ACCELERATING TOWARDS A CURE

2019 HCU Network America Conference | Indianapolis, IN

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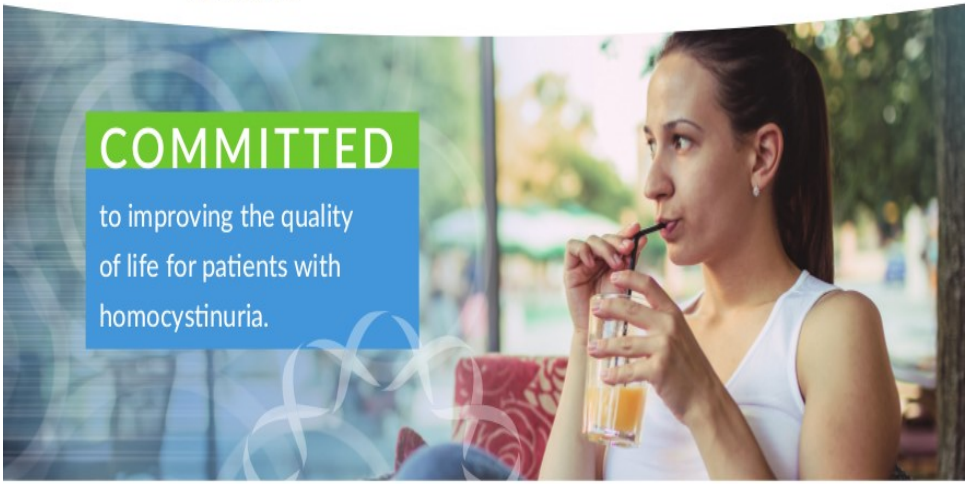
OrphanTechnologies.com



Orphan Technologies is dedicated to helping patients control their homocysteine levels.

Our lead program, OT-58, is an enzyme therapy that addresses the underlying CBS enzyme deficit for patients living with classical homocystinuria. The goal of OT-58 is to help patients reduce their homocysteine levels and restore a normal lifestyle.

Orphans Technologies has conducted rigorous preclinical evaluation of OT-58 and is now enrolling patients into a clinical trial in the US.

JOIN US AND MAKE A DIFFERENCE

Orphan Technologies has initiated a first-ever human study of OT-58*, a modified version of the human enzyme that is not functioning well in patients 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.

Orphan Technologies has an ongoing Natural History Study in classical homocystinuria to observe patients to learn how their disease is managed so as to provide information to researchers who are developing medications to treat the disease. This study does not involve any investigational medications and all exams are provided at no cost to the patient.

For more information contact us at: info@orphantechnologies.com

*For more information: https://clinicaltrials.gov/ct2/show/NCT03406611?cond=Homocystinuria&rank=2

HCU Network America

October 19, 2019

Dear Members of the Homocystinuria Community,

On behalf of the board of HCU Network America, we are so pleased you are able to join us for our second Patient/Family Conference, and we hope that you find it to be very informative and rewarding.

This meeting is all about helping you and/or your family member with HCU. Our goal is to help you as patients or caregivers more easily and successfully manage HCU. We know that patients who control their homocysteine levels thorough diet and supplements can avoid clinical issues and live a healthy and active life. But we also know that it can be complicated to adhere to diet or treatments, and to manage some of the clinical or other issues caused by the disease.

So what do we hope to accomplish with our two-day meeting? We planned the agenda based on feedback from our last meeting, and input from many of you as to what your greatest needs are, and we hope you leave here armed with resources and tools to help you address those needs, and information on new treatments in development to give you even better hope for the future. But as with the last meeting, we expect the most important take-away for all of you will come from the social interactions - the camaraderie you feel and the new friends you meet, and the ideas and support you all give each other.

We are proud of what we have been able to accomplish as HCU Network America, with your support, in our three years of existence. I hope you leave this meeting with even greater hope for the future and confidence that you or your loved one with HCU have a long, healthy and enjoyable life ahead of you.

