



CRN Family Conference

July 18-20, 2019

Draft Agenda – 5/13/19

Wednesday, July 17th

3:00 pm – 8:00 pm	CRN Board of Directors Meeting <i>Closed Session</i>	Columbus Ballroom A
-------------------	--	----------------------------

Thursday, July 18th

12:00 pm – 5:00 pm	Registration/Information Desk	Grand Ballroom Foyer
5:00 pm – 7:00 pm	CRN Welcome Reception <i>Hosts: CRN Board of Directors</i> <i>Complimentary</i>	Grand Ballroom

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

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 "Team Cysteamine" 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 <i>Complimentary</i>	Grand Ballroom Foyer
8:00 am – 5:00 pm	Registration/Information Desk	Grand Ballroom Foyer
8:00 am – 5:30 pm	Childcare Open <i>The CRN is proud to offer a day care for all the children and siblings of the conference. We will have a full staff of Nannies from the Philadelphia Nanny Company to watch the children. Childcare will be provided 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! Wii system and movies are also available for fun. We will have amazing entertainment coming in both mornings from 9-12!</i>	Columbus Ballroom

Parents **MUST** sign their child into daycare each morning and **SIGN OUT** every time they come to get their child(ren). 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. www.singmeastory.org

8:15 am – 8:30 am

Welcome and Opening Remarks
Clinton Moore, President

Grand Ballroom

8:30 am – 9:30 am

Keynote Speaker
David Fajgenbaum, MD, MBA, MSc

Grand Ballroom

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.

<https://www.cdcn.org/about-castleman-disease>

<https://bit.ly/2FVZT66>

<https://www.youtube.com/watch?v=95yqILftYHg&t=>

<https://www.youtube.com/watch?v=aM-DfYFUyt0>

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</i> <i>Minnie Sarwal, MD, FRCP, DCH, PhD</i>	
10:30 am – 10:45 am	Break	Grand Ballroom Foyer
10:45 am – 11:15 am	Cystinosis – A Review of Old and New <i>Joshua Zaritsky, MD</i> <i>A review of the epidemiology, pathophysiology and treatment options of Cystinosis.</i>	Grand Ballroom
11:15 am – 11:45 am	Anticipating Renal Replacement Therapy <i>Paul Grimm, MD</i>	Grand Ballroom
11:45 am – 12:00 pm	A Systematic Review of Adult Complications of Cystinosis <i>Rachel Kasimer, Medical Student/Craig Langman, MD</i>	Grand Ballroom
12:00 pm – 1:00 pm	Lunch <i>Complimentary</i>	Grand Ballroom Foyer

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 Caregiver/Infant & Child (0-10): Cystinosis 101 <i>Panelists: Drs. Katharina Hohenfellner, Larry Greenbaum, Joshua Zaritsky, Mihir Thacker, Ranjan Dohil, Jess Thoene, Ewa Elenberg, and Paul Goodyer</i>	Grand Ballroom A
	Teenager (11-17): Cystinosis 201 <i>Panelists: Drs. Rachel Bishop, Craig Langman, Rick Kaskel, Doris Trauner, Minnie Sarwal, Neveen Soliman, Invited panelists</i>	Grand Ballroom B/C
	Adult (18+): Cystinosis 301 <i>Panelists: Drs. William Gahl, Galina Nesterova, Paul Grimm, Maya Doyle, Invited panelists</i>	Grand Ballroom D
2:00 pm – 3:00 pm	Panel Session 2 Caregiver/Infant & Child (0-10): Physician Q & A <i>Panelists: Drs. Katharina Hohenfellner, Larry Greenbaum, Craig Langman, Paul Grimm, Joshua Zaritsky, Ranjan Dohil,</i>	Grand Ballroom A

Jess Thoene, Ewa Elenberg, Neveen Soliman, Paul Goodyer, Mihir Thacker, Invited Panelists

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

Grand Ballrooms B/C

Panelists: Maya Doyle, MSW, PhD, LCSW, Carrie Ostrea, Seth Rotberg, Ina Gardener, Licensed Professional Counselor

(session for teens 11-17 and their parents)

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

Grand Ballroom D

(closed session for adults 18 + and their partners)

Panelists: Drs. Rachel Bishop, William Gahl, Galina Nesterova, Invited panelists

Parents of Adults with Cystinosis Breakout

Room?

