

HEMOPHILIA ACTION

A PUBLICATION OF THE HEMOPHILIA FOUNDATION OF SOUTHERN CALIFORNIA
VOLUME 15 | ISSUE 1 | WINTER | 2019

10TH ANNUAL UNITE FOR BLEEDING DISORDERS WALK: GRIFFITH PARK, LOS ANGELES

Thank you to everyone who donated, walked, and promoted awareness for HFSC's 10th Annual Unite for Bleeding Disorders Walk in Griffith Park. Participants enjoyed a Circus theme with carnival games, face painting, popcorn, fun costumes and a magic show performed by the talented Robert Ramirez. A record number of walkers joined us this year at over 800!

More significantly, we raised over \$123,000 appreciably above our goal of \$100,000! With this result, HFSC is thrilled to announce that our 120 campers including Leaders in Training, are fully funded to attend Camp Blood Brothers and Sisters in July 2019. The HFSC Board of Directors has chosen to allocate the any remaining funds to support advocacy efforts in maintaining health access, medical research and studies to identify additional needs within our community.



Congratulations to Best T-Shirt Award: **No Fear Factor**



Thank you to the Redmond Family for being fun clowns and face painting, to Scott Carthey for facilitating the Pinwheel Ceremony, and to all the volunteers who helped make this great event possible. Thank you to Scout Burke for singing the National Anthem, Board Member Wayne Guzman for being our MC, Benjamin Cardenas for sharing "Why He Walks," Brooke Walsh for leading our warm up before walking, and to Board Members Shin Chen, Silvia Garcia, Rick Kelly, and Michele Warner for presenting the Factor Club awards.

Congratulations to our Top 10 Teams: We Clot This, No Fear Factor, We Are Grifols, Brothers Unite, Ninja Stars, Thousand Oaks Globe Clotters, Team Joshua Kim, It Runs in the Blood, Cole Train, and Amelie's GT Striders.

Congratulations to our Top 10 Individuals: Rose Cardenas, Michele Warner, Dr. Richard Metz, Michelle Dotson, Dr. Doris Quon, Michelle Kim, Veronica Diaz, Karlyn Johnson Brown, Misty and Paige Antonio, and Josie Kelly.

Congratulations to our Largest Team: We Are Grifols, who had 170 team members!

Finally, last December, the National Hemophilia Foundation honored HFSC with the Walk Chapter of the Year Award. HFSC thanks everyone who participated in the Walk for all of their hard work and dedication!

Thank you to our Local Sponsors! Gold Sponsors: AmeriPharma, CSL Behring, Grifols. Silver Sponsors: Bayer, Bioverativ, Novo Nordisk, and the Wingmen Foundation.

Booths provided by sponsors included National Presenting Sponsor Shire, Official Sponsors Bayer, CVS Specialty Pharmacy, Grifols, Hema Biologics, Octapharma, Pfizer, and Sanofi Genzyme, as well as Partner Sponsor Accredio.

ARTICLE CONTINUED ON PAGE 1

2019 CALENDAR OF EVENTS

- April 4-7 **HFA Symposium**
San Diego, CA
- April 17 **World Hemophilia Day**
Doubletree by Hilton
Downtown Los Angeles, CA
- April 28 **Blood Brotherhood & Dads in Action One-Day Retreat**
Irvine Ranch Outdoor Education Center | Orange, CA
- May 10 **Family Information Day Kickoff Dinner – Women’s Educational and Small Group Forum**
Knott’s Berry Farm Hotel
Buena Park, CA
- May 11 **Family Information Day**
Knott’s Berry Farm Hotel
Buena Park, CA
- May 13-15 **Future Leaders Program and Legislative Day (HCC)**
Sacramento, CA
- May 22 **Mujeres Enlazadas Dinner**
Los Angeles, CA
- June 7-9 **Retreat – “ROAR”**
Pali Retreat Center
Running Springs, CA
- June 28 **HFSC Scholarships Due (Christopher Mark Pitkin Scholarship & Dr. Earl James Fahringer Arts Scholarship)**
- June 29 **Screening of “Bombardier Blood”**
Laemmle 7 Playhouse
Pasadena, CA
- July 13 **Pain Management and Alternative Therapies Conference**
Pasadena, CA
- July 23-28 **Camp Blood Brothers & Sisters**
The Painted Turtle
Lake Hughes, CA
- September 6-8 **Familia de Sangre Conference**
Anaheim, CA
- October 3-5 **NHF Bleeding Disorders Conference**
Anaheim, CA

THANK YOU, DONORS

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HEMOPHILIA ACTION

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Congratulations to the 2018 Factor Club Winners: Jesus Avila, Paul Avila, Paige Antonio, Alondra Arias, Langston Brown, Karlyn Johnson Brown, Judy Ann Burgos, Amy Burke, Ana Cardenas, Jorge Cardenas, Rose Cardenas, Benjamin Cardenas, Rebecca Carmona, Jacqueline Ceja, Shin Chen, Sandra Davis, Veronica Diaz, Michelle Dotson, Julia Grove, Elena Iavicoli, Amelie Iavicoli, Joseph Jordan, Michelle Kim, Keely King, Corinne Love, Dr. Richard Metz, Vicky Michua, Christina Olivarria, Judy Redmond, Cristina Reyes, Marcel Sassola, Dr. Doris Quon, Michele Warner, Willie Zuniga.



