The HCU Herald

More HCU News Inside

HCU Hero: Kelly from Michigan
HCU Community Cook Book

News you should know!

- Retrophin Inc. Announces Corporate Name Change to Travere Therapeutics, Inc.
- Aeglea Presents at ASHG on ACN00177
- Aeglea BioTherapeutics Receives FDA Rare Pediatric Disease Designation for ACN00177 for the

Upcoming Events

- December Virtual Meet-ups
- Rare Across America
- 2021 Conference Agenda Now Available

Special Feature: Balancing Life and HCU
Help us Complete our Match - only $6,077 left to go!

HCU HERO:
KELLY FROM MICHIGAN

My name is Kelly and I was born in October of 1989. I appeared to be a perfectly healthy baby, the youngest of three, to loving parents and two wonderful older brothers. I appeared to be just another little girl until the age of three when it became obvious to my parents that I was becoming more and more agitated; and losing fine motor skills. At five years old, I was diagnosed with laterally subluxation in both eyes and high myopia (nearsightedness). My parents prayed new glasses would solve many of my problems. However, I was instead diagnosed with Homocystinuria, a rare autosomal genetic recessive Inborn Error of Metabolism. The familiarizing of medical phrases like "inability to produce the amino acid ***methionine***", "toxic buildup of ***homocysteine***" and "***low protein diet for life***" would become the normal from that moment on.

What life doesn't prepare you for is the disgusting taste of the medication that you are now expected to take multiple times a day, for the rest of your life. What no one can prepare you for are the social, financial, and emotional implications and complications that come with a rare diagnosis. What doesn't sink in at the time of the diagnosis is that this is for life. You can't take it off like you can a mask. It doesn't go away at eighteen or twenty-six; it doesn't go away at all until we are dead. How do you begin to explain any of that to a sick child, let alone a parent receiving what is easily one of the most overwhelming and terrifying pieces of news?

In order to not starve, protein from a "normal diet" must be replaced with modified protein medical formula, and a low protein diet for life. There is no way to modify the diet without also taking formula. This includes things like low protein pasta, bread, baking mix, etc. that can only be ordered from one of a few specialty medical food companies. These companies ask that you pay a certain amount before they will ship anything to you, so you end up paying in many cases, minimum of fifty dollars for food that doesn't last you a week.

As I said, I was diagnosed at five years old, about the time most children enter kindergarten and elementary school. This would play a critical role in not just my social development, but on how I would begin to view myself. Growing up, most of my peer's parents didn't want me to play with their children, either because they didn't know what they could feed me, or they didn't understand my disorder, either by choice or ignorance.

I remember taking out the elementary school phonebook; and calling each and every person in my grade, just looking for a friend to play with; just looking for some small bit of normalcy in a sea of things that set me apart socially or developmentally. Each time I called I would generally be told "they aren't around", "they are at (fill in the blank weekend activity)", etc.

So with very few friends who could or would come over to play, I took to movies for comfort and solace. I learned many social lessons from watching TV and movies growing up. I identified with the outcasts of the X-men, Spider-Man, Disney characters like the Beast, and Quasimodo; I saw what I wanted to be in characters like Batman, Frodo, and Harry Potter, characters on the outskirts, considered as monsters, but who still choose to not turn their backs on the very societies that would demonize them, for simply being different. The ones who still tried to do the right thing and make the world a little safer for those like them, and everyone by extension.

At home, I would have to go through the battle of taking medical formula, the thing keeping me alive, that tastes like vomit, no matter how you try to flavor or disguise it. This would be difficult for an average child, but I wasn't. I was hyperactive, easily frustrated and prone to violent mood swings as a result of the disorder. I also had sleeping difficulties and issues with low energy. My mother had to cut back on her work hours to take care of me, my diet, and to take me to doctor appointments. At the same time the additional financial struggle of paying for expensive food and formula, were also added to the family budget.

