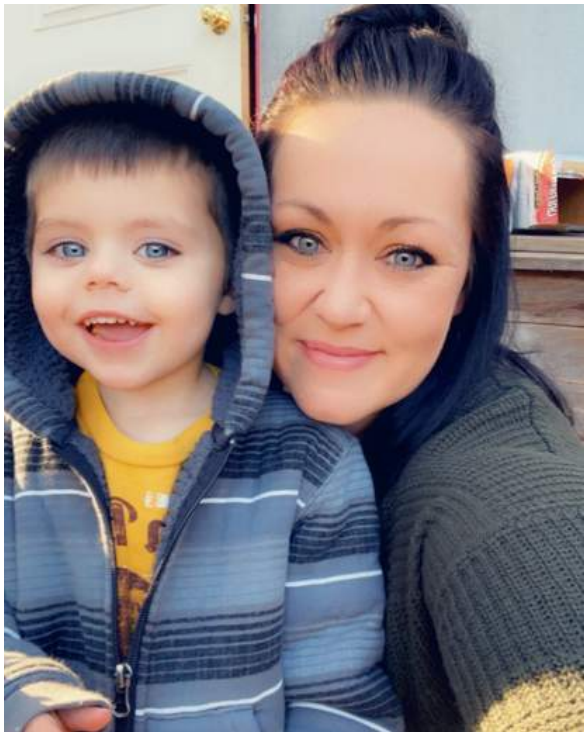
The HCU *Herald*

More HCU News Inside

HCU Hero: Kyler (CblC) from Texas

HCU HERO:
KYLER (CBLC) FROM TEXAS



Kyler was born November 2017 and seemed like a normal healthy newborn. I had no problems during my pregnancy, and he was born at 39 weeks weighing a little over 6lbs. In the hospital he ate just fine and within 48 hours was released to go home. He had his first newborn screening in the hospital; this newborn screening came back normal.

After a few days of being home and based on my experience with my other children, I felt like he slept a lot for a newborn and didn't want to eat much. Deep down I worried, but just blew it off thinking maybe he's just a sleepy baby. He would eat, just not on the normal schedule for a newborn, and most times I would have to wake him to eat. As time passed I kept telling my husband that he's really sleeping a lot and that I was worried. He assured me all was fine, and was probably just my anxiety from having a baby pass away of SIDS in 2011. We had our 2-week checkup and I mentioned he slept a lot and didn't eat as much as I thought. He had gained a little weight so the doctor wasn't concerned.

After this appointment we went for our second newborn screening, which is standard in Texas. A week later I got a call saying his screening came back with something showing signs of a metabolic problem. All they said was I needed to feed him every 2 hours no matter what and our doctor would contact us. I immediately panicked; I had so many questions and no answers. We spent the next week feeding every 2 hours and getting calls from doctors that we need to see a genetics specialist because Kyler's homocysteine levels were high.

His pediatrician had us set up with a genetics doctor within a week, which seemed like a lifetime when your being told something Is wrong with your child. Our first appointment was scary and confusing. They did blood work and told us that it was looking like cobalamin c disease, but could just be a b12 deficiency from pregnancy. So they asked to do the genetic testing and we agreed. To be on the safe side until we found out exactly what it was, they still wanted to treat him just in case because the sooner the better. We were told Kyler would have to take injections daily and medicine by mouth. This was so upsetting to me. I felt so bad, like it was all my fault, and I was broken because I brought a child into this world who would have to go through this. It was very hard on me, but I held it together the best I could. I tried so hard to find information online and just couldn't find much, which made me worry more. What information I was able to find was old, outdated and very worrisome. Once the genetic test came back it confirmed Cobalamin C disease. I cried for weeks knowing this was now our reality and future. I worried so much about his future; not knowing anything about this or knowing anyone with the same thing. Feeling alone in this was the worst feeling because you have no support. Over time I just accepted it and hoped one day I could find more answers.

Kyler is now 3 and a relatively healthy kid. He is delayed in some things; he walked late, talked late and will require speech therapy. We haven't had any major health issues thus far and hopefully it continues to stay this way. I'm so thankful for newborn screening to have caught this so early on. He has been treated since he was 3 weeks old and his levels stay fairly normal for the most part or at least where his doctor wants them. I still have so much to learn about all of this and just in the past month have finally found other families that battle this disease. It can all be confusing and too much. We take it day by day and appointment by appointment.

My biggest advice is don't stop looking, don't lose hope; you are not alone. Even though my child has a rare disease, he is thriving in life. He's so full of energy and always happy. I'm so thankful for him and pray over our journey in life.

Amber's Kitchen

Sweet Potato Toast

Makes 3 servings

Ingredients:

- 220 g Raw Sweet Potato
- 3 tsp Violife Just Like Cream Cheese - Original
- 1 berry, strawberry, fresh, medium, diced

Directions:

- Slice sweet potato into three slices longways. Each slice should be between 1/4 to 1/2 inch wide.
- Toaster: Place each slice in the toaster. Set toaster to high setting and start toaster. You want the toast to be fork tender, but not too crispy. Carefully remove from toaster to a plate and allow to cool a few minutes. Oven: Preheat oven to 350 degrees. Line a baking sheet with foil and spray with cooking spray. Place sliced sweet potatoes on baking tray and bake for 15-20 minutes until fork tender. Carefully remove from oven and allow to cool a few minutes.
- Spread 1 tsp of the cream cheese on each slice of toast. Top with sliced strawberries. You can also top with chopped fresh mint for added flavor, but optional.

