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# BIG RED

2016—Issue II

## Nebraska Chapter News

### Staffing Changes

It is with bittersweet feelings that we announce the departure of the NHF Nebraska Chapter's Executive Director, Tami Soper. During her time with the chapter, Tami has had the opportunity to meet and work with many in our community and everyone has appreciated her patience and willingness to listen to their needs. She was also involved with the chapter during a period of significant changes and evolution and we have respected her leadership throughout this process.

I believe that as she transitions to her new position in Marketing and Public Relations for Midwest Medical Transport she will be very successful and will be a valuable asset to the organization. I have personally enjoyed working with Tami and wish her the very best as she works towards furthering her career goals. Please join me in thanking Tami for her time and efforts throughout the last year with the chapter.

Maureen Grace, Chapter Development Manager, is assuming the role for interim executive director as we regroup and move the chapter forward. Please feel free to contact her with any questions or concerns going forward.

Michael Craciunoiu, Director of Chapter Development

#### \*\*\*\*\*ATTENTION\*\*\*\*\*

This issue will be the last printed issue of the  
NHF Big Red Newsletter.  
All future publications will be online.

Please email [mgrace@hemophilia.org](mailto:mgrace@hemophilia.org) to update  
your email address to ensure you continue  
receiving up to date information and our continued  
newsletter and event announcements.



### **Our Mission:**

The National Hemophilia Foundation—Nebraska Chapter is dedicated to finding better treatments and cures for inheritable bleeding disorders and to preventing the complications of these disorders through education, advocacy & research.

### **Staff**

***Interim Executive Director***  
Maureen Grace

### **Advisory Board of Directors**

President - Patrick Quintana  
Vice President - Geri Murphy  
Treasurer - Mark Harms  
Secretary - Dale Gibbs  
Zach Blackman  
Scott Gass  
Dale Gibbs  
Dan Henson  
Rebecca Runge  
Marlee Wernke

*The material in this newsletter is provided for your general information only. The Nebraska Chapter does not give medical advice or engage in the practice of medicine. NHF-NE does not recommend particular treatments for specific individuals and in all cases recommends that you consult your physician or local treatment center before pursuing any course of treatment.*

## **2016 Program and Events Calendar**

**July 21—23, 2016**

NHF 68th Annual National Conference  
Orlando, FL

**August TBD, 2016**

PING/New Parents Group  
TBD

**Tuesday, September 13, 2016**

NE Advisory Board Meeting

**Saturday, September 24, 2016**

NE Hemophilia Walk  
Chalco Hills Recreation Area

**Saturday, October 15, 2016**

Harvest Festival  
Vala's Pumpkin Patch

**Saturday, October 22, 2016**

Being Prepared for a Hemophilia  
Emergency sponsored by Bayer  
Nebraska Air National Guard

**Saturday, December 10, 2016**

PING and Holiday Party  
TBD

***\*\*Dates are Subject to change.  
More Activities Coming Soon!  
Watch our website for updates and register  
Online!***

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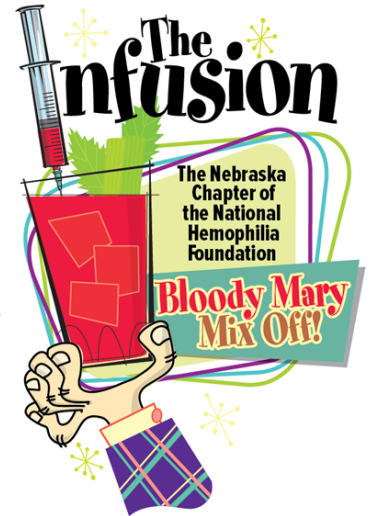
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## The Infusion: A Bloody Mary Mix Off

What's a better way to get people together on a Saturday morning to support the Nebraska Chapter of NHF? You get them together to vote on the best hand mixed Bloody Mary cocktails at The Infusion, a Bloody Mary Mix Off. This marked the inaugural year for the mix off. Six Nebraska bars gathered at the Ralston Arena to compete for the best Bloody Mary. Those competing were our Flagship bar, Liv Lounge, Beyond Golf, Krug Park, The Den, Report In and Jake's Cigars and Spirits. Each bar brought delicious drinks and an undeniable affinity for their craft.

After all the glasses were empty and attendees were stuffed like olives from a delicious brunch, Beyond Golf claimed their righteous spot on top of their competition as the People's Choice Bloody Mary trophy winners.



