HANJournal

Be sure to read the article on The ABC’s to Gene Therapy on page 20.
BDRN & HANJ

Bleeding Disorders Resource Network

BDRN’s Mission is to improve the quality of life for people living with bleeding disorders.

At BDRN we are dedicated to serving and making a difference in the bleeding disorders community. We take a team approach to address each set of circumstances. Our commitment to improving the lives of those living with a bleeding disorder is what motivates us and is the essence of everything we do.

Bleeding Disorders Resource Network

Hemophilia Association of New Jersey

HANJ’s mission is to improve the quality of life for persons with a bleeding disorder by providing and maintaining access to highly qualified medical treaters and successfully proven medical regimens.

- Ensure access to care
- Secure more comprehensive insurance coverage
- Ensure the NJ Standards of Care are met
- Provide financial grants to hemophilia and bleeding disorder patients
- Provide financial grants in support of the HTC’s
- Provide education programs and reimbursement support to patients of New Jersey

340B Program

The 340B Program is a federal drug discount program. It entitles certain safety net providers and clinics, including hemophilia treatment centers, to a discounted price for covered outpatient drugs. Rutgers/RWJ has registered to participate in the 340B Program as a Covered Entity. This will allow Rutgers/RWJ to purchase factor and other drugs at a discount. While other grant fundings suffer cutbacks, Rutgers/RWJ is able to use the cost savings and other program revenues to fund the services it provides to its patients. Rutgers has selected BDRN as one of its contract pharmacies under the 340B Program. BDRN and HANJ have agreed to work together to provide certain services for the Rutgers program, including patient education and financial assistance services.

Executive Director’s Corner

By Stephanie Lapidow

Summer is here, and boy, has it been HOT in the Garden State! For those of you who live here in New Jersey, I am sure you join in my excitement (and love) for fresh fruits and vegetables from local farms. If you are lucky enough, you may have your own garden in your backyard, which is even better! I have always loved the idea of growing your own fruits and vegetables. What starts as a simple seed, grows in time to be something you can enjoy and feel good about feeding your family.

Growth is often something we take for granted, particularly the amount of effort and nurturing that goes into making something flourish. It is a labor of love, really. With that love comes ups and downs. If you plant a garden you have to expect some rainy days, some hot days, some cloudy days, some days filled with sunshine. All in which you take in stride, day after day, until you see the fruit.

At the HANJ, we are moving into a space of growth ourselves. Despite the cuts in funding we have incurred both from manufacturers and government, we have begun exploring new and fresh ways of offering you the services you have come to expect, how we communicate our programs and events, and even what we look like to our community and the general public. Our Board of Trustees recently approved some really exciting things that we cannot wait to roll out to you. Sit tight for those updates, as I will be delivering those announcements in our next Executive Director’s Corner.

In the meantime, here’s a bit of update on things I can share with you today:

We continue to pursue Senate Bill 3100 and Assembly Bill S186 which will expand the definition of Hemophilia, specifically relating to our insurance grant program through the New Jersey Department of Health. This will allow more of our members to qualify for assistance so they can have access to the care they need.

In addition, I am happy to announce another piece of legislation that the HANJ has had introduced. Senate bill S3448 will require home care companies and specialty pharmacies providing clotting factor services related to bleeding disorders must comply with the New Jersey Standards of Care legislation regardless of their insurance affiliation. This bill will strengthen our standards and make certain outside pharmacies cannot provide clotting factor to anyone in New Jersey unless approved by the Department of Banking and Insurance. This will affect specialty pharmacies that are contracted with self-funded corporations. They will also have to meet our Standards of Care. For those of you unfamiliar with our Standards of Care law, please call the HANJ office and we will be happy to send you a copy.

That is all for now. Enjoy the harvest.

Warmly,
Steph
The Hemophilia Association of New Jersey was founded in August 1971 by 10 concerned families, and offers assistance to persons with hemophilia and their families from our office located in East Brunswick, New Jersey. Our mission is to improve the quality of life for persons with a bleeding disorder by providing and maintaining access to highly qualified medical providers and successfully proven medical regimens.

**PRESIDENT**
Ronald Grayzel, Esq.

**1st VICE PRESIDENT**
Steven Moersdorf

**VICE PRESIDENT**
Jeff Lynch, M.D.

**TREASURER**
Carl Piercey

**SECRETARY**
Chris Maniaci

**IMM. PAST PRESIDENT**
Joe Markowitz

**MEDICAL ADVISOR**
Jeff Lynch, M.D.

**TRUSTEES**
Tracie DeSarno

Milinda DiGiovanni

George Keelty

Richard Keelty

Elaine Kelly

Richard Kelly

David Kober

David Lechner

Robert Lessner

Richard Lloyd

Lisa Maniaci

Peter Marcano

Rajh Odi

Mary Petti

Mark Scudiery

Jerry Seitz

Issaiha Williamson

**MANAGING DIRECTOR**
Elena Bostick

**EXECUTIVE DIRECTOR**
Stephanie Lapidow

The HANJ scholarship is awarded for undergraduate level study. This year’s scholarship award recipient is Daniel Jenkins. Daniel will be attending Morehouse College in Atlanta, GA, to study Business Administration with a concentration in fashion.

The Julie E. Frenkel Scholarship

The Julie E. Frenkel Scholarship is awarded for undergraduate level study. This scholarship honors the memory of our beloved Hemophilia Association of New Jersey’s Assistant Executive Director, Julie E. Frenkel, who was fervent in her belief that continuing education is crucial for our community. This year’s scholarship award recipient is Danielle Jenkins. Danielle will be attending Spelman College in Atlanta, GA, as a Political Science and English double major.

