The HCU Herald

October is HCU Awareness Month!

Featuring...

HCU Hero
Marcus from Colorado

October 2024

HCU NETWORK AMERICA

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

HCU HERO: MARCUS FROM COLORADO

We found out I was pregnant with Marcus in April of 2021, after trying for nine months for baby number two. My pregnancy was manageable; I was exhausted the whole time and took a lot of naps, nauseous near daily for the first trimester, and miserable in the summer heat: all normal things! We were cautious from the start of my pregnancy as I had gestational hypertension with Marcus's sister Aria, but thankfully monitoring my blood pressure and taking a baby Advil daily helped keep that under control. By the time I was 36 weeks along I was done being pregnant and had a feeling Marcus was going to take his time to arrive so I asked if we could be induced. My doctor agreed to induce me at 39 weeks. On December 27th, after a full day of laboring, one failed epidural, and two pushes, Marcus was born at 10:46 pm weighing 7lbs 5oz. He had the sweetest little face and a full head of black hair, and we were in love instantly! During my labor, my sister pulled out her tarot cards and we did a reading that implied I'd need a lot of strength for this baby. I didn't know it at the time, but it was going to be a lifetime of strength.

After a few days in the hospital, we finally got to go home. Marcus wasn't gaining enough weight, so we had a few extra weight checks and were told to supplement with formula. About a week after he was born, I received a phone call from the pediatrician's office. The lady I spoke with was asking how Marcus was eating and sleeping and I thought she was just checking in on his weight gain, until she asked if he had been vomiting at all. She then said we would need to come in for additional testing because his first newborn screen was slightly 'off', and I remember her telling me it could be a false positive due to his jaundice. We took him in and got the bloodwork done, as well as a urine sample. The tests done had different results this

HCU HERO: MARCUS FROM COLORADO

time and showed other issues. I was so confused about everything going on that I could barely even explain to my husband Matthew. At this point, we didn't really tell anyone what was going on.

On January 17th, while cooking dinner, I received a call from the genetic and metabolic department at Children's Hospital. They told me Marcus' second newborn screen was also off and he required some additional testing, and to come in as soon as possible. Shortly after I received the call, Matthew came home from work, and he could tell immediately something was wrong; I broke down crying. I tried explaining the call I had just gotten but was still so confused that I really had little to say besides I that we needed to take Marcus in. We ate dinner in a hurry, I packed up the diaper bag and Marcus and I headed to Children's hospital in Aurora. Matthew and Aria stayed home; I didn't want Aria to see her little brother in the hospital and I was told we would just be waiting for lab results anyway. We arrived at the emergency department, ot checked in, and were taken back to a room.

A nurse came in and got his blood drawn right away, leaving the butterfly needle in his arm just in case, and then we waited. A few hours passed before a doctor came in and told me that Marcus was deficient in vitamin B-12. Originally, they were thinking he might have PKU which is why they retested everything. They administered two B-12 injections and told us we would need a follow up and more bloodwork. We went home to relax, still feeling a bit confused and tired, but a little hopeful that this all seemed to be over with.

Two days later I found myself on a telehealth call with the metabolic department at Children's. They explained the B-12 pathway to me and that said

HCU HERO: MARCUS FROM COLORADO

Marcus actually had high levels of B-12; that he not was deficient. Then they told me this could go one of two ways: One, his body was having a hard time breaking down B-12 and getting it into the cells. They were hoping that the injections they gave Marcus two days prior would help and that within a few weeks everything would be fine. Or two, that there may be an error in the B-12 pathway causing a bigger, lifelong problem. They asked if they could send off the extra blood they drew for genetic testing. I agreed and was told to watch Marcus for lethargy and excessive vomiting. Now we were back to waiting and worrying.

We finally shared with family and friends what was going on and asked everyone to send good vibes, positive thoughts and prayers that this was not a lifelong situation. Two weeks went by as we waited for the genetic results. During that time, I was a nervous wreck. I was trying to stay positive, but something in me told me to prepare for the news we didn't want. I tried to shove that feeling to the side, but it just kept poking at me for weeks. Marcus seemed to be doing just fine during this time, he wasn't sleeping any more than a newborn should, and he only threw up a few times; nothing that made me feel we should take him back to Children's. Finally, two weeks after I had gotten a phone call from the metabolic department, she told me Marcus did have a genetic disorder - homocystinuria with methylmalonic acidemia (Cobalamin C). The words were a blur, and the confusion and worry came rushing back; I felt my anxiety really set in.