A LETTER FROM THE BOARD PRESIDENT

As my term as Board President comes to an end, I'd like to thank the HFSC staff for an outstanding year. We held many events that supported our bleeding disorders community. Each of these events takes numerous hours of planning and execution. This happens while the staff is also participating in national events and supporting the community. I am so proud of this amazing team of dedicated individuals. They are not only hard working, but intelligent, diplomatic and dynamic. In the coming year we have many exciting events planned: Back to School Symposium; Holiday Parties; Unite Walk; Familia Dr Sangre; Industry Forum; emPOWERment Forum; Women's Retreat; Men's Retreat; Family Info Day; Family Retreat; Pain Management: Alternative Therapies Conference; and Camp Blood Brothers & Sisters. When I think about the roughly 2,000 (incredibly diverse) members and 9 counties that this small team supports, I am truly in awe.

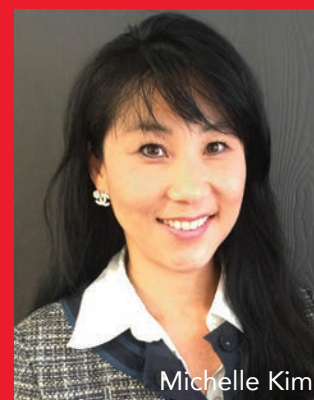
I have no doubt that our newest Executive Board which consists of Sandra Knight, President; Ron Staake, Treasurer; Rick Kelly, Secretary are fabulous passionate Board members that will lead the board to even greater success.

Thank you for the opportunity to serve you,

Sandy Davis, HFSC Board President

A LETTER FROM THE EXECUTIVE DIRECTOR

I have never been prouder of a group of people than I am to all of you who raised awareness and critical funding for HFSC at our 10th Annual UNITE Walk! As I accepted the "Walk Chapter of the Year" award from the National Hemophilia Foundation's National Walk Meeting, I couldn't help but reflect that this award is proudly shared among all of us! Our 2019 Camp Blood Brothers and Sisters is now fully funded and I encourage all of you to please apply as soon as possible. The application is a beast, so if you have any questions, please contact us. This is also the time for those that are in college, graduate, or trade school to think about applying for scholarships. Please reach out to us if you would like a list of the available scholarships or would like an HFSC scholarship application. I have written more than 50 letters of recommendation for our HFSC youth for both college and scholarships, so I hope your teens will continue to foster a relationship with us so I can continue telling the world how great they are! Finally, we are proud of all the ways that we can help support the community with our many hosted events. These events can be very costly. Therefore, if you cannot attend an event, please call or email us! I am intimately aware that things happen last minute, but if we don't know you've canceled, we (or a sponsor) still pays for you and your family. Last year alone we lost tens of thousands of dollars due to no-shows. Again, we understand that sometimes things come up at the last minute but please let us know. Thank you again for the privilege of serving this amazing community!



Michelle Kim

NOW APPROVED

FOR PEOPLE WITH HEMOPHILIA A WITH OR WITHOUT FACTOR VIII INHIBITORS

GO SEEK. GO EXPLORE. GO AHEAD.



Discover your sense of go. Discover HEMLIBRA®.

HEMLIBRA.com

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII, and the dose and schedule to use for breakthrough bleed treatment. HEMLIBRA may cause serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including thrombotic microangiopathy (TMA), and blood clots (thrombotic events). 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.

Please see Brief Summary of Medication Guide on following page for Important Safety Information, including **Serious Side Effects**.


HEMLIBRA
emicizumab-kxwh | 150
injection for subcutaneous use | mg/mL

Medication Guide
HEMLIBRA® (hem-lee-bruh)
(emicizumab-kxwh)
injection, for subcutaneous use

What is the most important information I should know about HEMLIBRA?

HEMLIBRA increases the potential for your blood to clot. Carefully follow your healthcare provider's instructions regarding when to use an on-demand bypassing agent or factor VIII (FVIII) and the recommended dose and schedule to use for breakthrough bleed treatment.

HEMLIBRA may cause the following serious side effects when used with activated prothrombin complex concentrate (aPCC; FEIBA®), including:

- **Thrombotic microangiopathy (TMA).** This is a condition involving blood clots 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
 - weakness
 - swelling of arms and legs
 - yellowing of skin and eyes
 - stomach (abdomen) or back pain
 - nausea or vomiting
 - 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 these signs or symptoms of blood clots 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
 - cough up blood
 - feel faint
 - headache
 - numbness in your face
 - eye pain or swelling
 - 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.

