BDRN & HANJ
340B Bleeding Disorders Program
In association with:
Rutgers: Robert Wood Johnson Medical School

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.

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.

Message from …
President of HANJ
Joe Markowitz

In July I became the President of the Hemophilia Association of New Jersey, and I am looking forward to working with everyone to improve the quality of life for all of us. Our organization, frankly the best of all of the state hemophilia chapters, has a proven record of success.

Over the years, HANJ has worked with NJ legislators to create Standards of Care that ensured we’d get quality service from our Specialty Pharmacies and manufacturers. The guarantee of readily available infusion nursing care and factor are mandated by the Department of Insurance. In addition, the Department of Health provides a grant, administered by HANJ, that ensures that hemophiliacs have the opportunity to get health insurance as long as they meet the guidelines for eligibility.

The reason I mention these items is that prior to being on the Board of HANJ I really had no idea of all of the work that was being done on our behalf. The HANJ staff works behind the scenes, with the HTCs, pharmaceutical and home health care companies, to provide NJ hemophiliacs with services unavailable in other locations.

To continue with this work, we need your support. And while cash is always nice, there are many other ways to make a valuable contribution. Consider joining a committee, possibly Education, Fundraising, Blood Brotherhood, that will help broaden the services we can provide.

What HANJ provides is not just valuable, it’s necessary. Imagine the young parents who were just told that their infant son has hemophilia. If not for HANJ where would they go for support? Where would they go to meet other parents who can explain that their son can have a wonderful long life.

Hemophilia comes in many forms. Some members have severe joint issues associated with chronic pain from bleeds as a child; some members have depressions associated with aging. Longevity may bring issues, but let’s celebrate that we are living longer.

But these advances don’t mean that HANJ has an easier job. We still have members with severe arthritis resulting in mobility issues; some members have depressions associated with chronic pain from bleeds as a kid; there’s clearly still insurance issues; and our young members and parents need support networks and guidance on how to handle hemophilia as you grow from an infant to college age. Clearly some members also need financial support.

The current federal trend in reducing or eliminating health care coverage, the issues around pre-existing conditions, and maximum life time caps, makes HANJ incredibly important to us.

So, in conclusion, I ask for your support to HANJ. And regardless of party affiliation, get out and vote and tell your representatives what is important to you.

Best Regards,
Joe
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.

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Social Worker Update
By Neidy Olarte, MSW
Social Service Coordinator

This year at the HANJ Annual Meeting on May 31, 2018 at the Pines Manor in Edison, NJ we announced the following 2018—2019 Scholarships:

**Robert and Dennis Kelly Memorial Scholarship — New:**
Andrew Cameron
Justin Najimian

**Renewal:**
Thomas Culp
Samuel Eglow
Justin Haring
Justin Horbach
Dominic Mistretta

**HANJ Scholarship — 2018 - 2019 New:**
Jake Lessner
Rhett Presti
Kaustubh Shankar

**Renewal:**
Anthony Dierna
Julie Frenkel Scholarship

**New:**
Victoria Lynn Young

**Renewal:**
Benjamin Cutler
Terynn Young
Paul D. Amitrani Graduate Scholarship

**New:**
Melissa Najimian

**Renewal:**
Daniel Guerriero

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**New:**
Julie Frenkel Scholarship

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Victoria Lynn Young

**Renewal:**
Benjamin Cutler
Terynn Young
Paul D. Amitrani Graduate Scholarship

**New:**
Melissa Najimian

**Renewal:**
Daniel Guerriero

**CAMP Update:**
This year the HANJ worked in collaboration with the camps and treatment centers to secure another fun year for campers interested in attending the Hole in the Wall Gang Camp and The Double “H” Hole in the Woods Ranch. The Hole in the Wall Gang Camp in Connecticut (Session 4) was from Friday, July 13th to Thursday, July 19th. The Double “H” Hole in the Woods Ranch in New York (Session 7) was held Thursday, August 9th to Tuesday, August 14th. A total of 22 campers will be attending camp this year. HANJ is providing transportation to these sessions for those who will be attending camp.