The Robert and Dennis Kelly Memorial Scholarship

The Robert & Dennis Kelly Memorial Scholarship awards two additional full-time undergraduate scholarships in memory of Robert & Dennis Kelly, beloved sons of longtime HANJ Board members Elaine and Robert Kelly. Each year, the Kelly family and friends host the Kelly Brothers Scholarship Picnic in order to raise the funds necessary to make these awards possible. This year’s scholarship award recipients are Christopher Maniaci and John Rodriguez.

The Paul D. Amitrani Graduate Level Scholarship

The Paul D. Amitrani Graduate Level Scholarship is awarded in memory of our former President and honored member of our community for many years. The contributor of this award is longtime HANJ Trustee, Mark Scudiery. Our new scholarship award winner of the Graduate Scholarship this year is Samuel Eglow. Samuel will be attending Law School at Syracuse University Law School in New York.

For more information about the scholarships offered through HANJ or a list of other scholarship resources, please visit our website at www.hanj.org or contact us at (732) 249-6000.
WHAT’S HAPPENING
New Jersey Hemophilia Treatment Centers

Rutgers Robert Wood Johnson Medical School Hemophilia Treatment Center

Rutgers RWJ Medical School 340B Program:
In order for the hemophilia program to maintain comprehensive hemophilia care in an era of increasing health care costs amidst dwindling levels of federal and state funding of hemophilia programs, the Rutgers Robert Wood Johnson Medical School Hemophilia Treatment Center is a 340B covered entity. Participation in the federal 340B program makes it possible for our HTC to continue to serve the hemophilia community with the high level of services and quality of care it expects. If you have questions about this program, please do not hesitate to contact the HTC directly at 732-235-6533.

Studies:
Currently, the HTC is participating in 2 studies: 1) TAURUS: A Multinational Phase IV Study Evaluating “Real World” Treatment Pattern in Previously Treated Hemophilia A Patients Receiving KOVALTRY (NovoSeven forte) for Routine Prophylaxis and 2) A Multicenter Phase 2 Open-Label, Single-Arm, Prospective, Interventional Study of Plasma-Derived Factor VIII/VWF (Alphanate®) in Immune Tolerance Induction Therapy in Subjects with Congenital Hemophilia A. A third study for women with Type 1 Von Willebrand’s disease is upcoming. If you are interested in or have questions regarding these studies, please call the HTC.

School & Camp Visits:
The staff at the HTC continues to provide in-service programs to school and camp personnel about a child’s hemophilia. If you are in need of an in-service program at your child’s school, please contact Lisa Cohen, MSW at 732-235-6533. Please contact Lisa ASAP, as the slots for these visits fill up very quickly!

Ongoing Training:
The staff at the HTC continues to provide hands-on training in infusion procedures to parents and their children. A series of thirty minute sessions are held over a period of weeks/months depending on the families’ needs, abilities and schedule. Please call Frances Macren, RN at 732-235-6542, if you are interested in arranging infusion training.

General Information:
For information regarding women with bleeding disorders and/or a family history of hemophilia, clinical trials, genetic counseling, insurance issues, educational sessions or school visits, please call the Hemophilia Treatment Center at 732-235-6531.

Rutgers Robert Wood Johnson Medical School Hemophilia Treatment Center
125 Paterson Street
Suite 5200
New Brunswick, NJ 08901
To make an appointment:
(732) 235-7226
Nurse Direct Line for Medical Issues:
(732) 235-6531
Social Worker:
Lisa Cohen, MSW, LSW
(732) 235-6533
cohenlr@rwjms.rutgers.edu

Newark Beth Israel Medical Center and Children’s Hospital of New Jersey

Happy Summer! The staff from the Comprehensive Hemophilia Treatment Center at Newark Beth Israel Medical Center and Children’s Hospital of New Jersey would like to share some updates, current programs, and plans with you.

Staff News:
New Staff:
We are happy to announce that Christelle Faustin, MHA has joined our team as our new Data Analyst. Welcome Christelle!

UPCOMING
Hemophilia Camp:
The time for summer camp is quickly approaching. This year, like last year, we will have several children attending a hemophilia camp at either Double H Ranch or Hole in the Wall Gang Camp. Hemophilia camp is an integral part of our patients’ journey towards independence. Learning how to self-infuse at camp is one of the many highlights of our camper’s experience, and both camps have many supportive counselors who encourage our patients in that process. Both camps also offer family programming in the spring and fall. For more information about camp, or if your child or family is interested in attending camp in the future, please contact Erica, our Social Worker, at the HTC.

NEW INITIATIVE
Treatment Logs:
The HTC is charging ahead with a new initiative to get all of our patients (who treat their bleeding disorder with medication) to track their infusions and/or treatment in a treatment log. In an effort to provide the best possible care for our patients, it is extremely important that our physicians and nurses can see a patient’s treatment logs in real time, for the management of their bleeding disorder. ATHNAdvo is a web-based application that allows patients, or their caregivers, to record any and all treatment related to their bleeding disorder in a user friendly way directly through their smart phone, tablet, laptop, or computer. When a user creates an account, they choose their treatment center linking the patient and the HTC and allowing the HTC to have access to view a patient’s treatment log. For more information, or to sign up, please contact our Social Worker, Erica.