Note:

This is just one of many options for the toast.
Others:

- Cookie butter with broken gluten free pretzels
- Season with Salt, pepper, italian seasoning and top with nondairy cheese, low protein scrambled eggs and crumbled vegan bacon

Nutritional Information: Serving size: 1 toast | Protein per serving: 1.3 g | Calories per serving: 76

April 2021: HCU and You: *Ask Methia*

Dear Methia,

The Quarantine 19 is Real!

No one tells you that the anxiety that accompanies a pandemic could also bring along its good friend: weight gain. I've packed on 20 pounds this year – YIKES! Sure, I've been eating more, but I've also been less active, being that I have been inside my house most of the time. One of my friends has had a lot of success with the ketogenic diet and has lost all of their "pandemic weight" in just 3 weeks. I'm thinking this is something I should try. Do you have any thoughts?

Sincerely,
Tight Jean Queen

Dear *Tight Jean Queen,*

Please know that you are not alone in your concerns. Many people have experienced weight gain as a result of the pandemic. Mindless snacking and overeating are common when bored and/or anxious, which are two sentiments that have dominated the past year.

Fad diets for weight loss, including the ketogenic diet and intermittent fasting, have become increasingly popular in our society. While they often do result in seeing lighter numbers on the scale (and quickly), it's important to note that this is not a healthy way to go about sustainable weight loss – PARTICULARLY with inborn errors of metabolism. Here are a couple of reasons to avoid these diets:

1. **They don't address the reason(s) why you gained weight in the first place.** Some people gain weight because they are too sedentary, while others gain weight simply because they added a bowl of ice cream to their nighttime routine. Completely overhauling your diet and drastically cutting your intake will help in the short-term, but the second the "reigns are loosened," it's likely that the weight will come back. The best way to address the weight gain is to address the issue at hand. Did you used to go for a nightly walk, but stopped? Think about re-starting! Did you start to eat that bowl of low protein ice cream at night because you love it, or because it was something to do when you were bored? If the answer is you love that bowl of ice cream, consider decreasing the frequency or switching to a light ice cream. If the answer is that you were bored, think about quitting it altogether.

2. **Very low-calorie diets can be dangerous for people with inborn errors of metabolism.** Cutting calories too fast and long periods of fasting forces the body to turn to its reserves (think fat and protein/muscle) for energy. This disrupts an already sensitive metabolic state for people with inborn errors of metabolism, and may affect methionine levels in homocystinuria (HCU) and homocysteine levels in both HCU and cobalamin defects. Because everyone is uniquely different and we don't know for sure how the body will respond to long fasts or drastic calorie deficits, the safest thing is to avoid them.

3. **For many people, they result in feelings of deprivation and are not sustainable.** Many fad diets leave people feeling worn out and hungry, and are the main reason they often fail. Also, having food freedom and the choice to be able to indulge from time to time is absolutely essential to your emotional well-being! Nutrition professionals talk about moderation being the key to success. If you know that chocolate donuts are your kryptonite, set a special day of the week where you can enjoy one (and keep it to JUST ONE by only keeping one in the house at a time!).

Your dietitian at your metabolic clinic has, more than likely, helped at least one metabolic patient to navigate the complexities of weight loss. They are your best resource if you need help achieving a healthy weight and improving the quality of your diet. They can also connect you with other patients. Remember, you have a support system, and within that support system there is almost always a solution!

Sincerely,
Methia

SPECIAL FEATURE: FITNESS AND HCU

March 20th marked the first day of spring! With the longer daylight hours and warmer weather many of us are looking forward to getting outside and enjoying the fresh air. For many the return of spring also means getting back on track with our fitness goals for the year and evaluating our nutritional goals for our soon to be summer bodies! While Ask Methia addressed the diet aspect – we thought it would be good to share some of our patients' experiences with the fitness side!

Marathon Training with HCU

by Kristin Rapp

It's common knowledge that what you eat before, during and after an endurance event can help or hurt your performance on race day. I've completed 12 full marathons, 19 half marathons, several 100-mile bike rides, a triathlon, and numerous other races. Over the years, many people have asked me how I fuel up for a marathon on such a restrictive diet. To be honest, even after competing in all these athletic events, I'm still learning. The good news is there are lots of books out there about running/exercise & nutrition. I've read some interesting things about runners who follow a plant based, vegan or fruitarian diet – which is very similar to what I eat.

These resources have been great finds for me, however, there isn't much out there in terms of guidelines for how a person with a metabolic disorder should nutritionally prepare for an endurance event. So, I thought I'd share with you some of my personal tips I've gathered over the years.

1. It should go without saying, but you should **always consult with your metabolic team before starting a new exercise plan**. They should be able to work with your unique dietary needs and come up with a nutritional game plan. I've been very fortunate as my nutritionist is also an avid marathon runner herself. I work with her to tweak my diet as my mileage increases during my training cycle.
2. **Take <u>all</u> your prescribed metabolic formula.** Since I'm not getting much in the way of natural protein from my diet, it's essential that I drink all the formula. It provides protein which is important for muscle building and repair. If you have a weak stomach like me, it might be hard to drink formula during an athletic event, but it's so important that you do take it before and after exercise.
3. **Stay hydrated!** I use a powder formula that I mix with water – so that's one way I stay hydrated. I also drink a sports drink such as Gatorade or Nuun to replace sodium and potassium – the electrolytes you lose in sweat. Recently, my dietician recommended another product called UCAN. They have an electrolyte replacement powder and an energy powder that I'll discuss in the next section. One thing I really like about UCAN is it was initially created for a boy with a rare metabolic disorder that required him to be tube-fed every two hours to maintain normal blood sugar levels. I just love the story behind the product.
4. **Carb-loading.** Many runners will probably tell you there favorite part about preparing for a race is that pre-race pasta dinner. Carbohydrates provide fuel so your body has enough energy to cover the distance of your athletic event. This can be a little challenging for someone with a metabolic disorder as some store-bought or restaurant dishes are too high in protein. However, there are an array of medical foods out there that can help if your protein allowance is low. Some of my favorite carb-

loading options include: low protein pasta, rice, pizza and baking mixes; bananas, oatmeal, potatoes, gluten-free waffles, pancakes and breads. Vegan, Vegetarian and Gluten-Free diets have massively increased in popularity over the past few decades which has been great news for me. This increase in popularity with these types of diets has resulted in a lot more HCU-friendly options in the grocery store and even at restaurants. If you're going out of town for an event, check with your hotel or a local restaurant ahead of your trip. They may be willing to cook a low protein dish if you bring your supplies (low protein pasta/rice/baking mix) with you.

