



CRN Family Conference

July 18-20, 2019

Draft Agenda – 6/11/19

Wednesday, July 17th

3:00 pm – 8:00 pm

CRN Board of Directors Meeting
Closed Session

Columbus Ballroom A

Thursday, July 18th

12:00 pm – 5:00 pm

Registration/Information Desk

Please stop by the Registration Desk to check in and pick up your welcome bag, t-shirts and name badges. If you have not yet downloaded the meeting app, staff can help you do so. The app contains all the information you will need for the conference such as the agenda, attendee and speaker contact information, and CRN Board and Speaker Bios.

Grand Ballroom Foyer

Grand Ballroom Foyer

Grip Strength Study

Dr. Larry Greenbaum from Emory University has been studying muscle strength in patients with cystinosis. Loss of muscle strength is a common complication in adults with cystinosis. This study measures grip strength (squeezing a grip for 5 seconds) in patients who are at least 6 years old. The goals of the study are to determine risk factors for decreased grip strength and to determine the severity of the problem in patients with cystinosis. Describing the problem will hopefully lead to studies of interventions to slow or reverse the loss of muscle strength. Grip strength may be a good way to monitor therapies. Dr. Greenbaum previously published a study in the Journal of Pediatrics showing that decreased grip strength is common in pediatric kidney transplant recipients. He has also recently submitted a paper showing decreased grip strength in children with chronic kidney disease as part of the NIH CKiD study (before dialysis or transplant). Dr. Greenbaum will be presenting the results of the first 50 participants in this study during this year's CRN conference. Many of these patients participated at the last CRN conference in Utah. Dr. Greenbaum hopes to obtain follow-up studies in some of these same patients. This will enable him to see how muscle strength changes over time. He also hopes to obtain results on more patients. Study participation will take about 5-10 minutes.

Grand Ballroom Foyer

5:00 pm – 7:00 pm **CRN Welcome Reception** **Grand Ballroom**
Hosts: CRN Board of Directors
Complimentary/Open Bar

7:00 pm – 10:00 pm **Family Introductions/Networking** **Grand Ballroom**
Hosts: Tim Wyman and Jen Wyman, VP Family Support

A great opportunity to put faces to names of each family attending the conference. Bring your entire family as this session will include introductions and reveal the third book in the “Journeys with Cystinosis” comic series. Author and illustrator, Kevin McCalla will be available to autograph complimentary copies of the book. In addition, families will all participate in a special project at the end of the evening!

Friday, July 19th

7:00 am – 8:30 am **Breakfast** **Grand Ballroom Foyer**
Complimentary

8:00 am – 5:00 pm **Registration/Information Desk** **Grand Ballroom Foyer**

Grip Strength Study **Grand Ballroom Foyer**

Dr. Larry Greenbaum from Emory University has been studying muscle strength in patients with cystinosis. Loss of muscle strength is a common complication in adults with cystinosis. This study measures grip strength (squeezing a grip for 5 seconds) in patients who are at least 6 years old. The goals of the study are to determine risk factors for decreased grip strength and to determine the severity of the problem in patients with cystinosis. Describing the problem will hopefully lead to studies of interventions to slow or reverse the loss of muscle strength. Grip strength may be a good way to monitor therapies. Dr. Greenbaum previously published a study in the Journal of Pediatrics showing that decreased grip strength is common in pediatric kidney transplant recipients. He has also recently submitted a paper showing decreased grip strength in children with chronic kidney disease as part of the NIH CKiD study (before dialysis or transplant). Dr. Greenbaum will be presenting the results of the first 50 participants in this study during this year’s CRN conference. Many of these patients participated at the last CRN conference in Utah. Dr. Greenbaum hopes to obtain follow-up studies in some of these same patients. This will enable him to see how muscle strength changes over time. He also hopes to obtain results on more patients. Study participation will take about 5-10 minutes.

WBC Cystine Testing **Discovery A**
Dr. Bruce Barshop

8:00 am – 5:30 pm

Childcare Open

Columbus Ballroom

The CRN is proud to offer a day care for all the children and siblings from ages 0-12 attending the conference! We will have a full staff of Nannies from the Philadelphia Nanny Company to watch the children. Ages 13 and up will be provided a safe and fun TEEN ROOM, where they can come and go as they please (parents must sign a waiver). Teens are welcome to participate in any entertainment or craft activity, which is located in the childcare rooms adjacent to the teen room.