Warm Regards,

Margie McGlynn
President, HCU Network America

THANKS TO OUR CONFERENCE SPONSORS

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A Special Thank You to:

The Cobalamin Steering Committee
The Fundraising Committee
The Patient-Parent Advisory Committee

SATURDAY LOW-PROTEIN

Breakfast

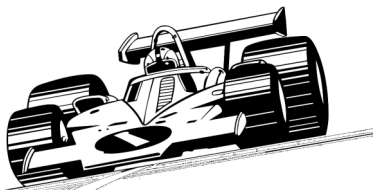
- *Egg Station (Using Country Sunrise Eggs)*
- *Mini Bagels and Cream Cheese (Cambrooke Foods)*
- *Homemade Low Protein Quick breads*
- *Seasonal Fruit*
- *Smoked Breakfast Carrots*
- *Morning Star Farms Veggie Bacon*
- *Yucca Taters (Cambrooke Foods)*
- *English Muffins (Taste Connections)*

Lunch

- *Bread (Taste Connections)*
- *Jackfruit Tuna*
- *BBQ Jackfruit*
- *"BLTs"*
- *Yucca Taters (Cambrooke Foods)*
- *Tortilla Chips (Cambrooke Foods)*
- *Toppings and Condiments of Choice:*
  - *Lettuce, Tomato, Pickles, Onions, Ketchup Mayo*
- *Dessert:*
  - *Cookies and Pie*

Dinner

- *Mixed Greens Salad*
- *Vegetable Medley*
- *Jackfruit Ragu*
- *Zucchini Lasagna*
- *Penne Pasta with Cream Sauce (Cambrooke Foods)*
- *Breadsticks (Cambrooke Foods)*
- *Dessert:*
  - *Cookies and Pie*



* Menu's are subject to change depending upon access to items

SUNDAY LOW-PROTEIN MENU

Breakfast

- *Egg Station (Using Country Sunrise Eggs)*
- *Mini Bagels and Cream Cheese (Cambrooke Foods)*
- *Homemade Low Protein Quick breads*
- *Seasonal Fruit*
- *Smoked Breakfast Carrots*
- *Morning Star Farms Veggie Bacon*

Lunch

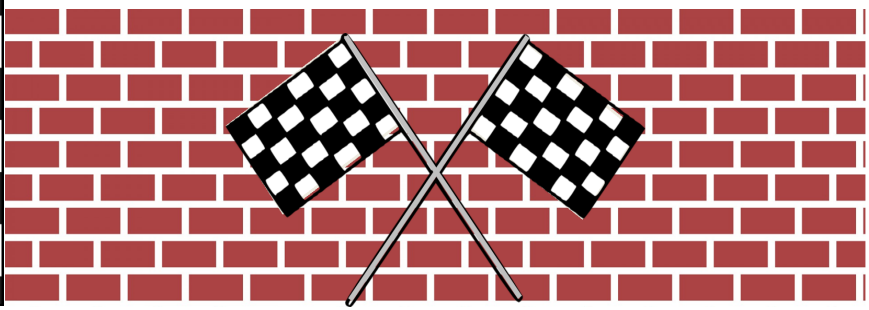
- *Garden Salad*
- *Coleslaw*
- *BBQ Jackfruit*
- *Low Protein Sloppy Joes*
- *Low Protein Corn Bread*
- *Slider Buns (Taste Connections)*

- *Dessert: Cookies and Pie*

* Menu's are subject to change depending upon access to items

We'd like to give a special thanks to Chef Amber Gibson and her husband Ben Gibson for helping prepare the delicious low protein food!

We'd also like to acknowledge Ajinomoto Cambrooke, Artisan Tropics, and Taste Connections for their generous in kind donations to help make our low-protein menu a possibility!



CONFERENCE SCHEDULE

Saturday, October 19

07:30-09:00 **Registration and Vendors Open**

07:45-08:45 **Breakfast**

09:00-09:45 **Introduction and Meeting Expectations**

09:45-10:45 **Natural History Study Update**
Presenter: Marcia Sellos, PhD
Orphan Technologies
In 2016, Orphan Technologies initiated the first comprehensive, longitudinal, prospective natural history study for classical homocystinuria. To date, we have enrolled close to 60 patients in 3 countries, with the majority of patients living in the United States. This study is providing novel insights into the clinical course of patients with classical homocystinuria on current therapy. In this presentation, we will discuss the interim findings of this study.

