



Supporting those affected
by Niemann-Pick



10th Interactive Workshop on Niemann-Pick Diseases &
26th Annual Family Conference
PROGRAMME

19th-22nd September 2019, at Wyboston Lakes, Bedfordshire, MK44 3AL

Overview of Events:

We would like to welcome you to the 26th NPUK Annual Family Conference and 10th Interactive Workshop on Niemann-Pick Diseases, a milestone event which seeks to celebrate the fantastic efforts of researchers and clinicians working in the Niemann-Pick field.

This programme contains information regarding every facet of the weekend, including schedules for the Interactive Workshop, Annual Family Conference, and Children & Young Persons' Activity Programme. If you have any questions or issues at any time, please do not hesitate to approach members of the NPUK Team (orange badges), who will be happy to help with your inquiry.

Thursday

19th September

International Niemann-Pick Disease Registry (INPDR)

**The Willows
Training Centre:
Birches Room**

Friday

20th September

10th Interactive Workshop on Niemann-Pick Diseases

**The Willows
Training Centre:
Willows
Conference Room**

Saturday

21st September

26th Niemann-Pick UK Annual Family Conference

**The Woodlands
Event Centre**

Sunday

22nd September

26th Niemann-Pick UK Annual Family Conference

**The Woodlands
Event Centre**



- A** Information Centre
- B** Conlan Restaurant and Bar
- C** The Woodlands Event Centre
- D** Potton House
- E** RIDE Leisure (Watersports Centre)
- F** Golf Driving Range
- G** 1Life Health and Leisure Club
- H** The Knowledge Centre

- I** Oakley Suite
- J** The Waterfront Hotel, Golf & Spa
- K** The Fairways
- L** Wyboston Lakes Golf Centre
- M** Y Spa
- N** Golf Course - Putting Green & 1st Tee
- O** Waterfront Restaurant & Bar
- P** Fountains Restaurant

- Q** The Willows Restaurant & Bar
- R** The Willows Training Centre
- S** Fishing Lake
- T** Firebrand Training Centre
- U** Rural Site
- Footpath**

Dear Families, Friends, and Supporters,

On behalf of the NPUK Board of Trustees, I have pleasure in welcoming you to our 10th Interactive Workshop and 26th Annual Family Conference.

NPUK works throughout the year on behalf of those affected by Niemann-Pick disease to ensure access to optimum care, increase understanding of the condition and to facilitate research and treatment options. The pace of change in scientific research and clinical care continues to accelerate, bringing hope to us all. This is demonstrated once again by our busy Conference programme; which includes updates from UK and International speakers covering the subjects of clinical trials, basic science research and practical care issues.

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.

At the end of the weekend, please take a moment to complete the online evaluation form which can be found on our website, npuk.org. Your feedback is very important to us and will help us to tailor future conferences to meet your needs.



Will Evans

**Dr. William Evans,
NPUK Trustee & Chair**

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NPUK Board of Trustees

NPUK is governed by a Board of Trustees who are responsible for delivering the aims and objectives of the Charity. We encourage you to get to know them over the course of this weekend, you can also learn more at: npuk.org/about-us



Will Evans



David Holton



Jackie Imrie



Richard Rogerson



David Roberts



Bill Owen



Janice Brooks



Helen Carter



Joella Melville



Fran Platt
(Co-opted)



Stefanie Rymsza
(Co-opted)



Fiona Dunne
(Co-opted)



Jenny Charman
(Co-opted)

NPUK Staff Team

NPUK has a small but dedicated staff team who are responsible for delivering the services of NPUK and provide much-needed care and support.

Please don't hesitate to approach members of our staff team at any time throughout the weekend, they will all be happy to help with any questions or issues you may encounter. You can also learn more about each individual and what their respective roles bring to NPUK, here: npuk.org/about-us/meet-our-team



Toni Mathieson
Chief Executive



John Lee Taggart
Communications Officer



Laura Bell
Clinical Nurse Specialist



Elizabeth Davenport
Senior Families Advocate



Steve Neal
Project Families Officer



Louise Metcalfe
Project Team Leader



Christine Jopling
Finance & Admin Officer

EVENT Information

Wyboston Lakes, Bedfordshire, UK

OVERVIEW OF EVENTS:

This year, NPUK's events will be situated across three of Wyboston Lakes primary venues, the Waterfront Hotel, The Willows Training Centre and the Woodlands Event Centre. The distance between venues is short, approximately a five-minute walk to each. If you prefer not to walk, transport between venues will be provided, further information will be included in your delegate pack and will be available from Reception.

Please note: Accommodation for guests arriving prior to Friday 20th September will be situated in the Waterfront Hotel. If you are transferring to the Woodlands Event Centre on Friday, assistance will be provided.

Thursday 19th September:

Willows Training Centre: The Birches Room
International Niemann-Pick Disease Alliance and International Niemann-Pick Disease Registry

09:30-12:00 INPDA Executive Committee
13:30-15:30 INPDR Working Group
16:00-18:00 INPDR Scientific Advisory Committee
18:30-19:00 Networking Reception, the Waterfront Hotel, Lakeview Room
19:00-21:00 Dinner at your leisure, the Waterfront Hotel, Fountains Restaurant

Friday 20th September:

Willows Training Centre, the Willows Conference Room
9:30-17:30 10th Interactive Workshop on Niemann-Pick Diseases

Friday 20th to Sunday 22nd September:

The Woodlands Event Centre
26th Annual Family Conference

Help and Information:

Throughout our events, please feel free to approach NPUK team members or Trustees for help or information at any time - they can be identified by the orange stripe on their badges.

