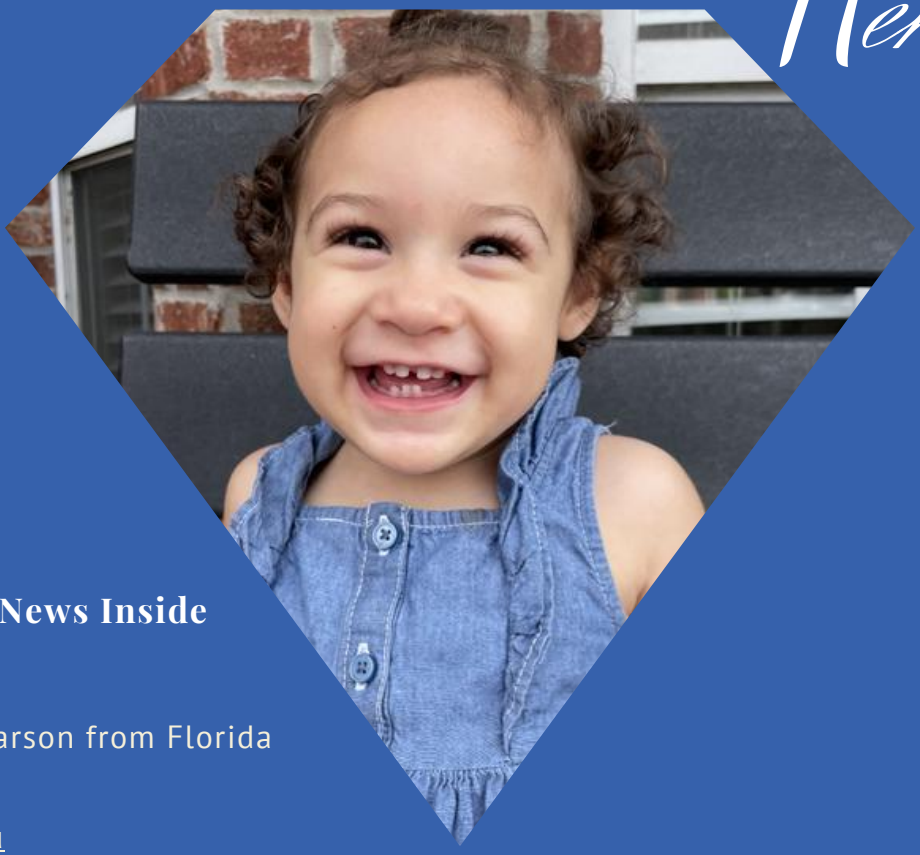
The HCU *Herald*



More HCU News Inside

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Time to Celebrate:

HCU Awareness Month

- Go Blue for HCU Campaign
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Get Connected & Involved:

- Join our Cobalamin Steering Committee

Tell us about you and help us clean up your inbox!

In order to provide the best user experience and programs, please let us know a little bit more about you. We are hoping to define your email experience, so you don't get as many emails from us in the future. These questions will give you a more tailored experience.

https://us14.list-manage.com/survey?u=f4df0c738edbf7df6565df2fe&id=842cefb887

LOW PROTEIN
SPOOKY CHARCUTERIE BOARD

Impress your little ghost and ghouls this Halloween with these treats
Here are some ideas to get you started!

- Snyder Gluten free pretzel rods dipped in white almond bark
  - then sprinkled with Halloween sprinkls
  - or, drizzled with melted Enjoy Life Ricemilk Chocolate Morsels and candy eyes.
- Low protein cheese slices cut with Halloween Cookie Cutters
- Apple slices, cookie butter or biscoff with Dandies Vegan Mini Marshmallows "teeth"
- Clementines with marker faces
- Banana's with Enjoy Life Ricemilk Chocolate Morsels
- Strawberries dipped in white almond bark with painted Enjoy Life Ricemilk Chocolate Morsel faces
- Cook for love pumpkin bread, made into a pancake maker then pressed with a pumpkin cookie cutter and drizzled with maple syrup.

HCU HERO: CARSON HOMOCYSTINURIA DUE TO MTHFR

My daughter, Carson, was born in June 2020. I received a call 1.5 weeks later about Carson's abnormal newborn screening. I was told it was most likely an error, but they were suspecting Homocystinuria and needed to redo the newborn screening. I ended the call confused and scared.

A week later, I received a call confirming that Carson had Homocystinuria due to severe MTHFR deficiency. A geneticist wanted to see us immediately. The first appointment consisted of explaining the condition, examining Carson, blood work, and a genetic test. She needed to start taking Cystadane (Betaine) and daily B12 injections (at least until we received the results of the genetic test at which point, she no longer needed it). Carson started both of those within two days of seeing the geneticist.

For these first three weeks, Carson barely gained any weight and she spent most of the time sleeping. By this point, she had been taking the medications for about 3 days and I noticed she started to become more alert. With her eyes now staying open longer, I noticed that she had trouble focusing them. She was trying to look at me, but her eyes were uncontrollably rolling up. I took a video and sent it to her doctor. I was told to get her to the ER immediately, that it may be a sign of seizures. I saw Carson have three seizures that day, one of which was in the presence of the ER doctor. After the doctor witnessed a seizure, she immediately called Carson's team of specialists. They wanted Carson flown to their facility 4 hours away to monitor her.

Carson was in the hospital for 3 days. She had an MRI and an EEG. Her Cystadane was also increased. I started noticing major improvements the second day. That was the first time Carson was able to open her eyes and stare at me. All tests were looking good so she was discharged. Carson had not even been taking Cystadane for a week at this point. I believe as the Cystadane was beginning to lower her homocysteine, and she was sleeping less and was more alert, we were able to notice symptoms that were previously hidden.

Carson had a one month well visit exactly one week after she was brought to the ER. After not gaining any weight for the first three weeks, she gained over one pound in only one week of starting Cystadane.