As a direct result of the diagnoses, I have had to have extensive dental and orthodontics work to correct my misaligned teeth. The oral work started at seven or eight years old, and continued on and off until I was in my early twenties. I was in and out of therapy, O.T., counselor's offices, resource classes to allow for extra time on homework and tests, etc. This was on top of the days I had to take off from school for doctor appointments. I remember in the early years of elementary school, praying to God for a cure for this disorder. I prayed every holiday and birthday, because all I wanted was a normal life. By fourth grade I stopped praying, because I no longer saw a point.

When I was fourteen years old, I got sick with sinusitis with bacteremia, causing me to throw up twenty pounds of my body weight, and having severe sensitivity to light and dehydration. I was in the hospital for a week and a half. During this time I found out the internal carotid artery in my head, leading to my brain, is narrowed, and as a result I was put on blood thinners. I was wheeled out of the hospital at fourteen, with twenty specialists now working around the clock to keep me alive. This incident left me almost dead, and for the next year I fell into such a deep depression that enjoying anything was not physically possible.

In 2006, when I was sixteen years old, my medical formula at that time cost $891 a month. Insurance companies refused to pay for it because it wasn't considered a prescription drug, despite needing a medical doctor's order for it. This was the reason chosen to discriminate against me because the medical formula had/has a different classification, so that it does not have to go through lengthy, rigorous FDA approval of chemical compound medications. Our insurance carrier at the time told my parents that my formula was "not medically necessary" because I can physically ingest it, and that it would not be covered unless taken via tube feeding. In 2006, twenty five states had passed laws mandating coverage of medical formula and low protein food products. This is to prevent insurance companies from taking advantage of this distinction.

Like many patients who often feel discriminated against and dehumanized, due to what is considered "otherness", and lacking access to resources, seeing how easily my life could be written off, and dismissed, not giving a thought to the consequences of not covering formula and low protein food. It was all I needed to go "off diet". The taste of the medical formula and low protein food didn't help this matter.

When I was first diagnosed, my parents were told I would not live to see thirteen. If I did, I would not live to see eighteen; and if I did live to see eighteen I wouldn't graduate high school. (I did graduate high school on time, with a B average.) At 16 years old, I started to do my own research on Homocystinuria. I read something that rocked me to my core, a statistic that said patients who are undiagnosed would not live to see thirty years old. So, for fifteen years I thought I would die by the age of thirty.

At some point in my life, I resigned myself to the fact that I was a monster who deserved every bad thing that had happened to her. At some point in my life, I felt abandoned by God, society and my government. The actions and failure to protect me, and people like me, never helped with that mentality.

While some insurance companies have changed their policies to cover us until the age of either eighteen or twenty-six, this leaves us wondering as patients, what happens afterwards? Our problems don't go away. No one waves a magic wand, and suddenly we're cured. Reality doesn't work like that, and pretending it does, is insulting to us, our families, our medical teams, our intelligence and the struggles we face every day.

At the time of writing this, I am thirty one years old, the election just took place, and while some are worried about how long Covid-19 will stick around, I have come to the realization that it is my moral, ethical, and social obligation to continue the work done by my parent's generation. People like my mother who fought for so long for medical food legislation in the state of Michigan; my aunt who worked with a medical formula company to assist children like me, her coworker who started her own company and offers cooking demonstrations to educate patients on how to properly prepare food, so walking into the kitchen isn't the worst part of their day. People like my geneticist and dietitian who have stuck by my side since that medical crisis in 2004 when I was fourteen and nearly died. They advocate for so much more than just medical needs. People like the endocrinologist who despite having Parkinson continued to advocate for people with rare disorders, until he was physically no longer able to do so. He was a giant, a rock and someone I miss every day.

I am the Homocystinuria rep for the Patients and Providers for Medical Nutrition Equity Coalition. I am proud to stand in the place of those who came before me, through this work I have found my voice. Through this work, I have realized I am not a monster; I didn't deserve what happened to me, I firmly believe that we owe it to the next generation to do better, so they don't have to go through what we did. If we want more for the next generation of patients, then we have to take actions that support, protect and defend them throughout their whole lives.

