

The HCU Herald

Featuring...



HCU Hero

Anniston from Mississippi



August 2026



All things Homocystinuria: patient stories, resources, research, events and more!

HCU Hero: Anniston from Mississippi



Anniston was born in November 2017. Like most new parents, we were filled with excitement as we welcomed our beautiful baby girl. When her newborn screening was completed, we didn't think twice about it. We were told that no news was good news, and we left the hospital believing everything was fine. About two weeks later, we received a phone call from the health department saying one of Anniston's tests needed to

be repeated due to an insufficient blood sample. At the time, we still weren't that concerned because we were new parents and did not know what to expect. When the repeat test results came back, her levels were right at the cutoff, and doctors wanted to investigate further. Shortly after, we learned that she likely had Homocystinuria (HCU).

Those early days were overwhelming. Our pediatrician had very limited, outdated information, and we were referred to a specialist three hours away to confirm the diagnosis. Hearing that our newborn had a rare metabolic disorder was heartbreaking. We were suddenly faced with frightening information, strict dietary guidelines, and so many unknowns. No one in our family had ever experienced anything like this.

After Anniston's diagnosis was confirmed, we were fortunate to begin care with an incredible genetics team closer to home. Their guidance, compassion,

HCU Hero: Anniston from Mississippi

and patience helped us begin to understand HCU and how to care for our daughter.

In her first few months of life, feeding became a major struggle. Anniston wasn't gaining weight and often refused her bottles. At first, it was dismissed as gas, but as time went on, we knew something wasn't right. After months of stress and worry, she was diagnosed with severe silent reflux. Once that was properly treated, feeding slowly improved, and we finally felt a sense of relief.

We also learned early on that Anniston is B6-responsive. Initially, introducing B6 caused her reflux symptoms to worsen, so her geneticist made the careful decision to wait until her reflux was under control. When B6 was reintroduced, it became a turning point in her treatment and long-term stability.

As Anniston grew, we learned how to manage her low-methionine diet — weighing foods, measuring portions, and adjusting as she transitioned from baby food to solids. What once felt intimidating eventually became part of our everyday routine.

Seven Years Later...

Starting school was always one of our biggest fears. We worried about food, safety, and whether others would understand her needs.

HCU Hero: Anniston from Mississippi



Now, Anniston is in second grade, and we have a routine in place that gives us confidence and peace of mind. Her school has been absolutely wonderful and provides low-protein meals from Cambrooke three times a week. We also have a plan where her teacher sends us pictures before and after lunch, and we are notified about any extra snacks ahead of time. What once felt terrifying has become manageable, and we are so grateful for the support from her school.

Anniston is doing incredibly well academically. She is constantly on the honor roll and has shown no signs of concern related to her condition. In fact, her doctor has recently increased her allowed protein intake to 14 grams per day, which feels like a huge milestone.

She is a bright, smart, and beautiful little girl with an incredible personality. Her classmates are kind and accept her condition, and we have even had other parents tell us that their children now read food labels to make sure snacks they bring to school are safe for Anniston to eat. Seeing that level of empathy and awareness at such a young age is truly heartwarming.

Outside of school, Anniston is becoming quite an artist. She has an amazing talent for drawing and can recreate almost anything just by looking at it.

HCU Hero: Anniston from Mississippi



Watching her creativity and confidence grow has been one of the greatest joys of our lives.

While Anniston is doing so well medically and developmentally, there are still ongoing challenges. Securing coverage for her medical formula and medications continues to be a constant battle. Commercial insurance will not cover them, and we have had to fight to get her enrolled in a state-funded program.

Even then, obstacles remain. Some durable medical equipment providers refuse to accept the coverage because reimbursement rates are too low, creating unnecessary stress for families like ours.

Despite these challenges, we remain deeply grateful — grateful for newborn screening, for early diagnosis, for dedicated doctors and dietitians, and for the HCU community that continues to



HCU Hero: Anniston from Mississippi

support us. Because Anniston was diagnosed early, she has been able to live a full, healthy, and joyful life.