See “What are the possible side effects of HEMLIBRA?” for more information about side effects.

What is HEMLIBRA?

HEMLIBRA is a prescription medicine used for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A with or without factor VIII inhibitors.

Hemophilia A is a bleeding condition people can be born with where a missing or faulty blood clotting factor (factor VIII) prevents blood from clotting normally.

HEMLIBRA is a therapeutic antibody that bridges clotting factors to help your blood clot.

Before using HEMLIBRA, tell your healthcare provider about all of your medical conditions, including if you:

- are pregnant or plan to become pregnant. It is not known if HEMLIBRA may harm your unborn baby. Females who are able to become pregnant should use birth control (contraception) during treatment with HEMLIBRA.
- are breastfeeding or plan to breastfeed. It is not known if HEMLIBRA passes into your breast milk.

Tell your healthcare provider about all the medicines you take, including prescription medicines, over-the-counter medicines, vitamins, or herbal supplements. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I use 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.

- Use HEMLIBRA exactly as prescribed by your healthcare provider.
- **Stop (discontinue) prophylactic use of bypassing agents the day before starting HEMLIBRA prophylaxis.**
- **You may continue prophylactic use of FVIII for the first week of HEMLIBRA prophylaxis.**
- HEMLIBRA is given as an injection under your skin (subcutaneous injection) by you or a caregiver.
- Your healthcare provider should show you or your caregiver how to prepare, measure, and inject your dose of HEMLIBRA before you inject yourself for the first time.

- Do not attempt to inject yourself or another person unless you have been taught how to do so by a healthcare provider.
- Your healthcare provider will prescribe your dose based on your weight. If your weight changes, tell your healthcare provider.
- You will receive HEMLIBRA 1 time a week for the first four weeks. Then you will receive a maintenance dose as prescribed by your healthcare provider.
- If you miss a dose of HEMLIBRA on your scheduled day, you should give the dose as soon as you remember. You must give the missed dose as soon as possible before the next scheduled dose, and then continue with your normal dosing schedule. **Do not** give two doses on the same day to make up for a missed dose.
- HEMLIBRA may interfere with laboratory tests that measure how well your blood is clotting and may cause a false reading. Talk to your healthcare provider about how this may affect your care.

What are the possible side effects of HEMLIBRA?

- See “What is the most important information I should know about HEMLIBRA?”

The most common side effects of HEMLIBRA include:

- redness, tenderness, warmth, or itching at the site of injection
- headache
- joint pain

These are not all of the possible side effects of HEMLIBRA.

Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store HEMLIBRA?

- Store HEMLIBRA in the refrigerator at 36°F to 46°F (2°C to 8°C). Do not freeze.
- Store HEMLIBRA in the original carton to protect the vials from light.
- Do not shake HEMLIBRA.
- If needed, unopened vials of HEMLIBRA can be stored out of the refrigerator and then returned to the refrigerator. HEMLIBRA should not be stored out of the refrigerator for more than a total of 7 days or at a temperature greater than 86°F (30°C).
- After HEMLIBRA is transferred from the vial to the syringe, HEMLIBRA should be used right away.
- Throw away (dispose of) any unused HEMLIBRA 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 HEMLIBRA 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 HEMLIBRA that is written for health professionals.

What are the ingredients in HEMLIBRA?

Active ingredient: emicizumab-kxwh

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

Manufactured by: Genentech, Inc., A Member of the Roche Group,
1 DNA Way, South San Francisco, CA 94080-4990
U.S. License No. 1048

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For more information, go to www.HEMLIBRA.com or call 1-866-HEMLIBRA.

This Medication Guide has been approved by the U.S. Food and Drug Administration
Revised : 10/2018



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MEMBER SPOTLIGHT!



HEMOPHILIA: THE MUSICAL

The **Breaking Through! Musical Theater Intensive** was a three-day musical theater and arts workshop for the bleeding disorders community, produced by **Believe Limited** and sponsored exclusively by **BioMarin**. Teen participants from around the country flew to New York City to learn and perform a 6-songs in, "Hemophilia: The Musical," about the psychosocial and general health aspects of being a young person with a bleeding disorder. Three Southern California teens were chosen to participate, and we are so proud of them!

Destini Danaye: "Getting the opportunity to go to New York and live out my Broadway dream and getting to represent the bleeding disorders community at the same time was truly the best and most humbling experience of my life. I have wanted to go to New York since I was 5! I got to meet so many people and make so many friends who also have bleeding disorders. This experience will forever hold a huge place in my heart!"

Emma Gould: "My experience in New York was amazing! The whole weekend was made about us kids, who all had a wonderful time connecting, singing, dancing, and just being ourselves while advocating for the community, throughout the country. This experience was truly amazing!"

