



# **Cystinosis Research Network – Your Advocacy Group**

**July 19 - 21, 2007  
San Antonio, Texas**

# Cystinosis Research Network - Your Advocacy Group

- Advocacy is defined as "the act of speaking or of disseminating information intended to influence individual behavior or opinion, corporate conduct, or public policy and law"

# Cystinosis Research Network - Your Advocacy Group

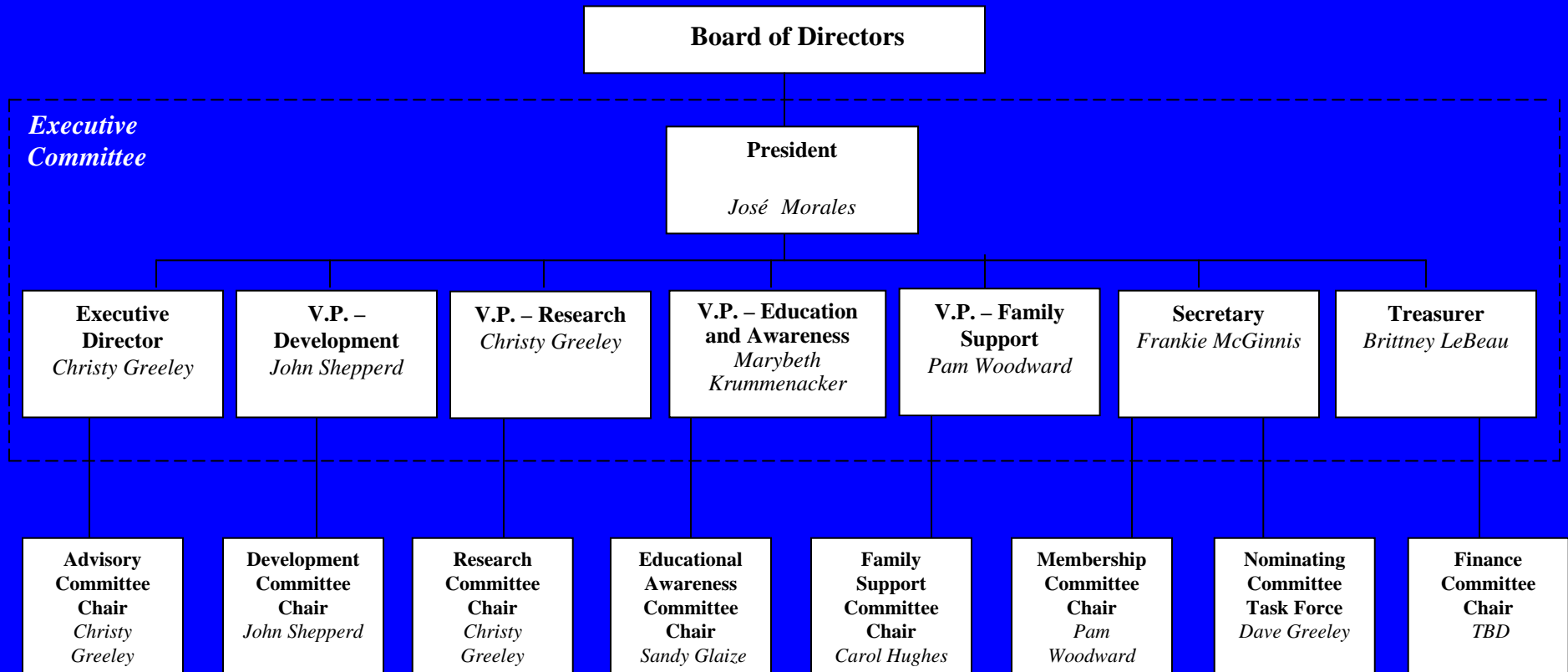
- There are multiple organizations :
  - Cystinosis Australia
  - Cystinosis Foundation Ireland
  - Cystinosis Foundation Germany
  - Cystinosis Foundation USA
  - Cystinosis Mexico
  - Cystinosis Research Foundation
  - **Cystinosis Research Network**
  - Cystinosis United Kingdom
- Each organization have distinct visions but with many common goals
- Collaboration amongst the organizations is a stated priority and objective

# Cystinosis Research Network - Vision and Mission

***Vision*** - the discovery of improved treatments and a cure for Cystinosis

***Mission*** - advocacy of scientific research, family support and raising of awareness amongst various critical communities

# Cystinosis Research Network



\* Denotes Acting

# Cystinosis Research Network - Board

- Glaize, Sandy
- Gledhill, Karen
- Greeley, Christy
- Greeley, Dave
- Harrison, Kathleen
- Hughes, Carol
- Julian, Dan
- Krummenacker, Marybeth
- LeBeau, Brittney
- Maxwell, Mack
- McGinnis, Frankie
- Morales, Jose
- *Morrill, Jill*
- *Shal, Paula*
- Shepperd, John
- Smith, Elva
- Thomas, Lynn
- Woodward, Pam

# Cystinosis Research Network - Executive Committee

- President Christy Greeley
- Education Paula Shal  
and Awareness
- Family Support Pam Woodward
- Development Jill Morrill
- Research Elva Smith
- Secretary Karen Gledhill
- Treasurer Brittney LeBeau

# CRN – Financial Overview

**Our emphasis continues to be the identification and funding of research to enhance the quality of life of the individual and the discovery of a cure for Cystinosis.**

## ***Credits***

- Fund Raising \$312,600

## ***Debits***

- Development \$5,400 1.7%
- Education and Awareness \$22,240 7.1%
- Executive Director \$7,400 2.4%
- Family Conference \$60,000 19.2%
- Family Support \$2,900 .9%
- Research \$192,701 61.6%
- Secretary \$200 .1%
- Treasurer \$2,900 .9%

Total: \$293,741  
Balance: \$18,859

# CRN – Development Committee

We continue to enhance our ability to raise funds and expand the number events.....you are the key to this success.