HCU is part of Anniston's story, but it does not define her. She is strong, compassionate, talented, and thriving. We are so incredibly proud of how far she has come and excited for everything her future holds.



WHAT IS LONGITUDINAL DATA?

**DATA TAKEN OVER
TIME THAT TRACKS
SUBJECTS AT
MULTIPLE POINTS**



**EXAMPLE:
TAKING THE SAME
SURVEY EVERY
YEAR**

WHY IS LONGITUDINAL DATA IMPORTANT?

**OBSERVE CHANGE AND PATTERN,
STUDY TRENDS,
CAUSE AND EFFECT RELATIONSHIPS,
LONG TERM IMPACTS**

THIS LEADS TO...

-  **BETTER UNDERSTANDING OF HCU**
-  **SHORTEN DIAGNOSTIC ODYSSEY**
-  **IMPROVE MANAGEMENT GUIDELINES**
-  **DEVELOP NEW AND BETTER THERAPIES**
- FIND A CURE!**

UPDATE OR TAKE THE HEAD TO TOE SURVEY HERE



IN CASE YOU MISSED IT...



The HC&U Podcast is back!!!

HC&U is a podcast about Homocystinuria, sponsored by HCU Network America and hosted by Ben & Lindsey.

Meet your hosts!



Welcome to the HC&U Podcast! We are Ben and Lindsey, your hosts. We are so excited to be starting this as extra resources for the Homocystinuria community. We hope you like our content!



To Listen:



<https://hcunetworkamerica.org/hcu-podcast/>

or click below on your favorite option!

LISTEN ON  Spotify

Listen on  Apple Podcasts

LISTEN ON  iHeartRADIO

Listen on  amazon music

The latest episode



During this inspiring episode, we follow the journey of an individual living with classical homocystinuria (HCU), from a difficult diagnosis at age 9 to becoming a marathon finisher. Through eye surgeries, a strict dietary regimen, and the daily challenges of managing a rare metabolic condition, he never let HCU keep him on the sidelines. From years of competitive basketball and team sports to discovering the personal challenge of distance running, his story highlights the power of preparation, perseverance, and support. Join us as we explore how small daily choices, consistent exercise, and a strong community helped turn obstacles into opportunities and athletic goals into extraordinary achievements. [owebo.com](https://www.owebo.com)



Still Dreaming About The...



2026 HCU Network America Conference





DR. GEORGE SPAETH **2026 HCU Hero** **Recipient**



READ MORE

The **HCU Hero Award** honors an individual whose lifetime of dedication has made a lasting impact on the homocystinuria community through leadership, research, advocacy, and compassionate care.

This year, we are proud to recognize Dr. George Spaeth, the first physician to diagnose a patient with HCU. His pioneering work and decades of unwavering dedication have transformed countless lives and helped shape the future of HCU care.

Thank you, Dr. Spaeth, for your extraordinary legacy and commitment to the HCU community.

Mark Your Calendars!

SUMMER VIRTUAL MEET UP



4:00 pm EST



REGISTER NOW

2026 HCU HEROES

REGISTER HERE



Cystadane; the last FDA approved drug for the homocystinurias was approved in 1996. That was 30 years ago!



Currently only one clinical trial in progress for classical HCU, but there are none in progress for cobalamin disorders and Severe MTHFR.



In collaboration with other organizations, HCU Network America has issued 10 research grants.



Help us accelerate better treatments for and help raise funds during our Race for



<https://bit.ly/hcurace26>

Pricing

- Register by July 19 to receive a medal
- Register by August 24 to receive a shirt
- Early bird pricing - \$30
- After July 13 - \$35



2026 HCU HEROES



2025 Team Spotlight

Proudly sponsored by:



TEAM HUNT
RAISED
\$6,961 AND
RAN **245**
TOTAL MILES!



Team Hunt For Research



TEAM ELLIES
ENTOURAGE
RAISED **\$5,011**
AND RAN **65.09**
TOTAL MILES!

Team Ellie's Entourage



Team Butter Stick



TEAM
BUTTERSTICK
RAISED **\$5,697**
AND RAN
5,300.48
TOTAL MILES!