Nathan Mermilliod: "The Breaking Through experience in New York was so much more than I even thought it would be - We worked hard, grew super close, and I loved every minute of it. The musical was really powerful; we even got a standing ovation right in the middle of the show. When I was on the stage performing "Hemophilia: the Musical," I realized that I wasn't alone in dealing with my disorder, and I'm so grateful for blessings like this to balance out the burdens."

TWO HFSC SCHOLARSHIPS NOW AVAILABLE

Up to \$1,000.00 each
Applications Due June 28, 2019



Dr. Earl James Fahringer Arts Scholarship

Awarded each year to 1-2 students pursuing a major in music, arts, drama, film, or dance.

Christopher Mark Pitkin Memorial Scholarship - 21st Annual Awards

Awarded to students seeking higher education who can show motivation and goal orientation.

Qualifications:

- Must be a person with a bleeding disorder OR must have an immediate family member with a bleeding disorder; however, priority will be given to those with bleeding disorders;
- Must be accepted into an educational/vocational institution and able to provide proof of enrollment;
- Must be a resident of, or have permanent address in, one of the following Southern California counties: Los Angeles, Orange, Riverside, San Bernardino, San Luis Obispo, Ventura, Kern, Inyo and Santa Barbara;
- Priority given to first time applicants;
- Priority given to applicants who are actively involved with HFSC.

Required Materials:

- Completed Application;
- 2 letters of recommendation (teacher, guidance counselor, employer, etc.);
- Official transcript;
- Evidence of enrollment at educational/vocational institution.

Please email laura@hemosocal.org or call 626-765-6656 for more information and an application.

VOLUNTEER SPOTLIGHT: IVAN AREVALO

Ivan Arevalo is a Los Angeles native. He was introduced to the Bleeding Disorders community two years ago through the Casas family, and has been volunteering ever since! This year he attended the North American Camp Conference for Hemophilia Organizations (NACCHO) 2019 to represent HFSC's Family Retreat. He learned several exciting and innovative activities for the kids, as well as how to monitor and be mindful of the unique needs of kids with bleeding disorders. "I look forward to working with everyone and spreading joy to the whole Hemophilia family in any way I can." HFSC is so thankful for Ivan's help, so please say "Hello" to him at our next event!



Thank you National Charity League for your help setting up!



Thank you volunteers!



Congratulations First Place
Tournament Winners!



Ron Staake, Board Member,
MC's the Golf Dinner



35TH ANNUAL GOLF TOURNAMENT LA CANADA, CA

On October 8, 2019, HFSC hosted its 35th Annual Golf Tournament at the La Canada Flintridge Country Club to raise funds for summer camp. It was a gorgeous day and the golfers enjoyed game holes, breakfast, and lunch. After the players finished the tournament, they enjoyed a cocktail reception with an extravagant Silent Auction. This year, HFSC honored Ms. Terry Hays-Horner. Ms. Hays-Horner has deep roots with HFSC within her family. Her father and mother, who had 2 sons with Hemophilia, were instrumental in founding HFSC with other local families in the late 1950's and early 1960's. Her father and brother were both on the HFSC Board for many years. Verne Hays also started the Golf Tournament Fundraiser. We appreciated hearing from her about challenges back then and her reflections about all the changes. Ron Staake, community member and Board Member emceed a heartfelt dinner and welcomed our camp appeal speakers: brothers Joseph Muñoz, Julian and Justin Guzman, Elizabeth Villanueva and Mia Castaneda, HFSC Teen Leader who did a fabulous job highlighting the impact and importance of camp. Ron then proceeded with a successful Camp Pledge appeal with many extraordinarily generous donors. HFSC is grateful to over 50 volunteers, 76 golfers, and dinner attendees who joined us to make this day a success and grossing more than \$51,000!



Congratulations Second
Place Tournament Winners!



Congratulations Third Place
Tournament Winners!

ROAR!



Set your course to the

HFSC Retreat

June 7-9, 2019

Pali Retreat Center

Register Here:

<https://hfscretreat2019.eventbrite.com>

Thank You To:

Platinum Sponsor Takeda

Hemophilia Federation of America

Orthopaedic Treatment Center

*New Young Adult (Singles ages 18-35) Program!



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antihemophilic factor
(recombinant) PEGylated-aucI

LET'S GO

SANTA BARBARA SNOWFLAKE FESTIVAL



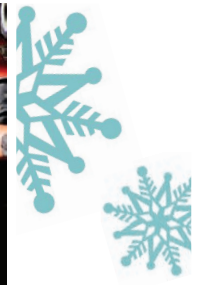
On a beautiful sunny morning on December 1, 2018, HFSC hosted the Santa Barbara Snowflake Festival at the Santa Barbara Zoo. Our participants were welcomed by Susan and Joe Cole (who have hosted many of our Santa Barbara gatherings) and Past President of the Board, Michael Franzen.