Columbus Ballroom A=Ages 13 and up

Columbus Ballroom B=Ages 6-12

Columbus Ballroom C=Ages 0-5

*Childcare rooms will be open both Friday and Saturday from 8am-5pm, excluding lunch time. There will be age appropriate activities, games, and tons of crafts to make and take! All ages are encouraged to participate in the entertainment and crafts in the rooms! Wii system and movies are also available for fun. **We will have amazing entertainment coming in both mornings from 9-12!***

Parents MUST sign their child into daycare each morning and SIGN OUT every time they come to get their child(ren) (ages 0-12). Parents need to take their children out of childcare for lunch. Dosing of medications can only be administered by parents-not nannies or volunteers. Snacks and plenty of water will be provided. Guaranteed to allow the kids to not only bond, but to have the time of their lives!

9:00 am - 12:00 pm: Party Princess and Superhero Performance! For all ages.

10:00 am - 1:00 pm: Photo Booth with Superheroes and Princesses! Mom and dad can join before or after lunch to get some great photos with the kids!

*3:00 pm – 5:00 pm: Sing me a Story Foundation
The kids will write & illustrate stories about whatever they want in their very own blank storybooks. Each story goes on to be transformed into a personalized song by artists & bands across the country, raising awareness about cystinosis through the power of music.
For all ages. www.singmeastory.org*

8:15 am – 8:30 am

Welcome and Opening Remarks

Grand Ballroom

Clinton Moore, President

8:30 am – 9:30 am

Keynote Speaker

Grand Ballroom

David Fajgenbaum, MD, MBA, MSc

Dr. David Fajgenbaum, MD, MBA, MSc, FCPP, is the co-founder and Executive Director of the Castleman Disease Collaborative Network (CDCN), Assistant Professor of

Medicine in Translational Medicine & Human Genetics at the University of Pennsylvania, and Associate Director, Patient Impact for the Penn Orphan Disease Center. Dr. Fajgenbaum is also a patient battling idiopathic multicentric Castleman disease (iMCD). As common as ALS and as deadly as lymphoma, iMCD involves the immune system attacking and shutting down the body's vital organs (liver, kidneys, bone marrow, heart). He became ill during his third year of medical school in 2010, spent five months hospitalized, had his last rites read, and has had four deadly relapses since. In 2012, Dr. Fajgenbaum began conducting Castleman disease research at Penn and co-founded the CDCN, through which he has spearheaded the "Collaborative Network Approach." He currently leads the Castleman Research Program at Penn as PI of 18 translational research studies, including an international natural history study and the first-ever NIH R01 grant studying iMCD. As a result of his work, Fajgenbaum is currently in his longest remission ever thanks to a treatment that he identified, which had never been used for iMCD.

9:30 am – 9:50 am	CRN – Your Advocacy Group <i>Christy Greeley, Executive Director, VP Research</i>	Grand Ballroom
9:50 am – 10:10 am	Cystinosis Network Europe Update <i>Anne Marie O'Dowd, Chair, Cystinosis Network Europe</i>	Grand Ballroom
10:10 am – 10:30 am	The Promise of New Drug Targets to Reverse Renal Injury in Cystinosis <i>Supported by HRB Ireland and Cystinosis Foundation Ireland Minnie Sarwal, MD, FRCP, DCH, PhD</i>	
10:30 am – 10:45 am	Break	Grand Ballroom Foyer
10:45 am – 11:10 am	Cystinosis – A Review of Old and New <i>J.J. Zaritsky, M.D.</i> <i>A review of the epidemiology, pathophysiology and treatment options of Cystinosis.</i>	Grand Ballroom
11:10 am – 11:35 am	Anticipating Renal Replacement Therapy <i>Anticipating Renal Replacement Therapy Paul Grimm, MD</i>	Grand Ballroom
	<i>Unfortunately, even when children are diagnosed with cystinosis at a young age and they are able to tolerate the cystine depleting therapy, there may be substantial damage</i>	

already done to the kidneys. This damage may occur without the usual tests of kidney function being abnormal. The creatinine might be "normal" even after significant amounts of kidney damage has occurred. We are usually born with a lot of extra kidney function, so we don't start feeling ill from kidney failure until we are down at 10 or 15% function. Therefore we have to rely on the kidney numbers that come with routine blood testing, but they are not so easily interpreted. So, as a parent or a patient, how worried should you be about your kidney numbers? What are the kidney numbers that are good, or bad, or worse? Are there any special considerations for a cystinosis patient compared to any other patient with Chronic Kidney Disease?

