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AmazonSmile customers can now support HCU Network America in the Amazon shopping app on iOS and Android mobile phones! Simply follow these instructions to turn on AmazonSmile and start generating donations.

1. Open the Amazon Shopping app on your device
2. Go into the main menu of the Amazon Shopping app and tap into 'Settings'
3. Tap 'AmazonSmile' and follow the on-screen instructions to complete the process

If you do not have the latest version of the Amazon Shopping app, update your app. [Click here for instructions.](#)

HCU COMMUNITY COOKBOOK

LET'S GET COOKING FEATURING RECIPES FROM NUTRICIA COOKING CLASS

See the recipes at: <http://hcunetworkamerica.org/hcu-community-cook-book/>





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HCU Lophlex LQ is a medical food for the dietary management of homocystinuria and must be used under medical supervision. Loprofin pasta products are medical foods for the dietary management of inborn errors of metabolism and must be used under medical supervision.

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July 2021: HCU and You: *Ask Methia*

Dear Methia,

Leaving on a Jet Plane, and the Panic is Setting In!

I just accepted my dream job! I could not be more excited, but I am starting to stress about one of the requirements of my new position: Domestic travel. I will be traveling to different sites for up to 10 weeks throughout the year. I will be able to drive to some of these locations, but others are across the country, and I will have to fly. I've never stressed about traveling before, but I have started to see my HCU geneticist and dietitian again and have never been more compliant with diet and medications than I am right now. Do you have any travel tips to help me manage my HCU while I make this transition?

Sincerely,
Jet Setter

Dear *Jet Setter*,

It's true – returning to diet and treatment, while it's absolutely the best thing for you, introduces a new set of considerations! I have good news for you: THERE ARE SOLUTIONS! With developments in security, packaging, and electronic prescriptions, traveling with necessary supplies is much easier than it used to be. Here is a “checklist” of things to consider when preparing for your next trip:

- ✓ **Formula/low protein foods.** If you are checking a bag, it is easy to check dry low protein foods before you go through security. The same is true for your formula, whether it be powder or liquid, but you will want to carry some formula on the plane with you in the event your luggage is delayed (or lost!). Powder sachets/cans (opened or sealed) and liquids that you attempt to bring on the plane with you may face some scrutiny by airport security. Ask your clinic to write you a travel letter explaining why you have to carry these products on the plane with you. They should include the airline you are flying, the date of departure, and the date of return, along with your specific formula and how much you are prescribed. Many patients have been successful in bringing their formula on the plane with these types of letters.
- ✓ **Emergency letter.** In the event you are sick while traveling and need to seek emergency medical care, it's a good idea to carry your emergency protocol that your metabolic clinic has prepared for you. This will include an explanation of your diagnosis, proper labs to draw in the event of an emergency, and how to reach your clinic for the next steps.
- ✓ **Medication storage and preparation.** If you take hydroxocobalamin injections (B12) and are traveling, this should be approached the same way as formula and medical foods: With a letter written by your clinic. You should pack your liquid and syringes (or prefilled injections) but also carry some on the plane with you in the event of lost luggage. In the event something happens to your medications while you are gone, your clinic may be able to call in to a local pharmacy for assistance. Communication is key!
- ✓ **Meal preparation...** and anticipation! If you are prescribed a low protein diet, meals while traveling can definitely be challenging. Research local restaurants or fast-food locations close to where you are staying so you can plan ahead. If you have a kitchen or kitchenette where you are staying, think about meals you will be eating on your own and walk or Uber to the nearest grocery store ahead of time.

This checklist is great to reference before any type of travel and be sure to check in with your clinic for any additional suggestions!

Sincerely,
Methia

Traveling with HCU

A REVIEW OF TRAVEL TIPS | TRICKS | EXPERIENCES

ABOUT ME

Danielle B. | Winter Park FL

My name is Danielle and I have Classical HCU B-6 Non-Responsive. I was diagnosed through Newborn Screening at 10 days old. My treatment plan consists of HCU Lophlex LQ formula, Cystadane (Betaine), Folic Acid, B-Complex and Aspirin. I live in a suburb of Orlando, FL with my husband, Irving Baez, 3 children (Irving, Julian, Christian), and 3 mini schnauzers (Mango, Bruce, Chichi). Lastly, we **LOVE** to travel!