Thanks to our exclusive sponsor, Shire, we were able to welcome 125 people to create festive crafts, meet a flock of Flamingos in the "Fla-Mingle" animal encounter, and learn about IV infusion on the practice arm. Before lunch, Cesme Caballero presented in English, and Susana Escojido and Amy Vega presented in Spanish about Resilience. Participants worked together to build structures with marshmallows, dry spaghetti, and other materials to see who could create the tallest structure. After the presentation, lunch was served. Teen Leader Carson Knight spoke to the group about the Teen Leadership Summit he attended in June, and then the group played a Trivia Game. Santa arrived, and the children took pictures and received gifts. After that, the families enjoyed a beautiful day at the Zoo!

Thank you to our Sponsor, Shire!



Thank you to our amazing volunteers: Photographer, Aileen O'Brien; Barb Pileri; Susan and Joe Cole; Michael, Melissa, Allison, and Charlie Franzen; Judy Mangione; Sandra and Carson Knight; Arely and Yatzared Michua; Randy DeSantis; Sochi Frank; and special thanks to Board Member Rick Kelly for your special role.

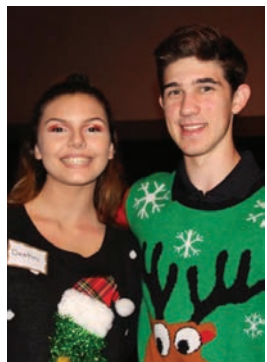


FESTIVAL OF LIGHTS AT THE MISSION INN | RIVERSIDE, CA

On December 18, 2018, 180 members met at National Historic Landmark, The Mission Inn Hotel and Spa, in Riverside, CA. The historic hotel has an annual Festival of Lights celebration where they showcase one of the largest holiday light displays in the country. Participants gathered in the Grand Parisian Ballroom for a dinner, courtesy of Platinum Sponsor, Bioverativ, a Sanofi Company. Edgar Vega presented on "Treating Hemophilia," and after dinner and the presentation, participants were treated to amazing dessert from Casey's Cupcakes. Christmas carols were sung by teens Nathan Mermilliod and Destini Danaye, followed by surprise guest, Olaf, who stopped by to say "Happy Holidays" to the kids. After the program, many families walked around to see the beautiful light display.

Thank you to our generous sponsor, Bioverativ, a Sanofi Company!

Thank you to our volunteers: Mathew Casas; Paul, Linda, and Erika Clement; Yatzared and Arely Michua; Cherie, James, and Jarett Guillow; Fiach Echandi; Judy, Brook, Loren, and Brook Redmond



Understanding New Discoveries in Genomic Medicine

While the exact cause for many genetic diseases remains unknown, for some patients, the problem can be traced to errors in DNA, or changes in a person's genes that can result in rare and severe disease. Emerging research in genomic medicine is working to address the underlying mistakes in DNA that lead to some genetic diseases.

The following answers five frequently asked questions about the emerging field of genomic medicine including gene therapy, genome editing and cell therapy, and what these technologies could mean for the rare disease community.



What is Gene Therapy?

Gene therapy is the treatment of disease by delivering a new gene into a patient's cells to replace an incorrect or damaged gene. Most often, gene therapy works by introducing a corrected copy of a defective gene into the patient's cells, without removing or modifying the defective gene. The goal of gene therapy is to treat or cure a genetic disease by adding back a normal copy of the gene that was responsible for the disease.

Although the concept sounds futuristic, gene therapy has been studied by researchers for more than 40 years. Along the way, much attention has been focused on ensuring this complex technology is implemented safely and effectively.



What is Genome Editing?

Genome editing (also called gene editing) makes permanent changes to the genetic code of a cell, by correcting, disabling, removing, or modifying the DNA.

Genome editing works by using enzymes called "engineered nucleases" to make precise cuts at specific DNA sequences. With these engineered nucleases the DNA of a cell can be changed permanently.

There are several types of engineered nucleases used in genome editing. These include:

- Zinc Finger Nucleases (ZFN)
- Transcription Activator-like Effector Nucleases (TALEN)
- Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR)

Each genome editing tool has slight differences, but all engineered nucleases are designed to do the same thing: act like molecular scissors to cut the DNA at a precise spot in the genome to remove, add, or replace the DNA. After cutting the DNA, the cell will naturally repair the break and researchers use these natural repair processes to introduce specific changes at the site where the DNA is cut.





What is Cell Therapy?

Cell therapy is the infusion or transplantation of human cells into a patient for the treatment of a disease. The origin of the cells can be from the patient or from another healthy donor. This technology uses the patient's own cells or cells from a healthy donor as a therapy to treat the patient for diseases caused by mistakes in DNA, infections or cancer.

Whole blood transfusions, packed red blood cell transfusions, platelet transfusions, bone marrow transplants, and CAR T-cells are all forms of cell therapy.



Can Genome Editing and Cell Therapy Be Combined?

Genome editing and cell therapy both have the potential to ease the underlying cause of genetic diseases and can be combined for certain treatments. Cells are collected from the patient or another healthy donor, modified in a laboratory using genome editing and re-administered to the patient.

