



## Thursday , April 16th

3:00 PM - 8:00 PM **Conference Registration Check-In**  
*The Cove*

4:00 PM - 6:30 PM **Childcare Hours**  
*Royal Tern (ages 0 - 3)*  
*Royal Tern (ages 4 - 7)*  
*Sandpiper (ages 8 - 12)*

3:30 PM - 4:30 PM **Teen Introductions**  
*Golden West*

4:30 PM - 6:30 PM **Family Introductions**  
*Moderators: Teresa and Kevin Partington, CRF Trustees*  
*Vista Ballroom*

6:30 PM - 9:00 PM **Dinner Reception**  
*Mariners Ballroom*

# Friday, April 17th

7:00 AM - 8:30 AM **Breakfast**  
*Pete's Sunset Grille*

8:15 AM - 12:00 PM **Childcare Hours**  
*Shoreline (ages 0 - 3)*  
*Shoreline (ages 4 - 7)*  
*Sunrise and Sunrise Foyer (ages 8 - 12)*

## Presentation Sessions      Huntington Ballroom

*Moderator: Marcu Alexander and Kristen Murray, CRF Trustees*

8:40 AM - 9:05 AM **Cystinosis Research Foundation**  
Clay Emerson, Trustee, Cystinosis Research Foundation

9:10 AM - 9:35 AM **Cystinosis: The Basics and Beyond**  
Stephen Jenkins, MD, CRF Trustee  
University of Utah Hospital

9:40 AM - 10:05 AM **Update on Cystinosis Medication and Role of Kidney Transplant**  
Paul Grimm, MD  
Stanford University School of Medicine and Stanford Children's Health

10:10 AM - 10:30 AM **Break**  
*Huntington Ballroom Foyer/Terrace*

## Break Out Sessions      Various Locations

10:35 AM - 11:55 AM **Parents of Children (ages 0 - 4)**  
**Golden West**  
*Discussion Leader: Jill Emerson, CRF Trustee, Ryan and Mariah Hub with Paul Grimm, MD*

10:35 AM - 11:55 AM **Parents of Children (ages 5 - 8)**  
**Salon B**  
*Discussion Leaders: Kristen Murray and Brian Sturgis, CRF Trustees with Julian Midgley, BM, BCh*

10:35 AM - 11:55 AM **Parents of Tweens (ages 9 - 12)**  
**Catalina**  
*Discussion Leader: Clay Emerson, CRF Trustee with Paul Goodyer, MD*

## Friday, April 17<sup>th</sup> (continued 1)

- 10:35 AM - 11:55 AM **Parents of Teens (ages 13 – 17)**  
**Salon C**  
*Discussion Leaders: Marcu Alexander and Stephen Jenkins, MD, CRF Trustees*
- 10:35 AM - 11:55 AM **Parents of Adults with Cystinosis**  
**Salon A**  
*Discussion Leaders: Teresa and Kevin Partington, Denice Flerchinger, CRF Trustees*
- 10:35 AM - 11:55 AM **Grandparents**  
**Vista 2**  
*Discussion Leaders: Bill and Diane Neumann*
- 10:35 AM - 11:55 AM **Breakout: Adults with Cystinosis and Partners**  
**Vista 1**  
*Discussion Leaders: Alex Stout and Heather Wegerif*
- 12:00 PM - 1:00 PM **Lunch**  
*Lighthouse Courtyard*
- 12:50 PM - 4:30 PM **Childcare Hours**  
*Shoreline (ages 0 - 3)*  
*Shoreline (ages 4 - 7)*  
*Sunrise and Sunrise Foyer (ages 8 - 12)*

### Presentation Sessions      Huntington Ballroom

*Moderators: Teresa and Kevin Partington, CRF Trustees*

- 1:05 PM 1:40 PM **Adult Panel**
- 1:45 PM - 2:20 PM **Breakout: Only Tweens and Teens with Cystinosis (ages 12 - 17)**  
*Discussion Leader: Stephen Jenkins, MD, CRF Trustee with Julian Midgley, BM, BCh*  
*Vista 2*
- 1:45 PM - 2:20 PM **Breakout: Adults with Cystinosis and Partners**  
*Discussion Leaders: Alex Stout and Heather Wegerif with Dr. Grimm*  
*Vista 1*

