



Hemophilia Outlook

A Newsletter of

The Hemophilia Association of New York, Inc.

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Pennies and Checks

By: Linda Mugford

Since 1952 the Association has depended and thrived on donations from corporations and individuals. In the past, there used to be volunteers who would stand on the corners of New York City and ask for donations (similar to the Salvation Army during the Holidays) pennies dropped in a can and checks from foundations are the foundation of our fundraising.

The Association is always seeking funding to support our efforts. At this time I thought that you would like to know what programs and services the Association provide with those "Pennies and Checks".

Since 2014

HANY has held **134 meetings** which have included: Blood Brotherhood events; Women's Meetings; Educational Meetings; the Night at the Museum event; our weekend Teen conference, S.A.I.L; our Steven L Margolies, MD Family Educational Conference; and Parent meetings.

The Association has received and acted on 325 individual requests for financial assistance in the areas of: Non-covered medical expenses; Insurance premiums; Emergency assistance for situations deemed necessary; and Medic Alerts (these requests number 60 so far this year!). **Over \$29,000 has been for healthcare costs. Other special services have exceeded over \$27,000.**

In the past five years **the Association has also provided almost \$8,000 in travel assistance** for appointments and camp.

Since 2014 there have been 98 Scholarships awarded to those with bleeding disorders or who are the children of those with bleeding disorders. These awards total \$258,475.

The Association accepts research proposals for consideration on an ongoing basis. **Since 2014, \$310,000 has been awarded for Research Projects.**

The Hemophilia Association's Board of Trustees and Staff are very proud of the **\$633,100** that has left the Association to assist, aid, and educate the members of the Bleeding Disorders Community which we serve.

HANY SCHOLARSHIP

A total of 13 applicants will receive awards through our scholarship program. This year's scholarship awards totaled \$50,000.

The scholarship ceremony for awardees will be held on Tuesday, July 23, 2019 at HANY'S main headquarters, located at 131 W 33rd ST Suite 11D.

For information about the upcoming 2020 scholarship program look out for the Fall 2019 newsletter.



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WHERE DOES YOUR FACTOR COME FROM?

By: Laurie Kelley

You may know the brand name of the factor concentrate your child or other loved one uses to treat bleeds. And you may have chosen the brand with the help of your hematologist. But where do you get your factor? Who provides it? Is your current brand the best way to meet your personal needs? Do you have choice of provider?

Pharmaceutical companies develop and manufacture factor. Then they sell the factor to a licensed pharmacy—a factor provider. You can't buy factor directly from the manufacturer, just as you can't buy a car directly from General Motors, or diapers from Kimberly-Clark. And you can't get factor from your local drug store. Your hematologist supplies a prescription to a factor provider, who delivers it to you. Who are factor providers?

Hospital Pharmacies

You want a factor provider that can meet your personal needs; this usually means being cost-effective and speedy, and supplying factor in the correct assay sizes with all the ancillaries (such as needles and syringes) you require. Unless you are a member of a health maintenance organization (HMO) and are required to buy factor from the hospital pharmacy, or your hospital runs a 340B program, obtaining your factor through a hospital pharmacy is usually not a good option. Why not? Hospital pharmacies are the least cost-effective factor provider, and often mark up the cost of factor several hundred percent to cover the high overhead costs of running the hospital. Also, hospital pharmacies are not set up for home delivery and unlike specialty pharmacies, do not offer any additional services, such as a home nurse. Factor is already very expensive without the hospital markup! You'll want a long-term solution, with a factor provider that ships to your home.

Specialty Pharmacies

Specialty pharmacies are one of the chief factor providers in the US. If your insurance payer approves a specialty pharmacy based on your

physician's prescription, you make a phone call, order your factor, and receive the order at your home within 24 to 48 hours, along with all necessary ancillaries and supplies. Reimbursement specialists handle your insurance paperwork. Specialty pharmacies stock most brands of factor, and usually can provide a size or assay that closely mirrors what you need for your child's infusions. Some specialty pharmacies will send a nurse to your home to perform or assist in the infusion process. There are many specialty pharmacies and home care companies that service hemophilia, and some are devoted only to hemophilia.

