

Hemophilia Outlook

A Newsletter of

The Hemophilia Association of New York, Inc.

131 West 33rd Street, Suite 11D, New York, NY 10001

Tel 212-682-5510 Fax 212-983-1114

admin@hemophiliany.com * www.hemophiliany.com

www.facebook.com/Hemophilia.Association.of.NewYork

SUMMER 2018 **Newsletter**

2018 HFA SYMPOSIUM

On April 26-28 over 1,000 members of the bleeding disorders community came together in Cleveland, Ohio to share information, learn new advancements, and build a network of support. The event was a tremendous success.



HANY SCHOLARSHIP

A total of 24 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 24, 2018 at the main office.

For information about the 2019 scholarship program please look for the Fall 2018 newsletter.

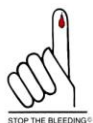
GIRLS WITH HEAVY PERIODS AREN'T BEING SCREENED FOR VON WILLEBRAND DISEASE

By: Reuters Staff

NEW YORK (Reuters Health) - The vast majority of young girls in the U.S. with heavy menstrual bleeding are not screened for von Willebrand disease, despite longstanding recommendations from the American College of Obstetricians and Gynecologists (ACOG), a new study indicates.

Up to 20% of girls with heavy menstrual bleeding will have an underlying bleeding disorder, most commonly von Willebrand disease, note Dr. Amanda Jacobson of Nationwide Children's Hospital and The Ohio State University in Columbus, Ohio, and colleagues in Obstetrics & Gynecology, online May 7.

"Onset of heavy menstrual bleeding at menarche is often the first sign of von Willebrand disease in affected patients. Early diagnosis of von Willebrand disease allows for additional treatment strategies for heavy menses if needed and



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prophylaxis for bleeding during future surgical procedures or childbirth," they point out. Factors associated with a higher frequency of von Willebrand disease screenings were younger age at diagnosis, inpatient stay for heavy menstrual bleeding, diagnosis of and testing for iron deficiency anemia, having commercial insurance, having an obstetrician-gynecologist at the initial encounter for heavy menstrual bleeding, and living in a metropolitan area.

"Increased awareness and adherence to recommended screening recommendations may increase diagnosis of von Willebrand disease," the authors conclude.

They note that a variety of health care providers will be "first responders" for girls and adolescents with heavy menstrual bleeding, "which emphasizes the need for increased awareness of von Willebrand disease among all health care providers taking care of adolescent females and recognition that heavy menstrual bleeding is the most common and often the first bleeding symptom in young women with von Willebrand disease."

The study was funded by the National Heart, Lung and Blood Institute. The authors have disclosed no conflicts of interest. Yet their analysis, of 2011-2013 national claims data for more than 23,800 postpubertal girls and adolescents (age 10 to 17) with a diagnosis of heavy menstrual bleeding, shows only 8% were screened for a deficiency of von Willebrand factor. Screening rates were somewhat better (16%) among those with severe heavy menstrual bleeding.

Source: www.medscape.com/viewarticle/896552

PATIENT ASSISTANCE PROGRAMS

The patient assistance programs are offered by factor manufacturers. Manufacturers offer assistance with co-pays and deductibles. If you would like information about these programs, please contact 212-682-5510.

YOU MUST REGISTER EVERY YEAR FOR YOUR SPECIFIC ASSISTANCE PROGRAM

Summer Camp Deadlines

Camp Little Oak

Date: Jul 22 to Jul 28 2018

Eligibility: Girls with a bleeding disorder

Child Age Range: 7-17 years old

Venue: Camp Little Oak

Deadline: July 10

Website: www.camplittleoak.org

Camp Carefree

Date: Jul 22 2018 to Jul 28 2018

Eligibility: Boys & Girls with a bleeding disorder

Children of parents with bleeding disorders
Unaffected siblings

Child Age Range: 6-16 years old

Venue: Camp Carefree, Inc.

