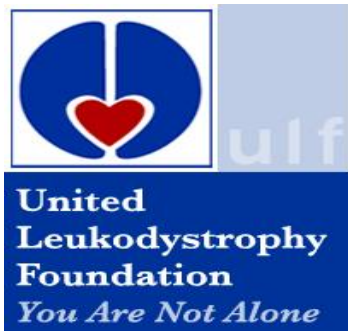


**PRE-REGISTRATION ONLY
NO WALK-INS ACCEPTED
Register by Monday
September 15, 2008**



**UNITED
LEUKODYSTROPHY
FOUNDATION**

LOVE AND SCIENCE 2008

Metachromatic Leukodystrophy (MLD)
Symposium

September 19, 20, 2008

DeKalb, Illinois

LOVE AND SCIENCE 2008

Metachromatic Leukodystrophy (MLD) Symposium

September 19, 20, 2008

The Conference Goals Are:

- To facilitate international collaboration to further research in Metachromatic Leukodystrophy
- To provide information to individuals and families concerning research, the genetic and clinical manifestations of Metachromatic Leukodystrophy
- To develop patient registries for Metachromatic Leukodystrophy

This conference is sponsored by the United Leukodystrophy Foundation and is intended as an educational and social opportunity for individuals, family members, and physicians.

LODGING:

Best Western, 1212 W. Lincoln Hwy., DeKalb, Illinois. One night lodging per family is included with your registration. You will be responsible for payment of extra night (s) at \$89.00 per night plus tax. Please let the ULF know the days you are planning on staying. We have blocked all rooms and will advise the hotel of your intentions.

IF YOU ARE FLYING.....:

Ground transportation between the Airport and DeKalb can be arranged in advance through Custom Limousine, please call: (800) 397-7921 or car rentals are available at the airport. These are transportation suggestions from the ULF.

CHECK-IN:

Check-in begins Thursday September 18th; 3:00pm at the Best Western.

MEALS:

Breakfast:

Continental Breakfast available in the Hotel

Lunch:

Friday 12:00 - 1:00pm

Saturday 12:00 - 1:00pm

Dinner:

Thursday night 6:00 pm,

Friday night 6:00 pm

Saturday night 6:00 pm

MLD SYMPOSIUM MEETING WEAR IS CASUAL!

MLD SYMPOSIUM AGENDA

**Friday
September 19th, 2008**

7:00 am – 8:00 am Continental Breakfast

Welcome and Introductions

8:00 am – 8:15 am Paula Brazeal, ULF President
William Kintner, ULF Vice-President

Peter L. Saltonstall, President, CEO of NORD

Florian Eichler, M.D.
ULF Medical & Scientific Advisory Board

Biochemistry and Genetics of MLD:

8:15 am – 8:45 am ARSA Mutations - Why Bother?
Arvan Fluharty, M.D.

8:45am – 9:15 am Diagnosis of MLD
David Wenger, Ph.D.

9:15 am - 9:45 am Experience with Newborn Screening for Lysosomal Disorders
Robert Vogt, Ph.D.

9:45 am - 10:00 am Break

Experimental Models of MLD:

10:00 am-10:30 am Increasing Sulfatide Synthesis in Myelin-forming Cells of Arylsulfatase
A-deficient Mice
Volkmar Geiselmann, M.D., Ph.D.

10:30 am - 11:00 am Neurological Deficits and Glycosphingolipid Accumulation in
Saposin B deficient Mice
Gregory Grabowski, M.D.

MLD SYMPOSIUM AGENDA

**Friday
September 19th, 2008**

Continued

Natural History of MLD:

- 11:00 am - 11:30 am Assessing Neurodevelopment in Infantile MLD
Maria Escolar, M.D.
- 11:30 am - 12:00 pm Assessing Behavior and Cognitive Functioning in Adult MLD
Nicole Baumann, M.D.
- 12:00pm - 1:00 pm Lunch
- 1:00 pm - 1:30 pm Peter L. Saltonstall, President, CEO of NORD
- 1:30 pm - 2:00 pm Quantitative MR Measures for Progression in MLD
Florian Eichler, M.D.
- 2:00 pm - 2:30 pm Database Options for a Natural History Study
Alfried Kohlschütter, M.D., Ph.D.

Clinical and Family Care Part I:

- 2:30pm - 4:00 pm Clinical and Family Care Panel Discussion, Part I
Moderator: William Kintner, D.Min., Th.M., LMFT
Michele Philippart, M.D., Maria Escolar, M.D.,
Colleen Kintner, M.A., LMFT
- 4:00 pm *International MLD Registry (Registry Members Only)*
- 4:00 pm - 6:00 pm Hospitality and R&R for all participants
- 6:00 pm Dinner & Hospitality with Scientists and Families

MLD SYMPOSIUM AGENDA

**Saturday
September 20th, 2008**

Immunology of MLD:

- 8:00 am - 8:30 am Dysmyelination and Demyelination Caused by Anti-Glycolipid Antibodies.
Jack Rosenbluth, M.D.
- 8:30 am - 9:00 am Epitope Spreading within the CNS
Stephen Miller, Ph.D.
- 9:00 am - 9:30 am Non-inhibitory Antibodies and Lysosomal Storage Reduction during ERT
Volkmar Gieselmann, M.D., Ph.D.

