

# Cystinosis Research Network, Inc.

## Annual Report 2007



302 Whytegate Court  
Lake Forest, IL 60045 USA

Toll Free: 866.276.3669  
Phone: 847.735.0471  
Fax: 847.235.2773  
Email: [crn@cystinosis.org](mailto:crn@cystinosis.org)  
[www.cystinosis.org](http://www.cystinosis.org)

Dear Friends:

We are pleased to present the first annual report for the Cystinosis Research Network (CRN). Founded in 1996, CRN's vision is the discovery of improved treatments and a cure for cystinosis. CRN is an all volunteer, non-profit organization dedicated to supporting and advocating research, providing family assistance and educating the public and medical communities about cystinosis. CRN funds research and programs primarily through donations from the public, grassroots fundraising events, and grants.

2007 was a year of great growth and accomplishment for CRN. We successfully transitioned new leadership for the organization on the Executive Committee and further expanded membership on the Board of Directors, Scientific Review Board, and Medical and Professional Advisory Committees. Key initiatives and program expansion took place in all major areas of concentration: Education and Awareness, Family Support, Research, and Development. Highlights of the year included:

### ***Education and Awareness***

- CRN raised awareness and reinforced strong relationships at three professional conferences which are very important to cystinosis: The University of Miami Pediatric Seminar (March), The American Society of Pediatric Nephrology meeting (May), and The American Society of Nephrology meeting (November).
- CRN continued to strengthen its affiliation with the National Organization for Rare Disorders (NORD) by attending the NORD Tribute Banquet in May honoring CRN Scientific Review Board Chairman Dr. William Gahl with the Public Health Leadership Award and NORD's retirement event honoring Abbey Meyers in October.
- CRN reformatted and expanded the information available on our comprehensive website and published two editions of our newsletter, which is distributed to over 2,000 families, donors, and cystinosis professionals.
- Three \$1,000 educational scholarships were awarded, two to individuals with cystinosis and one to a sibling of an affected individual.

### ***Family Support***

- The 2007 Family Conference, "Searching for a Cure...Deep in the Heart of Texas", took place July 19-21 in San Antonio, Texas. This was our largest conference to date as over 300 people attended and took advantage of the opportunity to become better informed, access medical professionals, and network. Individuals with cystinosis, families, and cystinosis clinicians and researchers from around the world were able to interact and share information in order to further their understanding of the disease. Future conference sites were announced, including 2009 in Atlanta, Georgia and 2011 in the San Francisco Bay Area.
- CRN continued its support of newly diagnosed and existing families through a variety of avenues including access to world experts in cystinosis via our advisory boards, moderation of an email support group and provision of information packets to families and professionals.

### ***Research***

- CRN announced the establishment and funding of a three year Cystinosis Fellowship at the National Institutes of Health under the direction of Dr. William Gahl. This will be the first community funded fellowship which will focus on clinical as well as research training in cystinosis, filling a crucial need in our community.
- CRN was designated as the lead non-profit organization for the International Pediatric Nephrology Association (IPNA) 15<sup>th</sup> Congress, August 29 – September 2, 2010, to be held in New York City under the direction of CRN Scientific Review Board member Dr. Frederick Kaskel.
- The 2007 Call for Research Proposals and subsequent Scientific Review Board review and recommendations resulted in funding for two new studies, for a total current research commitment of \$750,000 for eight studies:

“Early Intervention Trial for Visual Processing Deficit in Cystinosis”  
Doris Trauner, M.D., University of California, San Diego, La Jolla, CA  
August 2003 – Ongoing, Award amount: \$112,724

“Development and *in vivo* testing of novel therapies for cystinosis”  
Corinne Antignac, M.D., Ph.D., Vasiliki Kalatzis, Ph.D., Hôpital Necker-Enfants Malades,  
Paris, France, Grant Timeline: October 2005 – September 2007, Award Amount: \$50,000

“Design and Synthesis of Novel Prodrugs for the Treatment of Cystinosis”  
Donald Cairns, Ph.D., School of Pharmacy, The Robert Gordon University, Aberdeen,  
Scotland, Grant Timeline: October 2005 – September 2008, Award Amount: \$97,928