A patient's story is never just the medical aspect of a diagnosis, it's all the things we choose not talk about, it's all the things we have gone through, and continue to go through. So that's it, that's my story. I don't know what the end will be, but I know it's something of my own choosing.

SAVE ROOM FOR DESSERT

Click picture or name for recipe

NO BAKE CHEESECAKE

MAKES 3 SERVING
SERVING SIZE: 1 SLICE
PROTEIN PER SERVING: 1.5 G
CALORIES PER SERVING: 210

BREAD PUDDING

MAKES ABOUT 6 SERVINGS
SERVING SIZE: 102 G
PROTEIN PER SERVING: 1.0 G
CALORIES PER SERVING:205

CHOCOLATE PRETZLE PIE

MAKES 8 SERVING
SERVING SIZE: 1 SLICE
PROTEIN PER SERVING: 0.4 G
CALORIES PER SERVING: 229

SPRINKLE COOKIES

MAKES 18 SERVINGS
SERVING SIZE: 1 COOKIE
PROTEIN PER SERVING: 0.1 G
CALORIES PER SERVING: 113

MAKE A DIFFERENCE, LET YOUR VOICE BE HEARD!

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

HCU Network America

Will you be our next HCU patient Hero?

Tell us:

- How you or your child was diagnosed?
- How has HCU affected you, your family and relationships?
- What are some of your successes with HCU
- What are some of your challenges you have faced?
  - How have you overcome them?
- What words of advice would you give to newly diagnosed families?
- For other patient stories, visit: https://hcunetworkamerica.org/patient-stories
- Email your story to: info@hcunetworkamerica.org

Rare Bears for Kids with HCU

RARE science
Accelerating Cures 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

SURPRISE YOUR FAMILY!

A HCU GIFT FOR EVERYONE

https://www.bonfire.com/store/hcu-haberdashery

NEWS YOU SHOULD KNOW!

- **Retrophin Announces Corporate Name Change to Travere Therapeutics, Inc.**
  - SAN DIEGO, Nov. 16, 2020 (GLOBE NEWSWIRE) -- Retrophin Inc., (NASDAQ: RTRX) announced that the Company has changed its global corporate name to Travere Therapeutics, Inc. This new name reflects the Company's steadfast dedication to helping people as they navigate life with rare disease as well as the forward momentum of its promising pipeline of potential first-in-class therapeutic candidates. In conjunction with the name change, the Company expects to begin trading under the new ticker symbol "TVTX" on the Nasdaq Global Select Market at market open on November 19, 2020.
    - Read the full press release here: https://ir.travere.com/news-releases/news-release-details/retrophin-announces-corporate-name-change-travere-therapeutics
- **Aeglea Presents Poster at ASHG on Development of a Novel Human Engineered Enzyme (ACN00177) for the Treatment of Homocystinuria**
  - Abstract: https://www.abstractsonline.com/pp8/#!/9070/presentation/2479
  - Poster: https://ir.aegleabio.com/static-files/c8126df8-e7a9-48c8-96a8-b51c897349ed
- **Aeglea BioTherapeutics Receives FDA Rare Pediatric Disease Designation for ACN00177 for the Treatment of Homocystinuria**
  - AUSTIN, Texas, Dec. 1, 2020 /PRNewswire/ -- Aeglea BioTherapeutics, Inc. (NASDAQ: AGLE), a clinical-stage biotechnology company developing a new generation of human enzyme therapeutics as innovative solutions for rare and other high burden diseases, today announced the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease Designation to ACN00177 for the treatment of Homocystinuria, a serious metabolic disorder characterized by elevated plasma homocysteine which leads to a wide range of life-altering complications and reduced life expectancy.
  - Read full press release here: https://ir.aegleabio.com/news-releases/news-release-details/aeglea-biotherapeutics-receives-fda-rare-pediatric-disease

Open Enrollment for Health Insurance ends December 15 - ACT NOW!