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) has A&E facilities if required.

CHECKING IN/OUT:

You will be able to check into your accommodation from 15:00 on your arrival day.

The hotel respectfully request that you check out your accommodation by 11:00am on the day of departure, unless otherwise arranged.

Early check-in or late check-out rooms can be arranged, with prior notice. Please speak to a member of the NPUK team or book direct with the hotel reception. A secure luggage store is available at reception.



This is **YOUR** Conference!



We aim to create an informal atmosphere in which everyone feels comfortable



Please wear your badge at all times



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



If you need information, help, or advice, you can find a member of the NPUK team at the Registration Desk or just ask any team member with an orange stripe on their badge.



We encourage you to “be social” this weekend, please share your photos on Facebook, Instagram, and Twitter with relevant hashtags: e.g. #NPUKConf19, #NPUKFamily

PRINCIPLES FOR INTERACTION

Niemann-Pick UK (NPUK) works towards one clear goal: to improve the lives of people affected by Niemann-Pick Diseases (NPD). Our endeavours are directed into three key areas: offering care and support; sharing information; and the support of research and therapeutic development.

As research advances and as an increasing number of clinical trials are initiated, it is even more critical that the information we provide to our community is up-to-date, accurate and balanced. This includes ensuring all information provided is, fair, not misleading in any way and capable of substantiation. In addition, we encourage transparency and accountability in collaborative working and the opportunity for open debate to inform the patient community.

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. Throughout our activities and interactions, NPUK strives to maintain integrity, independence, impartiality and transparency in all we do.

Therefore, as we facilitate this exchange of clinical and scientific information, we would like to remind attending pharmaceutical and industry representatives that all communications and interactions at our conference must abide by the Association of the British Pharmaceutical Industry (ABPI) Code of Practice. Please visit www.abpi.org.uk for further information.

Another useful resource is the 'Principles for interactions with biopharmaceutical companies: the development of guidelines for patient advocacy organisations in the field of rare diseases'. These Guidelines, which were developed by an Independent Expert Panel, recommend best practices and standards for interactions between patient advocacy organisations and industry. The Guidelines can be found on the NPUK website www.npuk.org.

NPUK also complies with this code (in particular Clause 27, Relationships with Patient Organisations) in our interactions with all pharmaceutical companies engaged in developing therapies for NPD. As such, we will not endorse or promote one particular trial above another.

However, we recognise the significant benefits that can arise from working collaboratively with all stakeholders, and will continue to offer unstinting support, encouragement and guidance to all who share in our goals; including pharmaceutical companies, researchers and academic institutions, health care agencies and other charities.

NPUK believes these actions are in the best interests of the NPD community. We will continue to passionately support individuals and families affected by NP diseases and all those who share our goal.

NIEMANN-PICK UK AND CINEBITES PRESENT

vertical
supranuclear
gaze
palsy

go make memories!



Niemann-Pick UK (NPUK)

Contact us:

info@npuk.org

0191 415 06 93

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STARRING LLEYTON COOMBS EMILY STRIDE CHRISTOPHER HUGHES

BODHI RAE BREATHNACH ANDREA HALL

MUSIC BY CHRIS KÖBKE CINEMATOGRAPHER JAMES WESTLAKE

PRODUCTION DESIGNER ISA SHAW-ABULAFIA POSTER DESIGNER ROB HOTCHKISS

ANIMATION BY JAMES RICHARDSON WRITTEN BY ALEX SUTTON

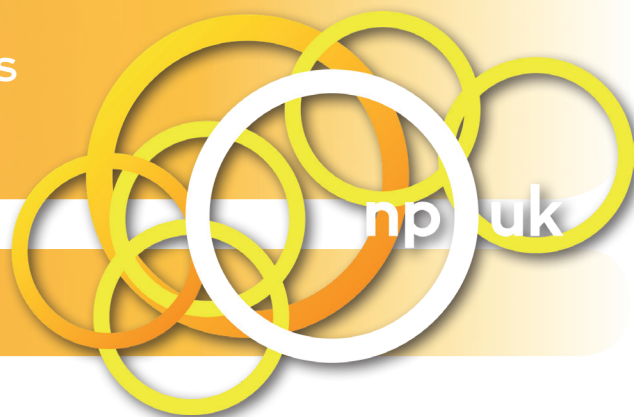
EXECUTIVE PRODUCER JOHN LEE TAGGART DIRECTED & PRODUCED BY CARL MASON



Supporting those affected by
Niemann-Pick

Niemann-Pick UK: 10th Interactive Workshop on Niemann-Pick Diseases

20th September 2019



09:00-09:30 Registration and Refreshments

09:30-09:35 Welcome and Introduction

Professor Frances Platt

Department of Pharmacology, University of Oxford, UK

09:35-11:20 Research Updates

09:35-09:45 A new target in NPC

Dr. Dan Sillence

Leicester School of Pharmacy, De Montfort University, Leicester

09:45-09:55 Novel Cyclodextrin Chemistry for Potential NPC Treatment

Professor John Spencer

Chemistry Department, School of Life Sciences, University of Sussex

09:55-10:05 The relationship between organelle contact sites, cholesterol transport and NPC