5. **Taking care of your body during the recovery process** after a long run or endurance event is just as important as the pre-race preparations. Make sure you're replacing the fluids lost during exercise as well as taking in the appropriate amount of carbs and protein. Some of my go-to options post-race include: formula, watermelon, apples, salty sweet potato fries, avocados, pickles/pickle juice and beets. Some of these foods may seem strange, but there's a reason each one is on my list. The formula provides much needed protein. Watermelon is incredibly hydrating, low in calories, and rich in vitamins and phytonutrients. Apples are a great low-protein choice for replacing carbs. Sweet potatoes are full of vitamin C and minerals that are essential for bone health and muscle recovery. Avocados provide "good fats" which are known to reduce inflammation in the body. Pickles and pickle juice are good for replenishing electrolytes and have been reported to help ease muscle cramps. Beets are not always my favorite, but I try to squeeze them in when I can. They are packed with iron, magnesium, folate and antioxidant properties. It's reported that beets can improve athletic performance, regulate blood pressure and increase blood flow.

Navigating fitness and HCU can be challenging, but it's definitely possible. Don't be afraid to ask for help!

I'm always happy to speak with other HCU patients about health, fitness, running marathons and managing the low-protein diet, so feel free to contact me via Facebook or email rappkristin@yahoo.com.

SPECIAL FEATURE: FITNESS AND HCU

How to Hike (with HCU)

By Benjamin Lewis

In many ways, living with a rare disease like Homocystinuria (HCU) is like hiking in the mountains. Both have high points and low points along a journey, and you are more likely to see success and enjoyment in both with planning and preparation. In my experience, it is also the case that the former should not hold you back from pursuing the latter. In other words, I and others with HCU are as able as anyone to set out on our own adventures, whether that be to points over a dozen miles into the woods of New Hampshire (NH), 6,288 feet above sea level at the top of Mount Washington, or the top of the Franconia Ridge in a -60-degree (Fahrenheit) windchill in the middle of winter. Over many years exploring the White Mountains of NH and other wild places, those are all situations I have found myself in and HCU and all of the considerations that come with it have not been a blocker. Therefore, drawing upon my experience over many hundreds of miles trekked and at least 60 or so high peaks climbs, I would like to offer others in the community a bit of advice on how to be a hiker, climber, or mountaineer with HCU. I think you will find that – spoiler alert – nearly all my advice applies to anyone who wants to hit the trail or climb a peak because, of the key steps to take to ensure your safety and enjoyment, none of them are all that different if you have HCU.

How to Hike (with HCU)

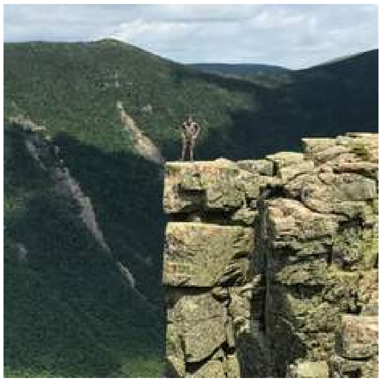
1. Set a goal! While you are more than welcome to pick any single trail or scenic mountain top and go through the rest of the steps, I highly encourage you to set a goal that goes beyond just one trip into the woods. For me, my love of hiking and mountaineering really got going after I set my sights on the Appalachian Mountain Club (AMC) Four Thousand Footer Club list of 48 peaks in NH. Not only did the list motivate me to keep getting out there, but it also forced me to try new trails and peaks I would have never otherwise visited. In northeastern United States, AMC and others offer many lists and achievements that you can choose to be your goal and other outdoors-oriented organizations around the world do the same. So set a goal and get outside!
2. Plan your outing! Regardless of whether you have picked a single hike to get your outdoor adventuring started, or a more extensive objective that will mean many miles are in your future, I highly recommend you do your homework before hitting the trail. A poorly planned trek may turn out to be miserable or even dangerous if you are caught in bad weather, get lost, or find yourself dealing with more than you bargained for. Using guidebooks, online blogs, and other resources, I always plan my route, check the weather and trail conditions, and estimate the hike time for my outing before I leave home. If you are venturing into an area with high peaks and/or areas above tree line where you will be exposed to the elements, take extra precautions and note your best 'bug out' or escape routes, triple check the weather in the 24 hours leading up to your hike – including, if available, a higher summits forecast, and know how much daylight you will have versus your arrival time and hike time. You will not regret doing this homework.
3. Prepare you and your gear! This is another step that may make or break your experience regardless of whether it is your first climb or you have been bagging peaks for years. Never leave home ill-prepared even if the outing is expected to be short. The best advice I can give is to listen to the experts and pack at least the "10 Essentials" for every hike. I always have some form of each essential system – a map, headlamp, sunblock, first aid kit, knife, fire-starting items, a form of emergency shelter, extra

food, extra water or a filter, and extra weather-appropriate clothes. I often like to hike solo and/or in the winter, and I take gear prep even more seriously in those cases. I will usually add a personal locator beacon (PLB), more first aid and clothing, as well as specialized equipment for winter mountaineering such as micro-spikes and an ice axe. In some cases, this gear is chosen to mitigate the risks of something going wrong, or to help respond more effectively if there is an issue. For the most part, I am thinking about situations anyone could encounter when selecting my gear, but certainly packing a pouch or two of HCU formula or having available medical care instructions for first responders are good practices with HCU in the picture. This line of thinking also extends to what I consume before a hike and then during. I always aim to have my formula before I set out to take advantage of the energy it contains, and I pick cereal bars, sports drinks, snacks, and sandwiches that are low protein but have some nutrition to give me a boost out on the trail. That preparation helps ensure I have the energy to make it through even the most taxing 20+ mile hikes, as well as essential equipment to deal with the unexpected!