Deadline July 10

Website: www.campcarefree.org

Camp Warren Jyrch

Date: Aug 5 2018 to Aug 11 2018

Eligibility: Boys & Girls with a bleeding disorder

Carriers

Child Age Range: 7-15 years old

Venue: Camp Warren Jyrch

Deadline: July 1

Medical Information Deadline: July 15

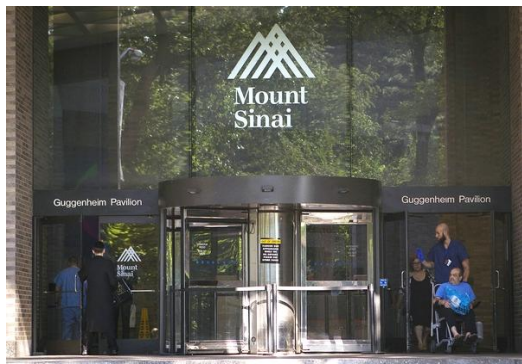
Website: www.bdai.org

If you are registered with a summer camp, we offer transportation reimbursement.

For more information email tconstantine@hemophiliany.com

PAST EVENTS

April 21- YOUNG WOMEN'S WORKSHOP



Mount Sinai staff held a unique integrative workshop introducing the new Young Women's clinic. This clinic will specialize in gynecological concerns for women living with bleeding disorders catered to women ages 22 and younger.

May 20- 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. Thank you to our sponsors Bayer, CSL, Genentech & Shire.

JUNE 1- "A NIGHT AT THE MUSEUM"



Our families enjoyed a sleep over at the Museum of Natural History. Thank you to Eileen San Juan & Michael Emma for the physical therapy session.

The 11th Annual Matthew L. Greer Liberty Mutual Golf Classic



On June 18th, the 11th 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 thank you to all of our sponsors for their generous support!

Shire & CSL

Embracing the Unique

By: Laurie Kelley

When children are diagnosed with hemophilia, they are each given an essential diagnostic label: for example, hemophilia A or hemophilia B, severe, moderate, or mild. These labels originate from a lab analysis of the child's blood. The diagnosis determines what type of factor replacement therapy each child will get. Labels like these can help draw a picture of who your child is and what he or she needs. But when it comes to dosing and prophylaxis regimen, sport choices and bleeding patterns, and even pain management, your child with hemophilia is unique. Diagnostic labels don't adequately explain a person's individuality and needs.

We asked parents from Facebook about their children with hemophilia: Has anyone ever used the labels of hemophilia to categorize your child, which resulted in limiting treatment options, or limiting what people think your child can do? What is it about your child that is not "typical" for someone with hemophilia? The responses poured in. While a child's uniqueness may be revealed in a preference for certain sports or a physiological reaction to a particular product, most of the parental responses we received were about each child's unique half-life, and about subsequent bleeding patterns.

Half-life was barely mentioned when my son was born. In the late 1980s and early 1990s, we dosed his factor using a chart based on his weight; it was very mathematical. We took one-half of his weight in kilograms times the factor level we desired, and this equaled the number of units of factor VIII we needed to infuse. Over time, as parents, we developed intuition about how much or how little factor our son needed based on his response to factor and his bleeding patterns, and we could adjust his dosage ourselves.

Up until about the last 10 years, hemophilia treatment centers (HTCs) often prescribed factor dosages based on weight, and determined a prophylaxis regimen based on a strict protocol from clinical studies. We now know that every child needs to have a pharmacokinetic (PK) or recovery study done to determine his or her

individual, unique half-life response to a specific factor VIII product. Determining the unique half-life can help hone the amount of factor a child should receive, or indicate the best prophylaxis regimen. A short half-life may mean more frequent infusions, higher doses, or the use of extended half-life products.

If anyone knows about the uniqueness of factor half-lives in children with hemophilia, it's June Reese, who has four sons with hemophilia. She says, "One son has always had a short half-life and has really struggled with bleeds. His teachers often compare him to his brothers, one of whom never bleeds." And this was a problem for the Reese family: in categorizing two brothers with textbook half-lives as "normal" for hemophilia, teachers dismissed the third brother's frequent bleeds—they thought he was being careless, or worse, that he was imagining the bleeds.

