

# The Clotting Connection

*A Newsletter for Families with Bleeding and Clotting Disorders*

January 2019  
Volume 9, Issue 1

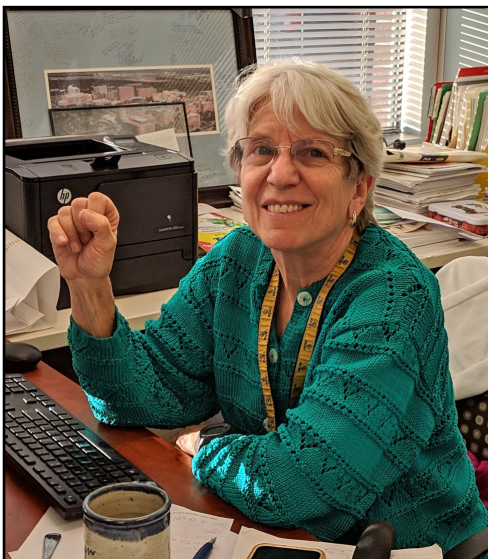


University of Colorado  
Anschutz Medical Campus  
Hemophilia and Thrombosis Center

Affiliated with  
Children's Hospital Colorado  
and University of Colorado Hospital

## DIRECTOR'S CORNER

January marks a New Year, new beginnings, new energies for growth while awaiting the bloom of spring. This winter the HTC staff are investing major energy in clinical research. Research is the process whereby ideas are translated into therapies that are subjected to scrutiny to prove their ability to advance health and improve lives. There is research that is easy to understand, such as testing a new product that will make hemophilia or thrombosis easier to manage. And there is research that is more global, such as a project to understand why healthy teenagers get large blood clots in their thighs and pelvis which can compromise their well-being for years to come. All of our faculty are engaged in research to improve the lives of persons with bleeding or clotting. I would like to use this newsletter to highlight some research projects that we are particularly excited about. Because of the many investigators at our HTC, I will only discuss the work of half of them, and continue the story with our next newsletter.



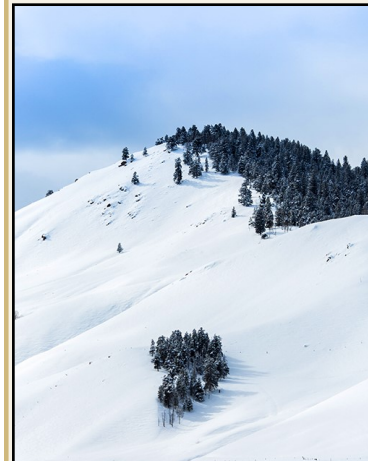
Our HTC Director, Dr. Marilyn Manco-Johnson.



Dr. Mike Wang oversees the gene therapy and novel factor studies at the HTC.

Gene therapy for hemophilia: Gene therapy is here and it appears to be ready to fulfill its promise as a credible cure, at least for some people with hemophilia. We are partnering with industry and the national HTC network to participate in gene therapy trials for hemophilia A and hemophilia B. Gene therapy is currently limited by the FDA to individuals 18 years and older. The primary reason some people are excluded from gene therapy trials is because they have been unknowingly exposed to the virus used to carry the factor gene into the liver, and thus their immune system would attack and destroy that virus carrying either the factor 8 or factor 9 gene, making gene therapy ineffective. We have already treated one person with hemophilia on a gene therapy trial, and while it is too early to know how it is working, we are excited to be part of this exciting time in hemophilia.

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Find us on Facebook, Twitter, and our website for frequent updates on news and events.



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## UPCOMING OPPORTUNITIES-SAVE THE DATE!

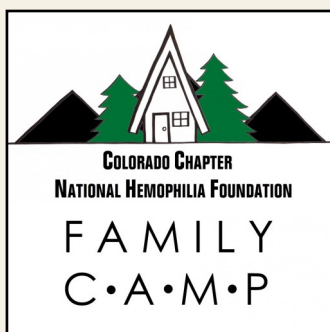


In 2019 we will again be hosting Self-Infusion Clinics, designed to help our patients master infusing! At each clinic our nursing staff is on hand to provide support, experience, and guidance as we walk patients and families through infusions. You can practice with saline or bring your own factor to infuse that day.