Do you find your insurance coverage inadequate for low protein foods, formula, betaine, or supplements? Don't fret - there is still time to make a change, but you need to act soon!

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

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

There is no fee to work with Raenette, but we do urge you to contact her immediately if you do need a new policy. Open enrollment for 2021 ends December 15, 2021.

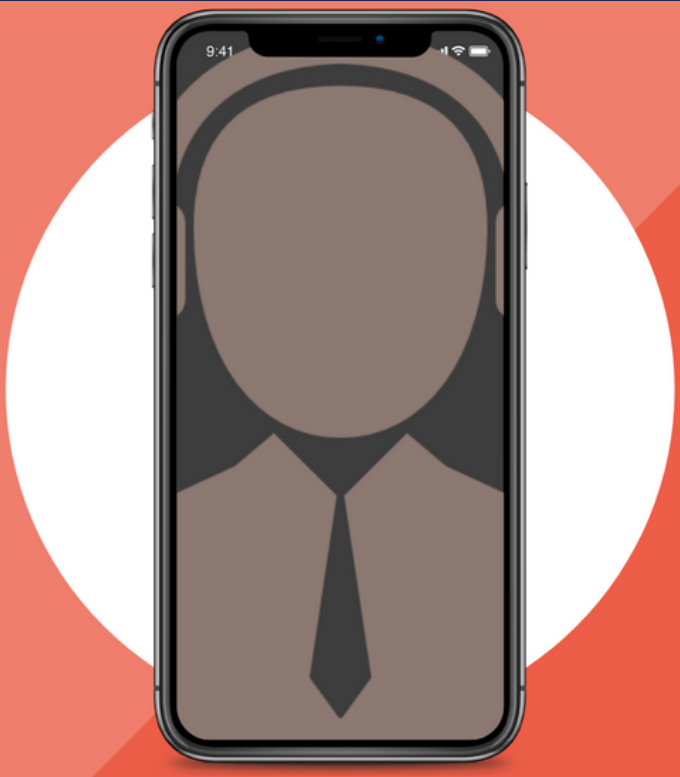
You may contact Raenette:
raenettef@compassionworksmrs.com | (973) 832-4736

UPCOMING EVENTS

HCU Community Virtual Meet-up

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

Sunday, December 13, 2020 @ 7 pm CT/8 ET
Monday, December 28, 2020 @ 1 pm CT/ 2 ET



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.

Sunday, December 20, 2020 @ 7 pm CT/8 ET

Organic Acidemia Association
www.oaanews.org

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/

- **Make an impact on federal policy from close to home.**
- **Share you rare disease story.**
- **Meet other rare disease advocates.**

Haz clic aquí para obtener información en español y para registrarte.

WHEN

February 22 - March 5, 2021 (Virtual Congressional meetings on March 3rd and 4th)

WHAT

Virtual meetings with your Senators and Representative AND virtual advocacy events. Rare Disease Legislative Advocates (RDLA) organizes meetings for rare disease advocates with their Members of Congress and/or the Member's staff. Meetings will take place virtually on March 3rd and 4th. The RDLA team also helps to prepare advocates for their meetings, provides legislative resource materials, and hosts pre-meeting training webinars.

SCHEDULE OF VIRTUAL EVENTS

Week 1: RARE Readiness

Monday, 2/22: Selfie Monday—Post a picture on social media and tag #RareAcrossAmerica
Wednesday, 2/24: Fast Forward for RARE Practice Your Pitch Webinar
Thursday, 2/25: Virtual Rare Disease Caucus Briefing
Friday, 2/26: Share Your Rare Story—Take a video and post on social media and tag #RareAcrossAmerica

Week 2: RARE Rally

Monday, 3/1: NIH Virtual Event
Tuesday, 3/2: Prepare for Your Virtual Hill Meetings (resources at www.RareAcrossAmerica.org)
Wednesday, 3/3: Virtual Hill Meetings with Senators (12-5 pm ET)
Thursday, 3/4: Virtual Hill Meetings with Representatives (12-5 pm ET)

No prior experience necessary. Registration for all RDLA events are free for rare disease advocates.