We will discuss the kidney numbers that include CKD Stages (1, 2, 3, 4 and 5/ESRD) serum creatinine, BUN and a relative newcomer called Cystatin C. These are used singly and in combination to get a better understanding of the kidney function. We may also discuss measuring protein loss in the urine as a way to help determine how far advanced the kidney function is.

We will talk about plotting these kidney numbers on a graph and using it to help predict when the kidney function might be falling to the level where some kind of kidney replacement therapy/renal replacement therapy will be necessary. At the end of the session, we will also discuss ways to slow the progress of the kidney failure; including nutritional choices, lifestyle choices and medication.

11:35 am – 12:00 pm **A Systematic Review of Adult Complications of Cystinosis** **Grand Ballroom**
Rachel Kasimer, Medical Student/Craig Langman, MD

12:00 pm – 1:00 pm **Lunch** **Grand Ballroom Foyer**
Complimentary

Panel Sessions 1, 2, & 3

Panel Sessions are targeted towards different stages of the Cystinosis journey, divided into tracks by age. They will be smaller group sessions with a panel of experts presenting information, guiding discussion and fielding questions. Attendees should feel free to attend sessions of most interest to them. Some sessions are closed as noted below.

1:00 pm – 2:00 pm **Panel Session 1** **Grand Ballroom A**
Caregiver/Infant & Child (0-10): Cystinosis 101
Panelists: Drs. Katharina Hohenfellner, Larry Greenbaum, Joshua Zaritsky, Mihir Thacker, Ranjan Dohil, Jess Thoene, Ewa Elenberg, and Paul Goodyer

Teenager (11-17): Cystinosis 201 **Grand Ballroom B/C**
Panelists: Drs. Rachel Bishop, Craig Langman, Rick Kaskel, Doris Trauner, Minnie Sarwal, Neveen Soliman

Adult (18+): Cystinosis 301 **Grand Ballroom D**
Panelists: Drs. William Gahl, Galina Nesterova, Paul Grimm, Maya Doyle, Patrick Gipson

2:00 pm – 3:00 pm

Panel Session 2

Caregiver/Infant & Child (0-10): Physician Q & A

Panelists: Drs. Katharina Hohenfellner, Larry Greenbaum, Craig Langman, Paul Grimm, Joshua Zaritsky, Ranjan Dohil, Jess Thoene, Ewa Elenberg, Neveen Soliman, Paul Goodyer, Mihir Thacker

Grand Ballroom A

Teenager (11-17): Social Relationships and Gaining Independence

*Panelists: Maya Doyle, MSW, PhD, LCSW, Carrie Ostrea, Seth Rotberg, Ina Gardener, MEd, LPC
(session for teens 11-17 and their parents)*

Grand Ballrooms B/C

Adult (18+): Living with Cystinosis as an Adult: Healthcare, Expectations, Self-Management, Reproductive Issues

*(closed session for adults 18 + and their partners)
Panelists: Drs. Rachel Bishop, William Gahl, Galina Nesterova, Patrick Gipson, Elena Levtchenko (via Skype)*

Grand Ballroom D

Parents of Adults with Cystinosis Breakout

*(closed session for parents of adults living with Cystinosis)
Panelists: Colleen Hammond, Carol Hughes*

Discovery C

The purpose of the session is intended to be a private setting for parents of adults with cystinosis to share information, insight, advice and encouragement. This session will provide an opportunity to learn from others who face similar challenges, and allow you to talk about your experiences.