The next day Dr. McCandless and his team showed me some diagrams of the B-12 pathway, and explained how Marcus even received a "bad" gene from Matthew and me. He told me the only treatment is a daily injection of a highly concentrated B-12, that I would have to administer. My heart shattered when I heard those words. He mentioned Betaine, but first wanted to wait and see how Marcus responded to the injections. He told me all the things that could be affected like his vision as well

HCU HERO: MARCUS FROM COLORADO

as mental and physical delays. All that shattered my heart even more, and the possibility of vision damage scared me.

On February 16th we received a delivery containing syringes, alcohol prep pads and vials of hydroxocobalamin. That evening we drove to Children's hospital to learn how to do the injections. It was snowing and I already get anxious driving in the snow, but I was even more anxious knowing what was about to happen. I put on a brave face and listened very intently to the nurse as she demonstrated how to administer the injection. When she said "okay, are you ready?", I absolutely was not ready, but I knew it had to be done. That strength from my laboring tarot reading needed to come on strong and fast! I couldn't even look Marcus in the eyes; I didn't want him to know I was the one causing temporary pain. I got the syringe filled, prepared his leg and took deep breath before giving him his very first injection. It wasn't physically hard to do but mentally I was a wreck and was holding back tears. After we finished our injection lesson the nurse took us to the lab to get bloodwork done before we headed home.

"I put on a brave face and listened very intently to the nurse as she demonstrated how to administer the injection. When she said "okay, are you ready?", I absolutely was not ready, but I knew it had to be done. I got the syringe filled, prepared his leg and took deep breath before giving him his very first injection."

At first doing the injections was rough for me mentally. Aria was scared and would run to the other room when she saw me get a syringe and vial out. But, after a few weeks of our new routine, I was finally able to get past the mental block I had and was able to just do the injections. It's funny to think I was once so anxious as now it's just a normal part of our day. The difference is, it's not as easy physically anymore: Marcus fights it hard! I was once able to swaddle him and do his injection on my own, now Matthew has to hold Marcus down so I can get it done. Aria finally came around after a few weeks too, and started watching me give Marcus his shot. She would try and calm him down by talking to him or distracting

HCU HERO: MARCUS FROM COLORADO

him with a toy. She's been the best big sister from the moment we brought him home and has continued to be amazing with him.

As Marcus continued to grow, we didn't really have many concerns for him. Even though he's on the slimmer side, he continued to gain weight nicely. He developed physically just fine; crawling by 8 months and walking by his 1st birthday. We had his first vision appointment when he was two and a half months old to establish a baseline, and we continue to get his vision checked every six months. So far, we have seen zero damage to his eyes!

When Marcus was six months old, we all got Covid. Marcus was fussy, wouldn't finish a bottle, and was sleeping a lot, so I knew we had to take him in. This visit to the ER landed us an overnight stay at Children's. After he was pumped with fluids and taking his normal feeding, we were able to go home. In April of 2023 Marcus got a stomach bug that took us back to the ER, but like before, after some fluids and rest, he was good to go home.

After Marcus turned one, he became picky with food and continues to be a snacker. We are extremely grateful he doesn't have to follow a low-protein diet because we have zero idea what he would ever eat. He did have some delayed speech, though we aren't sure if it's due to Cobalamin C or if it was just him listening until he was ready to talk more. Now, every day he picks up a new word, and I love watching his face when he's trying to form a new sentence with different words. Marcus has grown into a full-blown boy; he is rambunctious, loud, clumsy, independent and silly as ever. He is also kind and loving and gives the best hugs and kisses when he feels like it. He loves Spiderman, dancing, playing with his trucks, and copying everything Aria does. We were blessed with this little boy, and he fills our hearts every day.

HCU HERO: MARCUS FROM COLORADO

As I look back at the beginning of his life, I remember so much worry, frustration and confusion. Now I look at Marcus and am filled with happiness, hope, and the knowing that everything is okay. I wish there had been more support available when we were going through all this. I didn't find HCU Network America and the HCU community until last year, and I feel like it would have been beneficial for us to know this community existed in the beginning. I also wish we had sought support from our family sooner. We were too confused to explain anything, but being confused with people who love you and your child would have been easier, I think.