Nassau, Bahamas (2018)

AIR

Tip: Never Check Medicine!



Many airlines allow a medical bag as an additional carry on, free of charge, as long as the bag does not contain any other non-medical items. While this is a great offer, I actually have never tried it! For air travel, I use a backpack and roller-board. If questioned, I pack my purse in my backpack which satisfies the 1 overhead/1 under-seat limitations. Formula is packed in plastic bags and when time for TSA I pull out the plastic bags filled with the formula and place in the TSA "bin". Normally, 4oz liquids do not get pulled. Due to the amount I carry, that rule usually does not apply with HCU travels.

LAND

Tip: Insulated sandwich bags



Having traveled by Amtrak, North-South Road trips, Ubers from the city to the jungle, insulated bags/sandwich bags have been the biggest formula help. They keep food fresh and pre-mixed formula cold! Pairing with my hydro flask I have no worries maintaining formula. I also bring along snacks such as dried/fresh fruit, Original Pringles (to help with altitude changes), and granola bars. Keeping it lo-pro on the road ensures room for a good dinner to celebrate at your final destination.

SEA

Tip: Bring the formula and fun!



Cruising is by FAR the easiest experience. Use extreme detail when making your reservation. I have found referencing PKU has helped a lot. I have never brought lo-pro food on a cruise and have always eaten like a **QUEEN**. I have had my passport flagged and had to prove I had enough medication to sail plus emergency..

TRAVEL PREP TIPS



Travel Hacks

- When traveling internationally, now I bring enough medication to cover an additional 5 days in case I must quarantine and need to have more shipped to me.
- Have a comfortable backpack that can fit underneath an airplane seat. It usually is your backpack that goes with you everywhere
- Check your levels before you travel. I like doing this, so I know where I stand in case any emergencies arise.

TOP TRAVEL EATS



Explore through food!

- 1/4 cup rice + mixed veggies is my "safe meal" for all countries.
- Modify/mix-match menu items
- Eat local, it saves and usually provides fresh adaptable options
- Caribbean Favs: Local fruit, Plantains, Zucchini Boats with salsa/sauces
- Central American Favs: salads with salsa, vegetarian Casados, yuca fries
- African Favs: Fufu, curries, okra dishes
- European Favs: Eggless Crepes, Saffron rice

FUN MEMORIES



Traveling with Cobalamin C

AN INTERVIEW WITH ADAM SETTLE

ABOUT ADAM

Adam is a 21-year-old from the Harrisburg suburbs of Pennsylvania. Adam comes from a big family, and despite being the youngest, he is the only one to have been diagnosed with Cobalamin C deficiency. Adam was fortunate to be one of the first children diagnosed through the state's newborn screening program. Despite his being legally blind, and having Cobalamin C, those who know Adam would describe him as outgoing, compassionate, adventurous, and determined.

In the book about Adam's life - No Day Wasted, the Adam Settle Story his mom summed him up in a way I think fits Adam perfectly.

"With his desire to be normal, to overcome, to not be hampered by his medical condition, Adam wanted to try everything. He got hurt along the way, but that did not slow him down. No matter how many bumps or bruises Adam endured, he quickly bounced back, ready for the next new experience.... God made it very clear to me Adam was not to be boxed up or bubbled up. He is here for a purpose, and we need to let him go."

This drive and desire to be normal and to overcome is shown in all aspects of his life, especially in his desire to travel. In Adam's book, you read a lot about the adventures he has been on with his family - Canada, Cambodia, Africa, Italy, Florida, Dubai - he is quite the traveler! Recently though Adam took his first solo trip to Indiana, so we sat down with him to catch up.

Prior to your trip did you have any worries about traveling alone?

Yes! I've always had someone with me and this time there was not my family to depend on. I was very worried about how I was going to get my B12 shot, since my vision doesn't allow me to fill the syringe and I don't feel comfortable (also because of my vision) giving myself the shot. My dad is typically the one who fills the syringe and gives it to me.



Since you are traveling alone now, how does that change how you plan and prepare for your trip?

My parents helped me make sure I have all my medication ready to go. My dad prefilled the syringes of my B12 for the trip, typically the syringes are not prefilled and the B12, syringes and plunger are all packed separately, but in the same bag. Once all my meds are packed, they go into my backpack that I carry on with me. This makes it easier if they need to look at my medications.