Registration Open for **flok** Family Camps & Adult Retreat!

Registration is now open for September's family camp for those with inherited disorders of protein metabolism, their family members, and friends. Learn more and register at <https://flok.org/camp/>.

– **Family Camp East** on Lake Winnepesaukee in NH, September 17–20, 2026

Thanks to support from BioMarin Pharmaceutical Inc. flok's retreat and camp are **FREE** for those with Classical HCU, MSUD, an Organic Acidemia, PKU, Tyrosinemia or a Urea Cycle Disorder. Flexible pricing available for family members and friends.

Questions? Contact camp@flok.org.



Upcoming Events



REGISTER HERE



RARE
ACROSS AMERICA
EVERY VOICE, IN EVERY DISTRICT, MATTERS

Aug
10-21
2026



Rare Across America 2026 is the opportunity to meet with your Members of Congress at their in-district offices and educate them on the issues that are most important to the rare community by sharing your story.

Registration will close on Friday, July 17.

Where?

All Senate meetings will be scheduled virtually and House meetings will be in person at your Member's in-state, in-district office. Rare Disease Legislative Advocates (RDLA) will schedule meetings for you and help you to prepare. No prior advocacy experience is necessary.

When?

Meetings with Members of Congress will take place between August 10th and August 21st. We will be offering training webinars to help advocates prepare for their meetings.

Upcoming Events

2026

NEWBORN SCREENING BOOTCAMP

Empowering Advocates to Advance Life-Saving Screening

September 24, 2026



Washington, DC

- Learn about and discuss developments in newborn screening with experts
- Hear from patient advocates currently navigating the newborn screening process
- Learn how to get involved in the federal and state
- NBS systems

Register Now!



Upcoming Events



Webinar: Understanding Medical Nutrition and Insurance Coverage Challenges

Wednesday, August 19, 2026

1 p.m. to 2 p.m. ET



REGISTER NOW

Our speakers will help you understand:

- The meaning of “medically necessary nutrition products”
- Medical nutrition coverage challenges that impact the rare disease community
- How we evaluate medical nutrition coverage on our state report card



**Now
Enrolling**



Company: TraveTherapeutics

Total time in study: About 9 months

Goal: To learn more about classical homocystinuria (HCU),
and how safe and effective pegtibatinase may be in patients with HCU

Phase: This is a Phase 3 study that follows a Phase 1/Phase 2 study called COMPOSE

Who can take part?

- ✓ People with a diagnosis of HCU
- ✓ People 12 to 65 years of age

**Other factors and restrictions
also apply**

What is the study drug?

- ✓ Participants will receive
pegtibatinase or placebo
(an inactive medication)

Why participate in the study?

- ✓ You may help others with HCU
- ✓ You will help contribute to new research
and further understanding of HCU
- ✓ You will receive additional monitoring
and testing



Study locations include:
United States, Europe,
the Middle East, Asia
Pacific, and South
America.



About 70 people
with HCU will
take part in
the study.



Payment for time and
travel may be available
to participants and their
caregivers.

Periods during the study



SCREENING PERIOD

To check if
the right
study is
for you



PRE-TREATMENT PERIOD

To understand
your current diet
and how to keep
it stable during
the study



STUDY TREATMENT PERIOD

To receive pegtibatinase
or the placebo



FOLLOW-UP PERIOD

To check your
health after
the Study
Treatment
Period

After completing the **HARMONY Study**, it may be possible for participants to join a long-term follow-on study and continue or start receiving pegtibatinase.

To find out more, please contact:
MedInfo@traverse.com



For more information,
please scan the QR code or
visit:
www.hcuconnection.com

Back To School



Educators Guides

**también disponibles en español*



Classical HCU



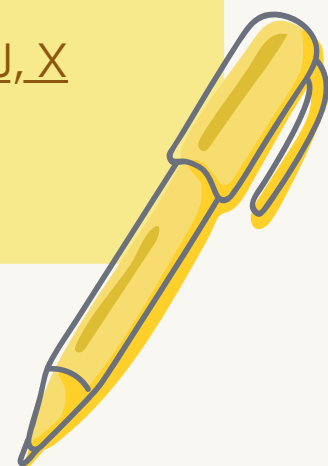
Cobalamin G & E



Cobalamin C, D, F, J, X



Severe MTHFR



Navigating Accommodations

**también disponible en español*



Parent Handbook for
Special Education Services

Low Protein Lunch resources



Cambrooke school lunch program
& more

College Transition



Off to College Guide

Customizable "All About Me" pamphlet

Click [here](#) see more Back to School & special edition topics!

