORG

What is Research Literacy?

The capacity to obtain, process and understand basic information needed to make informed decisions about research participation


RESEARCH LITERACY
A Primer for Understanding and Using Research
JEFFREY S. BEAUDRY
LYNNE MILLER

HemAware as a Resource



A Brief History of Gene Therapy: What It Means and Its Promise for the Hemophilia B Community

Posted: January 21, 2022
Updated: February 1, 2022

Over 10,000 individuals worldwide have been treated with various gene therapy products. Gene therapy is a medical treatment that uses DNA to treat a genetic condition, and it is transforming the treatment landscape in formerly incurable conditions where it has been studied.

The history of gene therapy begins with DNA, a set of step-by-step instructions that cells use to make proteins we need to survive and live healthily. But there are times when the DNA we have is altered and prevents our cells from making fully functional proteins, which can undermine our health. Gene therapy is an innovative, transformative treatment approach that aims to address these underlying genetic mutations to treat or cure a condition.

The first attempts at gene therapy began in 1970. Since then, the science advanced rapidly and in 2007, scientists identified effective vehicles for delivering new genes into cells. Designated as "vectors," these vehicles, commonly made from adeno-associated viruses (AAVs), are inactivated viral shells that excel at getting into specific targeted cells, which makes them ideal for situations where a specific tissue or organ is the cause of a disease. Currently, there are more than 250 AAV-based clinical trials underway across a variety of conditions.

In the case of hemophilia B, the liver cells, which normally make the blood-clotting proteins needed to stop bleeding, contain a mutation in the F9 gene that results in insufficient levels of Factor IX (FIX) being produced. Gene therapy offers great promise in replacing the single altered gene to allow the body to generate its own stable levels of FIX in the liver. The first gene therapy trial for hemophilia B was initiated in 1999 and by 2018 a number of late-stage clinical trials utilizing AAV-based gene therapy for hemophilia B were underway. In clinical trials, AAV-based gene therapy in hemophilia B has produced stable FIX activity for over eight years of follow-up with sustained decreases in annualized bleed rate (ABR) and FIX replacement therapy. Clinical trials for other gene therapies are still ongoing and currently no hemophilia B gene therapy has received FDA approval.

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In 2020, CSL Behring acquired global rights to commercialize the investigational AAV-based gene therapy etranacogene dezaparvovoec, also known as EtranaDez, which is in development in the ongoing, pivotal HOPE-B clinical study. The HOPE-B phase 3 pivotal trial enrolled 54 subjects with severe or moderately severe hemophilia B.

To learn more about the evolution of gene therapy for hemophilia B, please visit www.HemEvolution.com.

Demystifying Clinical Trials

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"I'm not sure a clinical trial is right for me."	"I had a bad experience with a clinical trial before."	"I don't have the time or money to be in a trial."	"My privacy would be at risk in a clinical trial."
Consult with your medical team and loved ones before committing to a trial. However, your participation can help better outcomes for others with similar conditions. Plus, you'll receive special care from health care professionals and receive access to cutting edge treatments before the general public.	So sorry to hear you had a negative experience. Each study is different and there is always the option to report any issues to the review board or the compliance officer.	Most studies compensate your time and travel! Organizations, charities, or foundations can also help offset any needs.	When you are involved in a trial, your data is anonymized when shared with relevant parties. Remember that your identity will be protected and that your involvement will help create positive outcomes for other patients and families.

Your Participation is Needed

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“In a 2020 analysis of the global participation in clinical trials, the FDA highlighted the vast difference between the enrolled participants and the global population. Of 292,537 participants in clinical trials globally, **76% were white, 11% were Asian and only 7% were Black**.”

