WE’RE BACK AND WE MISSED YOU

Saturday, June 5th dawned hot and sunny. Three hundred members and families from the bleeding disorders community as well as volunteers gathered to enjoy breakfast and a program, “Coming Out of Your Shell Life After the Pandemic.” The talk was given by Julie Taylor, Licensed Marriage and Family Therapist from Winter Garden Kids.

Following the program, everyone was free to enjoy the park, and they did! It was great to be back after the pandemic.

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Executive Director’s Column

Dear Friends,

Summer is off to a great start. We had our first in-person program June 5, 2021 at SeaWorld, and it was a blast. Special thanks to our speaker Julie Taylor, Licensed Marriage and Family Therapist, and our sponsors. It was wonderful seeing everyone.

Thank goodness we were able to have camp July 23-25, 2021…although we had to pivot from a week-long camp to a summer family retreat weekend, it was a lot of fun. Missed this one? We still have our annual fall Family Retreat Weekend scheduled for Oct. 8-10. We’d love to have you sign up for that one.

The Gator Clot went off on August 7th and was a huge success! The money raised will support the HFGF mission: to improve the quality of life for people with bleeding disorders. Every trotter counted! You can still sign up for one of our three upcoming fall walks.

We have a lot of fun programs scheduled (view our updated calendar at https://www.hemophiliaflorida.org/events/list/). We are following CDC guidelines.

I hope everyone stays healthy, and I look forward to seeing you soon.

Take care,

Fran

Calendar of Events

August 21, 2021  Bleeding Disorders Trivia - Adventure Island, Tampa

September 9, 2021  Being Prepared for a Hemophilia Emergency - Location to TBD

September 25, 2021  Super Hero Fun Walk Vinoy Park, St. Petersburg

October 8-10, 2021  Family Retreat Weekend Camp Boggy Creek, Eustis

More Information: https://www.hemophiliaflorida.org/events/list/
A BIG THANK-YOU TO ALL OUR SPONSORS WHO MADE THE DAY POSSIBLE!

HIGH SCHOOL STUDENTS

EARN COMMUNITY SERVICE HOURS
VOLUNTEER FOR THE HFGF
CONTACT US AT 800-293-6527
INFO@HEMOPHILIAFLORIDA.ORG

And a very special
Thank-you to our Friends at

SeaWorld ORLANDO
Scientific Overview of AAV Vectors and Hemophilia Gene Therapy


In this review, the authors discuss some of the primary considerations relevant to investigational gene therapies that employ adeno-associated viral (AAV) vectors, with particular focus on immunogenicity and genotoxicity – the former denotes the ability of a foreign substance to trigger an immune response, while the latter refers to a substance’s ability to damage genetic material.

AAV vectors act as delivery vehicles, carrying the genetic messaging that prompts the production of the factor VIII or factor IX proteins that are deficient in people with hemophilia A and B, respectively. Ideally, AAVs deliver this genetic material into living cells to sustain a therapeutic effect that is free of unintended adverse reactions in the short and long term. The challenge, and source of continued investigation by scientific researchers, is to better understand the nature of unwanted biological responses in patients who receive the one-time therapy. Foremost of these concerns are the potential for both innate and adaptive immune responses to AAVs and to the possible integration of the given vector into the genome of patients who have received the therapy. These types of responses could have safety and efficacy impacts, including inflammatory effects on the liver or the development of tumors or malignancies.

Authors of the JCM paper review the evolution of AAV-based gene therapy, including descriptions of the unique vector types that continue to be investigated in both preclinical research and in clinical trials. The article also sheds light on the nuances of each vector and how they interact with a person’s natural immunity, including insights into the disparate responses elicited in hemophilia A, versus hemophilia B patients, all with an eye towards achieving a better understanding of long-term safety and efficacy.

The authors go on to lay out various approaches to circumventing possible unwanted effects of AAV gene therapy, including enhanced screening for pre-existing neutralizing antibodies (Nabs), refinements to vector design, and adjustments in manufacturing that may allow the use of lower vector doses to forestall possible negative impacts of vector integration. They also reaffirm the importance of long-term follow-up of trial participants to glean valuable insights on the therapies’ overall impact for persons with hemophilia (PWH).

“Gene transfer is likely to play an increasingly important role in the treatment of genetic diseases such as hemophilia. Important challenges remain to be overcome, such as finding solutions to immune-related problems associated with viral vectors to provide safe, predictable, effective, and durable outcomes for PWH,” concluded the authors. They added, “Despite the challenges that remain to be overcome, the potential for gene transfer to improve therapeutic outcomes is significant. Novel frontiers, such as tolerance induction, show promise for the development of curative treatments for hemophilia.”

The paper, which was published online in JCM on June 2, 2021, is open access – Follow this link to read the full review.
The Gator Clot Trot, held in Gainesville on Saturday, August 7th was a great success, not only meeting but exceeding its fundraising goal, raising $31,225.60 for the HFGF to provide help for the bleeding disorders community.

More than 100 people attended the event and HFGF would like to thank all of our team captains, walkers, volunteers and Sponsors for making it possible. CDC guidelines were followed, and a fun time was had by all.

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Let’s All Get Outside and Walk!

Remember being cooped up at home for much of the pandemic? We do too. Did you know that studies show that being outside in nature has both physical and mental health benefits? You can enjoy all of those and the benefits of community as well, while helping to raise funds for the HFGF.

**Participate in one of our walks coming up in September and October**

**Super Hero Fun Walk**  
September 25, 2021  
Vinoy Park  
701 Bayshore Dr NE  
St. Petersburg, FL 33701

**Jacksonville Creepy Crawl**  
October 23, 2021  
Nocatee Station Field  
400 Nocatee Center Way  
Ponte Vedra, FL 32081

**Orlando Creepy Crawl**  
October 30, 2021  
Harbor Park at Baldwin Park  
4990 New Broad St  
Orlando, FL 32814

25th Annual Flight for Tomorrow Golf Tournament  
Monday October 25th, 2021  
East Lake Woodlands Country Club Oldsmar, FL  
For more information, to sign up, donate or become a sponsor, go to:  
https://www.hemophiliaflorida.org/fundraising/golf-tournament/