SAVE THE DATE: The Rare Across America training webinar will take place on 02/04/21 at 2:00 pm ET, First Time Advocates Webinar on02/09/21 at 2:00 pm ET, and the Team Leaders Webinar on 02/11/21 at 2 pm ET.

HCU Network America, Organic Acidemia Association, Propionic Acidemia Foundation | 2021 Conference

LAND OF THE FREE, HOME OF THE BRAVE

June 26-27, 2021 | Bethesda, Maryland

Homocystinurias Schedule of Events

07:00	Registration and Vendors Open	
07:30	Breakfast	
08:00	Kids Zone Opens	
08:30	Welcome to the HCUNA/OAA/PAF Conference	
09:00	Keynote Dr. Gerard Berry, MD	
09:30		
10:00	Vendor Break	
10:30	What are the Homocystinurias? Dr. Kimber Chapman, MD, PhD and Dr. Irini Manoli, MD	Breakouts TBD
11:00		
11:30	Lunch	
12:00		
12:30		
01:15	Cobalamin and Severe MTHFR Research Update Multiple Speakers: TBD	Breakouts for low protein die
01:45		
02:15	Vendor Break	
02:45	Classical HCU Research Update Multiple Speakers: TBD	Breakouts TBD
03:15		
03:45	Vendor Break	
04:00	Patient-Expert Panel	
04:30		
	Evening Break	
05:30	Cocktail Hour (Cash Bar)	
06:30	Dinner Reception	

*Agenda is subject to change

Homocystinurias | Organic Acidemias

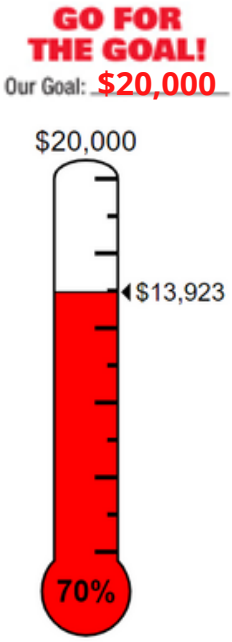
07:30	
08:00	Breakfast and Vendors Open
08:30	Group Picture
09:00	Emergency Preparedness Dr. Mark Korson, MD
09:30	
10:00	Vendor Break
10:30	Navigating Health Insurance Raenette Franco
11:00	
11:30	
12:00	Closing Remarks and Lunch

*Agenda is subject to change

To learn more or register, visit:

https://hcunetworkamerica.org/2021-conference

$20,000 DONOR MATCH IS BACK!