Your bags are packed, you're ready to go and now you are at the airport. Talk to me about navigating the airport - what tools are helpful and what assistance from people do you need.

I use an app called [Aira](#). Unfortunately, the Indianapolis airport is not partnered with them, and I didn't have the paid version, which would have let me have full access at this airport.

I almost got turned around in the airport. After I got Security, I asked them where to go and they told me to go left and take the stairs. I had my backpack on, my dog in my left hand, and my luggage in my right and started down the stairs - but the stairs had two sets down (with a platform in between). When I got to the platform, I had to ask someone else if they knew where I should go. I ended up using the free version of Aira which gave me about 5 minutes which got me close to my gate. I then was able to ask someone who worked at the airport where my gate was, and I was only a couple gates down from where I should be.



Coming back through the airport at Indianapolis, my friend's husband was able to get a pass at the luggage check area to come through security with me and help me get to my gate. I realized after we got to the gate, it was the same gate I came in on. If I would have known that it would have made it easier for me to navigate on my own since I had already been there.

How do you handle your medication needs once at your site, specifically your injections? Did you have any unanticipated obstacles?

I was lucky this time around that the friend I was visiting is a veterinarian and she could give me my shot. If she didn't, I would have needed to find someone or someplace that could. Everything went very smooth when I was visiting them.



What are your future plans for travel?

This October, I'm going overseas with my dad. I would never go by myself overseas - I'd either go with my dad or with a team of people. There is a lot more to navigate overseas with the cultural and language differences.

I'll be going back to Indianapolis in September by myself and going to Houston. When I go to Houston, I will need to get my syringes prefilled again - either by my dad or a pharmacy and I will need to find a clinic or pharmacy that can administer them to me.

Would you change anything in your future travels?

In the future I plan to upgrade to the full version of Aira so it's easier to navigate other airports. People were so friendly at both airports that I will likely be even more comfortable to ask others to help me if I need assistance.



How can people follow your adventures?

They can check out my website:

- <https://adamsettle.wordpress.com/>

Or follow me on instagram:

- @adamrobertsettle
- @adam_art__settle

SUPPORT EVENTS

To learn more or register, visit: <https://www.eventbrite.com/o/hcu-network-america-30163980100>



HCU Community Virtual Meet-up

Online meet-ups are an opportunity to connect patients and caregivers impacted by homocystinuria to one another virtually.

- Struggling with the diet and formula?
- Feeling in a food rut?
- Don't like your formula, or having trouble getting it covered?
- Having health issues you aren't sure are HCU related, or just part of being an adult?

Come join us

Sun., 7/11 @ 2 pm CT | 3 pm ET | 8 pm UK
and

Mon., 7/26 at 1 pm CT | 2 pm ET | 7 pm UK

HCU Kids Meet-Up

Patients, ages 7 and up with HCU, are invited to a zoom meet-up with familiar and new friends. We will have time to chat and play some fun games!

This meeting is being led by peer leaders with HCU.



Patients, ages 7 and up with HCU are invited to a zoom meet-up with familiar and new friends. We will have time to chat and play some games!

Come Join us!

July 12 at 2 pm CT | 3 ET

Classical HCU Parent-Caregiver Meetup

Parents, Grandparents and Caregivers of "kids" all ages with HCU need support too!
August 25, 2021 at 7pm CDT

Fall means back to school! Preschool, K-12, After School Care and College. No need to worry or fret, we are here to support you! Come chat with other parents about this transition and what has worked and hasn't worked, and learn what resources HCU Network America has to offer.



Fall means back to school! Preschool, K-12, after school care, or College, no need to worry or fret, we are here to support you!

Come chat with other parents and caregivers about this transition and what has worked, hasn't worked and learn what resources HCU Network America has to offer!

Come join us

Wed., 8/25 @ 5 pm PT | 7 pm CT | 8 pm ET

To learn more or register, visit: <https://www.eventbrite.com/o/hcu-network-america-30163980100>

LET'S GET COOKING

Cooking Demos for the Homocystinuria Community
with Chef Amber Gibson



Free low protein samples to complete recipes!

*Samples only available to those in the US

We encourage children with HCU to also participate in this event!

