WELCOME BACK!

April 8-9
2022

Washington, D.C.
Today’s Agenda

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
<th>Speaker(s)</th>
</tr>
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<tbody>
<tr>
<td>7:00 - 8:30 AM</td>
<td>Breakfast (Salons D, E)</td>
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</tr>
<tr>
<td>8:30 - 9:30 AM</td>
<td>NHF, Research, and YOU!</td>
<td>Michelle Witkop</td>
</tr>
<tr>
<td>9:30 - 10:30 AM</td>
<td>Think Different: SMEs as the Catalyst for Change</td>
<td>Maria Santaella &amp; Esmeralda Vazquez</td>
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<tr>
<td>10:30 - 11:00 AM</td>
<td>Break &amp; Check-out</td>
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<tr>
<td>11:00 - 12:00 PM</td>
<td>Community Engagement: Can You Hear Me Now?</td>
<td>Ilana Ostrin &amp; Brett Spitale</td>
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<tr>
<td>12:00 - 12:30 PM</td>
<td>Summary of Event &amp; Call to Action</td>
<td>Kevin Mills</td>
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<tr>
<td>12:30 PM</td>
<td>Lunch at the hotel (Salons D, E) and Departures</td>
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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.
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
- $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

**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

**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

**NHF-Takeda Clinical Fellowship**
- $100,000/year for 2 years
- Provides physicians hands-on clinical and research experience at highly regarded HTCs

**Excellence Fellowships**
- Nursing
- Social Work
- Physical Therapy
- Multidisciplinary advances in training, career development, and clinical care

$15,000 once a year
Evolution of NHF Research Funding

- **1972**: NHF Research Dept. Established
- **2000**: Social Work Excellence 15 awarded $155,000
- **2006**: JGP 106 awarded $9,124,000
- **2008**: Physical Therapy Excellence 10 awarded $105,000
- **2011**: IIR 6 awarded $360,000
- **2017**: Bridge Award 2 awarded $250,000
- **2018**: JML 2 awarded $312,000
- **2021**: 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
Clinical Trial Finder

www.Clinicaltrials.gov
### Clinical Trial Finder Eligibility

#### Study Title

<table>
<thead>
<tr>
<th>Row</th>
<th>Status</th>
<th>Study Title</th>
<th>Conditions</th>
<th>Locations</th>
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<tbody>
<tr>
<td>5</td>
<td>Recruiting</td>
<td>Evaluating Effectiveness and Long Term Safety of Damoctocog Alfa Pegol in Patients, Who Have Been Diagnosed With Hemophilia A</td>
<td>Hemophilia A</td>
<td>Drug: Damoctocog alfa pegol (Jivi, Bay94-9027)</td>
</tr>
<tr>
<td>6</td>
<td>Recruiting</td>
<td>A Study to Evaluate Seroprevalence and Seroconversion of Antibodies to Adeno-Associated Virus (AAV) in Patients With Hemophilia A</td>
<td>Hemophilia A</td>
<td>Procedure: Blood sample collection</td>
</tr>
<tr>
<td>7</td>
<td>Recruiting</td>
<td>Hemlibra in Mild Hemophilia A</td>
<td>Factor VIII Deficiency, Congenital</td>
<td>Covance Inc Madison, Wisconsin, United States</td>
</tr>
<tr>
<td>8</td>
<td>Recruiting</td>
<td>Outcomes of Prophylaxis With Emicizumab in Children With Severe Hemophilia A in Ivory Coast</td>
<td>Hemophilia A</td>
<td>Drug: Prophylaxis with Emicizumab</td>
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<tr>
<td>9</td>
<td>Recruiting</td>
<td>Needs Assessment of Knowledge, Beliefs, and Attitudes of Patients With Hemophilia</td>
<td>Gene Therapy, Hemophilia B</td>
<td>Behavioral: Interview</td>
</tr>
<tr>
<td>10</td>
<td>Recruiting</td>
<td>An Exploration of the Impact of Gene Therapy on the Lives of People With Haemophilia and Their Families</td>
<td>Hemophilia</td>
<td>Other: Qualitative interview</td>
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Showing: 1-10 of 153 studies 10 studies per page

Paging: | TO TOP |
## 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)

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**Add your experiences by joining CVR today!**

For more information, including how to enroll visit: [www.hemophilia.org/cvr](http://www.hemophilia.org/cvr) or scan the QR code.
CVR Dashboard

CVR Opening Screen

CVR Main Screen
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.

