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Get Your **SCARY** On!



October 26th, it's time for the Orlando Creepy Crawl 5K and Vampire Mile at Lake Baldwin on Broad Street in Baldwin Park, Orlando. Registration begins at 4 P.M., runners leave at 5 P.M., followed by walkers.

Join us for a scary Halloween-themed walk to raise funds for the HFGF. There will be costume contests and loads of fun, including Halloween parties afterward along with raising money for a great cause: helping the bleeding disorders community.

Can't make it this year? Plan ahead to walk and raise funds for the HFGF at one of our spring 2020 walks.

SAVE THE DATE!



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www.HemophiliaFlorida.org

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Mission
The mission of the Hemophilia
Foundation of Greater Florida is
dedicated to improving the qual-
ity of life for people with related
bleeding disorders and their fami-
lies through education, information
and referral services, advocacy
and research.

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individuals and recommends that
you consult your physician or
treatment center before pursuing
any course of treatment.

Dear Friends,

Can you believe it is fall already??? We have so much going on and we are looking forward to seeing you at our events and programs (see Calendar of Events).

We are so sad that JoAnn, who has been with us for more than 10 years, is really retiring and moving to South Carolina. We are going to miss JoAnn so much.

We had our first teen retreat this summer. It was so much fun that we are looking forward to making it an annual teen meeting.

What a great weekend at our Annual Family Retreat Weekend. We had 32 families gather at Camp Boggy Creek for a weekend of friendship and fun.

Do not forget to mark your calendars for the Creepy Crawls in Orlando and the Flight for Tomorrow Invitational Golf Tournament.

Warmest Regards,

Fran



October 26, 2019

Orlando Creepy Crawl

October 28, 2019

Flight for Tomorrow Golf Tournament

November 10, 2019

Product update, Tampa

November 17, 2019

Board Retreat, Orlando

December 1, 2019

Holiday Gathering, Jacksonville

December 7, 2019

Holiday Gathering, Gainesville

December 8, 2019

Holiday Gathering, Orlando

[CLICK HERE](#) to subscribe to our email to receive notices and updates of all our upcoming programs and events

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Meet the Recipients of the 2019 Calvin Dawson Memorial Scholarships

This year's 14 scholarship recipients, chosen on the basis of "merit, need, community service, and the aspirations of the applicant as reflected in an essay" are:

| Recipient Name | Hometown | Institution Attending |
|--------------------|----------------|---------------------------------|
| Alexia Barillas | Boca Raton | Palm Beach State College |
| Michael Berkman | Windermere | Syracuse University |
| Miguel Diaz-Burgos | Ocoee | Valencia College |
| Andrew Farren | St. Petersburg | St. Petersburg College |
| Xavier Fuentes | Orange Park | University of Florida |
| Olivia HoffSaint | Tampa | Mary's College |
| Justin Horbacz | Reunion | University of Florida |
| Tyler Nazario | Melbourne | Eastern Florida State College |
| Herman Robinson | Tampa | Webber International University |
| Natalie Rubin | Tampa | University of South Florida |
| Faith Runyon | Ocala | College of Central Florida |
| Hope Runyon | Ocala | College of Central Florida |
| James Solomon | Clermont | University of Central Florida |
| Raymond Torres | Valrico | University of Central Florida |



Calvin Dawson was the founder and first Executive Director of the Hemophilia Foundation of Greater Florida. The scholarships are given out annually to Florida residents with bleeding disorders who are pursuing post-secondary education at a college, university, or trade school. For more information, see the HFGF web site at HemophiliaFlorida.org/what-we-do/scholarships/ or to make a donation to the scholarship fund, go to HemophiliaFlorida.org/causes/

It's Not Too Late to Sign Up. . .

For the 23rd Annual Flight for Tomorrow Golf Tournament, to be held Monday, October 28th at East lake Woodlands Country Club in Oldsmar, Florida.

