



Cystinosis Research Network 2021 Virtual Family Conference

July 16-18, 2021 (all times Eastern Daylight Time)

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Friday, July 16th

6:00 pm – 8:00 pm **Conference Kickoff/Family Introductions**

*Hosts: Jonathan Dicks, VP Development and Jen Wyman, VP Family Support
Please join us for welcome and overview of the conference and family introductions. Bring your entire family!*

Saturday, July 17th

10:00 – 10:30 am **Welcome and CRN Overview**

Tim Wyman, President

10:30 am – 11:00 am **Cystinosis – A Review of Old and New**

*J.J. Zaritsky, MD
A review of the epidemiology, pathophysiology and treatment options of Cystinosis.*

11:00 am – 11:30 am **Anticipating Renal Replacement Therapy/Stanford protocol combined stem cell transplant and kidney transplant from same living donor**

Paul Grimm, MD

11:30 am – 12:30 pm **Panel Session 1**

Caregiver/Infant & Child (0-10): Cystinosis 101

*Moderator: CRN Board Member
Panelists: J.J. Zaritsky, Craig Langman, Ranjan Dohil, Paul Goodyer, Ewa Elenberg, Rachel Bishop*

Teenager (11-17): Cystinosis 201

Moderator: Herberth Sigler, Board Member
Panelists: Rick Kaskel, Larry Greenbaum, Mihir Thacker, Minnie Sarwal

Adult (18+): Cystinosis 301

Moderator: Marybeth Krummenacker, VP Education and Awareness
Panelists: Bill Gahl, Galina Nesterova, Jess Thoene, Elena Levtchenko, Roz Mannon

12:30 pm – 1:00 pm Break

1:00 pm – 2:00 pm Panel Session 2

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

Moderator: Jonathan Dicks, VP Development
Panelists: Doris Trauner

Teenager (11-17): Transplant and Dialysis

Moderator: CRN Board Member
Panelists: Paul Grimm, Rick Kaskel, Roz Mannon, Ewa Elenberg, Minnie Sarwal, Larry Greenbaum

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

(closed session for cystinosis adults and their partners)
Moderator: Sara Healy, Adult Leadership Advisory Board (ALAB) Member
Panelists: Maya Doyle, Bill Gahl, Galina Nesterova, Elena Levtchenko

Parents of Adults with Cystinosis

(closed session for parents of adults with cystinosis)

Moderator: 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.

2:00 pm – 3:00 pm Medical Panel

Moderator: William A. Gahl, MD, PhD

Panelists: Paul Grimm, Craig Langman, Jess Thoene, Rick Kaskel, Rachel Bishop, Doris Trauner, Larry Greenbaum, Paul Goodyer

Please join for the unique and informative opportunity to have your questions and concerns addressed by the leading physicians and researchers in Cystinosis. Questions will be gathered during the course of the conference.

Sunday, July 18th

10:00 am – 10:05 am **Welcome**

Christy Greeley, VP Research, Executive Director

10:05 am – 10:20 am **Cystadrops Update**

Recordati Rare Diseases/Anovo

10:20 am – 10:35 am **Cystaran Update**

Leadiant Biosciences

10:35 am – 11:05 am **Stem Cell Gene Therapy for Cystinosis**

AVROBIO

11:05 am – 11:35 am **Disease Specific Quality of Life Questionnaires
IMPACT – Improvement of Motoric Abilities in Patients with Cystinosis
Newborn Screening Update**

*Katharina Hohenfellner, MD, Harald Schubert, Novotec
Cystinosis Research Network Funded Investigator*

11:35 am – 12:05 pm **Cognitive Control Systems in Cystinosis**

*Sophie Molholm, PhD, John Foxe, PhD
Cystinosis Research Network Funded Investigator*

12:05 pm – 12:30 pm **Break**

12:30 – 1:00 pm **Chitotriosidase as a therapeutic monitor for cysteamine therapy in
cystinosis: a retrospective validation study**

*Elena Levtchenko, MD, PhD
Cystinosis Research Network Funded Investigator*

1:00 pm – 1:30 pm **Perturbations in the V-ATPase Pathway Drive Pathology in the Male Reproductive System in Cystinosis**
Minnie Sarwal, MD
Cystinosis Research Network and Cystinosis Ireland Funded Investigator

1:30 pm – 2:15 pm **Parents of Children and Adults with Cystinosis Panel**

Moderator: Jen Wyman, CRN VP Family Support

Panelists:

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.

2:15 pm – 3:00 pm **Adults Living with Cystinosis Panel**

Moderator: Maya Doyle

Panelists: Hannah Creel, Megan Morrill, Christina Morris, Brian Ensor

Panel presentation during which individuals living with cystinosis who serve on CRN's Adult Leadership Advisory Board (ALAB) 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