Invitation to Participate

Satellite Symposium to the 11th International Congress on Inborn Errors of Metabolism

Overcoming Barriers—New Developments and Future Directions for Urea Cycle Disorders

Estancia La Jolla Hotel
La Jolla, California
August 27-29, 2009
An Invitation

On behalf of the Organizing Committee and the National Urea Cycle Disorders Foundation, we are pleased to invite your participation in the Urea Cycle Disorders Symposium to be held August 27-29, 2009 as a satellite meeting of the 11th International Congress of Inborn Errors of Metabolism. We are excited to be hosting this third satellite meeting of its kind. The first international satellite on urea cycle disorders was held in Vienna, Austria in 1997, the second in Brisbane, Australia in 2003, attracting more than 200 participants from around the world. The proceedings of these meetings formed a foundation for improved understanding of urea cycle disorders, as well as a basis for advancing research. Our vision for this third satellite symposium is to continue the tradition, having it serve as a springboard for innovative collaborations and to provide stimulation for focused research over the coming years.

We have an outstanding scientific program planned to spotlight the most recent research findings in basic, translational and therapeutic investigations for urea cycle disorders. The Satellite will take place over one and a half days, consisting of invited lectures by a distinguished group of renowned clinicians and international scientists, a poster session and short presentation of abstracts. The format will also include formal and informal discussion. Publication of the proceedings is planned as a supplement to Molecular Genetics and Metabolism. Hospitality events include an opening reception the evening before the symposium and dinner following the full-day session.

We are excited to be hosting the satellite at the exclusive Estancia Hotel in the beautiful seaside village of La Jolla, California. The hotel is adjacent to University of California San Diego and the renowned Scripps Research Institute. Recreational activities include paragliding, scuba diving and snorkeling, as well as world-class golf at Torrey Pines Golf Course.

We hope that you will participate in this international effort to ignite new directions for urea cycle disorder research to save and improve the lives of affected children and adults who look to us for hope for the future.

We look forward to seeing you in La Jolla.

Stephen Cederbaum, M.D.
Brendan Lee, M.D., Ph.D.
Cynthia Le Mons, NUCDF
Satellite Co-chairs
Organizing Committee
Stephen Cederbaum, M.D.
University of California, Los Angeles CA

Brendan Lee, M.D., Ph.D.
Baylor College of Medicine, Houston TX
Howard Hughes Medical Institute

Cynthia Le Mons, Executive Director
National Urea Cycle Disorders Foundation

Andrea Gropman, M.D., Ph.D.
Georgetown University
Children’s National Medical Center, Washington DC

Soledad Kleppe, M.D., Ph.D.
CEMIC, Buenos Aires, Argentina

Claude Bachmann, M.D., Emeritus Professor
Bottmingen, Switzerland

Mendel Tuchman, M.D. ex officio
Children’s National Medical Center, Washington DC

Important Dates
Sponsor reservation deadline 15 January 2009
Sponsorship contract deadline 01 February 2009
Sponsorship receipt deadline 01 February 2009
Satellite Dates 27-29 August 2009

Meeting Venue
Estancia La Jolla Hotel
9700 North Torrey Pines Road
La Jolla, California 92037
USA

www.estancialajolla.com

Secretariat
National Urea Cycle Disorders Foundation
75 South Grand Avenue
Pasadena, California 91105
USA
Tel: (818)578-0833
Fax: (818)578-0823
www.nucdf.org

Contact: Cynthia Le Mons
CureUCD@nucdf.org
ABOUT NUCDF

The National Urea Cycle Disorders Foundation is a nonprofit organization dedicated to the identification, treatment and cure of urea cycle disorders. Committed to saving children and adults from the catastrophic effects of UCDs, NUCDF is a lifeline to families and medical professionals all over the world seeking crisis intervention, support and treatment information. The Foundation provides information to affected patients and families, promotes education for healthcare professionals on the identification, diagnosis and treatment of the disorder, stimulates and supports critical research, increases public awareness and educates legislators on the needs of UCD patients and families. NUCDF is dedicated to the development and support of multidisciplinary medical and clinical professionals, advocates, policymakers, industry and consumers working together to improve outcomes for persons affected with urea cycle disorders.