Dr Emily Eden, Research fellow, UCL Institute of Ophthalmology

10:05 -10:15 Discussion and Questions

10:15-10:25 Insights from NPC: Mechanistic convergence and shared therapeutic options in Tangier disease

Allie Colaço: Postdoctoral Researcher

Department of Pharmacology, University of Oxford

10:25-10:35 Impaired Lysosome transport to distal axons contributes to autophagic stress in NPC neurons

Joseph Roney, DPhil Student

Department of Pharmacology, University of Oxford

10:35 -10:45 Formation of ion channels by cyclodextrin provides a potential mechanism of action for NPC cellular correction

Dr. Emyr Lloyd-Evans, School of Biosciences, Cardiff University, UK

10:45-11:05 Enhancing the endocannabinoid system to treat brain pathology in NPA

Dr. Dolores Ledesma

Centro de Biología Molecular Severo Ochoa, Universidad Autónoma de Madrid

Dr. Melissa P. Wasserstein M.D. Chief, Division of Pediatric Genetic Medicine, Associate Professor of Pediatrics, Children's Hospital at Montefiore, New York, USA

11:05 -11:20 Discussion and Questions

11:20-11:50 Refreshment Break: Willow's Bar

11:50-13:30 **Interactive Workshop 1:**
Predicting the Clinical Path and Severity of NPC – Where are we now?
Chair: Dr. William Evans, Chair, NPUK

11:50-12:00 **Overview**
Dr. William Evans

12:00-12:10 **Bloodspot Diagnostics for NPC**
Dr. Wendy Heywood
Senior Research Associate, UCL Institute of Child Health

12:10-12:35 **Newborn Screening for NPD**
Dr. Melissa P. Wasserstein M.D.
Chief, Division of Pediatric Genetic Medicine, Associate Professor of Pediatrics,
Children's Hospital at Montefiore, New York, USA
William Owen
NPUK Trustee & Research Coordinator

12:35-12:45 **NPC Disability Scales: what are the differences?**
Christina Guldberg
Director, Clinical Outcomes, Orphazyme A/S

12:45-12:55 **The Role of Biomarkers**
Professor Marc C. Patterson, MD
Professor of Neurology, Pediatrics and Medical Genetics
Mayo Clinic Children's Center, Rochester, MN

12:55-13:05 **International Niemann-Pick Disease Registry**
Shaun Bolton
INPDR Registry Management Team and Inherited Metabolic Disorders
Research Co-ordinator, University Hospitals Birmingham NHS
Foundation Trust

13:05-13:30 **Discussion and Questions**

13:30-14:30 Networking Lunch & Posters: Willow's Bar

14:30-15:20 **Interactive Workshop 2**
New Developments in Niemann-Pick diseases
Chair: Professor Paul Gissen
Consultant in Paediatric Metabolic Diseases, Great Ormond Street Hospital,
Wellcome Trust Senior Research Fellow in Clinical Sciences at the UCL
Institute of Child Health

14:30-14:40 **Development of Gene Therapy for Niemann-Pick Type C**
Dr. Michael Hughes
UCL School of Pharmacy, London

14:40-14:50 Use of Efavirenz to treat brain pathology in NPC

Dr. Dolores Ledesma,
Centro de Biología Molecular Severo Ochoa
Universidad Autónoma de Madrid

14:50-15:00 S1P5 Receptor Agonists: Potential New Therapeutics for the Treatment of Neuronopathic Features of Niemann-Pick Disease Type C

Enchi Liu, PhD, Clinical Development, E-Scape Bio

15:00-15:20 Discussion and Questions

17:10-17:30 Clinical Updates

Co-Chairs:

Dr Marie Vanier, MD, PhD,
INSERM Research Director (em.) Lyon, France
Dr David J Begley, Kings College London,
Institute of Pharmaceutical Science

15:20-15:30 Clinical Development of Trappsol

Sharon Hrynkow, Chief Scientific Officer, CTD Holdings, Inc.

15:30-15:40 IB1001-201: An Innovative Trial Design for the Development of a Novel NPC Treatment

Taylor Fields, Vice President, IntraBio

15:40-15:50 Clinical Development of VTS-270 for NP-C1

Susan Van Meter, Senior Director, Clinical Research, Mallinckrodt Pharmaceuticals

15:50-16:00 AIDNPC: Development of Arimoclomol for NPC

Thomas Kirkegaard Jensen PhD, Chief Scientific Officer, Orphazyme A/S

16:00-16:20 Refreshment Break

16:20-16:30 Clinical Development of Olipudase Alfa for ASMD NPB

Gauri Krishna
Clinical Research Fellow, National Hospital for Neurology
and Neurosurgery, London

16:30-16:40 Impact of Long Term Intrathecal Adrabetadex Treatment on Clinical and Cognitive Decline in NPC

Elizabeth Berry-Kravis, MD, PhD
Professor of Pediatrics, Neurological Sciences and Biochemistry, Rush
Medical Centre, Chicago USA

16:40-17:10 Discussion and Questions

17:10-17:30

Patient Voice

Chair: Professor Frances Platt

Department of Pharmacology, University of Oxford, UK

17:10-17:25

Living with NPC

Helen and Hollie Carter

17:25-17:30


Discussion and Questions

17:30

Workshop CLOSE

17:30-18:30

Networking Reception - Willows Coffee Lounge



“...each one of us can
make a difference.
Together we can
make a change...”