Your HTC

Did you know your hemophilia treatment center might sell factor? There are about 140 HTCs in America as of this writing, and over 100 participate in the 340B program; all are licensed distributors of factor. So you also have the option—if your payer permits—to purchase factor from your HTC. Why and when would you consider buying from your HTC? Federally funded HTCs can take advantage of the federal Public Health Service (PHS) Act known as the 340B Drug Pricing Program. The PHS Act allows certain federally funded entities and public hospitals to purchase prescription outpatient drugs (including factor) at steeply discounted prices. So federally funded HTCs can buy factor from pharmaceutical companies at rock-bottom prices, and then sell it to you and make a profit.

In theory, 340B pricing is beneficial. It offers competition to help keep prices down, reduces costs for the government, and generates funds for the HTC to use for staff positions or overhead—which is truly needed. But not every eligible HTC uses the 340B program. And even when an HTC does offer factor through 340B, not all the HTC's hemophilia consumers take advantage of this. Why? Sometimes, 340B pricing doesn't guarantee lower prices to the consumer: some HTCs charge the same price per unit as specialty pharmacies. And some consumers simply prefer the personal relationship they have with their specialty pharmacy reps.

PBM Pharmacies

Pharmacy benefit managers (PBMs) are powerful, multi-billion-dollar companies hired by insurance

companies to manage the insurance benefits and prescription drug plans of private-sector entities, such as employers and labor unions. PBMs help determine the formulary—a limited list of preferred drugs that the payer will reimburse. PBMs also negotiate and manage contracts with pharmaceutical companies to buy the drugs needed by plan beneficiaries like you. The main function of a PBM is to keep prescription drug costs low for the insurance company.

PBMs are able to make high-volume drug purchases to receive substantial discounts from pharmaceutical companies. With their vast resources and negotiating skills, PBMs such as Express Scripts and CVS Health now serve most of the hemophilia patients in the US. Some PBMs have started their own specialty pharmacies to sell factor; and because they have a direct line to the payer, these PBMs are able to switch families from the factor provider of their choice to the PBM's specialty pharmacy. They have incredible power over pricing, product availability, and your payer.

Based on this, can you even choose a factor provider? Unfortunately, your healthcare payer—insurance company or government program—often chooses for you. Find out if your insurance company reimburses for specialty pharmacy services. Then, learn which companies are in-network for you. Your choices might be limited, because for the payer, working with a single factor provider is one way to lower costs. More and more often, choice is being restricted. You may face a struggle when choosing a preferred factor provider.

If you can choose, use this list of questions to ask your factor provider to make sure your personal needs are met:

- Which brands of factor concentrate do you provide?
- How much product will you provide at one time?
- How are products delivered to me?
- Do you ship during emergencies?
- Do you supply the assay size I need as a single dose?
- How much will I pay per unit of product?
- Do you (the HTC) offer 340B pricing?

- Are you recognized as an in-network provider by my insurance company?
- What are your hours of operation?
- Are a pharmacist and registered nurse available 24/7?
- Can I use your regular HTC services even if I choose to use a specialty pharmacy as my factor provider?
- Do you supply ancillaries: needles, syringes, and bandages?
- Do you provide needle disposal containers?
- Do you contract with local home nursing services?
- Is home nursing service included in the cost of product or billed separately?

Even though choice is being limited, you are not limited! Learn all you can about who supplies your factor, and continue to safeguard your needs. Ask questions, and get the answers that will help you make effective decisions.

FDA Finalizes Guidance on Developing Drugs to Treat Severe Hematologic Disorders

By: FDANEWS Drug Daily Bulletin

The FDA issued final guidance for developers of drugs to treat severely debilitating or life-threatening hematologic disorders (SDLTHDs), such as hemophilia and sickle cell disease.

Commissioner Scott Gottlieb noted the guidance is intended to streamline the development of pharmaceuticals used to treat patients with SDLTHDs other than cancer, while protecting patients' safety and avoiding the unnecessary use of research animals.

In vitro and/or in vivo proof-of-concept studies should be conducted prior to clinical trials to evaluate the mode of action and effects of the drug related to its intended therapeutic effect, the agency says.

