

# The Clotting Connection

A Newsletter for Families with Bleeding and Clotting Disorders

December 2022  
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Hemophilia and Thrombosis Center  
UNIVERSITY OF COLORADO ANSCHUTZ MEDICAL CAMPUS



Affiliated with  
Children's Hospital Colorado

## GENE THERAPY APPROVED



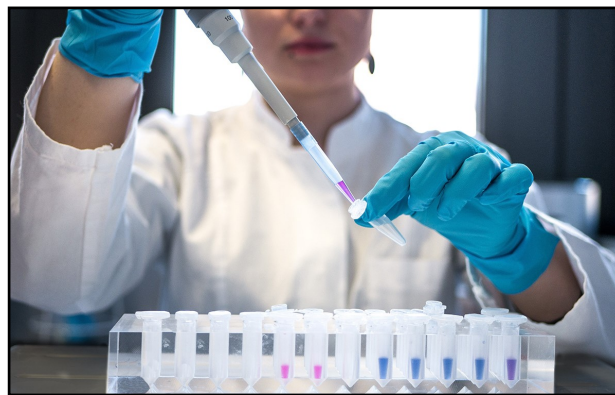
Graphic: FDA.gov

On November 22<sup>nd</sup>, 2022, the FDA approved the first gene therapy for hemophilia B. Etranacogene dezaparvovec-drlb (Hemgenix) is the first gene therapy approved in the United States for hemophilia. However, there are several other gene therapies that are in various stages of research,

including one for hemophilia A that has conditional approval in Europe and is submitted for review to the FDA.

### What is gene therapy and how will it help hemophilia patients?

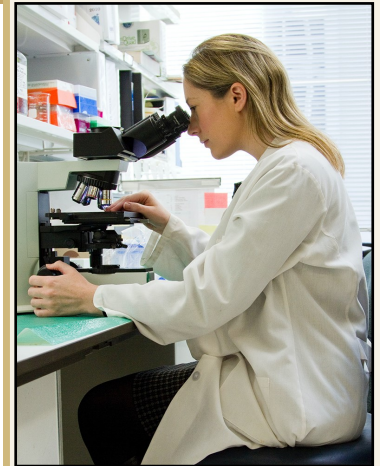
Research on gene therapies as treatment for various diseases or conditions has been explored for many decades. Instead of factor infusion, gene therapy acts to allow the patient's liver to produce factor on its own. In the approved hemophilia B gene therapy, an adeno-associated virus (AAV) is modified with copy of the gene that you want to express in the patient. The clinical research studies have shown that most patients can start producing factor in their livers, and this reduces bleeding without the infusion of additional factor. These studies are ongoing to follow the long-term effectiveness and safety of this new form of hemophilia treatment. New studies to follow patients who receive gene therapy for clinical care have begun, and these are vitally important to further our understanding of the benefits and risks of gene therapy.



### Who can get this treatment?

Criteria for patient selection for gene therapy is complex. Health and life-style are important factors in deciding who is safe to receive gene therapy. If hemophilia patients are interested in this treatment, it is important for them to talk to their HTC providers and clinical care team. This is a decision like no other and the HTC is preparing to be able to guide patients with the best advice and education.

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Years of research has led to the FDA approval of the first gene therapy for hemophilia B.

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



Go to [facebook.com/ColoradoHTC](https://www.facebook.com/ColoradoHTC)



Go to [twitter.com/HTCColorado](https://twitter.com/HTCColorado)



Website: Go to

[medschool.cuanschutz.edu/hemophilia-thrombosis](https://medschool.cuanschutz.edu/hemophilia-thrombosis)



Email:

[HTC.Newsletter@ucdenver.edu](mailto:HTC.Newsletter@ucdenver.edu)

# HTC STAFF HONORED BY NHF COLORADO

At the NHF Colorado “The Zoo and You!” event at the end of November, NHF Colorado honored several members of our community with awards recognizing their contributions.

