Connecting for a cure. There have been great things happening for the HCU Community and HCUNA. We strive to keep you informed and connected.

The HCU Herald

In This Issue

HCU Network America

Recipes from the Kitchen: *Back to School Edition*

Rather than provide you with a bunch of recipes (this newsletter would be endless), this month we thought it would be beneficial to provide you with some ideas for snacks and cold lunches. If there is a recipe, we will provide a hyperlink in the snack or meal name.

Snacks

Image Dish: Cammus
Guacamole and Tortilla Chips
Caummus and Tortilla Chips
Veggies and Dip
Veggie Straws
Loprofin Snack Mix
Yogurt (So Delicious or Chobani)
Crackers and Cheese (Chao, Follow Your Heart, Daiya, So Delicious)

Image Dish: Mini Churros
Fruit and Cool Whip
Crunchy Granola Bars
Spiced Pretzels
Low Protein Pretzels (Snyders Gluten Free or Glutino)
Flavis Fruit Bars

Sandwhiches | Wraps| Paninis

Image Dish: Phelafel Wrap
Grilled Cheese and Tomato
Caummus and Veggie Wrap
(many tortilla recipes on Cookforlove.org)
Jackfruit "Tuna" Salad
Quesadilla with Salsa
Phelafel Wrap
Honey and Banana
Better BLT (Use Morning Star Bacon or Homemade carrot, eggplant, or sweet potato bacon)

Image Dish: Broccoli Soup
Mac and Cheese
Ditalini and Marinara
Veggie Fried Rice
Cheesy Broccoli and Rice
Pho
Vegetable Noodle Soup
Thermos meals from Cambrooke

Thermos Meals

Dear Methia

Can I attend camp and still be compliant?

My two best friends just came back from spending three weeks at a summer sleepaway camp. They did the coolest activities – hiking, swimming, games like "Capture the Flag," team sports, you name it. I am so jealous! I've never been to summer camp, and I really want to go next year. Mom and Dad said that their only hesitation in sending me to camp is managing my HCU – they need to make sure that I will be able to stay on diet and be compliant with all of my medications. I want to start gathering information so that they know I am serious and becoming more responsible. Can you help me?

Sincerely,
Unhappy Camper

Dear Unhappy Camper,

The idea of summer sleepaway camp can be a scary thought for all parents, even parents of kids without special dietary restrictions. It's fantastic that you are thinking about what you will need to do to minimize the stress and be as prepared as possible ahead of time! Here's how to get started:

- *Contact the Camp Director and Food Service Manager.* Although HCU is not technically an "allergy" to methionine, it should be treated just as seriously. Many summer camps are used to preparing special menus for kids with food allergies. In fact, some camps even have a Food Allergy Director. This team of people is familiar with the necessary steps required to keep kids safe with special dietary needs. They will likely request that you and your family fill out some forms about your diet, what your restrictions are, and how to manage any dietary indiscretions that may occur while you are there.

- *Put your metabolic clinic in contact with the nurse on site.* Sleepaway camps are required to have a licensed physician or nurse on site at all times during the day. Your geneticist can write and send orders for you to have your formula, betaine, and other medications at specified times during the day. Additionally, ask your clinic to provide an emergency protocol (just in case!) for the camp to keep on file.

- *Put some meal plans together.* Your metabolic dietitian is able to work with you to put together meal plans for the camp to follow. Through the analysis of camp recipes, rotating menus can be created so that you meet your caloric and protein needs daily (but also don't have to eat the same thing every day!). For the foods and drinks that the camp does not already supply, many have storage space so that you can bring your own labeled (low protein) foods. Additionally, discuss any special preparations with the chef ahead of time. For example, if there is a low protein pasta recipe that you love at home, perhaps the camp chef can create it for you!

Keep in mind that going away for the summer requires the cooperation of many people, including your metabolic geneticist, dietitian, camp director, camp staff, your family, and especially YOU! All pieces of the puzzle must work together to make your experience fun and successful. In doing your research, you will discover what summer camps and activities are a good fit for you and HCU.