Barbara Mikulski

"...the NPUK Conference is a fantastic opportunity to spend the weekend with patients, families, and professionals..."

Laura Bell

NPUK Clinical Nurse Specialist

Session Guide:

For Joint Sessions follow

For NP-C Sessions follow

For ASMD Sessions follow

Children & Young Persons' Activity Programme

For Reflections follow

For Wellbeing follow

Go Make Memories



26TH ANNUAL FAMILY CONFERENCE

FRIDAY 20TH SEPTEMBER 2019

- 15:00-17:00 Arriving Guests:** please join us for welcome refreshments in the Olive Restaurant, where we can provide assistance with Check-in.
Please note: If you are arriving prior to 15:00 please join us at The Waterfront Bar
- 17:00-20:00 Annual Family Conference and Children & Young Persons' Activity Programme**
Registration (Cedar Lounge), please collect your badge and delegate pack!
- 18:30-21:30 Dinner:** The Olive Restaurant, at your leisure

20:00-21:30 Getting to Know You

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

Yew Lecture Theatre **Join us for a sing-a-long film showing of the Greatest Showman!**
All children and young people are welcome to this supervised session

Poplar **ASMD Niemann-Pick A and B**
Hosts: Sandra Cowie (ASMD NP-B) and Janice Brooks (ASMD NP-B Grandmother and NPUK Trustee)

Beech 1 **For Adults with NP-C**
Host: Louise Metcalfe, NPUK Project Coordinator

Ash 1&2 **For NP-C Mums/Carers**
Hosts: Helen Carter (NP-C Mum/NPUK Trustee) and Fiona Dunne (NP-C Mum)

Ebony **For Dads, Lads and Carers**
Hosts: Will Evans (NP-C Dad and NPUK Trustee) and Steven Neal (NPUK Project Families Officer)

Lime **For Extended Family and Friends**
Host: David Roberts (NPUK Trustee)

Cherry **For Teenagers and Young Adults**
Hosts: Laura Bell, NPUK Clinical Nurse Specialist, Katie Reynolds, NPUK Volunteer

21:00-21:30 Activity Programme Volunteers' Briefing
Cherry

SATURDAY 21ST SEPTEMBER

08:30-09:00 **Conference Registration – Cedar Lounge**
Please collect your badge and delegate pack from our helpful team!

08:30-09:00 **Registration - Children and Young Persons Activity Programme – Olive 1**
Please register promptly, we leave for Whipsnade Zoo at 9:15am!

09:00-10:45 **Reflections – The Apple Lounge**
Supportive Sessions for those who have lost a loved one
Elizabeth Davenport, Senior Families Advocate and
Fiona Dunne, NP-C Mum and NPUK Trustee (Co-opted)

09:00-10:45 **Joint Sessions - Rosewood Suite**
Session Chair: Dr William Evans, Trustee and Chair, NPUK

09:00-09:15 **Welcome and Overview**
Dr William Evans
Chair, NPUK

09:15-09:30 **Go Make Memories – An Introduction**
Carl Mason, Director/Producer
John Lee Taggart, NPUK Communications Officer

09:30-09:45 **Keynote Address:**
Professor Marc C. Patterson MD
FRACP Professor of Neurology, Pediatrics and Medical Genetics
Mayo Clinic, Minnesota, USA

09:45-10:00 **The International Niemann-Pick Disease Registry (INPDR)**
Jacqueline Imrie
INPDR Registry Management Team and NPUK Trustee

10:00-10:30 **The Importance of Newborn Screening**
Melissa P. Wasserstein MD
Chief, Division of Pediatric Genetic Medicine, Associate Professor
of Pediatrics, Children's Hospital at Montefiore, New York

Christopher Andrews
Co-Founder, The Firefly Fund, Austin, Texas

William Owen
NPUK Trustee and Research Co-ordinator

10:30-10:45 Discussion and Questions

10:45-11:15 Refreshment Break, Cedar Lounge

10:55-11:10 **Go Make Memories**
Screening in the Yew Technology Theatre

11:15-12:45 **Breakout Sessions:**

NP-C: Rosewood Suite

Session Chair:

Dr. Gregory M. Pastores, MD
Consultant, Adult Metabolic
Service, Mater Misericordiae
University Hospital, Dublin

11:20

Ongoing NP-C Patient Survey

Christina Guldberg
Director Clinical Outcomes
Orphazyme A/S

11:30

CTD's Phase II Intravenous Trial of Trappsol (R) Cyclo (TM) for NP-C

Dr. Reena Sharma
Co-ordinating PI for CTD's
EU Trial
Consultant Adult Inherited
Metabolic Disorders, Salford
Royal NHS Foundation Trust

11:40

Clinical Development of Arimoclomol for NP-C

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

ASMD: Poplar

Session Chair:

Sandra Cowie
ASMD Patient,
Director, Niemann-Pick
Canada
President, International
Niemann-Pick Disease
Alliance (INPDA)

11:20

ASMD Niemann-Pick types A and B: An Overview

Dr. Uma Ramaswami
Consultant in Inherited
Metabolic Disorders, Clinical
Lead, Lysosomal Disorders
Unit, Royal Free Hospital,
London