Sharma, A., Palaniappan, L. Improving diversity in medical research. Nat Rev Dis Primers ***7,*** *74 (2021). https://doi.org/10.1038/s41572-021-00316-8*

Become a Partner in Research

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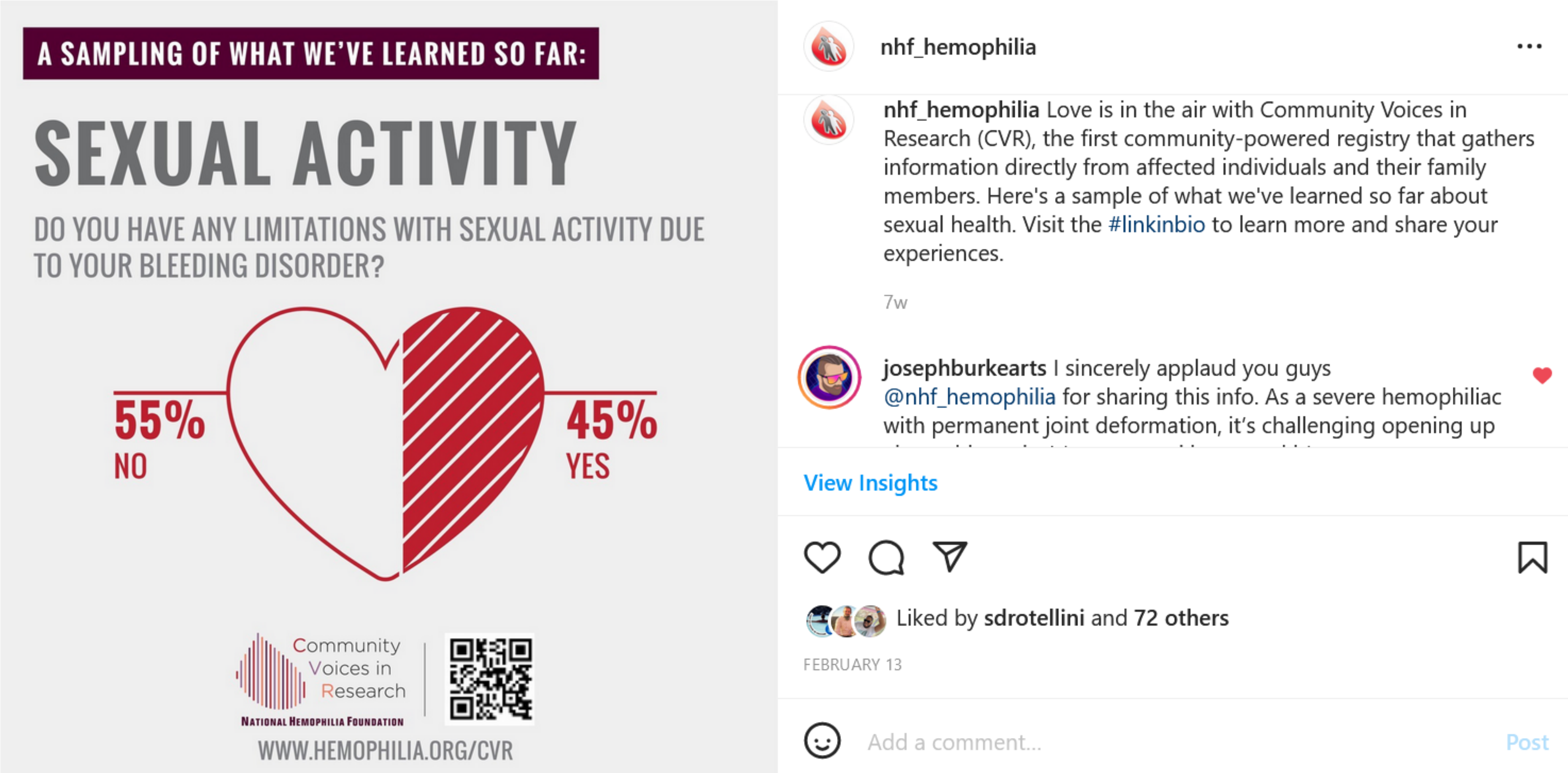

A SAMPLING OF WHAT WE'VE LEARNED SO FAR:

SEXUAL ACTIVITY

DO YOU HAVE ANY LIMITATIONS WITH SEXUAL ACTIVITY DUE TO YOUR BLEEDING DISORDER?