3:00 pm – 4:00 pm

Panel Session 3

Caregiver/Infant & Child (0-10): Neurocognitive & Educational Issues

Panelists: Marybeth Krummenacker, Doris Trauner, MD, Neveen Soliman, MD

Grand Ballroom A

This session will include a brief review of the effects of cystinosis on the developing brain, including possible motor, coordination, and cognitive differences, some of which may affect learning styles in the school age years. A brief summary of other possible neurological complications of cystinosis will also be presented.

Teenager (11-17): Transplant and Dialysis

Panelists: Drs. Rick Kaskel, Paul Grimm, Minnie Sarwal, Patrick Gipson

Grand Ballroom B/C

Adult (18+): Adults Living with Cystinosis: Focus on Mental Health and Wellness

*(closed session for Cystinosis adults and their partners)
Panelists: Maya Doyle, MSW, PhD, LCSW and Carrie Ostrea, Ina Gardener, MEd, LPC, Seth Rotberg, Galina Nesterova, MD*

Grand Ballroom D

4:00 pm – 4:15 pm

Break

Grand Ballroom Foyer

4:15 pm – 5:30 pm	Medical Panel <i>Moderator: William A. Gahl, M.D., Ph.D.</i>	Grand Ballroom
	<p><i>Please join the entire group for the unique and informative opportunity to have your questions and concerns addressed by the leading physicians and researchers in Cystinosis. All of the doctors who have presented at the Family Conference, all attending Medical Advisory Board and Scientific Review Board members, as well as other health care professionals involved in treating and researching Cystinosis are scheduled to participate. Questions for the panel will be collected during the proceedings today.</i></p>	
5:30 pm	Group Photograph <i>All conference attendees should gather for a group photograph and a photograph of all individuals in attendance living with Cystinosis. Please bring your green conference t-shirt to wear for a very special group photo! Also, please pick your children after the Medical Panel from Childcare and bring to the Grand Ballroom for this wonderful remembrance of the conference.</i>	Grand Ballroom
6:30 pm – 9:30 pm	Meet & Greet Family Buffet Dinner <i>Hosts: Adult Leadership Advisory Board Dinner complimentary/Cash Bar</i>	Grand Ballroom
	<p><i>Take time to refuel and relax with a casual dinner complete with good company. Share your learnings from the day and catch up with other families during our meet and greet buffet dinner.</i></p>	
7:00 pm – 10:00 pm	Speaker/VIP Dinner <i>Hosts: CRN Board of Directors Closed Session</i>	R2L Restaurant 50 S. 16th Street

Saturday, July 20th

7:00 am – 8:30 am	Breakfast <i>Complimentary</i>	Grand Ballroom Foyer
8:00 am – 4:00 pm	Registration/Information	Grand Ballroom Foyer
	Grip Strength Study <i>Dr. Larry Greenbaum from Emory University has been studying muscle strength in patients with cystinosis. Loss of muscle strength is a common complication in adults with cystinosis. This study measures grip strength (squeezing a grip for 5 seconds) in patients who are at least 6 years old. The goals of the study are to determine risk factors for decreased grip strength and to determine the severity of the problem in patients with cystinosis. Describing the problem will hopefully lead to studies of interventions to slow or reverse the loss of muscle strength. Grip strength may be a good way to monitor therapies. Dr. Greenbaum previously published a</i>	Grand Ballroom Foyer

study in the *Journal of Pediatrics* showing that decreased grip strength is common in pediatric kidney transplant recipients. He has also recently submitted a paper showing decreased grip strength in children with chronic kidney disease as part of the NIH CKiD study (before dialysis or transplant). Dr. Greenbaum will be presenting the results of the first 50 participants in this study during this year's CRN conference. Many of these patients participated at the last CRN conference in Utah. Dr. Greenbaum hopes to obtain follow-up studies in some of these same patients. This will enable him to see how muscle strength changes over time. He also hopes to obtain results on more patients. Study participation will take about 5-10 minutes.

8:00 am – 5:00 pm

Childcare Open

The CRN is proud to offer a day care for all the children and siblings from ages 0-12 attending the conference! We will have a full staff of Nannies from the Philadelphia Nanny Company to watch the children. Ages 13 and up will be provided a safe and fun TEEN ROOM, where they can come and go as they please. (parents must sign a waiver) Teens are welcome to participate in any entertainment or craft activity, which is located in the childcare rooms adjacent to the teen room.