Our next clinic is March 19, 2019 from 5:30-7:30 pm here at our clinic. This Self-Infusion Clinic we will be focusing on helping our adult patients, so we ask that only those 18 and up attend. There will be no childcare at this clinic. Please RSVP to Kelly Ryan of NHF Colorado at [kryan@hemophilia.org](mailto:kryan@hemophilia.org) or 646-499-0684 or see details at [cohemo.org](http://cohemo.org)

Education Days will be here before you know it! Join us for some great educational programs for parents, families, and patients and get to know others in the bleeding disorders community! Education Days will start Friday, April 12, 2019 at 5 pm and goes through Sunday, April 14th at 3 pm. Ed Days will again be at the Denver Marriott South at Park Meadows, located at 10345 Park Meadows Drive, Lone Tree, CO 80124.

For questions, details and to attend, please contact Kelly Ryan at NHF Colorado at [kryan@hemophilia.org](mailto:kryan@hemophilia.org) or 646-499-0684 and find more details at [cohemo.org](http://cohemo.org).



NHF Colorado invites the whole family to come up to Rocky Mountain Village and enjoy a family camping experience! Horseback riding, zip lining, archery and so much more! Plus it's a great chance to spend time with other families in our community! NHF Colorado especially encourages families with siblings who have always wanted to attend the experience at camp.

Family Camp will be May 4-5, 2019 and registration opens up on February 1st! Don't miss this opportunity to enjoy camp together! For more information or registration questions, please contact Kelly Ryan at [kryan@hemophilia.org](mailto:kryan@hemophilia.org), phone is 646-499-0684 and go to [cohemo.org](http://cohemo.org).

## PEDIATRIC STROKE PARENT SUPPORT GROUP

### Pediatric Stroke Parent Support Group

**Tuesday**  
**February 12, 2019**  
**6:00-7:30 pm**

Our Pediatric Stroke Parent Support Group resumed meeting in January and plans are in place for regular meetings in 2019! This support group is a wonderful opportunity for parents who have had a child experience stroke to meet with HTC staff and with other families who are in similar circumstances. The support group is open to families of children of all ages and of all types of pediatric stroke. Stroke patients do not need to be patients at our stroke clinic to join these free meetings. Dinner and childcare are provided at each meeting.

Our stroke team has several new members and new and experienced families are always welcome. If you were unable to come in January, our new social workers Bryn Dunham and Whitney Tedeschi, and our stroke nurse Cindy Nederveld would love to get to know you.

Our next meeting is Tuesday, February 12th, 2019 from 6-7:30 pm here at our HTC. Parking is available in the garage just to the east of our building at 13199 E Montview Blvd. Suite 100 in Aurora. We plan on keeping our meetings on the second Tuesday of the month.

For questions or to RSVP at [strokesupport@childrenscolorado.org](mailto:strokesupport@childrenscolorado.org) or 303-724-6163 so we know how many to plan for. If you are unable to attend in person but would like to participate via skype or phone call, please let us know so we can arrange for technical support.

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**Extended half-life products:** There is finally a human-engineered FVIII recombinant product that has a reasonable chance to have true effectiveness when given once weekly. This trial is not yet open, but we are very enthusiastic about its possibilities and will be recruiting for the study as soon as it opens.

**Non-factor replacement:** With the brisk approval of emicizumab (HemLibra) for all hemophilia A patients with and without inhibitors, non-factor approaches are the focus of a lot of interest. We will have three non-factor medication studies that target a molecule, tissue factor pathway inhibitor, open at the HTC this year. These new medications hope to “re-balance” the coagulation system leading to less bleeding and improved prophylaxis. We have had one patient on an early phase clinical trial of this novel approach to hemophilia therapy.

Joint forces, factor levels and what is good enough for the sports I want to play? Dr. Beth Warren is an engineer in addition to being a pediatric hematologist and she is melding her skills to determine just how to manage factor and new



Dr. Beth Warren is working on a study measuring stress on joints during various physical activities.

therapies to safely engage in the sports we love. Dr. Warren will be recruiting boys with hemophilia on prophylaxis to actually measure the stress on the joint

with various functions of kicking, jumping, running and sliding into base. She will pair these forces with the factor level achieved with various types of hemophilia therapies and logs of clinical bleeding to determine what factor levels are safe for which boys playing which sports. This is truly personalized medicine coming into its own!