An assessment of the drug's potential effect on vital organs—such as the respiratory, cardiovascular and central nervous systems—should be completed before beginning clinical trials. “Detailed clinical observations following

dosing and appropriate electrocardiographic measurements in nonrodents are generally considered sufficient,” the agency says, noting that stand-alone safety pharmacology studies aren’t needed.

Educating, Equipping, and Empowering Women with Hemophilia

By: Shellye Horowitz

Inspired to sponsor a national conference for women with hemophilia types A and B, The Hemophilia Foundation of Michigan (HFM) became a game changer last November when it hosted an event called “Being Visible.”

Gathering 103 women representing 32 states, the event featured physicians and researchers, conference attendees, and presenters, who united to discuss key issues affecting women with hemophilia and to disseminate critical information. Physicians and researchers volunteered their time to fly to Michigan to share their knowledge.

Recently, I spoke with HFM’s executive director, Susan Lerch. I wanted to learn the story behind the conference and Susan’s thoughts on the future for women with hemophilia.

Conference Inspiration

Susan said that many women inspired her desire to host the conference. She witnessed the struggles of women like Shelley Gerson, who has hemophilia type A, and Shari Luckey, who has hemophilia type B, and she saw the work of doctors like Roshni Kulkarni, who is passionate about disseminating information to assist women with bleeding disorders. Susan understood the power of bringing women and physicians together to seek understanding and motivate change. It was a previously unfilled void.

Physicians with the knowledge of how to diagnose and treat hemophilia types A and B needed to be paired with women desperate to understand their bleeding issues, she said. Combining the critical promulgation of information with community

building was key. HFM wanted to facilitate this linkage. Susan felt comfortable moving forward knowing that Patrice Thomas, HFM’s program services director, was on board and would be dedicated to making the conference happen.

Missing Services

When asked what services are missing for women with hemophilia, Susan responded, “Everything.” She noted, however, that some places do exist where women can get treatment. “I am so proud of the women who are vocal and addressing the issues,” she said. “If we can get their voices heard it will make an impact on long-term care.”

Susan has a deep desire to see women empowered. “Dynamic women are going to have to educate their healthcare providers. These providers will then have to educate their colleagues. Medical schools and emergency rooms need information about women with bleeding disorders. They need to pay attention and not disregard bleeding issues,” she said. Susan wants women with hemophilia and those who are diagnosed as “symptomatic carriers” to know that, “We hear you and we want to provide you with the information you need to get the care and support you need.”

In the future, women need to be able to benefit from all of the new hemophilia treatments coming to market. Understanding the importance of conducting research on effective treatments for women with hemophilia will be critical. This is necessary for women to be equally recognized as people with hemophilia, as well as attaining proper diagnosis and accessing appropriate care. Too often women are not treated respectfully and must wait years to obtain a proper diagnosis.

Barriers and Misconceptions

When asked about the term “symptomatic carrier,” Susan said she is “not a fan.” It is way too confusing and easily misinterpreted or misunderstood by medical providers, she said. Susan also sees a problem with a lack of consistent, innovative, patient-focused healthcare for women and girls with bleeding disorders. She believes that women with hemophilia need access

to leaders in the field who want to listen. Stories of late diagnosis and lack of treatment need to be told to motivate changes in care.

Gene therapy is an amazing advancement for men with hemophilia, possibly even curative. But Susan worries about what will happen when “cured” men pass hemophilia along to their daughters. Will obligate carriers access the treatment they need or be lost? As a person with a young granddaughter who is a carrier, Susan has both a personal and professional pull to be a change agent.

Misconceptions about hemophilia abound. Many people believe that only men can have it, and numerous women are told their excessive bleeding is normal. When crying out for help, they are labeled “hysterical.” Women are struggling with real issues and a disease that goes undiagnosed for years. These affected women need support. They must be educated, equipped, and empowered to advocate for the care they need and deserve.

Long-term Impact

The impact the National Conference for Women with Hemophilia had on attendees was incredible. They reported a sense of empowerment after the conference. Women are finding their voice in the fight to ensure that women with hemophilia obtain proper medical care. For Susan, this was the conference’s highlight.

