Vital Link

...For Hoosiers Living with a Bleeding Disorder

HEMOPHILIA OF INDIANA

September 2022

The Vital Link is published quarterly by Hemophilia of Indiana, Inc.

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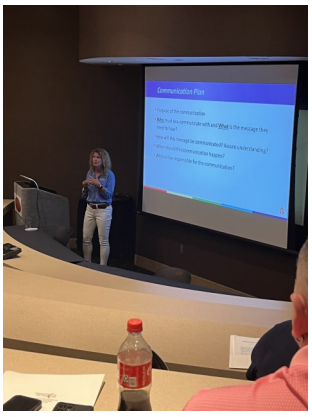
www.hoii.org

2022 Hemophilia of Indiana Annual Meeting

It's hard to believe that we are at the end of September! August was a busy month for the bleeding disorders community starting with the 2022 Annual Meeting on August 13th & 14th. Held at the Crowne Plaza Hotel - Indianapolis Airport, members of the bleeding disorders community joined together for a weekend of education and community spirit. The event started off Saturday with a multi-part presentation by staff from the Indiana Hemophilia & Thrombosis Center followed by education break out sessions in the afternoon. The annual awards dinner was held Saturday evening that honored the 2022 Camp and Scholarship award winners. The event continued Sunday morning with an incredible presentation by Zander Masser called "Unburying My Father". The program was a deep dive into Zander's process of exploring grief through creativity. The event concluded with breakout sessions for Holl's support groups. Go to our website to check out more pictures!



Thank you to our 2022 Annual Meeting Sponsors!

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The IHTC is home to Indiana's experts in rare bleeding & blood disorders, including:
Hemophilia | HHT | HVLM | Thrombosis & Clotting | Sickle Cell Disease | von Willebrand Disease

The Indiana Hemophilia & Thrombosis Center (IHTC) provides advanced care for both adult and pediatric patients with rare bleeding and blood disorders. As Indiana's only federally recognized hemophilia treatment center, IHTC is one of the nation's largest HTCs, delivering innovative, multi-disciplinary care in Indianapolis and at outreach clinics across the state. Visit ihtc.org

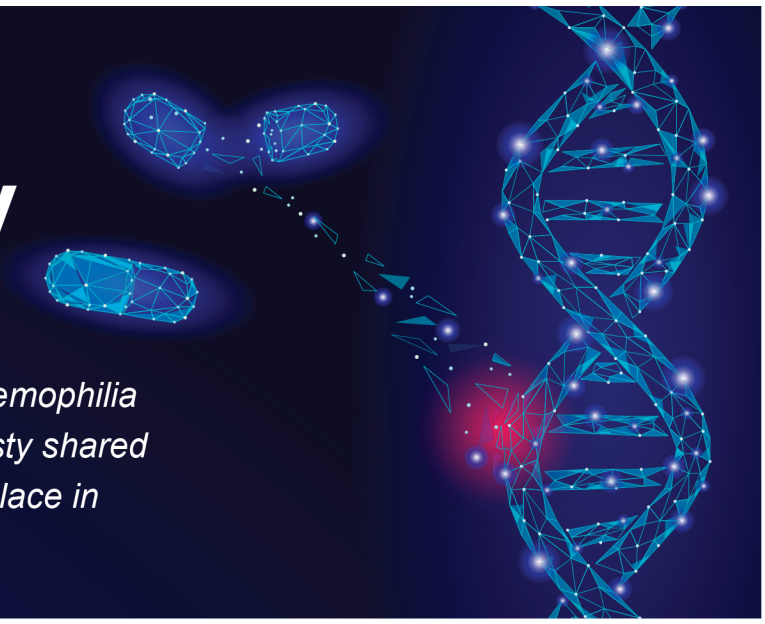
Indiana Hemophilia & Thrombosis Center, Inc. | 8326 Naab Road | Indianapolis, IN 46260 | 877.CLOTTER

2022 Unite for Bleeding Disorders Walk

The 2022 Unite for Bleeding Disorders Walk was held on Saturday, August 20th at the beautiful Fort Harrison State Park. The fundraising goal for this year was $40,000. and thanks to our Walk Teams, Sponsors, and Donors, we exceeded our goal and raised $50,820!!!! Special congratulations to Carolyn Salazar, Emiliano's Advocates for being this year's Top Team Fundraising Captain for the 2nd year in a row! Emiliano's Advocates raised $4,020! Thank you Carolyn and your incredible Walk team!!

