

Dear Colleagues,

Welcome to the 2005 WORLD (**World Organization for Research on Lysosomal Diseases**) Scientific Symposium presented by the Lysosomal Disease Network. This is the second annual symposium, the first was held in Minneapolis, Minnesota in 2004. This year's meeting will emphasize neurological aspects of lysosomal diseases, including potential treatments for many of the lysosomal diseases with central nervous system effects that are not yet in place, and exploration of CNS effects in currently treatable diseases. We are excited to have you join us for this important event!

We have assembled an outstanding program, which has been organized into four (4) sessions: Current Laboratory Approaches to Understanding and Treating Lysosomal Diseases, Promising Clinical Approaches in CNS Diseases, Innovative Therapies, Promising Clinical Approaches in CNS Diseases-Gene Therapy, and Longitudinal and Outcome Studies in CNS Diseases. Concurrent disease-specific lectures on the afternoon of May 22, designed specifically for patients and patient advocates will include educational presentations, and round table discussions. We look forward to welcoming you to the WORLD Scientific Symposium and to your participation in this exciting and important meeting.

Sincerely,

The **WORLD** Symposium Organizing Committee

SYMPOSIUM PURPOSE

This symposium is a multidisciplinary forum to present and discuss basic and clinical research discoveries in Lysosomal diseases, and related treatment, and quality of life issues. Specific aims are to: 1) foster interdisciplinary collaboration with the overall goal of improving knowledge of basic discoveries and clinical manifestations of these diseases; 2) identify and discuss the latest findings in the natural history of Lysosomal Diseases, diagnostic testing and screening, and treatment; and 3) identify areas requiring additional basic/clinical research public policy and regulatory attention.

SYMPOSIUM AUDIENCE

Clinicians, geneticists and genetic counselors, neurologists and neuropsychologists, researchers, health care professionals, patients and families, patient/family support organizations and industry professionals.

ORGANIZING COMMITTEE

Chester B. Whitley, PhD, MD, Chair
Lawrence Charnas, MD, PhD
Elsa G. Shapiro, PhD
Janet Hegland

SPEAKERS

Lorne A. Clarke, MDCM, FRCPC, FCCMG
Children's and Women's Hospital University of British Columbia, Vancouver, British Columbia, Canada

Lawrence Charnas, MD, PhD
University of Minnesota, Minneapolis, Minnesota

Ronald G. Crystal, MD
Weill Medical College, Cornell University, New York, NY

Christine Eng, MD

Baylor College of Medicine, Houston, Texas

Maria Escolar, PhD
Duke University, Chapel Hill, North Carolina

Gregory A. Grabowski, MD
Children's Hospital Research Foundation, Cincinnati, Ohio

Priya Sunil Kishnani, MD
Duke University, Durham, North Carolina

Donald Mahuran, Ph.D.
Toronto, Ontario, Canada

Margaret M. McGovern, MD, PhD
Mount Sinai School of Medicine of New York University, New York, New York

Joseph Muenzer, PhD, MD
University of North Carolina, Chapel Hill, North Carolina

Gail Ouellette, PhD
CHUS-Fleurimont Hospital, Sherbrooke, Quebec, Canada

Marco Passini, PhD
Genzyme Corporation, Cambridge, Massachusetts

Gregory M. Pastores, MD
New York University School of Medicine, New York, New York

Marc Patterson, MD, FRACP
Columbia University Medical Center, New York, New York

Raphael Schiffmann, MD, PhD
NINDS, National Institutes of Health, Bethesda, Maryland

Elsa Shapiro, PhD
University of Minnesota, Minneapolis, Minnesota

Robert Steiner, MD
Oregon Health Sciences University, Portland, Oregon

Neal Weinreb, MD
University Hospital, Tamarac, Florida

Chester B. Whitley, PhD, MD
University of Minnesota, Minneapolis, Minnesota

William R. Wilcox, MD, PhD
UCLA School of Medicine, Cedars-Sinai Medical Center, Los Angeles, California

DISEASE FOCUS

- Mucopolysaccharidoses, e.g., Hurler syndrome (I), Hunter syndrome (II), Sanfilippo syndrome (III), Morquio syndrome (IV), Maroteaux-Lamy syndrome (VI), Sly syndrome (VII)
- Fabry disease

- Gaucher disease
- Globoid Cell Leukodystrophy (Krabbe disease)
- Pompe disease
- Tay-Sachs disease
- Neuronal Ceroid Lipofuscinosis (Batten disease)
- Niemann-Pick disease
- Metachromatic leukodystrophy (MLD)

DISCLOSURE POLICY

It is the policy of the Lysosomal Disease Network to ensure balance, independence, objectivity and scientific rigor in all of its sponsored educational activities. All speakers participating in sponsored programs are expected to disclose to the program audience any real or apparent conflict of interest related to the content of their presentation.

