

AGENDA FOR 2015 AMDA/IPA INTERNATIONAL POMPE PATIENT AND SCIENTIFIC CONFERENCE

FRIDAY, OCTOBER 30TH, 2015

6:00-9:00

Welcome Dinner (meet in the Lobby of the Holiday Inn)
Dinner Presentation

SATURDAY, OCTOBER 31ST, 2015

Breakfast

7:00-8:00

Welcome Address

8:00-8:15

The Natural History of Pompe Disease by Dr. Hannerieke van den Hout

8:15-8:45

Question and Answer

8:45-8:55

IPA/Erasmus Survey and Pompe Registry

Report from the IPA/Erasmus Pompe Survey by Dr. Chris van der Meijden

8:55-9:15

Report from Genzyme's Pompe Registry by Dr. Virginia Kimonis

9:15-9:35

Question and Answer

9:35-9:45

Break

9:45-10:00

Diagnosis, Management, and Treatment of Pompe

Diagnosis and Genetic Counseling by Dr. Deeksha Bali and Stephanie Austin

10:00-10:20

Patient Management and Current Standard of Care by Dr. Hannerieke van den Hout

10:20-10:40

Respiratory Issues in Pompe Disease by Dr. John Bach

10:40-11:00

Diaphragm Pacemaker Presentation by Dr. Barbara Smith

11:00-11:20

Exercise Presentation by Dr. Linda van den Berg

11:20-11:40

Question and Answer

11:40-12:00

Lunch

12:00-1:00

Patient Perspective

1:00-1:20

Question and Answer

1:20-1:30

Gene Therapy

Introduction to Gene Therapy by Dr. Arnold Reuser

1:30-1:45

Dr. Andrea Amalfitano

1:45-2:00

Dr. Barry Byrne

2:00-2:15

Dr. Dwight Koeberl

2:15-2:30

Dr. Pim Pijnappel

2:30-2:45

Question and Answer

2:45-3:00

Break

3:00-3:30

ERT: What Have We Learned So Far Roundtable

Introduction

3:30-3:40

Dr. Nancy Chien

3:40-4:00

Dr. Priya Kishnani	4:00-4:20
Dr. Benedikt Schoser	4:20-4:40
Dr. Ans van der Ploeg	4:40-5:00
Open Discussion with Audience	5:00-6:00

SUNDAY, NOVEMBER 1ST, 2015

Breakfast	7:30-8:30
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Future Research

Pathogenesis of muscle damage in Pompe disease and new therapeutic approaches by Dr. Nina Raben	8:35-8:55
Stem Cell Presentation by Dr. Pim Pijnappel	8:55-9:15
Genotype/Phenotype Correlation by Dr. Arnold Reuser	9:15-9:35
Modifying Factors by Dr. Cesare Danesino	9:35-9:55
Biomarkers in Pompe Disease by Dr. Giancarlo Parenti	9:55-10:15
Question and Answer	10:15-10:45

Break	10:45-11:15
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Industry Updates

Question and Answer	11:15-12:15
	12:15-12:45

CONFERENCE CLOSE