Sincerely, Methia

Rare Bears are back *Limited time act fast !*

We are proud to partner with RARE Science, an all-volunteer, non-profit research organization that focuses on accelerating the identification of more immediate therapeutic solutions for kids with rare disease potentially through repurposing currently approved drugs and other therapeutic approaches. By combining efforts HCU Network America and RARE Science hope to bridge the divide in finding treatment, therapies and support for the HCU community. 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/

Open to those 19 years and younger
Those who have already received a bear, are not eligible
All requests need to be received by August 29, 2019.
We will be gifting and shipping RARE Bears out to families on Saturday, September 28, 2019!
hcunetworkamerica.org

HCU Network America

Featured Recipe: *Cheesecake Stuffed Strawberries*

These are a delicious treat to pack in your child's lunch as they head back to school!

Servings: 12
Serving Size: 1 Strawberry
Protein per serving: 0.2 g
Calories per serving: 31

Ingredients:

12 berrie(s) Strawberries, fresh, medium
1/4 c Cambrooke Cream Cheese, Plain, room temperature
1 TBSP Sugar, Powdered (Confectioners)
1/4 tsp Vanilla Extract
2 TBSP Butter, regular or unsalted, softened

Directions:
Rinse strawberries and pat dry. Use a paring knife to remove the tops of the strawberries. Scrape the inside of the strawberries to make a hollow cavity for the filling. Set aside.

Combine all the filling ingredients in a small bowl and mix until combined. Cover and refrigerate for 5 minutes. To fill the strawberries you can use a pastry bag fitted with a star tip or you can spoon the filling into each berry. Fill and serve right away or cover and refrigerate until ready to eat.
Notes : The size of the strawberries may vary, so try to take that into account.

*Advertisements are not an endorsement for products advertised.

HCU Heroes

Lindsey

From Washington

When I was 11 years old I started noticing I was having trouble seeing the chalkboard at school so my dad took me to have my eyes checked. They said I had a slight Astigmatism in both eyes, and I got my first pair of glasses. From then on, my eyesight progressively worsened. When I was about 14 years old, the eye doctor noticed that the lenses in my eyes had started to drop and the little threads that held the lenses in place were disintegrating. No one understood why nor did they recommend further testing. I did not have insurance and my parents were low income so I just lived with my eye issues. Every time I got new glasses the lenses were thicker and thicker.

When I was about 22 I started noticing that when I bent over, something would happen to my eyes and my pupils would get really big. I finally made an appointment with an ophthalmologist and he explained to me my lenses were completely dislocated, and when I bent over the lenses would fall to the front of my eyes, and I needed to have surgery to fix it. I was told it was approximately $20,000 per eye. I had no i nsurance and no money. At this point, I still was not given an explanation as to why it was happening. So again, I just lived with it.

Fast forward about 10-12 more years. I started getting really sick and the doctors couldn't figure out why. I was living in Idaho and had several blood tests to try to figure out what was wrong with me. The doctor finally put a generic name on it and said I had an autoimmune disorder. I ended up moving back to my home state of Washington. I needed new glasses so I made an appointment with an optometrist, an amazing man that wanted to help me get surgery even though he didn't know why my lenses were the way they were. He referred me to the Pacific Cataract and Laser institute.

Before I went to the institute, the optometrist dilated my eyes like any normal eye appointment. I went home and that night I had bent over while giving my daughter a bath and my lenses went in front of my pupils. Normally all I had to do was lay back and cover my eyes and they would go back to normal, but this time they didn't and I had to go to the Emergency room. They ended up not being able to help me but called the Pacific Cataract institute after hours and they told me to come in first thing in the morning. I was scared out of my mind thinking I was going to go blind. I got there first thing the next morning and they put the dilation serum in my eyes and eventually they went back to normal.

HCU Hero: Lindsey

The doctor told me it could be due to one of a few things that were going on with me, and he expected it was Homocysteinemia. So he contacted my primary physician to have my homocysteine levels checked. It took about 5 days for the results, but when they came back my levels were 350.