(closed session for parents of adults living with Cystinosis)

Panelists: Colleen Hammond, Carol Hughes

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

Grand Ballroom A

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

Teenager (11-17): Transplant and Dialysis

Grand Ballroom B/C

Panelists: Drs. Rick Kaskel, Paul Grimm, Minnie Sarwal, Invited panelists

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

Grand Ballroom D

(closed session for Cystinosis adults and their partners)

Panelists: Maya Doyle, MSW, PhD, LCSW and Carrie Ostrea, Ina Gardener, Licensed Professional Counselor, Seth Rotberg, Galina Nesterova, MD

4:00 pm – 4:15 pm

Break

**Grand Ballroom
Foyer**

4:15 pm – 5:30 pm

Medical Panel

Grand Ballroom

Moderator: William A. Gahl, MD, PhD

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.

5:30 pm	<p>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></p>	Grand Ballroom
6:30 pm – 9:30 pm	<p>Meet & Greet Family Buffet Dinner <i>Hosts: Adult Leadership Advisory Board Complimentary</i></p> <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>	Grand Ballroom
7:00 pm – 10:00 pm	<p>Speaker/VIP Dinner <i>Hosts: CRN Board of Directors Closed Session</i></p>	R2L Restaurant, 50 S. 16th Street

Saturday, July 20th

7:00 am – 8:30 am	<p>Breakfast <i>Complimentary</i></p>	Grand Ballroom Foyer
8:00 am – 4:00 pm	Registration/Information	Grand Ballroom Foyer
8:00 am – 5:00 pm	<p>Childcare Open <i>9:00 am-12:00 pm: Fabulous Faces of Philly - Fabulous Balloon Twisting, Balloon twisting school, Face painting and tattoos! For all Ages!</i></p> <p><i>1:00 pm-5:00 pm: Philadelphia Nanny Company to supervise in room activities!</i></p> <p><i>Tween/Teen- Field Trip and Team Building to Escape the Room Philly! (Must have permission slip signed, for ages 12-19)</i></p>	Columbus Ballroom
8:30 am – 8:45 am	<p>Opening Comments <i>Christy Greeley, Executive Director and VP Research</i></p>	Grand Ballroom
8:45 am – 10:15 am	Advocacy	Grand Ballroom
<i>8:45 am – 9: 15 am</i>	<i>Carrie Ostrea</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.

9:15 am – 9:45 am	Seth Rotberg	Grand Ballroom
	<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. https://www.youtube.com/watch?v=5_05TfMVqD8, https://www.youtube.com/watch?v=84QwwHOizGE</i>	
9:45 am – 10:30 am	Breakout Session	Innovation A & B
	<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>	
9:45 am – 10:15 am	Jean Campbell (communication/networking)	Grand Ballroom
10:15 am – 10:30 am	Break	Grand Ballroom
10:30 am – 1:00 pm	Research Updates <i>Host: Paul Grimm, MD</i>	Foyer Grand Ballroom
	<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>	
10:30 am – 10:50 am	Stem Cell Gene Therapy for Cystinosis: description of the upcoming clinical trial <i>Stephanie Cherqui, PhD</i>	
10:55 am – 11:15 am	A No-Nonsense Approach to Cystinosis <i>Paul Goodyer, MD</i>	
11:20 am – 11:40 am	Muscle Strength in Cystinosis <i>Larry Greenbaum, MD, PhD, FAAP</i>	
11:45 am – 12:05 pm	Genetic Newborn Screening for Cystinosis and SMA in Germany <i>Katharina Hohenfellner, MD</i> <i>Newborn screening (NBS) programs for treatable metabolic disorders have been enormously successful, but molecular-</i>	

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

12:35 pm – 12:55 pm

Foxe Labs CRN Neurocognitive Research Update

1:00 pm – 2:00 pm

Lunch

**Grand
Foyer**

Ballroom

Complimentary

12:30 pm – 2:30 pm

Poster Session

**Columbus
Foyer**

Ballroom

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

Parents of Children and Adults with Cystinosis Panel
Moderator: Jen Wyman, CRN VP Family Support

Grand Ballroom

Panelists: Invited

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.

4:00 pm – 5:00 pm

Adults Living with Cystinosis Panel

Grand Ballroom

Moderator: Carrie Ostrea

Panelists: Adult Leadership Advisory Board members, Invited panelists

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.

5:00 pm – 5:15 pm

Closing Remarks

Grand Ballroom

Clinton Moore, CRN President, Christy Greeley, Executive Director

6:00 pm – 10:00 pm

Farewell Dinner Dance

Grand Ballroom

Complimentary

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!