Dear Colleagues,

On behalf of the Scientific Committee, we are pleased to announce that this year's ***Steps Forward in Pompe Disease*** meeting will be held in Schiphol, the Netherlands, 11–12 November 2016.

Pompe disease is a progressive multi-systemic and disabling disorder that poses many clinical challenges on a daily basis. However, our understanding of Pompe disease continues to evolve with advances in the diagnosis, pathophysiology and therapeutic approaches of the disease, ultimately leading to improvements in patient care and the clinical management of the disease.

The Steps Forward in Pompe Disease meeting aims to be the main bi-annual meeting and platform for the presentation and discussion of newly published and evolving scientific insights. By bringing together the clinical and scientific community working on and interested in the treatment of patients with Pompe disease, we hope to stimulate and facilitate networking as well as discuss new opportunities that will hopefully lead to further advancement of diagnosis and clinical management of these patients.

This year's scientific programme will focus on the theme of ***10 years' Enzyme Replacement Therapy: achievements, limitations and future prospects.*** Within the programme we will explore infantile as well as late-onset Pompe disease, early recognition and new diagnostic approaches, as well as an overview of the experiences with alglucosidase alfa treatment. Key topics to be discussed include the latest developments in imaging, immunological factors and new advances in other emerging aspects of the disease including, novel enzyme replacement therapeutic approaches and gene therapy.

Our intent is to create an interactive meeting through a comprehensive programme, which we hope will provide a forum for questions, discussion, experience sharing and lively scientific debates.

We very much look forward to welcoming you to the Netherlands on 11–12 November for this highly interactive scientific event and look forward to meeting you in person at the meeting.

Prof. Ans van der Ploeg
Chair

Prof. Benedikt Schoser
Chair

Scientific Committee:

Professor Ans van der Ploeg
Erasmus Medical Center-Sophia Children's Hospital, Rotterdam, The Netherlands

Professor Benedikt Schoser
Friedrich-Baur Institute, Ludwig-Maximilians University, Munich, Germany

Dr Pascal Laforêt
Institute of Myology, Pitié-Salpêtrière Hospital, Paris, France

Professor Giancarlo Parenti
Telethon Institute of Genetics and Medicine, Napoli, Italy

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Steps Forward in Pompe Disease

10 years' Enzyme Replacement Therapy: achievements, limitations and future prospects

PERSONAL INVITATION

Programme and General Information

Steigenberger Hotel, Schiphol, The Netherlands

Friday 11 and Saturday 12 November, 2016

Abstract deadline: 1 October 2016
SFPD2016@lucid-uk.com

Sponsored by Sanofi Genzyme

Day 1 - Friday, 11 November 2016

12:00 - 13:00	**Welcome Lunch**	
13:00 - 13:15	Welcome to Steps Forward in Pompe Disease 2016	Ans van der Ploeg *(Erasmus Medical Center-Sophia Children's Hospital, Rotterdam, The Netherlands)* & Benedikt Schoser *(Friedrich-Baur Institute, Ludwig-Maximilians University, Munich, Germany)*
Session 1	**Natural and modified course of Pompe disease**	**Chairs: Pascal Laforêt & Giancarlo Parenti**
13:15 - 13:30	Effects of ERT in infantile Pompe disease and the new transition phenotype	Andreas Hahn *(University of Giessen, Giessen, Germany)*
13:30 - 13:45	The CNS in infantile Pompe disease	Hannerieke van den Hout *(Erasmus Medical Center, Rotterdam, The Netherlands)*
13:45 - 13:55	Israel and Gaza's long-term (13 years) experience of the treatment of Infantile Pompe disease	Hanna Mandel *(Rambam Health Care Center, Rappaport School of Medicine, Technion, Haifa, Israel)*
13:55 - 14:10	Effects of ERT in children with non-classic phenotypes	Chris van der Meijden *(Erasmus Medical Center, Rotterdam, The Netherlands)*
14:10 - 14:25	Natural course and effects of ERT in adults • Guidelines when to start/stop ERT	Pascal Laforêt *(Institute of Myology, Pitié-Salpêtrière Hospital, Paris, France)*
14:25 - 14:40	Results of 10 years of the Pompe registry (sponsored by Sanofi Genzyme)	Eugen Mengel *(Johannes Gutenberg University Mainz, Children's Hospital of the University of Mainz, Mainz, Germany)*
14:40 - 14:55	European Pompe Consortium (EPOC)	Benedikt Schoser
14:45 - 15:00	Panel discussion with audience Q&A	
15:05 - 15:30	**Break**	
Session 2	**Pathophysiology**	**Chairs: Nina Raben & Pim Pijnappel**
15:30 - 15:50	Role of autophagy - transcription factor EB (TEFB)	Nina Raben *(National Institutes of Health, Bethesda, MD, USA)*
15:50 - 16:10	Role of muscle regeneration and repair	Pim Pijnappel *(Erasmus MC University Medical Center, Rotterdam, The Netherlands)*
16:10 - 16:30	Genetic and modifying factors	Giancarlo Parenti *(Telethon Institute of Genetics and Medicine, Napoli, Italy)*
16:30 - 16:45	Audience Q&A	
16:45 - 17:05	Immunomodulation in infantile Pompe disease	Priya Kishnani *(Duke University School of Medicine, Durham, North Carolina, United States)*
17:05 - 17:25	Exploring the pros and cons of immunomodulation in adult Pompe disease	Pascal Laforêt
17:25 - 17:40	Panel discussion: The role of immune tolerance and modulation in Pompe disease	
17:40 - 17:50	Close Day 1	Ans van der Ploeg & Benedikt Schoser
19:00	**Depart for Dinner**	