This year's sponsors included Novo Nordisk, Emergent BioSolutions, Bayer, Baxalta, Biogen and CSL Bering.

We look forward to growing the event next year with more competitors so be sure to be on the lookout for the 2017 Infusion. Beyond Golf will be our Flagship bar challenging others and defending their trophy and NHF's Infusion Bloody Mary Mix Off Winner title.

## Hydrate for Hemophilia



The Road to Omaha once again brought 8 teams and the Nebraska Chapter of NHF to downtown Omaha to celebrate the NCAA Men's College World Series from June 17-28th. The Old Mattress Factory and Baseball Village once again hosted Flying Flags for Charity on opening night. Eight charities were honored and paired with the CWS team. NHF has been a Flying Flags charity for the previous two years. This year, we were honored with the Aim for the Fences Spirit Award for our involvement with the Flying Flags for Charity program and our past involvement in the College World Series. Senior Vice President of Chapter Development and Education, Dawn Rotellini traveled to Omaha to accept the award on

behalf of the National Hemophilia Foundation. We were honored to be the first charity to receive this award.

In addition to our service award, NHF also had a booth set up in Baseball Village selling water and sundry items to raise money and awareness for the chapter. A special thank you to all of the volunteers and board members who came out to run the tent and help bring awareness to bleeding disorders in Nebraska and to Walgreens for donating cases of bottled water to the cause.





**Presentation: Being Prepared for a Hemophilia Emergency**  
**Jessica Walker, RN, BSN**  
**Clinical Support Specialist, Bayer**

**Date: Saturday, 10/ 22/ 2016**

**Time: 12:00 PM**

**Where: Nebraska Air National Guard (Hangar)**  
**2601 NW 25<sup>th</sup> Street**  
**Lincoln, NE 68524**

**RSVP: Maureen Grace**  
**[mgrace@hemophilia.org](mailto:mgrace@hemophilia.org)**  
**402-499-8025**

**RSVP must be received by Friday, 10/ 7/ 2016**

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- You may be able to receive up to **\$12,000 in assistance** per year, regardless of income
- **Assistance is awarded per patient.** Multiple members of the same household can apply
- Enrollment can be completed in **one short phone call**

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- Spanish-speaking Case Specialists are also available

**CALL**  
**1-800-288-8374**

8:00 AM-8:00 PM (ET) Monday-Friday. Spanish-speaking Case Specialists are also available.

\*The Free Trial Program is available to newly diagnosed patients and patients who are currently using other therapy. Participation in the Free Trial Program is limited to 1 time only. This program is complimentary and is not an obligation to purchase or use a Bayer product in the future. Reselling or billing any third party for the free product is prohibited by law.

†The Free Trial Program includes up to 6 free doses to a maximum of 5,000 IU for new patients and 40,000 IU for previously treated patients.

‡The program does not guarantee that patients will be successful in obtaining reimbursement. Support medication provided through Bayer's assistance programs is complimentary and is not contingent on future product purchases. Reselling or billing any third party for free product provided by Bayer's patient assistance programs is prohibited by law. Bayer reserves the right to determine eligibility, monitor participation, determine equitable distribution of product, and modify or discontinue the program at any time.

§People with private, commercial health insurance may receive co-pay or co-insurance assistance based on eligibility requirements. The program is on a first-come, first-served basis. Financial support is available for up to 12 months. Eligible patients can re-enroll for additional 12-month courses. The program is not for patients receiving prescription reimbursement under any federal-, state-, or government-funded insurance programs, or where prohibited by law. All people who meet these criteria are encouraged to apply. Bayer reserves the right to discontinue the program at any time.



## Remembering John Indence

NHF Mourns the Loss of John Indence, VP Marketing and Communications.

John was our friend, and his contributions to our team and his place in our staff cannot be measured in accomplishments, as he was the heart and soul of our voice to this community.

John Indence was responsible for all marketing, communications, public relations and branding strategies for NHF. John was a valued member of our team and a creator and architect of our communication suite. Beyond the many awards and recognition for Hemaware, John broadened our communication abilities to include Hemaware online and extended our reach both nationally and internationally. His contributions to the agency and the community has changed the face of the National Hemophilia Foundation and brought us to a new and more inclusive image, representing everyone living with a bleeding disorder and their families. He will be missed.

Our thoughts and prayers our with his family, friends and those who worked closely with him at the National office and throughout NHF. His work touched so many lives and made a lasting impact in our community.