Carson is consistently 3-4 weeks behind developmentally. It was almost as if Carson completely "lost" that time before she started taking Cystadane.

In the beginning, we were traveling to Gainsville every two months to see a geneticist, metabolic specialist, neurologist, and ophthalmologist. We now have to go every 6 months. Carson also sees a physical therapist once a week.

Around 6 to 7 months, her development exploded! Within one month, Carson started rolling, sitting, and crawling. She started walking at 11 months. She is currently 14 months old. She recently started babbling, clapping, and saying “yum” for food. We have not had any major issues since she went to the hospital at 3 weeks of age. Carson is the sweetest and happiest baby. It is impossible to look at her and tell she has a rare disease.

Email us your patient story! info@hcunetworkamerica.org

Focused on the Few™...

At Recordati Rare Diseases, we focus on the few - those affected by rare diseases. They are our top priority and at the core of everything we do. Our mission is to reduce the impact of extremely rare and devastating diseases by providing urgently needed therapies. We work side-by-side with rare disease communities to increase awareness, improve diagnosis and expand availability of treatments for people with rare diseases.

We are proud to support the mission and vision of the HCU Network America.

RECORDATI RARE DISEASES GROUP
Focused on the Few™

www.recordatirarediseases.com/us
@RecordatiRareUS
NP-RRD-US-0231

October 2021, HCU and You: *Ask Methia*

Dear Methia

I have my annual visit with my geneticist and metabolic dietitian fast approaching. Over the past few years, I have realized that I leave these visits with more questions and end up reaching out to my clinic again afterward. They are very accommodating, but I would really love to go into my visit prepared and leave "no stone unturned." Can you give me some advice on how to have the most productive visit?

Sincerely,
A *Planning Patient*

Dear *Planning Patient*,
Your efforts to be prepared for your upcoming visit are sure to pay off. By being an active member of your own care team, you are helping your providers help YOU to be happy, healthy, and in the best metabolic control. I'd love to give you a sample "to-do" list!

- Know your height and weight. If your visit is in person, this will likely be measured for you. If your visit is via telemedicine, then your providers will be asking you for this information. Take note of any weight trends. Have you noticed any considerable changes since your last visit?
- Have an updated list of your medications. Make a word document of all of the medications you are taking. Note the doses and the number of times per day, week, or month you are taking them. Are there any medications you haven't been compliant with since your last visit? If so, what have the barriers been to being compliant with the medication?
- Make note of what has happened since your last visit. Were you hospitalized or diagnosed with any new medical problems? Are there any changes to your family history? Make sure your clinic knows about any new diagnoses, as this will influence their recommendations for your monitoring and follow-up.
- Think about how things are TRULY going – in all aspects of your life. Make sure your providers know about your comfort level with your daily routine. This includes who you live with, your school/employment status, how secure you feel financially, your support system, and your access to formula and medications. Are you not feeling supported by your partner in following your diet? Are insurance issues getting in the way of timely metabolic formula delivery? These are all important things to share with your team.
- Provide a diet record. A 24-hour recall is often helpful for your metabolic dietitian to assess your caloric and protein intake, but more is better! About a week prior to your visit, start writing down everything you are eating and drinking, including formula. This allows your dietitian to see variability in your diet from a weekday to a weekend and calculate averages.

Most importantly, make sure you approach the upcoming visit with honesty. Often times, we withhold important information that feels embarrassing or reveals our imperfections. Being honest about anything that is a struggle is the best way to show your providers how to help you. Always remember that they are here to help, and that YOU are the most important member of your care team!

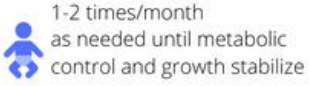
Sincerely,
Methia

NEW RESOURCE:

Cobalamin Disorder Monitoring Recommendations

Genetic or Metabolic Clinic Visits

Sources : Huemer et al. 2016 https://doi.org/10.1007/s10545-016-9991-4
Sloan et al. 2018 https://www.ncbi.nlm.nih.gov/books/NBK1328/



Monitor at Every Clinic Visit

Biochemical/Metabolic Control: Area to Monitor: Recommended Values

Plasma Total Homocysteine: <5y/o <40 umol/L <12y/o <60 umol/L Adults <100 umol/L Sick <100 umol/ L
Methionine: 20-50 umol/L
Serum Methylmalonic Acid: <10 umol/L

Anthropometry

Height: Poor Growth
Weight: Malnutrition, Poor Weight Gain
Head Circumference: Microcephaly

Diet

Food Diary: Poor feeding, Meet caloric and protein goals

Monitor Yearly

Nutritional/Metabolic Labs

Area to Monitor: Associated Issues

Vitamin B12
Plasma Amino Acids: Track methionine to adjust Betaine and OHCBL
Complete Blood Count: Cytopenia, Megaloblastic Anemia
Prealbumin: Malnutrition, Liver Function
Albumin/Total protein: Malnutrition, Liver Function
Urine Organic Acids
ALT/AST
Electrolytes
Renal Function
Carnitine plasma, total/free

Ophthalmological

Eye Exam: Maculopathy, Retinopathy, Strabismus detection and management
(retinal photography, optical coherence tomography, ERG (every 6 months first 2 years):
Ophthalmologic Assessment: Referral to resources/low vision services for Visually Impaired individuals

Kidney

Blood tests: Creatinine, Blood Urea Nitrogen, Cystatin C: Kidney disease, Thrombotic Microangiopathy, Hemolytic Uremic Syndrome
Urine tests: Urinalysis

Neurological and Neurodevelopment/Neuropsychological

Clinical Exam: Hypotonia, Seizures, Peripheral Neuropathy, Abnormal Movements, Developmental milestones, Need for services, IEP

Cardiovascular

Cardiovascular Risk Factor Review: Thromboembolic complications
Echocardiogram: Left Ventricular Non-Compaction, Other congenital heart disease
Blood pressure: Hypertension