My hope for the future is that diagnosis can be done for all patients quicker than what we experienced, without all of the additional testing and so much waiting. I also hope that a better treatment option can come about. I am the only one who has ever given Marcus his injection. Our family members find it hard to watch us give it to him, or even talk about it sometimes, and it's frustrating. Until Marcus is old enough to do his injection on his own, or until a better treatment option comes around, we will not be comfortable with leaving him for longer than 24 hours. I've been lucky to be a stay-at-home mom to Aria since 2020, and thankful that I have been able to continue to stay home with Marcus as we watch him develop and grow. Having a child with medical needs is not easy. Strength is something we had to embody from the start of his life and that is something we will never surrender. Marcus is so strong and brave; he's our little superhero! We are blessed to be his parents and walk this life with him, no matter how hard it might be.

Click here to read Marcus's & other patient stories on our website!

We need Patient Stories!

BY SHARING YOUR STORY, YOU CAN....

- Spread awareness
- Help others to feel connected
- Share insight beyond our patient community of what it's like to live with homocystinuria
- Provide hope to other families

Ready to share your story?

Send us a message on social media or email us!

info@hcunetworkamerica.org

HCU NETWORK AMERICA

JOIN OUR FUNDRAISING TEAM

We are looking for new community members to join our fundraising team!

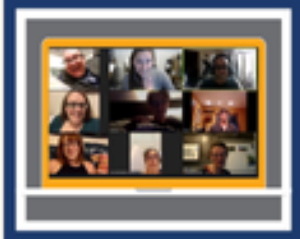
Help create, organize and support new and existing fundraising ideas.

These vital funds help support our outreach, programs and research!

To join, email Dbartke@hcunetworkamerica.org

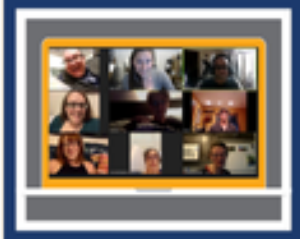
UPCOMING EVENTS

Our virtual meetups are an opportunity for patients & caregivers with any of the homocystinurias to connect, learn, and share experiences. We will also share important community updates!



https://bit.ly/fall-meetup

Our virtual meetups are an opportunity for patients & caregivers with any of the homocystinurias to connect, learn, and share experiences. We will also share important community updates!