**FALL EDUCATIONAL SYMPOSIUM:**
Our 2018 Fall Educational Symposium will be held on Thursday, September 13, 2018 at The Madison Hotel in Morristown, NJ. Invitations will be mailed to our members next month so please keep a look out for it.

**Pharmaceutical Educational Programs:**
HANJ hosts several educational programs in partnership with Pharmaceutical companies throughout the year. We will be hosting the following upcoming programs:

- **October—** Bioverativ—Topic: “Gateways to Education: Exploring Opportunities that may be right for you”

The HANJ is committed to serving the needs of our patients and their families in the bleeding disorder community and providing the most up-to-date information to the community.

Please do not hesitate to contact us with any questions.
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 pro-
gram to maintain comprehensive
hemophilia care in an era of in-
creasing health care costs amidst
dwindling levels of federal and
state funding of hemophilia pro-
grams, the Rutgers Robert Wood
Johnson Medical School Hemo-
philia Treatment Center is a
340B covered entity. Participa-
tion 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 ex-
pects. 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 partici-
pat ing in 2 studies: 1) TAURUS: A
Multinational Phase IV Study
Evaluating “Real World” Treat-
ment Pattern in Previously Treat-
ed Hemophilia A Patients Receiv-
ing KOVALTRY (Octocog alfa) for
Routine Prophylaxis and 2) A
Multicenter Phase 2 Open-Label,
Single-Arm, Prospective, Inter-
ventional Study of Plasma-
Derived Factor VIII/VWF
(Alphanate®) in Immune Toler-
ance Induction Therapy in Sub-
jects with Congenital Hemophilia
A. 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 or camp,
please contact Lisa Cohen, MSW
at 732-235-6533. Please do not
wait to contact Lisa, as the slots
for these visits fill up very quick-
ly!

Ongoing Training:
The staff at the HTC continues to
provide hands-on training in in-
fusion procedures to parents and
their children. A series of thirty
minute sessions are held over a
period of weeks/months depend-
ing on the families’ needs, abili-
ties and schedule. Please call
Frances Maceren, 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, clini-
cal trials, genetic counseling,
insurance issues, educational
sessions or school visits, please
call the Hemophilia Treatment
Center at 732-235-6531.

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

Happy Summer from the staff at
the Comprehensive Hemophilia
Treatment Center at Newark
Beth Israel Medical Center and
Children’s Hospital of New Jer-
sey! We are happy to share with
you the latest news from our
treatment center.

NEWS
Hemophilia Camp:
Camp season is here. Children
can attend a hemophilia camp at
either Double H Ranch or Hole in
the Wall Gang Camp. Both camps
also offer family programming.
Camp can be an integral part of
a patients’ journey towards inde-
pendence. Transportation for one
session (at each camp) is provid-
ed by HANJ. For more infor-
amation about camp, or if your
child is interested in attending,
please contact our Social Work-
er, Erica, at the HTC.

Scholarships:
Scholarships are now available.
Please be mindful of the dead-
lines to submit the applications
and the eligibility criteria for
each scholarship. Also, please
remember to complete your FAF-
SA forms as early as possible to
secure available funds. If you
have any questions about schol-
arships, internships, or coordi-
nating your care away from
home please contact our Social
Worker Erica.

ONGOING PROGRAMS
Hemophilia 340B Program:
Our HTC participates in the Fed-
eral 340B Program. As a compre-
hensive care center, we have
been improving the quality of life
for individuals with bleeding dis-
orders and providing cost effec-
tive care in the long term for
many years. In an effort to help
HTCs sustain themselves, and
provide better care for their eli-
gible 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, pa-
tients may have a variety of
pharmacy options to choose
from. Our HTC is contracted with
four different home care compa-
nies; Accredo, BDRN, Bioscrip,
and Option Care. Patients who
are not currently using one of
these four companies may volun-
tarily switch, if their insurance
company allows. Participation in
the 340B Program is voluntary.
Please contact our Program Man-
ger, Phyllis, for further infor-
mation.