Monitor As Needed

Area to Monitor: Associated Issues

Nutritional/Metabolic Labs

Ferritin/Iron: Periodic Nutritional Assessment
25-Hydroxyvitamin D
Essential Fatty Acids

Neurodevelopment /Neuropsychological

IQ Testing: Cognitive Impairment, Autism Evaluation

Psychological

Clinical Psychology or Psychiatric Assessment: Behavioral & Mental Disorders

Rehabilitation Medicine

Physical Medicine and Rehab, Physical, Occupational and Speech therapy

Neurology

Brain and Spine MRI: Hydrocephalus, Seizures, Movement Disorders. Subacute Combined Degeneration of the spinal cord
Electroencephalogram (EEG): Seizures
Electromyogram/Nerve Conduction Studies: Neuropathy

Liver Health

Abdominal Ultrasound: Fatty Liver
Every 3-5 years from adolescence
Unless clinically indicated earlier

Bone Health

DEXA Scan: Every 5 years from adolescence
Unless clinically indicated earlier: Bone health

An Interview with :

Alex Orange Drink

Alex Orange Drink is the solo moniker for Alex Zarou Levine, lead singer and songwriter of The So So Glos. Alex is known for his "streetwise and irreverent lyrics", which have drawn comparisons from William Wordsworth to the Beastie Boys.

Tell us about your diagnosis:

How old were you when you were diagnosed with HCU?

Luckily, I was picked up at birth in 1987 through New York Newborn Screening. I was pretty sick for the first 3-4 weeks, until diagnosis was confirmed.

How did you feel about the diagnosis growing up?

As a kid, I just ignored it. That carried into my late 20s. I didn't focus much brain energy on it, but definitely had an awareness I had it, and it had extreme consequences. I felt like I had to pretend that I didn't have it - It was my thing. I would just go to parties and figure it out.

What are some of the challenges you have faced with HCU?

Just having the diagnosis of Homocystinuria definitely messed me up because of all the restrictions and constant threat, and then the feeling of being a guinea pig. There is a lot of uncertainty and isolation – it was scary. So, I tried to distract myself with art and music, specifically punk rock, rock and roll and hip hop. Whether the struggle is medical, personal, economical, you can find a haven in this type of music, which is why I gravitated towards it.

Let's talk about your music

How old were you when you first got "into music"

I was 4 years old. We didn't know how to play our instruments, but we were given a lot of freedom to make a lot of noise; that was probably very annoying. I was compulsively writing songs and recording them – there were tapes, and tapes, and tapes of them at that age. Then as we grew up, we became The So So Glos – we became a staple in the DIY Brooklyn, NY scene. We got to play a lot of shows, including Letterman and traveled for over 10 years internationally. We are still a band working on a new album.

I started this solo album about 4 years ago while the band was taking a little break after touring for the past decade. I had a major shape shift in my personal life, and the songs had a different voice, a little bit more personal, more introspective. That's where this album came from.

When did you know that this is what you wanted to do professionally? Did you ever consider anything else – any jobs prior?

While I haven't mastered music, I've mastered what I do to an extent I'm like "I got this". So, it was never a conscious decision to do this, it was just automatic. Early on I didn't do anything else, and now I feel like it's too late to do anything else. It was never a conscious decision. I've had a lot of jobs though between gigs. Construction, art handler, you name it – whatever it took to pay the bills and stay on top of things.

Who are your musical idols/inspirations? Do you believe they influenced your ability to include bits of your personal life and diagnosis into your music?

The ones who inspire me are the ones who are brave enough to be honest. Joe Strummer of the Clash has a quote "You don't have to learn the 15-minute guitar solo to get up there, you can have 4 chords and the truth." Jimmy Clif & Notorious Big were very honest poets, who put all their strife and struggle into making a negative into a positive. Harry Belafonte and Fiona Apple are a couple others I draw from.

Diagnosis meets music:

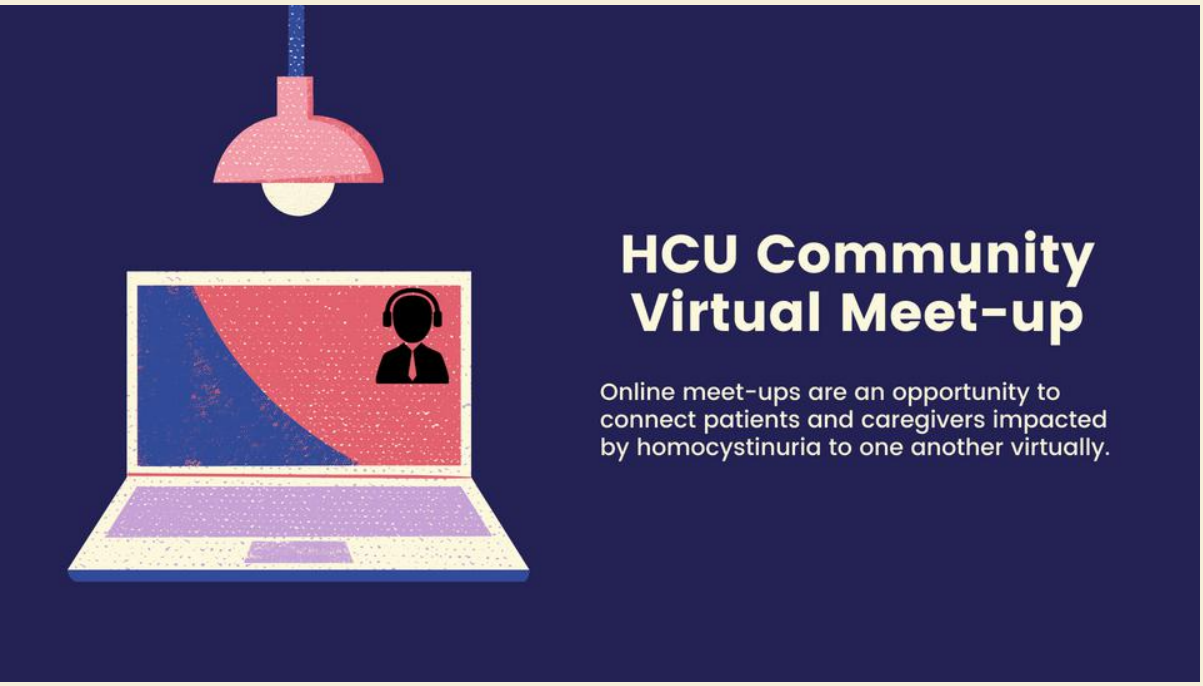
Do you ever feel that your diagnosis was a setback for your musical career?