Register today to play or to provide sponsorship at HemophiliaFlorida.org/fundraising/golf-tournament/





Attend the Red Party and Raise Funds for the HFGF

Join us Saturday, January 18, 2020 at the American Muscle Car Museum at 3500 Sarno Road in Melbourne, Florida at 6 P.M. Please wear red cocktail attire. Regular tickets include a reception with light hors d'oeuvres/drinks/live auction and access to the American Muscle Car Museum. Tickets are \$100 each.

A limited number of VIP tickets are available for \$200 each and include a VIP private tour of the Museum. VIP tour starts at 5 P.M.

Members of the Brevard County bleeding disorders community spearheaded the organization of this event to raise funds for the HFGF and bleeding disorders awareness on the East Coast of Florida.

 Hemophilia Foundation of Greater Florida

RED PARTY

January 18, 2020 • 6pm • 3500 Sarno Road Melbourne, FL 32934

Don your favorite red cocktail attire and support Florida's bleeding disorders community!

 American Muscle Car Museum

General Admission Tickets: \$100 and includes a reception with light hors d'oeuvres, drinks, a live auction and access to the exclusive museum.

VIP Tour Tickets: \$200 and includes GA ticket offering + private tour

hemophiliaflorida.org/RedParty



Supporting people with bleeding disorders



FINDING FREEDOM THE 411 ON GENE THERAPY

Tommy Maguire loves to travel. For the past two years, he has journeyed to South America to volunteer with the Columbian Hemophilia Foundation. While seeing the world and helping others is gratifying, he wishes that toting factor along was not part of the itinerary. His wish may soon be granted.

As part of worldwide trials for gene therapy being administered to those living with Hemophilia, the 22-year-old Clearwater paralegal is joining scores of other hopefuls who are riding the wave of this cutting-edge medical procedure.

“They are very careful not to call it a cure, but at the hospital they are very excited about it,” he says. And that really comes as no surprise.

Gene therapy has been referred to as a wonder treatment with the ability to tackle a disease by allowing lab-enhanced genes to “tell” genes in the body how to function, and in turn, fend off the disease in question. Gene therapy is currently being applied to incurable diseases including Hemophilia with positive results. In the case of Hemophilia, only trials are currently underway and gene therapy is commercially not available yet. Moreover, the therapy is not available within the Pediatric population and not every adult with a bleeding disorder is an eligible candidate. The selection process is rigorous, even if a patient’s Hemophilia Treatment Center team recommends them for the therapy.

Nonetheless, encouraging outcomes from both Hemophilia A and B ongoing trials do have the medical community enthusiastic about the therapy moving forward. “Researchers are interested in gene therapy for Hemophilia because it is a disorder that is caused by one gene, making it ideal to target with directed gene therapy,” says Anita Rajasekhar, MD, MS, Associate Professor, University of Florida, Department of Medicine, Division of Hematology/Oncology. “Also, gene therapy is ideal for Hemophilia because patients with severe Hemophilia [$<1\%$ factor] may be able to significantly reduce spontaneous bleeding risk by increasing their factor level by a few percentage points.” Dr. Rajasekhar says the goal of gene therapy in Hemophilia is to allow the person’s own body to make a functional form of the clotting factor they are missing, that being either FVIII or FIX.

How Does it Work?

According to Dr. Rajasekhar, normal factor is packaged in the laboratory into an outer shell of a virus called adeno-associated virus (AAV). The AAV, also known as a vector, contains the normal factor gene and is delivered to the person’s liver intravenously as a one-time infusion. The new gene then “tells” the liver cells to create the normal clotting factor that was previously missing “Some use the analogy of a car, passenger, and destination,” she says. “The AAV vector is the car and the gene is the passenger. The car (the vector) drives the passenger (the factor VIII or IX gene) to its destination (the liver).”

As relatively new therapy, Dr. Rajasekhar says side effects are being closely monitored. During the actual IV infusion of the gene therapy, patients can experience pain or irritation at the injection site called an infusion-related reaction. Other reported side effects include fever, chills, runny nose, sneezing, cough, fatigue, nausea, muscle pain, feeling light-headed, and generally having signs of an allergic reaction.