Columbus Ballroom

Columbus Ballroom A=Ages 13 and up
Columbus Ballroom B=Ages 6-12
Columbus Ballroom C=Ages 0-5

Childcare rooms will be open both Friday and Saturday from 8am-5pm, excluding lunch time. There will be age appropriate activities, games, and tons of crafts to make and take! All ages are encouraged to participate in the entertainment and crafts in the rooms! Wii system and movies are also available for fun. **We will have amazing entertainment coming in both mornings from 9-12!**

Parents **MUST** sign their child into daycare each morning and **SIGN OUT** every time they come to get their child(ren) (ages 0-12). Parents need to take their children out of childcare for lunch. Dosing of medications can only be administered by parents-not nannies or volunteers. Snacks and plenty of water will be provided. Guaranteed to allow the kids to not only bond, but to have the time of their lives!

8am-5pm: Childcare Open

9am-12:00pm: Fabulous Faces of Philly! Fabulous Balloon Twisting, balloon twisting school, face painting and tattoos! FOR ALL AGES!! All ages are encouraged to attend!

12:30- Ages 13-19 LUNCH IF going on the field trip
1:15 TEEN FIELD TRIP to The Escape Room of Philly!

(reserved trip for ages 13-19, must have RSVP'd)

Bus leaves at 1:15 SHARP!-meet in LOBBY by Concierge

8:30 am – 8:45 am	Opening Comments <i>Christy Greeley, Executive Director and VP Research</i>	Grand Ballroom
8:45 am – 10:15 am	Advocacy	Grand Ballroom
8:45 am – 9: 15 am	<i>Carrie Ostrea</i> <i>This interactive session will provide guidance and education on how to be a stronger advocate for the Cystinosis community. This session will explain the various types of advocacy to help each participant determine what they are most passionate about and what their next steps should be to pursue that direction. In addition, this will focus on tools and strategies to empower patients and their families to share their story impactfully with your community, local media, your healthcare team, and on social media.</i>	Grand Ballroom
9:15 am – 9:45 am	<i>Seth Rotberg</i> <i>This session will go through the patient journey of Seth Rotberg -a young adult in the rare disease space- and how he was able to turn challenges into opportunities. It will discuss how a person impacted by a rare disease can use his or her diagnosis as motivation to get more involved in fundraising, advocacy, and volunteer efforts. This session will also discuss some of the different ways to advocate for yourself and the next steps to take for a better tomorrow.</i> https://www.youtube.com/watch?v=5_O5TfMVqD8 , https://www.youtube.com/watch?v=84QwwHOizGE	Grand Ballroom
9:45 am – 10:30 am	Breakout Session <i>Teens and adults living with cystinosis to meet with Adult Leadership Advisory board members to discuss ideas and resources for the teen and adult community.</i>	Innovation A & B
9:45 am – 10:15 am	Life Lessons from the Rare Disease Community <i>Jean Campbell</i> <i>Over the course of 35 years, the rare disease community has evolved from the grassroots coalition to an assembly of like-minded individuals and organizations linked together with the common mission of raising awareness and finding effective treatments for their specific rare disease. This will be a discussion about how this unique band of people found ways to connect, commiserate, and collaborate to further each other's missions and visions while identifying ways to bring their voices together in a unified chorus.</i>	Grand Ballroom
10:15 am – 10:30 am	Break	Grand Ballroom Foyer
10:30 am – 1:00 pm	Research Updates <i>Host: Paul Grimm, MD</i> <i>Clinical research updates from around the world. Brief updates will be shared from a variety of researchers in order to better understand the future of cystinosis diagnosis and management.</i>	Grand Ballroom

10:30 am – 10:50 am

Stem Cell Gene Therapy for Cystinosis: Description of the Upcoming Clinical Trial

