



**International Niemann-Pick Disease Registry  
Annual Forum**

**Thursday 6<sup>th</sup> October 2022**

Venue: The Woodlands Event Centre, Wyboston Lakes,  
Great North Road,  
Wyboston, Bedfordshire, MK44 3ALUK

13.00 - 13.45	<b>Lunch:</b> The Olive Restaurant
13.45 – 17.30	<b>INPDR Forum:</b> Ash 1 & 2
13.45	<b>Welcome and Introduction</b> Jim Green, INPDR Board Chair
14.00	<b>Setting the Scene</b> Conan Donnelly, CEO, INPDR
14.15	<b>INPDR Progress Report</b> Shaun Bolton, Operations Consultant, INPDR
14.30	<b>Growing the INPDR</b> Jackie Imrie, Clinical Research Manager, INPDR
14.45	<b>Capturing and making sense of patient reported data</b> Conan Donnelly, CEO, INPDR
15.00	<b>Linking and Curating NPD Data</b> David Storey, DPO, INPDR
15.15	<b>Making use of data - commercial studies, challenges and opportunities</b> Shaun Bolton, Operations Consultant, INPDR
15.30	<b>Refreshment Break:</b> Paddy and Scott's Coffee House
15.45 - 16.45	<b>Breakout Sessions</b>
16.45	<b>Feedback and listening session</b>
17.00	<b>Discussion and round up</b>
17.30	<b>Networking Reception:</b> Olive 2
19.00	<b>Dinner:</b> Olive 2



**Niemann-Pick UK**  
**11<sup>th</sup> Interactive Workshop on Niemann-Pick Diseases**  
Friday 7<sup>th</sup> October 2022

Venue: The Woodlands Event Centre, Wyboston Lakes, Great North Road,  
Wyboston, Bedfordshire, MK44 3ALUK

**09.00-09.30 Registration and Refreshments**

**09.30-09.35**

**Welcome and Introduction**

**Professor Frances Platt**

Department of Pharmacology, University of Oxford, UK

**9.35-10.50**

**Research Updates**

**09.35-9.45**

**Dysregulated Inflammation in NPC**

Frances Platt, PhD, FMedSci, FRS

Head of Department of Pharmacology, University of Oxford

**09.45-09.55**

**NPC1-PMCA2 complex in Niemann-Pick disease type C**

Maria E. Fernandez-Suarez, PhD

Postdoctoral Researcher

Department of Pharmacology, University of Oxford

**09.55 -10.10**

**NPC1 and lysosome interactions**

Dr Andrea Martello

Research Associate, Institute of Ophthalmology, Faculty of Brain Sciences,

University College London

**10.10 -10.20**

**Role of NPC1 protein in the persistence of intracellular pathogens**

Yuzhe Weng

DPhil Student

Department of Pharmacology, University of Oxford

**10.20-10.35**

**In vitro attempts to treat the mitochondrial defect in NPCD**

Dr Simon Wheeler

Lecturer in Pharmaceutical Chemistry, De Montfort University, Leicester

**10.35-10.50**

**Discussion**

**10.50-11.15**

**Refreshment Break**

11.15-12.30 <b>Interactive Workshop 1: New Developments in Niemann-Pick diseases</b>	
11.15-11.35	<b>Developing an optimized gene therapy strategy for Niemann-Pick Disease Type C1</b> Hemanth Ramesh Nelvagal MBBS, PhD Postdoctoral Research Associate, Rahim Group, University College London
11.35-12.00	<b>Novel Therapeutic Strategies for Niemann Pick Type C Disease</b> Kevin T. Vaughan Associate Professor of Cell Biology, University of Notre Dame, Indiana, USA
12.00-12.20	<b>Niemann–Pick type C disease as proof-of-concept for intelligent biomarker panel selection in neurometabolic disorders</b> Apostolos Papandreou Molecular Neurosciences, Developmental Neurosciences Programme, Great Ormond Street Institute of Child Health, University College London
12.20-12.30	<b>Discussion</b>