## Harvest Festival 2016



Join us for the Harvest Festival at Vala's Pumpkin Patch

**Saturday, October 15, 2016**

Pre-registration is required!

In order to gain admission, you will need to be registered in advance of the event.

NHF will have a shelter reserved from 11:30 am to 3:30 pm. Lunch will be served.

Register by contacting Maureen Grace at 402-499-8025 or [mgrace@hemophilia.org](mailto:mgrace@hemophilia.org)

Registration deadline is October 5th, 2016





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- Von Willebrand's Disease
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# “As I See It” by Ian Muir

## A Letter to My Future Self

*Ian Muir*

Dear Ian of 2026,

How is 40 treating you? I have high hopes that it's going great in multiple respects (house, job, dog, wife, etc.).

Of equal or greater importance, I hope that the next hemophilia treatment revolution is well under way. And I certainly hope that you're not taking anything resembling clotting factor concentrate, or for that matter any product that lasts less than four weeks to treat your hemophilia.

The years 2015 and 2016 were so hopeful, remember? You had been primed with knowledge, friends, and perspective from the World Federation of Hemophilia Congress in Paris in 2012. Then, in 2015, you turned 30 and had a few mild health scares that reminded you of your own mortality. This set you off on a renewed quest of empathetic restlessness and searching. In 2015, novel approaches to treating hemophilia from some brilliant new players were being published, and the data looked promising. More accessible and effective treatment options were closer than the horizon for underserved people with inhibitors, and for some developing countries that lacked reliable access to any of the flavors of clotting factor. You felt excitement and hope every day (sometimes too much—do people still say you're too enthusiastic?), not only for you, but for your clotting-challenged friends and friends-to-be around the world.

What have you done with your new freedom to travel and be more “off the grid” when a single dose can last a month? I hope you've found time between stateside jobs to travel around the world and participate in preparing new markets that will do business with ambitious companies to expand access to hemophilia treatment. If all goes to plan, you should be far more concerned about what you're going to do with your now 12-year-old pit bull Charlotte for three months while hopping around the globe, rather than hassling with receiving \$50,000 of clotting factor a month. I hope you only need a couple of doses of your current product. This should give you and your family some peace of mind to be abroad for several months at a time without needing to meet up with someone for a covert factor handoff or something. Just kidding.

I hope that your prediction has come true: that we have a new pace and standard for meaningful advances in improving quality of life for patients in developed and developing countries, with and without inhibitors. At the time, it seemed like a lot to ask, but I hope we were right about the capability of the bright minds in the companies that seemed almost ready to release—and community members that seemed almost ready to embrace—a revolutionary, game-changing product. Are you still as fond of analogies? Introduction of the iPhone, going to Mars, you couldn't quite decide which analogy would do justice, but I hope it has been all of those things and more.

Have we progressed, from only 20% of hemophilia patients treated globally to at least 50% now? I sure hope so. If not, please send word back to me in 2016 from your iPhone 16s, and I will rattle some extra cages for you.

Keep up the good fight,

*Ian of 2016*

*Ian is a 31-year-old who has severe hemophilia A, and has thankfully been in remission for hepatitis C genotype 1A for the past 12 years. Ian graduated from California Polytechnic State University, San Luis Obispo, and currently works in Cambridge, Massachusetts, as an IT and informatics strategy consultant for early-stage biotechnology startups. He lives in Arlington, Massachusetts, with his fiancée Katie and their adorable mutt, Charlotte. Ian enjoys rock climbing, riding his road bike, and running outside on sunny days. He hopes to participate in bringing about the next era of hemophilia treatment for his friends with inhibitors and those with inadequate access to factor around the world.*

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# Hero Hemophilia Walk 2016



**Baxalta**

## 2016 NEBRASKA WALK FACT SHEET

- WHAT:** The Hemophilia Walk is NHF's largest event dedicated to finding better treatments and cures for bleeding and clotting disorders, and to preventing the complications of these disorders through awareness, education, advocacy and research.
- WHY:** Established in 1948, NHF is the premier organization in the U.S. serving the bleeding and clotting disorders community. Now in its 65th year, NHF continues to be a vital resource for prevention education and provides a network of support for Americans living with bleeding disorders and their families.
- WHEN:** **Saturday, September 24th, 2016**
- WHERE:** **Chalco Hills Recreation Area**— 8901 So. 154th Street, Omaha, NE 68164  
*Check-in begins at 9:00am / Walk begins at 10:00am*
- DISTANCE:** 1 mile, 4 or 6 mile route options
- HOW:** Sign up for the Walk online by visiting [www.hemophilia.org/walk](http://www.hemophilia.org/walk). Click the NE icon and register or gather information! Everyone is welcome to participate!
- VOLUNTEER:** Help us make our event a success. Volunteers are appreciated before and/or during the Walk. Unable to do the Walk but still want to participate? Enlist as a Volunteer. Contact Maureen Grace, Interim Executive Director at 402.499.8025.
- CONTACT:** Maureen Grace, Interim Executive Director, Nebraska Chapter of the National Hemophilia Foundation 402.499.8025 or [mgrace@hemophilia.org](mailto:mgrace@hemophilia.org)