10:45-11:15 **Vendor Break**

11:15-12:00 **Best Practices in Treatment of HCU**
Presenter: Kimberly Chapman MD, PhD
Children's National Hospital
Dr. Kimberly Chapman was a member of the E-HOD Guidelines Committee and was responsible for gathering the data from a review of the scientific literature that lead to the Guidelines for Diagnosis and Management for Cystathionine Beta-Synthase Deficiency that were published in the Journal of Metabolic Diseases.

12:00-01:15 **Lunch**

01:15-02:15 **Screening Family Members & Family Planning**
Presenter: Katie Sapp, MS, CGC
Indiana University Health Physicians | Riley Children's Hospital
In this talk, we will focus on issues related to the diagnosis and recurrence risk of homocystinuria. Beginning with newborn screening for this condition, we will discuss the various challenges that one may encounter when trying to diagnose or rule out homocystinuria. This will include conversations on the concerns that may arise when pursuing carrier testing for at-risk family members as well as different approaches to testing that may be considered. Finally, we will discuss the inheritance of homocystinuria and the chances for other family members or future generations to also be diagnosed with this condition. We will look at various scenarios which may be applicable to patients and family members to gain a deeper understanding of the recurrence risk for each individual.

02:15-2:45 **Vendor Break**

02:45-04:00 **Breakout Sessions:** By age group

04:00-05:30 **Free Time**

05:30-8:30 **Reception (Dinner Included)**

Sunday, October 20

07:30-08:30 **Breakfast, Registration and Vendors Open**

08:45-08:55 **Group Photo**

09:00-09:30 **Vendor Acknowledgement & HCU Hero Award**

09:30-10:30 **Therapies on the Horizon**
Presenter: Margie McGlynn, President, HCU Network America
Over the past few years, a project was sponsored by HCU Network America and HCU Network Australia to develop a global research map that describes the research underway for new therapies to treat homocystinuria, focusing on HCU caused by CBS deficiency. A global research strategy was developed and a Scientific Advisory Board was convened to define the top priorities for funding via a new global grants program. This presentation will provide an update on the research underway for HCU and the status of our global grants process.

10:30-11:00 **Vendor Break**

11:00-12:00 **Panel: Ask the Expert**

12:00-01:30 **Closing Remarks and Lunch**

End of Conference—Thank you for coming!

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HCU HERO AWARD

June 5, 1942 - July 3, 2019

Jan Kraus was a Professor in Pediatric of 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

"I first met Jan at lunch in Prague during summer 2006 interviewing for a postdoctoral position in his group. Next time I heard from him was before Christmas 2006 with a note that I can start in his lab working on cystathionine beta-synthase (CBS) as soon as possible. Later, I learned that out of 26 postdoctoral fellows that Jan trained and mentored over his career, most of them were from former Czechoslovakia, a country he and his family fled in 1968. Jan turned out to be demanding, but patient mentor always with bigger picture in mind and his feet stably on the ground. Our professional relationship quickly expanded into a more personal level. Jan liked to keep up with all the technological advances often asking me for advice with computers and smartphones. Jan was an avid skier looking for occasional challenges, which I gladly provided by leading us through trees or steep and bumpy slopes. On a lift chair, we caught our breath and continued chatting about history, politics, technology and, of course, science. His rigorous and meticulous approach to scientific questions was I inspirational and contagious and we became quite a productive team.

Jan's footprint in the field of homocystinuria (HCU) and our recent improvements in expression and production of CBS enzymes resulted in collaboration with Orphan Technologies on developing enzyme replacement therapy for HCU. This was an ultimate goal of Jan's career, i.e. to make a difference for and to address an unmet need of HCU patients. Proof of concept, lead molecule optimization and all the preclinical studies on ERT were performed under Jan's watchful eye in his laboratory at the University of Colorado Anschutz Medical Campus. In his last months, Jan assisted with reaching out to HCU patients with the opportunity to enroll into the phase I clinical trial of our ERT, which is currently under way.

