

3rd INTERNATIONAL CONFERENCE

**SANFILIPPO SYNDROME AND RELATED
DISEASES**

VIDEO-CONFERENCE

NOVEMBER 12 & 13, 2020



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PROVISIONAL SCIENTIFIC PROGRAM

Thursday 12 November 2020

Morning

➤ **Session 1: Clinical studies in MPS III: gene therapy**

- 1) **Prof. Rob Wynn, Blood and Marrow Transplant Unit, Royal Manchester Children's Hospital and University of Manchester, UK (CONFIRMED)**
Keynote presentation - Report of the first ever lentiviral vector, gene-modified stem cell transplant in a patient with MPS IIIA
- 2) **Dr. Simon Jones, Willink Unit, Manchester Centre for Genomic Medicine, St Mary Hospital, Manchester Academic Health Sciences Centre, University of Manchester, UK (CONFIRMED)**
Clinical trial design for treatment of Mucopolysaccharidosis Type III
- 3) **Mrs. Karen Pignet-Aiach, Lysogene, Paris, France**

➤ **Session 2: Clinical studies in MPS III: ERT, SRT and small molecules**

- 1) **Prof. Frits Wijburg, Dept. of Pediatrics, Academic Medical Centre, Amsterdam, The Netherlands**
Keynote presentation - Lessons from clinical studies of genistein and heparin-N-sulphatase
- 2) **Mr. Stephen Maricich, Allievex Corporation, USA (CONFIRMED)**
Study 250-202 of tralesenidase alfa in MPS IIIB
- 3) **Seelos Therapeutics, USA (CONFIRMED)**
Trehalose (SLS-005) a small molecule for MPS III, OPMD and SCA3

Afternoon

➤ **Session 3: Other MPS diseases**

- 1) **Dr. Bettina Cockroft, Sangamo USA (CONFIRMED)**
The ST-920 program for Fabry disease
- 2) **Inventiva Pharma, Daix, France (CONFIRMED)**
iMProveS: a Phase IIa clinical trial of odiparcil for the treatment of adults with MPS VI
- 3) **Roche/Spark Therapeutics (CONFIRMED)**
SPK-3006, an investigational gene therapy for the treatment of Pompe disease (possibly SPK-1001, an investigational gene therapy for CNL2 Batten disease)

➤ **Session 4: Early stage and investigational treatments**

- 1) **Mrs. Anastasia Henry, Denali Therapeutics USA (CONFIRMED)**
Intravenously-administered lysosomal enzyme using a blood-brain barrier transport vehicle
- 2) **Mrs. Maria Francisca Coutinho, Portugal (CONFIRMED)**
Targeting stable nucleic acid lipid particle (SNALP)-formulated siRNAs to the brain as a therapeutic approach in lysosomal storage disease-associated neuropathy
- 3) **Dr. Brian Bigger, Faculty of Medical and Human Sciences, University of Manchester, UK (CONFIRMED)**
Innate immunity in MPS - or - Impact of the immune system on the safety and efficiency of ERT in LSDs

Friday 13 November 2020

Morning

➤ **Session 5: Screening and diagnosis**

- 1) **Dr. Zoltan Lukacs, Institute of Clinical Chemistry/Dept. of Pediatrics, University Medical Center Hamburg-Eppendorf, Germany (CONFIRMED)**
Targeted screening for lysosomal storage diseases
- 2) **Prof. Lucy Raymond, Cambridge (CONFIRMED)**
The next generation of children project: Whole genome sequencing for rapid diagnosis of severely ill children in intensive care
- 3) **Mr. Francisco J. del Castillo, Spain (CONFIRMED)**
Early detection of lysosomal diseases by screening of cases of idiopathic splenomegaly and/or thrombocytopenia with a next-generation sequencing gene panel
- 4) **Mrs. Ana Carolina Brusius-Facchin, Brasil**
Sensitivity, advantages, limitations and clinical utility of targeted next-generation sequencing panels for the diagnosis of selected lysosomal storage disorders

➤ **Session 6: Families**

Round table Q & A session with scientific experts (45mn)

Chair: **Dr. Armand Bottani, Valais Hospital, Central Hospital Institute, Medical Genetics Services, Sion, Switzerland (CONFIRMED)**