11:40

Discussion and Questions

11:50

Clinical Development of Olipudase Alfa for ASMD NP-B Patients

Melissa P. Wasserstein MD
Chief, Division of Pediatric
Genetic Medicine, Associate
Professor of Pediatrics,
Children's Hospital at
Montefiore, New York

Wellbeing: Ebony

Session Chair:

Steve Neal
NPUK Project Families
Officer

Strategies to Support Emotional and Physical Health

11:30

Exercise Therapy for NPD Patients and Carers (Video Presentation)

Sean Van Velsen
AEP MCEP
Van Velsen Exercise
Physiology

11:40

Discussion and Questions

11:50

Music and Fitness (Video Presentation)

Felicity Munroe
BEXSPSC1 (HONS)
Future Directions Wellbeing
and Fitness, Australia

NP-C: Rosewood Suite

ASMD: Poplar

Wellbeing: Ebony

11:50

Clinical Development of VTS-270 for NP-C1

Professor Paul Gissen,
Consultant in Paediatric
Metabolic Diseases,
Great Ormond Street
Hospital

12:00

**Clinical Trial IB1001-201:
A novel study for the
treatment of Niemann-Pick
Disease type C**

Dr. Uma Ramaswami
Consultant in Inherited
Metabolic Disorders, Clinical
Lead, Lysosomal Disorders
Unit, Royal Free Hospital,
London

12:10-12:45

**Panel Discussion and
Questions**

12:10

**Discussion and
Questions**

12:20

ASMD Q&A Session

12:35-12:45

**Discussion and
Questions**

12:00

Discussion and Questions

12:10-12:45

Managing Adversity

**REMEMBER:
you are free
to come and go
from sessions as
you please!**

12:45-13:45

Lunch: Olive Restaurant

13:15-13:30

Go Make Memories

Screening in the Yew Technology Theatre

STOCK UP!

This weekend is a great opportunity for you to pick up copies of our latest publications, such as our Patient Passports (pictured), Medical Alert Cards, and more! Please visit our Registration Desk or speak to a member of the NPUK Staff Team for further details.



13:45-15:30 Breakout Sessions:

Wellbeing: Ebony (below)

ASMD: Poplar (below)

NP-C: Rosewood Suite (page 20)

Wellbeing: Ebony

13:45 Emotional Resilience and Managing Stress

Tony Somers
Counsellor, Author and Life Coach

This workshop will provide an insight into emotional resilience and provide coping strategies to help you to manage stress and build resilience.

ASMD: Poplar

13:45 ASMD Patient and Family Voices

Session Chairs:
David Roberts, NPUK Trustee
Janice Brooks, NPUK Trustee

14:30 Workshop: Awareness and Support for ASMD Patients and Families

John Lee Taggart, NPUK Communications Officer



15:30-15.45
Go Make Memories
Screening in the
Yew Technology
Theatre

NP-C: Rosewood Suite

13:45

NP-C Breakout Sessions

Session Chairs:

Dr. Marie Vanier, MD, PhD

INSERM Research Director (em.) Lyon, France

Professor Marc C. Patterson MD

FRACP Professor of Neurology, Pediatrics and Medical Genetics Mayo Clinic, Minnesota, USA

NPC and its relationship to other diseases

Professor Frances Platt,

Department of Pharmacology,

University of Oxford, UK

13:55

Discussion and Questions

14:00

Gene Therapy: What you need to know

Gregory M. Pastores, MD, Consultant,

Adult Metabolic Service, Mater Misericordiae University Hospital, Dublin

14:10

Gene Therapy for Niemann-Pick Type C

Dr. Ahad Rahim, Associate Professor

The School of Pharmacy, University College London

14:20

Discussion and Questions

14:30

Impact of Long Term Intrathecal Adrabetadex Treatment on Clinical and Cognitive Decline in NPC

Elizabeth Berry-Kravis, MD, PhD. Professor of Pediatrics, Neurological Sciences and Biochemistry, Rush Medical Centre, Chicago USA

14:40

Discussion and Questions

14:45

Use of Efavirenz to treat brain pathology in NPCDr. Dolores Ledesma, Centro de Biología Molecular Severo Ochoa
Universidad Autónoma de Madrid

14:55

Discussion and Questions

15:00

NP-C Patient Focussed Drug Development Meeting

William Owen, NPUK Trustee and Research Coordinator

Gail Koujaian, NP-C Mum and Co-Founder of Hope for Hayley

Christopher Andrews, NP-C Dad and Co-Founder of the Firefly Fund

15:30-
15:45**Refreshment Break, Cedar Lounge**

15:45-17:15

Joint Session: Rosewood Suite**Session Chairs:**

Dr David Holton, Trustee and Treasurer, NPUK

Dr William Evans, Trustee and Chair, NPUK

15:50

Niemann-Pick A, B and C: different diseases with some important scientific similarities

Dr. Emyr Lloyd-Evans

School of Bioscience, Cardiff University

16:00

Discussion and Questions

16:05

The 2019 Peter Carlton Jones Memorial Award

Introduced by: William Owen, Trustee and Research Coordinator, NPUK

Impaired lysosome transport to distal axons contributes to autophagic stress in the neurodegenerative lysosomal storage disorder Niemann-Pick Type C

Joseph Roney, DPhil student

Department of Pharmacology, University of Oxford

Apolipoprotein B-100 decreases in association with reduction in PCSK9 concentration in Acid Sphingomyelinase Deficiency following enzyme replacement therapy