Numerous approaches are currently under investigation, with a promising therapy involving a type of cell therapy called CAR T-cell therapy. With this treatment, T-cells, a type of human immune cell, are collected from blood, modified with engineered nucleases in the laboratory to make them better at attacking cancer cells and returned to the patient.

What Does This Mean for the Rare Disease Community?

Genomic medicine is making a positive impact in the fields of oncology, rare, and infectious disease. While early research and emerging clinical trials are encouraging and offer hope to patients and their families, many more studies are needed to ensure these therapies are safe and effective for adults and, someday, children.

Hundreds of clinical trials are being conducted to test gene therapy, genome editing and cell therapy as potential treatments for some genetic conditions, certain cancers and HIV/AIDS. This is an exciting new frontier in medicine and you can visit ClinicalTrials.gov to search for clinical trials that are accepting patients. Your physician or a genetics professional can help you understand if a clinical trial may be right for you.

Register Now!

Family Information Day Kickoff Dinner

Friday, May 10, 2019

Knott's Berry Farm Hotel

(Hotel rooms are not provided)

Family Information Day

Saturday, May 11, 2019

Knott's Berry Farm Hotel, Buena Park, CA

Educational Speakers,

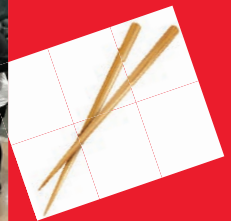
Youth and Teen Programs,

Breakout Sessions, and Sponsor Booths

REGISTER: <https://familyinfoday2019.eventbrite.com>



NEW FAMILIES DINNER



On November 14, 2018, HFSC welcomed new and old families to Goo Goo Ryoo, an all you can eat Korean Barbecue Restaurant in the city of Garden Grove. Some of HFSC's Teen Leaders opened the program by testing those in attendance with advocacy trivia; those who answered correctly won gift cards. The event was followed by an educational presentation by Bayer on Hemophilia 101. All families enjoyed the presentation while cooking and eating their favorite meats. HFSC welcomes all the new and old families who took time from their busy schedules to attend this dinner. Also, thank you to the Bayer Team: Matt, Silvia, and Karen for your support!

DO YOU OR SOMEONE YOU LOVE HAVE HEMOPHILIA A WITH AN INHIBITOR TO FACTOR VIII?

YOU MIGHT BE INTERESTED IN OUR CLINICAL RESEARCH STUDY.

Factor VIII inhibitors are antibodies that people with hemophilia A can develop in response to their Factor VIII treatment. They interfere with Factor VIII and prevent it from working the way it should. The development of FVIII inhibitors is one of the most serious complications for people with hemophilia A.

The INITIATE clinical study is examining the potential advantages of utilizing a patient-specific laboratory test called batch-selection to decrease the time to success in Immune Tolerance Induction (ITI) for hemophilia A patients with inhibitors to FVIII.

SPEAK TO A STUDY REPRESENTATIVE

For more information, or if you would like to be considered for participation in the study, please contact us at **916-734-3880**. We will be available to discuss any concerns or answer any questions.

Or go to www.initiateusa.com to learn more.



SNOWFLAKE FESTIVAL | SANTA ANA, CA

On December 8, 2018, over 500 HFSC members, volunteers and sponsors celebrated the close of 2018 at the Santa Ana Zoo with crafts, games, sponsor booths. HFSC's Teen Leadership Council presented on their experience and teen opportunities as well. With the Habit Truck serving delicious burgers, participants took their picture with Santa, danced with Olaf, and enjoyed several exciting animal presentations offered by the Zoo. Gifts and gift cards were distributed to all the kids and teens, and then families were free to explore the exciting Santa Ana Zoo!

Thank you to our Booth Sponsors: Bayer, CSL Behring, Genentech, Grifols, Octapharma, and Shire

Thank you to our Toy Sponsors: Motor4Toys Charitable Foundation, the Pasadena Police Department, and Good 360.

Thank you to Santa's Elves: Accredo, Advantage Infusion Care, Brothers Healthcare, Diplomat, Donna Gallegos, Factor Support Network, Guided Alliance, Santa Barbara Specialty Pharmacy Specialty Care, Quality Infusion and Soleo Health. Thank you to Brook Redmond as Olaf and Girlscout Troop # 3731 for creating beautiful factor boxes for their Silver Award.

Thank you to all our volunteers and special guests who helped wrap gifts and assisted the day of the event! We could not host this event without you!



The National Hemophilia Foundation's 70th Bleeding Disorders Conference (BDC) took place from October 11-13, 2018 in Orlando, CA. Exciting educational information was presented to families who traveled from all across the nation including HFSC members who received scholarships to attend. One highlight was a speech by HFSC Executive Director, Michelle Kim at the Opening Session, who shared her future hopes for the bleeding disorders community. These were included in a "time capsule" to be opened by NHF in 50 years!