School Visits:
As the school year ends, we
know that you might need forms
completed, letters for school, or
school visits scheduled. School
visits are a wonderful opportunity for our HTC to provide education and outreach to your child’s school or daycare center 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 or 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.

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.

Manufacturer Factor Programs: Manufacturers of clotting factor products have programs available to help patients continue to receive factor 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 contact 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.

St. Michael’s Medical Center

Happy Summer!!!

School Visits:
School is over for summer break, but remember we are always available for school visits. Our main goal is to educate school staff about the different types of bleeding disorders and new treatments. You may call The Blood Research Institute at (973) 877-5342.

Camp Applications:
Summer is here! I hope you all had the opportunity to sign up for this year’s camp experience. It’s a nice opportunity to meet others with same or similar bleeding disorders and learn from one another. For more information, please feel free to call Social Worker, Joanne Rodriguez, at (973) 877-2967.

Patient Education:
Our patients are always welcome to ask questions about
Meet the Board...

Mary Petti
HANJ Trustee

Hi. My name is Mary Petti and I am a Board member of HANJ. I am on the fundraising committee, and also serve as co-chairperson for our annual Dennis Keelty Memorial Golf Outing (our biggest fundraiser), helping plan the event and selling raffle tickets too!!

Just some background. I became involved with HANJ approximately 17 years ago because of my friendship with the family whose son the golf tournament is named after. Having a background in Pharmacy, I “knew” about hemophilia, but never really understood the daily physical, medical, emotional, and financial problems that were associated with this disease. I became even more interested in what HANJ does after learning my friends grandson also has the disease; it was a heartbreaking revelation!!

The more I learned about what HANJ does for their members and their families, the more involved I got. When the opportunity presented itself to join the board and really make a difference, I enthusiastically said yes. Let me add, that I am very fortunate to not have anyone in my family affected by the disease. This is truly a labor of love.

As to me personally, I retired as a pharmacist in 2003, and have been selling Real Estate since then. I am married since 1980 to my wonderful husband Florie, who is also involved with HANJ. We have two children, Matthew, (an account representative for Elizabethtown Gas) who is married to my daughter-in-life Sara (an elementary school teacher) and they have a gorgeous baby girl named Cora. Our daughter, Andrea, is a pastry chef and currently works in Red Bank. I love to fish, read, play golf, travel, and dance. My hobbies are crafting and gardening.

I cannot express enough how important it is for you to get involved in your organization, even if you only have a few hours to give. We do so much more behind the scenes, on so many levels, to make sure all our members have the quality of life they deserve. Here’s to the vision that one day hemophilia will be a disease of the past!!!
Please feel free to support The Hemophilia Association of New Jersey by donating. Your donations go a long way and every single dollar counts!

Donations go towards our Medical Insurance Grant Program, Scholarships, Financial Hardship Assistance, our Educational Programs and much, much more. All recipients are subject to eligibility.

Please make your checks 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 may always donate on our website at www.hanj.org
Thank You! Your Donations Make A Big Difference!

The Hemophilia Association of New Jersey would like to Thank Our Exhibitors at the Annual Meeting & Educational Forum on May 31st, 2018.

Accredo Specialty Pharmacy
Bayer US
BDRN, LLC
BiologicTX
Bioverativ
CSL Behring
Genentech
Kedrion Biopharma
Octapharma
Option Care
Pfizer Inc.
Shire

We truly appreciate your support!