https://bit.ly/winter-meetup

REGISTER HERE

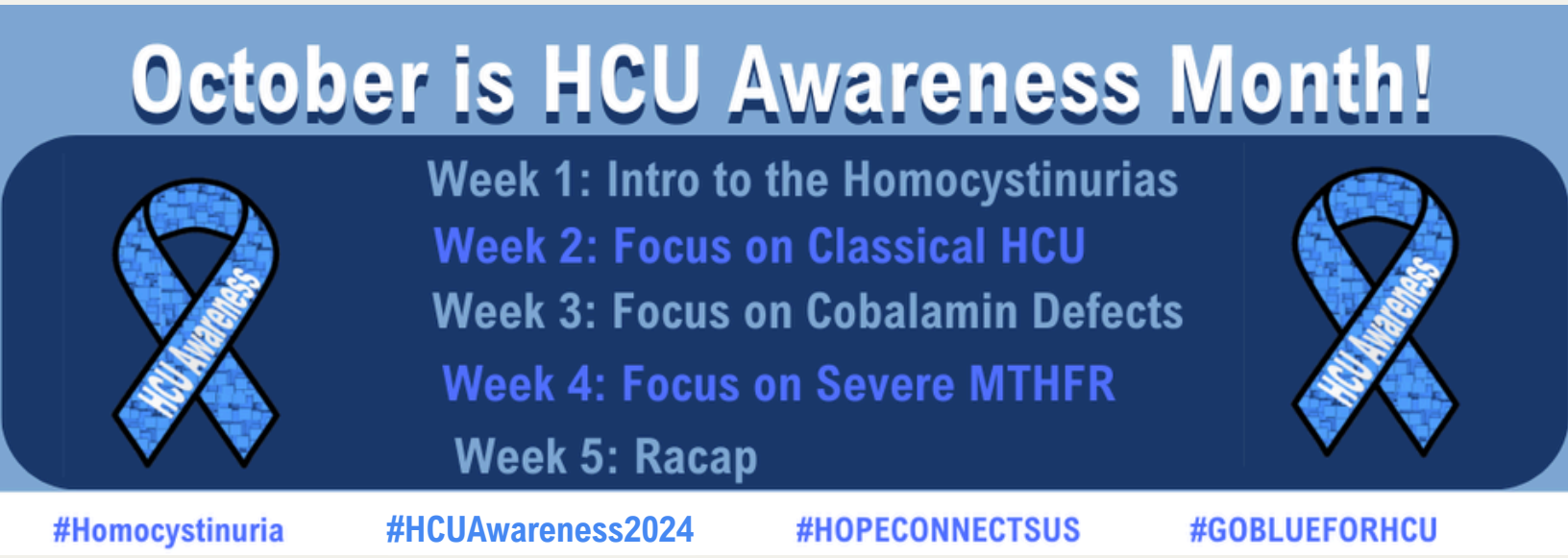
GO BLUE FOR HCU

October is HCU Awareness Month

Click here to order your gear!

All proceeds from the sale of our gear benefit HCU Network America!

HCU AWARENESS MONTH - LEVERAGING SOCIAL MEDIA!



One of the best ways to reach a wide audience to spread awareness for HCU is through social media!

How can you participate?

- Share HCU Network America's posts
- Create your own original posts (see our *Social Media Choiceboard* on the next page for ideas!)
- Share/comment on other posts from those in the community
- Start a social media fundraiser for HCU Awareness Month

Click to follow us!

HCU AWARENESS MONTH SOCIAL MEDIA CHOICEBOARD

How to use:

- Click **here** to download your copy with clickable links.
- Throughout the month, simply choose which items you'd like to post!
- Tag HCU Network America in your posts!

October is #HCUAwareness month! Show your support for the #HCU community by changing your profile to the awareness ribbon! **Awareness ribbon**	Start a fundraiser & share it out on your social media! Platform ideas: • Facebook or Instagram Fundraising • Gofundme (example) • Give Lively [Visit our fundraising page for more ideas]	#FacesofHCU: I am 1 in 200,000 people living with #HCU. *insert your photo*	Share a picture of you & an HCU Buddy, or tag a friend who is a great support. *insert your photo*
Share your/your child's patient story from our website (if you have one there). If not, share a little bit about your diagnosis. [Find the link to your story: https://hcunetworkamerica.org/patient-stories/]	Amplify another fundraiser in the community! Share out another person's fundraiser.	Share a picture of you/your family in an HCU tshirt/gear	Share a picture or video of your daily medications + formula (for those who take formula)
Share a favorite quote and how it connects to your HCU story or your outlook on living with HCU.	#HCUHero: Share/feature someone in the HCU Community who inspires you	#Hope4HCU: Share what gives you encouragement & hope	Travel with #HCU! Share a picture of your 'extra packing' to accommodate life with HCU, or your favorite travel hack!
Share a video from our Youtube Channel & why it resonates with you.	Listen to an episode of HC&U Podcast & share our your key takeaways or a quote that resonated with you	For those on a low-protein diet, share your daily allotment and a photo of a meal	Post of a picture of you/your child doing your favorite activity.
Share a picture of you from one of our conferences and tell why it was a meaningful experience for you.	For those on low-protein diet: Share your favorite low-protein meal or snack along with how many grams of protein per serving it contains.	For those on low-protein diet: Share your favorite restaurant with low-protein options & what you order there	Share something that you want people to know about HCU
Share something interesting / fact(s) from our Classical HCU Toolkit	Share something interesting / fact(s) from our Cobalamin Disorders Toolkit	Share out our MTHFR: Could it be homocystinuria? One-pager Sample post text: MTHFR, Severe MTHFR? What's the difference? MTHFR is one of the most confusing of the homocystinurias, as the common mutations are nothing like the severe form! Read to find out more.	For those with vision issues: share how this has impacted you.
Appreciate your clinic/team! Give a shout out to your clinic, team, or a specific provider who has been a champion for you in your HCU journey. If you can, tag them/the clinic in your post!	What is the most helpful piece of advice you've gotten? Share it out!	What does #community mean to you? How has finding a community of others who live with HCU been important to you?	#HopeConnectsUs: Share what your hopes are for a future therapy/treatment

It's HCU Awareness Month –
LET'S TALK FUNDRAISER SETUP!