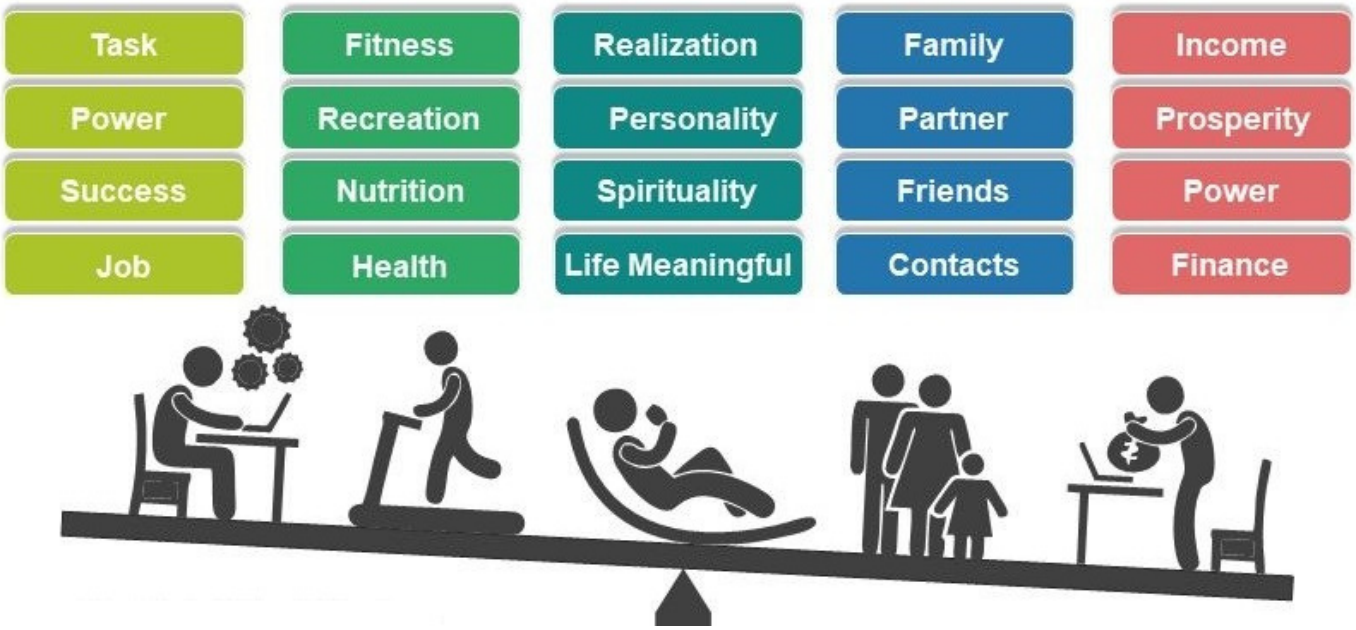
That's right, you heard us right!
Thanks to an anonymous donor, any **funds** you help raise through December 31, **will be matched up to $20,000!**

Don't forget to submit your donation receipt to your employer so it can be matched!

We are asking every patient and family to help us raise funds for homocystinuria. During the winter holiday's warm hearts and generosity can be felt near and wide. During this time, we ask that you share our appeal letter with your colleagues, friends and family.

See our appeal on the last two pages!
You can also print it here

Here are the steps to see if your company matches gifts!



Balancing Life and HCU

by Danae' Bartke

Life can get be busy; work, kids, school, extra-curricular activities and that's just the beginning. Now you add the demands of HCU to the pile and you feel ready to tap out. Unfortunately though for life there is no one to jump in and take over for when the responsibilities just seem like they are too much – this is the reality for parents and patients with Classical HCU. Know you aren't alone and we are here to help!

While life seems a bit crazy right now with the uncertainty of everything, we do have some advice on how to fit HCU into the picture without adding to the stress. While each piece does require time, they all will help save time in the long run.

- **Your time is limited, prioritize:**
  - There are 24 hours in a day and you ultimately have to decide what you are going to say yes or no too. Many have a hard time saying no, but in order to make room for priorities you have to learn to say no to things that might not matter as much or just less. This may mean instead of watching your favorite TV show one night, you sit down and meal plan or order your low protein foods.
  - Pro tip: Establish a schedule for when you are going to order and take your low protein foods and formula. Also set a consistent time for when you are going to fill your pill container for the day, week, or even month. Put it into your electronic calendar and set a reminder so you don't forget!