Hemophilia Association of New Jersey’s Hemophilia Walk
Sunday, May 6, 2018
At Raritan Valley Community College

Thank you to everyone who came out to support Hemophilia!
Hemophilia Association of New Jersey
38th Annual Testimonial Dinner Dance
Humanitarian
"Man of the Year"
Honoring

Paul Perreault
CEO & Managing Director
CSL Limited
May 11, 2018
Fiddler’s Elbow
Country Club
To Play or Not to Play?
Derek Markley
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Publication: PEN 05.18
Column: As I See It

As a father, I will be the first to say that learning about hemophilia leads to a number of questions about the life your son will live. This is highly dependent on your own childhood. Hemophilia mainly affects boys. There are women who suffer from hemophilia and other bleeding disorders, but hemophilia often discriminates. It likes to live in boys.

No one can stereotype exactly what a boy’s childhood will look like. Personally, I forecasted my son’s future based on my own experiences. We lived out in the country, and growing up, our daughter was happy being outdoors. With a son, I expected that we’d have someone who was simply a replication of me as a child, and also loved being outdoors.

I like to be outside. As a youth I played outdoors, shot BB guns, fished, and often did things that resulted in falling, tripping, slipping, or sliding. None of this was out of the ordinary. Little boys come back in the house dirty, sweaty, and bleeding sometimes. I was no different.

My fondness for being outdoors also meant that I was constantly playing a sport. My parents enjoyed that, and let me play year-round. Baseball, soccer, and basketballs took out of the ordinary. That little boy’s condition and our own fears, insecurities, and concerns. But prior to those incredibly helpful first meetings, we wondered exactly how we were supposed to raise a child who is different. We were desperate for someone to help us make sense of how we’d navigate the coming years.

As parents, we had to make a decision: either let Bubba play, or begin to guile his interests in a direction other than sports. When he started preschool in Tupelo, we learned that his teacher and her husband had lost a child at a young age. During one of our first discussions with this teacher, we found a common ground in our desire to make sure that Bubba was limited as little as possible by his condition. His teacher agreed fully, and told us that she and her husband had felt the same way about their son. I still remember her saying that she’d never regret the fact that they had always let him do as much as his medical condition would allow.

Overcoming our initial fears was not easy, nor would those fears disappear immediately when Bubba’s first soccer game ended without incident. So much of Bubba’s life is defined by the fact that he has hemophilia, but that does not have to be a bad thing. Playing soccer has been great for him, and a few people have even learned more about hemophilia because of his presence. The biggest leap wasn’t his to take; it was ours. Our decision to let him play sports ensures that we’ll always feel apprehension at game time, but it also means that our son will make valuable memories as a member of a team, and as a little kid having fun on the soccer field.

Derek lives in Saltillio, Mississippi, with his wife Ashley and their children Abbey and Bubba. He is executive director of two University of Mississippi regional campuses and assistant professor in the School of Education. Ashley is a fifth grade math teacher in the Tupelo Public School District.

Please feel free to contact me if you would like to share something to be considered for a future newsletter. You can tell your story. Send some pictures with captions. Anything that may help or enlighten our family, friends and members in the bleeding community to grow and thrive dealing with a bleeding disorder.

Please contact Gen Christo at hemnj@comcast.net. I look forward to hearing from you.
**ACE910: The First Disruptor**

Paul Clement

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Publication: PEN 2.18
Column: Inhibitor insights
Sponsored by Novo Nordisk Inc.

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**Disruptive innovation: an innovation that creates a new market and eventually disrupts an existing market, displacing established market-leading firms, products, and alliances.**

The standard of care for people with hemophilia in developed countries is clotting factor replacement therapy, and for those with inhibitors, therapy with bypassing agents (BPAs). These therapies have created an industry worth about $11 billion. That’s all about to change, especially for people with inhibitors, because of new “disruptive therapies”—which do not require factor replacement therapy or the use of BPAs. In addition to gene therapies, which will soon become a reality, several other disruptive therapies are poised to enter the market, and shake it up (see box, page 16).

