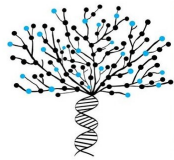


HCU Herald

Presented by



HCU Network America

Connecting for a Cure.

**There have been a lot of things happening for the HCU community & for HCUNA.
We strive to keep you informed and connected.**

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October Events:

- HCU Awareness Month
- Current Fundraisers
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Upcoming Events

- Save the Date: Rome 2019

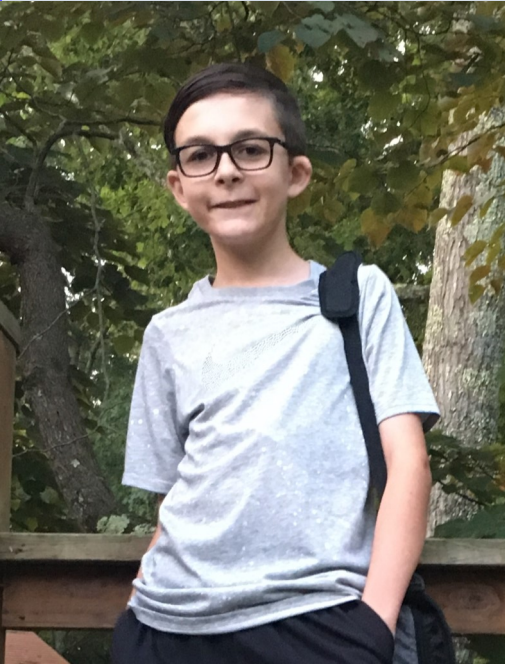
Ways to Get involved

- Contact Register
- Volunteer Opportunities



Heroes of HCU:

Landon from West Virginia



Landon was diagnosed with HCU at the age of 4. When he was born in 2007, they had not yet begun testing for Homocystinuria in our state. Our journey to diagnosis was a very long and hard one. Landon was misdiagnosed several times, and from 9 months until the age of 4 we went through 5 pediatricians, and traveled all over our state to see specialists. Landon's symptoms began with not meeting milestones on time with the other children. He also had some severe sensory issues that caused us concern. Upon recommendation of our pediatrician, we placed Landon in occupational and physical therapy. Landon did not talk or crawl until after the age of 1. He remained very weak and his sensory issues became worse. Landon would do a lot of repetitive play, and preferred to be left alone. Landon also began to lose the few words that he had learned to say. It was then suggested to us that Landon had autism. We immediately took Landon to a local Autism Center for an evaluation. We were told at the Autism center that Landon may be autistic, but they were not sure and they suggested we have some testing done on Landon by a psychologist, and we then began down that road.

In the time it took to get psychological testing at various places Landon's condition became worse. We were told on several occasions by numerous doctors that Landon was a boy and boys were slower to develop, and we were also told by some that Landon was mentally retarded and we needed to stop having him tested and accept the diagnosis of MR. But being with Landon every day and watching him, I knew that there was more going on. So I continued to take him to different doctors, ask for different testing and pursue an answer. And by the time Landon was 4, he had become unable to run and play without extreme tiredness. He could not retain things he had learned, and he became very weak on his left side. I went to a new appointment with our 5th pediatrician. When he saw Landon he was terribly upset and looked me in the eyes and said....."Your baby will die soon, he is very sick and he needs to get out of the state" and he sent us to Cincinnati Children's Hospital. After an initial misdiagnosis there, he was diagnosed properly with Homocystinuria and we began treatment. 6 months after we began diet and medications, Landon's body began to respond, and with a lot of OT, PT, and speech therapies Landon has finally been able to catch up to his peers in most ways.

We almost lost Landon. The damage to his body and brain were severe and caused a lot of health issues, muscle damage and developmental delays that could have all been avoided with a simple blood test. Through our journey we learned how to be better advocates for our child, and to encourage others to advocate for their children and to follow their gut instincts when it comes to children and their health and safety. Landon has his ups and downs with this disease, and the hardest part for us is the diet. Landon absolutely refuses to eat the low protein items that are offered from Cambrooke and PKU Perspectives, we have tried everything, so Landon's diet mainly consists of fruits and veggies. This makes variety challenging, especially when packing his lunch every day for school. I hope that someday he will choose to branch out and try new foods. We encourage him often.

