

HFGF CONNECTION



The Quarterly Newsletter of the HFGF Summer 2022 Volume 37 Issue 63

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TOLL FREE 800-293-6527
www.HemophiliaFlorida.org

Volunteer Spotlight: Joe Sena

Joe Sena with Aairius DJ Entertainment has been a part of the walks with the Hemophilia Foundation since 2014, providing the soundtrack for these events. He's taken part in our Gainesville, Tampa & Jacksonville walks.



Joe started out as a DJ back in 2008 in Tallahassee and eventually started his company in June of that year. He has been a DJ ever since (along with holding down a regular job)! He's DJed many corporate events such as weddings, birthday parties, Gulf Winds Track Club events in Tallahassee and recently added the Monticello Watermelon Festival to his resume.

Even though he's based out of Tallahassee, he travels all over the state, and we're always happy to have him help out at our HFGF events.

You can contact Joe through his website at www.aairiusdj.com, Facebook (www.facebook.com/aairiusdjentertainment), Email: joe@aairiusdj.com, or call him at (850) 212-5083.



Save the Date!

Family Retreat Weekend
October 7, 2022

For more information, go to: <https://www.hemophiliaflorida.org/what-we-do/family-retreat/>

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HFGF Connections

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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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consult your physician or treatment
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 (view our updated calendar at <https://www.hemophiliaflorida.org/events/list/>).

Our annual family program: New Day, Fresh Start...Lets Go! at SeaWorld was a blast. Folks enjoyed a fun program facilitated by Michael Rosenthal. Michael always knows how to create fun. Special thank you to SeaWorld for their generosity and wonderful support. They are the best!

Camp was a lot of fun. Campers enjoyed swimming, archery, theater and so many more activities.

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

Take care,

Fran

mark  your **Calendar**

Superhero Walk

September 24, 2022 – Tampa

Family Retreat Weekend

October 7, 2022, Camp Boggy Creek, Eustis

University of Miami Program

October 16, 2022 – Miami

Golf Tournament

October 24, 2022 – Tampa

Orlando Creepy Crawl

October 29, 2022 – Orlando

Teen Retreat

November 5, 2022 – Location TBD



SeaWorld Outing Was a Great Success!

More than 450 people with family members who have a bleeding disorder met for breakfast and to enjoy a program entitled "New Day. Fresh Start, Let's Go!" The program got the audience involved in a discussion of the impacts of Covid on their lives, and how life is returning to normal, and everyone gets a fresh start as they return to work and school and other activities. The program was presented by Michael Rosenthal, an award-winning speaker for the bleeding disorders community with more than 30 years of leadership experience. 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:

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TAKEDA





Alexander Monica spends his work day and some of his free time working with members of the bleeding disorders community. HFGF got Alex to sit down for a Q&A session to learn some things about him and his volunteer work for the HFGF.

Why do you volunteer for HFGF?

"I volunteer for HFGF because of the community and HFGF's mission. I am professionally and personally connected with lots of members in this community and feel that HFGF tirelessly works to improve the quality of life for these folks. I couldn't think of a better group of people to support."

How long have you been an HFGF volunteer?

"I have been a volunteer for a little less than two years now but don't plan on going away anytime soon."

What is one thing about you that would surprise people if they knew it?

"I am surprisingly athletic. I know my figure doesn't paint the picture, but I played four sports in high school including tennis, basketball, football, and golf."

Tell us about yourself in a couple of sentences.

"I am a Native Floridian, raised in the Midwest. I am a husband to my lovely wife Kylie and a father of two, Georgia and Brandt. I love being outdoors, staying active, and finding ways to challenge myself mentally and physically. My life motto is 'life is short, choose happiness'."

Come to the Superhero Walk on September 24, 2022, and you can meet Alex yourself. The Walk is at Vinoy Park in St. Petersburg. Registration starts at 9 A.M. and the Walk begins at 10 A.M.



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Greater Florida



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Flight for Tomorrow Golf Tournament

The Flight for Tomorrow Golf Tournament is coming up Monday, October 24th, 2022, at East Lake Woodlands Country Club in Oldsmar, Florida. This is the HFGF's single largest fundraising event.