ONGOING PROGRAMS
Hemophilia 340B Program:
Our HTC participates in the Federal 340B Program. As a comprehensive care center, we have been improving the quality of life for individuals with bleeding disorders and providing cost effective care in the long term for many years. In an effort to help HTCs sustain themselves, and provide better care for their eligible patients, Congress created the 340B Program as part of the Veteran’s Health Care Act of 1992. Across the United States almost all of the HTCs participate in 340B Programs. Depending on their healthcare coverage, patients may have a variety of pharmacy options to choose from. Our HTC is contracted with four different home care companies;Accredo, RBHN, Biocorp, and Option Care. Patients who are not currently using one of these four companies may voluntarily switch if their insurance company allows. Participation in the 340B Program is voluntary. Please contact our Program Manager, Phyllis, for further information.

School Visits:
School may be out for the summer however it is not too early to start thinking about your child’s school or educational needs for the fall. School visits are a wonderful opportunity for our HTC.
to provide education and outreach to your child’s school or daycare about hemophilia and other bleeding disorders. Whether the visit is with the staff at your child’s school, the daycare staff, or even the child study team, a school visit opens the lines of communication between the child’s school or daycare and the HTC. If you are going to want a school visit scheduled for your child’s school or daycare center, or need a letter for school/forms completed, please contact Erica, our Social Worker. Erica will make sure that we have a release on file, and will coordinate your needs with the school and schedule a visit. If you will need any forms or letters for your child’s school or daycare center, please be mindful that it may take up to two weeks for forms or letters to be completed. For more information, please contact us at the HTC.

Comprehensive Evaluations:
It is really important to schedule and attend an annual comprehensive evaluation at the HTC. The annual evaluation is an essential component in the provision of an individual’s comprehensive care. Members of the HTC treatment team will complete medical, musculoskeletal, psychosocial and laboratory evaluations to assess the patient’s current health and to develop a treatment plan for the upcoming year. Education and referrals for medical and psychosocial services will also be provided as needed. At the time of an annual evaluation, patients will be asked to participate in the ATHN (American Thrombosis & Hemostasis Network) Data Set. This is a voluntary program conducted by HTC’s with support from ATHN to improve the health of people with coagulation disorders. Patients will also be educated about any other available studies that they might be eligible to participate in. Please note that any individual receiving medication through the HTC to treat their bleeding disorder must be seen by the HTC on an annual basis.

Manufacturers Factor Programs:
Manufacturers have programs available to help patients continue to receive products during a lapse of insurance coverage. They also offer co-pay assistance programs. Each program has enrollment requirements and many require yearly re-enrollment. Enrollment in these programs can be beneficial. For more information, please contact your home care company or us at the HTC.

Please contact us at The Hemophilia Treatment Center to sign up for one of the above programs or to request further information on available groups or services for children and adults. We can be reached at (973) 926-6511.

Travel Letters:
Are you going to be travelling? Are you going to need a travel letter? If you answered yes to either of those questions, this information is for you. Please remember to let the HTC staff know if you are going to need a travel letter at least two weeks prior to your scheduled trip so you can rest assured that your letter is in your hand as you embark on your journey.

Newark Beth Israel Medical Center and Children’s Hospital of New Jersey
Hemophilia Treatment Center
201 Lyons Ave. (E2)
Newark, NJ 07112
Main Number: (973) 926-6511
Social Worker: Erica Stuppler, LCSW
(973) 926-4197
Erica.Stuppler@RWJbh.org
Fax: (973) 391-0048

St. Michael’s Medical Center
Patient Education:
Our patients are always welcome to ask questions about New Therapies and Insurance updates. If you have any questions or concerns, please give us a call and we will provide you with the most up to date medical and insurance information. You may call Dominique Joseph, Nurse at (973) 877-5340 or Joanne Rodriguez, Social Worker at (973) 877-2967.

Psychosocial Yearly Evaluations:
It is very important to visit your HTC yearly to follow up on treatments and with any issues or concerns that may arise during the year. Please call The Blood Research Institute at (973)877-5340 to schedule your next visit.

School Visits:
School is over but remember that every year at the beginning of October we are available to start visiting schools for the purpose of staff education. If interested in scheduling a school visit, please contact Joanne Rodriguez, Social Worker at (973)877-2967.

Camp:
Camp is currently back in session. This is always a great opportunity for our children to meet other children and learn from each other. Always remember that every year gives your children an opportunity to attend. If you are interested in your child attending camp next year, please contact us at The Blood Research Institute at (973)877-5342.

Here at St. Michael’s Medical Center we would like to wish everyone a wonderful, happy and safe summer 2019!!!
FACTOR REPLACEMENT REFLECTS THE PROTECTION WITHIN

For people with hemophilia, Factor treatment temporarily replaces what's missing.\(^1,2\) With a long track record of proven results, Factor treatment works with your body's natural blood clotting process to form a proper clot.\(^3\)

Brought to you by Takeda, dedicated to pursuing advancements in hemophilia for more than 70 years.\(^\text{4}\)

Stay empowered by the possibilities.


Meet the Board…
Lisa Maniaci
HANJ Trustee

In 2001 my husband and I adopted a baby boy who was perfectly round and perfectly healthy. It wasn’t until about 10 months later, when a babysitter told me that he had a very dark bruise, in a very unusual place, that we had our first encounter with a bleeding disorder. She suggested we get it checked out, and that is where our HANJ story begins.

Our son was diagnosed with Severe Hemophilia A before his first birthday. Our first thought was, “What is Hemophilia?” and our second, “How do we fix this?” As you know, Hemophilia cannot be fixed…yet, but treating it and helping our child live a normal life is possible.

In the past 17 years I have made it my mission to be involved in the organization that does so much for so many in our community. I have served HANJ on the Fundraising Committee for almost all of those years; I am the current Chair of the Publicity and Education Committee, I did a brief stint on the Women with Bleeding Disorders Committee (although I do not have a bleeding disorder), I am on the Golf Tournament Planning Committee and, as of last year, became a member of the Board of Trustees. Whew!