We are very excited to announce that the date for the 2023 Unite for Bleeding disorders Walk has moved and will be held Saturday, **MAY 20th!!!**

The basics of Gene Therapy



Among the many expert presenters at the HOII Annual Meeting in August was ***Dr. Brandon Hardesty*** *of the Indiana Hemophilia & Thrombosis Center (IHTC). Dr. Hardesty shared information about gene therapy and its place in the bleeding disorder landscape.*

Dr. Brandon Hardesty
Adult Hematologist, IHTC

How gene therapy works
In hemophilia, patients can have unexpected or prolonged bleeding because of a missing or low clotting factor in the blood. To increase the factor level and lower the number of bleeding episodes, patients often use a factor product or another form of medicine taken by needle.

These treatments can be expensive and time consuming. Gene therapy is an alternative that adds a working copy of the gene. This copy tells the body how to make the missing factor on its own.

The working gene is created in a lab and packaged in a vector—something that will deliver the gene to the body's cells. In gene therapy, a virus is used as the vector because it's easy for viruses to enter cells.

When a patient undergoes gene therapy, a healthcare provider gives the vector through an IV. The vector delivers the gene to the liver, where it starts to make the needed clotting factor protein. If gene therapy is successful, the patient can stop regular factor or nonfactor prophylaxis.

Benefits of gene therapy
Patients who use prescribed factor products and other types of hemophilia medicines often have to adjust their daily or weekly routines because of time-consuming treatments. These treatments can be expensive.

If a patient undergoes successful gene therapy, their body will produce the missing factor on its own without these products. Usually, patients will still need factor infusions for injuries or surgeries.

Ongoing follow-up
Once a patient undergoes gene therapy, they will need to make frequent visits to take and review blood tests. Frequent blood tests are critical to detecting gene rejection and preventing loss of the new gene. Most patients receiving gene therapy will need steroids for weeks to months to prevent the body from rejecting the newly implanted gene. If patients are unable to commit to intensive twice-weekly blood test monitoring for 3-5 months, it would be better to wait until a future time when they are able to make that commitment. There is currently no way to give a second dose of gene therapy if the first dose fails—so catching problems early is crucial.

Understanding gene therapy's limitations
Gene therapy is not a cure for hemophilia. It won't change the fact a patient has the condition. In fact, a patient who undergoes successful gene therapy can still pass the hemophilia gene on to their children. The factor levels achieved by gene therapy vary depending on the product.

Gene therapy will not work for everyone, and it may not last forever. A patient's ability to produce factor can change over time. The body's immune system might identify and eliminate the altered cells. The cells can also naturally die off over time and can impact a patient's ability to undergo future gene therapy. Gene therapy might not work in children due to their growing and changing bodies.

IHTC's approach to new treatments
The IHTC often participates in clinical trials for new treatment, including gene therapy. Our healthcare team cautiously considers each new therapy with patient safety as top priority. We also work with the hemophilia community to share experiences and report success and concerns. Patients are encouraged to speak with their care team if they are interested in information about gene therapy or other advances in hemophilia treatment.

Indiana Hemophilia & Thrombosis Center
8326 Naab Road, Indianapolis, IN 46260 • 317.871.0000 • ihtc.org

Let's make today brilliant.

Takeda is here to support you throughout your journey and help you embrace life's possibilities. Our focus on factor treatments and educational programs, and our dedication to the bleeding disorders community, remain unchanged. And our commitment to patients, inspired by our vision for a bleed-free world is stronger than ever.

bleedingdisorders.com | Takeda

WE ARE HERE FOR YOU!

SUPPORTING INDIANA'S BLEEDING DISORDERS COMMUNITY!!

HEMOPHILIA OF INDIANA

WHAT WE OFFER

- Education Programs
- Emergency Financial Assistance
- Summer Camps
- Scholarship Programs
- Dental Program
- Advocacy Programs
- Medic Alert IDs

How to get more information?

- www.hoii.org
- Social Media - Facebook, Twitter, and Instagram (@HEMOINDY)
- Vital Links Newsletter (also available on website)

OR CONTACT US DIRECTLY

317-570-0039

A **ONCE-WEEKLY** TREATMENT OPTION FOR HEMOPHILIA B.

