



Niemann-Pick UK
8th Interactive Workshop on Niemann-Pick Diseases
22nd September 2017

Venue: The Mercure Haydock Hotel, Penny Lane, Haydock, WA11 9SG, UK

9.00-9.30 Registration and Refreshments

9.30-9.40

Welcome and Introduction

Professor Frances Platt

Department of Pharmacology, University of Oxford, UK

9.40-11.00

**Interactive Workshop 1:
Newborn Screening and
Preventative Strategies**

Chair: Professor Frances Platt

Department of Pharmacology, University
of Oxford, UK

9.40-9.50

Overview

Professor Marc C. Patterson M.D.,
FRACP Professor of Neurology,
Pediatrics and Medical Genetics
Mayo Clinic, Minnesota, USA

9.50-10.30

Contributors:

Dr. Daniel S. Ory MD
Washington University School of
Medicine, St Louis, Missouri, USA

Pat Roberts
Executive Director, Save Babies UK and
Chair, UK Patient Advocates for Newborn
Screening Group (UK PANS)

Professor Peter Clayton
UCL Institute of Child Health
University College Hospital Medical
School, London

William Owen
Trustee and Research Coordinator,
Niemann-Pick UK

10.30-11.00

Discussion and Questions

11.00-11.30

Refreshment Break

11.30-13.15 Research Updates Chair: Prof David Begley Institute of Pharmaceutical Science, King's College London		
11.35-11.45	smpd1-/- loss of function zebrafish synergistically enhances phenotypes of gba1-/- deficiency	Dr. Marcus Keatinge University of Sheffield
11.45-11.50	Discussion and Questions	
11.50-12.00	The role of calcium signalling in the pathogenesis and treatment of Niemann-Pick disease	Dr. Emyr Lloyd-Evans School of Biosciences, Cardiff University, UK
12.00-12.05	Discussion and Questions	
12.05-12.15	New methods for monitoring disease progression and stratifying patients for clinical trials	Professor Frances Platt Department of Pharmacology, University of Oxford, UK
12.15-12.20	Discussion and Questions	
12.20-12.30	Biomarkers for NP-C	Dr. Daniel S. Ory MD Washington University School of Medicine St Louis, Missouri, USA
12.30-12.35	Discussion and Questions	
12.35-12.45	Fingolimod Prodrugs: A New Treatment For NPC?	Dr. Fabrizio Pertusati Research Fellow School of Pharmacy and Pharmaceutical Sciences, Cardiff University, UK
12.45-12.50	Discussion and Questions	
12.50-13.10	Miglustat: effectiveness in NP-C	Alison Reid International Medical Scientific Manager Actelion Pharmaceuticals UK Ltd
13.10-13.15	Discussion and Questions	

13.15-14.15

Lunch – Beechers Restaurant

14.15-16.30

Clinical Updates

Chair: Dr William Evans
Chair, NPUK

14.20-14.30

Development of health-related quality of life scales for patients affected by Niemann-Pick Disease Type C

Dr Rebecca Knibb CPsychol AFBPsS
Senior Lecturer and Programme Director MSc Health Psychology, Aston University, Birmingham, UK

14.30-14.35

Discussion and Questions

14.35-14.45

NPUK CNS role and the UK NP patient cohort

Laura Bell
NPUK Clinical Nurse Specialist
Salford Royal Foundation NHS Trust

14.45-14.50

Discussion and Questions

14.50-15.00

ASCEND: A phase 2/3 trial of olipudase alfa in Adults with ASMD

Dr. Tarek Hiwot
Consultant in Inherited Metabolic Disorders
University Hospitals Birmingham NHS Foundation Trust

15.00-15.10

Clinical Development of Olipudase Alfa for ASMD NP-B (Paediatric)

Dr. Simon Jones
Consultant in Paediatric Inherited Metabolic Disease, St Mary's Hospital, Manchester, UK

15.10-15.15

Discussion and Questions

15.15-15.30

CTD's Phase I and Phase I/II Intravenous Trials of Trappsol (R) Cyclo (TM) for NP-C

Dr. Sharon H. Hrynkow
Senior Vice President for Medical Affairs
Co-Chair, Scientific Advisory Board
CTD Holdings, Inc.

15.30-15.35

Discussion and Questions

15.35-15.50

AIDNPC: Development of Arimoclomol for NP-C

Christine i Dali MD
VP Clinical Development
Orphazyme Aps

15.50-15.55

Discussion and Questions

15.55-16.15 Refreshment Break

16.15-16.30

Clinical Development of VTS-270 for NP-C

Dr. Daniel S. Ory MD
Washington University School of Medicine
St Louis, Missouri, USA

16.30-16.35

Discussion and Questions

16.35-16.45

Intrabio

Dr. Michiko Mann,
Senior Scientific Researcher
IntraBio Inc.

16.45-17.30

Interactive Workshop 2
Improving diagnosis and therapeutic efficacy

Chair: Dr. Uma Ramaswami
Consultant in Inherited Metabolic Disorders, Royal Free Hospital, London

16.50-17.05

INPDR

James Green
President,
International Niemann-Pick Disease Alliance

17.05-17.20

Clinical Guidelines ASMD and NP-C

Marc C. Patterson
FRACP Professor of Neurology,
Pediatrics and Medical Genetics
Mayo Clinic, Minnesota, USA

17.20-17.30

Discussion and Questions

17.30

Workshop CLOSE - Drinks Reception

NPUK is a charitable organisation dedicated to making a positive difference to the lives of those affected by Niemann-Pick diseases. We raise much needed awareness, provide practical and emotional support, advice and information and facilitate research into potential therapies.

Niemann-Pick UK

Registered Charity Number: 1144406 | Registered as a Charity in Scotland No. SCO45407

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Website npuk.org

With grateful thanks to our Sponsors and Grant Funders:

We rely entirely on voluntary donations, charitable grants and fundraising to support our work with those affected by Niemann-Pick diseases, their families and the health professionals caring for them. We are hugely grateful to all who have provided support for this event:



Actelion Pharmaceuticals has financially sponsored this event with no input into the content of the agenda or selection of speakers.



CTD Holdings, Inc. provided financial support for this event via an unrestricted educational grant without control or influence of the content of the programme or the selection of speakers.



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Niemann-Pick UK aims to ensure the patients and families we support are equipped and empowered with up-to-date and accurate information about Niemann-Pick disease, including the latest research and clinical trial information.

Our annual events provide an ideal opportunity to be able to do this, as many of our families attend, alongside our healthcare professional colleagues and pharmaceutical company representatives.

As research advances and an increasing number of clinical trials are initiated, it is even more critical that the information we provide to our families at our conference and through all our other communication is up-to-date, accurate and balanced.

Therefore, as we facilitate this exchange of scientific information, we would like to remind attending pharmaceutical companies that all communications and interactions at our conference must abide by the Association of the British Pharmaceutical Industry (ABPI) Code of Practice available here: <http://www.pmcpa.org.uk/thecode/InteractiveCode2016/Pages/default.aspx>

This includes ensuring all information provided is balanced, fair, up-to-date, accurate, not misleading in any way and capable of substantiation.

We aim to be as transparent and as fair as possible with our families, healthcare professional colleagues and pharmaceutical company representatives and we ask for the same conduct in return.

Making a difference to the lives of those affected by Niemann-Pick

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