In order to study the capacity of new agents, such as Hemlibra, our HTC research lab (Katherine, Chrissy and Linda) have developed special assays to measure the hemostatic effects of non-factor products on the blood of persons with hemophilia. Our lab folk will enable us to tell you what effect you are getting from Hemlibra (and in the future with other agents) in planning whether a specific therapy will be adequate if used alone for prophylaxis.

Platelets are small blood cells that are critical to form the surface on which blood clots form. It has been difficult to study platelets from persons because the tests need to be performed on fresh blood and mild stresses can affect the test results. Dr. Pavel Davison-Castillo is interested in whether platelets are key to normal aging as well as thrombosis. In pursuit of these questions, Dr. Davison-Castillo is establishing research and clinical platelet labs to perform very sophisticated assessments of platelet function. These tests may be able to determine if genetic platelet differences are at work in the predisposition to blood clots as well as to offer better platelet testing to patients with bleeding or clotting.

You can see there is a lot of progress to be excited about. Next month I will continue the story with the equally exciting work of more of the HTC investigators.

Marilyn Manco-Johnson, M.D., Director

## RETURN YOUR PACKING SLIPS

Our Pharmacy is required to have records on file about the medications and supplies they send out. When you receive factor or other items from our HTC Pharmacy, please remember to return the delivery receipt **stamped in red ink**. Instructions are included in the packaging.

These packing slips must be signed, dated and mailed to us. There is a self-addressed and stamped envelope provided to make returning the delivery receipt easy and painless.

Unfortunately, failure to return the signed packing list verifying that you received the medications could result in a delay in receiving your next factor order or possibly being held financially responsible for the order.

**THANK YOU for your help!** Returning this packing slip is a requirement for us to comply with regulations.

Please contact us if you have questions. Our main pharmacy line is 303-724-0168 or toll free at 888-724-7427 and our email is: [hemophilia@ucdenver.edu](mailto:hemophilia@ucdenver.edu).

## RECENT HEADLINES

These are a few of the headlines that we've recently featured on our Newsroom page or on Facebook.

### **Non-Profit Save One Life Welcomes Chris Bombardier as New Executive Director**

### **Study Finds Hepatitis C is a Major Predictor of Liver Cancer in Hemophilia Patients**

### **Phase 3b Study Shows Novoeight Safe and Effective Over Long Term**

### **Sangamo Treats 1st Patient in Trial of In Vivo Genome Editing for Hemophilia B**

### **Data on Elocate and Alprolix Show Efficacy and Safety over Four Years**

We maintain a Newsroom page at our website with a list of the news we feel may be of interest to our patients and families. To see more go to: [www.medschool.ucdenver.edu/htc](http://www.medschool.ucdenver.edu/htc) then find the **RESOURCES** tab, and go to the **NEWSROOM** page. Find other stories at our Facebook page at [www.facebook.com/ColoradoHTC/](http://www.facebook.com/ColoradoHTC/)

## UPCOMING EVENTS

### **Mark Your Calendars:**

**Feb 12:** Pediatric Stroke Parent Support Group

**Feb 18:** Clinic, Pharmacy, & Admin closed for Presidents Day

**Feb 22-24:** RMHBDA Education Weekend & Annual Meeting—Bozeman, MT.

**Feb 28:** Rare Disease Day

**March is Bleeding Disorders Awareness Month**

**Mar 19:** Self-Infusion Clinic at the HTC for Adults (ages 18+)

**Mar 27-29:** NHF Washington DC Days

**Apr 12-14:** NHF CO Education Days—Lone Tree, CO

**May 4-5:** NHF CO Family Camp—Easter Seals, Rocky Mountain Village, Empire, CO

**July 12-13:** Leadership Training at Mile High Summer Camp

**July 14-19:** Mile High Summer Camp

**See more at our Events Page on our website:**  
[www.medschool.ucdenver.edu/htc](http://www.medschool.ucdenver.edu/htc) find **RESOURCES** tab, go to **EVENTS**

### **Hemophilia & Thrombosis Center**

13199 E. Montview Blvd., Suite 100  
Aurora, CO 80045

Clinic Main: 303-724-0724

or toll free at 888-297-0724

Pharmacy Main: 303-724-0168

or toll free at 888-724-7427

Web page:

<http://medschool.ucdenver.edu/htc>

PLEASE  
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HERE