“Tissue Repository for Cystinosis”  
Jess G. Thoene, M.D., University of Michigan Medical Center, Ann Arbor, MI  
Grant Timeline: Fall 2006 – Ongoing, Award Amount: \$26,206

“Study of ATP metabolism in human cystinotic proximal tubular cells and in humans with  
cystinosis *in vivo*”, Elena Levtschenko, M.D., Ph.D., University Medical Center Nijmegen,  
The Netherlands, Grant Timeline: January 2007 – December 2007, Award Amount: \$68,090

“Development of a cysteamine *in situ* gelling system for the topical treatment of corneal crystals  
in cystinosis”, Dr. C. Tuleu, University of London, School of Pharmacy, Dr. Olufemi Rabiou,  
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  
Grant Timeline: Fall 2006-Fall 2009, Award Amount: £ 103,000 (approximately \$203,500)

“Gene transfer studies for cystinosis”  
Vasiliki Kalatzis, Ph.D., Eric J. Kremer, Ph.D., Institut Genetique Moleculaire de Montpellier,  
France, Grant Timeline: Fall 2007 – Fall 2009, Award Amount € 75,900 (approximately  
\$101,000)

“Evaluation of Novel Prodrugs for the Treatment of Nephropathic Cystinosis”  
Professor Donald Cairns, Dr Rachel M Knott, Dr Graeme Kay  
Grant Timeline: Fall 2007 - Fall 2008, Award Amount: £ 35,000 (approximately \$70,000)

### ***Development***

- CRN raised nearly half a million dollars in 2007 through the extraordinary generosity of many families and individuals. Funding was received from a variety of sources, including individual donations, grassroots fundraisers (sporting events, letter campaigns, family fun days, themed events, dinner dances, home business donations, etc.), CRN sponsored programs, and corporate and government grants.
- Of special note, The Harry A. and Margaret D. Towsley Foundation awarded CRN a matching grant in the amount of \$150,000 dedicated to the newly established NIH Cystinosis Fellowship.

CRN had a landmark year in many respects in 2007. Please accept our profound gratitude for all of those whose generosity of financial gifts, time and spirit made all of this possible. We thank you and look forward to your continuing support.

Sincerely,  
Christy Greeley  
President and Executive Director  
Cystinosis Research Network, Inc.

**CRN Board of Directors 2007-2008**

**President and Executive Director**

Christy Greeley, Illinois

**Vice President Research**

Elva Smith, Georgia

**Vice President Development**

Jill Morrill, Michigan

**Vice President Education and Awareness**

Paula Shal, Wisconsin

**Vice President Family Support**

Pam Woodward, Utah

**Secretary**

Karen Gledhill, New York

**Treasurer**

Brittney LeBeau, Illinois

**Directors**

Sandy Glaize, Florida

Dave Greeley, Illinois

Kathleen Harrison, Arizona

Carol Hughes, Florida

Dan Julian, Illinois

Marybeth Krummenacker, New York

Mack Maxwell, Texas

Frankie McGinnis, South Carolina

José Morales, Connecticut

John Shepperd, Texas

Lynn Thomas, New York



*Pictured from top left: Dan Julian, Sandy Glaize, John Shepperd, Karen Gledhill, Elva Smith, Frankie McGinnis, Dave Greeley, Lynn Thomas, Carol Hughes, Mack Maxwell, and Paula Shal. Bottom row from left: Marybeth Krummenacker, Brittney LeBeau, José Morales, Pam Woodward, and Christy Greeley. CRN is an all volunteer non-profit organization.*

**CRN Advisory Board Members 2007-2008**

***Scientific Review Board***

**William A. Gahl, M.D., Ph.D., Chairperson**

Clinical Director, National Human Genome Research Institute, Head, Section on Human Biochemical Genetics, Medical Genetics Branch, Head, Intramural Program, Office of Rare Diseases, National Institutes of Health, Bethesda, Maryland

**Adam J. Jonas, M.D.**

Chairman, Department of Pediatrics, Harbor-UCLA Medical Center, California

**Frederick Kaskel, M.D., Ph.D.**

Professor of Pediatrics, Director, Division & Training Program in Pediatric Nephrology Children's Hospital at Montefiore, Albert Einstein College of Medicine of Yeshiva University, New York