Two of our HTC staff were honored as a part of their ceremonies! The awards presented represent two of the core values of NHF Colorado and their mission to support the bleeding disorders community.



Bryn Dunham with NHF Colorado Director Perry Jowsey. Photo: NHF Colorado.

Congratulations to Social Worker Bryn Dunham who received the “Support” award. This award was given “In special recognition of going above the call to action and your exceptional performance with your continued positivity, commitment, and service to the bleeding disorder community.” Many of you may know Bryn and know that she does go above and beyond to help provide support to our patients in her role as a social worker.

She shared, “I am deeply honored by this from an organization that works tirelessly to support the bleeding disorder community. Working with patients and families in this community is a gift – hearing their stories, their

accomplishments, and challenges brings meaning to my work as a social worker and is a testament to the resiliency of families in this community.”

Congratulations to Joseph Mah who received the “Service” award. Joseph is currently working at our HTC in research with Dr. Beth Warren on a project studying how to use clinical gait analysis to learn more about the movement of hemophilia patients. He is a vital part of this research group and spends many hours analyzing computer data. His award was given “In special recognition for your continued commitment and service to the bleeding disorder community through acts of kindness and extensive volunteerism.”



Joseph Mah receiving his award for service from NHF Colorado Director Perry Jowsey. Photo: NHF Colorado.

Joseph regularly helps at NHF Colorado events but noted that he particularly enjoyed working with the 7-year-olds at Mile High Summer Camp where he was a counselor. We thank both Bryn and Joseph for their continued support and service to the NHF Colorado community. They are wonderful assets to our HTC! Congratulations!

## NHF COLORADO UPCOMING WINTER EVENTS

### January 20-22, 2023: Winter Teen Retreat



NHF Colorado is offering a fun weekend in the mountains for young adults and teens at The YMCA of the Rockies—Snow Mountain Ranch. A younger, teens through age 17 group will have an overnight adventure on the 21st, and those 18 and older are invited to a two-day program Friday Jan 20-21 to prepare participants for mentorship and community building.

Learn more or sign up at [cohemo.org](http://cohemo.org).

### February 2023: Indoor Climbing Clinic Series (4 dates)

Each Friday in February, Backpacks + Bleeders will host a special Indoor Climbing Clinic at Movement Gym in Englewood. Each week in the series will focus on different topics, but you can attend any clinic you are able to. This special series is available for those with a diagnosed bleeding disorder, a carrier, or caregiver of a minor with a blood disorder.

There are 15 spots available for each week’s climb and you are invited to sign up for as many weeks as you can attend. See more information on and register for this exciting program at [cohemo.org](http://cohemo.org).



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### How much does it cost? Is it covered by insurance?

This therapy made national news when it was approved because of the high price. At the moment, who and how payment for gene therapy will occur is still undecided.

These are great questions to ask your HTC providers when discussing this treatment.

### What happens next?

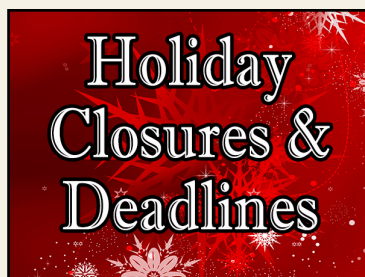
Our doctors, researchers, and providers are very familiar with the research around gene therapy and are actively

involved in understanding the impact this will have on patients.

If you have questions and want to understand more how gene therapy works, do not hesitate to reach out to us at the HTC.



## HOLIDAY CLOSURES & SHIPPING DEADLINES



Happy Holidays! Please be aware of several upcoming clinic closures as well as shipping deadlines for pharmacy products.

**Clinic, Pharmacy, & Admin offices are closed:**

**Fri-Mon Dec 23-26: Christmas Holiday**

**Mon Jan 2, 2023: New Year's Day (observed)**

**Mon Jan 16, 2023: Martin Luther King Jr. Day**

Last date to ship before each holiday:

Before Christmas	Wed., Dec 21, 2022
Before New Year's	Wed., Dec 28, 2022
Before MLK Day	Thurs., Jan 12, 2023

If you need to contact our clinic or pharmacy during any holiday, see our [contact page](#) for our after-hours info.