4. Have fun adventures! The last step is the easiest - get out there and experience the fresh air, scenic views, and exciting trials near you! The long and short of it is that HCU has not held me back from doing that here in New England and beyond. The key steps to ensure a safe and enjoyable hiking or mountaineering trip are the same for virtually everyone. Yes, having HCU means you may benefit from drinking formula before you hit the trail, or that you may find yourself eating pretzels instead of the traditional trail mix as you take in the views from a high peak (you can always pick at a friend's trail mix – I suggest M&Ms over raisins any day). However, I can assure you that if you are going to focus on anything in advance of a hike or climb, I suggest it be the planning and preparation that will make for a successful trip and not HCU. Get out there, seek out those high points, and discover your own adventures. I hope to see you on the summit!

At the "finish line" of my WM4K

At a trail crossing near Mt. Adams



On the outcropping of Bondcliff

Mark your calendars and start your training now for HCU Network America's Race for Research!

You're RARE We CARE

Introducing NEW Homactin AA Plus Powder In Refreshing Lemon Lime

Continuing to innovate for our #SmallButMighty communities!

15 PE HOMACTIN™ AA Plus
METHIONINE FREE
Lemon Lime
POWDER

- **Great Fresh Flavor:** **Lemon Lime**
- **Flexible For All Ages:** **15g PE & 150 kcals**
- **Low Volume:** **Mix With 5 oz Water**
- **Optimized Bone Health Profile**

**Feedback card included in each Homactin AA Plus Powder Sample Kit. For every feedback card completed and returned to us, Cambrooke will donate $10 towards the HCU Network America. Ends May 31, 2021. Terms and conditions apply.*

OUR PROMISE

We will donate $10 to the HCU Network America for each Sample Feedback Card received!*

Get prepared for spring & upcoming Easter holidays!

Make your favorite desserts, such as delicious cookies, muffins and cakes and much more!

cambrooke.com 866 456 9776

Wel-Made Baking Mix

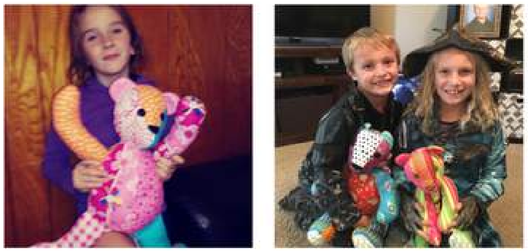
Sugar Cookie Mix

HCU Network America

Eat Well, Live Well.
AJINOMOTO.

CAMBROOKE™

RARE BEARS FOR RARE KIDS!



We are thrilled to announce that the Rare Bears for HCU Campaign in partnership with Rare Science has been re-opened!

To enroll in the RARE Bear Program and to request a RARE Bear, please click the link and complete the form: https://www.rarescience.org/hcu/

- Those who have already received a bear, are not eligible
- Date for gifting will be announced later
- You will not receive a confirmation email or be notified when your bear has been shipped

