Cystinosis Research Network 2021 Virtual Family Conference

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

Draft 5/12/2021

<table>
<thead>
<tr>
<th>Friday, July 16th</th>
</tr>
</thead>
<tbody>
<tr>
<td>6:00 pm – 8:00 pm Conference Kickoff/Family Introductions</td>
</tr>
<tr>
<td><strong>Hosts:</strong> Jonathan Dicks, VP Development and Jen Wyman, VP Family Support</td>
</tr>
<tr>
<td>Please join us for welcome and overview of the conference and family introductions. Bring your entire family!</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Saturday, July 17th</th>
</tr>
</thead>
<tbody>
<tr>
<td>10:00 – 10:30 am Welcome and CRN Overview</td>
</tr>
<tr>
<td>Tim Wyman, President</td>
</tr>
<tr>
<td>10:30 am – 11:00 am Cystinosis – A Review of Old and New</td>
</tr>
<tr>
<td>J.J. Zaritsky, MD</td>
</tr>
<tr>
<td><em>A review of the epidemiology, pathophysiology and treatment options of Cystinosis.</em></td>
</tr>
<tr>
<td>11:00 am – 11:30 am Anticipating Renal Replacement Therapy/Stanford protocol combined stem cell transplant and kidney transplant from same living donor</td>
</tr>
<tr>
<td>Paul Grimm, MD</td>
</tr>
<tr>
<td>11:30 am – 12:30 pm Panel Session 1</td>
</tr>
<tr>
<td>Caregiver/Infant &amp; Child (0-10): Cystinosis 101</td>
</tr>
<tr>
<td><strong>Moderator:</strong> CRN Board Member</td>
</tr>
<tr>
<td><strong>Panelists:</strong> J.J. Zaritsky, Craig Langman, Ranjan Dohil, Paul Goodyer, Ewa Elenberg, Rachel Bishop</td>
</tr>
<tr>
<td>Teenager (11-17): Cystinosis 201</td>
</tr>
</tbody>
</table>
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 Levchenko, 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 Levchenko

**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 Levchenko, 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.