In terms of learning music in general, I felt dyslexic in a way. I couldn't learn conventional music. I couldn't do it by the book. I couldn't learn the notes, music theory, I'm not good at math – my brain didn't work in that way. Not sure if it's psychological or not, nature or nurture. But it caused deep insecurity from not grasping certain concepts musically and feeling like I was limited by having a disorder.

See page 23 to continue with interview

LET'S CELEBRATE, OCTOBER IS HCU AWARENESS MONTH

2021 is our 5th year of going blue for HCU – we will be releasing a limited run of Go Blue for HCU 2021 apparel. You will not be able to get it after this year. Get yours now!
https://www.bonfire.com/store/hcu-haberdashery/



Sunday, October 3 at 2 pm CT | 8 pm Ireland & UK

Online meetups are an opportunity to connect other patients and caregivers.

- **Struggling with treatment? Or you are having trouble getting it covered?**
- **Having health issues, you aren't sure if it's related to the diagnosis, or just a normal person thing?**
- **Or just looking to connect?**

Come join us for one of our Community Meetup

Register now at: https://www.eventbrite.com/o/hcu-network-america-30163980100

Sunday, October 17 at 10 am CT | 4 pm Ireland & UK

HCU Awareness Month Activity List

We know this is a rather long list, but we love your participation as it helps raise awareness for our small but mighty community. Please try to do as many as you can. If you do one each day, you'd complete the list!

- ☐ Change your social media picture to the HCU Awareness Ribbon
- ☐ Start a HCU fundraiser
- ☐ Share an infographic about HCU
- ☐ Share a patient story
- ☐ Share your diagnosis story
- ☐ Challenge your friends to the same amount of protein and three normal protein shakes a day #ToastTocHCU
- ☐ Share a pic of an item that has the same amount of protein you can have
- ☐ Share your daily diet record –completed
- ☐ Share a low-protein meme
- ☐ Share your favorite low protein recipe! Bonus if you cook it and share a pic
- ☐ Dining out, low protein style. Where do you like to eat?
- ☐ Share a pic of what your grocery store haul looks like
- ☐ Real cost of HCU: Grocery Cost Comparison #Medical Nutrition Equity Act, or share some patients with HCU require injectable B12. B12 on average is $300-400 a month and most insurance companies don't cover it!
- ☐ Share a picture or video capturing all the medication you take (this includes formula for those who need it).
- ☐ Share a picture of your first pair of glasses, or a device that helps you navigate or communicate due to lack of vision
- ☐ Share something you wish people understood about HCU
- ☐ #HaikuforHCU—Write and share a Haiku describing life with HCU
- ☐ Wear jeans for your rare genes #ItsInOurGenes
- ☐ Wear your HCU Shirt and share a pic online—#HopeConnectsUs
- ☐ #GoBlueforHCU
- ☐ #HCUAwareness post in a public place
- ☐ Share with a stranger what HCU is and why it's important to you
- ☐ #Create4Cure—Create a work of art that brings awareness for HCU— can be a song, dance, a painting—get creative!
- ☐ #High5forHCU—List 5 ways HCU makes you a stronger, better person
- ☐ All states test for classical HCU, but many are still missed
- ☐ Share a picture of you and a HCU buddy! Or tag a friend who is of great support
- ☐ #FacesofHCU—Share a picture of you saying, I am one of the 1 in 200,000 people with HCU
- ☐ #Hope4HCU—Share 4 things that give you hope and encouragement
- ☐ Share the HCU timeline—if you know other facts, let us know!
- ☐ Cutting Edge of HCU: Share about a therapy that is in the works!

To find additional information and resources, visit:
https://hcunetworkamerica.org/hcu-awareness-month/

OUR $25K MATCH IS BACK!

That's right, you heard us right! Thanks to two anonymous donors, any **funds** you help raise from October through December 31, 2021 **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 Fundraiser or host your own alternative fundraising event and invite your family and friends to participate. Alternatively, they can donate directly to HCU Network America. **Anyone who creates a fundraiser and raises over $100, will receive a HCU Awareness car magnet!**

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!

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 Does this Benefit my Employer?
Companies of all sizes match donations their employees make to nonprofits because it's an easy way for them to support good work in their communities. Corporate Social Responsibility (CSR), is an important factor in how the public perceives brands and companies these days.

Corporate matching gifts are an efficient and straightforward way for companies to build relationships with charities.

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
• BP (British Petroleum)	1:1
• Gap Corporation	1:1
• ExxonMobil	3:1
• CarMax	1:1
• Johnson & Johnson	2:1
• Microsoft	1:1
• Pfizer	1:1
• Coca-Cola	2:1
• Avon	2:1
• IBM	1:1
• And many, many more!	

Did you know some companies will match donations from their retired employees?

EXPLORE NEW RESEARCH!