SCHOLARSHIP OPPORTUNITIES

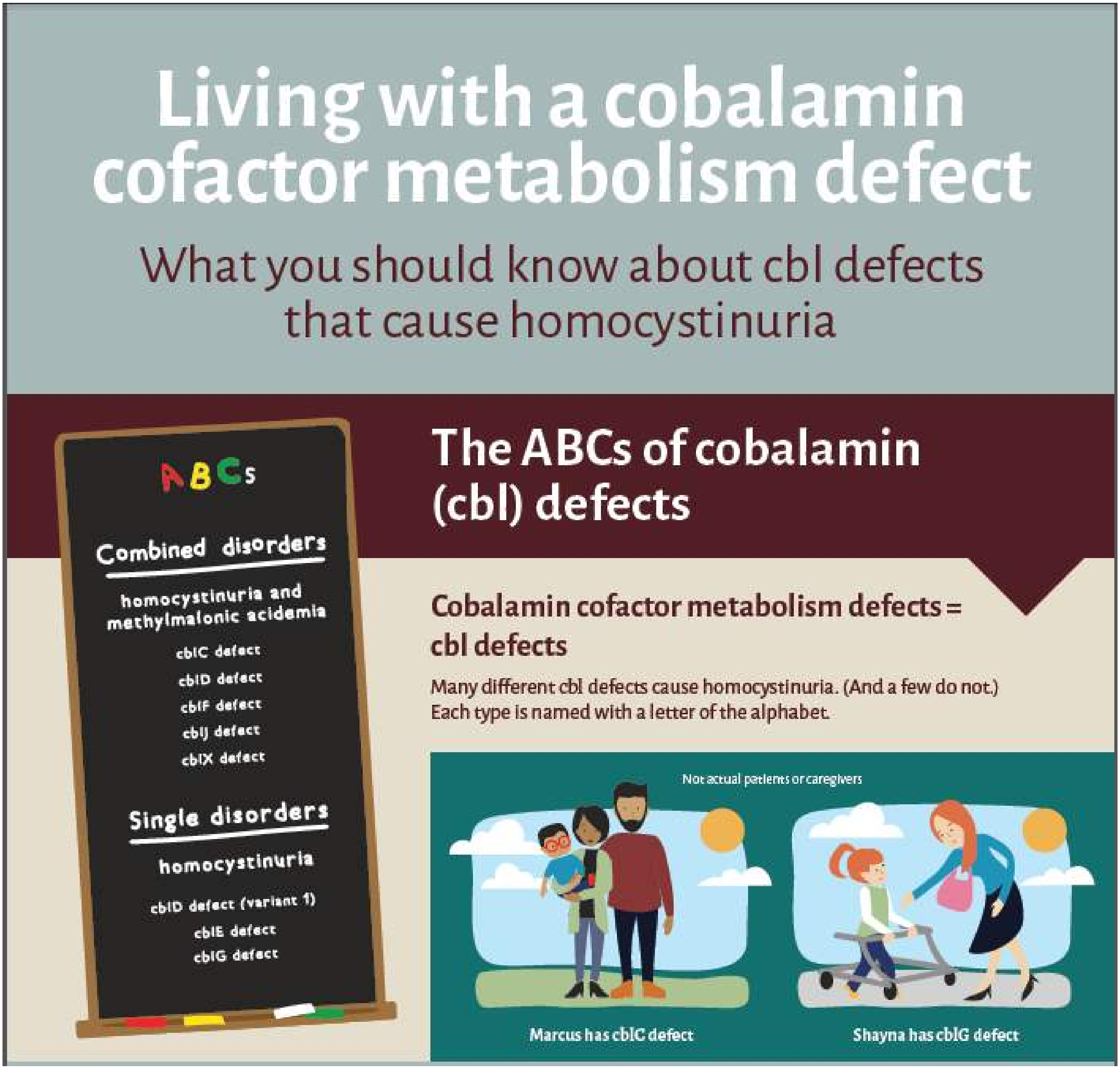
Living with a rare disease means managing unique challenges, including frequent doctor visits, rigorous treatment regimens and hospitalizations, and exposure risks. While quality and duration of life continues to improve thanks to improved diagnosis and treatment approaches, individuals living with rare diseases still face disparities in achieving traditional life milestones.

That's why The EveryLife Foundation for Rare Diseases established the #RAREis Scholarship Fund – to enrich the lives of adults living with rare diseases by providing support for their educational pursuits. Thanks to the support of Horizon Therapeutics RAREis, one-time awards of $5,000 each will be granted to up to 32 recipients for the Fall 2021 semester.

Deadline for Fall Semester 2021application is May 7, 2021 at 3:00 p.m. CDT Learn more and apply here: https://everylifefoundation.org/rare-scholarship/

NEWS YOU SHOULD KNOW

Check out this amazing new resource from Recordati Rare Diseases! Click the here, or the infographic to see the full **infographic on *Living with a Cobalamin Cofactor Metabolism Defect***.



Living with a cobalamin cofactor metabolism defect

What you should know about cbl defects that cause homocystinuria

The ABCs of cobalamin (cbl) defects

Cobalamin cofactor metabolism defects = cbl defects

Many different cbl defects cause homocystinuria. (And a few do not.) Each type is named with a letter of the alphabet.

ABCs

Combined disorders

homocystinuria and methylmalonic acidemia

- cblC defect
- cblD defect
- cblF defect
- cblJ defect
- cblX defect

Single disorders

homocystinuria

- cblD defect (variant 1)
- cblE defect
- cblG defect

Not actual patients or caregivers

Marcus has cblC defect

Shayna has cblG defect

NEWS YOU SHOULD KNOW

NEWS YOU SHOULD KNOW: PUBLICATIONS

In case you missed it (#ICMY)

- Homocystinuria Patient and Caregiver Survey: Experiences of Diagnosis and Patient Satisfaction Publication

We are delighted to share that a paper from HCU Network Australia's survey of the homocystinuria community has recently been published in Orphanet Journal of Rare Diseases. This paper describes the often long delay in diagnosing the homocystinurias unless this is achieved by newborn screening; this paper also highlights problems with the availability and cost of treatment and the palatability of protein substitutes. (https://ojrd.biomedcentral.com/articles/10.1186/s13023-021-01764-x)

NEWS YOU SHOULD KNOW:
MEET OUR NEW COMMITTEE MEMBERS

- Fundraising Team - Tom Hawkins

Tom Hawkins is an HCU Dad, with two children (Virgil & Annie) that have HCU and a third child (Charlie) that is HCU-free. Tom & Kristen had never heard of HCU but learned they were both carriers when Virgil's diagnosis was caught via newborn screening. With both Virgil and Annie being diagnosed from birth, they have luckily been on a steady diet of HCU Early Years and HCU Next and gladly drink their "milk" every day. The Hawkins family resides in Park Ridge, IL; Tom works as an M&A business consultant at PricewaterhouseCoopers.

- Fundraising Team - Annette Settle

Gig (George) and Annette Settle live in the Harrisburg suburbs of Pennsylvania with their son Adam (21) who was diagnosed with CblC deficiency under the then new newborn screening. They believe Adam was the third child diagnosed prior to the onset of serious medical complications. Adam is the youngest of 8 of Gig and Annette’s children. Adam’s story was captured in a book for children; No Day Wasted: The Adam Settle Story. The Settle family enjoys mission work in developing countries. They look forward to getting involved and helping raise awareness and much needed funds to help accelerate research for the Homocystinuria community!