It is my hope that Jan's legacy will live not just through his scholarly achievements in the field and the researchers like me he trained or worked with, but mostly through the treatment for HCU we developed together."

Tomas Majtan, Assistant Research Professor at the University of Colorado Anschutz Medical Campus

CONFERENCE SPEAKERS

Marcia Sellos-Moura, PhD,
Marcia Sellos-Moura, PhD, is Vice President of Program and Portfolio Management at Orphan Technologies, a company dedicated to developing novel therapies to dramatically improve the lives of patients suffering from the rare disorder homocystinuria and related diseases. She has over 20 years of research, development, and program management experience with emphasis in preclinical and clinical development as well as bioanalytical and translational sciences.

Kimberly Chapman, MD, PhD, FAAP, FABIM
Kimberly Chapman is an attending physician in Genetics and Metabolism at Children's National and an Assistant Professor of Pediatrics and Integrated Systems Biology at George Washington University in Washington DC, United States. She specializes in taking care of all ages of individuals with defects of methylation, homocysteine and propionate metabolism.

Katie Sapp, MS, CGC
Katie Sapp is a genetic counselor with the Metabolism Clinic at IU Health Physicians and Riley Hospital for Children here in Indianapolis. Katie obtained her undergraduate degree in Biology from Indiana University-Bloomington in 2008, and completed her Masters degree in Medical Genetics at the Indiana University School of Medicine in 2010. Since that time, she has worked in her current role providing genetic counseling services as part of a multi-disciplinary clinic to patients with all types of inborn errors of metabolism, including those with positive newborn screens.

Margie McGlynn, HCUNA President
Margaret (Margie) McGlynn is President of the Board of HCU Network America, a patient advocacy organization she co-founded to provide support for patients and families affected by homocystinuria. She is also President of the Hempling Foundation for Homocystinuria Research, a fund she established to support research on new therapies for HCU in honor of her late sisters, Judy and Susie Hempling.

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BREAKOUT SESSION

Session: Birth—Age 4 (Early Childhood)
Leader: Kimberly Chapman
Job Title: Metabolic Geneticist
Clinic Affiliation: Children's National, DC

Session: 5-12 (Elementary)
Leader: Abby Hall
Job Title: Registered Dietitian
Clinic Affiliation: IU Health Physicians at Riley Hospital for Children

Session: 13-18 (High School)
Leader: Danielle Drake
Job Title: Registered Dietitian
Clinic Affiliation: IU Health Physicians at Riley Hospital for Children

Session: 19+ (College and Beyond)
Leader: Katie Sapp
Job Title: Genetic Counselor
Clinic Affiliation: IU Health Physicians at Riley Hospital for Children

PATIENT-EXPERT PANEL

Moderator:

Mark Lewis

Mark Lewis is a director on the HCU Network America Board and VP, Program and Outsource Management at TARIS Biomedical. He has three children, two of which have HCU. His older son, Ben was diagnosed through newborn screening and his daughter, Gabbi was the first child to be diagnosed through amniocentesis. He hopes to apply his professional knowledge and personal experience to help support HCUNA in achieving our goals for the betterment of those challenged by HCU and related disorders.

The Panel:

Kimberly Chapman, MD, PhD — Geneticist

Kimberly Chapman is an attending physician in Genetics and Metabolism at Children's National and an Assistant Professor of Pediatrics and Integrated Systems Biology at George Washington University in Washington DC, United States. She specializes in taking care of all ages of individuals with defects of methylation, homocysteine and propionate metabolism. She is also is one of the authors on the Guidelines for Diagnoses and Management of Cystathionine Beta-Synthase.

Katie Sapp, MS CGC — Genetic Counselor

Katie Sapp is a genetic counselor with the Metabolism Clinic at IU Health Physicians and Riley Hospital for Children here in Indianapolis. In her current role. she provides genetic counseling services as part of a multi-disciplinary clinic to patients with all types of inborn errors of metabolism, including those with positive newborn screens.

