Vital Link

HEMOPHILIA OF INDIANA

...For Hoosiers Living with a Bleeding Disorder

March 2017

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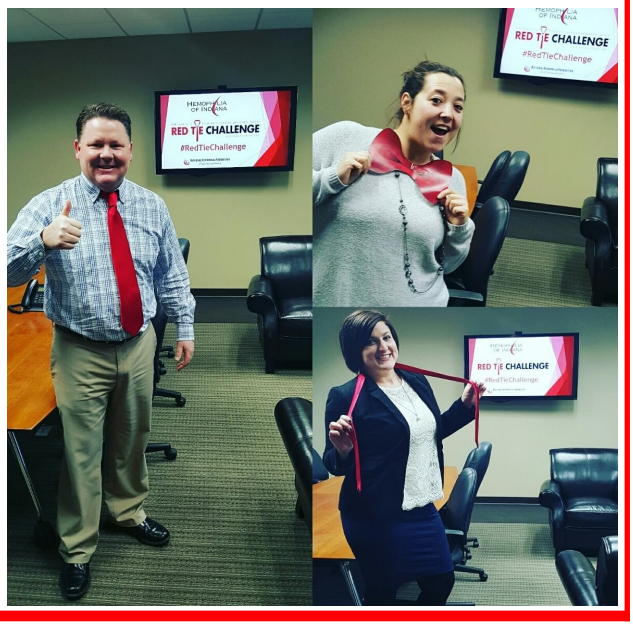
Hemophilia of Indiana Launches the 2017 Red Tie Challenge

March is Bleeding Disorder's Awareness Month and we need your help to spread the word! How do you get involved? Participate in the National Hemophilia Foundation's Red Tie Challenge!

Join the Red Tie Challenge to:
Raise awareness of bleeding disorders – and the importance of early detection and treatment.
Help raise $20,000 to support research, advocacy, and services for families living with bleeding disorders. NHF hopes to raise $1 for each person affected by hemophilia in the U.S.
Show your support for the bleeding disorders community – and challenge others to do the same!

Hemophilia of Indiana's Executive Director, Scott Ehnes says, "taking the Red Tie Challenge is as easy as 1-2-3!"

1. Make a donation at **www.redtiechallenge.org**
2. Get a red tie, then record and share your best red tie style with the hashtag #RedTieChallenge.
3. Challenge your friends to join you in the fight against bleeding disorders.



Get your Red Tie Ready!

IXINITY®
coagulation factor IX
(recombinant)

is now brought to you by

Aptevo Therapeutics—
a company that's anything but ordinary

At Aptevo, we pride ourselves on:

Providing high-quality, specialized therapies for people with rare conditions

Connecting with people to learn about their needs

Developing empowering programs that enrich peoples' lives

Learn more at IXINITY.com

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Research...

Potential Therapy Employs Addition by Subtraction Approach

Recent research suggests that a new understanding of furin, a common protein found in most cells, could have therapeutic implications for people with factor VIII deficiency, or hemophilia A. The new findings, "Circumventing Furin Enhances Factor VIII Biological Activity and Ameliorates Bleeding Phenotypes in Hemophilia Models," were published October 6, 2016, in the journal *JCI (Journal of Clinical Investigation) Insight*. The lead investigator of the study was Valder R. Arruda, MD, PhD, a hematology researcher at The Children's Hospital of Philadelphia. Arruda is also a faculty member of the Perelman School of Medicine at the University of Pennsylvania.

Until now, scientists understood that among its many roles, furin contributes to coagulation. Factor replacement therapies, including factor VIII (FVIII) in hemophilia A and factor IX (FIX) in hemophilia B, contain amino acids that identify and interact with furin as part of the clotting process. However, Arruda and his team have discovered that while the furin/FIX interaction is a key "clotting-contributor," furin may not be necessary for a healthy clot to form in people with hemophilia A. They made this discovery by first bioengineering a new variant FVIII protein designed so that it would not interact with furin. They then used that variant in gene therapy experiments in mice with severe hemophilia A, which triggered increased FVIII levels and improved clotting activity in the animals.

Collaborating on the study were investigators from the University of North Carolina (UNC) at Chapel Hill led by Timothy C. Nichols, MD. The UNC team used the same gene therapy on dogs with hemophilia A. They also observed decrease bleeding as a result. In addition, no unwanted immune response occurred from the therapy.