# How to be a super fundraiser!

It can be challenging to ask for donations, however you never know what one might say or give for that matter until you ask. We hope the information provided about fundraising in the workplace, utilizing social media and how to raise \$500 in 7 days are beneficial in your fundraising efforts.



## Workplace Fundraising

Organize some fun activities to raise funds at your office— here are a few examples but limit yourself to other options.

1. Jeans Day/Dress Down Day: Arrange with your employer a Jeans Day or Dress Down Day for a donation of \$5 to the Hemophilia Walk.
2. Food/Beverage Sale: For a donation, sell cookies, donuts, bagels, popcorn etc.
3. 50/50 Drawing— Sell tickets and the winner gets half of the money received while you keep the other half for the Hemophilia Walk.
4. Coin War: If you work in a office with several departments start a little competition by placing jars in the office for “extra change” and compete to raise the most money.
5. A Day of Silence for the Hemophilia Walk: Accept donations to silence an “exuberant” employee for the day.

## Utilizing Social Media

Here are a few suggestions you can use social media to increase your Hemophilia Walk Fundraising!

1. Use your status updates to motivate and fundraise. Share your progress and be sure to give the link to your fundraising page.
2. Say thanks & get sponsors— when someone makes a donation to your page, make a big deal out of it.
3. Create a Team Page— Encourage your team captain and help with the efforts.
4. Download the new Hemophilia Walk App for your smart phone! Fundraising has never been so easy!
5. Retweet! Become a follower of the Nebraska Chapter and the National Hemophilia Foundation.
6. Have a Facebook Fundraising Sale— Facebook allows you to sell stuff by “Selling for a Cause.”

## HOW TO RAISE \$500 IN 7 DAYS!

1. Sponsor yourself first:  
**\$50.00**
2. Ask 4 family members to sponsors for \$25 each:  
**\$100.00**
3. Ask 5 co-workers to contribute \$15 each:  
**\$75.00**
4. Ask 5 friends to contribute \$15 each:  
**\$75.00**
5. Ask 5 neighbors to sponsor you for \$15 each:  
**\$75.00**
6. Ask your boss for a company contribution of \$50;  
**\$50.00**
7. Ask 3 businesses you frequent for a donation of \$25 each:  
**\$75.00**

**Add it all up and what do you get?**

# **\$500.00**

Who else could you ask? Here are some ideas.....

Mother, Father, Brother, Sister, Cousins, Uncle, Aunt, Doctor, Neighbor, Roommates, Pharmacist, Accountant, Landlord, Dentist, Hair Stylist, Dry Cleaner, Banker.....the list goes on and on. Remember, you never know until you ask.



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### Indications

ELOCTATE [Antihemophilic Factor (Recombinant), Fc Fusion Protein] is a recombinant DNA derived, antihemophilic factor indicated in adults and children with Hemophilia A (congenital Factor VIII deficiency) for: control and prevention of bleeding episodes, perioperative management (surgical prophylaxis), and routine prophylaxis to prevent or reduce the frequency of bleeding episodes. ELOCTATE is not indicated for the treatment of von Willebrand disease.

### Important Safety Information

Do not use ELOCTATE if you have had an allergic reaction to it in the past.

Tell your healthcare provider if you have or have had any medical problems, take any medicines, including prescription and non-prescription medicines, supplements, or herbal medicines, have any allergies, are breastfeeding, are pregnant or planning to become pregnant, or have been told you have inhibitors (antibodies) to Factor VIII.

Allergic reactions may occur with ELOCTATE. Call your healthcare provider or get emergency treatment right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash, or hives.

Your body can also make antibodies called, "inhibitors," against ELOCTATE, which may stop ELOCTATE from working properly.

Common side effects of ELOCTATE are joint pain and general discomfort. These are not all the possible side effects of ELOCTATE. Talk to your healthcare provider right away about any side effect that bothers you or that does not go away, and if bleeding is not controlled after using ELOCTATE.

