

# Conference Agenda

October 25, 2019 – October 27, 2019

San Antonio, Texas

## FRIDAY, OCTOBER 25<sup>TH</sup>, 2019

<b>Conference Registration</b>	1:00-5:30
Lobby of the Holiday Inn Riverwalk	
<b>Welcome Dinner</b>	6:00-9:00
(Buses depart from the Lobby of the Holiday Inn at 6 PM)	

## SATURDAY, OCTOBER 26<sup>TH</sup>, 2019

<b>Breakfast</b>	7:00-8:00
<b>Welcome Address</b>	8:00-8:10
<b>Crash Course in Pompe</b>	8:10-8:40
<i>Dr. Arnold Reuser (Erasmus University)</i>	
<b>Natural History vs. New History with Treatment</b>	
Chair: Dr. Pascal Laforêt	
<b>Natural History vs New History: Infantile-Onset</b>	8:40-9:00
<i>Dr. Priya Kishnani (Duke University)</i>	
<b>Natural History vs New History: Late-Onset</b>	9:00-9:20
<i>Dr. Ans van der Ploeg (Erasmus University)</i>	
<b>Newborn Screening: Taiwan Experience</b>	9:20-9:40
<i>Dr. Yin-Hsiu Chien (National Taiwan University Hospital)</i>	
<b>Question and Answer</b>	9:40-10:00
<b>Break</b>	10:00-10:20
<b>Integrative Care of Pompe Patients</b>	10:20-10:50
<i>Dr. Hannerieke van den Hout (Erasmus University)</i>	
<b>Question and Answer</b>	10:50-11:00
<b>The Basics of Clinical Trials</b>	
Chair: Dr. Priya Kishnani	
<b>Clinical Trial Relate Issues for Pompe Disease</b>	11:00-11:25
<i>Dr. Benedikt Schoser (Friedrich-Baur-Institute)</i>	
<b>Patient Perspective on Clinical Trials</b>	11:25-11:40
<i>Ryan Colburn (United States)</i>	
<b>Patient Organization View on Clinical Trials</b>	11:40-11:55
<i>Ria Broekgaarden (IPA, VSN)</i>	
<b>Question and Answer</b>	11:55-12:00
<b>Lunch</b>	12:00-1:00
<b>Management and Care of Pompe Patients</b>	
Chair: Dr. Ans van der Ploeg	
<b>Improved Muscle Function in a Phase I/II Clinical Trial of Albuterol in Pompe Disease</b>	1:00-1:20
<i>Dr. Dwight Koeberl (Duke University)</i>	
<b>Exercise and Nutritional Considerations for the Treatment of Pompe Disease</b>	1:20-1:40
<i>Dr. Mark Tamopolsky (McMaster University)</i>	
<b>Respiratory Muscle Training in Late-Onset Pompe Disease: Randomized Controlled Trial Results</b>	1:40-2:00
<i>Dr. Harrison Jones (Duke University)</i>	
<b>Noninvasive Respiratory Management of Pompe Disease</b>	2:00-2:20
<i>Dr. John Bach (Rutgers University)</i>	
<b>Question and Answer</b>	2:20-2:40
<b>Break</b>	2:40-3:00
<b>Expert Round Table: 20 Years of Treatment and Care</b>	
Chair: Tiffany House (IPA, AMDA)	
<i>Dr. Pascal Laforêt (Versailles-Saint Quentin University)</i>	3:00-3:10
<i>Dr. Priya Kishnani (Duke University)</i>	3:10-3:20
<i>Dr. Benedikt Schoser (Friedrich-Baur-Institute)</i>	3:20-3:30
<i>Dr. Ans van der Ploeg (Erasmus University)</i>	3:30-3:40
Open Discussion with Audience	3:40-4:30
<b>Break</b>	4:30-4:45