ABOUT THE INTERNATIONAL CONGRESS

The International Congress on Inborn Errors of Metabolism (ICIEM) is held every three years and is designed to bring the best scientists and physicians dedicated to inborn errors from around the world together to discuss advances in the understanding, diagnosis and treatment of this family of conditions. The sponsoring society’s board of directors is made up of the heads of the various national and international organizations dedicated to inborn errors and the sites for the meeting rotate around the globe to maximize the opportunities for more junior colleagues to attend. This year’s meeting is sponsored by the Society for Inherited Metabolic Disorders.

ENDORSING ORGANIZATIONS

National Institutes of Health Office of Rare Diseases
Rare Disease Clinical Research Network Urea Cycle Disorders Consortium
Society for Inherited Metabolic Disorders

ABOUT THE VENUE

Estancia La Jolla is located adjacent to the University of California San Diego (UCSD) and Scripps Research Institute, near the village of La Jolla and world-class shopping, beaches, and San Diego’s attractions.

The hotel features 26,000 square feet of state-of-the-art IACC certified conference facilities, a full-service spa and fitness center and multiple award-winning restaurants and lounges amongst exquisitely landscaped gardens.

Selected by Condé Nast Traveler as one of the "Hottest New Hotels in the World" and as one of the "World's Best Places to Stay" in 2006, the Four-Diamond Estancia La Jolla is renowned as a coastal retreat where tradition, environment and design blend to renew and inspire in the setting reminiscent of a sprawling early California ranch estate. Just a "chip shot" away from Torrey Pines Golf Course, host of the 2008 US Open, and a short stroll from the majestic coves, those seeking natural beauty and renowned golf have discovered the perfect location. Estancia La Jolla Hotel & Spa is truly a retreat from the ordinary.
**PROGRAMME TOPICS AND SPEAKERS**

**Introduction and Goals of the Conference**  
Stephen Cederbaum, M.D. - CONFIRMED  
Associate Professor, Psychiatry and Pediatrics  
Associate Director for Clinical Research, UCLA Mental Retardation Research Center  
Principal Investigator, Urea Cycle Disorders Consortium  
University of California at Los Angeles, CA

**Current Concepts in the Pathogenesis of Urea Cycle Disorders**  
Olivier Braissant, Ph.D., MER - CONFIRMED  
Head, Research Unit on Inborn Errors of Metabolism  
Laboratoire de Chimie Clinique  
Lausanne, Switzerland

**UCD Longitudinal Study Update: Rare Diseases Clinical Research Network Urea Cycle Disorders Consortium**  
Mark L. Batshaw, M.D. – CONFIRMED  
Chief Academic Officer & Director, Clinical Research Center  
Principal Investigator, Urea Cycle Disorders Consortium  
Children’s National Medical Center

**N-Acetyl Glutamate Synthetase Deficiency and Carbamylglutamate Therapy**  
Mendel Tuchman, M.D. - CONFIRMED  
Vice Chairman for Research  
Scientific Director, Children’s Research Institute  
Professor of Pediatrics, Biochemistry and Molecular Biology  
Principal Investigator, Urea Cycle Disorders Consortium  
Children’s National Medical Center, Washington, DC

**Measurement of Urea Cycle Flux in Health and Disease**  
Marc Yudkoff, M.D. - CONFIRMED  
Chief, Division of Child Development, Rehabilitation and Metabolic Disease  
Children’s Hospital of Philadelphia  
Philadelphia, PA

**Nitrogen Sparing Therapy**  
Greg Enns, M.D., Ch.B. - CONFIRMED  
Director, Biochemical Genetics Program  
Division of Medical Genetics  
Stanford University  
Palo Alto, CA

**New Insights in Nutritional Management and Amino Acid Supplementation**  
Fernando Scaglia, M.D., FACMG - CONFIRMED  
Associate Professor of Genetics  
Department of Molecular and Human Genetics  
Baylor College of Medicine and Texas Children’s Hospital  
Houston, TX

**The Urea Cycle and Nitric Oxide Synthesis**  
Brendan Lee, M.D., Ph.D. - CONFIRMED  
Investigator, Howard Hughes Medical Institute  
Professor, Department of Molecular and Human Genetics  
Principal Investigator, Urea Cycle Disorders Consortium  
Baylor College of Medicine  
Houston, TX