Bethanie Garside MSc

Research Technician, Lipid Research Group, University of Manchester

16:20

Discussion and Questions

16:25

Access to Treatments and Services for NPD**Overview of the Access Environment**

Josie Godfrey, JG Zebra Consulting

Patient and Family Panel Discussion

Contributors: Miriam Evans, Gabriella Walker, James Dyson

17:15

CLOSE

18:30

Join us in the Cedar Lounge and take time to write a message for our wish tree

18:45

Dinner: The Rosewood Suite

Check the seating plan for your table

20:30-
23:00

Entertainment: Disco by MKFM

17:45-18:00
Go Make Memories
Screening in the
Yew Technology
Theatre

SUNDAY 22ND SEPTEMBER

09:15 **Ash 1: Children and Young Persons' Activity Programme Opens**

09:30-10:45 **Joint Session: Rosewood Suite**

Welcome to Day 2
Toni Mathieson
Chief Executive, NPUK

09:45 **NPUK Care and Support Team**

Meet our Team – NPUK's Clinical Nurse Specialist, Senior Families Advocate, Project Team Leader and Project Families Officer will explain their roles and answer your questions regarding care or treatment for NPD, or practical day to day issues including benefits, housing and education, aids and adaptations or local support services.

10:30 **Therapeutic-driven whole genome sequencing of Niemann-Pick type C disease: an update on an international study (video presentation)**

Andrew Munkacs, Ph.D. Senior Lecturer (Assistant Professor)
Victoria University of Wellington, School of Biological Sciences

10:45-11:15 **Refreshment Break and Check Out**

11:15-12:45 **Cherry: NPUK Youth Council**

11:15-13:00 **Joint Session: Rosewood Suite
Patient and Family Voices**

Session Chairs: Elizabeth Davenport, NPUK Senior Families Advocate, David Roberts, NPUK Trustee

11:15-12:00 **Sibling Voices**

12:00-12:50 **Family Voices**

12:50-13:00 **Closing Remarks**
Dr William Evans
Trustee and Chair, NPUK

thank
you

13:00-14:00 **Lunch - The Olive Restaurant**
Please remember to return your badges!



Be **social** with NPUK:



NPUK CHILDREN & YOUNG PERSONS' ACTIVITY PROGRAMME 2019



FRIDAY 20TH SEPTEMBER 2019

- 17:00-20:00** **Registration - Children and Young Persons' Activity Programme**
Cedar Lounge
- 18:30-21:30** **Dinner: The Olive Restaurant**
- 20:00-21:30** **Join us for a sing-a-long film showing of the Greatest Showman!**
The Yew Technology Theatre
All children and young people are welcome to this supervised session
- 20:00-21:00** **Getting to Know You**
Join us at the following informal session to say hello, get to know others and meet NPUK Staff members, Volunteers and Trustees
- Cherry: For Teenagers and Young Adults**
Hosts: Laura Bell, NPUK Clinical Nurse Specialist, Niemann Pick Diseases and Katie Reynolds, NPUK Volunteer
- 21:00-21:30** **Cherry: Activity Programme Volunteers' Briefing**

SATURDAY 21ST SEPTEMBER 2019

- 8:30-09:00** **Olive 1: Registration**
- 09:15** **Coaches depart for Whipsnade Zoo - We leave promptly!**
Be ready for a Fun Packed Day!
A packed lunch will be provided
- Please ensure you wear sensible shoes or trainers**

WHAT TO BRING WITH YOU:

- Waterproof Coat / Sunscreen
- Any Medication
- Any other emergency essentials
- Spending money up to a maximum of £5 in a named envelope

17:15	Children and Young Persons' Activity Programme Closes
18:30	Join us in the Cedar Lounge and take time to write a message for our wish tree
18:45	Dinner: The Rosewood Suite Check the seating plan for your table name
20:30-11:00	Entertainment: Disco by MKFM

SUNDAY 22ND SEPTEMBER 2019

08:45-09:15	Ash 1: Registration Ash 1 will also be available throughout the morning as a 'chill out' room!
09:30-11:00	Movie Time in the Yew Technology Theatre!
09:30-11:00	Crazy Golf Competition and Table Tennis Tournament in the Cedar Courtyard
11:00-13:00	Cherry: NPUK Youth Council The NPUK Youth Council provides an opportunity for younger members of our community (aged 11+) to be involved in the work of NPUK, by providing their views and ideas about the activities and services we provide now and contributing to the design of future resources and services.
11:00-13:00	Beech 1 and 2 Crafty Monkey Ceramics Workshop
13:00	Children & Young Persons' Activity Programme CLOSSES Lunch: The Olive Restaurant



Thank you to our Speakers:

We are incredibly thankful to each and every one of our speakers over the course of both the Annual Family Conference and the Interactive Workshop on Niemann-Pick Disease, your contribution to this weekend and your ongoing support as an advocate to those in the NPUK community is much appreciated.

Chris Andrews

Chris Andrews is an attorney at Amherst Holdings and philanthropist by day and an avid medical researcher and citizen scientist by night.

Chris is a Co-founder and Director of the Firefly Fund; most importantly, he is the proud father of Belle & Abby Andrews, who are affected by NP-C 1.



Dr David Begley

David J. Begley PhD has recently retired as Senior Lecturer in Physiology at Kings College London. He continues to work with the laboratory within in the Institute of Pharmaceutical Sciences at Kings College investigating the blood-brain barrier and drug delivery to the CNS with a special emphasis on lysosomal storage diseases.