“The vector that is used in most clinical trials, the AAV, cannot multiply or cause infection,” she adds. “People who have been treated with gene therapy in many studies have not been shown to ‘infect’ others.”

One of the biggest challenges facing AAV gene delivery is the host immune response, she explains, especially liver toxicity. This destruction of liver cells can lead to the loss of therapy efficiency. Further, some patients house antibodies for the AAV, which means they are immune to current therapy.

One and Done

Jonathan Lebron of Orlando, diagnosed with severe Hemophilia A as a baby, underwent the procedure at the Hemophilia Treatment Center at the University of Miami and is now being monitored at the University of South Florida Hemophilia Treatment Center. He says after a lot of testing, he spent two days and nights in Miami receiving gene therapy.

“My hematologist recommended me as a good candidate for gene therapy,” he says. “I was one of the last patients in

cont’d on page 6



FINDING FREEDOM THE 411 ON GENE THERAPY

cont'd

On arrival at the HTC, Jonathan says the product was first defrosted and then infused, which took about six hours. That was followed by continuous monitoring for the next day and night. Since close, vigilant monitoring is required, clinical trials are only conducted at Hemophilia Treatment Centers or similar locations with expertise in Hemophilia and gene therapy.

Back at home, Jonathan's twice-a-week infusions of factor are now history. Jonathan only needs to infuse in the case of an emergency. He feels great and looks forward to positive results from tests that will be administered for a year. That includes lab work twice a week for three months, then once a month until given the all clear.

Diagnosed with severe Hemophilia A during circumcision, Tommy Maguire will receive his gene therapy at St. Joseph's Hospital Center for Bleeding and Clotting Disorders. He initially consulted his hematologist about a new drug designed to drop the level of his Hemophilia from severe to mild.

"He said, 'how about the cure?' He was talking about gene therapy. But we don't like to use the word cure. We don't want to jinx it."

Tommy says that after the initial levels of testing are through, the actual procedure is simple.

"It's one time and done," he says about the infusion process. "It will be exciting not to take factor anymore. I have self-infused since I was six."

Future Looks Bright

No one can predict a target date for if and when gene therapy for Hemophilia will be widely accessible for the bleeding disorders community and how affordable it may be but initial findings are cause for hope.

"Since studies are still in clinical trials with relatively short follow-up time, I think it is too soon to say one way or another," says Dr. Rajasekhar. "But preliminary data is very promising and with multiple studies showing similar positive results, we are probably on the right track. There have been studies in both Hemophilia A and Hemophilia B that have shown positive clinical results in terms of increased factor activities, decreased bleeding events, decreased need for

factor prophylaxis, and improved quality of life."

Hemophilia B trials began earlier and are farther along than Hemophilia A trials.

Brett Palaschak, diagnosed with severe Hemophilia A when he was three days old, embraced his affliction by making a career out of gene therapy research and production.

At the University of Florida, Brett worked in labs conducting research with mouse models, and today he is employed by a gene therapy drug manufacturer.



Based on his work, Brett believes gene therapy treatments will be widely available to the bleeding disorders community in the next five years, if not sooner, pending FDA approval. "It is really, really promising," he says of the advancements being made. "The science is there, it just takes time. The trouble will be how to pay for it."

Fran Haynes, executive director of the Hemophilia Foundation of Greater Florida, says gene therapy for the bleeding disorders community could be a game changer, but she remains cautious.

"This is an opportunity, not a cure," she says. "The therapy may last five years, maybe ten years. We don't know."

In the meantime, the National Hemophilia Foundation is developing a Gene Therapy Initiative to offer a multi-pronged approach to ensure accurate information, tools and resources are available to everyone from patients to policy makers. In the future, Dr. Rajasekhar believes gene therapy may also be available to patients with mild or moderate disease or patients with current inhibitors or a history of inhibitors.

"It is an exciting time for Hemophilia since we now have several different types of therapies for Hemophilia patients, not just simply replacing the clotting factor they are missing."