12.30-13.30	<b>Lunch</b>
-------------	--------------

13.30-14.20 <b>Interactive Workshop 2: Drug Repurposing in Rare Diseases</b>	
13.30-13.55	<b>Drug repurposing in rare diseases; the benefits and opportunities</b> Rick Thompson CEO, Rare Beacon
13.55-14.10	<b>Drug repurposing: a company perspective</b> Taylor Fields Chief Product Development Officer, IntraBio Ltd.
14.10-14.20	<b>Discussion</b>

14.20-15.20 <b>Interactive Workshop 3: Newborn and Future Screening</b>	
14.20-14.30	<b>Introduction</b> William Owen Trustee and Research Coordinator, NPUK
14.30-14.40	<b>A Rare Life Lived Better; Newborn Screening</b> Bob Stevens Group CEO, MPS Society & Rare Disease Research Partners Vice Chair, LSD Collaborative
14.40-15.20	<b>Panel Session:</b> The benefits, challenges, and practicalities of newborn and future screening for Niemann-Pick and other rare genetic conditions. <ul style="list-style-type: none"> <li>• Dr Robin Lachmann, PhD, FRCP, Consultant in Metabolic Medicine, National Hospital for Neurology, London</li> <li>• Prof. Paul Gissen, Consultant in Paediatric Metabolic Diseases, Great Ormond Street; Wellcome Trust Sr. Research Fellow in Clinical Sciences, UCL</li> <li>• Karen Harrison, Support Services Manager, AlexTLC</li> </ul>

<b>15.20-15.35</b>	<b>Refreshment Break</b>
--------------------	--------------------------

<b>15.35-16.30</b>	<b>Interactive Workshop 4: Predicting the Clinical Path and Severity of NPC</b>
--------------------	-------------------------------------------------------------------------------------

<b>15.35-15.50</b>	<b>Challenges and Opportunities with the NPC Clinical Severity Scale (NPCCSS)</b> Professor Marc C. Patterson, MD Professor of Neurology, Pediatrics and Medical Genetics Mayo Clinic Children's Center, Rochester, MN, USA
<b>15.50-16.05</b>	<b>Natural History and Progression of NPC; Progression rates in NPC vary between individual patients due to the heterogeneity of the disease; What factors appear to influence annual progression rates?</b> Christine í Dali, MD Chief Medical Officer, KemPharm
<b>16.05–16.30</b>	<b>International Niemann-Pick Disease Registry (INPDR): Supporting Research</b>  <b>Taking the next steps – supporting research</b> Conan Donnelly, Chief Executive Officer, INPDR  <b>Niemann-Pick Type C – The patient reported experience, an NPUK led survey</b> Solomon Mbua, CRA Consultant, INPDR  <b>Clinical disease characteristics of patients with NPC: findings from the INPDR</b> Shaun Bolton, BSc, MSc, Operations Consultant, INPDR  <b>Quality of life in patients with Niemann-Pick disease and their carers</b> Jackson Pountney, Psychology PhD student, Aston University

<b>16.30 - 17.35</b>	<b>Interactive Workshop 5: Clinical Updates</b>
----------------------	-----------------------------------------------------

<b>16.30-16.40</b>	<b>The impacts of olipudase alfa on paediatric patients with ASMD and their families</b> Justin L. Hopkin, M.D. University of Rochester Medical Centre
<b>16.40-16.50</b>	<b>Clinical Development of Olipudase Alfa for ASMD NPB</b> Dr Robin Lachmann PhD, FRCP National Hospital for Neurology and Neurosurgery, London
<b>16.50-17.05</b>	<b>Fifteen years from discovery to bedside: HPβCD, where to now?"</b> Caroline Hastings MD UCSF Benioff, USA
<b>17.05-17.15</b>	<b>Oral, dental and craniofacial features in chronic acid sphingomyelinase deficiency</b> Dra Cláudia Viegas Bender Dentist and Researcher, Universidade Federal do Rio Grande do Sul, Brazil
<b>17.15-17.25</b>	<b>Development of Clinical Guidelines for ASMD</b> Shaun Bolton, BSc, MSc Operations Consultant, INPDR
<b>17.25-17.35</b>	<b>Discussion</b>

**17.35-17.45      Community Voices: Living with Niemann-Pick Diseases**

**17.45                      Workshop CLOSE**  
**18.00-19.00          Networking Reception:**  
Rosewood Foyer and Conservatory

**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  
Suite 2, Vermont House, Washington, Tyne and Wear, NE37 2SQ, UK  
Telephone: 00 44 (0)191 415 0693 Email: [info@npuk.org](mailto:info@npuk.org)  
Website [npuk.org](http://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:**



Cyclo Therapeutics, 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.