Aside from all of that, I also have two careers. My full time work is in the Trade Show industry working for Skyline Exhibits. We design and manufacture trade show exhibits from small tabletops to large islands. I have had the pleasure of working with almost every industry under the sun, including one that takes me to Des Moines every year for the World Pork Expo, which is very interesting to a girl born and raised on Long Island. I have been with Skyline for 15 years.

My second career is in live sports broadcasting, as a Freelance Technical Director for CBS Sports. For 23 years I have had the pleasure of airing three Olympics, French Open Tennis, NFL Football regular season games, several Super bowls, NCAA Basketball and the March Madness Tournament, and I’m sure a few things I have forgotten.

In my spare time you can find me at yoga, taking my 12 year old daughter to Field Hockey tournaments or softball games, or helping my son get ready for his European singing tour and tournaments or softball games, or helping my son get ready for his European singing tour and.

Many of you may know my husband, Chris, who served as HANJ President a few years ago. When one of us is not traveling for work or tag teaming our kids’ activities, we actually find that we like spending time together. We enjoy getting away for extended weekends when we can, just to stop the world for a few days. We manage to find a wine or vodka tasting occasionally too. We have been married for 27 years and have lived in East Brunswick for 14.

Volunteerism is very important to us. It is something that was instilled in me as a child, watching my parents get involved in their church and community for all of my life. It is important that we instill this in our kids as well. Without active participation of our volunteers our organization would not survive to support those who need it. It costs nothing to participate for just a few hours a month, and the rewards are tenfold.

After all, “Community is in our Blood!”
Thank you to everyone who exhibited at our meeting. We greatly appreciate your support!
A little threat of rain doesn’t scare anyone away in the hemophilia community. We moved the Walk inside Raritan Valley Community College and had a great turn out. Thank you all for participating!

Thank you to everyone who supported our Walk!
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. 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.1

Tools that can help girls manage symptoms include:

- 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 a hemophilia should have a discussion with their health care team in order to coordinate management and care.

The signs and symptoms of menorrhagia include:

- 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

Tips for Parents1

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.

Parenting a child with a chronic disorder has a whole new set of challenges and worries. Hemophilia is unpredictable, inconsistent, and sometimes invisible. One time when we lived in south Florida, my son received a handicap decal for our car because he was unable to walk due to his ankle bleed. We received stares in the parking lot of our local grocery store. A woman questioned, “Why the decal? You both look fine. Do you have that handicap card illegally?” I was floored. But I responded politely, “We are okay, and have a nice day, ma’am.”

Some chronic conditions are not always obvious, and many patients are limited in their work or daily activities; sometimes they’re labeled lazy, overdramatic, or even a liar. Many patients try to explain their disability after hearing, “But you look so good.” It’s crucial that we spread awareness about invisible disorders to everyone we encounter and dispel any judgment calls.

Here are three incidents our family experienced with our invisible disorder—hemophilia:

Story 1. Omar was in fifth grade when he had a little sprain in his ankle, which happens to be his target joint. Just to be safe, my husband and I took Omar to the hemophilia treatment center (HTC) to get examined. The very next day, Omar went to school with crutches to avoid permanent damage. With high levels of factor in him, Omar decided not to use crutches at school for the next three days. He was fine, walking normally. Judging started right away. When Omar returned home, he said that his teacher and friends had called him a liar, and had assumed that all of this was made up. The next day, I visited school and clearly explained again about Omar’s bleeding condition, distributed additional information, and stated that this invisible illness is something to take seriously.

Story 2. In the fall of 2015, I was asked to teach at a special needs school in Livingston, New Jersey. I was so excited to teach this population and wanted to take up the challenge. At the time, it was my 15th year teaching special needs, ranging from age six months to 72 years. But I too judged someone, something I had never done in 15 years of teaching. I judged one of my students because he was not in a wheelchair; all of my students were in wheelchairs. I assumed that he didn’t have an intellectual disability. I thought, “Why is he in this school? He looks perfectly fine, and he is walking well.” I then discovered that this student had a major visual problem and indeed did have an intellectual disability. He was 14, and was learning at a third-grade level. This was a reminder to not judge others, even as...
a parent of a child with a chronic disorder. It was a learning moment for me.

Story 3. Omar’s wish was granted by the Make-A-Wish Foundation in 2014 to attend WrestleMania 30 in New Orleans, Louisiana, at the Mercedes-Benz Superdome. On the third day of the trip, we were invited to attend one of the six WWE WrestleMania Axxess sessions at the Morial Convention Center. At the session, there was a replica of the WWE stage, complete with music and video screen. All of the Make-A-Wish children lined up to make their special appearances on stage as if they were wrestlers. Omar decided he would walk in with the music and video of wrestler John Cena. As we approached the beginning of the line, an attendant stopped us and said, “He can’t go in.” I quickly responded, “And why not?” The attendant continued to stare at our son and at us. He said, “This line is for the Make-A-Wish kids only, and he is not in a wheelchair.” I replied, “Oh, you don’t see his illness, but can you see his badge that says ‘Make-A-Wish.’” I finished by saying, “He is making his entrance.”

As a parent and teacher, I have learned that some disabilities are invisible. And just as we can’t assume that a child or young adult in a wheelchair has limited intellectual abilities, we can’t assume that a child or young adult who is walking normally doesn’t have a chronic disorder like hemophilia.

I work closely with my students and take inventory of their strengths, weaknesses, likes, and dislikes—whether visible or not. At times, our disorders may be invisible, but we need to speak up and dispel any misconceptions and misunderstandings by sharing our knowledge. Folks, continue to advocate for yourself, for your children, and for others. Knowledge is power and empowering!