“I feel very grateful to the women who came and the presenters who gave their time,” she said. “We had the privilege of starting a movement.”

Starting a movement they did. This is, and will continue to be, incredibly significant in the hemophilia community.

PAST EVENTS

March 29 - 31 – Self Advocacy & Independence Training (S.A.I.L)



*HANY teamed up with GutMonkey and held our very first Parent/Teen retreat at Camp Quinipet. The event was a success! We are grateful for the support of our sponsors **Genentech, Takeda, Pfizer & CSL***

May 19 - DAY AT THE RACES



*Thank you to all of the families who attended & a special thank you to HFA for our crafty Art therapy session with Susan Bifano. We are grateful for our sponsors **Kedrion, Bayer, Takeda, CSL, & Octapharma***

**Hematology and Pregnancy:
Problems and Issues
(H.A.P.P.I)**



On June 4, 2019 HANY Partnered with Albert Einstein Medicine College to provide a symposium for Medical Professionals (Including C.M.E). Dr.Henny Billet arranged the speakers and program. Topics included: Managing the Pregnant Patient with Sickle Cell Disease; Evaluation and Management of Thrombosis in Pregnancy; From Pre-eclampsia to HELLP to aHUS and Beyond: TMA in Pregnancy; Von Williebrands Disease and other Bleeding Disorders in Pregnancy; and Iron Deficiency in Pregnancy.

PATIENT CO-PAY ASSISTANCE PROGRAMS

Patient assistance programs are offered by medicine manufacturers. These programs offer assistance with co-pays and deductibles. If you would like information about these programs, please contact your hemophilia treatment center or HANY at 212-682-5510.

IT IS MANDATORY TO REGISTER EVERY YEAR FOR YOUR SPECIFIC ASSISTANCE PROGRAM

HANY UPCOMING EVENTS

July TBD – Outside the Clinical Box

July 21 - HANY 67th Anniversary Gala at Yankee Stadium - Yankees VS. Colorado Rockies

July 23 – Scholarship Reception

September TBD – Blood Brotherhood

Sept/Oct TBD – Women’s Group

December 13 - 15 – 4th Annual Steven L. Margolies Family Retreat at Mohonk

JUNE 7 - “A NIGHT AT THE MUSEUM”



Children ages 6-13 were able to spend a night at the Museum of Natural History & participate in a physical therapy session led by Eileen San Juan, DPT

The 12th Annual Matthew L. Greer Liberty Mutual Golf Classic

On June 17th, the 12th Annual Matthew L. Greer Liberty Mutual Golf Classic was held at the Willow Ridge Country Club in Harrison, NY. More than 130 people attended for a day full of golf and fun!

A special thanks to all of our sponsors for their generous support!
Takeda, CSL & Bayer

New name, stronger commitment.

Over the years, Baxalta and Shire have provided products and services that have stayed in step with the changing needs of the bleeding disorders community. And now as Takeda, we are committed to building on that legacy. We've got a new name, but our commitment to you is stronger than ever.

Takeda Baxalta and Shire are now part of Takeda

Alex Hemophilia A Massachusetts
bleedingdisorders.com

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Special Considerations for Girls

The Onset of Menstruation May Be a Time to Focus on a Hemophilia Care Plan



For more information, visit b2byourvoice.com to download *Hemophilia B: Her Voice, Her Life*.

This content is brought to you by Pfizer.

Puberty on its own can be a difficult experience, but it can become even more complicated with a hemophilia diagnosis. The start of menstruation is one of the many aspects of a girl's life that may be affected by a bleeding disorder. For girls who are showing symptoms of hemophilia, puberty may be a good time to identify a health care team and develop a care plan.