Cambrooke

Sunday, July 25
1 pm EDT

Deadline to register:
July 5th, 2021

Flavis

Sunday, August 22
1 pm EDT

Deadline to register:
August 4, 2021



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

NAVIGATING THE FUTURE OF ALL HOMOCYSTINURIA PATIENTS!

Exciting things are happening in research for the homocystinuria community!

HCU Network America and HCU Network Australia established a research grant program and a Scientific Advisory Board (SAB) in 2017 to identify and advise on research priorities for classical homocystinuria (CBS deficiency) and now has expanded to include other homocystinurias (including classical homocystinuria, select cobalamin disorders and severe MTHFR). To date, we have awarded two research grants: the first in 2018 and the second in 2020. The research periods have ended and the data from the first grant was shared recently at [The National Human Genome Research Institute \(NHGRI\)](#) and the [National Center for Advancing Translational Sciences \(NCATS\) virtual meeting on June 24-25, 2021](#).

This summer we are gearing up to issue our third call for Expressions of Interest (EOI) to identify a small number of potential researchers whose programs will be considered for grants and invited to submit a full Grant Application.

The areas in which these expressions of interest are focused include:

- New therapies to treat classical homocystinuria and remethylation disorders by exploring novel mechanisms to obtain proof of concept to enable progression to clinical trials
- Technologies to improve early detection of classical homocystinuria and remethylation disorders, particularly via primary markers for newborn screening

How can we ensure this happens?

The past two grants we have issued were \$40,000 USD (each). To continue this momentum, we need to call upon our community to help us fundraise to make this happen! This September 100% of the funds raised during our [Race for Research](#) will go towards the priorities set forth above. By fundraising, you can ensure that these priorities can be met. In addition to this – you can decide if your funds go towards classical homocystinuria, cobalamin disorders or severe MTHFR.

After registering for our [Race for Research](#) and setting up your fundraiser – reach out to our Executive Director, Danae' Bartke and let her know which area you'd like them to be used.





Virtual Race

Benefiting HCU Network America

Walk, Run, Ride | September 2021

What is a virtual race?

A virtual race is a race that can be walked, ran, or biked from any location you choose. You can participate on the road, on the trail, on the treadmill (or stationary bike), at the gym or on the track (or even at another race). You get to run your own race, at your own pace, and time it yourself. You do not have to complete the miles all at once, in one day, or even a week. You can use the entire month to complete the race.

How do my miles translate to money raised?

After a racer is registered, they are set up with their own personal donation page. You can direct those who would like to donate to your race link.

How do you know how many miles I completed?

- We rely on the honor system. You don't have to use a device to prove your miles.
- If you'd prefer to use an app to track your miles, we recommend Strava. You can join the HCU Network America Club.
- Please use intentional miles – this means no step counting
- Please log all your miles by 11:59 pm ET September, 30, 2021.

Learn more or register at <https://runsignup.com/Race/IL/Batavia/HCURaceforResearch>

WANT TO LEARN MORE ABOUT WHAT IS HAPPENING FOR HCU?

Join us for these exciting webinars!

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



Progress on Gene Therapy for Classical Homocystinuria (Cystathionine Beta-Synthase)



Informational Webinar

Tuesday, July 13 @ 7 pm ET

Warren Kruger, PhD
Fox Chase Cancer Institute



Roadmap to Innovation for HCU

Driving Data to Drug Development

August 28, 2021 - 10 am CDT

Natural History Studies

Clinical Trials

New Approved
Therapies

Patient Registries

Join us for a one hour webinar on:

- Patient Registries
- Natural History Studies
- The Drug Development Pathway
- Industry Updates from Aeglea BioTherapeutics and Travele Therapeutics
- Perspectives on Clinical Trials

Aeglea BioTherapeutics Doses First Patient in a Phase 1/2 Clinical Trial of AGLE-177, a Potential First-in-Class Treatment for Homocystinuria

June 24, 2021

AUSTIN, Texas, June 24, 2021 /PRNewswire/ -- Aeglea BioTherapeutics, Inc. (NASDAQ: AGLE), a clinical-stage biotechnology company developing a new generation of human enzyme therapeutics as innovative solutions for rare metabolic diseases, today announced the dosing of the first patient in the Company's first-in-human Phase 1/2 clinical trial investigating AGLE-177 for the treatment of Homocystinuria. The markedly elevated plasma homocysteine levels lead to a wide range of life-altering complications and reduced life expectancy in people with Homocystinuria. AGLE-177 is a novel engineered human enzyme therapy designed to lower the total level of homocysteine in the plasma. Aeglea anticipates providing a clinical update on the program prior to the end of 2021.