My doctor didn't know anything about Homocysteinemia so she had to do some research. They put me on high dosages of vitamin b12, b6, and folic acid, as well as baby aspirin. Six weeks later they tested my levels again, which came back at 175. It was better than 350, but still not good. Since the levels were coming down they had me continue taking the dosage and I had my levels checked every 6 weeks for 6 months. The lowest it got too was 150, but kept going up and down. My primary physician referred me to a blood specialist because she didn't know what else to do for me. They ended up increasing my doses and monitoring my levels for the next 6 months with no success of it going lower than 125 but kept yo-yoing.

So they finally referred me to a geneticist. It took about another year, but now my levels are finally starting to come down and stabilize after being put on Cystadane, Foltanx, and a limited protein diet. My last level check was 27 and I just had my 39th birthday in April 2019. Woohoo! I did have my lenses replaced when I was 34.

HCU Hero: Lindsey

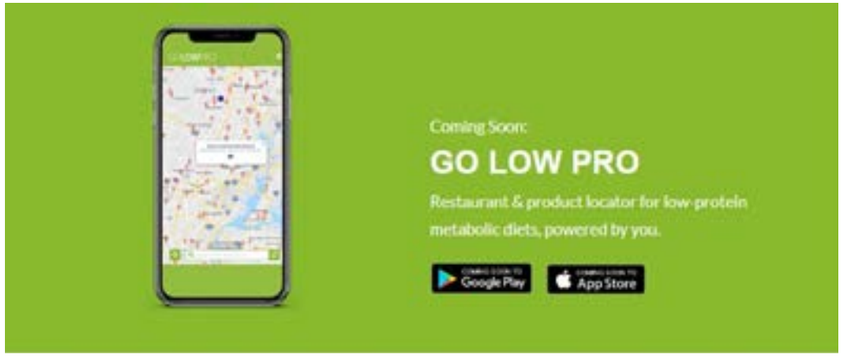
HCU has greatly affected my life in a negative way. I have had severe headaches and migraines ever since I can remember. I was always coming home from school and work because of the debilitating headaches. I couldn't participate in sports as a result of my eye condition. Since doctors couldn't figure out what was wrong with me, my children's father thought it was all in my head which strained our relationship to the point of separating.

Some successes I would say was having my eye surgery and restoring my eyesight and finding the right doctors and medications to finally stabilize my homocysteine levels. Words of advice: be an advocate for yourself or family member going through this genetic disorder. Never give up trying to find the right doctors to help along in the journey. Take the necessary medications even though there will be some days you just want to throw in the towel. Oh, and join a support group like this one! It helps knowing you're not alone and/or crazy. I was officially diagnosed when I was 34 years old in 2014.

Are you the next HCU Hero ?

A Hero is an ordinary individual who finds the strength to persevere and endure in spite of overwhelming obstacles.
– Christopher Reeve

Go Low Pro *(an App): Taking Low Pro on the Road*



We all know managing the low protein diet takes a lot of planning, and sometimes it's just not possible to be THAT prepared. Or maybe you're traveling and need a few low-protein staples, stayed later than you expected at work, and need to refuel while staying within your allowance. Maybe you're a teen who is suddenly finding it harder to stick to diet now that you're out and about on your own more often.

Go Low Pro, a new smartphone app from National PKU News aims to eliminate the unfair situation so many who require low-protein diets often face: go hungry or go over your protein allowance; stay with your friends or go home and eat your low-protein foods, alone. By leveraging Google Maps, the store data of the members of the Partnership for PKU, and the power of thousands of community members on low-protein diets, we'll create an always-updated directory of restaurants and stores compatible with the low-protein diet, all accessible from your smart phone.

Go Low Pro, a smartphone-native app (iOS and Android) will allow the user to find:
1. Restaurants that serve meals compatible with a low-protein diet
2.Restaurants that are accommodating to a low-protein diet (allow substitutions without charge or allow you to bring your own pasta or low-protein tortillas)
3.Stores that sell products that work with a low-protein diet

GoLowPro isn't just for people with PKU or to replace diet management tools. Since the app focuses on restaurants and products compatible with the low-protein diet, it will be a fantastic tool for a full range of metabolic communities. We're also looking forward to working with our colleagues in the UK, Ireland, and beyond to incorporate locations in those countries.
To learn more visit: https://golowpro.org

Back to School with HCU

August means back to school! Exciting new backpacks, notebooks and pencils! For caregivers of children with HCU there is also anxiety: Will the staff understand HCU? Will they follow the diet as well? Will other students tease my child for being different? We are here to help!

