

AGENDA FOR 2024 AMDA/IPA INTERNATIONAL POMPE PATIENT AND SCIENTIFIC CONFERENCE

FRIDAY, MAY 3RD, 2024

Workshops	1:00-4:30
First Session	1:00-2:00
• Mental Health—	
• Caregivers Breakout Room—led by Julie Wells (Give an Hour)	
• Patient Breakout Room—led by Dr. van den Hout	
• Parent's Breakout Room—led by Julienne Williams	
Break	2:00-2:15
Second Session (Two Workshops)	2:15-3:15
• How to monitor LOPD patients diagnosed through NBS—Dr. Cocanougher, Amanda Joost, and Erin Huggins	
• Transitioning from Pediatric Care to Adult Care—Dr. van den Hout and Zach DuMond	
Break	3:15-3:30
Third Session (Two Workshops)	3:30 -4:30
• Exercise Recommendations for IOPD—Dr. Laura Case	
• Exercise Recommendations LOPD— Dr. van den Hout and Keyuna (Coach K) Milam	
End of Workshops	
Welcome Dinner (meet in the Lobby of the Holiday Inn for departure)	5:30-9:00

SATURDAY, MAY 4TH, 2024

Breakfast	7:00–8:00
Welcome Address	8:00-8:10
Understanding Pompe: What Do We Know, and What Is Left To Learn by Drs. Arnold Reuser and Nina Raben	8:10-8:40
Natural History of Pompe vs. New History with ERT	
Chair: Dr. Priya Kishnani	8:40-8:50
Infantile-Onset by Dr. Hannerieke van den Hout	8:50-9:10
Late-Onset by Dr. Mark Roberts	9:10-9:30
Effects of Newborn Screening by Dr. Nancy Chien	9:30-9:50
Question and Answer	9:50-10:05
Break	10:05-10:25
The Whole Patient—Monitoring of Patients	
Chair: Dr. Benedikt Schoser	10:25-10:30
Integrative Care of Pompe Patients by Dr. Andreas Hahn	10:30-11:00

Monitoring of Patients: Biomarkers by Dr. Giancarlo Parenti	11:00-11:15
Monitoring of Patients: Muscle MRI by Dr. Jordi Diaz-Manera	11:15-11:30
Monitoring of Patients: Muscle Ultrasound by Dr. Neha Regmi	11:30-11:45
Question and Answer	11:45-12:00

Lunch 12:00 -1:00

Management and Care of Pompe Patients

Chair: Dr. Ans van der Ploeg	1:00-1:05
Non-Invasive Ventilation for Pompe by Dr. Grazia Crescimanno	1:05-1:20
Respiratory Muscle training Project by Elena Compalati and Rosario Di Marco	1:20-1:35
Utilizing Nutritional Ketosis to Improve the Infantile Onset Pompe Disease Phenotype in C57/BL6 NJ Mice by Dr. Dominic D'Agostino	1:35-1:50
Diet and Exercise Interactions for the Treatment of Pompe Disease by Dr. Mark Tarnopolsky	1:50-2:05
Speech Pathology by Dr. Harrison Jones	2:05-2:20
Pain Management for Pompe by Dr. Heidi Peters	2:20-2:35
Question and Answer	2:35-2:50

Break 2:50-3:10

Expert Roundtable: 25 Years of Treatment and Care of Pompe Patients

Chair: Tiffany House (Dr. Priya Kishnani, Dr. Benedikt Schoser, Dr. Ans van der Ploeg, Dr. Mark Roberts)	
Open Discussion with Audience	3:10-4:15

Challenges of Managing Pompe-The Patient Perspective Roundtable

Chair: Dr. Ria Broekgaarden	
Panel: Fabio Di Pietro, Wilma Treur, Coburn Burroughs, Krystal Hayes, and Hayley Hayes	4:15-5:15

End of Day 1

Patient Reception 6:30-9:00

SUNDAY, MAY 5TH, 2024

Breakfast 7:00-8:00

Next Generation Therapies: Presentations from Industry 8:00-9:35

Chairs: Dr. Federico Mingozzi and Dr. Nina Raben	8:00-8:10
Amicus By Brian Fox	8:10-8:20
M6P By Russell Gotschall	8:20-8:30
What's Next for Pompe from Sanofi - Dr. Nadia Daba	8:30-8:40
Introduction to Gene Therapy	8:40-8:50
Astellas	8:50-9:00
LentiCure - Dr. Pim Pijnappel	9:00-9:10

Regeneron	9:10-9:20
Question and Answer	9:20-9:35
Break	9:35-9:55
IPA/Erasmus Survey and Pompe Registries	
Chair: Brad Crittenden	9:55-10:00
Report on IPA/Erasmus Pompe Survey/Mutation Database by Dr. Nadine van der Beek	10:00-10:15
Report from Genzyme's Pompe Registry by Dr. Tiziana Mongini	10:15-10:25
Report from French Registry by Dr. Pascal Laforet	10:25-10:35
Where Do We Go From Here and Why is it Important? By Dr. Benedikt Schoser	10:35-10:45
Question and Answer	10:45-11:00
Break	11:00-11:20
Future Research	
Chair: Dr. Ans van der Ploeg	11:20-11:30
How to Fast Track Preclinical Testing New Treatments for Pompe disease by Dr. Nina Raben	11:30-11:50
Substrate Reduction Therapy in Pompe by Dr. Virginia Kimonis	11:50-12:10
PS Gene-editing for Pompe disease by Dr. Chester Whitley	12:10-12:30
Stem Cell and Muscle Regeneration in Pompe by Dr. Pim Pijnappel	12:30-12:50
Question and Answer	12:50-1:00
CONFERENCE CLOSE	1:00-1:30