KemPharm 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



Azafaros 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



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: <https://www.pmcpa.org.uk/>

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

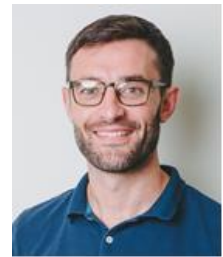
Registered Charity: England and Wales (1144406) / Scotland (SCO45407)  
Registered as a company limited by guarantee in England and Wales (07775835)



# **Annual Family Conference Programme of Events**

7<sup>th</sup> – 9<sup>th</sup> October 2022

Woodlands Event Centre  
Wyboston Lakes, Great North Road,  
Wyboston, Bedfordshire, MK44 3AL, UK



Dear Families, Friends, and Supporters,

On behalf of the NPUK Board of Trustees, I have pleasure in welcoming you to our flagship events - the Interactive Workshop on Niemann-Pick Diseases and our Family Conference. We are pleased to be back at Wyboston Lakes; and to be able to bring our community together with once again.

As in previous years, the programme is full of interesting and valuable talks, presentations, and workshops, which will cover many thought-provoking areas of research and advances in treatments. As we reach new milestones in terms of developments and stages in research, I think it is always important to take a look at just how far we have come - such advances that we speak of today would have been simply unthinkable when NPUK began in 1991.

Although we still have a long way to go, and at times the road can appear unsure, I find it encouraging to look upon the progress we have achieved together as a community, and to remind myself that it is as a community that we will continue to make progress.

I for one am very much looking forward to being able to reunite with many familiar faces in the community, as well as offer a warm welcome to those who have recently joined NPUK. If you feel a little apprehensive as this is your first time at Conference, just remember that everyone has been in your position at one stage, we are a very friendly bunch, and I am sure you will find it an inspiring experience - it's no coincidence that our community members return year on year!

The NPUK staff and trustees, along with the friendly and efficient team at the Woodlands Event Centre, are here to ensure you have an enjoyable stay at our event. Please feel free to speak to a member of either team should you have any questions about the venue or your accommodation.

I hope you will find your experience at our Conference informative and rewarding. If you need any help at all, please ask – NPUK representatives will be happy to assist you.

With best wishes,

Dr. William Evans  
Chair, NPUK



# Delegate Information – The Woodlands Event Centre

## On Arrival

The Woodlands Event Centre has free parking on-site including designated spaces for disabled guests (located close to the main entrance).

## The Woodlands Event Centre Reception

Reception is open from 7.00am until 10.30pm; a night porter is on duty outside these hours.

You will be able to check into your accommodation any time from 3.00pm on your arrival day. Early check-in rooms can be arranged if required. The Hotel respectfully request that you check out of your accommodation by 11.00 on the day of departure, unless otherwise arranged. To pre-book a late checkout room, please speak to a member of the NPUK team or book direct with the hotel reception. A secure luggage store is available at reception.

## Niemann-Pick UK Registration Desk

The NPUK registration desk will be available in the Cedar lounge from 08.30 on Friday 7<sup>th</sup> October, until 12.30 on Sunday 9<sup>th</sup> October. NPUK team members will be available to assist you with any queries regarding the hotel or the Conference programme/activities. You can also approach team members or NPUK Trustees for help or information at any time.

## Accommodation

All bedrooms are en-suite and include tea and coffee making facilities, hairdryer and toiletries, ironing facilities, TV, and Wi-Fi internet access. There is a strict **“No Smoking or Vaping”** policy in bedrooms and throughout the Woodlands Event Centre.

## Meals

All meals will be served in the Olive Restaurant:

Breakfast	Lunch	Dinner
Weekdays: 7.00-9.00 Weekends: 7.00-10.00	Please refer to the Conference programme for timings.	Thursday 6 <sup>th</sup> October, 19.00-21.00 Friday 7 <sup>th</sup> October 18.30- 21.00 Saturday 8 <sup>th</sup> October: Gala Dinner 19.00 prompt

Refreshments will be available throughout the event from Paddy and Scott’s Coffee House, located close to Reception.