To find out about a prescription option, talk to your doctor or visit

OnceWeeklyForHemophiliaB.com

Pfizer

2022 Doug Thompson Teen Leadership Camp

Doug Thompson Teen Leadership Camp (DTTLC) is a leadership camp traditionally for teen boys with bleeding disorders ages 14-18. This year's camp also included teen girls ages 14-18 affected with a bleeding disorder! DTTLC was created in memory of our beloved friend and colleague, Doug Thompson. Doug had a passion for teens and enjoyed working with them during the impressionable years of life. The Doug Thompson Teen Leadership Camp Program was designed to provide teen members of the bleeding disorder community the opportunity to develop and refine life skills while participating in an adventure camp experience. Each year 10 teen "leaders" are chosen to participate in the teen leadership program. DTTLC has four main goals: To develop self-confidence through challenging activities, to prepare participants to be responsible about their health condition as adults through education and by completing steps necessary to pursue college admission, and to develop their innate ability through reasonable risk-taking through physical activities, and to foster relationships and build a sense of community between young people with bleeding disorders. This year's DTTLC took place at Ace Adventure Park in Oak Hill, WV. The campers participated in white water rafting, zip lining, hiking, and much more! Check out pictures from this year's DTTLC!

2022

FitLivin THXRUN

Get a healthy start to your holiday and help support Hemophilia of Indiana and the bleeding disorders community!!

Where: Dr. James A Dillon Park
Noblesville, IN

When: November 24th

Time: Free Kids Run - 8:45am
THXRUN - 9:00am

www.fitlivinthanksgivin.com

September is Suicide Prevention Awareness Month

Suicide is the 12th leading cause of death in the United States. On average, this comes out to 130 suicides per day, but the evidence shows talking about suicide can help decrease the likelihood that someone will die by suicide. This is one reason that September has been designated as Suicide Prevention Month.

While it is not uncommon for people to have occasional suicidal thoughts, this is a sign that more help may be needed. Some other warning signs include:

- Talking about wanting to die or kill themselves
- Feeling hopeless or having no reason to live
- Feeling trapped or in unbearable pain
- Feeling like a burden to others
- Increasing use of alcohol or drugs
- Sleeping too little or too much
- Withdrawing or isolating socially
- Agitation or talking about seeking revenge
- Extreme mood swings

988 is an easy-to-remember number to connect to compassionate, confidential support for anyone experiencing a mental health crisis. ***This can include thoughts of suicide, mental health or substance use crisis.***

You recognize that someone in your life is suffering – *now what*?

- Talk openly and honestly.
- Don't be afraid to ask questions like: "Have you had thoughts of ending your life?"
- Express support and concern.
- Don't argue, threaten, or raise your voice.
- Be patient.
- Remove items like guns, knives, or stockpiled pills.
- Connect the patient to help. You can help them call their therapist or psychiatrist, contact a local crisis center, or call the Suicide and Crisis Lifeline.

If you or someone you know is currently experiencing thoughts of suicide, or a mental health or substance use crisis, please call 9-8-8 to reach Suicide & Crisis Lifeline and speak with a trained crisis specialist 24/7.

988 SUICIDE & CRISIS LIFELINE

Indiana Hemophilia & Thrombosis Center

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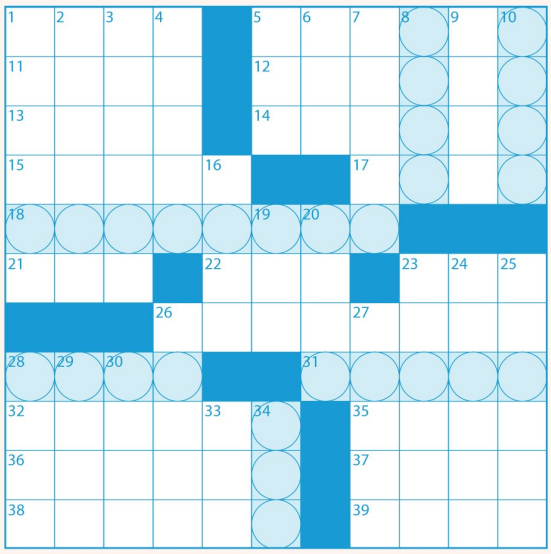
CAN YOU SOLVE FOR A DIFFERENT HEMOPHILIA A TREATMENT?