"AGLE-177 is designed to address the markedly elevated homocysteine levels that are believed to be a key driver of the risk of serious complications in Homocystinuria, and has the potential to transform management of this burdensome and life-threatening condition," said Anthony Quinn, M.B Ch.B, Ph.D., president and chief executive officer of Aeglea. "We believe this clinical trial will provide us with proof of concept of the homocysteine lowering effects of AGLE-177 in patients and provide us with the information needed to transition rapidly into a Phase 3 registrational trial."

Cystathionine beta synthase (CBS) deficiency, also known as Classical Homocystinuria, is an inherited disorder of methionine metabolism that results in elevated homocysteine and homocystine in the plasma and urine. Disease management strategies, which are limited and include dietary protein restriction with amino acid replacement either alone or with vitamin B6, and betaine supplementation, are challenging, have poor adherence and many patients are not responsive to treatment. It is estimated that there are more than 30,000 people living with Homocystinuria in the global addressable markets, which includes approximately 15,000 to 18,000 B6-non-responsive patients.

"Untreated Homocystinuria is a severe disease as a result of which individuals can develop significant complications, including visual impairment, skeletal abnormalities, reduced intellectual capacity, and recurrent thromboembolic events which may be life-threatening," said Principal Investigator Dr. Elaine Murphy, Charles Dent Metabolic Unit, National Hospital for Neurology and Neurosurgery, London, UK. "Currently available treatment options are complex and do not always result in optimal metabolic control of the condition, leaving patients at risk of lifelong complications. There is an unmet need, therefore, for new therapies that can improve the outcomes and quality of life for this patient community."

Read the full press release here: <https://www.prnewswire.com/news-releases/aeglea-biotherapeutics-doses-first-patient-in-a-phase-12-clinical-trial-of-agle-177-a-potential-first-in-class-treatment-for-homocystinuria-201218772.html>

ADVOCACY IN ACTION AT WORK: THE PENROSE AMENDMENT



Jim and Pamela Penrose

For the past four months my husband, Jim, and I have been struggling with our insurance company over its refusal to cover the cost of my medical formula. I'm sure many of you can relate. We live in Nevada and our state law mandates that health insurers provide coverage for "enteral formulas" when prescribed for metabolic patients. Our insurance plan documents also state that the cost of enteral formula is a covered expense "as mandated by law."

Jim spent many hours preparing appeals to our insurance company regarding its most recent refusal to pay for my formula. He is a retired attorney and did an excellent job of preparing the proper documents, complete with exhibits, only to have the appeals denied. Our last chance was an external appeal with an "independent review organization," coordinated by a state agency. This time we were successful, and the previous denials were overturned.

During this process, Jim had also been contemplating asking the Nevada Legislature to clarify the existing coverage mandate for enteral formulas. Our insurance company (and I am sure many others) chose to define "enteral" as referring only to a formula administered through a feeding tube or intravenously. Since most formulas for inborn errors of metabolism are taken orally, our insurer's interpretation was obviously problematic for patients and – we argued – inconsistent with the statutory coverage mandate. Jim formerly worked at the Legislature as a bill drafter, and so he set about preparing an amendment to the current law, making it explicit that coverage is also required for formulas taken orally. He sent in his proposed language and contacted legislative leaders for help. He also testified in legislative committee meetings in support of the proposed changes. Ultimately, his hard work paid off and his language is now set to become law as of July 1, 2021. We hope that the amendment will help all metabolic patients in Nevada.

To those of you in similar situations, keep fighting! Stand up for what you believe in. You may be surprised at what can happen. As individuals we are rare, but together we can be strong, powerful voices!

Join Pamela and the others in sharing your story about medical nutrition equity at <https://nutritionequity.org/>



RAREARTIST

Submissions due July 23, 2021 at 5 pm.



- Children (4-11) – \$500
- Teen (12-19) – \$5000
- Young Adult (20-30) – \$500
- Adult (31+) – \$500





Live better, together!

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. By registering, you will be able to identify other patients in your state and request their contact information. You will also 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 patients and families with resources, 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/>

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US***