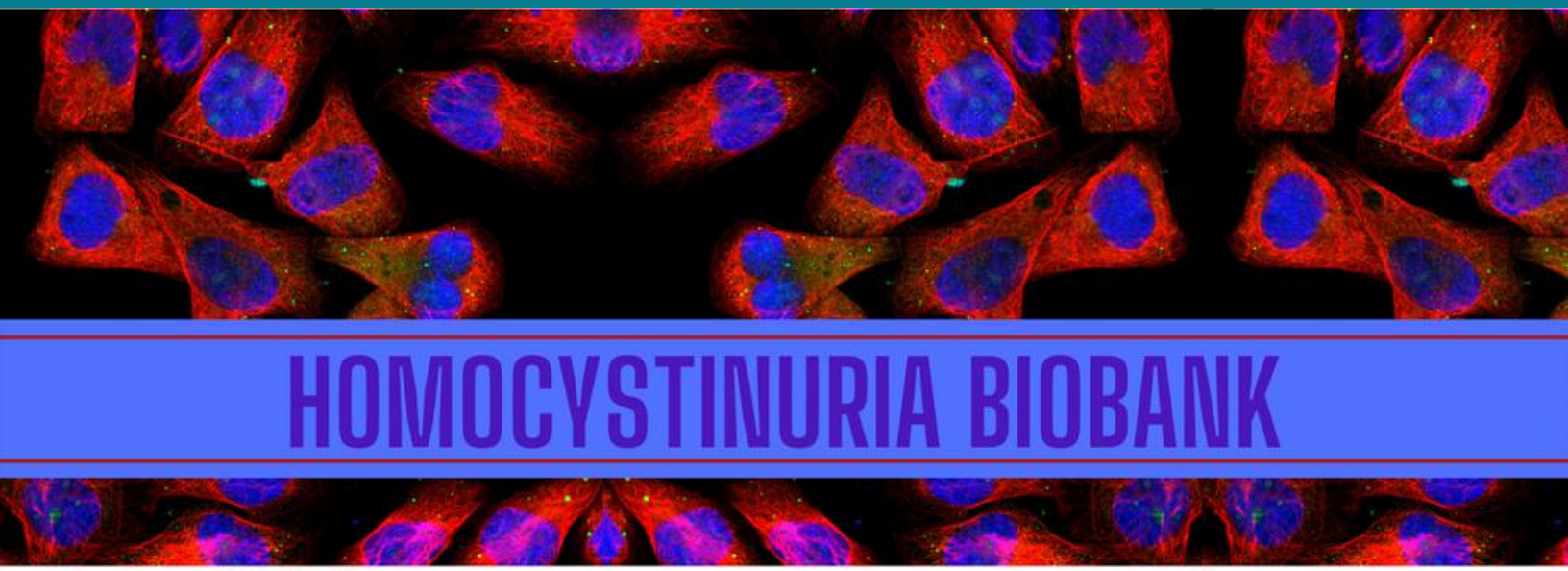
Join us Saturday, October 9, 2021 at 11 am Central time (US/Canada) | 5 pm United Kingdom While Dr. Andrea Bordugo of University of Verona Italy shares his center's experience on administering hydroxocobalamin by a subcutaneous catheter device. Some patients/caregivers may have heard of having a port installed for injections, and this is that concept that will be reviewed.

Register now: https://www.eventbrite.com/e/167915442609

Join us Saturday, October 30, 2021 at 10 am Central time (US/Canada) | 4 pm United Kingdom to learn about the Data Collection Program the HCU Networks will be launching a this fall. Your participation in the Data Collection Program is one of the most important and critical contributions you can make. Patient reported outcomes can accelerate research and the development of future treatments and cures.

Register now: https://www.eventbrite.com/e/homocystinurias-data-collection-program-informational-webinar-tickets-183723641357

HELP DRIVE RESEARCH AND AWARENESS



HCU Network America

Coriell Institute

What is a biobank?
A cell repository is a biobank that stores cell lines. To make a cell line, human cells are taken from a blood or tissue sample and placed in a container with a growth solution. This process is called cell culturing. The cultured cells can then be “harvested,” frozen, and stored. The medical and family history of the sample donor is also collected. Scientists can access banked cells and use them for a variety of purposes, including: finding new genes, studying how cells function, and developing new ways to diagnose, treat and possibly prevent genetic diseases.

Participating in the biobank
Participating in the Coriell biobank allows scientists around the world with a unique opportunity to learn more about homocystinuria and how the specific mutations influence the course of the disease.

Learn more:
https://www.coriell.org/1/NIGMS/About/Information-for-Patients

Join the Program
Participating in the biobank means you are contributing to understanding homocystinuria, which paves the way for scientists to test potential drug compounds or gene- or cell-based therapies. Testing candidate drugs or therapies using cells from individuals with rare genetic diseases can help scientists determine which approaches are most likely to work and be safe enough to test further in human clinical trials. To participate email nigms@coriell.org. or follow the link below.

Participate:
https://www.coriell.org/1/NIGMS/Affected-Families-Donate-Here/Donor-Portal-Home

Coriell Institute
FOR MEDICAL RESEARCH

https://www.coriell.org/1/NIGMS/About/Mission-and-Organization

VMP, LLC
expanding genetic horizons...

Voice: 404.793.7800
Fax: 866.744.5665
www.vmpgenetics.com

HELP US TEACH PHYSICIANS/ HEALTH PROFESSIONALS ABOUT AMINO ACID DISORDERS

THE PATIENT-TEACHER REGISTRY HAS GONE LIVE!!!

(https://www.vmpgenetics.com/edu-services/patient-teacher)

We are proud to announce that the Patient-Teacher Registry website is now up and operational! The Registry is a resource intended for educators and clinicians who want to enhance their teaching about genetic metabolic disorders by including the patient voice and perspective through live presentations… in person or remote.

So far, we have 41 speakers fully enrolled in the registry representing 19 unique diagnoses. These individuals wish to participate in the teaching process. They have medically confirmed diagnoses, or they are the parents/family members of patients. They are seasoned speakers (having spoken to public audiences before) or have been coached to speak in a story-telling format to better engage an audience. **Thirteen have already presented their stories to a variety of medical audiences!**

WE ARE LOOKING FOR MORE!

- ***Patients and/or family members who are interested in telling their stories in a medical classroom setting…*** If medical educators are looking to include the patient story in a teaching session, the Registry will connect the educators and the "patient-teachers."