Day 2 - Saturday, 12 November 2016

09:00–09:05	Welcome to Day 2	Ans van der Ploeg & Benedikt Schoser
Session 3	**Novel diagnostic approaches**	**Chairs: Antonio Toscano & Pascal Laforêt**
09:05–09:20	MRI as a diagnostic tool in neuromuscular disorders	Robert-Yves Carlier *(Hôpital Raymond Poincaré, Garches, France)*
09:20–09:30	MRI of the cerebral vessels and brain	Antonio Toscano *(University of Messina, Messina, Italy)*
09:30–09:40	MRI of the diaphragm	Nadine van der Beek *(Erasmus Medical Center, Rotterdam, The Netherlands)*
09:40–09:55	Assessment of high-risk patients in respiratory clinics	Michael Polkey *(Royal Brompton Hospital & National Heart & Lung Institute, London, UK)*
09:55–10:10	Blood-based testing and whole genome screening • What is the current state in neuromuscular disorders?	Volker Straub *(Institute of Human Genetics, University of Newcastle upon Tyne, Newcastle Upon Tyne, UK)*
10:10–10:30	Audience Q&A	
10:30–11:00	**Break**	
Session 4	**10 years' of alglucosidase alfa: achievements, limitations and future prospects**	**Chairs: Ans van der Ploeg & Benedikt Schoser**
11:00–11:05	Introduction	
11:05–11:25	A company perspective	Henk Schuring *(Vice President – Head Rare Neurological diseases, Sanofi Genzyme)*
11:25–11:55	A doctor's perspective	Ans van der Ploeg
11:55–12:15	A patient´s perspective	Ria Broekgaarden *(Dutch Pompe patient organization VSN [Vereniging Spierziekten Nederland])*
12:15–12:40	Panel discussion and audience Q&A: How has ERT changed Pompe care?	*Moderated by* Ans van der Ploeg & Benedikt Schoser
12:40–13:25	**Lunch**	
13:25–14:25	**Poster session**	
Session 5	**Next therapeutic approaches**	**Chairs: Giancarlo Parenti & Nina Raben**
14:25–14:45	Results of NeoGAA and other ERT therapies under development	Mark Roberts *(Salford Royal NHS Foundation Trust, Salford, UK)*
14:45–15:05	Exosomes – a new way to transport large molecules?	Mark Tarnopolsky *(McMaster University, Hamilton, Ontario, Canada)*
15:05–15:20	Gene therapy – AAV/lentiviral	Federico Mingozzi *(University Pierre and Marie Curie, Paris, France)*
15:20–15:35	Stem cells what is possible today?	Francesco Saverio Tedesco *(University College London, London, UK)*
15:35–15:50	Audience Q&A: Future perspectives in Pompe disease	
15:50–16:00	Close of meeting	Ans van der Ploeg & Benedikt Schoser