Why should I play in the tournament?

The funds raised from the tournament go to further the work of the HFGF in helping the bleeding disorders community by supporting the Foundation's many programs including Camp Spirit, a week long camp for children 7-16 with a bleeding disorder; the emergency assistance fund that helps families who have a family member with a bleeding disorder with rent, utilities, medical or dental care, health insurance and more; education programs that the HFGF sponsors all over the state to raise awareness of bleeding disorders and get the community vital information.

Not to mention having a fun day on the course.

How much does it cost to play in the tournament?

\$250 for an individual golfer or \$1000 for a foursome.

How do I register for the tournament?

Just go to: <https://www.hemophiliaflorida.org/fundraising/golf-tournament/> and sign up.

Are there other ways that I can help besides playing golf?

Sure, you can make a onetime donation at the web address above.

You could also help the HFGF secure experiences (like golfing, theme park visits, professional or college sports tickets) or travel options (airline tickets, hotels, restaurants) or autographed sports memorabilia to be part of the auction that occurs the day of the tournament. Call Us: (800) 293-6527.



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MASAC Updated Recommendations

NHF's Medical and Scientific Advisory Council (MASAC) has approved a new recommendation on genotyping for people with hemophilia, which were endorsed by the NHF Board of Directors on July 6th.

The document provides a brief historic background of genotyping, including NHF's efforts to advance its usage in the hemophilia community. It also highlights its proven utility and many benefits, including enhanced diagnosis and screening for carrier status, inhibitor risk, complementing personalized therapy, and for anticipating an individual's potential candidacy for gene therapies as they become available.

MASAC subsequently issued a series of specific recommendations geared towards broadening access to genotyping, plus emphasizing the importance of specialized laboratory testing.

MASAC Document 273 - Recommendations on Genotyping for Persons with Hemophilia

Revisions: 248; 214; 96

Since the genes for factors VIII and IX were identified and sequenced in the 1980s, numerous gene changes have been identified in persons with hemophilia A and B. This information has led to increased understanding of the molecular biology of these genes and has established new correlations between a person's genotype and phenotype. High through-put technology and strategies for more efficient genotyping have reduced the costs of doing genotyping significantly.

In 1998, MASAC recommended that NHF identify public and private funding sources that could facilitate widespread genotyping efforts in the hemophilia community. In 2003, this concept was endorsed but not funded by Congress. Another event that made a community-wide genotyping project feasible was the passage by Congress in 2008 of the Genetic Information Nondiscrimination Act (GINA). This act guarantees that genetic information cannot be used to discriminate against an individual. A multi-sector partnership formed between NHF, the American Thrombosis and Hemostasis Network, and Bloodworks to meet unmet needs for genotyping in the community through the My Life, Our Future (MLOF) program. MLOF provided free hemophilia A and B genotyping for patients with a diagnosis of hemophilia and at-risk female relatives in the U.S. from 2013-2017. After the completion of MLOF, individuals have had to have testing performed as part of standard clinical practice.

Barriers to testing include access and financial considerations. Free genotyping services, such as the 8Check Gene Mutation Testing Service for patients with hemophilia A, can be a testing resource for patients and their at-risk relatives, when eligible.

Genotyping is high yield, with >98% of individuals with hemophilia A or B having an identifiable DNA change in their factor gene. Occasionally, individuals may have more than one DNA

change that can cause hemophilia. More than one genotype can also be detected in different affected members of the same family. In females, genotype is the most reliable method for diagnosis, as factor levels correlate less well with bleeding in females than in males, and factor levels may even be normal in a symptomatic (bleeding) carrier. Thus, individual genotype information is needed for each patient.

Genotype information allows individuals and healthcare providers to make or refine the diagnosis of hemophilia, predict hemophilia severity, determine inhibitor risk, identify female genetic carriers, help with reproductive counseling and birth planning, and lead towards improved, individualized treatments. Also, with the emergence of experimental gene therapies, genotyping is important in predicting those individuals who might be potential candidates and in selecting the most appropriate form of gene therapy for each individual.

An important aspect of genotyping is genetic counseling. Counseling should be provided to individuals and families pre- and post-genotype testing to ensure that they understand the implications of the test results.