If you or a loved one have been diagnosed with amino acid disorders, we are always accepting new speakers! If interested, please complete the registration form and consent form (https://www.vmpgenetics.com/edu-services/patient-teacher) and submit them to Jacob Athoe at PatientTeacherRegistry@vmpgenetics.com

Please help us in our efforts to raise awareness about amino acid disorders through this innovative educational outreach to the medical and health care communities. For more information about this project, please contact Jacob Athoe at PatientTeacherRegistry@vmpgenetics.com

Mark Korson, MD
VMP Genetics
Director of Education

Jacob Athoe, BA
Boston University
Biochemistry and Molecular Biology








COBALAMIN STEERING COMMITTEE

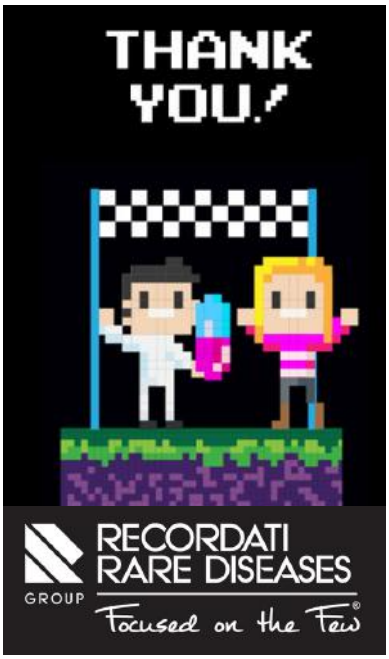
Representing Cbl C, F, G - with hopes of E, J and X to join us!

JOIN US!

HELP DRIVE THE FUTURE OF COBALAMIN DISORDERS

LEARN MORE ABOUT THE COMMITTEE AT:
HTTP://BIT.LY/HCUNACBLSC
OR EMAIL: BPARKE.HCUAMERICA@GMAIL.COM

CLICK CAMPAIGN RECAP



Click Campaign Recap

September 23 kicked off our 4th annual Homocystinuria click campaign sponsored by Recordati Rare Diseases. For every "click" Recordati Rare Diseases donated up to $5 (up to $5k) to HCU Network America. This was an exciting year for us, we not only met our goal, but surpassed it! **Last year we had 639 clicks, but this year we had 1,561 clicks. That's more than double compared to the year before.** We'd like to thank Recordati for partnering with us to raise awareness and funds for the Homocystinuria community

GO THE EXTRA MILE FOR HCU, RACE RECAP

Thank you to the 147 racers and countless people who donated to make our third virtual race a smashing success! The first week started off very strong. We had 720 miles logged and 3 racers cross the finish line! First place in our cycling division went to Nannette Bradley of Team Recordati, and in our running division first place went to Allison Trucillo of Team Aeglea, and second place went Shane Petrelli and In addition to the strong lead by racers, teams Race with C & G, Mighty Marchese's, Grayson's Gang, Brooke's Blazers, Carson Crusaders, the Bartke Ruff Ruffs, Ellie's Entourage, Rare Runners, Recordati, Aeglea. Codexis and Team Will for HCU had already almost met our fundraising goal and had collected $18,418 in donations!

As week two started, the momentum picked up and racers logged another astounding 1276.06 miles! As the competition began to increase, we started to see racers cross the finish line and 19 more racers crossed the finish line. As week two came to a close teams Codexis and Team Will for HCU added over $300 each to our fundraising goal, with Ellie's Entourage not far behind, for a total of $19,663.

The second leg of the race many new racers entered the leaderboard and 885 miles were added to the total completed. With that extra competition another 18 racers crossed the finish line. With only 40 of 147 registered racers having crossed the finish line, those watching the race became slightly skeptical if everyone was going to finish with so many mile left completing.

With only a few days left to go, our racers picked up their pace. In the end, 102 racers logged over 4,684 miles and 68 crossed the finish line. With time ticking, donations continued to come in making for a final total of approximately $26,878! This truly was an amazing event. Thank you to all our amazing racers and donors for making this an exceptional event!

MEET OUR FABULOUS FUNDRAISING TEAMS!

Race with C & G - $8,410

Colin (3.5) and Garrett (1) Bicklemann were diagnosed earlier this year with Classical HCU by a genetic test administered by their doctor for an unrelated reason. As a teacher and the boys' mother, Angela wanted to learn as much as she could about HCU. Dr. Google was very unhelpful and anxiety-inducing! Even the boys' doctors lacked confidence in the answers to her questions. HCU Network America provided the family with a wealth of information, resources, and patient success stories. Angela pushed the stroller 26+ miles to raise money for awareness, research, and to support this important organization that provided her family with comfort and answers in the early months following diagnosis. They are filled with gratitude for the outpouring of support from friends and family in this fundraiser.

Team Ellie's Entourage - $3,650

Ellie's entourage walked on behalf of 3-year-old Ellie who was diagnosed with Classical HCU at 10 days old. She was diagnosed through newborn screening and spent 10 days in the NICU. Since then, her disease has been maintained though diet, except a few times where illness's led to short hospital stays. Walking to raise awareness means more research and better treatment options for a better future. Not only are we walking for Ellie, but Ellie's adult cousin, Craig, has been living with HCU for almost 30 years. Better treatment options for both would ultimately mean a healthier life free of complications.

Team Will for HCU - $3189

Chris and Tara Hummel tag teamed to raise money for Team Will for HCU. Chris has run a few marathons in the past, so he was happy to have a month to run 26.2 miles instead of only 4 hours. Tara is a new runner, but did a great job getting her miles in! Both Chris and Tara gain motivation and inspiration from their son Will, who has HCU. He is such a trooper with regards to his diet and treatment. It would be easy for him to say he's had enough, stray from his diet, and give up. However, he never does. Chris and Tara try to duplicate that mentality while running... just keep going even when you want to stop. We've learned that where there's a Will there's a way! Thanks to all our friends and family who have supported us this month on our runs!

