

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

FRIDAY, MAY 3RD, 2024

Workshops	1:00-4:30
First Session (Two Workshops)	1:00-2:00
<ul style="list-style-type: none"> How to monitor LOPD patients diagnosed through NBS—Dr. Cocanougher and Amanda Joost Transitioning from Pediatric Care to Adult Care—Dr. van den Hout and Zach DuMond 	
Break	2:00-2:15
Second Session	2:15-3:15
1) Mental Health—Breakout Rooms TBD based on enrollment	
Break	3:15-3:30
Third Session (Two Workshops)	3:30 -4:30
2) Exercise Recommendations for IOPD)—Dr. Laura Case	
3) Exercise Recommendations LOPD—Dr. Linda den Berg	
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:	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 TBD	11:30-11:45
Question and Answer	11:45-12:00

Lunch 12:00 -1:00

Management and Care of Pompe Patients (15 min each)

Chair: Dr. Ans van der Ploeg	1:00-1:05
Diet approach by Dr. Dominic D'Agostino	1:05-1:20
Exercise and Nutritional Considerations for the Treatment of Pompe Disease by Dr. Mark Tarnopolsky	1:20-1:35
NIV for Pompe by Dr. Grazia Crescimanno	1:35-1:50
Respiratory Muscle training Project by Compalati and Di Marco (Respiratory Therapists)	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: TBD	
Panel TBD	4:15-5:15

End of Day 1

Patient Reception/Speaker Dinner	6:30-9:00
----------------------------------	-----------

SUNDAY, MAY 5TH, 2024

Breakfast 7:00-8:00

IPA/Erasmus Survey and Pompe Registries

Chair: Brad Crittenden	8:00-8:05
Report on IPA/Erasmus Pompe Survey/Mutation Database by Dr. Nadine van der Beek	8:05-8:20
Report from Genzyme's Pompe Registry by Dr. Tiziana Mongini	8:20-8:30
Report from French Registry by Dr. Pascal Laforet	8:30-8:40
Where Do We Go From Here and Why is it Important? By Dr. Benedikt Schoser	8:40-8:50
Question and Answer	8:50-9:05

Break 9:05-9:25

Next Generation Therapies: ERT, Gene Therapy and Substrate Reduction (All Industry)

Chair: Dr. Federico Mingozzi—ERT	9:25-9:35
Amicus	9:35-9:45
M6P	10:55-11:05

Sanofi —Next Gen ERT	9:45-9:55
Chair Nina Raben—Gene Therapy	9:55-10:05
Astellas	10:05-10:15
LentiCure	10:15-10:25
Regeneron	10:25-10:35
Question and Answer	10:35-10:45
Chair Dr. Federico Mingozzi—Substrate Reduction Therapy	10:45-10:55
Maze-TBD	11:05-11:15
Question and Answer	11:15-11:24
Break	11:25-11:45
Future Research	
Chair: Dr. Ans van der Ploeg	11:45-11:50
Topic TBD by Dr. Nina Raben	11:50-12:10
TBD	12:10-12:20
TBD	12:20-12:40
Stem Cell and Muscle Regeneration in Pompe by Dr. Pim Pijnappel	12:40-1:00
Question and Answer	1:00-1:15
CONFERENCE CLOSE	1:15-1:30