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

**Please see Brief Summary of full Prescribing Information on the next page.**

**This information is not intended to replace discussions with your healthcare provider.**



**Biogen.**

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## **FDA-Approved Patient Labeling**

### **Patient Information**

#### **ELOCTATE™ /el' ok' tate /**

#### **[Antihemophilic Factor (Recombinant), Fc Fusion Protein]**

Please read this Patient Information carefully before using ELOCTATE and each time you get a refill, as there may be new information. This Patient Information does not take the place of talking with your healthcare provider about your medical condition or your treatment.

#### **What is ELOCTATE?**

ELOCTATE is an injectable medicine that is used to help control and prevent bleeding in people with Hemophilia A (congenital Factor VIII deficiency).

Your healthcare provider may give you ELOCTATE when you have surgery.

#### **Who should not use ELOCTATE?**

You should not use ELOCTATE if you had an allergic reaction to it in the past.

#### **What should I tell my healthcare provider before using ELOCTATE?**

Talk to your healthcare provider about:

- Any medical problems that you have or had.
- All prescription and non-prescription medicines that you take, including over-the-counter medicines, supplements or herbal medicines.
- Pregnancy or if you are planning to become pregnant. It is not known if ELOCTATE may harm your unborn baby.
- Breastfeeding. It is not known if ELOCTATE passes into the milk and if it can harm your baby.

#### **How should I use ELOCTATE?**

You get ELOCTATE as an infusion into your vein. Your healthcare provider will instruct you on how to do infusions on your own, and may watch you give yourself the first dose of ELOCTATE.

Contact your healthcare provider right away if bleeding is not controlled after using ELOCTATE.

#### **What are the possible side effects of ELOCTATE?**

You can have an allergic reaction to ELOCTATE. Call your healthcare provider or emergency department right away if you have any of the following symptoms: difficulty breathing, chest tightness, swelling of the face, rash or hives.

Your body can also make antibodies called, "inhibitors," against ELOCTATE. This can stop ELOCTATE from working properly. Your healthcare provider may give you blood tests to check for inhibitors.

Common side effects of ELOCTATE are headache, rash, joint pain, muscle pain and general discomfort.

These are not the only possible side effects of ELOCTATE. Tell your healthcare provider about any side effect that bothers you or does not go away.

#### **How should I store ELOCTATE?**

- Keep ELOCTATE in its original package.
- Protect it from light.
- Do not freeze.
- Store refrigerated (2°C to 8°C or 36°F to 46°F) or at room temperature [not to exceed 30°C (86°F)], for up to six months.
- When storing at room temperature:
- Note on the carton the date on which the product is removed from refrigeration.
- Use the product before the end of this 6 month period or discard it.
- Do not return the product to the refrigerator.

Do not use ELOCTATE after the expiration date printed on the vial or, if you removed it from the refrigerator, after the date that was noted on the carton, whichever is earlier.

After reconstitution (mixing with the diluent):

- Do not use ELOCTATE if the reconstituted solution is not clear to slightly opalescent and colorless.
- Use reconstituted product as soon as possible.
- You may store reconstituted solution at room temperature, not to exceed 30°C (86°F), for up to three hours. Protect the reconstituted product from direct sunlight. Discard any product not used within three hours.

#### **What else should I know about ELOCTATE?**

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

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## Eeny, Meeny, Miny, Mo...

## Choosing Factor? What Do You Know?

Laurie Kelley

Last year we invited patients via email to complete a survey. We asked specifically for people who used only plasma-derived factor. Lots of patients replied, but most used recombinant. Had we phrased the invitation correctly? One young guy with hemophilia even asked, “What’s the difference?” I answered, “Yikes.”

If you aren’t sure what type and brand of factor you’re using and why, how will you be able to successfully choose from the plethora of factor brands coming your way? As PEN’s feature article reveals, we have many products to choose from now, and more in the pipeline.

The good news: lots of factor to choose from.

The bad news: lots of factor to choose from.

It’s up to YOU to know what you are injecting into your (or your loved one’s) veins. Sure, you can ask your hemophilia treatment center (HTC) hematologist, who is well versed in product choice, to help you decide what product to use. But if you’re a parent or young person with hemophilia, don’t you want to participate in making the decision? Use the talking points in this guide to review your choices with your HTC, and to prepare for choices in the future.