There are many ways that you can participate in HCU Awareness Month.

One way is by hosting a fundraiser!

Not only do fundraisers raise vital funds to help us support the Homocystinuria community, they spread awareness of the Homocystinurias and the challenges of living with the conditions.

Not sure how to get started?
Let our Fundraising committee help!

Email
info@HCUnetworkamerica.org

Click here for a list of additional HCU Awareness and Fundraising Event Ideas!

OUR MATCHING GIFT IS BACK!!!

That's right, you heard us right! Thanks to anonymous donors from our community, **any funds** you help raise from October through December 31, 2024, **will be matched... up to $25,000!**

We are asking every patient and family to help us raise funds for homocystinuria. Set up a Facebook or Instagram Fundraiser, Give Lively, GoFundMe, or host your own alternative fundraising event and invite your family and friends to participate! Alternatively, they can donate directly to HCU Network America.

Have an idea for a fundraiser, but not sure how to get started? Let our fundraising committee help you get started Email info@hcunetworkamerica.org and we will connect you!

EMPLOYER MATCHING GIFT PROGRAM

Did you submit for your Employers Corporate Matching Gifts Program?

What are Matching Gifts?
Corporate matching gifts are a type of gift giving in which companies financially match donations that their employees make to nonprofit organizations. When an employee makes a donation, they need to request the matching gift from the employer, who then makes their own donation. Companies usually match at a 1:1 ratio, but some will match 1:2 or 2:1.

Why are Corporate Matching Gifts Valuable?
Corporate matching gifts are valuable because they are free money for your nonprofit of choice, HCU Network America! Your donation has double the power without you having to give double the amount.

Does HCU Network America Really Benefit?
If corporate matching gifts weren't valuable, we wouldn't be sending this letter. In 2020 we received approximately $45,000 in corporate matching gifts!

How Do I Find out if my Employer has a Corporate Matching Gifts Program?
Your company website or websites such as doublethedonation.com make it easy for you to search and see if your employer takes part in corporate matching gifts. If you can't find it online, please consult with your employee handbook, HR rep or manager to find out.

How Do I Request my Donation is Matched by my Employer?

1. The donor completes their donation
2. The donor submits matching gift request
3. Company reviews donation and nonprofit eligibility and reaches out to nonprofit
4. Nonprofit verifies the donation was made
5. If eligible, the nonprofit will receive the matching gifts request!

Top Matching Gift Companies

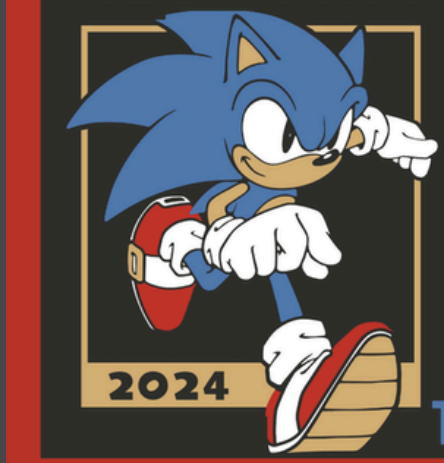
Company	Match Ratio
General Electric	1:1
Gap Corporation	1:1
ExxonMobil	3:1
Johnson & Johnson	2:1
Microsoft	1:1
Pfizer	1:1
Coca-Cola	2:1
And many more!	

Did you know some companies match retired employees donations?

With 4,014 total miles logged, Race Participants raised an astounding $58,867 for HCU research grants!