By omitting the furin component, researchers have found a novel way to streamline delivery of the genetic material. "In gene therapy, size matters," said Arruda. "It's important to reduce the gene package for FVIII to the smallest effective size." Deleting the furin-recognition portion both decreases the size of the gene therapy "payload" and enhances its benefits for treating hemophilia A, he added.

Further research is needed before clinical trials in people can be conducted, but the researchers are optimistic about furin's future. "Because this variant provides more efficient bleeding control than currently available replacement drugs, while avoiding immune reactions, this could address the unmet needs of hemophilia A patients worldwide," added Arruda. "It may also advance gene therapy for this disorder as well."

Source: Science Daily, October 17, 2016

Hearts for Hemophilia Gala, "Masquerade Ball"

The 27th Annual Hearts for Hemophilia Gala, was held on Friday, February 10th, at the Union Station Grand Ballroom, downtown Indianapolis. This year's theme was "Masquerade Ball." Guests sported masks and enjoyed an evening of mystique, where they enjoyed dinner, live and silent auctions, close-up magic and illusions by magicians Jay McLaughlin and Doug Jave, and danced the night away to live music by *Greta Speaks.* We even had special guest, National Hemophilia Foundation CEO, Val Bias deliver an inspiring speech. All proceeds from the Gala will support Hemophilia of Indiana's *Project Lifeline,* which provides more than 1,400 Hoosiers affected with bleeding disorders vital services such as education, scholarships, dental insurance, MedicAlert identification, emergency financial assistance, and more. It was a wonderful evening and we had a record turn out with over 250 people in attendance! Whether you personally attended, sponsored the event in some way, donated an item to our silent auction, or volunteered, know that we are grateful! Mark your calendar for next year's Hearts for Hemophilia Gala, February 9th, 2018.

INDIANA HEMOPHILIA & THROMBOSIS CENTER, INC.

The Best Outcomes for You and Your Family

Indiana's only Center of Excellence in bleeding disorders

- Comprehensive clinics with our expert multidisciplinary team
- Integrated pharmacy program accessible 24/7
- Nationally renowned for the treatment of bleeding and clotting disorders
- First HTC to receive Medical Home certification (AAAHC)
- Free CME education and 24 hours, 7 days a week consultative services for providers throughout the state
- Outreach clinics across Indiana, education and training for families
- Research to advance care and provide savings to patients and families

For more information, please contact us at 317.871.0000

To contact our Pharmacy, please call 317.829.7778

Visit us online at www.ihtc.org and www.facebook.com/IndianaHemophilia

2017 Hemophilia of Indiana Judy Moore Memorial Scholarship

Judy Moore joined the Indiana Hemophilia & Thrombosis Center as a social worker in 1999. She first entered the hemophilia care arena in 1990 through employment at Hemophilia of Indiana, Inc. Judy's experience serving the hemophilia community spanned the HIV/AIDS epidemic, the transition to recombinant clotting factor concentrates, and most recently, healthcare reform. Judy was an inspiration to all – patients, families, friends and coworkers. She leaves a proud legacy of care, having touched many lives during her thirteen years at the IHTC and her prior years serving the bleeding disorders community.

Scholarships will be awarded to those inflicted with a bleeding disorder, including but not limited to von Willebrand's disease, Hemophilia, and Hemophilia carriers. The scholarship will be presented to the applicant that provides the best combination of a creative and persuasive essay, excellent recommendations, and superior academic standing.

For the 2017-2018 academic year, Hemophilia of Indiana will award up to $10,000 in college scholarships, including vocational schools.

To be eligible, you must:

- Have been diagnosed with a bleeding disorder, be an Indiana resident attending a school in the United States, and meet one of the following criteria:
  -Be a high school senior or graduate, or
  -Have completed high school or an equivalent (i.e. general equivalency diploma /GED or
  -Be currently accepted to or enrolled in a junior college, college (undergraduate or graduate), or vocational school

Completed applications must be received via mail or sent via e-mail no later than **May 8, 2017** . It is the applicant's responsibility to make sure that all original transcripts **(copies will not be accepted)** are postmarked by this deadline. **We recommend you request your transcripts from your school no later than April 5, 2017.**

An application is complete when the application form (including education form), essay, completed release form, 2 personal recommendations, and all original transcript documents have been received or sent via e-mail to the program administrator **no later than May 8, 2017.**

To download an application, visit **www.hoii.org,** select Member Resources, then Scholarships. If at any time you have questions, please call 317-570-0039 between 9 AM and 5 PM EST or e-mail your questions to sehnes@hoii.org.