Our advice to families beginning this life changing journey would be to take one day at a time, talk to each other and support each other. It can all be so overwhelming. With proper diet and medications the future can be as bright as anyone's!

HCU and You: Ask Methia

DEAR Methia,

What Can I Eat and Stay on Diet? I'm Always Hungry!

I don't know how else to say this: I'm hungry! Being on such a protein-restricted diet, I find that the foods I am able to eat simply don't keep me satisfied. I can't be the only patient with homocystinuria who feels this way. There has to be a way for me to be able to eat more – any suggestions?

Love,

Starvin' Marvin'

Dear *Starvin' Marvin'*,

You definitely are not alone. Many people do not realize just how protein-restricted the diet for homocystinuria really is, and how difficult it makes it to maintain good caloric, vitamin and mineral intake. Fortunately, over the past few years, many advancements have been made in metabolic formulas and foods to make the diet easier to follow, while still meeting nutritional needs. Here are some suggestions to help you fill up and feel satisfied!

Fill up on low protein foods. The number of companies producing low protein medical foods has skyrocketed. These foods, with little to no protein, help you fill up on calories without affecting your daily protein budget. From products like low protein pasta, to meat alternatives, to cookies and other desserts, these products have improved in taste over the years and are a staple in many low protein diets. Some states and insurance companies offer programs to cover some of the cost of these foods, which can be expensive. Contact your clinic's metabolic dietitian to inquire about companies, new products, and coverage. Don't forget to ask for samples!

Shop smart. Many gluten free and vegan products are much lower in protein, and are obtainable at your local grocery stores. Some examples are Daiya Foods (only one gram of protein per cheese slice), Shirataki noodles, and Enjoy Life! Cookies and desserts. Simply swapping a regular carbohydrate product (pasta, pretzels) for a gluten free alternative, depending on the brand, can decrease the protein content by 50% or more. Be sure to read your labels and pay attention to serving sizes.

Increase your formula intake. Talk with your dietitian about how you're feeling. Everyone is different, and your clinic will make recommendations regarding how much medical food you can and should be drinking daily. Formula certainly helps to fill you up and satisfy hunger.

Remember, your clinic is always available to you to help you brainstorm solutions to your concerns. They are not only your best resource, but a gateway to becoming connected to others who can help!

Sincerely,
Methia

New News

Orphan Technologies Starts to Recruit Patients for Enzyme Replacement Therapy Trial

Orphan Technologies has initiated a first in human study of OT-58, an enzyme replacement therapy that addresses the underlying enzyme deficit for patients living with classical homocystinuria (HCU). The goal of this trial is to evaluate the safety and efficacy of OT-58 in patients with classical HCU and identify the appropriate dose.

There are four sites in the US currently participating in the trial; Children's Hospital of Philadelphia, Boston Children's Hospital, Indiana University, and Children's Hospital Colorado. Patients between the ages of 12 and 65 years of age with classical HCU may be eligible to join.

For additional information on the sites and criteria for eligibility, please go to: <https://clinicaltrials.gov/ct2/show/NCT03406611?cond=Homocystinuria&rank=1>

To learn more about OT-58 please visit: <http://www.orphantechologies.com/ot-58/overview/>

November Marks Beginning of Open Enrollment for Health Insurance

Do you find your insurance coverage inadequate for low-protein foods, formula, betaine, or supplements? Don't fret—November marks the beginning of open enrollment for new health insurance policies.

Feeling overwhelmed? Not sure what policies cover your doctors and your medications? Don't worry, we can assist you with that!

HCU Network America has contracted Raenette Franco of Compassion Works Medical to assist you with your needs. Raenette can help you find a policy that works for you, or work with your current policy to help you get low-protein foods, medical formula, betaine and "supplements" covered.

There is no fee to work with Raenette, but we do urge you to contact her immediately if you do need a new policy. Open enrollment for 2019 ends December 15, 2018.

You may contact Raenette:
raenettef@compassionworksmrs.com
(973) 832-4736

HCU Network Australia releases Classical HCU Patient Information Booklet and Quick Guide



HCU Network Australia is excited to announce the release of two new resources, the Classical Homocystinuria Patient Information Booklet and Quick Guide. These new resources were produced by HCU Network Australia as a part of our continued commitment to improve health outcomes through education, research and support. The goal of these new resources is to help the patient, their family or caregiver understand more about classical homocystinuria, how it is diagnosed and the therapies it requires.