One of these disruptive therapies has already arrived: in November 2017, the US FDA approved Genentech’s Hemlibra® (emicizumab or ACE910) for treating hemophilia A with inhibitors. Two things make this product a standout: it’s administered subcutaneously under the skin—and the clinical results have been remarkable. Genentech describes Hemlibra as the first innovation for inhibitor patients in 20 years.

Hemlibra has put the wheels of change in motion.

How Does Hemlibra Work?

Hemlibra is a bispecific monoclonal antibody that mimics (copies) the function of factor VIII. What’s a bispecific antibody? Antibodies are Y-shaped proteins that serve as the immune system’s first line of defense against foreign substances including pathogens such as viruses, bacteria, and fungi. Each antibody is unique in that it recognizes and binds only to a specific antigen: a specific molecule on a specific pathogen. The unique part of the antibody—the binding site that attaches to an antigen—is located near the tips of arms of the Y-shaped antibody. In nature, both arms of the antibody bind to the same antigen. Bispecific antibodies are a little different from natural antibodies—they are artificial antibodies that can simultaneously bind to two different types of proteins (antigens), instead of to a single antigen (as is normal in nature). The adjective “monoclonal” simply means that all the bispecific antibodies are of the same type.

When an antibody binds to an antigen, it may neutralize or inactivate the antigen. Inhibitors, for example, are antibodies that attach themselves to infused factor and inactivate it, preventing it from helping to clot the blood. All antibodies tend to persist in the blood for a week to a month (they have long half-lives).

If an inhibitor to factor VIII is an antibody, then how can antibodies be used to treat hemophilia?

You know that factor VIII is one of more than a dozen clotting factors that participate in the complex blood coagulation process known as the clotting cascade. Factor VIII's job is to activate factor X. Factor VIII does this by becoming activated itself (after injury to a blood vessel) and then binding to activated factor IX (written as IXa) and factor X. When factor IXa and X are brought close to each other, the factor X becomes activated (now written as Xa). This allows the clotting process to continue, and eventually to form a clot. When you have hemophilia A, you have little or no factor VIII. With no factor VIII, no Xa is produced, and the clotting process stops. The bleeding continues.

Researchers at Chugai Pharmaceuticals asked: How can we bring factors IXa and X together to activate factor X without the need for factor VIII? They settled on investigating bispecific antibodies. And they undertook a daunting molecular engineering task: to design a bispecific antibody capable of activating factor X by simultaneously binding factors IXa and X. The bispecific antibody would act like a person grabbing factor IXa in one hand and factor X in the other and then bringing the two together to create activated Xa.

There would be no need for factor VIII.

**A Breakthrough for People with Inhibitors?**

In two phase 3 clinical trials, Hemlibra demonstrated impressive results, with a significant reduction in bleeding episodes, and in many cases, with no reported bleeds!

Two additional phase 3 clinical trials of Hemlibra are also underway: Haven 3 is evaluating prophylactic use of Hemlibra versus no prophylaxis in hemophilia A patients without inhibitors (to be completed in September 2019). And Haven 4 is looking at higher doses of Hemlibra, but monthly dosing as opposed to weekly dosing (to be completed in July 2018).²

**A Black-Box Warning**

Despite the impressive results, Hemlibra carries an FDA “black-box” warning—the strictest of warnings the FDA can require a manufacturer to put on prescription drug or drug product labels. Hemlibra’s product insert carries extensive warnings of thrombotic events if the product is used with FEIBA. Thrombotic events are clots that cause partial or total obstruction of a blood vessel.

Why the need for warnings? In late 2016 and early 2017, Roche reported that five participants (out of 109) who...
were being treated for breakthrough bleeds in the first clinical trial had experienced serious thrombotic events. In all of these cases, the affected patients had received repeated doses of aPCC (FEIBA) to control breakthrough bleeding. Roche concluded that simultaneous use of aPCC and ACE910 is associated with an elevated risk of thrombotic events. So the company issued a new protocol in its clinical trials that recommends avoiding aPCC whenever possible and only administering at low doses. After the new protocol was implemented, no new cases of thrombotic events were reported.