**Craig B. Langman, M.D.**

Issac A. Abt, M.D. Professor of Kidney Diseases, Feinberg School of Medicine, Northwestern University, Head, Kidney Diseases, Children's Memorial Hospital, Chicago, Illinois

**William Rizzo, M.D.**

Professor of Pediatrics, Chief, Inherited Metabolic Diseases, University of Nebraska Medical Center, Omaha, Nebraska

**Jerry A. Schneider, M.D.**

Research Professor of Pediatrics, Dean of Academic Affairs Emeritus, UCSD School of Medicine, San Diego, California

**Joseph D. Schulman, M.D.**

Retired, Palm Springs, California

**Jess G. Thoene, M.D.**

Director, Biochemical Genetics Laboratory, Active Professor Emeritus of Pediatrics, University of Michigan, Ann Arbor, Michigan

***Medical Advisory Committee***

**Donald Cairns, Ph.D.**

Associate Head of School, School of Pharmacy, The Robert Gordon University, Schoolhill, Aberdeen, Scotland

**Ranjan Dohil, M.D.**

Associate Professor, Department of Pediatrics, UCSD School of Medicine, San Diego, California

**Ewa Elenberg, M.D.**

Pediatric Nephrologist, Texas Children's Hospital, Baylor College of Medicine, Houston, Texas

**Julie R. Ingelfinger, M.D.**

Senior Consultant in Pediatric Nephrology, Mass General Hospital for Children, Professor of Pediatrics, Harvard Medical School, Deputy Editor, The New England Journal of Medicine, Boston, Massachusetts

**Robert Kleta, MD, PhD**

Potter Chair of Nephrology, University College, London, UK

**Minnie Sarwal, M.D., Ph.D.**

Nephrologist and Director, Pediatric Renal Transplantation, Stanford University, Stanford, California

**Susan Thomas Schendel, M.D.**

Clinical Assistant Professor, Medical Director, Pediatric Kidney Transplant Program, Division of Pediatric Nephrology & Transplantation, C.S. Mott Children's Hospital, University of Michigan Medical Center, Ann Arbor, Michigan

**Doris A. Trauner, M.D.**

Professor, Department of Neurosciences, UCSD School of Medicine, San Diego, California

***Professional Advisory Committee***

**Maya Doyle, LCSW-R**

Senior Social Worker, Pediatric Nephrology, The Children's Hospital at Montefiore, Bronx, New York

**David Glaize, Ed.D.**

Student Services Director, Seminole Community College, Lake Mary, Florida

**Nick Smith, LCMHC**

New Hope Counseling, Satellite Beach, Florida

**Charles E. Morrill, M.D.**

Retired, Grand Haven, Michigan



*Members of CRN's Scientific Review Board and Medical Advisory Committee participate in the Medical Panel during the July 2007 Family Conference in San Antonio, Texas.*



*Dr. Ranjan Dohil and Dr. William Gabl present during the 2007 Family Conference.*

Cystinosis Research Network  
Statement of Income and Expenses  
For the year ending December 31, 2007

	<u>Jan - Dec 07</u>
<b>Ordinary Income/Expense</b>	
<b>Income</b>	
<b>Direct Public Support</b>	
Corporate Contributions	92,805.00
Individual, Business Contributions	39,197.78
Direct Public Support - Fundraisers	<u>343,816.57</u>
<b>Total Direct Public Support</b>	475,819.35
<b>Indirect Public Support</b>	
United Way, CFC Contributions	20,442.64
Indirect Public Support - Other	<u>10,079.31</u>
<b>Total Indirect Public Support</b>	30,521.95
<b>Investments</b>	
Interest-Savings, Short-term CD	<u>2,370.99</u>
<b>Total Investments</b>	2,370.99
<b>Other Types of Income</b>	
Miscellaneous Revenue	50.00
Other Types of Income - Other	<u>1,309.84</u>
<b>Total Other Types of Income</b>	1,359.84
<b>Program Income</b>	<u>1,926.00</u>
<b>Total Income</b>	511,998.13
<b>Expenses</b>	
<b>Awards and Grants</b>	
Cash Awards and Grants	3,000.00
Awards and Grants - Other	<u>142,275.16</u>
<b>Total Awards and Grants</b>	145,275.16
<b>Bank fee</b>	150.34
<b>Business Expenses</b>	
Business Registration Fees	404.50
Business Expenses - Other	<u>5.00</u>
<b>Total Business Expenses</b>	409.50
<b>Contract Services</b>	
Accounting Fees	<u>1,540.00</u>
<b>Total Contract Services</b>	1,540.00