Mily Cepeda lives in New Jersey. She is a special education teacher and motivational speaker. Mily has an MA in special education and a BA in psychology. She is currently a doctoral student in education, dedicating her degree to her son and her father.

HANJ Pharmaceutical Educational Programs

HANJ partnered with Genentech for a program June 19, 2019 at Costa’s Restaurant in Roselle Park, NJ. Robert Szybist, from UPENN Positive Psychology Center, gave a very valuable presentation about strengthening relationships titled “The Science Connection”. We all left a little more knowledgeable about how to connect with the people in our life.

HANJ hosts several educational programs in partnership with various pharmaceutical companies once a month throughout New Jersey. These educational programs are a time for our members and their families to get together and enjoy the program. You may also meet members of the community you have never met before and make some new friends. Keep an eye out for program mailings. These programs are packed with valuable information you won’t want to miss. Please contact the HANJ office to RSVP for any program you would like to attend.

Upcoming Program: September 25, 2019
HANJ partnering with Sanofi
“Evaluating Your Insurance Options”

October 9, 2019
HANJ partnering with Biogen
“Managing Your Cowden Syndrome”

November 6, 2019
HANJ partnering with Hereditary Hemorrhagic Telangiectasia Outreach
“Navigating the Healthcare System”

December 4, 2019
HANJ partnering with Bristol Myers Squibb
“Understanding Your Blood Platelet Disorder”

Please contact the HANJ office to RSVP for any program you would like to attend.

Hemophilia Community Liaison

Tommy Russomano
Patient supporter

About Tommy
Tommy is a Hemophilia Community Liaison who is also a real patient and loves getting involved in the community. He served on the HFA and HANJ boards for years, supporting other hemophilia patients and families in the East Coast community.

Hobbies
• All things food-related
• Traveling

“The bleeding disorder community is very tight knit; we really support each other.”
Gene replacement therapy is designed to target the root cause of a disease—the gene that doesn’t work properly—by delivering a new, working copy of the gene. The new gene carries the instructions for making the protein the body needs that it couldn’t make before. Once the protein is being made in those cells, there is the potential to stop disease progression.

Gene replacement therapy works by creating a new, working copy of a specific gene in the laboratory. Then, the new gene is put inside a delivery vehicle, called a vector. Vectors are chosen based on which type of cells in your body need the new gene. This is because vectors are very particular about the type of cell they choose to enter. For example, a certain vector may be used to deliver a gene to cells in the brain, while another vector may be used to deliver a gene to cells in the liver.

Vectors are the delivery vehicles used to carry a new, working copy of the missing or non-working gene into the right cells inside the body. These delivery vehicles are typically made from naturally occurring viruses. Viruses are used because they are very good at getting inside of cells. However, scientists remove DNA from the virus so that it won’t make people sick when used as a vector.

A commonly used virus is the adeno-associated virus, or AAV. It is used because it can get inside many different types of cells, such as those in the liver, kidney, eyes, and the central nervous system. There are several different types of AAVs, and each has a specific affinity for certain types of cells in people, allowing them to target different cells and tissues. AAV is also not known to cause illness in people. This makes the AAV a potentially promising vehicle for use in the treatment of a wide range of genetic diseases.

In this kind of gene replacement therapy, the new gene sits separate from your DNA, inside the nucleus, or control center, of the cell. The vector is then naturally broken down by the body, but the gene stays right where it was delivered. The gene starts working to make protein the body needs to work as it should.

The Different Types of Gene-Based Therapies

There are several types of therapies that aim to treat diseases by using genes—and gene replacement therapy is just one type.

Gene Replacement Therapy

Gene replacement therapy is what we have been focusing on thus far in this article. It has been an area of study in humans since 1989. It has taken nearly 30 years for the first gene replacement therapy to be approved by the Food and Drug Administration (FDA) for use in people outside of a clinical study. In late 2017, a gene replacement therapy was approved for the first time to treat a rare, inherited form of vision loss. Many gene-based therapies are currently being investigated in clinical trials.
A Timeline of Progress in Gene-Based Therapies

Scientists have been exploring gene therapy for decades as a way to treat genetic illness. Take a look at this timeline of major milestones in gene-based therapies:

1865 - Working with pea plants, a scientist named Gregor Mendel discovers the fundamentals of heredity and how genetic information is passed from parent to child.

1909 - The term "gene" is coined.

1953 - James Watson, Francis Crick, and Maurice Wilkins characterize the structure of DNA. They were awarded the Nobel Prize in Physiology or Medicine in 1962 for this discovery.

1962 - Waclaw Szybalski demonstrates the first successful gene repair in mammalian cells.

1965 - The adeno-associated virus (AAV) is discovered. It will eventually be modified by scientists and used as a vector to deliver a new, working gene to cells.

1968 - Rogers and Pfuider demonstrate that virus-mediated gene transfer is possible.

1972 - The concept of gene therapy is first considered as a treatment for genetic diseases.

1973-1975 - The first direct human gene therapy trial took place, and while the trial was unsuccessful, there were many important learnings.

1984-2000 - Scientists embark on a 15-year odyssey that culminated in successfully mapping every piece of DNA in the human body.

1985 - A gene-based therapy is shown to be able to correct a genetic defect in human cells.

1989 - Rosenberg conducts the first in-human gene transfer.

1990-1999 - The FDA, for the first time, approves a human gene therapy trial. Gene therapy is used to treat a 4-year-old girl and a 9-year-old girl with a genetic disease. During this time, an 18-year-old boy undergoing gene therapy in a clinical trial passed away. This temporarily slows down gene therapy research so scientists can understand what happened. Scientists continue in their pursuit of a way to safely treat genetic diseases.