- **Meal Plan:**
  - While meal planning initially takes some time, it will save time and help keep you on diet in the long run. Those days where you are short on time, you won't have to figure out what you are going to make, because it's already decided for you. Meal planning saves a lot of time and ensures you have the products and supplies on hand. Sit down and figure out what you are going to make for the week and then add it to your grocery list. Try to make the protein a side, rather than the star of a dish. That way your regular protein meal and low protein meal can be basically the same. Instead of throwing that meal plan away, file it away in a 3 ring binder for reuse later on.
  - Pro tip: Add the protein and any other nutrient information to each meal so you can quickly add it to your daily log. **(We included a sample without nutrient info below for you)**

- **Cook and Freeze Extra:**
  - Again, since you are meal planning, plan on making extra! We inevitably all have those evenings where either something comes up, or you just can't fathom cooking. When you do cook a meal, cook extra and freeze it.
- **Educate and ask for help from those close to you that you can trust:**
  - Without a doubt there will be a time where something comes up and you need help. We cannot recommend enough finding a friend, relative, or babysitter – or maybe all 3 and educating them about Homocystinuria – what it entails, how to read labels, how to prepare the food, where to find low protein items. This way you can get the well-deserved break you need. It's important for your mental and emotional health you find people who can help you carry this load.
  - As your child grows, do not forget to start instilling some self-care skills and teaching them how to take over their diet– they will one day become an adult and need to know how to do all the stuff that HCU encompasses! Bonus is, that as they become independent with their care– you can remove it from your plate!

Remember, there is no one that can tell you exactly how to make HCU work for you and your family, but remember there is a community behind you to support you and cheer you on along the way!

	Thursday 11/12/	Name	Items	Directions
2	Lunch	Open faced grilled cheese	2 slices of cambrooke bread	
3			Violife sliced cheese	
4		Veggie straws	28 grams	
5	Dinner	Sloppy janes	90 grams of mixture	warm and top on bun
6			Ener-g hamburger bun	
7		Green beans		
8	Dessert	Cambrooke cookie		
9				
10				
11	Friday 11/13	Name	Items	Directions
12	Breakfast	Hashbrown Patty	One	
13		bacon' patties	country sunrise bacon (in pantry)	prepare as directed. form into rounds and Saute until browned.
14		Free Fruit		Serve any free fruit
15				
16	Lunch	Tacos	2 siete taco shells	warm as directed
17			30 grams mushroom	saute
18			taco seasoning	mix with cooked mushrooms
19			1 cup Shredded Violife or FYH Cheese	Top cheese on prepared tacos
20		Rice	52 grams dry rice	Cook until tender
21			mix with taco seasoning and butter	
22	Dinner	Pierogies	5 pierogies	boil water andl add frozen pierogies. cook until tender then saute in buttered pan until slightly crispy
23		Free vegetable		
27	Saturday 11/14	Name	Items	Directions
28	Breakfast	Butter bread	2 slices Cambrooke bread	toast and butter
29			1 tablespoon Butter	
30			Free Fruit	
31	Lunch	Spaghetti & marinara	52 grams pasta	
32			any amount marinara	
33			grated violife parmesan (in right door of cooler)	
34		Applesauce	Any amount applesauce	
35	Snack	Wise onion rings	1 bag	
36	Dinner	Pizza	Mellow mushroom pizza	SHE CAN HAVE HALF OF THE PIZZA---Gluten free, vegan cheese, mushroom
37		Marinara rice	52 grams rice (weigh dry)	cook with oil and salt
38			any amount marinara	mix with butter, salt, and marinara
39			Violife parmesan	grate on top of rice/marinara
40		Free vegetable		
41	Dessert	Cambrooke cookie		

HCU Network America
15 S Mallory Ave
Batavia, Illinois 60510

(630) 360-2087
info@hcunetworkamerica.org
http://www.hcunetworkamerica.org
Tax ID Number: 81-3646006

The year 2020 has been an extraordinary challenge to us all. Everyone at HCU Network America hopes that you and your loved ones are managing to get through this safely, and if you have suffered from COVID-19 illness or losses in any way, you have our deepest sympathies.

These times are extra demanding for people with unique medical needs and special diets, who spend time at clinics and waiting for life giving medical supplies. People with HCU have to balance all the variables in their lives that affect their health and that is tough, especially this year, and can sometimes seem overwhelming.