To view and download these tools, please visit:
<https://www.hcunetworkaustralia.org.au/patient-information-booklet-quick-guide/>

HCU Awareness & You

GO BLUE FOR HCU

October is HCU Awareness Month

HCU Network America — Hcunetworkamerica.org — Hcunetworkamerica@gmail.com

Thanks to an anonymous donor, any **funds** you help raise from October through December 31 **will be matched up to \$20,000!**

We are asking every patient and family to help us raise funds asking family and friends to donate, which you can do through a Facebook Fundraiser or by asking them to donate, and/or through a fundraising event. Have an idea but not sure how to get started? Let our fundraising committee help you get started!

Email hcunetworkamerica@gmail.com and we will connect you!

Maximize your impact



with a matching gift!

15,000 Companies
Match Gifts...

Does Yours?

Please contact your HR Department
to find out if your gift or volunteer hours
can be matched!

Need information to complete your match?

Visit: <https://hcunetworkamerica.org/company-matching/>

HCU Awareness Month Calendar

See what you can do each day to help raise awareness and funding!

Sunday	Monday	Tuesday	Wednesday	Thursday	Friday	Saturday
	1) Change Facebook picture to the HCU Awareness Ribbon	2) Start your HCU Facebook fundraiser & invite your friends	3) Share the HCU Network America Infographic	4) Share a HCU patient story video!	5) Share a pic of your first pair of glasses, or the fact: by age 10, 55% -82% of patients will have their lenses dislocate	6) Share the HCU timeline - if you know other timeline facts, let us know!
7) Share your diagnosis story!	8) #GoBlueForHCU	9) #HCUAwareness post in a public place!	10) Share a low protein meme	11) #Create4Cure create a work of art that brings awareness for HCU	12) Share with one stranger what HCU is & why it's important to	13) Wear jeans for our rare genes
14) Wear your HCU Shirt and share a pic online!	15) Challenge your friends to eat 10 g of protein and a normal protein shake 3 times a day. #ToastToHCU	16) Share a pic of an item that has the same amount of protein you can have	17) Share your daily diet record - completed	18) Real cost of HCU: Grocery Cost Comparison #MedicalNutritionEquityAct	19) Share a pic of what your grocery store "haul" looks like	20) Share your favorite low protein recipe! Bonus if you cook it and share a pic.
21) Dining out, low - protein style!	22) #HaikuForHCU Write and share a Haiku describing life with HCU	23) Share something you wish people would understand about HCU	24) #High5forHCU List 5 ways HCU makes you a stronger, better person	25) #Hope4HCU Share 4 things that give you hope and encouragement	26) #FacesofHCU Share a picture of you saying "I am one of the estimated 1 in 250,000 people with HCU"	27) Share a picture or video capturing all the medication and formula you take in a day!
28) Share a picture of you and a HCU buddy! Or tag a friend who is a great support!	29) Newborn Screening: All 50 states for HCU at birth, but many patients are still	30) Cutting Edge of HCU: Share about a HCU therapy that is in the works!	31) Your message to the world about HCU!			

Confused about one of the activities?

Find examples and resources on our website: <https://hcunetworkamerica.org/hcu-awareness-month/>

Follow us on Facebook, Twitter and Instagram during the month for examples and additional activities!

Help spread awareness, share your participation on social media and your Facebook Fundraising page!

Current Fundraisers

Love, Hope and HCU T-Shirt Fundraiser

Organized by Danae' Bartke



\$24.99 per T-Shirt

To order:

Adult sizes:

<https://www.bonfire.com/hope-love-and-hcu/>

Youth sizes:

<https://www.bonfire.com/youth-hope-love-and-hcu/>

Last day to order: October 13th

Made to Order Bake Sale

Organized by Amber Gibson

Who doesn't love homemade baked goods? If you live in the Indianapolis, Indiana area or know some one who does—tell them to check out this tasty fundraiser!

Amber can also do low protein baked goods as well!
See flyer for further details.