AFSTYLA®, Antihemophilic Factor (Recombinant), Single Chain
For Intravenous Injection, Powder and Solvent for Injection
Initial U.S. Approval: 2016

BRIEF SUMMARY OF PRESCRIBING INFORMATION
These highlights do not include all the information needed to use AFSTYLA safely and effectively. See full prescribing information for AFSTYLA.

----------INDICATIONS AND USAGE----------
AFSTYLA®, Antihemophilic Factor (Recombinant), Single Chain, is a recombinant, antihemophilic factor indicated in adults and children with hemophilia A (congenital Factor VIII deficiency) for:

- On-demand treatment and control of bleeding episodes,
- Routine prophylaxis to reduce the frequency of bleeding episodes,
- Perioperative management of bleeding.

Limitation of Use
AFSTYLA is not indicated for the treatment of von Willebrand disease.

----------DOSAGE AND ADMINISTRATION----------
For intravenous use after reconstitution only.

- Each vial of AFSTYLA is labeled with the amount of recombinant Factor VIII in international units (IU or unit). One unit per kilogram body weight will raise the Factor VIII level by 2 IU/dL.
- Plasma Factor VIII levels can be monitored using either a chromogenic assay or a one-stage clotting assay – routinely used in US clinical laboratories. **If the one-stage clotting assay is used, multiply the result by a conversion factor of 2 to determine the patient's Factor VIII activity level.**

Calculating Required Dose:

Dose (IU) = Body Weight (kg) x Desired Factor VIII Rise (IU/dL or % of normal) x 0.5 (IU/kg per IU/dL)

Routine Prophylaxis:

- Adults and adolescents (≥12 years): The recommended starting regimen is 20 to 50 IU per kg of AFSTYLA administered 2 to 3 times weekly.
- Children (<12 years): The recommended starting regimen is 30 to 50 IU per kg of AFSTYLA administered 2 to 3 times weekly. More frequent or higher doses may be required in children <12 years of age to account for the higher clearance in this age group.
- The regimen may be adjusted based on patient response.

Perioperative Management:

- Ensure the appropriate Factor VIII activity level is achieved and maintained.

----------DOSAGE FORMS AND STRENGTHS----------
AFSTYLA is available as a white or slightly yellow lyophilized powder supplied in single-use vials containing nominally 250, 500, 1000, 2000, or 3000 International Units (IU).

----------CONTRAINDICATIONS----------
Do not use in patients who have had life-threatening hypersensitivity reactions, including anaphylaxis to AFSTYLA or its excipients, or hamster proteins.

----------WARNINGS AND PRECAUTIONS----------

- Hypersensitivity reactions, including anaphylaxis, are possible. Should symptoms occur, immediately discontinue AFSTYLA and administer appropriate treatment. (5.1)
- Development of Factor VIII neutralizing antibodies (inhibitors) can occur. If expected plasma Factor VIII activity levels are not attained, or if bleeding is not controlled with an appropriate dose, perform an assay that measures Factor VIII inhibitor concentration.
- If the one-stage clotting assay is used, multiply the result by a conversion factor of 2 to determine the patient's Factor VIII activity level.

----------ADVERSE REACTIONS----------
The most common adverse reactions reported in clinical trials (>0.5% of subjects) were dizziness and hypersensitivity.

To report SUSPECTED ADVERSE REACTIONS, contact the CSL Behring Pharmacovigilance Department at 1-866-915-6958 or FDA at 1-800-FDA-1088 or *www.fda.gov/medwatch*.

----------USE IN SPECIFIC POPULATIONS----------

- Pediatric: Clearance (based on per kg body weight) is higher in pediatric patients 0 to <12 years of age. Higher and/or more frequent dosing may be needed.

