



Together We Are Strong

**7th International Glycoproteinoses Conference
Minneapolis, MN**

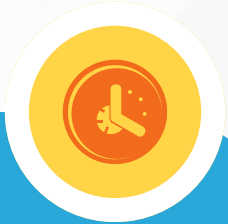


**THURS. AUG 7TH
thru SAT. AUG 9TH**

This is a Preliminary Program Only. All times and speakers will be subject to change. Be sure to check back as updates are made.

More Information:  www.ismrd.org

Program



THURSDAY, AUGUST 7TH

TOGETHER WE ARE STRONG

ISMRD is delighted to introduce you to its 7th International Glycoproteinoses Family and Scientific Conference. The theme of our conference is keeping with ISMRD's mission of seeking to detect and cure the Glycoprotein Storage Diseases, and to provide a global network of support and information, through partnerships built with medicine, science and industry. Working together is our **GREATEST STRENGTH!**

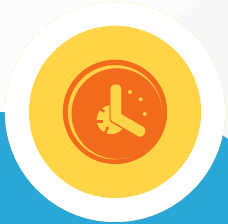
WELCOME

3:00 PM -6:00 PM

6:00 PM - 8:00 PM

- REGISTRATION
- OPENING RECEPTION
Welcome & Introduction
Marc Patterson, Ph.D., M.D. &
Carolyn Paisley-Dew, ISMRD President

Program



FRIDAY, AUGUST 8TH

7:00 AM - 8:30AM

- BREAKFAST

8:30 AM - 8:50 AM

- **Keynote Presentation** - Welcome and Perspectives on ISMRD - Chester B. Whitley, Ph.D., M.D.

8:50 AM - 9:05 AM

- Therapy for Genetic Diseases: Overview and Considerations for the Glycoproteinoses
Marc Patterson, Ph.D., M.D.

9:10 AM - 9:25 AM

- Resilience

ALPHA-MANNOSIDOSIS

9:30 AM - 9:35 AM

- Overview of Alpha-Mannosidosis

9:35 AM - 9:50 AM

- Long-Term Effects of BMT in Alpha-Mannosidosis

9:55 AM - 10:10 AM

- Gene Therapy for Alpha-Mannosidosis

10:15 AM - 10:30 AM

- Alpha-Mannosidosis Patient Perspective

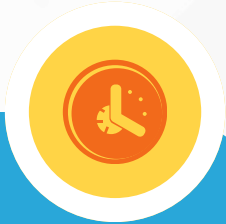
10:35 AM - 10:50 AM

- Enzyme Replacement Therapy - A European model

10:50 AM - 11:05 AM

- *Coffee Break*

Program



FRIDAY, AUGUST 8TH *continued*

BETA-MANNOSIDOSIS AND ASPARTYLGLUCOSAMINURIA (AGU)

- 11:05 AM - 11:10 AM
 - 11:15 AM - 11:30 AM
 - 11:35 AM - 11:50 AM
 - 11:55 AM - 12:10 PM
 - 12:15 PM - 12:30 PM
 - 12:30 PM - 1:30 PM
 - 1:30 PM - 2:30 PM
- Overview of Beta-Mannosidosis and AGU
 - The Lost Enzyme Project
 - Beta-Mannosidosis
 - Beta-Mannosidosis Parent and Patient Advocate
 - Aspartylglucosaminuria (AGU)
 - LUNCH
 - MEMORIAL SERVICE & CONFERENCE PHOTO

FUCOSIDOSIS

- 2:30 PM - 2:35 PM
 - 2:35 PM - 2:50 PM
 - 2:55 PM - 3:10 PM
 - 3:15 PM - 3:30 PM
 - 3:35 PM - 3:55 PM
- Overview of Fucosidosis
 - HSCT for Fucosidosis
 - Fucosidosis
 - Fucosidosis Parent and Patient Advocate
 - Recap of Day 1 Sessions

- 4:00 PM - 5:00 PM
 - 5:00 PM
- Breakout Sessions for Alpha-Mannosidosis, Beta-Mannosidosis, combined AGU & Fucosidosis
 - CLOSE OF DAY
Dinner on Your Own

Program



SATURDAY, AUGUST 9TH

7:30 AM - 9:00 AM

BREAKFAST

MUCOLIPIDOSIS

9:00 AM - 9:10 AM

- Overview of Mucopolidosis

9:15 AM - 9:30 AM

- Heart Defects and Gene Therapy

9:35 AM - 9:50 AM

- S1S3 GNPTAB Gene Therapy in MLII Mice

9:55 AM - 10:10 AM

- Small Molecule Therapy for MLII

10:15 AM - 10:30 AM

- Mucopolidosis Patient Advocate Perspective

10:30 AM - 10:45 AM

- *Coffee Break*

10:50 AM - 11:00 AM

- Mechanisms of Drug Resistance in MLII

11:05 AM - 11:20 AM

- New MLII-Related Disease Caused by LYSET Mutations

11:25 AM - 11:40 AM

- Mucopolidosis

11:45 AM - 12:00 PM

- Lysosomal Exocytosis in ML and Sialidosis

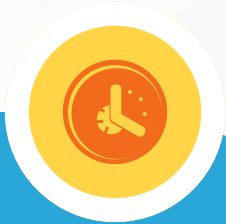
12:05 PM - 12:20 PM

- Childhood Dementia

12:25 PM - 1:30 PM

- LUNCH

Program



SATURDAY, AUGUST 9TH *continued*

SIALIDOSIS AND GALACTOSIALIDOSIS

1:30 PM - 1:35 PM
1:40 PM - 1:55 PM
2:00 PM - 2:15 PM
2:20 PM - 2:35 PM
2:40 PM - 2:55 PM

- Overview of Sialidosis and Galactosialidosis
- Therapeutic Approaches for Sialidosis
- Gene Therapy in Large Animal Models
- Small Molecule Therapies for Sialidosis
- Galactosialidosis Network

SCHINDLER DISEASE

3:00 PM - 3:15 PM
3:20 PM - 3:25 PM

- Schindler Disease
- Recap Day 2 Sessions and Closing Remarks

3:30 PM - 4:30 PM

- Breakout Sessions for
combined Mucopolidosis & Sialidosis,
combined Galactosialidosis & Schindler Disease

4:30 PM - 5:15 PM

- POSTER SESSION

6:00 PM - 7:00 PM

- Pre-Dinner Networking

7:00 PM - 9:30 PM

- GALA DINNER