REGISTRATION is NOW available for NHF's 2019 BDC conference to take place in ANAHEIM from October 3-5, 2019. If you would like to apply for a hotel scholarship for three nights, please email cynthia@hemosocal.org (this does not include parking or registration to the conference) Don't miss this fantastic opportunity! Register directly for the conference here: <https://events.hemophilia.org/ehome/index.php?eventid=385113&>



UNLOCKING YOUR SELF-POTENTIAL



ONLY ADVATE® HAS 15 YEARS OF EXPERIENCE IN THE REAL WORLD AS A RECOMBINANT FACTOR VIII[†]

- Proven in a pivotal clinical trial to reduce the number of bleeding episodes in children and adults when used prophylactically^{2*}
- Third-generation full-length molecule, similar to the factor VIII that occurs naturally in the body^{1,2}

*Multicenter, open-label, prospective, randomized, 2-arm controlled trial of 53 previously treated patients with severe to moderately severe hemophilia A. Two different ADVATE prophylaxis regimens (standard, 20-40 IU/kg every 48 hours, or pharmacokinetic-driven, 20-80 IU/kg every 72 hours) were compared with on-demand treatment. Patients underwent 6 months of on-demand treatment before 12 months of prophylaxis.²

The market leader in hemophilia A treatment[†]

[†]Based on 2016 data published July 2017.³

The myPKFIT™ for Patients Mobile Application offers visibility into your personalized ADVATE treatment. Talk to your doctor to see if myPKFIT for ADVATE may be right for you. Learn more at ADVATE.com.

ADVATE Important Information

What is ADVATE?

- ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called “classic” hemophilia).
- ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A. Your healthcare provider (HCP) may give you ADVATE when you have surgery.
- ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

DETAILED IMPORTANT RISK INFORMATION

Who should not use ADVATE?

Do not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your HCP if you are pregnant or breastfeeding because ADVATE may not be right for you.

What should I tell my HCP before using ADVATE?

Tell your HCP if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.
- Are or become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What important information do I need to know about ADVATE?

- You can have an allergic reaction to ADVATE. Call your HCP right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.
- Do not attempt to infuse yourself with ADVATE unless you have been taught by your HCP or hemophilia center.

What else should I know about ADVATE and Hemophilia A?

- Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Talk with your HCP to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

What are possible side effects of ADVATE?

- Side effects that have been reported with ADVATE include: cough, headache, joint swelling/aching, sore throat, fever, itching, unusual taste, dizziness, hematoma, abdominal pain, hot flashes, swelling of legs, diarrhea, chills, runny nose/congestion, nausea/vomiting, sweating, and rash. Tell your HCP about any side effects that bother you or do not go away or if your bleeding does not stop after taking ADVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA.

Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

For additional safety information, please see Important Facts about ADVATE on the following page and discuss with your HCP. For Full Prescribing Information, visit www.ADVATE.com

myPKFIT for ADVATE Patients Mobile Application Intended Use

- The myPKFIT for Patients Mobile Application (“myPKFIT Mobile App”) is intended for use by patients with hemophilia A being treated with ADVATE [Antihemophilic Factor (Recombinant)] who are 16 years of age or older with a body weight of 45 kg or higher, and their caregivers.
- The myPKFIT Mobile App is designed to make it convenient for you to record your infusion and bleed events, track your estimated Factor VIII levels following a prophylactic infusion, and export the data for review by your health care provider (“HCP”).
- Your HCP can use the myPKFIT software to generate ADVATE dosage amount and frequency recommendations for routine prophylaxis using your age, body weight information, and laboratory tests that measure your Factor VIII clotting activity. Using myPKFIT software, HCPs can evaluate various prophylaxis dose regimens tailored to your individual needs and treatment plan.
- myPKFIT Mobile App should only be used by hemophilia A patients treated with ADVATE, as per the ADVATE Prescribing Information.
- myPKFIT Mobile App is not indicated for use by patients with von Willebrand disease. myPKFIT Mobile App should not be used by patients who have developed inhibitors to Factor VIII products.

myPKFIT for Patients Mobile Application is Rx only. For safe and proper use of the myPKFIT Mobile App, please refer to the complete instructions for use in the User Manual.

References: 1. Grillberger L, Kreil TR, Nasr S, Reiter M. Emerging trends in plasma-free manufacturing of recombinant protein therapeutics expressed in mammalian cells. *Biotechnol J*. 2009;4(2):186-201. 2. ADVATE Prescribing Information. 3. The Marketing Research Bureau, Inc. The plasma proteins market in the United States. 2016. 4. myPKFIT Mobile Application v2.0 User Manual. 2018.

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[Antihemophilic Factor (Recombinant)]

Important facts about

ADVATE [Antihemophilic Factor (Recombinant)]

This leaflet summarizes important information about ADVATE. Please read it carefully before using this medicine. This information does not take the place of talking with your healthcare provider, and it does not include all of the important information about ADVATE. If you have any questions after reading this, ask your healthcare provider.