Elizabeth Berry-Kravis MD PhD

Elizabeth Berry-Kravis MD, PhD is a Professor of Pediatrics, Neurological Sciences, and Biochemistry at Rush University Medical Center in Chicago. She has run an NPC program since 2013, been Co-PI of the Vtesse phase 2/3 registration trial of VTS-270 (hydroxypropyl-beta cyclodextrin) for NPC and has been PI of an investigator-initiated expanded access program for VTS-270 through which 42 patients with NPC have been treated at 20+ sites in the USA.



Shaun Bolton

Shaun Bolton is a Clinical Research Coordinator based at the Queen Elizabeth Hospital Birmingham who is working with the International Niemann-Pick Disease Registry team. Shaun has worked in the NHS since 2013 and through his experiences and interactions with NPUK has an appreciation of the value a patient-centric registry can provide to the Niemann-Pick Disease community. Shaun helps to support the INPDR as it moves into the future.



Allie Colaço

Allie completed her PhD on Niemann-Pick type C disease: Pathogenesis and Therapy in 2016 in Fran Platt's lab at University of Oxford. After spending two years working on lysosomes in cancer in Copenhagen, Allie is now back in the Platt lab as a postdoctoral researcher where she is focusing on therapeutic targets for NPC1 disease.





Fran works in the Pharmacology Department at the University of Oxford. Her laboratory is interested in understanding and treating lysosomal storage diseases. A major current focus of the lab is in NPC disease with ongoing studies on the pathogenic cascade, mechanisms of immune activation, biomarker identification and trialling therapies in a mouse model of this disorder.

Professor Frances Platt has kindly hosted the Interactive Workshop on Niemann-Pick Diseases for ten years.

Sandra Cowie

Sandra is an adult with ASMD Niemann-Pick Disease Type B who serves as a Director of Niemann-Pick Canada and was recently elected as President of the International Niemann-Pick Disease Alliance at the INPDA "Face to Face" Meeting 2019 in Lyon, France. Sandra currently lives and works in Toronto, Ontario, Canada.



Emily Eden

Dr Eden Senior Research Fellow at UCL Institute of Ophthalmology; her current research surrounds defects in lipid transport resulting in lipid accumulation in lysosomes that is associated with several neuro-degenerative diseases, including Niemann Pick type-C (NPC).



Taylor Fields

Taylor Fields is Vice President of External Affairs at IntraBio Inc., a global leader in developing therapies and treatments for rare and common neuro-degenerative diseases, including lysosomal storage disorders. IntraBio's lead drug candidate was recently granted Orphan Drug Designation by the European Medicines Agency (EMA) for the treatment of Niemann-Pick Disease Types A, B & C.



Bethanie Garside MSc

Bethanie is a Research Technician, Lipid Research Group at the University of Manchester. She is a recipient of the 2019 Peter Carlton Jones Memorial Award, which is given annually by NPUK in response to the submission of a 'research project' (not a literature review) which provides an original contribution to the scientific or public understanding of the Niemann-Pick diseases and/or their treatment or cure.



Prof. Paul Gissen

Prof. Gissen got his PhD in Human Molecular Genetics from the University of Birmingham investigating genetic causes of rare paediatric diseases when he developed specific research interest in intracellular trafficking disorders. Paul is currently a Consultant in Paediatric Inherited Metabolic Diseases at Great Ormond Street Hospital, London and a group leader at the Laboratory for Molecular Cell Biology, University College London.



Josie Godfrey

Josie is the founding Director of JG Zebra Consulting, which provides independent consultancy services with a focus on market access strategy, government and public affairs and patient and stakeholder engagement. Her current projects include leading Duchenne UK's Hercules project, and international multi-stakeholder collaboration to develop support tools for Health Technology Assessments for new treatments for Duchenne Muscular Dystrophy.



Christina Guldberg

Christina Guldberg is Director Clinical Outcomes at Orphazyme A/S and is responsible for the validation and standardized implementation of outcomes measures in clinical trials. Christina has more than 30 years of experience in the pharmaceutical industry working within clinical development.



Dr Wendy Heywood

Dr Heywood is a senior research fellow at the UCL Institute of Child Health. She has research interests in various diseases but work mostly in the area of lysosomal storage disorders. She also co-manages the UCL Biological Mass Spectrometry centre that provides routine diagnostic assays to great Ormond Street Hospital and Industry.



Dr Sharon Hrynkow

Dr. Sharon H. Hrynkow is Senior Vice President for Medical Affairs at CTD Holdings, Inc., a Florida-based biotechnology company working to develop cyclodextrin-based drugs for the treatment of Niemann-Pick Type C and other indications. At CTD, Dr. Hrynkow leads the clinical and scientific programs. She is Co-Chair of CTD's Scientific Advisory Board and Co-Chair of CTD's Family and Physician Listening Circle.



Michael Hughes

He completed his PhD in Dr. Ahad Rahim's lab at the UCL School of Pharmacy investigating the development of novel viral vectors for the use of gene therapy to treat Niemann-Pick Type C. Michael continues the work on developing a gene therapy approach for Niemann-Pick Type C as an MRC funded Postdoctoral Research Associate in the lab of Dr. Ahad Rahim.