Abby Hall. RD– Dietitian

Abby Hall is a Metabolic Dietitian for IU Health Physicians at Riley Hospital for Children. Abby loves the challenge of metabolism and the relationships formed through working so closely with families. She is honored to be able to help her patients and to be in the ever evolving field of metabolism.

Ben Lewis—Adult Patient

Born in Massachusetts, Ben was diagnosed with HCU through newborn screening. He has led a relatively normal life, free of complications, because of his early diagnosis and the exceptional support provided by his parents, siblings, and specialists such as Dr. Mary Ampola. Ben is an avid hiker and member of the Appalachian Mountain Club (AMC) Four Thousand Footer Club, having summited all 48 White Mountain Four Thousand Footers. He holds a Bachelor of Science for Rensselaer Polytechnic Institute, an MBA from Babson College, and the role of Director at Liberty Mutual Insurance.

Rachel Skeens—Mother of Patient

Rachel Skeens is the mom to Landon, a HCU patient who wasn't diagnosed till he was 4 years old. After multiple misdiagnosis and failing to meet milestones, she remained strong and advocated for Landon until they received a diagnosis that explained his symptoms. Landon is now 11 years old and on track in most areas. She encourages families to stay strong and follow their gut instincts.

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KIDS ZONE SCHEDULE

Saturday	Ages: 5-8	Ages: 9-12
Time	**Activity**	**Activity**
08:45-09:00	Kids Zone Opens—	Kids Zone Opens—
09:00-09:30	Ice Breaker & HCU Story	Ice Breaker & HCU Story
09:30-10:00	“Racecar” Craft	Vendor Scavenger Hunt
10:00-10:30	Vendor Scavenger Hunt	“Racecar” Craft
10:30-11:00	Mask Activity	Jeopardy
11:00-11:30	Bingo	Mask Activity
11:30-12:00	Bathroom Break	Bathroom Break
12:00-01:15	**Lunch with Parents**	**Lunch with Parents**
01:15-01:30	Bathroom Break	Bathroom Break
01:30-02:00	Dietitian Activity	Jeopardy
02:00-02:30	Movie	Dietitian Activity
02:30-03:00	Movie	Movie
03:00-03:30	Movie	Movie
03:30-04:00	Clean up	Movie

Schedules and activities are subject to change without notice

Parents, Please be prompt in picking up your child.

Sunday	Ages: 5-8	Ages: 9-12
Time	**Activity**	**Activity**
08:45-08:55	Group Photo	Group Photo
08:55-09:00	Back to Kids Zone	Back to Kids Zone
09:00-09:30	Finish Movie	Outdoor Relay Race
09:30-10:00	LP Cookie Decoration	Bathroom Break
10:00-10:30	Outdoor Relay Race	LP Cookie Decoration
10:30-11:00	Bathroom Break	Finish Movie
11:00-11:30	Sign T-shirts & Pictures	Picture Frame Craft
11:30-12:00	Picture Frame Craft	Clean up
12:00-12:30	Clean up	Sign T-Shirts & Pictures
12:00-01:15	**Lunch with Parents**	**Lunch with Parents**

Schedules and activities are subject to change without notice

Parents, Please be prompt in picking up your child.

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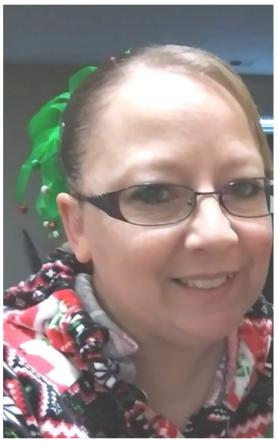
KIDS ZONE TEAM LEADERS



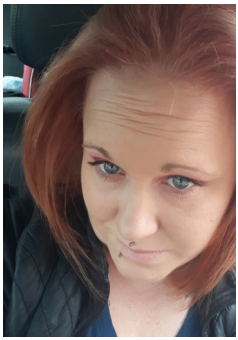
Jen has a 5 year old son and is currently a stay at home mom. She has 23 years experience with children of all ages. In that time, she has worked in several childcare centers, as well as, ran her own in-home daycare. Jen has always had a passion to work with children and aid in their developmental growth. She says, "there is nothing more satisfying than seeing a child succeed."