NEWS YOU SHOULD KNOW

- Patient–Parent Advisory Committee (PPAC) - Elizabeth Carter

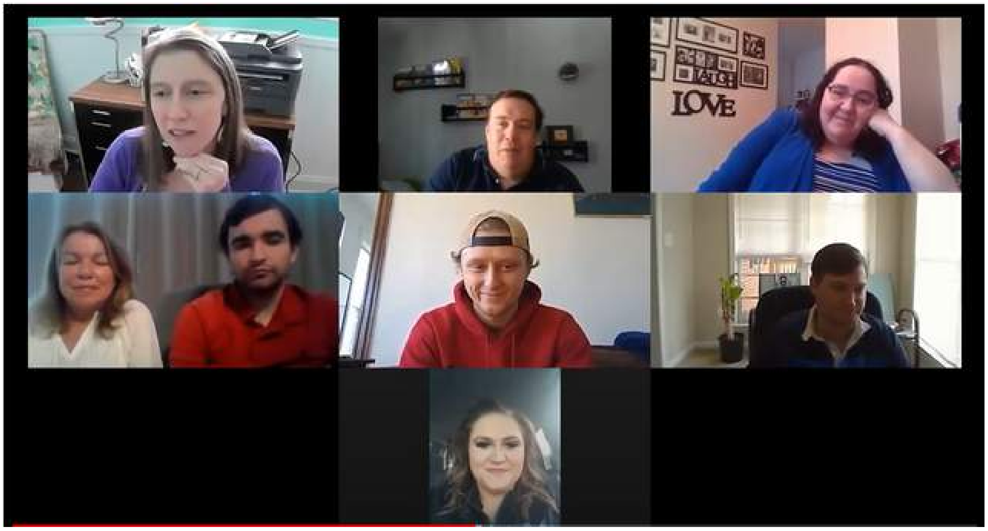
Elizabeth is the mom of Elliott, a 5 year old boy in South Carolina who has Homocystinuria. Elliott was diagnosed with HCU at age 2 after suffering from an unexplained illness and clotting in the brain, which left him fighting for his life. Upon receiving this diagnosis, Elizabeth found the HCU Network America online and immediately connected with other families living with HCU. Elizabeth says that the HCU Network America has been an invaluable resource and that the connections and relationships that her family has built through the network have been such a source of comfort and knowledge. Elizabeth hopes that by serving on the advisory council, she can help other families to make these connections and to help further the mission of the network.

To learn more about our committee's:

- Fundraising Committee, visit: https://hcunetworkamerica.org/fundraising-committee/
- PPAC, visit: https://hcunetworkamerica.org/patient-parent-advisory-committee/

NEWS YOU SHOULD KNOW: EVENTS

- Navigating Relationships: Low Protein Community Chat - Synopsis



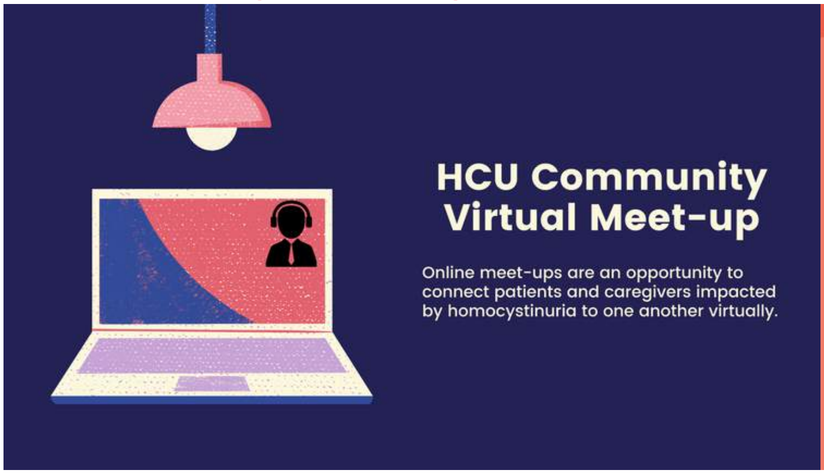
February 27 HCU Network America along with MSUD Family Support, Organic Acidemia Association, PKU Organization of Illinois and PKU News co-hosted Navigating Relationships: Low Protein Community Chat, A Patient-Panel Perspective. The hour long panel explored different ideas of how the low protein diet affects relationships regarding family dynamics, dating, co-workers, and roommates. The key take home was “everybody’s story got better, as they got older. So, no matter where you are in this journey, it gets better”. “Today is not every day, every day will get better”.

You can watch the recording here: https://www.youtube.com/watch?v=WFs46B6Va7M&t=159s

UPCOMING EVENTS

Come check out our Virtual Homocystinuria Meet-ups!
Join our virtual meet-up for a chance to meet, connect, and learn from other patients and caregivers who are facing similar challenges. Whether it's navigating adherence issues, insurance, clinic visit, or life transitions, you are not alone.

