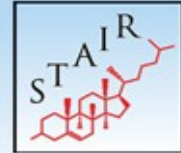




STEROL & ISOPRENOID
RESEARCH CONSORTIUM



**THE UNITED LEUKODYSTROPHY FOUNDATION
AND THE STAIR COMMITTEE**

CTX (Cerebrotendinous Xanthomatosis) 2012

Celebrating 30 years of service to
Leukodystrophy Families

Friday
July 20, 2012

Best Western Hotel
DeKalb, Illinois

**The United Leukodystrophy Foundation
would like to express our sincere
thanks to our sponsors:**

**Manchester Pharmaceuticals, Inc.
And
Sigma-Tau Pharmaceuticals, Inc.**

Manchester 
Pharmaceuticals, Inc.™



CTX SCIENTIFIC & FAMILY MEETING

THURSDAY, JULY 19, 2012

3:00 pm Registration
6:00 pm Buffet Dinner and Hospitality

FRIDAY, JULY 20, 2012

6:45a.m – 8:00am Registration & Breakfast

8:00am - 8:20am Welcome to CTX 2012
Paula K. Brazeal President
William Rizzo, M.D. ULF Medical &Scientific Advisory Board Chairman
Shailesh Patel, M.D., Division of Endocrinology, Metabolism and Clinical Nutrition, Medical College of Wisconsin, Milwaukee

8:25am - 8:55am CTX: From Bed to Bench and Contrary in the Siena Experience
Professor Antonio Federico, Direttore, U.O. Neurologia e Malattie Neurometaboliche, Universita degli Studi di Siena

9:00am – 9:30am CTX: Update on Genetic Data and Methods
Maria-Jesus Sobrido, M.D., PhD, Neurogenetics Group, Fundacion Publica Galega de Medicina Xenomica, Santiago de Compostela, Spain

9:35am-10:05am The Mouse Model for CTX
Sandra Erickson, PhD, Professor, Medicine/Endocrinology and Metabolism, Department of Medicine, University of California, Veterans Affairs Medical Center, San Francisco, California

10:05am-10:25pm BREAK

10:30 pm - 11:00pm Diagnosis of CTX
Kenneth Setchell, PhD, Pathology and Laboratory Medicine, Department of Pediatrics, Cincinnati Children's Hospital Medical Center, Cincinnati, Ohio.

11:05pm – 11:35pm Improved Blood Tests For CTX
Andrea DeBarber, PhD, Physiology & Pharmacology Department (L334), Oregon Health & Science University, Portland, Oregon

11:40pm - 12:10pm Mechanisms Behind Development of Xanthomas in Cerebrotendinous Xanthomatosis
Ingemar Bjorkhem, Senior Professor, Division of Clinical Chemistry, Department of Laboratory Medicine, Karolinska University Hospital Huddinge, Sweden

12:10pm - 1:40pm LUNCH

CTX SCIENTIFIC & FAMILY MEETING

- 1:40pm - 1:55pm Topics of Interest to CTX Families
William Rizzo, M.D., ULF Medical & Scientific Advisory Board Chairman
- 2:00pm - 2:30pm “Summary Current CTX Research/Future Research Directions/Funding Opportunities”
Q & A Speaker Panel Session
- 2:35pm - 2:55pm Long Term Treatment in CTX
Gerald Salen, M.D., Department of medicine, University of Medicine and Dentistry, New Jersey Medical School, Newark, New Jersey
- 3:00pm - 3:30pm Cholic Acid Treatment for CTX
James Heubi, M.D., Department of Pediatrics, University of Cincinnati College of Medicine, Cincinnati, Ohio
- 3:35pm - 4:05pm “Ask the Doc” Questions & Answers Speaker Panel Session
William Rizzo, M.D., ULF Medical & Scientific Advisory Board Chairman
- 4:10pm - 4:30pm CTX Treatment Update
Paula Brazeal, President
Brian Schreiber and GianFranco Fornasini, Sigma-Tau Pharmaceuticals, Inc., Gaithersburg, Maryland
- 4:35pm - 4:55pm John Wolf, ULF Board of Directors, Family Liaison, Aloha, Oregon
- 5:00pm - 5:30pm Break for Dinner and Poster Session Set-up
- 5:30pm DINNER
- 6:30pm Evening Poster Session & Hospitality

INVITED SPEAKERS AND FACILITATORS

Ingemar Bjorkhem
Senior Professor
Division of Clinical Chemistry
Department of Laboratory
Medicine
Karolinska University Hospital
Huddinge, Sweden

Paula K. Brazeal
ULF
President
Sycamore, IL

Andrea DeBarber, PhD
Research Assistant Professor
Physiology & Pharmacology
Department
Oregon Health & Science
University
Portland, OR

Sandra Erickson
Department of Medicine
University of California
Veterans Affairs Medical Center
San Francisco, CA 94121

Professor Antonio Federico
Direttore
U.O. Neurologia e Malattie
Neurometaboliche
Universita degli Studi di Siena
Siena, Italy

James Heubi, M.D.
Department of Pediatrics
University of Cincinnati
College of Medicine
Cincinnati, OH

Ann Moser, B.A.
Kennedy Krieger Institute
Peroxisomal Diseases Lab
Baltimore, MD

Shailesh Patel, M.D.
Division of Endocrinology
Metabolism & Clinical Nutrition
Medical College of Wisconsin
Milwaukee, WI

William Rizzo, M.D.
Department of Pediatrics
University of Nebraska Medical
Center
Omaha, NE

Jean-Baptiste Roulet, PhD
Oregon Health & Science
University
Portland, OR

Gerald Salen, M.D.
Department of Medicine
University of Medicine &
Dentistry
New Jersey Medical School
Newark, NJ

Brian Schreiber
GianFranco Fornasini
Sigma-Tau Pharmaceuticals, Inc.
Gaithersburg, MD

Kenneth Setchell, PhD
Pathology & Laboratory
Medicine
Department of Pediatrics
Cincinnati Children's Hospital
Medical Center
Cincinnati, OH

Maria-Jesus Sobrido, M.D., PhD
Neurogenetics Group
Fundacion Publica Galega de
Medicina Xenomica
Santiago de Compostela
Spain

Robert Steiner, M.D.
Professor
Oregon Health & Science
University
Portland, OR

John Wolf
ULF Board of Directors &
Family Liaison
Aloha, OR

HOTEL GENERAL INFORMATION:

- Complimentary coffee and iced tea available 24 hrs., in Hospitality Center; coffee makers in rooms; cold drink vending machines.
- Pool Hours: 9:00 am - 10:00 pm
- Incidental expenses: Telephone calls, TV, extra hotel nights, etc., are the responsibility of the participants and must be paid at check out time.
- Business Center: Fax and photocopy machines, printer for laptop computer.
- Fitness Center: 6:30am - 9:00 pm
- Check-in 3:00pm and Check-out 11:00am

HOSPITALITY HOURS BEST WESTERN INN

Thursday Registration: 3:00pm
Buffet Dinner & Hospitality: 6:00pm
Welcome Reception for Speakers & STAIR Guests: 6:00pm- 10:00pm
Friday Lunch and Dinner Buffet

BREAKFAST:

Friday Buffet 6:45am - 8:00am
Saturday Continental Breakfast

EMERGENCY TELEPHONE NUMBERS:

- 1) Best Western (815) 758-8661
- 2) ULF Office, Sycamore, IL: (answering machine only):
(815) 895-3211 or (800) 728-5483