What is the most important information I need to know about ADVATE?

Do not attempt to do an infusion to yourself unless you have been taught how by your healthcare provider or hemophilia center.

You must carefully follow your healthcare provider's instructions regarding the dose and schedule for infusing ADVATE so that your treatment will work best for you.

What is ADVATE?

ADVATE is a medicine used to replace clotting factor (factor VIII or antihemophilic factor) that is missing in people with hemophilia A (also called "classic" hemophilia). The product does not contain plasma or albumin. Hemophilia A is an inherited bleeding disorder that prevents blood from clotting normally.

ADVATE is used to prevent and control bleeding in adults and children (0-16 years) with hemophilia A.

Your healthcare provider may give you ADVATE when you have surgery. ADVATE can reduce the number of bleeding episodes in adults and children (0-16 years) when used regularly (prophylaxis).

ADVATE is not used to treat von Willebrand disease.

Who should not use ADVATE?

You should not use ADVATE if you:

- Are allergic to mice or hamsters.
- Are allergic to any ingredients in ADVATE.

Tell your healthcare provider if you are pregnant or breastfeeding because ADVATE may not be right for you.

How should I use ADVATE?

ADVATE is given directly into the bloodstream.

You may infuse ADVATE at a hemophilia treatment center, at your healthcare provider's office or in your home. You should be trained on how to do infusions by your healthcare provider or hemophilia treatment center. Many people with hemophilia A learn to infuse their ADVATE by themselves or with the help of a family member.

Your healthcare provider will tell you how much ADVATE to use based on your weight, the severity of your hemophilia A, and where you are bleeding.

You may have to have blood tests done after getting ADVATE to be sure that your blood level of factor VIII is high enough to clot your blood.

Call your healthcare provider right away if your bleeding does not stop after taking ADVATE.

What should I tell my healthcare provider before I use ADVATE?

You should tell your healthcare provider if you:

- Have or have had any medical problems.
- Take any medicines, including prescription and non-prescription medicines, such as over-the-counter medicines, supplements or herbal remedies.
- Have any allergies, including allergies to mice or hamsters.
- Are breastfeeding. It is not known if ADVATE passes into your milk and if it can harm your baby.
- Are pregnant or planning to become pregnant. It is not known if ADVATE may harm your unborn baby.
- Have been told that you have inhibitors to factor VIII (because ADVATE may not work for you).

What are the possible side effects of ADVATE?

You can have an allergic reaction to ADVATE.

Call your healthcare provider right away and stop treatment if you get a rash or hives, itching, tightness of the throat, chest pain or tightness, difficulty breathing, lightheadedness, dizziness, nausea or fainting.

Side effects that have been reported with ADVATE include:

cough	headache	joint swelling/aching
sore throat	fever	itching
unusual taste	dizziness	hematoma
abdominal pain	hot flashes	swelling of legs
diarrhea	chills	runny nose/congestion
nausea/vomiting	sweating	rash

Tell your healthcare provider about any side effects that bother you or do not go away.

These are not all the possible side effects with ADVATE. You can ask your healthcare provider for information that is written for healthcare professionals.

What else should I know about ADVATE and Hemophilia A?

Your body may form inhibitors to factor VIII. An inhibitor is part of the body's normal defense system. If you form inhibitors, it may stop ADVATE from working properly. Consult with your healthcare provider to make sure you are carefully monitored with blood tests for the development of inhibitors to factor VIII.

Medicines are sometimes prescribed for purposes other than those listed here. Do not use ADVATE for a condition for which it is not prescribed. Do not share ADVATE with other people, even if they have the same symptoms that you have.

The risk information provided here is not comprehensive. To learn more, talk with your health care provider or pharmacist about ADVATE. The FDA approved product labeling can be found at www.ADVATE.com or 1-888-4-ADVATE.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

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If you would like a copy of this newsletter in Spanish, please contact the HFSC office at 626-765-6656 or info@hemosocal.org, and we would be happy to send one to you!

¡Si desea una copia de este boletín de noticias en español, por favor póngase en contacto con la oficina de HFSC al 626-765-6656 o info@hemosocal.org!



The Painted Turtle
Held at
July 23-28, 2019

CAMP BLOOD BROTHERS
AND SISTERS

Apply Now!

Call us with questions!
<http://thepaintedturtle.org/campers-families/families/application/>

Saturday, June 29, 2019
Laemmle 7 Playhouse
Pasadena, CA
Registration: 9:30AM
Film Screening:
10:30AM-12:00PM
Panel Q&A with Director,
Producer, and Cast:
12:00PM-12:30PM
This event made possible by generous support from Octapharma.
REGISTER AT:
<https://hfsqbombardierblood.eventbrite.com>



HFSC SCREENING OF
BOMBARDIER BLOOD