2003 - China approves the first gene therapy in the world for head and neck cancers.

2009 - Scientists show that an AAV vector has the potential to cross the blood-brain barrier in the body. This is a major advancement toward the treatment of genetic diseases with gene replacement therapy.

2012 - The first gene therapy is approved by the European Medicines Agency to treat a rare genetic disease.

2017 - The FDA approves the first CAR-T cell therapy in the United States, which is used to treat acute lymphoblastic leukemia. By the end of 2017, the FDA approves another gene therapy for an inherited form of vision loss.

2019 - More than 2,600 gene therapy clinical trials completed, ongoing, or approved worldwide. A major medical journal declares, "Gene Therapy Comes of Age."

2019 - Rosenberg conducts the first in-human gene transfer.

2009 - Scientists show that an AAV vector has the potential to cross the blood-brain barrier in the body. This is a major advancement toward the treatment of genetic diseases with gene replacement therapy.

2012 - The first gene therapy is approved by the European Medicines Agency to treat a rare genetic disease.

2018 - Major medical journal declares, "Gene Therapy Comes of Age."


For people affected by monogenic diseases like hemophilia, gene replacement therapy has the potential to help them in the future. As gene-based therapies are approved for genetic disorders, the advancements appear to have the potential to greatly benefit many.

Follow the Future of this Scientific Advancement

For additional information about the potential of gene replacement therapy for genetic diseases, please visit:

ClinicalTrials.gov

ClinicalTrials.gov provides patients, their family members, healthcare professionals, researchers, and the public with easy access to information on publicly and privately supported clinical studies on a wide range of diseases and conditions.

Genetics Home Reference

Genetics Home Reference is the National Library of Medicine’s website for consumer information about genetic conditions and the genes or chromosomes associated with those conditions.

National Human Genome Research Institute (NHGRI)
genomics, its mission is to accelerate scientific and medical breakthroughs that improve human health. They do this by driving cutting-edge research, developing new technologies, and studying the impact of genomics on society.
The Benefits of Physical Therapy: A Customized Approach

By Dr. Michael Zolotnitsky

As humans, we get tired of performing the same tasks over and over again, so we switch our routine. One month we like Facebook, then Instagram, then Twitter, then Snapchat. Why do we always switch our social media, diets, pillows, destinations, or fashion trends? Our minds tell us that if we do the same thing repetitively, we’ll get bored. The same goes for exercise.

We have to change our physical routines to avoid plateauing. Exercise needs to be consistently modified to help improve our bodies, to keep them from getting “bored.” But how do we do that safely to avoid injury? People with bleeding disorders find it hard to exercise for various reasons. I had difficulty when I first began, because I was afraid of causing more damage to my joints. When you start a new routine, you may face challenges: soreness, increased joint pain from overload, increasing low back or neck pain from improper technique, or maybe just not enjoying the workout. I wanted to find workouts that would be fun, so I could see results, not get bored, and improve my overall well-being to reduce my joint bleeds. That’s when I decided to pursue a career in physical therapy -- to increase my knowledge and to help others.

Attaining my doctorate in physical therapy to assist people with bleeding disorders was a huge accomplishment in my life. I knew what it was like growing up with hemophilia. I endured persistent joint bleeds, had trouble walking, and felt different from my peers. When I learned that with proper exercise, I could greatly improve my joint health, I felt like I had my life back, and I swore never to lose that health. From age 13, I have been running, weight lifting, and playing sports. I haven’t had a joint bleed in over 15 years, I attribute this to strength training and living a healthy lifestyle.

I wanted to educate the bleeding disorder community about what exercise can do for us all. I didn’t want anyone to feel they couldn’t do something because their bleeding disorder prevented them. I had the same concerns at a young age, but with hard work, I was able to overcome adversity and live like a “normal” person.

Exercise is my key to wellness, staying fit, and avoiding joint damage. I believe it’s crucial for people with bleeding disorders. Maintaining optimal joint health will increase functional mobility, strength, and endurance; but most important, it will reduce the number of joint bleeds and improve overall quality of life.

And not just any physical therapy, but a personalized approach. No two people are the same, especially when it comes to bleeding disorders. When I sit down with a patient, I ask “What do YOU want to get back to doing?” From there, we develop a personalized and customized treatment plan that relates to the patient’s goals. For example, if someone wants to run, we watch him run on our antigravity treadmill, which uses three camera angles and allows dramatic reductions in impact and gravitational forces, helping the patient increase mobility without pain. This allows the patient to walk, run, squat, and jump in a pain-free environment.

If a goal is more sports-related--soccer, basketball, golf -- it’s imperative to assess the overall quality of movements: the golf swing, running, jumping, or shooting a basket. We find the root of the pain or dysfunction by assessing all of these movements, and by looking at the ankle, knee, hip, pelvis, and spine to make sure we aren’t letting our patients put unnecessary strain on the body. A full-body approach is essential, so that every joint is covered.

Footwear is also important because increasing strain on the feet can affect overall walking and can cause joint pain from the feet to the low back. As we age, our bodies change, so it’s crucial to be reevaluated by a professional experienced in dealing with people with bleeding disorders. Flexibility is important, strength is important, but we need to make sure our bodies as a whole are symmetrical. We can use methods such as kinesio taping to place the joints in improved alignment, for joint support, and for reducing joint inflammation.