**Operations**

Books, Subscriptions, Reference	10.49
Postage, Mailing Service	1,785.26
Printing and Copying	12,898.55
Supplies	156.95
Telephone, Telecommunications	6,521.16
Operations - CRN Credit Cards	13,302.91
<b>Total Operations</b>	<u>34,675.32</u>

**Other Types of Expenses**

Insurance - Liability, D and O	3,406.00
Other Costs (Fundraiser expenses)	40,289.06
Other Types of Expenses - Other	125.00
<b>Total Other Types of Expenses</b>	<u>43,820.06</u>

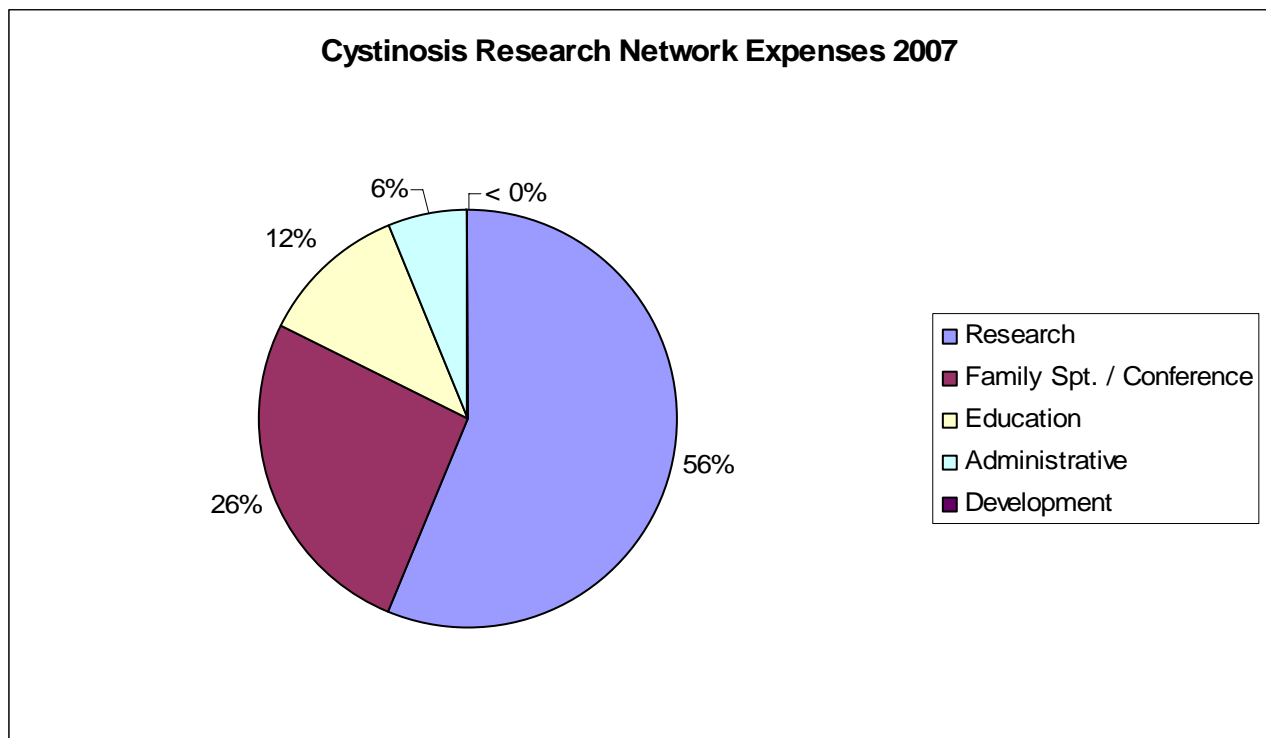
**Travel and Meetings**

Conference, Convention, Meeting	54,321.96
Travel	5,993.29
Travel and Meetings - Other	3,114.00
<b>Total Travel and Meetings</b>	<u>63,429.25</u>