**Disruptive Therapies for Hemophilia in Development**

- **Gene therapy for hemophilia A and B** is underway in almost a dozen clinical trials. These trials have produced significant and sustained results in some cases, curing patients of their hemophilia.
- **Catalyst Biosciences and ISU Abix** are conducting a phase 1/2 clinical trial of their highly potent, next-generation factor IX product (CB 2679d). This drug is administered subcutaneously to treat hemophilia B.
- **Alnylam Pharmaceuticals and Sanofi** are restarting a phase 1/2 clinical trial of a subcutaneously administered RNA interference (RNAi) therapy, ALN-AT3 (also called fitsusiran), that targets antithrombin (AT, a protein that inhibits clotting) to improve thrombin production. Fitusiran can be used to treat hemophilia A or B, with or without inhibitors.
- **Bayer Healthcare Pharmaceuticals** and Novo Nordisk each have phase 1/2 clinical trials underway testing concizumab, a subcutaneously administered monoclonal antibody that is an anti-tissue factor pathway inhibitor (anti-TFPI). Like AT, TFPI is a protein that inhibits clotting. By blocking the function of TFPI, concizumab improves thrombin production and can be used to treat hemophilia A or B, with or without inhibitors.
- **Chugai Pharmaceutical Co., Ltd., and Genentech** (both subsidiaries of the Swiss healthcare company Roche Group) have underway two phase 3 clinical trials (Haven 3 and Haven 4) of Hemlibra (emicizumab-KXWH, formerly ACE910), a subcutaneously administered bispecific antibody that mimics the function of factor VIII, to treat hemophilia A with or without inhibitors.

### Hemlibra’s Potential Impact on the Marketplace

Right now, Roche is targeting only people with hemophilia A and inhibitors. Roche revealed its pricing for Hemlibra in November 2017: “At an average weight of about 127 pounds, the cost is approximately $482,000 for the first year of treatment and then approximately $448,000 per year, which is less than half of the WAC [wholesale acquisition cost] of the only other approved prophylactic treatment for these [inhibitor] patients.”

Hemlibra’s price still places it within the top 10 most expensive drugs for rare conditions. And for people without inhibitors, the cost is significantly higher than prophylaxis with factor concentrates. It remains to be seen whether Roche will retain the present pricing structure in a year or two—after obtaining an additional FDA indication for use of Hemlibra on patients without inhibitors. If the price doesn’t drop significantly, Roche will encounter pushback from insurance companies, slowing its move into the hemophilia marketplace for patients without inhibitors.

You can be sure that current leaders in the hemophilia marketplace won’t “go without a fight.” Assuming Roche can navigate legal challenges, Hemlibra is certain to make waves in the annual $2 billion BPA marketplace, affecting mainly Novo Nordisk (manufacturer of NovoSeven® RT, the most popular recombinant factor VIIa BPA used in inhibitor patients) and Shire (manufacturer of FEIBA, the most commonly used aPCC BPA for inhibitor patients).

Hemophilia patients are famous for their reluctance to switch products. But Roche has a winning combination with its new product: Hemlibra is significantly less expensive than current inhibitor therapies and significantly more effective at preventing bleeds. So Roche is poised to unseat the hemophilia inhibitor market. And, if Roche can lower the cost when offering the product to people with hemophilia and without inhibitors, then the company can potentially unseat the entire clotting factor industry—which mainly consists of producing factor VIII and BPAs.

Hemlibra is only the first of several disruptive therapies that will probably enter the market over the next several years. The wheels of change have been set in motion. Will the manufacturers lower their prices to stay competitive? Possibly, but prices did not drop with the introduction of any of the new clotting factors over the past few years. Will some manufacturers go out of business? Also possible, but not likely—all the big players in the hemophilia market have been planning for the arrival of disruptive therapies for some time, and they’re also developing their own disruptive therapies. This is the first rumbling of what will be a seismic shift in the hemophilia industry: within a decade, the hemophilia industry—and how we treat bleeds—will look very different.