### It Takes Two, Baby

If you remember nothing else, please remember that you can use only one of two types of factor products: (1) *plasma derived* or (2) *recombinant*. Choose one or the other. (Well, some people may use both, but that’s probably an article for our Insights column).

What’s the difference between the two? Plasma-derived factor comes from...plasma! It originates from the plasma of up to 60,000 blood donors, which is pooled together and then processed. Recombinant factor is made in the lab, from the human gene that produces factor, which is “recombined” or inserted into the DNA of a host cell (often a hamster cell) to produce human factor. That human factor-producing gene is spliced into the cell, and popped into a large tank called a bioreactor to keep it fed and alive. Then the cells grow to large numbers, and go to work producing factor—without ever having seen or touched blood.

So one factor product type originates from blood, and the other from the lab. Blood versus lab. Which type is your product? And why are there two types, anyway?

Plasma-derived factor concentrate was developed first, in 1968. But as you may (and should) know, it was vulnerable to transmitting human viruses from the donors because it was not subjected to any method of viral inactivation. In 1985, in the wake of the HIV epidemic, new manufacturing processes were implemented to kill most blood-borne viruses, such as HIV. Then in 1992, in response to the bleeding disorder community’s demand for greater safety, recombinant factor was developed and commercially released as a virus-free source of factor. It doesn’t originate from blood.

Must know: Since 1987, no US FDA-approved factor product has transmitted hepatitis C or HIV. All factor distributed in the US is considered safe, whether it’s plasma-derived or recombinant. For the record, National Hemophilia Foundation’s (NHF) Medical and Scientific Advisory Council (MASAC) recommends using recombinant factor.



## Safety versus Purity

Parents and patients often confuse *safety* and *purity* of factor products. You need to know that safety and purity are not the same, and why.

Purity is a measure of the presence of other proteins, sometimes including other clotting factors, in addition to the specific factor supplied in the concentrate.

Safety is the removal or inactivation of potentially harmful substances, including blood-borne viruses, from factor concentrate.

So purity refers to how much of your factor concentrate contains just factor, with no other proteins. Safety refers to reducing the risk of viral transmission.

I sometimes think of it like a choice of drinking water source. US tap water is generally safe to drink (unless, sadly, you live in Flint, Michigan). But it isn't that pure: it contains minerals, metals, and possibly chlorine and fluoride. Bottled water is safe and of higher purity: it still contains minerals, but other compounds such as chlorine and fluorine are removed. Distilled water is both safe and pure: it contains no minerals or other compounds.

Because of varying production methods and based on the source of the factor, the relative purity of the final products—whether plasma-derived or recombinant—varies. Plasma-derived products are classified as intermediate purity, high purity, and ultrapure or monoclonal. The intermediate purity products are still pure (and all are safe), but some are more pure than others. Recombinant products are the purest of the factor concentrates.

## Talking 'Bout My Generation

Recombinant products are all über pure. Beyond purity, they're further classified by *generation*. Generation refers to when the products were first commercially available, but also to the presence of animal or human proteins used in the production process or the final product. Yeah, even though I just said that recombinants are produced in a lab, and they don't come from human blood...a blood product may be put into the finished product to stabilize the factor and add bulk. Generation, then, relates to how the product is manufactured.

First-generation recombinant products were introduced in 1992 (points if you know which product was first!). They use human or animal proteins in the growth medium—the serum used to feed the host cells that produce the factor. These products also contain human albumin<sup>1</sup> added at the final production stage to help stabilize and bulk up the product. So they don't come from human blood, but they do contain a human blood product added in the manufacturing process.

Second-generation recombinant products, introduced in 2000, contain no human albumin added to the final product, but like first-generation products, they still use human or animal proteins in the growth medium.

Third-generation recombinant products, first available in 2003, contain no human or animal proteins in the growth medium or added to the final product. Because they are not exposed to any animal or human proteins outside of the manufacturing process, they have no risk of transmitting blood-borne viruses.

So if you use a recombinant product, which “gen” is it?

Continued on next page

# Continued...You sponsored by Baxalta

## **New Kids on the Block: Prolonged Half-life**

Finally, we have what so many families were waiting for: prolonged half-life products. Because they last longer in the bloodstream, they should require fewer infusions per week or possibly only a single infusion to treat a bleed. But read up: some prolonged half-life factor VIII products have only a marginally longer half-life, and may require the same dosing schedule you are currently using. One prolonged factor IX product has a significantly longer half-life, allowing you to infuse less often.