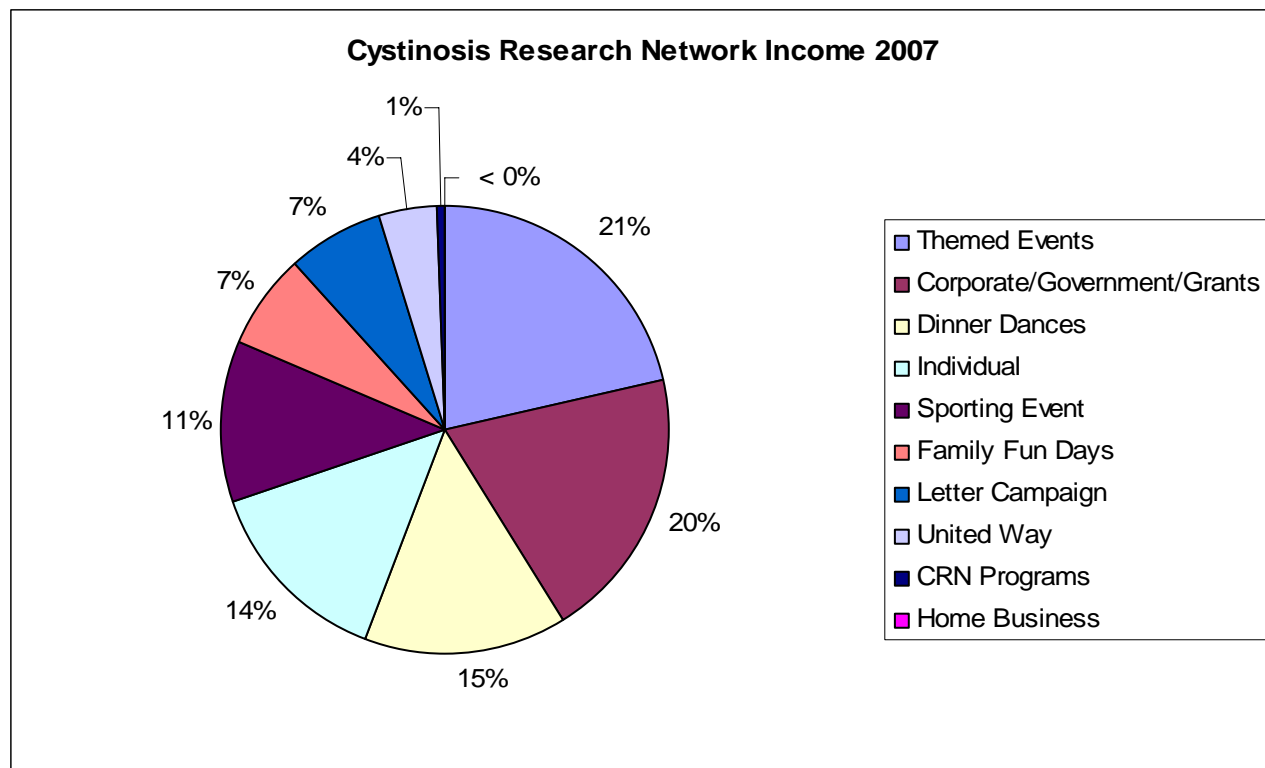
<b>Total Expenses</b>	<u>289,299.63</u>
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<b>Net Ordinary Income</b>	<u>222,698.50</u>
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<b>Net Income</b>	<u><u>222,698.50</u></u>
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**Contact Information**

The Cystinosis Research Network, Inc. is an all volunteer, non-profit organization dedicated to supporting and advocating research, providing family assistance and educating the public and medical communities about cystinosis. We are a private, nonprofit 501(c)(3) corporation, Federal Tax ID 04-3323789.

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For more detailed financial information, please check with GuideStar – The National Database of Nonprofit Organizations at [www.guidestar.org](http://www.guidestar.org) and search using “Cystinosis Research Network”.

For more information about cystinosis or to download a copy of this report, please visit our website at [www.cystinosis.org](http://www.cystinosis.org).