CONFERENCE LOCATION / OVERNIGHT ACCOMMODATIONS / PARKING

Conference organizers have selected:

Wigwam Resort and Golf Club
300 Wigwam Boulevard
Litchfield Park, Arizona, 85340

Reservations: 623-935-3811 or toll-free 1-800-SAY PUTT (1-800-729-7888)

website: www.wigwamresort.com

Lodging

A block of sleeping rooms is available at The Wigwam Resort and Golf Club at the special conference rate of \$175.00 single or double occupancy per night plus resort fee (\$15.00) applicable taxes (currently 8.3%).

All participants requiring accommodations should call the hotel directly at 623-935-3811 or toll-free 1-800-SAY PUTT (1-800-729-7888) before **April 21st**. When making your reservations, be sure to identify yourself as an attendee of the **WORLD Symposium**. If you are also attending the Genzyme Registries meeting, please make two hotel reservations, one for the Genzyme meeting and a second for WORLD for the night of May 22.

Reservations received after **April 21st**, will be subject to space availability and at prevailing room rates.

REGISTRATION FEES

Please register by April 21st using the registration form in this brochure. The fees listed on the Registration Form includes: records processing, conference materials, continental breakfast, lunch, dinner and refreshment breaks.

Seating is limited, so early registration is strongly advised. A letter of confirmation will be sent to you once we receive your completed registration form.

REGISTER BY FAX, EMAIL, OR MAIL,

You may choose ONE of the following methods to register for this conference.

FAX: (612) 626-0665

EMAIL: hegla005@umn.edu

MAIL: WORLD Symposium, Attn: Janet Hegland, University of Minnesota,, 420 Delaware St. SE,
MMC 395, Room 12-258 Moos Tower, Minneapolis, MN 55455

CANCELLATION POLICY

In the event that you need to cancel your registration, the registration fee, less a \$50 administrative fee, will be refunded if you notify us by 4:30 p.m. CDT on April 29th, 2004. No refunds will be made after this date.

OUR WEB SITE:

WWW.WORLD.UMN.EDU – Additional information on this symposium and general information on WORLD (World Organization for Research on Lysosomal Diseases) Symposium, presented by the Lysosomal Disease Network.

PRELIMINARY PROGRAM

Sunday, May 22, 2005

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|------------|--|--------------------|
| 7:00 a.m. | REGISTRATION & CONTINENTAL BREAKFAST | |
| 7:45 a.m. | Welcome and Opening Remarks | Chester Whitley |
| 8:00 a.m. | Session I - Current Laboratory Approaches to Understanding and Treating Lysosomal Diseases
<i>MODERATOR: Chester Whitley</i> | |
| | A New Murine Model for Gaucher Disease | Lorne Clarke |
| | Microarray Studies of Tissues in Murine Models of Prosaposin Deficiency and Gaucher Disease | Gregory Grabowski |
| | Genetic Testing for Rare Disorders | Christine Eng |
| | A Difficult Journey But a Happy Outcome for an Orphan Disease Patient: Early Screening and Early Treatment of MPS I | Gail Ouellette |
| 9:20 a.m. | Session II - Promising Clinical Approaches in CNS Diseases-Innovative Therapies
<i>MODERATOR: Robert Steiner</i> | |
| | Long Term Experience with Recombinant Human Acid Alpha Glucosidase Enzyme Replacement Therapy for Infantile Pompe Disease | Priya Kishnani |
| | Modifier Genes and the Vasculopathy of Fabry Disease | Raphael Schiffmann |
| 10:00 a.m. | MORNING REFRESHMENT BREAK, EXHIBITS | |
| 10:20 a.m. | Resume Session II - Promising Clinical Approaches in CNS Diseases-Innovative Therapies | |
| | Pharmacological Chaperones: Unraveling the Mutational Knot in Adult Tay-Sachs Disease. | Donald Mahuran |

