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

FRIDAY, OCTOBER 30TH, 2015
Welcome Dinner (meet in the Lobby of the Holiday Inn)
Dinner Presentation

SATURDAY, OCTOBER 31ST, 2015
Breakfast
Welcome Address
The Natural History of Pompe Disease by Dr. Hannerieke van den Hout
Question and Answer

IPA/Erasmus Survey and Pompe Registry
Report from the IPA/Erasmus Pompe Survey by Dr. Chris van der Meijden
Report from Genzyme’s Pompe Registry by Dr. Virginia Kimonis
Question and Answer

Break

Diagnosis, Management, and Treatment of Pompe
Diagnosis and Genetic Counseling by Dr. Deeksha Bali and Stephanie Austin
Patient Management and Current Standard of Care by Dr. Hannerieke van den Hout
Respiratory Issues in Pompe Disease by Dr. John Bach
Diaphragm Pacemaker Presentation by Dr. Barbara Smith
Exercise Presentation by Dr. Linda van den Berg
Question and Answer

Lunch

Patient Perspective
Question and Answer

Gene Therapy
Introduction to Gene Therapy by Dr. Arnold Reuser
Dr. Andrea Amalfitano
Dr. Barry Byrne
Dr. Dwight Koeberl
Dr. Pim Pijnappel
Question and Answer

Break

ERT: What Have We Learned So Far Roundtable
Introduction
Dr. Nancy Chien
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

**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

**Industry Updates**
11:15-12:15
Question and Answer 12:15-12:45

**CONFERENCE CLOSE**