The customized approach yielded fantastic results when my first patient with hemophilia came to me for treatment. After developing inhibitors at an older age, being diagnosed with severe osteoarthritis of the hip, having brittle bones and severe weakness due to prior joint bleeds, he was facing total hip replacement. My evaluation showed that he had difficulty walking, balance deficits, poor core stability, and weakness in his knees, hips, ankles, core, and overall upper extremity strength. He used to walk a mile, but now it was hard for him to stand for five minutes with his cane. So his three months of treatment included endurance training on the antigravity treadmill, manual therapy to improve mobility of his hips, knees, and ankles; laser therapy to reduce joint inflammation in his hip; and balance and retraining to reduce his risk of falling and improve his overall gait. At the end of treatment, he had stopped using a cane, and his hip surgery was canceled. This was unbelievable: a person with hemophilia could overcome his pain and even cancel his surgery!

I can’t emphasize it enough: Physical activity is key. And it’s not too late to begin a program. It’s essential to make sure the movements are performed correctly to improve your joint function and not be detrimental. When you’re being assessed by a physical therapist, make sure the approach is customized and personalized, and that your program will constantly be updated based on your progress. I have lived by this approach for over a decade, and I have lived life to the fullest.

Dr. Michael Zolotnitsky, PT, DPT, who has hemophilia, is director of physical therapy at New Jersey Spine and Wellness in Old Bridge, New Jersey. Trained as an orthopedic and neurological physical therapist, Mike ensures his patients are offered a customized approach, including personalized aquatic therapy programs in indoor and outdoor pools. Mike is a national speaker for the hemophilia community, and lectures on safe exercises and alternatives to pain management, demonstrates kinesio taping, and runs aquatic therapy sessions. Mike has run three marathons in one year, and enjoys traveling and hanging out with his family, including his two nephews and his girlfriend. He is fluent in Russian.
**Blood Brotherhood**

**For Adult Men with Hemophilia**

The NJ Blood Brotherhood program holds free events for men with bleeding disorders. This group is open to anyone over the age of 21 who has a bleeding disorder. Each of our events incorporates a bit of education, socializing and a physical activity, but we typically use the time to get to know other guys in the community. The events are completely free and there is no commitment to attend every event.

If you'd like to join the Blood Brotherhood group and attend one of our events, please reach out to Peter Marcano (petermarcano@gmail.com, 201-401-7080) or Rajh Odi (odi.apd@gmail.com, 862-215-7944) or HANJ directly.

HANJ has partnered with the Hemophilia Federation of America (HFA) to offer the Blood Brotherhood program. Blood Brotherhood is a men’s group open to adult men (21+) with bleeding disorders. The purpose of this group is to provide an opportunity for older men with bleeding disorders to connect with their peers in a fun, relaxed setting. There is NO COST to attend any Blood Brotherhood event and once you sign up, there is no obligation to attend every event.

**DONATE! DONATE! DONATE!**

Please show your support by donating to The Hemophilia Association of New Jersey. Every single dollar counts!

Your donations go towards providing crucial programs to support persons with hemophilia in New Jersey.

Please make your check out to The Hemophilia Association of New Jersey or HANJ. We are a non-profit 501(c) organization. You will receive a receipt when we receive your donation for tax purposes.

Name: ____________________________________________
Address: __________________________________________
City: ______________________ State: _____ Zip: ______

Phone: Cell/Home : ____________________________
Amount of Donation: ____________________________

You can always donate on our website at www.hanj.org
Thank You! Your Donations Make A Big Difference!

---

**Summer Vegetable Whole Wheat Orzo Salad**

Recipe provided by Monica Hansen, Registered Dietitian at Greater Morristown Shop Rite.

This recipe is a great way to use up summer vegetables. The whole wheat orzo adds protein and fiber, and if you serve with grilled chicken or fish, you have the perfect summer meal!

**Ingredients:**

- 1/2 small Jersey Fresh eggplant, peeled and diced
- 1/2 medium Jersey Fresh yellow squash, diced
- 1/2 medium Jersey Fresh green zucchini, diced
- 1/2 red bell pepper, diced
- 1 small red onion, diced
- 2 garlic cloves, minced
- 2 teaspoons red wine vinegar
- 1/2 cup olive oil, divided
- Salt and pepper, to taste
- 1 cup dry whole wheat orzo
- 1 lemon, juiced
- 2 teaspoons red wine vinegar
- 2 scallions, greens only, thinly sliced
- 1/4 cup shredded parmesan cheese
- 10 fresh basil leaves, chopped

**Directions:**

1. Heat large skillet over medium-high heat. Add ¼ cup olive oil and sauté eggplant, squash, zucchini, bell pepper, onion, and garlic for about 5-6 minutes, until al dente. Season with salt, and pepper.

2. Meanwhile, cook whole wheat orzo according to package directions. Drain and transfer to a large serving bowl.

3. Add sautéed vegetables to cooked orzo in a large bowl. Add lemon juice, red wine vinegar, remaining ¼ cup olive oil and season with salt and pepper and toss well. Let cool to room temperature then add scallions, shredded parmesan, and basil and toss again, right before serving.
Tips for the Traveler

Some of us travel for fun, business, vacation and even emergency. But when you factor in someone who has a bleeding disorder you really need to be prepared for the trip to avoid any problems along the way.