Note our new registration page: https://hcunetworkamerica.org/virtual-meet-ups/



HCU Community Virtual Meet-up

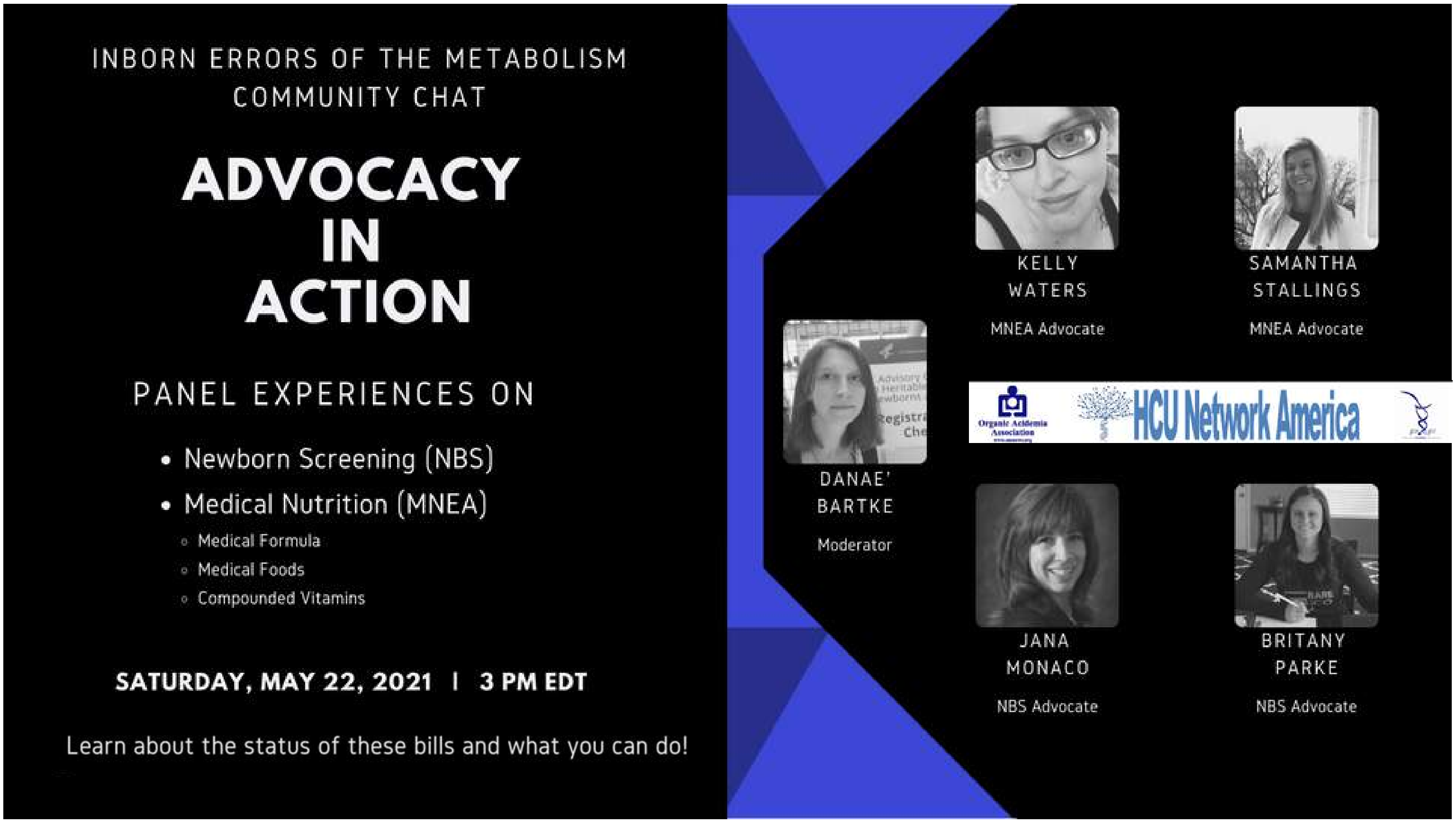
Online meet-ups are an opportunity to connect patients and caregivers impacted by homocystinuria to one another virtually.

MMA & Cobalamin Community Virtual Meet-up

Online meet-ups are an opportunity to connect patients and caregivers impacted by methylmalonic acidemia and coblamamin disorders to one another virtually.

- **Sun., April 11 @ 2 pm CDT | 8 pm UTC**
- **Mon., April 26 @ 1 pm CDT | 7 pm UTC**

- **Sun., April 25 @ 7 pm CDT**



INBORN ERRORS OF THE METABOLISM COMMUNITY CHAT

ADVOCACY IN ACTION

PANEL EXPERIENCES ON

- Newborn Screening (NBS)
- Medical Nutrition (MNEA)
  - Medical Formula
  - Medical Foods
  - Compounded Vitamins

SATURDAY, MAY 22, 2021 | 3 PM EDT

Learn about the status of these bills and what you can do!

KELLY WATERS
MNEA Advocate

SAMANTHA STALLINGS
MNEA Advocate

DANAE' BARTKE
Moderator

JANA MONACO
NBS Advocate

BRITANY PARKE
NBS Advocate

Register now: https://zoom.us/webinar/register/4016172070335/WN_x5T3l1r3RF-o60MMgBt8CQ

Dear HCU, OAA, and PAF Families,
In October 2019, HCU Network America hosted their second patient/family-expert conference. The families and experts were so excited to come together and share their knowledge and experiences with one another over the course of the two days. The weekend was packed with speakers, panels, children's programs, time to visit vendors and of course time to network and catch up with old friends and make some new ones. It really is a one-of-a-kind experience for patients, families, medical professionals and researchers.

This amazing experience made coming back and starting to plan our 2021 conference very easy. By December 2019 we had a location already selected and we were so excited to start reaching out to possible speakers and putting together the agenda. We had no clue how quickly the next few months would change the course of our lives. Like many others, by spring we understood the true impact that the pandemic would have on our community. We were hopeful with the work of the biotech and pharma companies, and with the guidance of the CDC we would soon have a vaccine. We thought for sure we'd have something by December. Well December was right, just not to the volume we had hoped for and so here we are today writing this letter. It is with great disappointment we are announcing the postponement of our 2021 conference. Based on the timing of the vaccine rollout in the US, we expect that not all adults will have been fully vaccinated and vaccines will not have been made available yet to teenagers and young adults. We are tentatively planning to have our in person conference June 25-26, 2022. While we know this is a disappointment for all of our communities, we believe it is in the best interest of all parties involved.

To help fulfill the needs of our communities, we will be hosting several virtual events over the summer – please stay tuned for a schedule of events.

