

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

## FRIDAY, MAY 3<sup>RD</sup>, 2024

Workshops	1:00-4:30
<b>First Session</b>	<b>1:00-2:00</b>
• 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
<b>Second Session (Two Workshops)</b>	<b>2:15-3:15</b>
• 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	3:15-3:30
<b>Third Session (Two Workshops)</b>	<b>3:30 -4:30</b>
• Exercise Recommendations for IOPD—Dr. Laura Case	
• Exercise Recommendations LOPD—Dr. Linda den Berg	
<b>End of Workshops</b>	
Welcome Dinner (meet in the Lobby of the Holiday Inn for departure)	5:30-9:00

## SATURDAY, MAY 4<sup>TH</sup>, 2024

<b>Breakfast</b>	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
<b>Natural History of Pompe vs. New History with ERT</b>	
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
<b>Break</b>	10:05-10:25
<b>The Whole Patient—Monitoring of Patients</b>	
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
Diet approach by Dr. Dominic D'Agostino	1:35-1:50
Exercise and Nutritional Considerations 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: TBD	
Panel TBD	4:15-5:15

**End of Day 1**

**Patient Reception** 6:30-9:00

**SUNDAY, MAY 5<sup>TH</sup>, 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: Presentations from Industry**

Chairs: Dr. Federico Mingozzi and Dr. Nina Raben

Amicus

M6P

Sanofi

Introduction to Gene Therapy

Astellas

LentiCure

Regeneron

Question and Answer

**9:25-11:00**

9:25-9:35

9:35-9:45

9:45-9:55

9:55-10:05

10:05-10:15

10:15-10:25

10:25-10:35

10:35-10:45

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**