Amy's Army	$1,535 0 miles
Brooke's Blazers	$725 106 miles
Carson's Cure Chasers	$1,405 37 miles
Carter Crew 4 HCU	$500 284 miles
CblC Brazil	$30 36 miles
Cure for Casey	$655 27 miles
Ellie's Entourage	$5,560 67 miles
Grayson's Gang	$505 384 miles
"Hunt" for research	$23,590 406 miles
Leo Frank	$285 6 miles
March for Mariella	$1,895 7 miles
Masen's Mad Dawgs	$750 44 miles

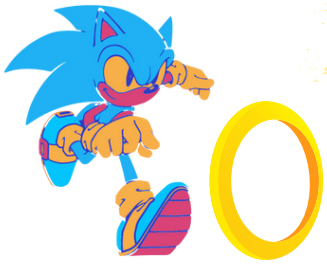
Miles for Marcus	$3,110 234 miles
Miles for Marley	$1,090 25 miles
Sarah's Sidekicks	$5 0 miles
Sonic's Sprinters	$30 0 miles
Team Anniston	$590 37 miles
Team Butter Stick	$6,380 73 miles
Team Dayton	$3,395 340 miles
Team Recordati	$505 1,129 miles
Team Will for HCU	$2,460 128 miles
Teo's Trailblazers	$940 8 miles
The Bartke Ruff Ruffs	$1,288 465 miles
Traverians	$725 165 miles



HCU HEROES RACE FOR RESEARCH

THIS SEPTEMBER WALK | RUN | RIDE

HCU NETWORK AMERICA



Thank you to our sponsors for helping to make our race a success!

RECORDATI RARE DISEASES

Learn more about Recordati Rare Diseases

eTon PHARMACEUTICALS

Learn more about Eton Pharmaceuticals

RACERS IN ACTION!

NOWCAST **KMBC 9 First News on KCWE at 7AM**

MENU KMBC NEWS 9 abc

Overland Park student rides 150 miles to raise awareness for rare disorder

Carson plays saxophone in Blue Valley Northwest High School's Howlin' Husky Marching Band.

OVERLAND PARK, Kan. – A 17-year-old Overland Park high school student is nearing the completion of an extraordinary bike ride to raise research funds and awareness for his exceptionally rare metabolic disorder.

Carson Hunt is legally blind and has minimal vision. He was born with homocystinuria **cblG (HCU)**, one of only 51 known cases worldwide for this strain.

Carson and **"Team Hunt"** in the news!

CLICK HERE

to see the full story + video

HCU ON THE MOVE - SEPTEMBER ADVOCACY RECAP!

SSIEM Annual Symposium

(Society for the Study of Inborn Errors of Metabolism)

-Porto, Portugal-

At the symposium, our Executive Director Danae was able to network with approximately 3,000 clinicians and researchers from around the globe and heard the latest research on inborn errors of metabolism, including Homocystinuria. There were over a dozen posters and presentations related to HCU! Your support has helped drive progress forward!

The EveryLife Foundation for Rare Diseases Newborn Screening Bootcamp & CDC Tour

-Atlanta GA-

- Danae & Liz, and Austin from the CDC, participated as panelists in a Fireside Chat, highlighting the collaboration between HCU Network America & CDC to improve the Newborn Screen for HCU. (click to watch)
- Grace & Joanna also represented the HCU community!
- The event also included a tour of the CDC!

Flok (low protein) Family Camp East

-Lake Winnipesaukee, NH-

Danae had the incredible experience of attending Flok Family Camp East! She shared resources, and our organization's experiences, and heard stories from those with other low-protein disorders. She also got learn about the groundbreaking platform that Flok is building. To learn more about Flok, visit: https://flok.org/research/

SIMD/NAMA

-Buford, GA-

Liz was able to chat with trainees in medical & biochemical genetics & share the resources and support that HCU Network America provides. It was an excellent opportunity to start building relationships with our future providers!

ZOIA Pharma is proud to offer a wide range of medical formulas, low protein foods and enteral nutrition from leading manufacturers to support your nutritional management needs.

ZOIA PHARMA

A Pentec™ Company

Nourishing **your unique nutritional journey**

As your one-stop for metabolic home medical and supplies, we are pleased to recognize Homocystinuria in our October blog. In honor of HCU Awareness Month, learn how ZOIA Pharma is dedicated to serving patients with this inherited metabolic disorder.

To discover how ZOIA Pharma empowers patients on the journey towards better health and well-being, visit: zoiapharma.com.

Access our rare disease spotlight on HCU

www.zoiapharma.com

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Incase 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

This month's episode is a Special Edition for Newborn Screening Awareness Month!

This time, Ben welcomes Dylan Simon, Senior Director of Policy at EveryLife Foundation for Rare Diseases & Dr. Neena Champaigne, Division Chief of Pediatric Genetics at Medical University of South Carolina (MUSC)!