1. The clotting process does not stop completely in the absence of factor VIII. Another branch of the clotting cascade, the extrinsic pathway, does allow for some clotting, but it’s inefficient compared to the intrinsic pathway that uses factor VIII.
2. The Haven 1 clinical trial studied once-weekly prophylaxis with Hemlibra compared to on-demand bypassing agents in adults and adolescents with hemophilia A with inhibitors. Results showed a reduction in bleed rate of 87% with Hemlibra prophylaxis, compared with on-demand treatment with BPA. And after a median observation time of 31 weeks, 62.9% of patients receiving Hemlibra—a comparison to 5.6% of those receiving on-demand BPAs—experienced zero treated bleeds.
3. For more information on Roche’s clinical trial involving Hemlibra, see [www.otcmarkets.com](http://www.otcmarkets.com)

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**Mark Your Calendar:**

**October 27th, 2018**

**Pines Manor**

**Casino Night**

The Hemophilia Association of New Jersey will be hosting our annual Casino Night. We would appreciate any type of donations: Gift Cards, Ready Made Baskets, Any New Items. All new items are welcomed. Call the HANJ office to let us know what you’d like to donate: (732)249-6000.

All donations are welcomed and appreciated!
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 Joe Markowitz (Joe.Markowitz@gmail.com, 201-650-0335) or Peter Marcano (petermarcano@gmail.com, 201-401-7080) 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.

Hemophilia Association of New Jersey

2018 Upcoming Events*

**Fall Educational Symposium**
September 13, 2018

**Kelly Brothers Scholarship Benefit**
October 13, 2018

**Casino Night**
October 27, 2018

**PACT Workshop**
November 2018

*This information is subject to change. Please check your mail for invitations with meeting dates and topics as well as RSVP instructions. We hope you will consider attending some of, or at least one, of these events. We look forward to seeing you.

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**HEMPLIBRA**

A once-weekly subcutaneous (given under the skin) injection for people with hemophilia A with factor VIII inhibitors

We extend our appreciation to the individuals, families, and healthcare providers who participated in the clinical trials that led to the approval of HEMPLIBRA®.

We thank you and celebrate with the community who made it a reality.

Discover HEMPLIBRA.com

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Discover HEMPLIBRA.com
Hemophilia A is a bleeding condition people can be born with where the body does not make enough of a blood clotting factor needed to form clots. It causes bleeding episodes that may last longer than usual and may cause muscle or joint bleeding.

WHAT IS THE MOST IMPORTANT INFORMATION I SHOULD KNOW ABOUT HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Discontinue prophylactic use of bypassing agents before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider’s instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with aPCC (FEIBA®), including:

- Thrombotic microangiopathy (TMA). This is a condition involving blood clot and injury to small blood vessels that may cause harm to your kidneys, brain, and other organs. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - confusion
  - stomach (abdomen) or back pain
  - weakness
  - swelling of arms or legs
  - feeling sick
  - decreased urination

- Blood clots (thrombotic events). Blood clots may form in blood vessels in your arm, leg, lung or head. Get medical help right away if you have any of the following signs or symptoms during or after treatment with HEMLIBRA:
  - swelling in arms or legs
  - pain or redness in your arms or legs
  - shortness of breath
  - chest pain or tightness
  - fast heart rate
  - trouble seeing

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.

WHAT ARE THE POSSIBLE SIDE EFFECTS OF HEMLIBRA?

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection; headache; and joint pain. These are not all of the possible side effects of HEMLIBRA.

WHAT IS HEMLIBRA?

HEMIBRA is a prescription medicine used for routine prophylaxis to help prevent or reduce the frequency of bleeding episodes in adults and children with hemophilia A with factor VIII inhibitors.