Therefore, MASAC makes the following recommendations:

1. MASAC recommends that individuals with hemophilia A or B undergo genotyping
2. MASAC recommends that affected male relatives (or other individuals with a single X chromosome) undergo genotyping
3. MASAC recommends that females (or other individuals with more than one X chromosome) at-risk to have inherited a hemophilia-causing genetic change undergo hemophilia genotyping, regardless of factor VIII or factor IX level
4. MASAC recommends genotyping should be performed in a laboratory experienced in hemophilia genetics and results interpretation

References:

Johnsen JM, Fletcher SN, Huston H, Roberge S, Martin BK, Kircher M, Josephson NC, Shendure J, Ruuska S, Koerper MA, Morales J, Pierce GF, Aschman DJ, Konkle BA. Novel approach to genetic analysis and results in 3000 hemophilia patients enrolled in the My Life, Our Future initiative. *Blood Adv.* 2017 May 18;1(13):824-834. doi: 10.1182/bloodadvances.2016002923. PMID: 29296726; PMCID: PMC5727804. Srivastava A, Santagostino E, Dougall A, Kitchen S, Sutherland M, Pipe SW, Carcao M, Mahlangu J, Ragni MV, Windyga J, Llinás A, Goddard NJ, Mohan R, Poonnoose PM, Feldman BM, Lewis SZ, van den Berg HM, Pierce GF; WFH Guidelines for the Management of Hemophilia panelists and co-authors. *WFH Guidelines for the Management of Hemophilia*, 3rd edition. *Haemophilia*. 2020 Aug;26 Suppl 6:1-158. doi: 10.1111/hae.14046. Epub 2020 Aug 3. Erratum in: *Haemophilia*. 2021 Jul;27(4):699. PMID: 32744769. Jul 15, 2022

Paper Features Q&A Resource on Shared Decision Making and Gene Therapy

As investigational hemophilia gene therapies move closer to regulatory authorization, community stakeholders have recognized the acute need for people with hemophilia (PWH) and healthcare professionals (HCPs) to be fully engaged in shared decision making (SDM). While the concept of SDM, whereby patient and provider collaborate to reach informed treatment decisions, has gained support in recent years, the arrival of such a paradigm-shift will present unique challenges and opportunities.

Disparities in health literacy, the proliferation of inaccurate and contradictory content on social media, direct-to-patient marketing, plus the sheer complexity of gene therapy, may prevent a PWH from fully engaging in SDM. While some HCPs may also lack a thorough enough understanding of gene therapy, hindering their full participation in the SDM model. In addition, an HCP's perception of their patient's comprehension of this therapy may not align with their patient's actual understanding, a discordance that further compromises SDM, increasing the potential for delayed treatment decisions and other negative outcomes.

In light of these anticipated obstacles, an international and multidisciplinary group known of as the Council of the Hemophilia Community (CHC) was convened. Composed of independent advisors, HCPs, industry and patient representatives, the goal of the CHC was to fill these information gaps through the development of a resource that would help generate an ongoing dialogue between PWH/HCP, with patient-centricity as its guiding principle.

The CHC held three roundtable meetings between November 2020 and May 2021 wherein they fleshed out a series of questions and answers that would best foster a genuine SDM process amongst PWHs/HCPs. The majority of the decided-upon questions fell under several over-arching categories including treatment regimen/adherence requirements, treatment predictability and variability, treatment durability, and the risk/benefit profile.

Each of the questions were subsequently assigned to the five stages of the patient "decisionmaking journey." These

included 1) Pre-gene therapy information seeking 2) Pre-gene therapy decisionmaking 3) Treatment initiation 4) Short-term post-gene therapy follow up (less than one year since receiving gene therapy) 5) Long-term gene therapy follow up (more than one year after receiving gene therapy).

A recent paper published online in the journal Patient Preference and Adherence (PPA), describes in greater detail the process of developing the resource and context in which it was created.

The authors highlight the value of this tool to enhance SDM relevant to hemophilia gene therapy, while also hinting at its potential utility in other disease groups.