First, download and print our Educators Guide to HCU and give a copy to your child's teachers. In our Educators' Guide we give you an easy explanation of HCU, Helpful Tips for Teachers and Nurses, Educational and Nutritional Accommodations tools, as well as ways to ensure students are not left out of classroom celebrations. Schedule a time to speak with the school nurse and teachers about HCU.

Think about how your student's diet will be accommodated. Federal law requires that "substitutions to the regular meal must be made for children who are unable to eat school meals because of their disabilities, when that need is certified by a licensed physician." This is true for any school accepting USDA funds. The law also states that families cannot be charged extra to be provided substitutions on the menu. This means you pay whatever the regular cost is for students.

Although it's required by law, the law does not provide funding and schools must look within their own budgets to purchase these medical foods. Examples include the food service budget, district general funds, and Parent-Teacher Organizations. Many schools will tell families that there is absolutely no funding available to provide meal substitutions. This does not release them from the requirement.

Schools can use resources like Cookforlove.org which uses many store bought items and limited medical low protein items. Many grocery stores also carry items and brands that happen to be low or lower in protein. Please work with your school and provide them this list – if you need assistance, please reach out to us. To learn more about the National Lunch Program, please watch this webinar from Georgia PKU Connect – School Lunch Program: It's Your Child's Civil Right or view the Cambrooke School Lunch Program Materials.

With that being said, some kids will still prefer to take their own lunch. An additional accommodation you can have added is that your child will have access to a microwave – this could be one in the school cafeteria or in the staff lounge. Cold lunches do not have to be boring, so get creative and involve your child in meal planning!

Remembering Jan Kraus

June 5, 1942 – July 3, 2019

HCU Network America mourns the loss of another metabolic hero and pioneer, Dr. Jan Kraus. Jan passed away on Thursday, July 4th after a courageous battle with lung cancer.

Jan was a Professor in Pediatric - Clinical Genetics and Metabolism at the University of Colorado. Jan, considered by many as the "Father of Homocystinuria", dedicated his life's work to the understanding, diagnosis and treatment of Homocystinuria (HCU) and Proprionic Acidemia (PA). Since the 1960's, Jan has authored and co-authored over 160 publications regarding HCU and PA. Jan's career highlights include building a database of all the genetic mutations associated with Homocystinuria, as well as being the inventor for the OT-58 product (a pegylated version of the CBS enzyme) that is in human trials for Classical Homocystinuria patients. The community will never forget Jan and his commitment to patients and families suffering from these diseases, and we send our sincere condolences to his wife Eva and family.

"Jan was a committed and bright researcher who wanted to use his talent to alleviate and prevent suffering from rare metabolic diseases. I first met Jan in 2009 when I was searching for information on how to screen family members for HCU, and he helped my family unravel the genetic puzzle of the defects that caused HCU in my sisters, determine which family members were carriers and how we could screen their children and partners to give peace of mind that HCU would not be passed along. I was so excited to hear about the Enzyme Replacement Therapy he had discovered, which he made sure advanced into development so it could get to patients. Jan also helped set up the first ever patient/family meeting in Denver in 2011, and encouraged us to establish HCU Network America so we could help patients and families deal with the disease. He also served as a member of our Global Scientific Advisory Board for HCU Research Grants. I will miss Jan and his kind demeanor and warm smile, but know his legacy lives in though his research contributions as well as the Researchers he mentored, including Tomas Majtan at the University of Colorado and Viktor Kozich at Charles University in Prague." - HCU Network America President, Margie McGlynn

VMP, LLC
expanding genetic horizons...