Prolonged half-life products are recombinant. They are still subject to all the criteria of purity and safety. They are exciting. They might be more expensive. Be sure to check your insurance coverage before switching to any product. Your HTC team should always be involved in any product choice discussion, for medical, safety, and insurance considerations.

There you have it in a nutshell. The Hitchhiker's Guide to the Galaxy of Factor Products. Please know your product: What's its name? Why do you use it? Who selected it? Might another product better meet your needs? What are your personal needs, anyway? Which products does your health insurance cover, and what's your copay?

So many choices. But there's only one *you*, and you'll want to decide on the right factor for you. We've only scratched the surface here, but remember, you're injecting this factor into your own or your child's vein. Know what you're putting in there. Bring this article to your HTC for a good, long chat about products...and about product choice.

## **Ask this (with your HTC team):**

- Do I want a plasma-derived or recombinant product?
- If recombinant, what generation is the product?
- Which product does my (or my child's) doctor recommend? Why?
- What is the viral inactivation process for the product?
- What is the purification process for the product?
- Does the product have a prolonged half-life?
- Is my child (or am I) at risk of an inhibitor?
- Do I need an intermediate or high-purity product?
- Does the assay size range meet my (or my child's) needs?
- Is the product covered by my insurance policy?
- How will the price per unit affect my out-of-pocket costs?

## **Know this (with or without your HTC team):**

- All US FDA-approved factor concentrates are considered safe.
- No plasma-derived US factor concentrate has transmitted hepatitis C or HIV since 1986.
- Recombinant factor products are generally more expensive than plasma-derived products.
- NHF's MASAC recommends recombinant factor concentrate for hemophilia treatment.

1. Albumin (or human serum albumin) is a protein found in blood plasma that makes up about 50% of plasma proteins. In some brands of recombinant factor concentrate, albumin is added to the final product as a stabilizer or used in the production process.

# Inhibitor Summit



The National Hemophilia Foundation (NHF) is recruiting participants for their Inhibitor Summit in St. Louis, Missouri for families impacted by hemophilia with inhibitors, August 18-21. There are a limited number of spots available. If you're interested in attending this educational summit visit:

<https://www.hemophilia.org/Events-Educational-Programs/Inhibitor-Education/Inhibitor-Education-Summits>

These Summits will provide a dynamic weekend of inhibitor-related education alongside other inhibitor families.

- Is available to anyone with hemophilia A or B who currently has or once had inhibitors
- Caregivers that live in the same household as the person with an inhibitor (active or tolerized)
  - Provides complimentary travel, hotel accommodations and registration are provided
- This includes such expenses as tolls, mileage, airfare and hotel accommodations. Some meals will be provided

## Scholarship Opportunities

### Josh Gordy Educational Scholarship

If you're entering or attending a 2- or 4- year undergraduate institution in the US for the 2016-2017 school year and have hemophilia, you are eligible for the Josh Gordy Educational Scholarship. The scholarship was created by Josh Gordy, a National Football League free agent in honor of his nephew, Nolan, who has severe hemophilia A. This year, three \$1,000 scholarships are awarded to eligible students. Deadline is Friday, August 5, 2016. For more information about this scholarship please visit:

<https://www.hemophilia.org/Community-Resources/Scholarships/Josh-Gordy-Educational-Scholarship>

### Factor One Source Pharmacy Scholarship

To apply, applicants must send the following:

- Photograph of applicant (must own the copyright) \*
- 1-2 page letter of intent and/or qualifications
- Resume
- Letter of recommendation

All items are to be sent to [contact@fosrx.com](mailto:contact@fosrx.com) as attachments in one combined email by July 31, 2016. Applications will be reviewed by an internal committee. Applicants will be notified in August if they have been selected. Full eligibility details can be found on our website: <http://fosrx.com/wp/scholarships/>



# SIPPET Study

## SIPPET Study Results Published in NEJM

The detailed findings of the much anticipated SIPPET (Survey of Inhibitors in Plasma-Products Exposed Toddlers) study were published today, May 26, 2016. The study, “A Randomized Trial of Factor VIII and Neutralizing Antibodies in Hemophilia A,” appeared in The New England Journal of Medicine. The lead investigator was Flora Peyvandi, MD, University of Milan.

Peyvandi and fellow investigators found that previously-untreated patients (PUPs) had a significantly higher incidence of inhibitors when treated with recombinant factor VIII (rFVIII) than those treated with plasma-derived factor VIII (pdFVIII) containing von Willebrand factor (VWF). Developing an inhibitor to treatment remains the most prominent and challenging complication for clinicians, occurring in approximately 30% of hemophilia patients globally.