## Entertainment and Leisure Facilities

The hotel has a Health and Fitness Club situated on site, with a heated swimming pool, sauna, and steam room plus a fully equipped gym. Access is free of charge for NPUK Conference delegates. Please be advised that due to health and safety regulations children under the age of 16 years need to be accompanied by an adult, one for every 2 children, regardless of their swimming abilities.

The site also boasts a Spa ([www.yspa.co.uk](http://www.yspa.co.uk)), a superb 18-hole golf course, an all-weather driving range and a water sports centre. Charges for these facilities are payable locally. For further information, please contact hotel reception or visit <http://www.wybostonlakes.co.uk/golf>

Bar facilities will be available in the main lounge from 15.00 until 23.00. **Please note that the Woodlands Event Centre does not accept cash - card payments only.** Following our Gala Dinner on Saturday evening, delegates will be entertained with live music from party band, **Makapaka**. Throughout the weekend, you can join our raffle, browse our craft stall, and try your hand at our tombola, all staffed by members of our wonderful volunteer team.

## Medical Assistance

Bedford Hospital (MK42 9DJ) and Hinchbrook Hospital (PE29 6NT) are within 10 miles of the locality and Addenbrooke’s Hospital in Cambridge (CB2 0QQ) have A&E facilities if required.

## Session Guide:

For Joint Sessions follow:	
For NP-C Sessions follow:	
For ASMD Sessions follow:	
Children and Young Adults' Activity Programme:	
Reflections	

### This is YOUR Conference!

- ✓ We aim to create an informal atmosphere in which everyone feels comfortable
- ✓ Please always wear your badge
- ✓ Drop in and drop out - feel free to come and go during sessions
- ✓ You will be able to connect with family and professional delegates during coffee, lunch, and social time
- ✓ Refreshment stations will be available throughout the weekend, please help yourself at any time
- ✓ If you would like to, you can add your personal thoughts and messages to our Wishing Tree
- ✓ You will find our registration desk in the Cedar Lounge. For help or advice, please approach a member of the NPUK Team at any time
- ✓ Be "Social"! Help NPUK to raise awareness by sharing your photos and experiences of Conference across social media, using any of the following hashtags #NPUKAFC22 #NPUKIW22 #TeamNPUK #NPUKFamily #NiemannPickUK #NPUK

08.30	<b>Registration</b> The Interactive Workshop on Niemann-Pick Diseases
09.00-17.45	<b>The Interactive Workshop on Niemann-Pick Diseases</b> Rosewood Suite
15.00	<b>Check-in and Registration</b> NPUK Family Conference
16.00-18.00	<b>Patient and Carer Focus Group</b> (Closed session – invitation only) Beech 1
18.30 - 21.00	<b>Dinner</b> The Olive Restaurant
19.00 - 20.30	<b>Children and Young Persons' Activity Programme: Movie Time!</b> Beech 2 All children and young people are welcome to this supervised session.
19.00 - 20.30	<b>Getting to Know You</b> Rosewood 2 & 3  Join us at one of the following informal sessions to say hello, get to know other family delegates and meet NPUK Staff members and Trustees
20.30	<b>Children and Young Persons' Activity Programme: Volunteer Briefing</b> Beech 2

8.30 - 9.00	<b>Conference Registration</b> Cedar Lounge
-------------	------------------------------------------------

8.30 - 9.00	<b>Children and Young Adults' Activity Programme Registration</b> Beech 2 Please register promptly! All activities located within the Salix Suite, Wyboston Lakes.
-------------	--------------------------------------------------------------------------------------------------------------------------------------------------------------------------

09.00- 10.40	<b>Joint Sessions</b> Rosewood Suite
09.00	<b>Welcome and Overview</b>  <b>Dr William Evans</b> Chair, NPUK
09.10 - 10.00	<b>Where are we now?</b>  <b>Research update on NPC: where are we now?</b> Frances Platt, PhD, FMedSci, FRS Head of Department of Pharmacology, University of Oxford  <b>The clinical trial landscape for Niemann-Pick type C disease</b> Professor Marc C. Patterson, MD FRACP Professor of Neurology, Pediatrics and Medical Genetics Mayo Clinic Children's Center, Rochester, MN, USA  <b>ASMD: an overview of research and clinical options</b> Dr Robin Lachmann, PhD, FRCP Consultant in Metabolic Medicine, National Hospital for Neurology, London  <b>Questions and discussion</b>
10.00 - 10.40 Cedar Lounge	<b>Where do we want to be?</b>  <b>Research and Therapy Development:</b> Community voices, hopes and expectations
10.40 - 11.00	<b>Refreshment Break</b> Paddy and Scott's Coffee House