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HELP US TEACH PHYSICIANS ABOUT HOMOCYSTINURIA

FACT! Teaching about metabolic diseases in medical school and residency programs is poor.
FACT! Most patients live and die without a diagnosis being made, especially when the disease presents in adulthood.
FACT! Patients cannot access effective therapies unless a proper diagnosis is made.
FACT! The sooner a diagnosis is made and treatment begun, the better the outcome.

WE NEED YOUR HELP!

We at VMP Genetics believe in the power of "patient-teaching" and are bringing patients and families into lectures and presentations - at conferences and in the classroom around the country. While doctors teach facts, patients tell stories. Story-telling is a more compelling teaching method with better recall over time than didactic lecturing. We also believe that doctors are more likely to make a diagnosis if they have already seen a patient and heard her/his story. Story-telling can be live or taped...

WE ARE LOOKING FOR...

- ***Patients and/or family members who are interested in telling their stories in local medical classroom settings...*** We are developing a Patient Teacher Registry. If a medical school faculty member is looking to introduce the patient story in a teaching session, the Registry can tell him/her if there are patient-speakers in the area and what diagnoses they have.

- ***Patients and/or family members who are interested in having their stories videotaped...*** As we secure funding, we are interested in recording stories that reflect the broader patient experience. The more variety in the stories, the richer the learning potential.

- ***Videos of patients and families telling their stories...*** A 5-or 10-minute clip can be downloaded into a lecture about that disease or relevant biochemistry to enhance the learning potential of the session.

Please help us in our efforts to raise awareness about Homocystinuria through this innovative educational outreach to the medical community. To Volunteer or participate, or for more information about this project... please contact Jacob Athoe at PatientTeacherRegistry@vmpgenetics.com

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OT-58, Enzyme Replacement Therapy Clinical Trial Recruitment

Orphan Technologies has initiated a first in human (Phase 1) clinical trial of OT-58, an enzyme replacement therapy that addresses the underlying enzyme defect for patients living with classical homocystinuria. The goal of this trial is to evaluate the safety and efficacy of OT-58 in patients with classical homocystinuria and identify the appropriate dose. Patients between the ages of 12 and 65 years of age with classical homocystinuria may be eligible to join. For additional information on criteria for eligibility, please go to:
https://clinicaltrials.gov/ct2/show/NCT03406611?cond=Homocystinuria&rank=1
There are four sites in the US currently participating in the trial:
• Children's Hospital of Philadelphia – open to patient enrollment
• Boston Children's Hospital – open to patient enrollment
• Indiana University – open to patient enrollment
• Children's Hospital Colorado - open to patient enrollment
Payment for time and travel may be available to patients who participate in this trial.
To inquire about participation into the trial, please email: info@orphantechnologies.com

Contact Register

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

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

How do I participate?
The contact register form takes approximately 3-5 minutes to complete. You can find the form either by visiting our website and clicking on the "Contact Register" tab, or you can fill it out by going directly to: https://hcunetworkamerica.org/contact-register/

We'd like to thank the following content contributors:
Editor in Chief: Danae' Bartke
Heroes of HCU: Lindsey from Washington
HCU and You: Recipes from the *Kitchen:* Amber Gibson
Ask Methia: Angela Pipitone
Change Wars: Fundraising Committee
Back to School Guide: Patient-Parent Advisory Committee

Want to get more involved, but don't qualify for the Enzyme Replacement Therapy Trial – Drive research by joining the Natural History Study!

Current sites include: Atlanta, Boston, Denver, Indianapolis and Philadelphia. Joining the Natural History Study allows researchers to find out more about Homocystinuria and issues that patients face. Natural history studies help drive new therapies and a cure! If you qualify, we highly suggest you participate if there is a center in your area. You do not have to be a patient at one of these clinics to participate.

Click here to learn more

Want to register but not sure what you are going to do with your kids?
Don't worry – we have a children's program for children ages 5-12.
Register now at https://hcunetworkamerica.org/2019-conference

Vendor Scavenger Hunt
Build your own race car
Food Bingo / HCU Jeopardy
Dietitian lead activity
Movies
Relay Races
Low Protein Cookie Decoration
And more !

Fundraising Tip

Schools back in session, so get your kids and their school involved in a simple, yet fun fundraiser for Homocystinuria!