



The Quarterly Newsletter of the HFGF Summer 2023 Volume 41 Issue 67

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## The Hemophilia Foundation of Greater Florida Will Be Changing Its Name to the **Bleeding Disorders Foundation of Florida**

The newly named, Bleeding Disorders Foundation of Florida will be serving people with all bleeding disorders such as hemophilia, von Willebrand's Disease, platelet disorders, factor deficiencies, and other rare bleeding disorders. The new name reflects the Foundation's efforts in creating a more inclusive culture.



**Bleeding  
Disorders**  
*Foundation of Florida*

A bleeding disorder is a defect in the body's blood clotting system. Clotting factors are either missing or do not work as they should, which may cause problems with bleeding too much after an injury or surgery.

"We are excited to announce the name change and our new name will be inclusive of other bleeding disorders besides hemophilia," said Fran Haynes, Executive Director. "This will allow our organization to serve more Floridians with bleeding disorders."

For over 27 years, the Hemophilia Foundation of Greater Florida has been helping those affected by and living with bleeding disorders throughout the state of Florida to live without limits through much-needed services and programs.

As a 501(c)(3) non-profit organization, the Foundation has been successful in achieving its mission to improve the quality of life for people with bleeding disorders and their families through education, information and referral services, advocacy and research.

## HFGF Connections

Published quarterly by  
The Hemophilia Foundation of  
Greater Florida

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Mission  
The mission of the Hemophilia  
Foundation of Greater Florida is  
dedicated to improving the quality  
of life for people with related bleed-  
ing disorders and their families  
through education, information and  
referral services, advocacy and  
research.

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center before pursuing any course  
of treatment.

## Executive Director's Column

Dear Friends,

Happy Summer! I hope everyone is enjoying their summer fun...it goes by so fast; thank goodness we live in Florida so we can enjoy the warm weather longer. We have a lot of fun and educational programs scheduled ([Click Here to view our updated calendar](#)).

If you have not heard, the HFGF will be now be doing business as the Bleeding Disorders Foundation of Florida (BDFF). Thank you to the name change committee, and community to help us navigate this change.

Our Annual Family Program, Bleeding Disorders Jeopardy was a blast. Folks enjoyed a fun family program testing their bleeding disorders knowledge. Special thank you to [SeaWorld](#) for their generosity and wonderful support. They are the best!

Camp was June 12-16th and it was a lot of fun. Campers enjoyed swimming, archery, theater and so many more activities. We have been a camp partner for more than 26 years.

It is time to register for the Superhero Walk—we are looking forward to a stellar event with lots of guest appearances, face painting and superhero fun.

I hope everyone has an amazing summer, and I look forward to seeing you soon.

Take care,

*Fran*



- August 10, 2023** Getting the Game Program – Topgolf – St. Pete  
**August 16-20, 2023** Bleeding Disorders Conference - Maryland  
**August 26, 2023** Family Program - Tampa  
**September 23, 2023** Superhero Walk – St. Pete  
**October 23, 2023** Flight for Tomorrow Golf Tournament – Oldsmar  
**October 28, 2023** Creepy Crawl 5K/Walk – Orlando

HIGH SCHOOL STUDENTS  
EARN COMMUNITY SERVICE HOURS

**VOLUNTEER FOR THE HFGF**

CONTACT US AT 800-293-6527 or  
[info@hemophiliaflorida.org](mailto:info@hemophiliaflorida.org)



**DONATE**



## Annual Family Program Was Fun For Everyone

More than 500 people with family members who have a bleeding disorder met for a fun filled educational day, playing **Bleeding Disorders Jeopardy**. The participants had fun as they competed and tested their bleeding disorder knowledge and while learning new information.

Special thanks to our game show hosts Hector Heer, Regional Care Coordinator & John Martinez, Regional Care Coordinator, Education, Development Specialist from BioMatrix. Both Hector and John are part of the bleeding disorders Community and have been involved for more than 20 years.

After the event, attendees were free to enjoy the SeaWorld experience. HFGF wants to especially thank our volunteers and sponsors for making the day possible:



For your generosity and support!

### THANK YOU DISPLAY SPONSORS

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TAKEDA



Join us as we run and walk together to raise funds and awareness for the bleeding disorders community. From dressing up as your favorite superhero to wearing your Halloween costume, these are events you will not want to miss. Join us **September 23rd for the Superhero Fun Walk**, and/or **October 28th for the Creepy Crawl 5K Run & Walk**. Gather friends, family, neighbors, and coworkers to join the fun.

Don't delay, register today!

### SUPERHERO FUN WALK



### Creepy Crawl 5K Run & Walk



## The HFGF has received the Newman Circle of Friends Award

The Hemophilia Foundation of Greater Florida (HFGF) received the Newman Circle of Friends Award from Camp Boggy Creek for donating more than ONE MILLION DOLLARS over the last 27 years to the HFGF Camp Spirit Camping Programs.

Your generous support of the Golf Tournament, Theater Event, Walks, Grants and other fundraising events, made this possible.

Thank you from the bottom of our hearts!