WWW.HEMOPHILIA.ORG/CVR

nhf_hemophilia

nhf_hemophilia Love is in the air with Community Voices in Research (CVR), the first community-powered registry that gathers information directly from affected individuals and their family members. Here's a sample of what we've learned so far about sexual health. Visit the #linkinbio to learn more and share your experiences.

7w

josephburkearts I sincerely applaud you guys @nhf_hemophilia for sharing this info. As a severe hemophiliac with permanent joint deformation, it's challenging opening up

View Insights

Liked by **sdrotellini** and **72 others**

FEBRUARY 13

Add a comment... Post

Become a Partner in Research (Cont’d)

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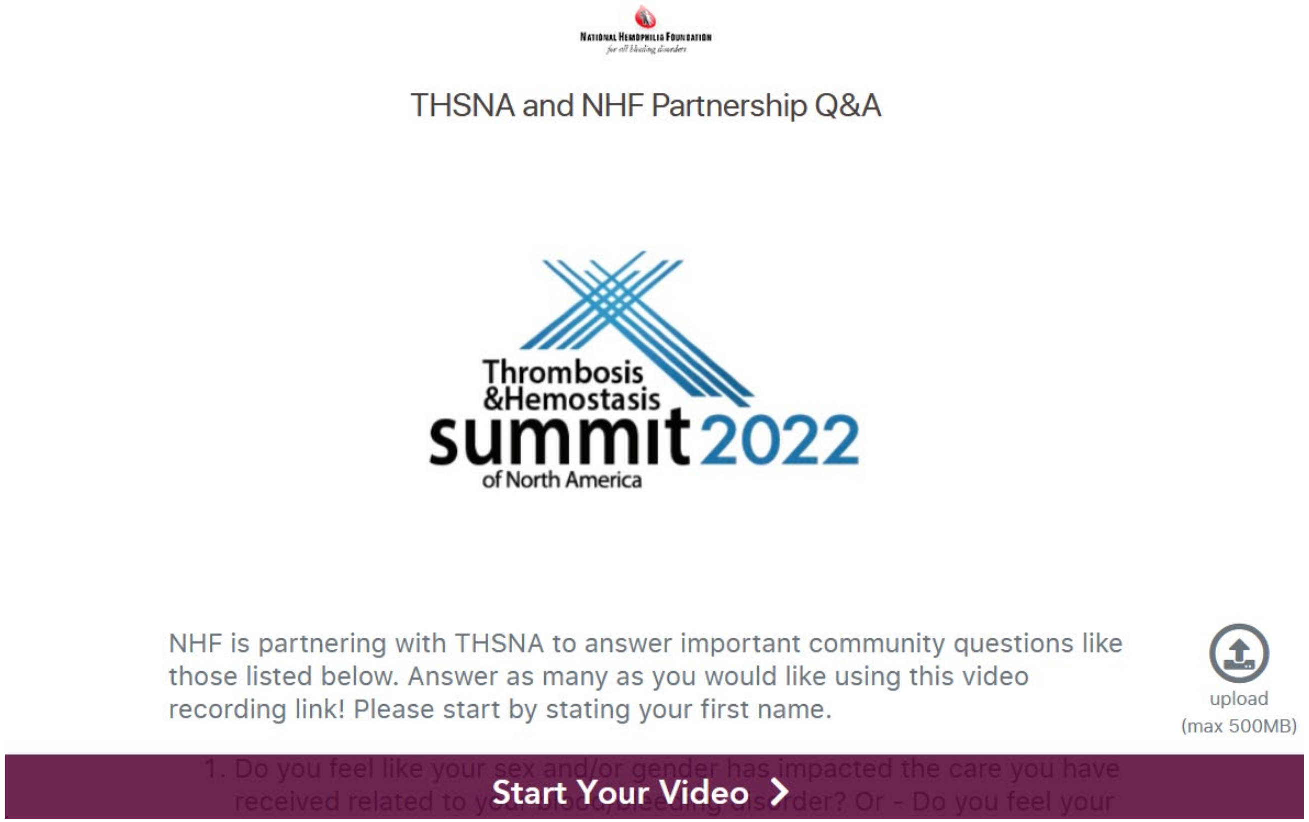
https://gather.video/sAqs