Identifying a Health Care Team

The lack of knowledge about how hemophilia impacts girls can affect the level of medical care and emotional support received by a girl with this condition.¹ It may be recommended that a girl who is diagnosed with hemophilia wear a medical identifier at all times so that medical personnel are aware of her bleeding disorder in an emergency. It is important for every female with hemophilia to enlist a team that includes a primary care physician, a gynecologist, and a hematologist who can coordinate care and needs.²

Tools that can help girls manage symptoms include³:

- Care plans designed for patients by their team of health care providers to help facilitate care coordination
- Apps that allow patients to track their hemophilia symptoms and care
- Self-monitoring assistance for better symptom accuracy

Heavy Menstrual Bleeding

Periods with heavy blood loss (called *menorrhagia*) can lead to anemia and have a negative effect on quality of life. Girls with bleeding disorders who are experiencing symptoms of menorrhagia should have a discussion with their health care team in order to coordinate management and care.⁴

References: 1. Aldridge S. The carrier barrier: women push for mild hemophilia diagnosis. HemAware.org Web site. www.hemaware.org/story/carrier-barrier. Published July 19, 2012. Accessed February 20, 2019. 2. Canadian Hemophilia Society (CHS). Precautions for pregnant women with a bleeding disorder. Hemophilia.ca Web site. www.hemophilia.ca/en/women/precautions-for-pregnant-women/. Accessed February 20, 2019. 3. Carr S. A new look at patient communications in outcomes-driven healthcare. Pharmaphorum.com Web site. http://pharmaphorum.com/views-and-analysis/a_new_look_at_patient_communications_in_outcomes-driven_healthcare/. Published November 5, 2012. Accessed February 20, 2019. 4. National Hemophilia Foundation (NHF). Effects of puberty on girls with a bleeding disorder. StepsforLiving.Hemophilia.org Web site. <https://stepsforliving.hemophilia.org/next-step/maintaining-a-healthy-body/growing-up-puberty/effects-of-puberty-on-girls-with-a-bleeding-disorder>. Accessed February 20, 2019.

The signs and symptoms of menorrhagia include⁴:

- Having a menstrual period that lasts longer than 7 days
- Needing to change pads or tampons at least every 2 hours
- Passing blood clots larger than a quarter
- Bleeding that affects daily activities

"I am a true testament to the fact that factor replacement therapies can help when it comes to being a woman with hemophilia. Not only do we have to deal with the joint bleeds that men do, but we also have specific issues as women."

- ELIZABETH
Has hemophilia B

Tips for Parents⁴

Parents of a girl with a bleeding disorder can ease their daughter's transition into puberty by preparing her for the experience of having periods and helping her learn how to manage them. It can also be helpful for parents to ensure that a supply of feminine products is available and provide a way for their daughter to carry the products discreetly if needed. Parents can also help by providing honest, accurate information about menstruation and the impact hemophilia may have.

The beginning of menstruation, which can already be a confusing and demanding time in the life of any girl, brings special concerns for those showing symptoms of hemophilia. It's important for girls to talk with health care providers and caregivers about their periods, especially if bleeding becomes heavy.



Patient Affairs Liaisons are Pfizer hemophilia employees who are dedicated solely to providing support to the community. Your Pfizer Patient Affairs Liaison is available to help you access the support and information you need. To find your Patient Affairs Liaison, go to hemophilivillage.com/support/patient-affairs-liaison-finder or call Pfizer Hemophilia Connect[®] at 1.844.989.HEMO (4366).



HANY's RESOURCE CENTER

MISSION STATEMENT

The mission of the Hemophilia Association of New York is to provide information, education, advocacy and direct assistance to and on behalf of people with bleeding disorders, and to encourage and support scientific research to improve medical treatments and develop cures for hemophilia and related disorders.

About this Newsletter

The Hemophilia Outlook has been around since 1952. It is produced quarterly and distributed to all the members of the bleeding disorder community.

Electronic versions of our newsletters are available on our website.

HANY does not release any personal information without consent.

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Resources Information
Hemophilia Federation of America

800-230-9797

www.hemophiliafed.org

National Hemophilia Foundation

800-42-HANDI

www.hemophilia.org

Coalition for Hemophilia B

212-520-8272

www.coalitionforhemophiliab.org

HEMOPHILIA TREATMENT CENTERS

New York Presbyterian

www.cornellpediatrics.com

Mt. Sinai Medical Center

www.mountsinai.org

Northwell Health (formerly LIJ)

www.northwell.edu

Albany Medical Center

www.amc.edu

New Comprehensive HTC

Montefiore Hospital

www.montefiore.org