11.00- 11.30	<b>Joint Sessions</b> Rosewood Suite  <b>How do we get there?</b>	
11.00 - 11.30	<b>Drug repurposing in rare diseases; the benefits and opportunities</b> Rick Thompson CEO, Rare Beacon	
11.30	<b>Reflections Activity</b> Meet in Reception	
11.30 - 12.45	<b>Parallel Sessions</b>	
<b>NPC Sessions:</b> Rosewood Suite		<b>ASMD Sessions:</b> Ash 1 & 2
<b>NPC Research Updates</b>  <b>11.35 -12.00</b> <b>Altered Inflammation in NPC</b> Frances Platt, PhD, FMedSci, FRS Head of Department of Pharmacology, University of Oxford  <b>12.00-12.20</b> <b>Role of NPC1 Protein in the persistence of intracellular pathogens</b> Yuzhe Weng DPhil Student Department of Pharmacology, University of Oxford  <b>12.20-12.45</b> <b>Patient-directed efforts at the University of Notre Dame</b> Kevin T. Vaughan Associate Professor of Cell Biology, University of Notre Dame, Indiana, USA		<b>ASMD Research and Clinical Updates</b>  <b>11.35 -11.55</b> <b>Clinical Development of Olipudase Alfa for ASMD NPB</b> Dr Robin Lachmann PhD, FRCP National Hospital for Neurology and Neurosurgery, London  <b>11.50 -12.20</b> <b>The impacts of olipudase alfa on paediatric patients with ASMD and their families</b> Justin L. Hopkin, M.D. University of Rochester Medical Centre  <b>12.20 - 12.45</b> <b>Development of Clinical Guidelines for ASMD</b> Shaun Bolton, BSc, MSc Operations Consultant, INPDR and Justin L. Hopkin, M.D., University of Rochester Medical Centre
12.45-13.45	<b>Lunch</b> The Olive Restaurant	

13.45- 14.30 Rosewood Suite	<b>Joint Sessions</b>	
13.45-14.30	<p><b>Newborn screening: opportunities and challenges: Introduction</b> William Owen Trustee and Research Coordinator, NPUK</p> <p><b>A Rare Life Lived Better; Newborn Screening</b> Bob Stevens Group CEO, MPS Society &amp; Rare Disease Research Partners Vice Chair, LSD Collaborative</p> <p><b>Panel Discussion:</b> The benefits, challenges, and practicalities of newborn and future screening for Niemann-Pick and other rare genetic conditions.</p> <ul style="list-style-type: none"> <li>○ Dr Robin Lachmann, PhD, FRCP, Consultant in Metabolic Medicine, National Hospital for Neurology, London</li> <li>○ Prof. Paul Gissen, Consultant in Paediatric Metabolic Diseases, Great Ormond Street; Wellcome Trust Sr. Research Fellow in Clinical Sciences, UCL</li> <li>○ Karen Harrison, Support Services Manager, AlexTLC</li> </ul>	
14.30 – 15.20	<b>Parallel Sessions</b>	
<b>NPC Sessions: Rosewood Suite</b>		<b>ASMD Sessions: Ash 1 &amp; 2</b>
<p><b>14.30</b> <b>Focus on Gene Therapy for NPC</b></p> <p>Dr Ahad Rahim Associate Professor, The School of Pharmacy, University College London</p> <p>Cristin Davidson PhD Researcher, National Institutes of Health, Maryland, USA Project Manager, SOAR-NPC</p> <p>Prof. Paul Gissen, Consultant in Paediatric Metabolic Diseases, Great Ormond Street; Wellcome Trust Sr. Research Fellow in Clinical Sciences, UCL</p> <p><b>15.00</b> <b>Gene Therapy: your questions answered</b></p>		<p><b>14.30</b> <b>Sanofi Rare Disease Programme</b> Ben Whitehouse, Head of Rare Diseases, UK Sanofi</p> <p><b>14.55</b> <b>An approved therapy for ASMD; what can we expect?</b> Discussion facilitated by ASMD community members James Brooks and James Dyson, with guest speaker Bob Stevens, Rare Disease Dad.</p>