THSNA and NHF Partnership Q&A

NHF is partnering with THSNA to answer important community questions like those listed below. Answer as many as you would like using this video recording link! Please start by stating your first name.

1. Do you feel like your sex and/or gender has impacted the care you have received related to your blood/bleeding disorder? Or - Do you feel your

1. Do you feel like your sex and/or gender has impacted the care you have received related to your blood/bleeding disorder? Or - Do you feel your blood/bleeding disorder care has been impacted by your sex and/or gender.
2. Have you ever suffered from depression because of your blood/bleeding disorder?
3. Are you aware of the new recommendations for care of those with VWD?
4. Does your lab or workplace have active equity and diversity efforts?
5. What would make your care better?
6. Has a medical professional -- or even a friend or family member -- ever told you that pain was just in your head?
7. What BD do you have? How long did it take your rare bleeding disorder to get diagnosed?
8. If gene therapy were approved this year and you were eligible to receive it, would you a) receive gene therapy right away b) wait until some time has passed to see how others do with it outside clinical trials or c) never consider having it

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Summary & Call to Action

Kevin Mills
NHF, Research Department

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National Research Blueprint

Please scan this QR code to provide additional comments

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THANK YOU!

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An Extraordinary Meeting

Nothing About Us Without Us
Building the National Research Blueprint

April 7-9, 2022 - Washington D.C.

VISION

National Hemophilia Foundation
for all bleeding disorders

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

La Fundación Nacional de 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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What did we hear?

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- Inclusion → Bringing the community to research; bringing research to the community
- Communication → Listening becomes hearing become understanding
- Collaboration → Pooling our talents; pulling together
- Participation → Every grain of sand builds the sandcastle

The Blueprint: Putting the Puzzle Together

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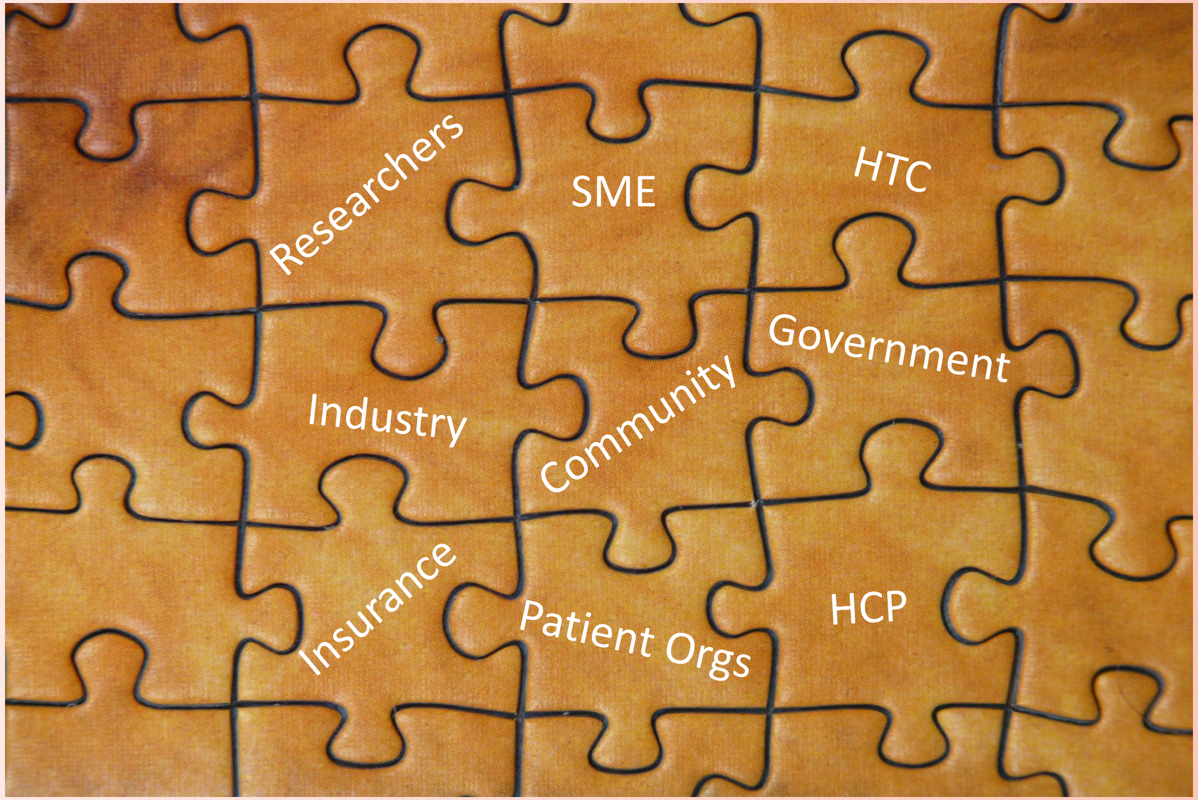
The Blueprint: Putting the Puzzle Together