Crystal Eskine has two sons with severe hemophilia A, ages 9 and 10. "I expected two similar stories," she laughs. Despite having the same diagnosis as his brother, Crystal's 10-year-old bleeds spontaneously, "if you look at him too hard." Her younger son "never needs factor," and "he isn't even on prophylaxis he bleeds so little!" When Crystal's doctor wanted her to adhere to a traditional dosage and infusion schedule with her older son, her gut instinct told her it wasn't good enough. She knew her children's unique responses to factor. "I started giving my older son double doses. I took notes, showed our doctor, but he still he thinks I'm worrying too much, while I still don't think the dosing regimen is good enough." Crystal continues, "I've asked for a PK test, with blood samples taken over a much longer time period, but he has said no."

And then there is Jen Miller's five-year-old with severe hemophilia A. Jen calls him a "typical boy" who enjoys video games, swimming, T-ball, and playing with his friends. His factor half-life is very short, which is not typical, but this doesn't seem to impact his bleeding patterns.

When a shorter half-life does impact bleeding patterns, and parents instinctively know something isn't right, they need to alert their HTC staff, sometimes to prove that their child does not

fit a category or label. In these cases, parents should request a PK study. Crystal laments, “My boys’ hematologist makes me feel like I’m doing something wrong, but refuses to do a PK study.” June adds, “For years, our medical staff acted as though we were to blame when he’d have bleeds—even though he was infusing regularly.”

Kate Stotz, who has a 22-month-old with severe hemophilia A, felt she had to fight against the standard prophylaxis infusion schedule of three times a week. “This was not working for our son,” she explains. “He was having frequent bleeds on Sunday, the day he was unprotected. Trough levels indicated that in order to maintain a minimal 1% trough, we could not exceed 48 hours between infusions.” Though Kate wanted to infuse every other day to keep him protected, her son’s hematologist didn’t want to break from the traditional schedule the HTC normally prescribed. “It took a lot of advocating on our part and ultimately finding a new doctor at a different HTC.”

Sarah Hueston successfully advocated for a new prophylaxis regimen for her 16-year-old son with severe hemophilia A, who plays two varsity sports. When they determined he had a short half-life, the HTC team, Sarah, and her son developed his treatment plan together. He now infuses standard factor daily. “It’s what works for us,” says Sarah, “and his doctors are so proud of him, as are we, his family! Never did we think he’d be doing the things he’s doing even 10 years ago!”

By logging her son’s bleeds, Stacey Mollinet was able to convince her HTC team to change the treatment schedule. When her son with severe hemophilia A was a young teenager, he didn’t bleed like a typical severe and was not very active by nature. “I had to push the HTC,” she recalls, “so he could treat only twice a week, instead of a standard prophylaxis schedule.” Around age 14, he started to bleed more like a typical severe. So Stacey worked with the HTC to adjust her son’s dosing schedule, and ended up dosing every other day until he switched to extended half-life factor two years ago.

“There’s not a one-size solution for everyone,” Stacey has learned. “Keeping good

infusion and bleed logs so you know what schedule works best to prevent bleeding is important.” Crystal laughs, “I could probably write a book about all the ways my boys ‘differ’ from the typical definition of hemophilia.”

And in a community where boys “typically” have hemophilia while women are carriers, women are now advocating to redefine what it means to have hemophilia. Labels have their place, but when we define hemophilia and determine treatment plans, we sometimes need to look outside the box at hemophilia—and trust the parents and patients when they describe their own uniqueness and needs.

Publication: PEN 05.18

HANY UPCOMING EVENTS

July 24 - Scholarship Reception

July 29 - HANY 66th Anniversary Gala at Yankee Stadium

Tickets- \$220/Per Person

TICKETS ARE LIMITED

Current Sponsors: CSL, Shire, Novo, Kedrion

September 14-16 - S.A.I.L

(Self Advocacy Independence & Leadership)

Training at Camp Quinipet at Shelter Island, NY with Guttmonkey

(Ages 13-18)

Current Supporters: CSL, Genentech

December 7-9 - Steven L. Margolies, MD Family Retreat at Mohonk Mountain House

Current Supporters: Genentech

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.

HANY's Contacts

Linda Mugford, Executive Director

lindamugford@hemophiliany.com

Tyshawn Constantine, Program Director

tconstantine@hemophiliany.com

Jessica Blanco, Staff Associate

jblanco@hemophiliany.com

Website:

www.hemophiliany.com

Facebook:

www.facebook.com/hemophilia.association.of.newyork

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.coalitionforhemophilia.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

Montefiore Hospital

www.montefiore.org