Stephanie Cherqui, PhD

*We showed in the mouse model of cystinosis that bone marrow stem cells, especially the hematopoietic stem cells (HSCs), holds great promise to treat cystinosis. Indeed, HSCs led to significant decrease of cystine in all the organs as well as the long-term preservation of the kidney, eyes and thyroid for the life of the *Ctns*^{-/-} mice. Thus, these results suggest that one-time HSC transplantation could prevent the development of the different complications associated with cystinosis for the life of the patients. Because the use of allogeneic (foreign) HSCs is very risky, we developed an autologous HSC transplantation for cystinosis, i.e. the use of the patients' own stem cells. Thus, patients' HSCs have to be gene-corrected to introduce a functional CTNS gene. The CTNS gene will be introduced using a lentiviral vector (HIV-derived vector) that will stably integrate into the patient's DNA. The preclinical studies for testing such a treatment have been done in the *Ctns*^{-/-} mice. We have now received FDA-approval to start a phase 1/2 clinical trial for transplantation of autologous gene-corrected HSCs for cystinosis. The trial will be conducted and overviewed by the Cystinosis Stem Cell and Gene Therapy Consortium which include 14 experts in the field of bone marrow transplantation, gene therapy and cystinosis: Stephanie Cherqui - Study director, UCSD; Nancy Stack – Director of the Cystinosis Research Foundation; Edward D. Ball – Director of Bone Marrow Transplantation at UCSD; Bruce Barshop – Metabolic, UCSD; Ranjan Dohil – Gastroenterology, UCSD; Magdalene Dohil – Dermatology, UCSD; Robert Mak – Nephrology/Muscle, UCSD; Nadine Benador – Nephrology, UCSD; Doris A. Trauner – Neurology, UCSD; Eric Nudleman – Ophthalmology, UCSD; Susan Phillips – Endocrinology, UCSD; Kathleen Rickert - Orthopedy, UCSD; Paul Grimm – Nephrology, Stanford; Donald B. Kohn – HSC Gene Therapy, UCLA. We will describe the different steps of the phase 1/2 clinical trial that will occur at the University of California, San Diego. This study will enroll 6 subjects, male or female, affected with cystinosis and will consist of:*

- Screening period (verification that meet inclusion criteria),*
- Baseline period (clinical evaluations to determine the health status of the patients before transplant),*
- Stem Cell Isolation period to mobilize peripheral blood HSCs and perform apheresis (white blood cell isolation),*
- Stem cell gene-correction period while patients are back home,*
- Treatment and Recovery period consisting of a 4-week inpatient stay for conditioning and transplantation, and weekly follow-up outpatient visits for about 10 weeks,*
- Five one-week Post-Transplant Study Follow-up visits over 2 years at 3, 6, 12, 18- and 24-months post-transplant.*

10:55 am – 11:15 am

A No-Nonsense Approach to Cystinosis

Paul Goodyer, MD

11:20 am – 11:40 am

Muscle Weakness in Patients with Cystinosis

Larry Greenbaum, MD, PhD, FAAP

Muscle weakness is a complication of cystinosis. It tends to worsen over time. It is most commonly seen in adults with cystinosis. Many believe that cysteamine therapy prevents or delays the development of muscle weakness. Grip strength is one way of measuring muscle strength. We have used grip strength to measure muscle strength in children in the CKiD study. This is a study sponsored by the NIH of approximately 1000 children with chronic kidney disease. It only includes a small number of children with cystinosis. We showed that children with chronic kidney disease have decreased grip strength compared to healthy children. At our clinic at Emory, we have conducted a study of grip strength of children and adults with cystinosis. In order to study more patients, we measured grip strength of children and adults with cystinosis at the last CRN Conference in Utah. We were very appreciative of the many children and adults who participated in the study. We also asked patients about problems with weakness in their daily lives and if they have had times during their lives when they did not take cysteamine. In this study, we showed that grip strength was decreased in patients with cystinosis. We are now measuring grip strength every 1-2 years in patients with cystinosis to see if it decreases over time.

11:45 am – 12:05 pm

Genetic Newborn Screening for Cystinosis and SMA in Germany

Katharina Hohenfellner, MD

Newborn screening (NBS) programs for treatable metabolic disorders have been enormously successful, but molecular-based screening has not been broadly implemented so far. This prospective pilot study was performed within the German NBS framework. DNA, extracted from dried blood cards collected as part of the regular NBS program, was screened for cystinosis and spinal muscular atrophy (SMA). Between January 15, 2018 and May 30 2019, more than 260,000 newborns were screened in Germany for cystinosis and SMA. Until now 1 patient with Cystinosis and 26 patients with SMA have been diagnosed. No false screening result have been reported so far. Communication of findings to parents, and confirmation of diagnosis were accomplished in a multi-disciplinary setting and the program was well accepted by hospitals, physicians, and parents. This pilot study demonstrates the efficacy of a molecular-based neonatal screening program for cystinosis and SMA using an existing national screening framework.