HOMOCYSTINURIAS
DATA COLLECTION PROGRAM

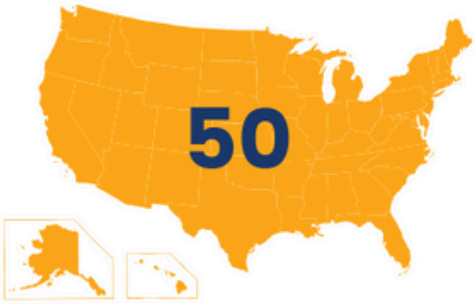
POWERED BY RARE X

Diagnosis Survey

Newborn Screening & the Homocystinurias

50%

The approximate number of babies with Classical HCU that are missed at Newborn Screening (however, some reports suggest up to 80% are missed.)



The number of states that screen for classical homocystinuria. Classical HCU was added to the RUSP (Recommended Uniform Screening Panel) in 2007, thanks to the Newborn Screening Saves Lives Act.

Cobalamin Disorders & Severe MTHFR

Combined Cobalamin Disorders are a part of the RUSP Secondary Conditions. This means they are picked up as a result of a different disorder, typcally Methylmalonic acidemia. These condtions are Cobalamin C, F, J, K, X and TC II.

Some states set a low methionine cut off and these will flag the conditions above, but also flag Isolated Cobalamin Disorders, Cbl D, E, G & Severe MTHFR.

Complete the Diagnosis Survey at https://homocystinuria.rare-x.org

CANPCU+ CANPKU+ HCU

HCU NETWORK AMERICA

HCU Network Australia

POWERED BY RARE X

Now Enrolling

Acappella

Sponsor: Travere Therapeutics
Study type: Natural History (no investigational medicine given)
Study duration: About 6.5 years
Goal: To learn more about classical HCU & the course of the disease

TO QUALIFY*	AGE OF PARTICIPANTS	DETAILS
Diagnosis of Homocystinuria due to CBS Deficiency (Classical Homocystinuria)	Currently enrolling 1 to 4 years old	The study will include three key stages (screening, enrollment, and observational follow-up) and will last approximately 6.5 years.

**Your child will need to meet all other study criteria to take part in the ACAPPELLA Study.*

Study Locations
United States: Colorado, Washington DC, Georgia, Pennsylvania
Countries outside of the US: Ireland and Qatar

Approximately 150 people aged between 1 and 65 will participate at sites in the US, Europe and other countries around the world.

150

The ACAPPELLA Study has already enrolled 100 adults and children over 5 years old, and is now looking for children aged 1 to 4 years old to take part.

You may be able to receive payment for time and travel if your child participates in this study.

Talk to your doctor and family members about your child joining the ACAPPELLA study.

Sites are open and currently enrolling participants. For the most current information about the ACAPPELLA Study and to see additional eligibility criteria, please visit:

https://www.clinicaltrials.gov/study/NCT02998710

If you have any questions, please email:

medinfo@travere.com

For more information, please scan the QR code or visit:

www.hcuconnection.com.

MA-PE-24-0008. Version 2.0; en-US; March 2024

TRAVERE THERAPEUTICS

Sign our NBS Screening Petition!

In this petition, we call for state newborn screening labs to **revise screening protocols for Classical Homocystinuria** to *ensure fewer false negative screening results and delayed diagnoses.*

Click here to sign the petition!

Get your kit!

Our FREE Customizable Kits are here!

Request yours today!

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*!

UPDATE

Now includes funding assistance for medical formula AND low protein foods!

NORD®

CLASSICAL HOMOCYSTINURIA MEDICAL ASSISTANCE PROGRAM

MEDICAL ASSISTANCE

What is the purpose of this program?

Having a rare disease is difficult. Adding in the complex care required to treat or manage that disease and figuring out how to pay for it makes a rare diagnosis even harder.

NORD's Classical HCU Medical Assistance Program offers eligible individuals diagnosed with Classical Homocystinuria financial support to pay for the low protein foods and physician prescribed medical formulas necessary in managing this HCU diagnosis.

NORD's HCU Medical Assistance Program opened thanks to a generous donation from the HCU Network America.

Who is eligible to apply?