Our Collective Call to Action

Get Involved! – There's No Time Like Now To Get in The Mix

Make Your Voice Heard! – Don't Just Sing in the Shower

Lead From the Front! – Be the Bold Bird

We Will Reach Our Goal Together

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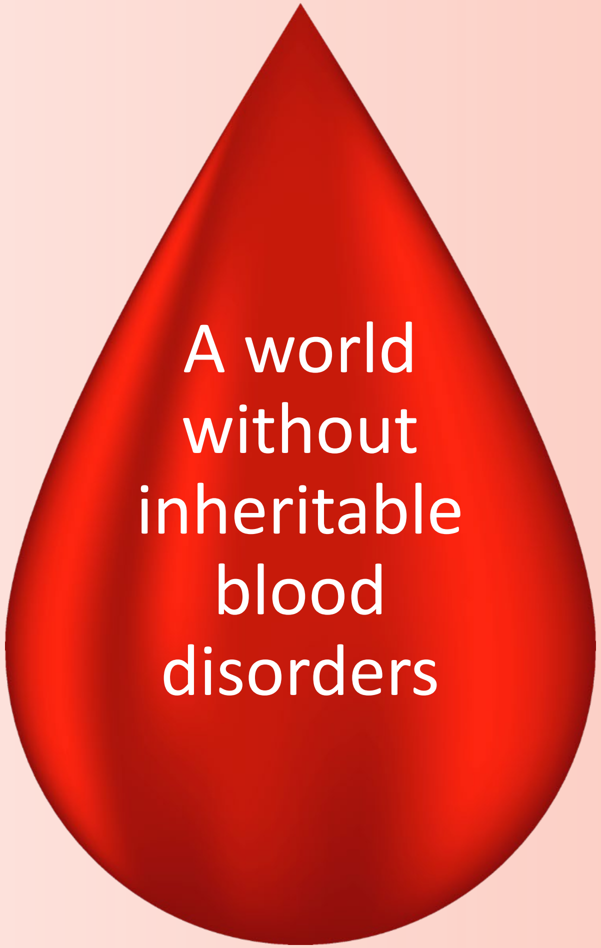

A world without inheritable blood disorders

THANK YOU

Michelle Witkop

Donna DiMichele

Maria E. Santaella

- Samantha Carlson
- Allison Hartless
- Felix Olaya

- Panelists
- Working Group Chair

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