12:10 pm – 12:30 pm

The Lifecourse Journey of Cystinosis

Frederick Kaskel, MD, PhD, FAAP, FASN

The concept of lifecourse grew from life course theory: sociological approach developed in 1960's to study social structure & cultural influences on health. It examines effects of the life history of a variety of human outcomes and focuses on pathways, mechanisms and research processes across multiple levels genetic to socio-structural. It places importance on developmental and biologic trajectories considered over time. Investigators are interdisciplinary in nature and strive to develop tools to understand & integrate health disparities in later life. The concept of exposures along the continuum of life from prenatal, postnatal, early childhood, adolescence into adulthood and old age, may affect developmental plasticity and the effect of intergenerational exposures on future generations. From lifecourse research came efforts to study special population differences in the progress and treatment of disease processes which could be applied to the cystinosis population. A goal would be to develop an integrative approach to the multiorgan involvement during growth and development in cystinosis using the principles of lifecourse research. This would involve EMRs, biobanks, registries, and other longitudinal studies aimed at achieving a more integrated approach to cystinosis throughout the continuum.

12:35 pm – 12:55 pm

Foxe Labs CRN Neurocognitive Research Update

1:00 pm – 2:00 pm

Lunch

Complimentary

Grand Ballroom Foyer

12:30 pm – 2:30 pm

Working Lunch - Adult Leadership Advisory Board

Moderator: Carrie Ostrea

Closed Session – ALAB Members

Innovation A

2:00 pm – 3:00 pm

Poster Session

Columbus Ballroom Foyer

This session will showcase a mix of science, medicine, industry, advocacy group and patient experiences to provide an interactive experience for both family and professional attendees. Researchers, clinicians, industry, advocacy representatives, students, patients, and caregivers will be invited to exhibit their latest research findings, treatment breakthroughs, advocacy group updates and real patient and family experiences. This will be an interactive session where exhibitors will be available to discuss their work or experiences with those attending. We invite you to browse the posters and take this opportunity to ask the authors and presenters questions.

3:00 pm – 4:00 pm	<p>Parents of Children and Adults with Cystinosis Panel Moderator: Jen Wyman, CRN VP Family Support Panelists: The Meschke, Bartkowski, Stilke, Schleuder, Hammond, Pruitt and Reed Families</p>	Grand Ballroom
	<p><i>Panel presentation during which parents of children and adults with cystinosis will answer prepared questions and address topics related to the use of coping mechanisms through the ups and downs that cystinosis brings related to not only developmental and transitional issues of daily life but also medical issues. Parents of individuals at every stage of the disease will be featured and will share how they have managed the variety of challenges they have faced. Audience participation will be encouraged.</i></p>	
4:00 pm – 5:00 pm	<p>Adults Living with Cystinosis Panel Moderator: Carrie Ostrea Panelists: Adult Leadership Advisory Board members, Invited panelists</p>	Grand Ballroom
	<p><i>Panel presentation during which individuals living with cystinosis will answer prepared questions and address topics related to the use of coping mechanisms and strategies for success through the ups and downs that Cystinosis brings. Audience participation will be encouraged. Adult Leadership Advisory Board (ALAB) members in attendance will participate. ALAB is a new CRN initiative and is comprised of adults living with cystinosis. Ultimately, this program will develop programming and opportunity for adults affected by Cystinosis.</i></p>	
5:00 pm – 5:15 pm	<p>Closing Remarks Clinton Moore, CRN President, Christy Greeley, Executive Director</p>	Grand Ballroom
6:00 pm – 10:00 pm	<p>Farewell Dinner Dance Dinner complimentary/cash bar</p>	Grand Ballroom
	<p><i>All conference attendees – pack your dancing shoes for our final event of the week – the dinner dance! Before saying goodbye, recharge with a delicious dinner then show your moves on the dance floor! After all, this evening is to celebrate YOU and our cystinosis community....Ring in the Future!</i></p>	