<b>IPA/Erasmus Survey and Pompe Registry</b>	
Chair: Thomas Schaller (IPA, Pompe Germany)	
<b>The IPA/Erasmus Pompe Survey: Past, Present, and Future</b>	4:45-5:00
<i>Dr. Nadine van der Beek (Erasmus University)</i>	
<b>Report from Genzyme's Pompe Registry</b>	5:00-5:15
<i>Dr. Virginia Kimonis (University of California-Irvine)</i>	
<b>Question and Answer</b>	5:15-5:30

<b>PATIENT MIXER</b>	<b>7:00-9:00</b>
Skyline Atrium of the Holiday Inn Riverwalk	

END OF DAY ONE

## SUNDAY, OCTOBER 27<sup>TH</sup>, 2019

<b>Breakfast</b>	7:00-8:00
<b>Next Generation Therapy: Enzyme Replacement Therapy (ERT)</b>	
Chair: Dr. Nina Raben (NIH)	
<b>How Enzyme Replacement Therapy Works</b>	8:00-8:10
<b>Update on Amicus Trials with ERT Associated with Chaperone Molecule</b>	8:10-8:25
<i>Dr. Pascal Laforêt (Versailles-Saint Quentin University)</i>	
<b>Update on Genzyme's Neo-GAA</b>	8:25-8:40
<i>Dr. Benedikt Schoser (Friedrich-Baur-Institute)</i>	
<b>Preclinical studies for Oral-ERT with a Tobacco Seed-Derived a Recombinant Acid Maltase</b>	8:40-8:55
<i>Dr. Frank Martiniuk (JME Group)</i>	
<b>Safety and Efficacy Data Using VAL-1221 in Patients with LOPD</b>	8:55-9:10
<i>Dr. Priya Kishnani (Duke University)</i>	
<b>Question and Answer</b>	9:10-9:30
<b>Break</b>	9:30-9:50
<b>Next Generation Therapy: Gene Therapy</b>	
Chair: Arnold Reuser (Erasmus University)	
<b>Basics of Gene Therapy</b>	9:50-10:00
<b>A Phase 1 Study of the Safety of AAV8-LSPHGA (ACTUS-101) in Late-Onset Pompe Disease (LOPD)</b>	10:00-10:15
<i>Dr. Dwight Koeberl (Duke University)</i>	
<b>Lentiviral Gene Therapy and Antisense Oligonucleotides for Pompe Disease</b>	10:15-10:30
<i>Dr. Pim Pijnappel (Erasmus University)</i>	
<b>AAV-Mediated Gene Therapy in Pompe Disease: Clinical Immunology Considerations</b>	10:30-10:45
<i>Dr. Barry Byrne (University of Florida)</i>	
<b>Gene Therapy for Pompe Disease: From Bench to Bedside</b>	10:45-11:00
<i>Dr. Giuseppe Ronzitti (Genethon-France)</i>	
<b>Question and Answer</b>	11:00-11:20
<b>Break</b>	11:20-11:40
<b>Future Research</b>	
Chair: Dr. Benedikt Schoser (Friedrich-Baur-Institute)	
<b>Skeletal Muscle Abnormalities in Pompe Disease Can Be Reversed by Enzyme Replacement Therapy</b>	11:40-12:00
<i>Dr. Nina Raben (NIH)</i>	
<b>The Common c.-32-13T&gt;G Splicing Variant of GAA Gene: From Functional Characterization to the Identification of New Therapeutic Targets for Pompe Disease</b>	12:00-12:20
<i>Dr. Andrea Dardis (Regional Coordinator Centre for Rare Diseases, Udine)</i>	
<b>Targeting Glycogen Synthase (GYS1) with Antisense Oligonucleotide Treatment in a Mouse Model of Pompe Disease</b>	12:20-12:40
<i>Dr. Virginia Kimonis (University of California-Irvine)</i>	
<b>Stem Cell and Muscle Regeneration in Pompe</b>	12:40-1:00
<i>Dr. Pim Pijnappel (Erasmus University)</i>	

CONFERENCE CLOSE