If you are an adult member of the bleeding disorders community and would like to look into gene therapy treatment, talk to your hematologist at your Hemophilia Treatment Center. Gene therapy trials are currently enrolling patients with severe Hemophilia since these are the patients who would benefit the most. Clinical trials are enrolling only patients without concurrent liver disease. Inhibitor patients are generally excluded from current open trials.



Meet the New Outreach and Education Nurse



Please join us in welcoming the HFGF’s new Outreach and Education Nurse, Leanne Buchanan. Her background has been in public health and education. Most recently, she served as a School Board Nurse for Seminole County Public Schools. She graduated from the University of Central Florida.

Leanne says, “From the moment I stepped into the HFGF office, I felt like it was home. The staff has welcomed me into the family and the entire bleeding disorders community has been so kind.” Her goal is to increase independence through education. “Living with a chronic medical condition myself, I have learned that I am not my diagnosis. I want to help each of you embrace your disease and learn to live your best life,” she said.

Leanne is a native Floridian currently living in Winter Springs with her husband, Greg, and two dogs. When she is not at work, she enjoys spending time with her family, going to estate sales, Disney visits, going to the dog beach, and traveling.

If you have a question or just want to say hello, you can reach Leanne at Leannebuchanan@hemophiliaflorida.org or by calling the HFGF office at 800-293-6527.

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Research Update

WFH-Led Research Points to Higher Global Hemophilia Prevalence

The World Federation of Hemophilia (WFH) recently announced the publication of a new study on the global prevalence of hemophilia, the results of which suggest a number that is significantly higher than the previous estimates. Findings from this eye-opening study were published in the *Annals of Internal Medicine*.

WFH recognized that having a clearer understanding of hemophilia prevalence will only enhance efforts to improve health care and quality-of-life for patients worldwide. They therefore put out a call to action that ultimately inspired researchers from McMaster University, North Carolina State University, Aix-Marseille University, and the University of Sheffield to conduct a meta-analysis of registry data in countries with comprehensive patient registries. These included Australia, Canada, France, Italy, New Zealand, and the United Kingdom. Investigators, working in concert with WFH's Data and Demographics Committee (DDC), uncovered a prevalence, per 100,000 males, of 17 for all severities of hemophilia A, six for severe hemophilia A, four for all severities of hemophilia B, and one for severe hemophilia B.

The study was unprecedented for also estimating hemophilia prevalence at birth, which is defined as the number of people born with hemophilia per 100,000 male births. Investigators' analysis established that prevalence at birth per 100,000 males is 25 for all severities of hemophilia A, ten for severe hemophilia A, five for all severities of hemophilia B and two for severe hemophilia B. The study generated an overall global estimate of approximately 1,125,000 males with hemophilia, representing a dramatic increase when compared to the previous estimate of 400,000 worldwide. Further, 418,000 of these are estimated to have severe hemophilia, the majority of whom are undiagnosed.

"This paper is a milestone in our journey to providing care to hemophilia patients worldwide. Knowing how many patients are expected in each country given its population is an important measure of the efficiency of the health care system. Knowing how many patients should be there, and how many less are reported to national and international registries is a measure of the work left to be done," said Alfonso Iorio, MD, lead author and past chair of the DDC. "Knowing how

many patients are out there will enable health care systems to estimate the resources needed to treat the disease, and will enable drug manufacturers to increase the investment in research to match the demand of a patient population three times larger than previously thought. It will also serve to underline the importance of outreach and identification campaigns."

The article, "Establishing the Male Prevalence and Prevalence at Birth of Hemophilia: A Meta-Analytic Approach Using National Registries," appeared September 10, 2019 in *Annals of Internal Medicine*.

Source: WFH press release dated September 10, 2019



WFH

WORLD FEDERATION OF HEMOPHILIA



We Miss You Already

We were so sad to say goodbye and good luck to Joann Brownrigg as she heads off into her second retirement in Sun City, South Carolina near Hilton Head. Joann gave tirelessly of her time and effort to the HFGF for more than 15 years as both a volunteer and an employee.



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