

AGENDA FOR 2011 AMDA/IPA POMPE PATIENT AND SCIENTIFIC CONFERENCE

FRIDAY, OCTOBER 7TH, 2011

Day at Sea World!

Welcome Dinner at Sea World

Time is TBD

Buses depart Holiday Inn at 5:30

SATURDAY, OCTOBER 8TH, 2011

Breakfast

7:00–8:00

Welcome Address

8:00-8:10

Introduction: What is Pompe? by Dr. Paul Plotz

8:10-8:25

History of Pompe by Dr. Kevin O'Donnell

8:25-8:50

Enzyme Replacement Therapy and the IPA/Erasmus Survey

12 Years ERT experience with Infants by Dr. Priya Kishnani

8:55-9:15

12 Years ERT experience with Late-Onset by Dr. Ans van der Ploeg

9:15-9:35

Report from the IPA/Erasmus Pompe Survey by Dr. Deniz Gungor

9:35-9:55

Question and Answer

9:55-10:15

Break

10:15-10:30

Industry Updates (presentations by Genzyme, BioMarin and Amicus)

10:35-11:35

Question and Answer

11:35-12:00

Lunch

12:00-1:00

Living with Pompe Panel

Respiratory Issues in Pompe Disease by Dr. John Bach

1:05-1:25

Swallowing, Dysphagia and Lingual Weakness in Pompe Disease by Dr. Harrison Jones

1:25-1:45

Report on Erasmus Exercise Trial by Dr. Linda van der Berg

1:45-2:05

Patient Perspective on Erasmus Exercise Trial by Wilma Treur

2:05-2:25

Question and Answer

2:25-2:55

Break

2:55-3:10

Newborn Screening Panel

Results from Newborn Screening Study by Ria Broekgaarden

3:15-3:35

Results from Taiwan Newborn Screening Study by Dr. Y.T. Chen

3:35-3:55

Challenges with Newborn Screening by Dr. Arnold Reuser

3:55-4:15

Question and Answer

4:15-4:45

SUNDAY, OCTOBER 9TH, 2011

Breakfast	7:30-8:30
Future Research	
Autophagy and Pompe by Dr. Nina Raben	8:35-8:55
The Role of Antibodies by Dr. Priya Kishnani	8:55-9:15
Muscle Regeneration and Pompe by Dr. Ans van der Ploeg	9:15-9:35
Question and Answer	9:35-9:55
Break	9:55-10:10
Gene Therapy	
Introduction to Gene Therapy	10:15-10:25
Dr. Andrea Amalfitano	10:25-10:40
Dr. Barry Byrne	10:40-10:55
Dr. Dwight Koeberl	10:55-11:10
Dr. Gerard Wagemaker	11:10-11:25
Question and Answer	11:25-11:55
Round Table Patient Discussion: Gaining Perspective (over lunch)	
Led by Maryze Schoneveld van der Linde	12:00-1:00

CONFERENCE CLOSE