Enzyme Replacement in MLD:

- 9:30 am-10:00 am Enzyme Replacement Therapy for Late-Infantile MLD
Norman Barton, M.D., Ph.D.
- 10:00 am - 10:30 am Ameliorating Lysosomal Storage Diseases by Restoring Proteostasis
Ting Wei Mu, Ph.D.

MLD Therapy of the Future Part I:

- 10:30 am – 11:00 am Prospects for Treating the CNS Disease in LSDs
Edward Kolodny, M.D.,

Cell Based Therapies in MLD Part I:

- 11:00 am - 11:45 am HSCT in MLD
Lawrence Charnas, M.D., Ph.D.
- 11:45 - 1:00 pm Lunch

MLD SYMPOSIUM AGENDA

**Saturday
September 20th, 2008
Continued**

MLD Therapy of the Future Part II:

1:00 pm - 1:30 pm Combination Therapies for Lysosomal Disorders
Mark Sands, Ph.D.

Cell Based Therapies in MLD Part II:

1:30 pm - 2:00 pm Gene Therapy with Lentiviral Vector for MLD
Maria Sessa, M.D.

2:00 pm - 2:15 pm Break

Clinical and Family Care Part II:

2:15 pm - 4:30 pm Clinical and Family Care Panel Discussion, Part II
Moderator: William Kintner, D.Min., Th.M., LMFT
William Sweet, R.N., Lawrence Charnas, M.D., Ph.D.,
Colleen Kintner, M.A., LMFT

4:30 pm – 5:00 pm Open Forum & ULF Summary

6:00 pm Dinner & Hospitality with Scientists and Families

INVITED SPEAKERS

Norman Barton, M.D., Ph.D.
Shire Human Genetic Therapies, Inc.
Cambridge, MA

Nicole Baumann, M.D.
Hôpital de la Salpetriere
Paris, France

Paula K. Brazeal
ULF President
Sycamore, IL

Ron Brazeal
ULF Executive Director
Sycamore, IL

Lawrence Charnas, M.D., Ph.D.
University of Minnesota
Minneapolis, MN

Victor Raúl De Jesús, Ph.D.
The Center for Disease Control
Atlanta, GA

Florian Eichler, M.D.
Massachusetts General Hospital
Boston, MA

Maria L. Escolar, M.D., M.S.
The University of North Carolina
Chapel Hill, NC

Arvan Fluharty, Ph.D.
U.C.L.A.
Los Angeles, CA

Volkmar Gieselmann, M.D., Ph.D.
Rheinische Friedrich-Wilhelms
University of Bonn
Germany

Gregory A. Grabowski, M.D.
Cincinnati Children's Hospital Med. Center
Cincinnati, Ohio

William Kintner, D.Min., Th.M., LMFT
ULF Vice-President,
Pastor, Psychotherapist
Redlands, CA

Colleen Kintner, M.A., LMFT
ULF Vice-President
Redlands Community Hospital
Redlands, CA

Alfried Kohlschuetter, M.D.
University of Hamburg
Hamburg, Germany

Ed Kolodny, M.D.
NYU Langone Medical Center
New York, NY

Stephen D. Miller, Ph.D.
Northwestern University
Medical School
Chicago, IL

Ting Wei Mu, Ph.D.
The Scripps Research Institute
La Jolla, CA

Michel S Philippart, M.D.
U.C.L.A.
Los Angeles, CA

Jack Rosenbluth, M.D.
NYU Langone Medical Center
New York, NY

Peter L. Saltonstall
National Organization
for Rare Disorders (NORD)
Danbury, CT

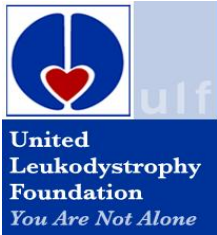
Mark S. Sands, PhD
Washington University
St. Louis, MO

Maria Sessa, M.D.
San Rafael Scientific Institute
Milano, Italy

William Sweet, R.N.
Community Medical Alliance
Boston, MA

Robert Vogt, Ph.D.
The Center for Disease Control
Atlanta, Georgia

David A. Wenger, Ph.D.
Jefferson Medical College
Philadelphia, PA



Metachromatic Leukodystrophy (MLD)

Symposium

Registration Form

September 19, 20, 2008

**NO
WALK-INS
Register by
Monday
September 15, 2008**

First & Last Name: _____

First & Last Name: _____

First & Last Name: _____

Address: _____

City: _____ *State:* _____ *Zip:* _____ *E-mail:* _____

Telephone :() _____ *Cell Phone: ()* _____

ROOM INFORMATION:

_____ *Vegetarian* _____ *# in room* *Handicapped Pls. Explain:* _____

ARRIVAL INFORMATION:

_____ *Staying: Thursday September 18, 2008*

_____ *Staying: Friday September 19, 2008*

_____ *Staying: Saturday September 20, 2008*

Approx. arrival time is _____, *Check in at hotel 3:00p.m. (Central Time)*

ULF SPONSORED MLD Symposium

Return Registration Form To:

ULF MLD Symposium
2304 Highland Drive
Sycamore, IL 60178

FAX registration form to: (815) 895-2432
or call (800) 728-5483 - (815) 895-3211
or e-mail: Office@ulf.org

This two day Metachromatic Leukodystrophy Symposium is Sponsored by the United Leukodystrophy Foundation, including supporting sponsorship by Shire Human Genetic Therapies

There is no registration fee.

The ULF will be providing: hotel room for a night, and dinner September 18; lunch, & dinner September 19, 20, 2008.