15.20 - 15.40	<b>Refreshment Break</b> Paddy and Scott's Coffee House
15.40- 17.00 Rosewood Suite	<b>Joint Sessions</b>
15.40 - 16.30	<b>Panel Session: Clinical Development, Past, Present and Future</b> This is your opportunity to ask our experts any burning questions you have about clinical trials and development.
16.30 - 17.00	<b>Invisible Interviews: Niemann-Pick diseases</b> John Lee Taggart NPUK Communications Manager and Lingxi Zhang, Animator and Creative Director
17.00	<b>Day 1 Close</b>
19.00	<b>Gala Dinner</b>  <b>Rosewood Suite</b> Please check the seating plan for your table number.
20.00	<b>The 2022 Peter Carlton Jones Memorial Award</b> Introduced by: William Owen, Trustee and Research Coordinator, NPUK  <b>The role of the NPC1-PMCA2 complex in Niemann-Pick disease type C</b> Dr Maria E. Fernandez-Suarez Platt Lab, University of Oxford
20.00 – 11.00	<b>Entertainment:</b> Makapaka Party Band

09.00	<b>Children and Young Persons' Activity Programme</b> Beech 2 and Salix Suite
09.30	<b>Welcome to Day 2</b> Rosewood Suite
09.35 – 10.45	<b>Breakout Session:</b> <b>Emotional Resilience against anxiety, stress, and depression</b> Poplar  Steven Neal NPUK Counselling and Wellbeing Officer
09.35 -10.45	<b>Joint Sessions</b> Rosewood Suite
09.35	<b>INPDR: International Niemann-Pick Disease Registry</b>  <b>Patient Reported Data</b> Jackie Imrie, Clinical Research Manager  <b>Niemann-Pick Type C – The patient reported experience, an NPUK led survey</b> Conan Donnelly, Chief Executive Officer, INPDR  <b>Quality of life in patients with Niemann-Pick disease and their carers</b> Jackson Pountney, Psychology PhD student, Aston University  <b>Home Based Monitoring</b> Claire Fyvie, NPUK Trustee (Co-opted) Solomon Mbua, CRA Consultant, INPDR Jackie Imrie, Clinical Research Manager
10.30	<b>Web-based animations to inform and connect patients and support organisations</b> Maria E. Fernandez-Suarez, PhD Postdoctoral Researcher Department of Pharmacology, University of Oxford
10.45 - 11.15	<b>Refreshment Break and Check-out</b> Paddy and Scott's Coffee House



11.15-12.30	<b>Joint Sessions</b> <b>Rosewood Suite</b>		
11.15 -11.35	<b>Physical Therapy in Niemann-Pick Diseases</b> Alvaro Berzal Gomez Paediatric Physiotherapist		
11.35 -12.00	<b>NPUK: Meet our Care &amp; Support Team</b> Laura Bell, Clinical Nurse Specialist Louise Metcalfe, Families Officer Steve Neal, Counselling and Wellbeing Officer		
12.00 -12.20	<b>The Impact of Fundraising</b> Jenny Geere NPUK Fundraising Officer		
12.20-12.30	<b>Closing Remarks</b>  <div> <b>Dr William Evans</b> Chair, NPUK </div> <div> <b>Helen Carter</b> Trustee, NPUK </div> <div> <b>Joella Melville</b> Trustee, NPUK </div>		

12.30-13.30	<b>Lunch</b> The Olive Restaurant  Please return your badges and completed evaluation forms to the NPUK Reception Desk. Thank you!		
-------------	---------------------------------------------------------------------------------------------------------------------------------------------	--	--



Huge thanks to everyone who has attended or contributed to this event, we simply couldn't do it without you!

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.

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:**



Cyclo Therapeutics, 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.



KemPharm 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



Azaforos 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



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

Registered Charity: England and Wales (1144406) / Scotland (SCO45407)  
Registered as a company limited by guarantee in England and Wales (07775835)