Test your HEMLIBRA knowledge



ACROSS

1. Wine barrel
5. Deep fissures
11. Mideast gulf port
12. District
13. Ripped
14. Familiar with
15. Mean
17. Roost
18. The #1 prescribed prophylaxis for people with hemophilia A without factor VIII inhibitors*

*According to IQVIA claims data from various insurance plan types from April 2020 - May 2021 and accounts for usage in prophylaxis settings in the US.

21. Calendar divs.
22. Regret
23. Banquet hosts (abbr.)
26. International travel necessity
28. Check out the _____ treated bleeds data with HEMLIBRA
31. Number of dosing options HEMLIBRA offers

† Number of people with hemophilia A treated as of October 2021.

32. Small hole in lace cloth
35. Central Plains tribe
36. Melodic
37. Towering
38. Reduce
39. Spanish cheers

DOWN

1. Memorable, as an earworm
2. Devotee
3. Medical fluids
4. Prepare to propose, perhaps
5. PC's "brain"
6. Owns
7. Concert venue
8. See Medication Guide or talk to your doctor about potential _____ effects
9. Winter hrs. in Denver and El Paso
10. HEMLIBRA is the only prophylactic treatment offered this way under the skin
16. Pre-Euro currency in Italy
19. Subway alternative
20. Relax
23. Human
24. New Orleans cuisine
25. Mentally prepares
26. Collared shirts
27. Instagram post
28. Ardent enthusiasm
29. Brontë heroine Jane
30. Old Portuguese coins
33. Opposite of WNW
34. More than_____ thousand patients have been treated with HEMLIBRA worldwide†

SOLUTIONS

Across: 1. cask, 5. chasms, 11. Aden, 12. parish, 13. tore, 14. used to, 15. cruel, 17. nest, 18. HEMLIBRA, 21. yrs, 22. rue, 23. MCs, 26. passport, 28. zero, 31. three, 32. eyelet, 35. Otoe, 36. anose, 37. tall, 38. lessen, 39. oles

Down: 1. catchy, 2. adorer, 3. serums, 4. kneel, 5. CPU, 6. has, 7. arena, 8. side, 9. MSTs, 10. shot, 16. lira, 19. bus, 20. rest, 23. mortal, 24. Creole, 25. steels, 26. polos, 27. photo, 28. zeal, 29. Eyre, 30. Reis, 33. ESE, 34. ten

Discover more at HEMLIBRA.com/answers

INDICATION & IMPORTANT SAFETY INFORMATION

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. People who use activated prothrombin complex concentrate (aPCC; Feiba®) to treat breakthrough bleeds while taking HEMLIBRA may be at risk of serious side effects related to blood clots.

These serious side effects include:

- **Thrombotic microangiopathy (TMA),** a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs
- **Blood clots (thrombotic events),** which may form in blood vessels in your arm, leg, lung, or head

Please see Brief Summary of Medication Guide on following page for Important Safety Information, including **Serious Side Effects.**

HEMLIBRA®
emicizumab-kxwh
injection for subcutaneous use
150 mg/mL

Medication Guide
HEMLIBRA® (hem-lee-bruh)
(emicizumab-kxwh)
injection, for subcutaneous use

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - confusion
  - weakness
  - swelling of arms and legs
  - yellowing of skin and eyes
  - stomach (abdomen) or back pain
  - nausea or vomiting
  - feeling sick
  - decreased urination
- **Blood clots (thrombotic events).** Blood clots may form in blood vessels in your arm, leg, lung, or head. Get medical help right away if you have any of these signs or symptoms of blood clots during or after treatment with HEMLIBRA:
  - swelling in arms or legs
  - pain or redness in your arms or legs
  - shortness of breath
  - chest pain or tightness
  - fast heart rate
  - cough up blood
  - feel faint
  - headache
  - numbness in your face
  - eye pain or swelling
  - trouble seeing

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

Your body may make antibodies against HEMLIBRA, which may stop HEMLIBRA from working properly. Contact your healthcare provider immediately if you notice that HEMLIBRA has stopped working for you (eg, increase in bleeds).

See "**What are the possible side effects of HEMLIBRA?**" for more information about side effects.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.

HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

Before using HEMLIBRA, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA.
- are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use HEMLIBRA?