## Friday, April 17<sup>th</sup> (continued 2)

- 1:45 PM - 2:05 PM **Cystinosis and the Skin**  
Umber Dube, MD, PhD  
University of California, San Diego
- 2:10 PM - 2:20 PM **Family Story: From Helpless to Hopeful: Zoé's Story**  
Bianca Provost
- 2:25 PM - 6:00 PM **Teen Activity**  
*Slyders Poolside Cabanas*
- 2:25 PM - 2:45 PM **Changing Urine Into Kidney to Combat Cystinosis**  
Benjamin "Beno" Freedman, PhD  
University of Washington
- 2:50 PM - 3:05 PM **Break**  
*Huntington Ballroom Foyer/Terrace*
- 3:10 PM - 3:20 PM **Family Story: Aarav's Journey**  
Mukund Khalasi
- 3:25 PM - 3:45 PM **Validation of the University of California, Irvine Corneal Cystine Crystal Score: Correlation with Clinical and Patient-Reported Outcomes in Cystinosis**  
Donny Suh, MD, FAAP, MBA, FACS  
Gavin Herbert Eye Institute (GHEI) & Fellowship Program & Eye Mobile Program, University of California, Irvine
- 3:50 PM - 4:05 PM **What Have We Learned Today?**  
Stephen Jenkins, MD, CRF Trustee
- 6:00 PM - 9:00 PM **Dinner Under the Stars**  
*Lighthouse Courtyard*

# Saturday, April 18th

7:00 AM - 8:30 AM **Breakfast**  
*Pete's Sunset Grille*

8:15 AM - 12:00 PM **Childcare Hours**  
*Shoreline (ages 0 - 3)*  
*Shoreline (ages 4 - 7)*  
*Sunrise and Sunrise Foyer (ages 8 - 12)*

## Presentation Sessions      Huntington Ballroom

*Moderator: Denice Flerchinger and Stephen Jenkins, MD, CRF Trustees*

8:30 AM - 8:40 AM **Welcome**  
Marcu Alexander, CRF Trustee

8:45 AM - 9:05 AM **Interventional Swallow Study**  
Reza Sadjadi, MD and Melanie Krause G., MA, CCC-SLP  
Massachusetts General Hospital, Harvard Medical School

9:10 AM - 9:30 AM **Exploring Skeletal Muscle Repair in the Context of Cystinosis**  
Feodor Price, PhD  
Harvard Medical School

9:35 AM - 10:00 AM **A Preliminary Report on mRNA Therapy for Cystinosis in Mice**  
Paul Goodyer, MD  
McGill University Health Center, Montreal, Canada

10:05 AM - 10:20 AM **Break**  
*Huntington Ballroom Foyer/Terrace*

# Saturday, April 18<sup>th</sup> (continued)

## Presentation Sessions

## Huntington Ballroom

*Moderators: Jill Emerson and Brian Sturgis, CRF Trustees*

- 10:25 AM - 10:55 AM **Long Term Follow-Up of the First Clinical Trial of Autologous Hematopoietic Stem Cell Gene Therapy for Cystinosis**  
Stéphanie Cherqui, PhD  
University of California, San Diego
- 11:00 AM - 11:30 AM **Novartis Trial Update**  
Melissa Wigderson, MD  
Novartis
- 11:35 AM - 11:55 AM **Conference Wrap-Up**  
*Day of Hope Co-Chairs: Marcu Alexander, Jill and Clay Emerson, Denice Flerchinger, Stephen Jenkins, Kristen Murray, Teresa Partington and Brian Sturgis, CRF Trustees*
- 12:00 PM - 1:00 PM **Lunch**  
*Lighthouse Courtyard*

Conference Sponsors and Supporters:

# Nancy and Geoffrey Stack Family Foundation



Representatives from Amgen, Novartis, Leadiant and Recordati will all be available outside the main session room throughout the conference to provide information and answer your questions.