## HTC EMPLOYMENT OPPORTUNITIES AVAILABLE

Did you know that our HTC is hiring? We have several job openings available and some that are coming soon. A few that have been posted include:

- Pharmacy Technician
- Senior Clinical Pharmacist
- Nurse Practitioner/Physician Assistant
- Finance Professional
- Clinical Science Professional/Research

Go to [cu.edu/cu-careers](http://cu.edu/cu-careers) and click on the Anschutz Medical Campus link, and their [CU Anschutz Openings](#) page for details. If you put "Hemophilia" in the Keyword section of the search bar, the current jobs available at our center will appear. Be aware there may be some listed that are not at our HTC, so read the descriptions carefully.



## RETURN YOUR PHARMACY RECEIPTS & RECYCLE THOSE BOXES!

Our pharmacy is required to have records on file about medications and supplies they send out to our patients. 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 receipts must be signed, dated and mailed to us.** There is a self-addressed and stamped envelope provided. Thank you for your help!



Our pharmacy also has made it easier for you to recycle your Certis, specialized shipping materials! We include recycling instructions with each of our factor shipments that explain the process to have FedEx pick up your box and return it. The pharmacy is happy to print out as many return labels as you need. [Contact our pharmacy](#) if you have questions about this program or other questions about your medications at 303-724-0168 or at [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 Social Media.

**Data Shows SerpinPC Reduces Bleeds for All Hemophilia Types**

**Phase 1/2 Study Shows Durability of Investigational Gene Therapy SPK-8011 in Patients with Hemophilia A**

**FDA Approves First Gene Therapy for Hemophilia B**

**BioMarin Faces Possible Delay for Hemophilia A Gene Therapy**

**Study Shows Heart Risk Higher in Less Active Adult Hemophilia Patients**

**Real World Study Shows Switch to Kovaltry Safe, Effective for Hemophilia A**

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. Go to [medschool.cuanschutz.edu/hemophilia-thrombosis/resources/newsroom](https://medschool.cuanschutz.edu/hemophilia-thrombosis/resources/newsroom) to read more. We also share news on our [Facebook](#) and [Twitter](#) accounts.

Sign up to get this newsletter delivered directly to your email box each month! Just go to [eepurl.com/bnszoz](http://eepurl.com/bnszoz) and fill out the form!

## UPCOMING EVENTS

### Mark Your Calendars:

**Please note. These dates may be tentative and subject to change. Please check appropriate websites for confirmation of events.**

**Dec 23-26:** Clinic, Pharmacy, and Admin offices closed for Christmas Holiday

**Jan 2, 2023:** Clinic, Pharmacy, and Admin offices closed for New Year's Day (observed).

**Jan 16:** Clinic, Pharmacy, and Admin offices closed for Martin Luther King, Jr. Day

**Jan 20-22:** NHF Colorado Teen Winter Retreat

**Feb 3:** Backpacks + Bleeders Climbing Clinic

**Feb 10:** Backpacks + Bleeders Climbing Clinic

**Feb 17:** Backpacks + Bleeders Climbing Clinic

**Feb 20:** Clinic, Pharmacy, and Admin offices closed for Presidents Day

**Feb 24:** Backpacks + Bleeders Climbing Clinic

**See more at our Events Page on our website:** [medschool.cuanschutz.edu/hemophilia-thrombosis/resources/events-calendar](https://medschool.cuanschutz.edu/hemophilia-thrombosis/resources/events-calendar)

### Hemophilia & Thrombosis Center

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or toll free at 888-297-0724

Pharmacy Main: 303-724-0168

or toll free at 888-724-7427

Web page:

[medschool.cuanschutz.edu/hemophilia-thrombosis](https://medschool.cuanschutz.edu/hemophilia-thrombosis)

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