See the detailed "Instructions for Use" that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- **Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis.**
- **You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.**
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.
- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.
- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- You will receive HEMLIBRA 1 time a week for the first four weeks. Then you will receive a maintenance dose as prescribed by your healthcare provider.
- If you miss a dose of HEMLIBRA on your scheduled day, you should give the dose as soon as you remember. You must give the missed dose as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. **Do not** give two doses on the same day to make up for a missed dose.
- HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

What are the possible side effects of HEMLIBRA?

- See "**What is the most important information I should know about HEMLIBRA?**"

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
- Store HEMLIBRA in the original carton to protect the vials from light.
- Do not shake HEMLIBRA.
- If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator for more than a total of 7 days or at a temperature greater than 86°F (30°C).
- After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.
- Throw away (dispose of) any unused HEMLIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

General information about the safe and effective use of HEMLIBRA.

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMLIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMLIBRA that is written for health professionals.

What are the ingredients in HEMLIBRA?

Active ingredient: emicizumab-kxwh

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

Manufactured by: Genentech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94080-4990
U.S. License No. 1048

For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.
This Medication Guide has been approved by the U.S. Food and Drug Administration
Revised: 12/2021

Genentech
A Member of the Roche Group

Upcoming Educational Dinners:

Contact Angel DiRuzza at adiruzza@hoii.org to register. Check out our Facebook (@HEMOINDY) page and website calendar for more details on upcoming in person educational dinners!

Topic: **Community U Speaker Program...the female connection**
Sponsored by Novo Nordisk
When: Thursday, October 13th
Where: Livery - Noblesville
13225 Levinson Lane
Noblesville, IN 46060

Topic: **SevenFACT, Coagulation Factor VIIa (Recombinant)-jncw**
Sponsored by Hema Biologics
When: Tuesday, October 25
Where: Giordano's
4110 E 82st St
Indianapolis, IN 46250

Topic: **Learn more about Idelvion, a Coagulation Factor IX (Recombinant) product**
Sponsored by CSL Behring
When: Thursday, November 3rd
Where: **Restaurant TBD - Indianapolis, IN**

Topic: **Topic TBD**
Sponsored by Takeda
When: Tuesday, November 15th
Where: **Restaurant TBD - Fort Wayne, IN**

Topic: **Topic TBD**
Sponsored by Sanofi
When: Tuesday, November 29th
Where: **Restaurant and City TBD**

saavy (270-701)

Help the hemophilia community by sharing this research study with your patients

Sponsored by BIOMARIN

The SAAVY study is a prospective, observational study to evaluate the seroprevalence and the rate of seroconversion of adeno-associated virus (AAV) serotypes AAV5, AAV6, and AAV8 in patients with hemophilia A, over a 3 month or 6 month timepoint.

The aim of this study is to find out how common it is for people with hemophilia A to have existing antibodies against AAV. By participating, your patient can help scientists understand how the body may develop these antibodies and what it could mean for the development of future treatments.

Your patient may qualify for the *SAAVY* (270-701) study if s/he is:

- 18 years of age or older; and
- Diagnosed with hemophilia A

How does the study work?

- Participants will be asked to complete 2 blood draws and answer questions on a convenient mobile app.
- They will receive compensation after each blood donation for their time and participation.
- No medication, therapy, or experimental procedures are part of this study.

What value will the results of the study bring to the hemophilia community?

Different types of AAV are frequently used in clinical trials for gene therapy. Understanding the presence of AAV antibodies will help guide researchers in developing innovative therapies for people with hemophilia A.

Scan the code to Learn More

Hear what the experts are saying

Learn more about the value of this important research from experts in the community.

Kim Schafer, MSN, NP-C
Nurse Practitioner, Hemostasis and Thrombosis Center, UC Davis Health

Dawn Rotellini
Chief Operating Officer, NHF

Watch the video at saavy-study.com

This event was created for educational purposes only.

HEMOPHILIA OF INDIANA

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*The material provided in Vital Link is only for general information purposes. HoII does not give medical advice or engage in the practice of medicine. HoII recommends in all cases that you consult your physician or HTC before pursuing any course of treatment.

Mark your Calendars!

- FitLivin THXRUN - November 24
- Year End Education Event - December 4th
- 2023 Hearts for Hemophilia Gala - January 27th

Call our office @ (317) 570-0039 or email Kristy McConnell @ kmcconnell@hoii.org if you would like to get involved in any of our events!

- Check out our social media pages for updates!!!