Thank you for your understanding, and please feel free to reach out to us -

Danae' Bartke	Kathy Stagni	Jill Franks
HCU Network America	Organic Acidemia Association	Propionic Acidemia Foundation

---

Save the Date for these Upcoming Events!

- 2021 Recordati HCU Click Campaign
- Race for Research, Virtual Race – September 1-30, 2021
- HCU Awareness Month – October 1-31, 2021

GET INVOLVED

RARE on the ROAD
Rare Disease Leadership Tour

Virtual State-Specific Training and Networking Events*

May 4, 2021 – Nevada (10:00am – 12:15pm PT)
May 11, 2021 – Florida (10:00am – 12:15pm ET)
May 18, 2021 – Illinois (10:00am – 12:15pm CT)

#RAREOnTheRoad RARETOUR.ORG

Calling All Nevada, Florida, & Illinois Residents! Registration for the RARE on the Road is now OPEN!

You're invited to participate in a RARE on the Road Virtual State-Specific Training and Networking event! Brush up on your rare disease advocacy skills and connect with other advocates in your area.

Join the EveryLife Foundation for Rare Diseases and Global Genes this May to...
- Discuss what advocacy means to you and the different ways to make your voice heard and advance change for the rare disease community...
- Participate in a guided, step-by-step workshop on how to tell your own rare story, no matter the audience...
- Network with rare disease advocacy leaders and peers from your local community...
- Gain insights and updates on COVID-19 vaccination distribution in your state...
- Learn next steps for connecting with even more rare disease community members in your state to keep the momentum going on your advocacy journey.

Registration is now OPEN! Don't miss the opportunity to build upon the skills and interests already in your toolbelt while virtually meeting others in your state to share experiences and ideas.

*Note: You must be a resident of the state event you select to attend.

May 4, 2021: Virtual State-Specific Training and Networking Event | Nevada
May 11, 2021: Virtual State-Specific Training and Networking Event | Florida
May 18, 2021: Virtual State-Specific Training and Networking Event | Illinois

Click Here to Register Now

GET INVOLVED

Rare Disease Week on Capitol Hill 2021
July 14th to July 22nd

New dates, new format...
same life-changing experience!

Rare Disease Week on Capitol Hill brings together rare disease community members from across the country to be educated on federal legislative issues, meet other advocates, and share their unique stories with legislators. While we had hoped to host this event in-person, for the safety of all advocates, Rare Disease Week will be going virtual for 2021.

Virtual Rare Disease Week on Capitol Hill 2021 will be held July 14th through July 22nd and will include the same opportunities as in-person Rare Disease Week, plus more!

Registration opens April 21: https://everylifefoundation.org/rare-advocates/rare-disease-week/

HCU NETWORK AMERICA
IS LOOKING FOR STATE AMBASSADORS

Looking for active and outgoing members of the HCU community

What does an ambassador do?

Ambassadors...

- *Connect with local HCU families*
- *Share their story*
- *Advocate and raise awareness for HCU*
- *Amplify and support our mission*
- *Help fund-raise*

Get involved today! Contact Danae'
dbartke@hcunetworkamerica.org

BECOME A
STATE AMBASSADOR
FOR HCU NETWORK AMERICA

PaTH Network
patient empowered research

University of Pittsburgh UPMC

MyPaTH Story Booth

Would you like to

Share your story?

The University of Pittsburgh and their collaborators are conducting a research study and want to know about patients' experiences with health and illness. By sharing these stories with researchers, we hope to focus research on topics that are of importance to patients and likely to improve health and health care. Using MyPaTH Story Booth, patients can record a conversation to share with researchers. Let them know what it's like to live with your health issue, what you would like health care professionals to know when they care for someone with a diagnosis like yours, or how the health care system could have improved your experience among other topics. **We'd love to hear your point of view!**

How: Share your story by phone

Who: Anyone 18 or older and able to speak and understand English over the phone

Why: To focus health research on topics that matter, by amplifying patient and caregiver voices

Phone: 412-692-2697

Email: mystory@pitt.edu

APRIL FUNDRAISING REMINDER

Not using Amazon Smile? It's easy, here's how

Support us with
amazonsmile
You shop. Amazon gives.

What is Amazon Smile?

Amazon Smile is a simple and automatic way for you to support HCU Network America every time you shop, at no cost to you. When you shop, you'll find the exact same low prices, vast selection and convenient shopping experience as, with the added bonus that Amazon will donate a portion of the purchase price to us.

How do I set it up?

Simply, go to smile.amazon.com, the first time you go it will ask you to designate an organization. Type in HCU Network America and select us from the list (or go to our direct link: click here). It is important to note that in order for the donations to go to HCU Network America, you MUST check out from this url every time - see best practices below for some pointers on how to do this.

Best practices for using Amazon Smile

Now that your account is set up to use Amazon Smile, it is important to note that Amazon only makes donations to HCU Network America when you checkout from your cart from this url. This is the only way HCU Network America gets any donations from Amazon Smile. Since this is the case here are some best practices to help you make the most of your Amazon Shopping.

Desktop Users:

If you do your Amazon shopping from you desktop/laptop then you can simply bookmark/favorite this url and do your shopping from this web page

Mobile Users:

Most Amazon shoppers use the app on their mobile or tablet. If you are using your smartphone, be sure to download the Amazon Smile App and follow the directions.

What if I'm already set up and would like to switch to HCU Network America?

1. From your desktop, simply select "Your Account" from the navigation at the top of any page
2. Then select the option to "Change your Charity". From your mobile browser, select "Change your Charity" from the options at the bottom of the page.
3. Type "HCU Network America" in the search bar and search for the charity.
4. Select HCU Network America charity to update your account

NEED HELP HOSTING A FUNDRAISER? EMAIL US - INFO@HCUNETWORKAMERICA.ORG