Back in December, a preview of the SIPPET findings presented during the American Society of Hematology’s annual conference raised considerable interest among patients, providers and industry. While earlier studies have assessed the overall risk of inhibitor development in patients with hemophilia, the SIPPET study is the first large-scale international trial to randomize patients prospectively for the immunogenicity of pdFVIII vs. rFVIII usage.

SIPPET was a prospective randomized study which took place between January 2010 and December 2014 and collected data on 251 children (<6 years of age with severe hemophilia A) from 42 sites in 14 countries in Africa, North and South America, Asia and Europe. The authors reported that rFVIII was associated with an 87% higher incidence than pdFVIII. Half of the patients were randomly assigned to receive either pdFVIII or rFVIII. The authors reported an overall inhibitor incidence rate of 26.8%.

The National Hemophilia Foundation’s Medical and Scientific Advisory Council (MASAC) will be reviewing the full study, making a thorough assessment of these findings and best determine what changes may be needed to the current MASAC recommendations for PUPS.

Source: New England Journal of Medicine, original article, published May 26, 2016



# Kid's Corner

## Kids Who Volunteer Make a Difference



Illustration by John Haslam

Volunteering and helping other people makes you feel good about yourself. No matter how old you are or what type of bleeding disorder you have, you can make a difference.

If you want to volunteer, begin by asking yourself some questions: Do I want to help kids or older folks? Do I want to help raise money for a good cause, like a hemophilia walk? Could I help at an animal shelter or pet rescue? Next, talk with your family about what's available in your community.

Search online for groups that can help you find the right fit for volunteering. GenerationOn helps organize local Kids Care Clubs for elementary and middle school children. Clubs are formed at schools, houses of worship and other community-based organizations. Learn more at: [generationon.org/kids-care-clubs/about](http://generationon.org/kids-care-clubs/about). Visit a local school, pet shelter or nursing home to see what kind of help they need. There are lots of ways to get involved in

your community. Just find a cause that means something to you and get started.

### 10 to Try

Here are some other ideas for volunteering:

1. Read books to people who are visually impaired through a community organization.
  2. Make care packages for the homeless.
  3. Help adults pack donations of clothes, books and toys.
  4. Present a talent show with your friends at a local nursing home.
  5. Offer to do yard work for a neighbor.
  6. Run a lemonade stand and donate your earnings to a charity.
  7. Pitch in to clean up a local park or beach.
  8. Organize a penny drive with your friends and neighbors. Donate the money to a local charity.
  9. Make handmade cards to send to people in a nursing home or soldiers serving far away. Your cheery surprise could make someone's day.
- Start a club at your school to help stop bullying.



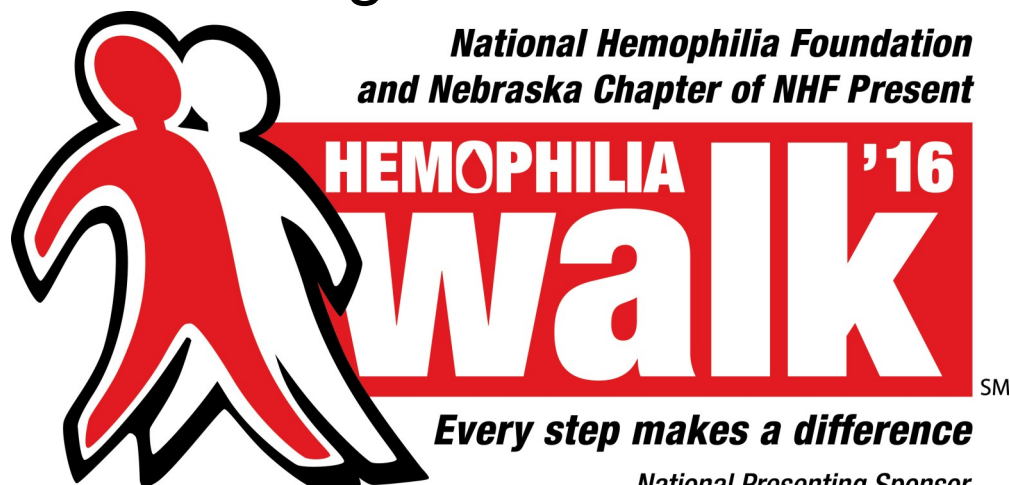
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Saturday, September 24, 2016  
Register Now!



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