**The Urea Cycle and Creatine Metabolism**  
John Brosnan, Ph.D. - CONFIRMED  
Department: Biochemistry, Memorial University  
Newfoundland & Labrador, Canada

**Brain Imaging in Urea Cycle Disorders**  
Andrea Gropman, M.D., FAAP, FACMG - CONFIRMED  
Associate Professor of Neurology and Pediatrics  
Department of Neurology  
Children’s National Medical Center  
Washington, DC

**Citrin Deficiency and Current Treatment Concepts**  
Takeyori Saheki, M.D., Ph.D. - CONFIRMED  
Institute for Health Sciences  
Tokushima Bunri University  
Tokushima, Japan

**Liver Transplantation for Urea Cycle Disorders**  
Georg Hoffman, M.D. - CONFIRMED  
Department of General Pediatrics, Division of Metabolic and Endocrine Diseases  
University Children’s Hospital  
Heidelberg, Germany
Breaching the Blood-Brain Barrier
Edward Neuwelt, M.D.
Professor, Departments of Neurology and Neurosurgery
Director, Neuro-Oncology Blood Brain Barrier Program
Oregon Health & Science University
Portland, OR

Amplitude Integrated EEG
Christiane Theda, M.D., Ph.D, MBA - CONFIRMED
Assistant Professor, Department of Pediatrics
Johns Hopkins University School of Medicine

Neuroimaging: New Frontiers
Andrea Gropman, M.D. - CONFIRMED
Associate Professor of Neurology and Pediatrics
Department of Neurology
Children's National Medical Center

Ammonia Toxicity to the Brain and Creatine
Olivier Braissant, Ph.D., MER - CONFIRMED
Head, Research Unit on Inborn Errors of Metabolism
Laboratoire de Chimie Clinique
Lausanne, Switzerland

Session Moderators:

Marshall B. Summar, M.D. - CONFIRMED
Associate Professor of Pediatrics, Molecular Physiology and Biophysics
Division of Medical Genetics
Vanderbilt University Medical Center
Nashville, TN

Robert Steiner, M.D.,FAAP, FACMG
Vice Chairman for Pediatric Research
Doernbecher Children's Hospital
Professor of Pediatrics, Molecular and Medical Genetics, Oregon Health & Science University School of Medicine
Portland, OR

Marc Yudkoff, M.D. - CONFIRMED
Chief, Division of Child Development, Rehabilitation and Metabolic Disease
Children’s Hospital of Philadelphia
Philadelphia, PA

Michaël Johnston, M.D. - CONFIRMED
Professor of Neurology and Pediatrics
Johns Hopkins University School of Medicine
Research Investigator
Kennedy-Krieger Institute, Baltimore, MD

Mechanisms of Neural Brain Injury, Application to IEMs
Michael Johnston, M.D. - CONFIRMED
Professor of Neurology and Pediatrics
Johns Hopkins University School of Medicine
Research Investigator
Kennedy-Krieger Institute, Baltimore, MD

Mechanism of Age-Dependent Susceptibility and Novel Treatment Strategy in GA Type I
William Zinnanti, Ph.D. - CONFIRMED
Penn State College of Medicine
Hershey, PA

Brain Metabolism in Action: In Vivo 13C MRS
Metabolic Modeling with Neuronal-Glial Models
Brian Ross, M.D., Ph.D., FRCS - CONFIRMED
Director of MR Spectroscopy Unit
Huntington Medical Research Institutes
Pasadena, CA

Blood Brain Barrier Drug Targeting: The Future of Brain Drug Development
William Pardridge, M.D.
Professor of Medicine
Director, Blood Brain Barrier Research Laboratory
University of California at Los Angeles, CA

Invited NIH Participants:

Stephen C. Groft, Pharm.D
Director
NIH Office of Rare Diseases

Mary Lou Oster-Granite, PhD
Health Scientist Administrator
Intellectual and Developmental Disabilities Branch
Center for Developmental Biology and Perinatal Medicine
Eunice Kennedy Shriver National Institute of Child Health and Human Development