Listed below are some tips you should consider:

- ALWAYS wear your MedicAlert bracelet or Medical Identification information.
- ALWAYS carry your own treatment products, supplies, equipment and Factor with you. Especially if you are flying. Bring them in your carry on. You need to have them accessible in case of delays or emergency. They could also be at risk of loss, breakage, or damage due to temperature variations.
- Carry a letter for security staff at your port of exit or entry explaining why you are carrying treatment products, supplies, equipment and Factor with you. Especially if you are flying. Bring them in your carry on. You need to have them accessible in case of delays or emergency. They could also be at risk of loss, breakage, or damage due to temperature variations.
- Carry a letter for security staff at your port of exit or entry explaining why you are carrying treatment products, supplies, equipment and Factor with you. Especially if you are flying. Bring them in your carry on. You need to have them accessible in case of delays or emergency. They could also be at risk of loss, breakage, or damage due to temperature variations.
- Carry a letter to present to customs, if requested, to explain why you are carrying treatment products, prescribed drugs, needles, syringes etc... and the serious implications of not having them immediately on hand. See sample letter for customs.
- Carry a letter from your treating doctor with information about your bleeding condition, any blood-borne viruses you may have, and the usual treatment you receive. This letter should, if possible, be in the language of the country being visited. You may not always be able to treat yourself, so this information is important. See sample letter from doctor.
- Carry a letter to present to customs, if requested, to explain why you are carrying treatment products, prescribed drugs, needles, and syringes. Again, this letter should be in the language of the country being visited. See sample letter for customs.

Sample Travel Letter from Treating Doctor
To Whom It May Concern:
RE: (Patient’s name)
This patient has a bleeding disorder called .
indicating a deficiency (and/or malfunction) of factor . She/he is well known to me. If internal or external bleeding occurs, the patient responds well to early self-infused transfusions of anti-hemophilic factor plasma-derived or recombinant concentrates. ___’s (patient’s name) judgement as to when these products should be used and as to the quantity may be relied upon.
Yours truly, (Doctor’s signature, name, position)

Sample Travel Letter for Customs, Port of Entry
Dear Sir or Madam:
A person with hemophilia or other inherited bleeding disorder can control internal or external bleeding only with infusion of plasma-derived or recombinant concentrate.

(patient’s name), who suffers from _______ (hemophilia, von Willebrand disease, or other inherited bleeding disorder), is travelling to . It would not be possible for him/her to travel without a supply of concentrates. She/he carries with him/her sufficient units for self-infusion as maintenance therapy and additional amounts in the event of an emergency. She/he also carries syringes, needles, etc., necessary for infusion.

prescribed drugs are also being carried for management of _______.

A customs official who inadvertently withholds bottles of plasma-derived or recombinant concentrate may place ______’s (patient’s name) life in jeopardy. For purposes of comparison, it is as if insulin vials were withheld from a person with diabetes.

In anticipation of your cooperation, I am, Yours very truly, (Doctor’s signature, name, position)

Sample Letter for Airport/ Airline Security
Dear Sir or Madam,

Re: (name & address of person)

The above named is a person with a bleeding disorder. The bleeding to which they are subject to can only be treated with infusion of plasma-derived or recombinant concentrate. Since bleeding may occur at any time, (patient’s name) carries supplies with him/her together with syringes, needles, etc... for infusion. It is therefore of vital importance that these essential medications are permitted to travel with the bearer as hand luggage.

In no circumstances should the concentrates be withheld or placed in the aircraft hold as to doing so may cause unnecessary delay in treatment and, additionally, would increase the risk of loss or damage.

A security official who inadvertently withholds bottles of plasma-derived or recombinant concentrate may place ______’s (patient’s name) life in jeopardy. For purposes of comparison, it is as if insulin vials were withheld from a person with diabetes.

In anticipation of your cooperation, I am, Yours very truly, (Doctor’s signature)

The Hemophilia Association of New Jersey provides MedicAlert Memberships and IDs for patients with hemophilia and von Willebrand Disease.

Medical ID jewelry is essential for people with bleeding disorders. Wearing a Medical ID ensures emergency responders and hospital staff have the most up-to-date medical information the moment they need it, to make informed decisions about treatment and care.

For more information and an order form, please contact Cindy Hansen at HANJ at 732-249-6000 or chansen@hanj.org.
HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider’s instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

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

- Thrombotic microangiopathy (TMA), a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs.
- Midline venous thrombosis (MTV), a type of blood clot in veins that are located in the body's central nervous system.
- Renal impairment, a condition in which there is damage to the kidneys.
- Ocular hemorrhage, a condition in which there is bleeding into the eye.
- Thrombotic microangiopathy (TMA), a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs.
- Midline venous thrombosis (MTV), a type of blood clot in veins that are located in the body's central nervous system.
- Renal impairment, a condition in which there is damage to the kidneys.
- Ocular hemorrhage, a condition in which there is bleeding into the eye.
- Thrombotic microangiopathy (TMA), a condition involving blood clots and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs.
- Midline venous thrombosis (MTV), a type of blood clot in veins that are located in the body's central nervous system.
- Renal impairment, a condition in which there is damage to the kidneys.
- Ocular hemorrhage, a condition in which there is bleeding into the eye.

If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total. See “What are the possible side effects of HEMLIBRA?” for more information about side effects.

What is HEMAIRA?

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

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

HEMIRAIRA increases the potential for your blood to clot. Carefully follow your healthcare provider’s instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMIRAIRA may cause serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including thrombotic microangiopathy (TMA), and blood clots (thrombotic events). If aPCC (FEIBA®) is needed, talk to your healthcare provider in case you feel you need more than 100 U/kg of aPCC (FEIBA®) total.

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

Upcoming Events

27th Annual Kelly Brothers Scholarship Benefit
In Memory of Bob & Dennis
Saturday, August 17, 2019

Dennis Keelty Memorial Classic
Monday, August 19, 2019

Casino Night
Saturday, October 26, 2019

Fall Educational Symposium
November 2019
Date to be decided

PACT Workshop
December 2019

Super Bowl Raffle
January 2020
Date to be decided

Winter Membership Gathering
Sunday, February 29, 2020