Jen is certified in CPR/First Aid and has her CDA in Early Childhood Development. She dreams of one day opening her own non-profit childcare for low income families. She believes that all children deserve access to an enjoyable education.

Kathy is a mom of two children, ages 18 and 13. Besides raising her own kids, she has taken care of other children in both a daycare setting and also at her home. Five years prior to receiving her bachelors degree in Healthcare Administration, she worked with children from infancy to 5 years old. She currently is a Certified Clinical Medical Assistant and works at a Cancer Center full time. In addition to her full time job, she cleans offices at night and on most weekends run a photo booth associated with her parents business. As a single mother, Kathy's main focus is providing the best life possible for her children. Watching children enjoy life and being part in that joy is very gratifying.

Joanna is a mother of two children, a 9 year old boy and a 2 year old girl. In 2010 she graduated college with an Associates Degree. She is currently enrolled at Purdue Northwest University and hopes to earn her Bachelors degree in Early Childhood Education. She currently is a lead teacher at the Head Start Program in Indianapolis. She is certified in both CPR and First Aid.

Joanna loves taking her children out to do things and making lasting memories. Joanna believes all children deserve the same opportunity to receive quality education no matter their race, religion, financial status, etc.



Desiree is a single mom of four children, 2 boys and 2 girls, ranging in ages from 2 to 15 years old. For the past six years she has been working in the in-home health service field. Desiree has recently started working for the Indiana Family and Social Services Administration Head Start program. She holds the position of Preschool Assistant. Additionally, she is currently pursuing a bachelor's in criminal psychology. Her ultimate goal is to receive a master's in art therapy. She really enjoys her job and going to school.

Desiree is CPR certified for infants, kids and adults. Being in health care she has worked with a wide range of health complications and dietary plans. She looks forward to this experience with excitement

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SPONSOR INFORMATION

Orphan Technologies is committed to reduce the burden of patients suffering from homocystinuria. OT-58, our lead drug development candidate, has been optimized as an enzyme replacement therapy for classical homocystinuria, a genetic disease characterized bycardiovascular, skeletal, neurologic, and ophthalmologic complications. OT-58 is designed to reduce homocysteine levels via a targeted mechanism of action and may have therapeutic applications in other diseases. To learn more, visit www.orphantechnologies.com/ot-58/

Recordati Rare Diseases is a biopharmaceutical company committed to providing often overlooked orphan therapies to the underserved rare disease communities of the United States. Our experienced team works side-by-side with rare disease communities to increase awareness, improve diagnosis, and expand availability of treatments. For more information, please visit www.recordatirarediseases.com

Aeglea BioTherapeutics, Inc. is a clinical-stage biotechnology company that engineers next-generation human enzymes with enhanced properties and novel activity to provide solutions for diseases with unmet medical need. To learn more about Aeglea, please visit aegleabio.com.

Founded in 2000, Cambrooke Therapeutics recognizes the critical nature of managing serious medical disorders through medically based nutrition therapies. For people with serious unmet medical nutrition needs, it is a daily challenge to find flavorful, easy options that can sustain their diets and maintain their nutritional needs. To check out our line of low protein foods and methionine free formula, please visit Cambrooke.com

At Nutricia we believe in the power of nutrition to make a positive and proven difference to health. Everyday we apply our collective expertise in nutrition to some of the world's biggest health challenges in life in the nutritional management of diseases, disorders and medical conditions. To find more out about our products please visit www.medicalfood.com

Rubius Therapeutics is genetically engineering red blood cells to create an entirely new class of cellular medicines, called Red Cell Therapeutics™. They can potentially be used to replace missing enzymes for patients living with certain rare metabolic disorders, such as homocystinuria, kill tumors in cancer and regulate the immune system for the treatment of autoimmune diseases. To learn more about our investigational therapies, please visit www.rubiustx.com

Solace Nutrition prides itself on its main reason for existence – the advancement of disease management through nutrition. Our products are designed by healthcare professionals specialized in the area of disease for which our products are intended. Our team is consistently developing scientifically proven products that fill the unmet need of the many people struggling with inborn errors of metabolism and chronic disease management. Our products are in concentrated & convenient forms using only safe, clinically-validated ingredients that are manufactured in FDA registered & audited facilities.