This program is designed to help patients who:

- Have a diagnosis of Classical Homocystinuria.
- Are a United States citizen or U.S. resident of six (6) months or greater with evidence of residency such as a utility bill showing the patient's name and address.
- Meet the program's financial eligibility criteria.

What is the application process?

Patients may be referred to the program by their health care provider, their case managers, or they may self-refer.

A NORD Patient Services Representative will guide the applicant through the application process and verify eligibility for inclusion in the HCU Program.

Awards are based on meeting eligibility criteria, funding availability, and are made on a first-come, first serve basis.

NORD is Here for You

NORD, a 501(c)(3) organization, is a patient advocacy organization dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 300 patient organization members, is committed to the identification, treatment, and cure of rare disorders through programs of education, advocacy, research, and patient services.

NORD was founded by families struggling to obtain access to treatments and whose advocacy for change led to the passage of the Orphan Drug Act in 1983. NORD assists eligible patients (those with medical and financial needs) in affording the treatments and medical services their healthcare professionals have prescribed.

Alone we are rare. Together we are strong.®

How do I get more information and apply?

Contact NORD's Classical HCU Medical Assistance Program

Monday-Thursday 8:30am – 7:00pm ET
Friday 8:30 am – 6:00pm ET

203-616-4327

203-635-4163

hcu@rarediseases.org

US MAIL to: NORD
Attention: HCU Program
55 Kenosia Avenue
Danbury, CT 06810

What assistance does NORD provide?

- The Classical HCU Medical Assistance Program assists eligible individuals with out-of-pocket costs to purchase low protein foods and physician prescribed medical formulas. Individuals approved for assistance in this program will be issued a PEX card. The PEX card is a prepaid expense card to be used for the purchase of low protein foods or physician prescribed medical formulas only.
- *Upon receipt of the card, the cardholder will contact NORD to request card activation. The card will be funded based on program award caps set for the program (this cap will be discussed with individuals upon enrollment in the program..*
  - *> It is necessary for the individual to submit receipts on a monthly basis evidencing card utilization for the purchase of low protein foods or medical formulas for the previous month.*
  - *> Funds will not be added to the card until the previous month's receipts have been received by NORD.*
  - *> The card may only be utilized for the purchase of low protein foods up to the monthly program limit.*

Once a patient is accepted into the assistance program(s) how long are they eligible?

Awards are issued for a calendar year.

Patients are encouraged to reapply annually if continued assistance is needed.

What happens if an applicant does not meet the criteria of the Electronic Income Verification?

The NORD Patient Services Representative will offer to e-mail, fax, or mail the brief program application and disclosure forms to the patient. The applicant may then complete the application, sign the disclosure form, provide the appropriate financial documentation to verify financial need, and return them via fax, email, or USPS mail.

How does NORD demonstrate compliance with regulations required of charities?

- NORD independently designs its patient assistance programs based on the needs of specific patient communities.
- No pharmaceutical company or donor controls or influences our programs.
- Our patient assistance decisions are based on consistently applied financial eligibility criteria and diagnosis only.
- Patients have their choice of health care provider, treatment and treatment location, and can make changes at any time.
- Patients' privacy and well-being are priorities at NORD. We do not share or provide patient names or data with donors, nor do we disclose or identify donors to patients. Patients are able to make the choices that are best for them because NORD's assistance covers all FDA-approved products available for a diagnosis. Our programs also help with more than medication: patients can use their funds to pay for other physician prescribed services related to their diagnosis, such as laboratory and diagnostic testing, physical and occupational therapy, durable medical and adaptive equipment, and travel to medical appointments.

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NORD®

rarediseases.org

Stay connected: Join our Contact Register!

What is a contact register?

- a secured private survey that allows you to share information on you or your family member with HCU with us. (general contact info, diagnosis, etc)
- kept confidential and will not be shared unless you permit us

Why join?

- subscribe to our monthly newsletter and other communications
- identify other patients in your state and request their contact information
- access information posted over time that can only be shared with the patient community
- helps us plan events and inform the development of resources and educational tools
- supports our advocacy efforts and enables us to have informed conversations with doctors, pharmaceutical companies, state newborn screening labs, and lawmakers.

I want to participate! What's next?

You can find the form either by visiting our website and clicking on the "Contact Register" tab, or you can fill it out by going directly to:
https://www.surveymonkey.com/r/HCUContact

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FOLLOW US