Melissa Parisi, M.D., Ph.D.
Chief, Intellectual and Developmental Disabilities Branch
Center for Developmental Biology and Perinatal Medicine
Eunice Kennedy Shriver National Institute of Child Health and Human Development

Catherine McKeon, Ph.D.
Senior Advisor for Genetic Research
National Institute of Diabetes, Digestive, Kidney Diseases

Danilo Tagli, Ph.D.
Program Director, Neurogenetics
National Institute for Neurological Disorders and Stroke

Giovanna Spinella, M.D.
Science and Program Consultant
NIH Office of Rare Diseases
General Information

REGISTRATION

Full registration $295.00 (US) Late registration: $340.00 (after July 27)
Student $150.00 (US) Student late registration $195.00 (after July 27)
Includes:
- Sessions
- Breakfast Friday and Saturday
- Afternoon Breaks
- Lunch Friday
- Satellite Dinner Friday
- Welcome Reception Thursday

To register: http://www.nucdf.org/UCD_Satellite.htm
ICIEM Delegates may register for the UCD Satellite on their ICIEM registration form:
http://www.iciem2009.org/registration.html

CME Accreditation provided by Children’s National Medical Center of Continuing Medical Education

Welcome Reception
For all delegates to relax, network and renew friendships over cocktails and hors d’oeuvres on the beautiful grounds of the Estancia La Jolla Hotel, a renowned coastal retreat reminiscent of a California rancho estate, selected by Conde Nast as one of the “World’s Best Places to Stay.”

Date: Thursday, August 27
Time: 7:00 pm
Cost: Included in full registration

Accompanying persons (US $35.00)

Symposium Dinner
Our dinner will be prepared by the Estancia’s renowned chefs and held on the grounds of the gracious Estancia Hotel and Spa, a short stroll from the Pacific Ocean and the majestic coves of La Jolla.

Date: Friday, August 28
Time: 7:00 pm
Cost: Included in full registration

Accompanying persons (US $50.00)

POSTER SESSION AND ABSTRACTS
Call for electronic submission of abstracts is open through June 1, 2009. Participation of young investigators is encouraged. Students and junior investigators are eligible to compete for travel stipends based on need and quality of submission. Download applications at http://www.nucdf.org/UCD_Satellite.htm

TRAVEL INFORMATION
Estancia La Jolla Hotel is located a short 15-mile driving distance from the San Diego International Airport. Shuttle service, cabs and private carpools with other delegates will be available to satellite registrants (contact Satellite Secretariat for information and to reserve space).

Temperatures in La Jolla in August typically range from 81°F daytime to 62°F evening.

ACCOMMODATIONS

Accommodations have been reserved at a reduced rate of US $195.00 plus tax per night at the Estancia La Jolla Hotel and Spa (meeting venue). Reservation deadline is July 27, 2009 (regular rate of $495.00++ subject to availability apply). For reservations call Estancia La Jolla at (877)427-8262, using Group Code NUCDF09, or make reservations securely online at: https://reservations.synxis.com/XBE/rez.aspx?Hotel=12323&Chain=5156&group=NUCDF09

The Satellite meeting will be held at the world-class Estancia Hotel and Spa, adjacent to University of California at San Diego and Scripps Research Institute, in the beautiful seaside village of La Jolla, California. The Estancia offers a non-traditional approach in a premiere conference destination. Charming courtyards, patios, native gardens and the Executive Learning Retreat encompass more than 21,500 square feet of sophisticated meeting space. Renowned for its ideal learning environment and certified by International Association of Conference Centers, Estancia La Jolla raises the bar in achievement.

The four-diamond Estancia La Jolla Hotel and Spa is adjacent to University of California San Diego, Scripps Research Institute and the seaside village of La Jolla, California, home of world-class shopping and beaches. The Estancia is a “chip shot” away from Torrey Pines Golf Course, host of the 2008 US Open and a short stroll from paragliding cliffs, majestic coves and water sports. The hotel can make special arrangements for guests for the golf course and other activities. The hotel concierge would be pleased to help you with activity planning.

Estancia La Jolla Hotel
9700 North Torrey Pines Road
La Jolla, CA 92307
Phone: (858)964-6558
http://www.estancialajolla.com