Abbott Nutrition is one of the world's leading authorities in science-based nutrition for all stages of life. We offer a wide range of metabolic products including for HCU patients to help patients grow and achieve greater wellbeing. Visit: www.AbbottNutrition.com to find product information, patient education materials, clinical research papers and more.

POA Pharma North America is a pharmaceutical and healthcare company that manufactures and distributes medical food for patients with inborn errors of metabolism. Our mission is to offer unique products that make a special diet easier to follow. POA Pharma North America is committed to adding new product offerings and are collaborating with our partners in Europe (POA Pharma Scandinavia) to introduce exciting new products to the US and Canadian markets.

Taste Connections, LLC

Taste Connections specializes in low-protein products such as bread mix, multi-baking mix, etc. breads, cookies, and other baked goods that are suitable for metabolic conditions such as Phenylketonuria, Homocystinuria, etc. We are committed to providing best quality low-protein products to our customers at lowest possible price. In the near future, we will be adding several new low-protein products for your convenience.

Visit Tasteconnections.com to view our low protein items and mixes.

Vitaflo®

Innovation in Nutrition

Vitaflo® is at the forefront of developing innovate specialized foods for metabolic disorders, pediatric renal disease, ketogenic diet, and other areas of disease
specific nutrition. Our aim is to create nutritional products that combine the best of cutting-edge research with the lifestyle demands of modern living, ensuring the most acceptable products are available for the patient. By constantly evolving to meet patient needs, Vitaflo will continue to develop products that offer patients choice and help support them in complying with restrictive therapeutic diets. For more information, visit www.nestlehealthscience.us/vitaflo-usa

FLAVIS

Flavis is a line of medical foods designed for those with Chronic Kidney Disease and Inherited Metabolic Disorders. The brand is the result of Dr. Schär's experience and dedication to research in the field of Medical Foods for Specific Nutritional Needs for over 30 years.

ACCELERATING TOWARDS A CURE

2019 CU Network America Conference | Indianapolis, IN

THANK YOU FOR ATTENDING

Dear Friends,

I would like to thank you again for attending our second conference and for your support in *Accelerating Towards a Cure.* As I reflect on the past year and a half since our last conference, I wouldn't have imagined our organization and community would come this far. In this short time, we have:

- Formed three new committee's:
    - The Fundraising Committee
        - Completed First Virtual Race—Go the Extra Mile for HCU
    - The Patient-Parent Advisory Committee
        - Created 3 new toolkits
    - Cobalamin (CBL) Steering Committee
        - Published New Diagnosis Checklist for Cbl patients

- Issued our first research grant. HCU Network and HCU Network Australia awarded Kenneth Maclean of the University of Colorado School of Medicine, Denver a $40,000 research grant to investigate "New Metabolic Strategies for Improving Treatment of Homocystinuria Due to CBS Deficiency and Remethylation Defects.

- Presented testimony to the Advisory Committee on Heritable Disorders in Newborn and Children (ACHDNC). Part of HCU Network America's mission is to support the advancement and diagnosis of Homocystinuria and related disorders. Under current newborn screening approaches an estimated 50% of patients are missed. HCU Network America is advocating for a two-tier method that would dramatically decrease the number of missed cases.

And those are just the highlights! Help us continue the momentum and Acceleration Towards a Cure in 2020 and get involved.

With gratitude and hope,

Danae' Bartke

Danae' Bartke, Executive Director

RECORDATI RARE DISEASES

Focused on the Few™...

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

We are proud to support the 2019 HCU Network America
***Accelerating Towards a Cure* Conference**

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NP-RRD-US-0124

HCU Network America 's mission is to improve the lives of individuals with HCU and to pursue a cure.