HCU Network America is here to help this special group of people with the support and resources they need to navigate daily life. We are proud of the reach we have and the way these communities have knit themselves together. But we can't do it without your help! As a 501(C) (3), we need your donations, which are tax deductible, to continue with our mission and meet our goals. What you do to help us, can make a huge difference in the lives of all HCU patients and their families.

We would like to share a story of a family's journey of diagnosis.

> *Our daughter had just turned 3 in the spring of 2012 when she suffered a large blood clot on the brain and a series of small strokes. After a week's stay in a children's hospital we were released without any explanation as to the cause of the large blood clot. Six weeks later, during our follow up, the doctors ordered extended blood tests which resulted in a test coming back with elevated homocysteine levels. The level of homocysteine in our daughter's blood was so high that it was actually toxic to her body and prone to clot. It was shortly after this test result came back that we had our explanation to the blood clot as well as a new lifelong diagnosis - our daughter had Homocystinuria. During our crash course on this rare metabolic disorder we learned that she was born with this & that it is hereditary - it was recommended that we have our 18 month old son tested as well. His results came back positive - both of our children were diagnosed with HCU. Homocystinuria was missed on initial newborn screening for both of our children and the state we reside in doesn't offer a second newborn screening where HCU should be flagged. Thankfully the doctors were able to diagnose our daughter correctly after her event and fortunately we were able to get our son on the treatment regimen preventing any events for him. So began our journey with Homocystinuria.*

You can imagine what that felt like and at that time there was no HCU Network America to give this family support and vital information. In June of 2016, HCU Network America was incorporated; bringing hope to families living with HCU that they had advocates to help them get the latest and best advice from the medical community. HCU Network America also financially supports research that can help find new treatments. Since 2016 we have communicated with metabolic clinics all over the country to reach out to new patients and provide them toolkits which are filled with helpful tips and guidelines for living with HCU.

Here are some of the highlights that your donations helped with:

1. Help fund Research Grant: In 2020, HCU Network America, in partnership with HCU Network Australia, issued its second research grant in the amount of $40,000 to Dr. Tomas Majtan, PhD from the University of Colorado Anschutz Medical Campus, Aurora for the evaluation of benefits of thiol-based reductants in classical Homocystinuria
2. Expand understanding and awareness of the need for new therapies for Classical Homocystinuria by conducting Patient-Listening Session with the FDA
3. Distributed Improved Newborn Screening Talking Points for the Homocystinurias to medical professionals and professional Inherited Metabolic Disorder Organizations
4. Helped secure session on improved Newborn Screening for the Homocystinurias at the Association of Public Health Laboratories 2020 Newborn Screening Symposium
5. Published an Emergency Planning Guide with Emergency Protocol Template for all Homocystinurias
6. Published educators guide for Cobalamin Disorders and MTHFR
7. Provide a consultant with experience in the insurance industry to fight for coverage for medications and food at no cost to HCU patients
8. Support for bi-monthly community meetups

Our goal this year is $50,000. Thanks to an anonymous donor, any funds you donate up to December 31, 2020 will be matched up to $20,000. Please consider a donation to HCU Network America in 2020. We need your help and appreciate any donation. In addition, if your employer matches charitable donations, they will match those too!

Take a minute to look at our website to see what we are up to and meet some our "heroes": https://hcunetworkamerica.org. You can donate through our website or by mail.

Thank you for all you do to help us - we will all get through this year and we look forward to 2021!

Thank you,
Danae' Bartke
HCU Network America, Executive Director

Donor levels:

- Leadership Circle - $5,000 or more
  Donor's name, HCU Patient's name and photo on homepage of website, along with certificate donation
- HCU Champion - $1,000 or more
  Donor's name, HCU Patient's name and photo on HCUNA donation page along with donation certificate
- HCU Supporter - $500 or more
  Donor's name, HCU patient's name on website, along with a donation certificate
- HCU Ally's - $100 or more
  Donors name on website along with a donation certificate
- HCU Friend - $99 or less
  Donor's name listed on website