Based on May 2016 version

8th Annual Bowling Marathon

Sunday, April 30, 2017 @ Pinhead's in Fishers, IN.

Contact Scott Ehnes at sehnes@hoii.org or visit our website at www.hoii.org for more information.

ADYNOVATE [Antihemophilic Factor (Recombinant), PEGylated] Important Information

Indications

ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

You should not use ADYNOVATE if you:
- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE [Antihemophilic Factor (Recombinant)]

Tell your healthcare provider if you are pregnant or breastfeeding because ADYNOVATE may not be right for you.

You should tell your healthcare provider if you:
- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to Factor VIII.

You can have an allergic reaction to ADYNOVATE.
Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do not go away.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see following page for ADYNOVATE Important Facts.

For full Prescribing Information, visit www.ADYNOVATE.com.

References: 1. ADYNOVATE Prescribing Information. Westlake Village, CA: Baxalta US Inc. **2.** Mullins ES, Stasyshyn O, Alvarez-Román MT, et al. Extended half-life pegylated, full-length recombinant factor VIII for prophylaxis in children with severe haemophilia A. *Haemophilia*. 2016 Nov 27. doi: 10.1111/hae.13119 [Epub ahead of print]. **3.** Data on file; Shire US Inc.

Shire

ADYNOVATE
[Antihemophilic Factor (Recombinant), PEGylated]

Patient Important facts about

ADYNOVATE® [Antihemophilic Factor (Recombinant), PEGylated]

This leaflet summarizes important information about ADYNOVATE. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about ADYNOVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADYNOVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADYNOVATE so that your treatment will work best for you.

What is ADYNOVATE?

ADYNOVATE is an injectable medicine that is used to help treat and control bleeding in children and adults with hemophilia A (congenital Factor VIII deficiency). Your healthcare provider may give you ADYNOVATE when you have surgery. ADYNOVATE can reduce the number of bleeding episodes when used regularly (prophylaxis).

ADYNOVATE is not used to treat von Willebrand disease.

Who should not use ADYNOVATE?

You should not use ADYNOVATE if you:

- Are allergic to mice or hamster protein
- Are allergic to any ingredients in ADYNOVATE or ADVATE® [Antihemophilic Factor (Recombinant)]

Tell your healthcare provider if you are pregnant or breastfeeding because ADYNOVATE may not be right for you.

How should I use ADYNOVATE?

ADYNOVATE is given directly into the bloodstream.

You may infuse ADYNOVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADYNOVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADYNOVATE to use based on your individual weight, level of physical activity, the severity of your hemophilia A, and where you are bleeding.

Reconstituted product (after mixing dry product with wet diluent) must be used within 3 hours and cannot be stored or refrigerated. Discard any ADYNOVATE left in the vial at the end of your infusion as directed by your healthcare professional.

You may have to have blood tests done after getting ADYNOVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

How should I use ADYNOVATE? (cont'd)

Call your healthcare provider right away if your bleeding does not stop after taking ADYNOVATE.

What should I tell my healthcare provider before I use ADYNOVATE?

You should tell your healthcare provider if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADYNOVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADYNOVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADYNOVATE may not work for you).

What are the possible side effects of ADYNOVATE?

You can have an allergic reaction to ADYNOVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

The common side effects of ADYNOVATE are headache and nausea. Tell your healthcare provider about any side effects that bother you or do not go away.

These are not all the possible side effects with ADYNOVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADYNOVATE and Hemophilia A?

Your body may form inhibitors to Factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADYNOVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to Factor VIII.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ADYNOVATE for a condition for which it is not prescribed. Do not share ADYNOVATE with other people, even if they have the same symptoms that you have.

The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADYNOVATE. The FDA-approved product labeling can be found at www.shirecontent.com/PI/PDFs/ADYNOVATE_USA_ENG.pdf or 855-4-ADYNOVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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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!

Here are some of our upcoming events...

- Bowling Marathon, Pinhead's, Fishers - April 30
- Men's Group Meeting, Victory Field - May TBD
- Course to a Cure Golf Outing, Maple Creek Golf Club - June 5
- Camp Brave Eagle, Camp Crosley - June 11-16
- Doug Thompson Teen Leadership Camp - June 26-30
- Annual Meeting, Crowne Plaza Airport - August 4-6

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

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