



Center for Rare Disease Therapies
KECK GRADUATE INSTITUTE
of Applied Life Sciences

MISSION

Our mission is to advocate new therapies for rare diseases and to increase awareness of rare diseases through education, science, and ethical business practices. The Center is a unique academic non-profit unit that interacts with government agencies, patient advocates, and life science companies.



ADVISORY BOARD

Chair

Dennis Fenton, PhD

Former Executive Vice President
Amgen

Daniel Bradbury, DMS

President and CEO
Amylin Pharmaceuticals

Tim Cote, MD, MPH

Director
Office of Orphan Products Development,
Food and Drug Administration

Robert Curry, PhD

Partner, Alliance Technology
Ventures
Chairman of the KGI Board of
Trustees

Diane Dorman

Vice President for Public Policy
National Organization for
Rare Diseases (NORD)

Steven Groft, PharmD

Director
Office of Rare Diseases
National Institutes of Health (NIH)

Marlene Haffner

Executive Director
Regulatory Affairs
Amgen

Richard Haas, MD, B Chir, MRCP

Professor of Neurosciences and
Pediatrics
Director of UCSD Mitochondrial
Disease Laboratory

Warren Holmes

Vice President Organizational
Resources & Health Policy
Sigma-Tau Pharmaceuticals

Peter Barton Hutt, LLB, LLM

Senior Counsel
Covington & Burling

Andy Johnson

Professor, Dean of the School of
Global and Community Health
Claremont Graduate University

Richard Jove, PhD

Director, Beckman Research Institute
City of Hope

Greg Lapointe, MBA

Chief Operating Officer
Sigma-Tau Pharmaceuticals

Peter Saltonstall

President and Chief Executive Officer
National Organization for Rare
Disorders (NORD)

Sheldon M. Schuster, PhD

President
KGI

Barbara Wuebbels, RN, MS

Manager, Medical Affairs Investigator
Relations
Biomarin Pharmaceuticals Inc.

BOARD OF EXPERTS

Blanca Aguiar, MD

Pediatrician

Jon Bui, MD, PhD

Neurologist, UCSD

Daniel Darvish, MD

Co-Founder, ARM & HIBM Research
Group (HRG)

Edward R. B. McCabe, MD, PhD

Physician-in-Chief
Mattel Children's Hospital
UCLA

Alan Rothfeld, MD

Clinical Professor of Pulmonary and
Critical Care Medicine
Keck School of Medicine
USC

Joseph Villafranca, PhD

Senior Vice President Operations
Tunnell Consulting

CONTACT:

M. Ian Phillips, PhD, DSc, FAHA

Director
Center For Rare Disease Therapies
Keck Graduate Institute

535 Watson Drive, Claremont CA 91711

Phone: (909) 607-7487

Fax: (909) 607-8086

E-mail: ian_phillips@kgi.edu



Center for Rare Disease Therapies
KECK GRADUATE INSTITUTE
of Applied Life Sciences

Center for Rare Disease Therapies

*"Advancing new therapies and
education to improve lives of
people with rare diseases"*



KGI.edu

Center Functions:

Working with corporate partners, patient organizations, and venture philanthropists, the Center:

- Assesses and evaluates undeveloped orphan drugs and biologics held in biomedical companies.
- Works with patient organizations to document the prevalence of rare diseases, identify potential therapies, and devise strategies for promoting development and commercialization.
- Works with the FDA to run workshops on orphan drug designations.
- Publishes papers on orphan drug regulations.
- Assists in preparation and filing of orphan drug designations.
- Funds internships with FDA/OOPD and PhRMA for KGI students.

Visit our website:

www.KGI.edu/CRDT.xml



Ian Phillips (center) with FDA officials Erica McNeilly and Dr. Mathew Thomas during the student workshop with the FDA.

The Center for Rare Disease Therapies (CRDT) was established under Dr. Ian Phillips and President Shelly Schuster at KGI to increase awareness of rare diseases through education, research, and new therapies. The center works with key groups and trains a student workforce skilled in rare disease therapies and regulation.

CRDT NEWS:

1. October 2009: CRDT organized a national symposium on “New Strategies for Rare Diseases” for top leaders from industry, government, science and patient advocates.
2. CRDT hosted the first FDA/industry “Orphan Drug Designation Workshop” in February 2010. The 2-day workshop attracted 29 pharmaceutical/academic sponsors and generated 18 orphan designation applications. The model was

repeated at the Univ. of Minnesota, and as a result 25% more orphan designations were approved than during the previous year.

3. KGI student Silviya Meletath drafted an orphan drug designation for start-up company BPT pharmaceuticals. The application was approved.

CRDT PUBLICATIONS:

CRDT has published in *Nature*, *Drug Discovery*, *Pediatrics* and *Tuftscoop* and appeared in *Wall Street Journal* stories.



Timothy Cote, Director of FDA–Office of Orphan Products Development, at the Orphan Designation Workshop for companies hosted at KGI.

CRDT SUPPORT:

CRDT acknowledges the support of:

- The Norris Foundation
- Sigma-Tau Pharmaceuticals
- PhRMA
- The Gross family