ORGANIZATION OF THE CONFERENCE

The Organizing Committee

- Dr. Danielle Bertola-Reymond, Member of the Sanfilippo Foundation Switzerland Scientific Committee
- Dr. Armand Bottani, Chief Physician, Department of Medical Genetics, Valais Hospital, Sion, Switzerland; Member of the the Sanfilippo Foundation Switzerland Scientific Committee
- Ms Isabelle Costes, Directrice of the Sanfilippo Foundation Switzerland
- Ms Corinne Féry-von Arx, Member of the Sanfilippo Foundation Switzerland Board
- Dr. Ron Hogg, Scientific Consultant, Medical Editor, Omniscience SA, Geneva, switzerland
- Mr Frédéric Morel, President of the Sanfilipo Foundation Switzerland

The Scientific Committee

- Dr. Danielle Bertola-Reymond
- Dr. Armand Bottani
- Dr. Susan Hill
- Dr. Nicolas Lantz

Our Partners



PROVISIONNAL BUDGET

180-200 participants

Video-conference

Virtual conference organisation package 30'000.-

Technical equipment

3 conference translators 4'200.-

Rental of equipment for simultaneous translations 10'000.-

Communication & graphics design

Online registration (website), flyers, booklet 15'000.-

Media relations

Contacts with the regional and international press,
Press releases 12'000.-

Indirects costs

Scientific consultant, coordinator, miscellaneous 12'000.-

Total **CHF 83'200.-**

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FINANCING PLAN

1) Solicited Foundations

Fondation Adrian et Simone Frutiger
Fondation Aga Khan
Fondation Allegra
Fondation Anita Chevalley
Fondation Beausobre et Jayet
Fondation Cyril de Coulon
Gebert Rűf Stiftung
Fondation Hans Wilsdorf
Fondation privée des HUG
Fondation Roger de Spoelberch
StopSanfilippo Fundacion
Fondation Hubert Tuor
Sanfilippo Children's Foundation

2) Solicited Companies

Allievex Corporation
Denali Therapeutics
Inventiva
Lysogene
Orchard Therapeutics
Roche / Spark Therapeutics
Sangamo
Seelos Therapeutics

SPONSORSHIP PROPOSALS

Main Sponsor CHF 15'000.-

- Opportunity to speak at the opening of the congress;
- Official sponsor of the conference;
- Page(s) of advertising either a cover or a double page in the program;
- Conference Registration for delegates;
- Your company logo (large format) projected on the plasma screens during the event;
- Company mentioned as one of the official sponsors in all information brochures;
- Inclusion in press and radio announcements;
- Links from the conference website to the sponsor's website;
- Name and logo mentioned in the Foundation's annual report.

Executive Sponsor CHF 7'500.-

- Possibility to be a moderator during a session;
- Advertising page in the program;
- Conference registration for delegates;
- Your company logo (large format) projected on the plasma screens during the event;
- Inclusion in press and radio announcements;
- Links from the conference website to the sponsor's website;
- Name and logo mentioned in the Foundation's annual report.

Associate Sponsor CHF 2'500.-

- Half-page ad in the program;
- Conference registration for delegates;
- Your company logo (medium format) projected on the plasma screens during the event;
- Inclusion in press and radio announcements;
- Links from the conference website to the sponsor's website;
- Name and logo mentioned in the Foundation's annual report.

CONFERENCE 2015, SOME DATA

The 2 day conference opened with **2 welcoming speeches** and a **plenary lecture**.

3 keynotes : Prof. John Hopwood, Prof. Marc Tardieu, Prof. Hans Aerts.

17 orales presentations and **4 posters** explored the following issues:

- 1) LSD screening and diagnosis
- 2) Treatment options
- 3) Specific clinical aspects of MPS III and new treatment approaches
- 4) Clinical and preclinical development in MPS and related LSD
- 5) Regulations

The families session consisted of **3 presentations** on:

- 1) Genetic counseling and prenatal diagnosis
- 2) Main highlights of the scientific conference
- 3) Round table and Q&A