- Corporate Alliances
- Fund Raising
- Marketing
- Public Relations
- Strategic Alliances

# CRN – Fund Raising

**Fund raising comes from a diverse array of individual / family sponsored events. The key is deciding to make a difference!**

• CRN Programs	\$4,000	1.2%
• Corporate	\$33,500	10.7%
• Dinner Dances	\$72,600	23.2%
• Family Fun Days	\$48,000	15.4%
• Home Businesses	\$500	0.2%
• United Way	\$42,000	13.4%
• Individuals	\$54,000	17.3%
• Letter Campaigns	\$29,000	9.3%
• Sporting Events	\$26,600	8.5%
• Theme Events	\$2,500	0.8%

Total: \$312,600

# CRN – Research Committee

**Working with leading medical and research professionals to ensure the allocation of resources is aligned with our vision and mission.**

- Identification and Prioritization of Research
- Oversight of Medical Advisory Committee
- Coordination of research proposal requests
- Liaison with Scientific Review Board and Medical Advisory Board
- Creation of National Institutes of Health Fellowship, Bethesda, Maryland

# CRN – Research

**Doris Trauner, M.D., UCSD, La Jolla, CA**

- Early Intervention Trial for Visual Processing Deficit in Cystinosis

**Corinne Antignac, M.D., Ph.D., Vasiliki Kalatzis, Ph.D.,  
Hospital Necker-Enfants Malades, Paris, France**

- Development and in vivo testing of novel therapies for Cystinosis

**Donald Cairns, PH.D., School of Pharmacy,  
The Robert Gordon University, Aberdeen, Scotland**

- Design and Synthesis of Novel Prodrugs for the Treatment of Cystinosis

# CRN – Research

**Jess Thoene, M.D., University of Michigan Medical Center,  
Ann Arbor, MI**

- **Tissue Repository for Cystinosis**

**Elena Levtchenko, M.D., Ph.D., University Medical Center  
Nijmegen, The Netherlands**

- **Study of ATP metabolism in human cystinotic proximal tubular cells and in humans with Cystinosis in vivo**

**Dr. C. Tuleu, University of London, School of Pharmacy,  
Dr. Olufemi Rabi, Guy's & St. Thomas' NHS Foundation  
Hospital, Mr. Ken K. Nischal, Mr. Rajnish Sekhri, and Dr.  
William Van't Hoff, Great Ormond Street Hospital and Institute  
of Child Health**

- **Development of a Cysteamine in situ gelling system for the topical treatment of corneal crystals in Cystinosis**

# CRN – Education and Awareness Committee

**Focused on elevating the awareness of Cystinosis amongst key groups of service providers and members of our community.**

- Internet Support Group and Web Site Management
- Bi-Annual Newsletter – Fall and Spring
- Medical and Genetic and Rare Disorder Advocacy Conferences
- Liaison and collaboration with other key advocacy organizations
- 2010 International Nephrology Pediatric Conference – New York City, New York

# CRN – Family Support

**Focused on elevating the awareness of Cystinosis amongst members of our community.**

- Family Support – Identification and on-boarding
- 2008 Regional Family Gatherings
- 2009 Family Conference – Atlanta, Georgia, Hosts – Elva and Lorna Smith and Cheri Friend
- 2011 Family Conference – Bay Area, California, Hosts – Mary and Rich Jordan

# CRN – Future Commitments

**We have the strongest resolve to “stay the course”.**

- 2007 New Research Grants to be awarded
- 2008 National Institutes of Health Fellowship, Bethesda, Maryland
- 2009 Family Conference – Atlanta, Georgia
- 2010 International Pediatric Conference – New York City, New York
- 2011 Family Conference – Bay Area, California

# “Searching for a Cure..... Deep in the Heart of Texas”

## Conference Objectives

- Increase awareness and understanding of medical and research advances
- Provide access to leading medical and research professionals
- Raise awareness and understanding
- Network, network, network.....

# “Searching for a Cure..... Deep in the Heart of Texas”

## Family Conference Highlights

- Medical and Education and Family Support Sessions
  - Age appropriate tracks (Infancy/Preschool; Childhood, Adolescence and Adult)
- Poster Session
- Medical Panel

# “Searching for a Cure..... Deep in the Heart of Texas”

- This is a key, signature event for all of us. It represents our commitment to each other and to our extended community. Over the next few days we all have an opportunity learn information which will aid us in our journey. Our request of you is to share and contribute as much as you can so that we can all benefit.
- Welcome and Thank You!