Made to Order

Bake Sale

Items include, but are not limited to:
Cheesecakes, cookies, brownies, bars, breads

All proceeds will go to HCU Network America
Samantha was diagnosed with Homocystinuria (HCU) at birth.
When not treated, Homocystinuria can be life threatening!

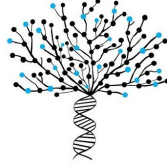
To Order contact Amber at:
agibson11d@outlook.com or 317-847-5350

Current Fundraisers



**RECORDATI
RARE DISEASES**

Focused on the Few™



HCU Network America

During the month of October, Recordati Rare Diseases will make a \$1 donation (up to \$5,000) for every click to HCU Network America!



WORKING TOGETHER TO IMPROVE EDUCATION AND AWARENESS OF HOMOCYSTINURIA



During Homocystinuria Awareness Month, Recordati Rare Diseases will make a \$1 donation (up to \$5,000) for every click to HCU Network America.

[Click here to make a difference](#)

(Click the picture to make a difference!)

Navigating our Future

Global Research Map Update

October 23, 2018—7pm EST

Featuring, **Margie McGlynn, HCU Network America President**

Over the past few years, a project was sponsored by HCU Network America and HCU Network Australia to develop a global research map that describes the research underway for new therapies to treat homocystinuria, focusing on HCU caused by CBS deficiency. A global research strategy was also developed and Scientific Advisory Board was convened to define the top priorities for funding via new global grants program. This presentation will summarize the findings from this project and status of the global grants process and the status of trials underway.

For more details and to register: <https://hcunetworkamerica.org/global-map-webinar/>



2019 Rome Conference

rome
2019 28 FEB - 1 MAR

3rd International Patient - Expert Meeting



See the link for more info: <https://www.hcunetworkaustralia.org.au/patient-expert-meeting-2019/>



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- Cinnamon-Sugar
 - Sold in packages of 8—\$10.00
- Onion-Parsley
 - Sold in packages of 8—\$10.00

Specials

Buy 3 loaves of White Bread or Cinnamon— Swirl bread or combination of, and get the 4th one for 5\$



Buy either 3 pizza bread, Sub Rolls, Hamburger Buns or any combination of, and get the 4th one for \$6

Seasonal Items —Order by October 22, 2018 to receive by Monday, October 29, 2018



- Quick Breads
 - Pumpkin-Craisin and Apple-Cinnamon
- Carmel "Granola" Tapioca Mix
 - Includes dried apple, pineapple, raisins and lemon bits
- Cookies
 - Halloween Frosted Sugar Cookies
 - Apple Chunk, Hawaii Mania, Lemon Blast, Cinnamon Roll
- Bars
 - Pumpkin and Blueberry

Specials for September 15—October 15, 2018

Contact Register



Did you know that just under one year ago we launched our contact register?

What is the contact register?

The contact register is a secured private survey that allows you to share information on you or your family member with HCU with us. This includes where you are from, your relationship to homocystinuria, the patient's birthdate, gender, their exact diagnosis (e.g. CBS, cobalamin, or MTHFR), how they were diagnosed, and if the patient was diagnosed through newborn screening. This information is kept confidential and will not be shared unless you give us permission to. By registering, you will be also be able to identify other affected patients in your state and request their contact information, and you will be able to access information posted over time that can only be shared with the patient community. (For example, we may have webinars that the expert presenter does not want to be publicly available, but is willing to share with the HCU community.)

What will this information be used for?

HCU Network America strives to inform and provide resources for patients and families, create connections, and support advancement of diagnosis and treatment of HCU and related disorders. The information you provide helps us succeed in our mission - plan events, develop resources and educational tools, and ensure everything is being done to support timely and accurate diagnosis from birth. It also allows us to have informed conversations with doctors, pharmaceutical companies, and law makers. Your information helps us understand the landscape better so we can better advocate for you!

How do I participate?

The contact register form takes approximately 3-5 minutes to complete. 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://hcunetworkamerica.org/contact-register/>

VOLUNTEER

GET INVOLVED

You can make a difference!

Our fundraising committee is looking for volunteers!
E-mail HCU Network America and we will connect you



We'd like to thank the following content contributors:

Editor in Chief: Danae' Bartke

Heroes of HCU: Landon from West Virginia

HCU and You: *Ask Methia*: Angela Pipetone

[Click to donate directly](#)