Team Codexis - $1,975

Team Codexis is a group of 64 passionate employees and their families. Codexis is a leading enzyme engineering company dedicated to the discovery and development of novel biotherapeutics and high-performance enzymes. Codexis is proud to support the Homocystinuria community, as well as other rare disease communities, through action. Our goals are to build disease awareness, improve quality of life for patients, and discover better treatments. For more information, visit: https://www.codexis.com/therapeutics/

The Bartke Ruff Ruffs - $1,150

Danae' and Garrett were diagnosed with classical homocystinuria in 1995 at the ages of 10 and 5 after Garrett's lenses became dislocated. For years they struggled with the low protein diet and dealing with issues related to non-compliance. In 2009, after Danae' suffered a blood clot they were connected to the PKU Organization of Illinois and Allied Disorders, where they were invited to a low protein cooking classes and educational events. Danae' loved the support and got involved with their organization. Her time volunteering lead her to realize the gap in support for the homocystinuria community. In 2016, with Margie McGlynn at her side, they co-founded HCU Network America. Danae' feels fundraising is a way to give back to the community that has provided so much for her.

Mighty Marchese's - $1,615

Aimee was diagnosed in 1997 at age 8 with HCU after having spent years bouncing between specialists and being misdiagnosed with Marfan syndrome. She has had an uphill battle with compliance all her life. Only over the past year and a half she has finally been able to take control of her low protein diet with the help of her new husband and supportive family. The HCU network has been an important community that helps her push through tough days. Aimee and her family walk for a brighter future for adults and newborns with HCU and are grateful to the scientists and doctors working to make it happen.

Grayson's Gang - $934

We were excited to participate as a family in the Race for Research again. This is our second year running 26.2 miles each during the month of September. It was a tad bit tricky this year with school schedules, but we were proud to have the opportunity to raise awareness and funds for HCU Network America. Grayson is one tough little boy. He inspires our whole family. It was fun to have him join us in the running this year. He wants to keep up with his sisters so he didn't let anything stop him from running with them too. Every mile we log during this race reminds us of how far medicine has

come in just 10 years since the birth of our first son, Drew. Drew passed from undiagnosed Cbl G at 3 1/2 months. We look forward to seeing the amazing advances in science and technology in the next 10 years to improve Grayson's life and all the wonderful families we have met since being involved with this wonderful organization.

Mill Creek Early Childhood Program - $500

Mill Creek Early Childhood Program was so excited to support one of our families and join the fundraiser! The two-year-old class was especially excited to be a part of the "big walk." They sat down together and decided what their walking goal would be. The children had recommendations from one street all the way to 400 miles. After some discussion the class decided to set their goal at 4 miles. Every time the class set out on a walk around town, they were so excited to color in the miles they walked when they got back to the room. The class surpassed their four miles goal! Those tiny legs walked many miles all around town. We were so thankful that our little class could bring some light to this great cause! We never thought we would raise the money we did but are so thankful we passed our goal many times over!

Team Recordati- $770

Team Recordati Rare Diseases is a team composed of 34 of their current employees and 4 former employees. Recordati Rare Diseases is a biopharmaceutical company committed to providing often overlooked orphan therapies to underserved rare disease communities of the United States. Our experienced team works side-by-side with rare disease communities to increase awareness, improve diagnosis and expand availability of treatments. Recordati Rare Diseases is proud to support HCU Network America and the Homocystinuria community. For more information, please visit www.recordatirarediseases.com

Team Aeglea- $535

Team Aeglea proudly participated in this year's Race for Research to bring awareness and show support to the HCU community. Consisting of 39 employees and completing a total of 1316 miles throughout the month of September, Team Aeglea raised $535 !

Aeglea BioTherapeutics is committed to helping people with rare and devastating metabolic diseases like HCU who have limited treatment options - because having a rare disease doesn't mean that you are in this fight alone. We are building a pipeline of novel therapies designed to modulate the chemical imbalances that drive disease. By leveraging our unique protein engineering expertise to develop human enzyme medicines, we aim to improve lives today and create hope for new tomorrows.

To learn more and to check out our Patient & Families resources, please visit: www.aeglea.com