CHOOSE ONE:

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!
CDC Data Visualization Tool
https://communitycountsdataviz.cdc.gov/blooddisorders/#/!

Patient Characteristics

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

Demographics

<table>
<thead>
<tr>
<th>Category</th>
<th>View</th>
<th>Download</th>
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<tbody>
<tr>
<td>Sex</td>
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<td>Race</td>
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<td>Ethnicity</td>
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<tr>
<td>Age</td>
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Clinical Characteristics

- Patient Geographic Distribution
- Insurance Type
- Education Level
- Employment Status
CDC Data Visualization Tool
https://communitycountsdataviz.cdc.gov/blooddisorders/#!/
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.

What CBRN Members Will Do

- Attend the training & monthly meetings (date and time TBD per the group)
- Communicate outside of meetings through HFA’s engagement 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
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 Distanted (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 women 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?

• 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

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

• 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
What does being an SME mean to me

Our lived experience cannot be taught!
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?

We would love to hear from you!
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 ...

Follow NHF on social media!

@nhf_hemophilia
@nhf_hemophilia
Youtube.com/NHFVideo
Community Engagement 101

Community informed

Community involvement

Community direction

= Community as advisors

= Community as collaborators

= Community as vocal leaders

GREATER COMMUNITY ENGAGEMENT
Community Engagement 101

Community informed

Community involvement

Community direction

=  

=  

=  

Your involvement is key!!!
Recruitment & Engagement

You can help!

Research goals

- Where your audience engages (online vs. in-person, etc.)
- Audience (age, demographics, disease state, location, etc.)
- Budget & capabilities (traditional outreach vs. digital)
Recruitment & Engagement (Cont’d)

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
Recruitment & Engagement (Cont’d)
What is Research Literacy?

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

In 2020, CSL Behring acquired global rights to commercialize the investigational AAV-based gene therapy etranacogene dezaparvovec, 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.
<table>
<thead>
<tr>
<th>“I’m not sure a clinical trial is right for me.”</th>
<th>“I had a bad experience with a clinical trial before.”</th>
<th>“I don’t have the time or money to be in a trial.”</th>
<th>“My privacy would be at risk in a clinical trial.”</th>
</tr>
</thead>
<tbody>
<tr>
<td>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.</td>
<td>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.</td>
<td>Most studies compensate your time and travel! Organizations, charities, or foundations can also help offset any needs.</td>
<td>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.</td>
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</table>
“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.”

A SAMPLING OF WHAT WE’VE LEARNED SO FAR:

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

55% NO
45% YES

Visit www.hemophilia.org/cvr for more insights.

nfh_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.

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

View Insights

9 April 2022
Become a Partner in Research (Cont’d)

https://gather.video/sAqs

THSNA and NHF Partnership Q&A

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

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.

9 April 2022

National Research Blueprint
Summary & Call to Action

Kevin Mills
NHF, Research Department
National Research Blueprint

Please scan this QR code to provide additional comments
THANK YOU!
An Extraordinary Meeting

Nothing About Us Without Us
Building the National Research Blueprint
April 7-9, 2022 - Washington D.C.
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

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.
What did we hear?

- **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
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

A world without inheritable blood disorders
THANK YOU

- Samantha Carlson
- Allison Hartless
- Felix Olaya

- Panelists
- Working Group Chair
THANK YOU SO MUCH!