- HEMIBRA is a monoclonal antibody that binds to factor VIII, preventing factor VIII from forming blood clots in the body.

- HEMIBRA is given as a daily or twice daily subcutaneous injection under your skin.

- HEMIBRA is not a treatment for bleeding episodes.

HOW SHOULD I STORE HEMLIBRA?

- Store HEMIBRA as a refrigerated solution between 35°F (2°C) to 86°F (30°C) or below. Do not freeze.

- Store HEMIBRA in the original carton to protect the vials from light.

- Do not shake HEMIBRA.

- If needed, unopened vials of HEMIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMIBRA should not be stored out of the refrigerator for more than 7 days at 86°F (30°C) or below.

- HEMIBRA is transferred from the vial to the syringe.

- Throw away (dispose of) any unused HEMIBRA left in the vial.

WHAT ARE THE INGREDIENTS IN HEMLIBRA?

Active ingredients: emicizumab

Inactive ingredients: L-arginine, L-histidine, poloxamer 188, and L-aspartic acid.

WHAT IS A MEDICATION GUIDE?

A Medication Guide is a document that has been approved by the U.S. Food and Drug Administration (FDA). It is meant to help you and your healthcare provider understand and use a medicine properly. The information in this Medication Guide has been approved by the FDA. Your doctor may describe the medicine in other ways. A Medication Guide is not a commercial advertisement for the medicine.

How can I get additional information about HEMLIBRA?

See the detailed “Instructions for Use” that comes with your HEMLIBRA for information on how to prepare and inject a dose of HEMLIBRA, and how to properly throw away (dispose of) used needles and syringes. You may also report side effects to Genentech at (888) 835-2555.

Please see Brief Summary of Medication Guide on the following page for more important safety information, including Serious Side Effects.

Medication Guide Brief Summary

HEMLIBRA® (hem-lee-bruh)

(emicizumab-kxwh)

Injection for subcutaneous use

injection, for subcutaneous use

WHAT IS THE MOST IMPORTANT INFORMATION I SHOULD KNOW ABOUT HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Discontinue prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis. Carefully follow your healthcare provider’s instructions regarding when to use an on-demand bypassing agent, and the dose and schedule you should use. HEMLIBRA may cause the following serious side effects when used with aPCC (FEIBA®), including:

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  - stomach (abdomen) or back pain
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- HEMIBRA is transferred from the vial to the syringe.

- Throw away (dispose of) any unused HEMIBRA left in the vial.

Keep HEMLIBRA and all medicines out of the reach of children.

GENERAL INFORMATION ABOUT THE SAFE AND EFFECTIVE USE OF HEMLIBRA.

Medicines are sometimes prescribed for purposes other than those listed in a Medication Guide. Do not use HEMLIBRA for a condition for which it was not prescribed. Do not give HEMIBRA to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about HEMIBRA that is written for health professionals.

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How can I get additional information about HEMLIBRA?

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WHAT ARE THE OTHER POSSIBLE SIDE EFFECTS OF HEMLIBRA?

The most common side effects of HEMLIBRA include: redness, tenderness, warmth, or itching at the site of injection; headache; and joint pain. These are not all of the possible side effects of HEMLIBRA. You may report side effects to the FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Genentech at (888) 835-2555.

Please see Brief Summary of Medication Guide on the following page for more important safety information, including Serious Side Effects.
26th Annual
Kelly Brothers Scholarship Benefit
*In Memory of Bob & Dennis*

**Saturday, October 13, 2018**
2-6 PM

Friendly Sons of the Shillelagh
15 Oak Street, Old Bridge, NJ

$25 per Adult  *(Children free)*

Includes:
Food, Draft Beer, Wine, Soda, Water
DJ & Games for Kids
Any questions please call 732-679-5679

If unable to attend, donations greatly appreciated.

Checks can be sent to:
Hemophilia Association of NJ or H.A.N.J.
197 Route 18 South, Suite 206 North
East Brunswick, NJ 08816