Carson's Crusaders - $210

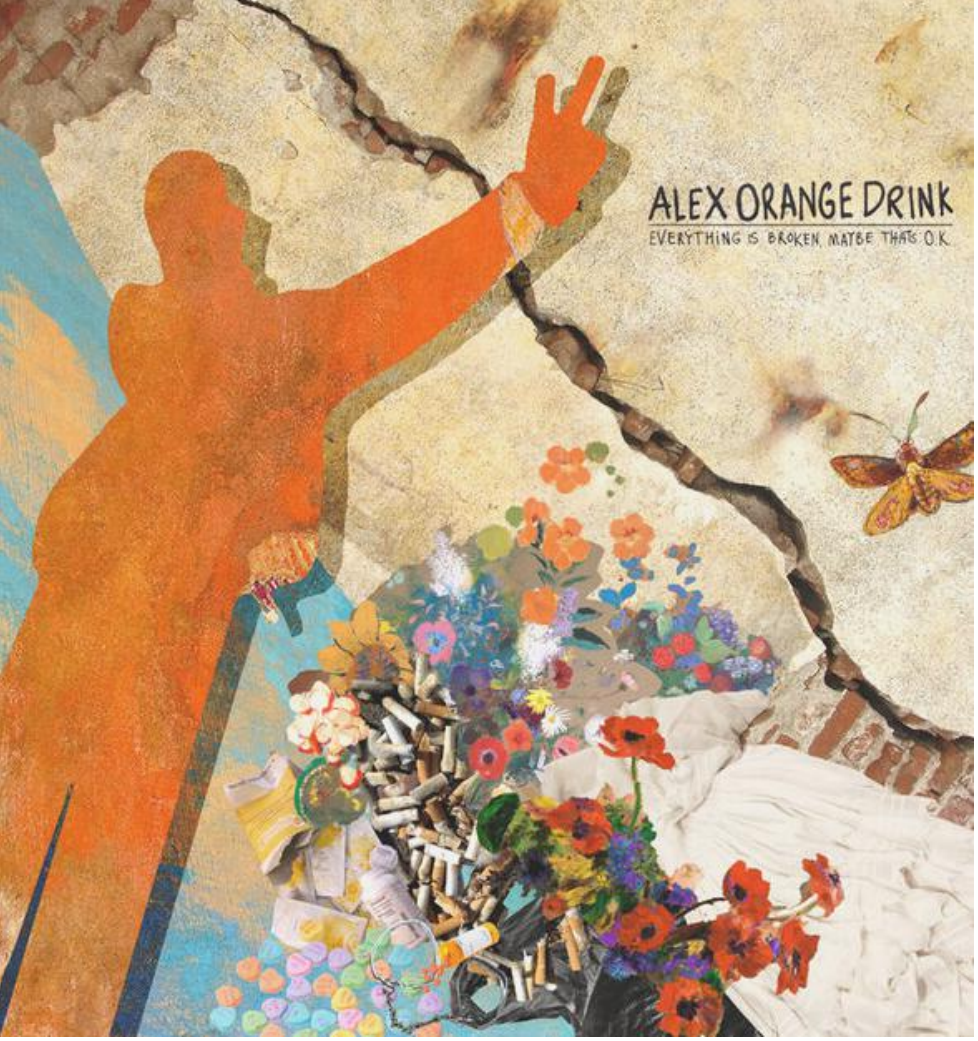
Carson was diagnosed with Homocystinuria due to severe MTHFR deficiency through newborn screening. This is a rare genetic condition which is currently incurable. There are only about 50 documented cases worldwide. Since not all states test for this condition, most children are diagnosed later in life after symptoms have developed. Early diagnosis leads to better outcomes.Our hope is to raise awareness for our daughter's condition and raise money to go towards research.

Other Fundraisers of $100+

Brooke's Blazers - $2,650

The Rare Runners - $250

South Tampa Strides $100



But I think I'm a stupid optimist where I think I can't do that; I can do this. Maybe I'm not supposed to be a classically trained musician, but a punk rocker and that's what I felt anyway. It was the thing that made me feel like I didn't need to bounce off the walls. I've learned to embrace my insecurities.

Letting the diagnosis inspire you:

Tell us about adopting your moniker - Alex Orange Drink

Alex Orange Drink comes from my formula; orange drink is the slang. I always called it that because it was this disgusting orange flavor. I felt like this was taking a negative and switching it into a positive, and making it this super power, super drink, for this unique rock star who has this power. Inspired by the hip-hop mentality, which tends to celebrate and embrace its struggle.

What influence did HCU have on your new album?

Homocystinuria part 1: 1994-1997 – is all about living with this disease, then feeling like an outsider/outcast. It is all about checkups at the hospital, taking trips, feeling different at birthday parties, and the inspirations of artists that could relate to me and helped me get through this period. Those artists made me feel safe, it was my medicine.

Regardless if you want to think about it or not, you are always reminded of this thing, your mortality, every time you sit down to eat, every time you go out, every time you are with friends. Food is a connector, but in

our case, it was a reminder of feeling like an outcast or a bit different. It's not something to glamorize, but it helped me find my voice artistically, and fit into a timeline of rock and roll.

To those who would like to use their talents to advocate and raise awareness, what advice do you have to give?

I've learned through a lot of other heroes and inspirational figures that the thing you think is holding you back, is something you should embrace and be proud of and will give you a special voice and be your savior too. There are a million ways to learn, a million ways to triumph, and a million ways to fail, and it's all fine - they don't teach you that.

How can our audience keep up with Alex Orange Drink or the So So Glos?

Alex Orange Drink:

- Instagram: @alexorangedrink
- Facebook: @alexorangedrink
- Soundcloud: alexanderorangedrink
- Spotify: https://open.spotify.com/artist/6MjoFNppz6Tm6qCdHrzcvx
- Bandcamp: https://alexanderorangedrink.bandcamp.com/
- Website: alexorangedrink.com

So So Glos

- Facebook: @sosoglos
- Instagram: @sosoglos
- Spotify: https://open.spotify.com/artist/3WrUzXz7uxtNFZuapRMhiP

Alex is currently selling unstructured, snapback hats, hand screen-printed to help raise awareness and funds for Homocystinuria. 20% of all proceeds will go to HCU Network America. Contact Alex via Instagram DM, or email to order.

*Often we share information from our Network Affiliates - this information is not an endorsement for the product. Consult your medical team to make sure it's right for you.

NUTRICIA

Try Low Protein Spaghetti Puttanesca for Dinner!

Spaghetti alla Puttanesca

Makes 2 servings.

Ingredients:

- 1 Tbsp extra virgin olive oil
- 1 garlic clove, minced
- 8.5 oz (240 g) canned crushed tomatoes
- Salt and pepper
- ½ tsp of crushed chili flakes
- 1 Tbsp chopped parsley
- 1 tsp dried oregano
- 200 g Loprofin Spaghetti
- 1 Tbsp capers, chopped
- 4 black olives, pitted and sliced

Method:

1. Heat a little oil in a frying pan. Saute the garlic until it is just starting to brown.
2. Add the tomatoes, salt, pepper, chili flakes and cook the mixture over medium heat for 3–4 minutes until the tomato liquid is slightly reduced.
3. Lower the heat, add the parsley and oregano and simmer the sauce gently for 20 minutes.
4. Cook the Loprofin Spaghetti as per packet instructions.
5. Add the capers and olives to the pan.
6. Toss the cooked Loprofin Spaghetti into the sauce. If the sauce appears dry, add a splash of water from the pasta pot.

Nutritional Information	Per Recipe	Per Serving
Calories	1008	504
Protein	6.2 g	3.1 g
Phenylalanine	168 mg	84 mg
Leucine	257 mg	129 mg
Tyrosine	95 mg	48 mg

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