Customize Your Kit For Free!

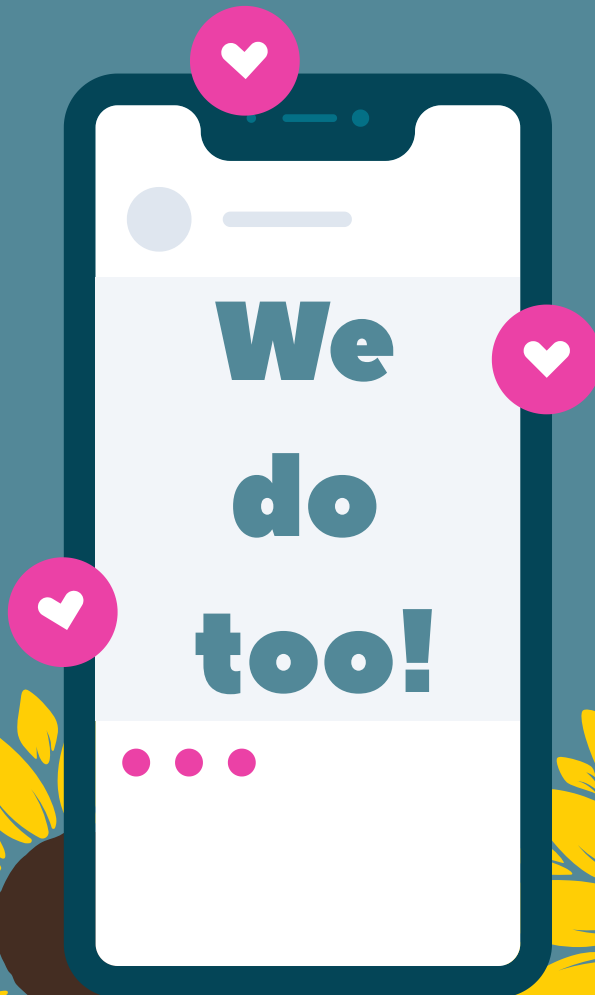


At HCU Network America, we believe that one of the most important steps to empowering patients and caregivers is giving them the support and tools needed to succeed! We know that a new diagnosis can be overwhelming and riddled with concerns and questions. To us, one way to combat those feelings, and give you the confidence you need, is by providing you with one-on-one support, educational resources, and practical tools, such as scales, cooler bags, and more! Our request for a kit survey allows you the opportunity to request a one-on-one introductory call (with more opportunities to connect), and then a customized kit to the patient's needs. Don't want a call or a Zoom? That's fine too - we are happy to send you the customized kit.

Request your kit now - <https://www.surveymonkey.com/r/HCUKitSurvey>

**Kits can only be sent to patients in the continental US. However, we are happy to connect virtually and share the educational materials with you via weblinks!*

Do you have social media??



Follow us



@hcu_network_america



HCU Network America



@HCUAmerica

- **What** is it?
 - A secure private survey for individuals or families affected by Homocystinuria
- **What** will I share?
 - Patient's birthdate, gender, exact diagnosis, and how they were diagnosed
- **What** will my info be used for?
 - Confidential and will not be shared unless we have permission
 - Helps HCUNA achieve our goals

- **Why** should I join?
 - Able to find other families and patients in your state and request contact information
 - Access to exclusive materials (ex: we may have a webinar that a presenter doesn't want to share publicly but is okay sharing with just our community)

What?

Why?

Contact Register

How?

- **How** do I participate?
 - The form takes 3-5 minutes to complete
 - Visit our website and click on "contact register" tab or...

[Click Here](#)

Follow Us!