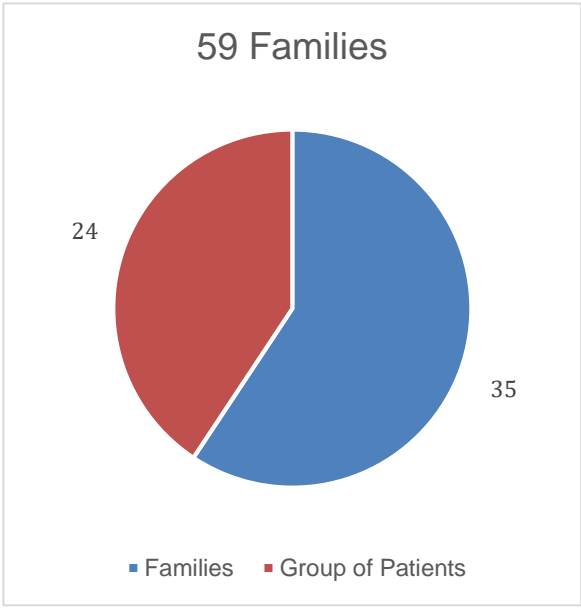
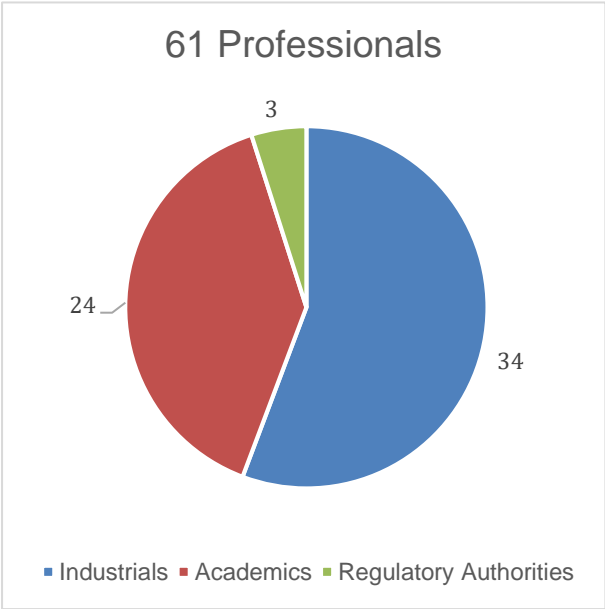
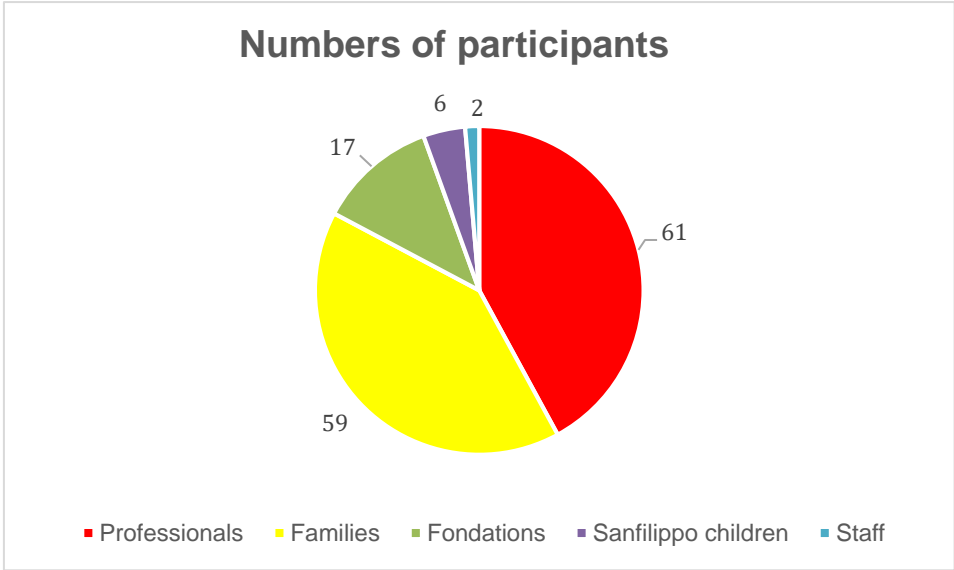
In the frame of the conference, a program of activities for children with Sanfilippo syndrome was organised by a team of educators from the Foundation Cap Loisirs.

8 attended companies: Abeona Therapeutics (USA), Amicus Therapeutics (USA), Armagen (USA), Dorphan SA (Switzerland), Genzyme (USA), Lysogene (France), Minoryx Therapeutics (Spain), Waypoint Capital (Switzerland).

6 attended universities: South Paris University (France), Manchester University (UK), Hambourg-Eppendorf University (German), Manchester University (UK), Leiden University (Pays-Bas), Geneva University (Switzerland).

145 participants from 19 countries:

Saudi Arabia	United States	Kazakhstan	Russia
Germany	France	The Netherlands	Sweden
Australia	Greece	Poland	Switzerland
Bosnia	Ireland	Portugal	Turkey
Spain	Italy	Great Britain	



CONTACTS

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