## 27th Annual Flight for Tomorrow Invitational Golf Tournament



Have fun while supporting the bleeding disorders community at the 27th Annual Golf Flight for Tomorrow Golf Tournament on Monday, October 23, 2023 at the beautiful East Lake Woodlands Country Club. Form a team and join the fun!

For more information, email [info@hemophiliaflorida.org](mailto:info@hemophiliaflorida.org) or call (800) 293-6527.

## Pat Solomon Memorial Bike Ride and Fun Walk - December 9, 2023

Patrick (Pat) Solomon was a very special Hemophilia Foundation of Greater Florida friend and active volunteer. With several family members having hemophilia, Pat had a personal commitment to the HFGF mission. He was an extraordinary individual. We were fortunate to be the recipients of his dedication and kindness. We are beyond excited for this fundraising event in memory of Pat.

Join us on December 9th at the Clermont Trail in Waterfront Park, 300 3rd Street Clermont, FL, for the Inaugural Pat Solomon Memorial Bike Ride and Fun Walk!

For more information call  
800-293-6527  
[info@hemophiliaflorida.org](mailto:info@hemophiliaflorida.org)



## The HFGF Receives Grant to Support those with von Willebrand Disease

The Hemophilia Foundation of Greater Florida is grateful to be a recipient of the Community Foundation of Sarasota County's Grant Award. With their help, we will be able to better support those with von Willebrand Disease in our community.



After several decades of preclinical and clinical research, pitfalls and progress, gene therapies for hemophilia A and B have become a reality with recent FDA approvals. While these therapies along with others still in development, represent both promise and a notable scientific achievement, outstanding questions relevant to long terms efficacy and safety remain. Authors of a new review “Hemophilia Gene Therapy: First, Do No Harm,” published in the Journal of Thrombosis and Haemostasis (JTH), tackle these concerns.

The authors address fundamental considerations for hemophilia patients, cognizant that each individual has their own personal health goals and comfort level with open-ended questions of long-term risk vs. therapeutic benefits. Safety, in context of the hemophilia communities’ unique history, is a prominent theme through line in this paper. They acknowledge concerns of the potential for both innate and adaptive immune responses to adeno-associated viral (AAV) vectors and to the possible integration of the given vector into the genome of gene therapy recipients. These types of responses could have safety and efficacy impacts, including inflammatory effects on the liver or the development of tumors or malignancies.

“Gene therapy is a complex biological ‘drug’ for which, despite 30 years of development, there are many unresolved questions, and the unknowns remain top of mind for clinicians and PwH alike. Evaluation of the risks and benefits of any new therapy requires the careful consideration of all the available information and a shared decision-making approach should be employed, explain the authors. “This is particularly important in the consideration of gene therapy, given the fact that AAV-mediated gene therapy is a one-time irreversible therapy. A fully informed decision must be ensured, and a robust shared decision-making approach is mandatory for these therapies.”

The review summarizes clinical trial data that supported the regulatory authorization of valoctocogene roxaparvovec in Europe to treat hemophilia A and etranacogene dezaparvovec-drlb in Europe and the United States to treat hemophilia B.

Since the publication of this paper, valoctocogene roxaparvovec also received U.S. Food and Drug Administration [approval](#) under the brand name ROCTAVIAN™.

The authors highlight initiatives taken by NHF and other organizations to prioritize safety for hemophilia patients who are either considering gene therapy or for those who have received the one-time treatment. A primary example of this is NHF’s submission of a Citizen Petition to the FDA in 2022 requesting that a Risk Evaluation and Mitigation Strategy (REM) be required as a condition of approval for both valoctocogene roxaparvovec and etranacogene dezaparvovec. Read the full petition to learn more.

While the aforementioned products received FDA approval

without meeting the REM requirements, the document’s key elements remain relevant to protecting long term patient safety. These include training and education for healthcare providers (HCPs) on gene therapy and its management in patients with hemophilia, with particular emphasis on the central role of shared decision making (SDM). The crucial function of facilities charged with administering gene therapies, specifically federally funded hemophilia treatment centers, is also emphasized.



The authors stress the importance of high enrollment in the World Federation of Hemophilia’s global gene therapy registry, ideally placed to collect adverse event data and other developments from patients who receive these products. Steps towards achieving greater health equity, including broader representation in clinical trial design, is also a focus.

The paper concludes with a series of recommended steps that could be taken within the hemophilia community to ensure the safety and optimal outcomes for PwH who might opt to receive a gene therapy product. These recommendations dovetail closely with the elements of the earlier REM. This article is currently available in a pre-proof PDF version on the JTH website.

NHF also recorded a webinar that serves as an excellent companion resource to the review article. It was presented by lead author Leonard A. Valentino, MD, President, and CEO of NHF.

Lastly, please see this additional paper highlighting a great tool for SDM and gene therapy.

### Citation

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**Disclaimer: NHF provides periodic synopses of articles published in peer reviewed journals, the purpose of which is to highlight papers that cover a wide range of topics and speak to a broad spectrum of the inherited blood disorders community. Topics include shared decision making, gene therapy, health equity, and more. NHF hopes you find this content to be informative and engaging.**

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