"The educational and decision support resources described herein recognize that each patient's decision journey will evolve throughout their lifetime with their individual preferences at different life stages, and with the emergence of new therapies and a growing evidence base," explain the authors. "The Q&A resource provides HCPs and PWH with timely, relevant information, facilitates discussions, and empowers PWH to engage in shared decisionmaking. As gene therapy products enter the market, the themes and questions mapped here should stimulate discussion and aid interactions among HCPs, PWH, and family members, to ensure that they are fully informed and realize the clinical potential of this treatment. While the issues discussed here pertain to hemophilia, they could also be applied to other hereditary diseases with multiple treatment options."

Citation

Wang M, Negrier C, Driessler F, Goodman C, Skinner MW. The Hemophilia Gene Therapy Patient Journey: Questions and Answers for Shared Decision-Making. Patient Preference Adherence. 2022 Jun 9;16:1439-1447. doi: 10.2147/PPA.S355627. PMID: 35707346; PMCID: PMC9191577.

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**CHANGE THEIR WORLD. CHANGE YOURS.
THIS CHANGES EVERYTHING.**

BECOME A VOLUNTEER

OR

DONATE



Samantha Nazario is a National Ambassador for bleeding disorders awareness and was chosen as the 2021 National Advocate of the Year.

Ty Benoit works from home with the Washington State Pandemic EBT Program. HFGF got these two busy people to pause and answer a few questions about volunteering for the HFGF.

Samantha Nazario

Why do you volunteer for the HFGF?

"I like helping another family on their journey with a bleeding disorder; helping other people to not feel alone and to know that the HFGF is there to help them."

How long have you been an HFGF volunteer?

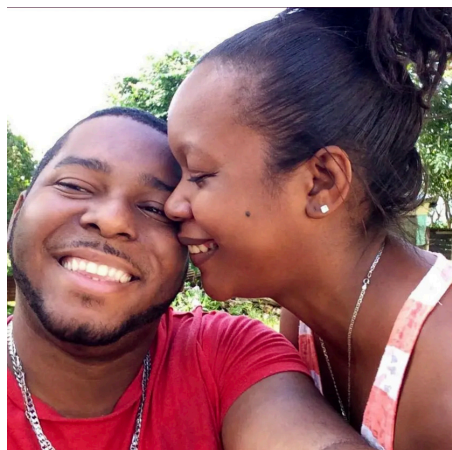
"For 17 years now, it has been my honor to volunteer with the HFGF family."

What is one thing about you that would surprise people if they knew it?

"I studied Performing Arts and at the age of 14, I recorded a song with Cyndi Lauper called "Tear Hate Apart."

Tell us about yourself in a couple of sentences.

"I am a mother, wife, small business owner, radio cohost and an advocate not only for bleeding disorders but also for my Latino community here in Brevard. I am a Bronx-born native of Puerto Rican descent."



Ty Benoit

Why do you volunteer for the HFGF?

"Being able to see that something I did can put a smile on someone else's face and feeling that I'm helping with something that's bigger than me as an individual."

How long have you been an HFGF volunteer?

"For five years now."

What is one thing about you that would surprise people if they knew it?

"That I've only been on Factor for five years now."

Tell us about yourself in a couple of sentences.

"I am originally from Dominica and moved to Orlando in 2017. I married my wife, whom I met in college. I have severe Hemophilia A and am currently part of a hemophilia gene therapy research program."

Come to the Orlando Creepy Crawl on October 29, 2022, and you
can meet Samantha and Ty yourself.
The walk is at Harbor Park in Orlando.
Registration starts at 4 P.M.

16th Annual Orlando
Creepy Crawl
FUN WALK or 5K

Benefiting the
**Hemophilia Foundation
of Greater Florida**

October 29, 2022

Harbor Park at Baldwin Park
4990 New Broad Street
Orlando, Florida
Registration starts at 4pm

Food & Fun, Music & Prizes
Special Guests
Costume Contest
for All Ages (pets included)
Trick or Treating
Raise \$25 or more and receive a
event t-shirt!

More Info: info@hemophiliaflorida.org or 800.293.6527
Register at hemophiliaflorida.org/walks-5ks/