Panel Discussion: Neurological Response to Therapy: Stabilization, Slow Decline or Improvement
Lawrence Charnas

11:10 a.m. **Session III - Promising Clinical Approaches in CNS Diseases-Gene Therapy Approaches**

MODERATOR: Gregory Grabowski

AAV-Mediated Gene Therapy of Niemann Pick A Disease Marco Passini

AAV Gene Therapy for Hunter Syndrome and Sanfilippo Syndrome Type B Joseph Muenzer

Lentivirus Gene Therapy for Hurler Syndrome Chester Whitley

Gene Therapy for Batten Disease Ronald Crystal

12:30 p.m. LUNCH

1:45 PM – 5:00 PM **CONCURRENT DISEASE-SPECIFIC FAMILY/SUPPORT GROUP BREAKOUT SESSIONS**

2:00 p.m. **Session IV - Longitudinal and Outcome Studies IN CNS Diseases**

MODERATOR: William Wilcox

Spectrum of Disease Manifestations in Type B Niemann Pick Margaret McGovern

Clinical Studies of Niemann-Pick Disease Type C Marc Patterson

Late-Onset Tay-Sachs Disease: Assessment of Functional Disease Severity Gregory Pastores

Neurodevelopmental Outcomes of Umbilical Cord Blood Transplantation for Infantile Krabbe Disease Maria Escolar

3:20 p.m. AFTERNOON REFRESHMENT BREAK, EXHIBITS

3:50 p.m. **Resume Session IV - Longitudinal and Outcome Studies In CNS Diseases**

Longitudinal Outcomes of Early- and Late-Onset Metachromatic Leukodystrophy after Hematopoietic Stem Cell Transplantation Elsa Shapiro

Challenges in Treating Neuronopathic Gaucher Disease: A Report From the Gaucher Registry Neal Weinreb

4:30 p.m. Closing Remarks

4:45 p.m. **ADJOURN**

6:00 pm - DINNER AND LYSOSOMAL DISEASE NETWORK BUSINESS MEETING
Join your fellow symposium participants for dinner. A brief business meeting will follow dinner.

EXHIBITS

Exhibits will be open to all participants throughout the symposium.

WORLD SYMPOSIUM REGISTRATION FORM

Please Type or Print Clearly

Name _____

Title/Organization/Affiliation _____

Address _____

City _____ State _____ Zip _____

Office Telephone Number _____ FAX _____

E-mail _____

Degree PhD MD DO PA NP RN Other _____

REGISTRATION FEES:	On or Before April 29, 2005	On Site Registration
<input type="checkbox"/> <i>Corporate</i> (scientists, physicians, other representatives employed by for-profit corporations/organizations):	\$ 450.00	\$ 500.00
<input type="checkbox"/> <i>Scientists/Physicians</i> (academic, government, non-profit or private health care institutions):	\$ 150.00	\$ 200.00
<input type="checkbox"/> <i>Other Health Professional</i> (patient support advocacy group representatives, adult LSD patient/parent):	\$ 100.00	\$ 150.00
<input type="checkbox"/> <i>Medical Students, Residents, Fellows and Trainees:</i>	\$ 100.00	\$ 150.00
<input type="checkbox"/> <i>Adult LSD patient/parents attending only Family/Support Group Sessions:</i>	Fee Waived	Fee Waived

FAMILY/SUPPORT GROUP BREAKOUT SESSION:

Please indicate the topic(s) of interest that you would like to see presented at the Patient/Family support group breakout session:

- Fabry disease
- Mucopolysaccharidosis (Please circle one: I II III IV VI VII)
- Mucopolidosis
- Neuronal ceroid lipofuscinosis (Batten disease)
- Pompe disease
- Gaucher disease
- Tay-Sachs disease
- Niemann-Pick disease
- Globoid cell leukodystrophy (Krabbe disease)
- Metachromatic leukodystrophy (MLD)
- Mannosidosis
- Other: _____

Payment Method

Check (payable to University of Minnesota in U.S. Dollars)

VISA MasterCard American Express

Card Number _____

Expiration Date _____

Signature _____

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MMC 395, Room 12-258 Moos Tower, Minneapolis, MN 55455