Jacqueline Imrie

Jackie is a Trustee of NPUK and obtained a Degree in Biochemistry, Genetics and MSc Genetic Counselling after being a paediatric nurse. She worked at the Willink Metabolic Unit, Manchester seeing patients with many inborn errors of metabolism. In 1999, funded by NPUK, Jackie became the first support nurse for families with Niemann-Pick disease. She currently serves as a consultant for the International Niemann-Pick Disease Registry (INPDR).



Thomas Kirkegaard

Thomas founded Orphazyme A/S, in 2009, where he currently holds the position of Chief Scientific Officer. Orphazyme develop Heat Shock Protein based therapies for orphan diseases, with a particular focus on lysosomal storage diseases and neuromuscular diseases and are currently in late stage clinical trials in four rare diseases, Niemann-Pick type C, Gaucher's disease, sporadic Inclusion Body Myositis (sIBM) and Amyotrophic Lateral Sclerosis (ALS).



Dr Gauri Krishna

Dr Krishna is Clinical Research Fellow at the National Hospital for Neurology and Neurosurgery in London. From 2011 to 2014 Gauri served as a Doctor at Cloudnine Healthcare Facility, specialising in inpatient care for newborns, outpatient services in infant, child and adolescent healthcare, and with a special interest in rare diseases.



Dr. Dolores (Lola) Ledesma

Dr Ledesma's scientific career in neuroscience started during her PhD at Autonomia University of Madrid (1992-1995), studying the posttranslational alterations of Tau protein in Alzheimer's Disease. Dr Ledesma currently leads a team focussing on the contribution of cholesterol and sphingolipids to neuronal physiology and on the pathological consequences of their alterations in lipid storage disorders such as Niemann-Pick Disease.



Enchi Liu

Enchi Liu is the Head of Clinical Development and Translational Biomarkers at E-Scape Bio, a Bay Area (California, USA) biotechnology company focused on developing therapies for genetically defined neurodegenerative diseases such as Niemann Pick disease type C and LRRK2 Parkinson's disease. Enchi has 20 years of experience in drug development in the pharmaceutical industry.



Dr. Emyr Lloyd-Evans

Dr Lloyd-Evans is Research Group Leader and Senior Lecturer at Cardiff University. His interest in lysosomes and lysosomal storage diseases started during his first PTY placement at the Weizmann Institute where he worked on the role of Ca²⁺ dyshomeostasis in Gaucher disease. Since then he has worked extensively on Niemann-Pick disease type C and more recently the Neuronal Ceroid Lipofuscinoses.



William (Bill) Owen

Bill is a retired Chartered Electrical Engineer and a trustee of Niemann-Pick UK, with an interest in scientific and therapeutic developments for rare diseases. Bill is acutely aware of the challenges facing families in relation to the disease as his daughter was affected by Niemann-Pick type C and sadly died in August 2005, aged 30. Bill's interest in the biosciences relates to his desire to see rare diseases given greater prominence in research.



Gregory Pastores

Dr Pastores is a Consultant with the Adult Metabolic Service, at the Mater Misericordiae University Hospital, Dublin. He has extensive clinical and research experience in the diagnosis and management of patients with the lysosomal storage disorders and other inborn errors of metabolism. He has published over 200 papers, 20 book chapters and two textbooks.



Professor Marc C. Patterson

Professor Patterson's research has focused on neurometabolic disorders, with special interests in Niemann-Pick disease, Type C, Gaucher disease and Congenital Disorders of Glycosylation, areas in which he has published and spoken widely. His awards include a Fulbright Award, The Lewis P. Rowland Teaching Award at Columbia University, and several named professorships. Professor Patterson is strongly committed to care and advocacy for children and families with neurologic disabilities



Dr. Ahad Rahim

Ahad has worked on gene therapy and the development of gene transfer vectors for 10 years while working at the Institute of Cancer Research and more recently University College London in the laboratories of Professor Adrian Thrasher and Dr Simon Waddington. Ahad's laboratory at UCL focuses on the development of gene therapy strategies for a range of intractable and ultimately lethal neurological disorders.



Dr. Uma Ramaswami

Dr Ramaswami is involved in transition services for young people with lysosomal disorders (LSDs). She leads a family clinic at the Royal Free Hospital, which is a one stop service for parents and their children with Fabry disease. Uma's research relates to LSDs in children and young adults and she also has an interest in paediatric lipid disorders.



Joseph Roney

Joseph Roney received a BSc in Microbiology and Cell Science from the University of Florida in 2012 and is currently a DPhil student in the NIH OxCam program. He is co-supervised by Prof. Fran Platt at the University of Oxford and Dr. Zu-Hang Sheng at the NIH. His dissertation research focuses on the axonal transport of endolysosomal-autophagic organelles and its relevance to axonal homeostasis and neurodegeneration in NPC.



Dr. Reena Sharma

Dr Reena Sharma joined the department of Adult Inherited Metabolic disorders at Salford Royal Hospital in 2011 after completing her speciality training in metabolic medicine in the Northwest deanery. The metabolic medicine department at Salford Royal provides adult metabolic service for the North of England and is also centre for treatment and management of patients with lysosomal storage diseases.



Dr Dan Sillence

Dr Sillence is a Reader in Cell Biology, Leicester School of Pharmacy at De Montfort University, Leicester UK. Dan was awarded the Peter Carlton Jones Memorial Award (2006) for new therapy for Niemann-Pick C disease with Emyr Lloyd-Evans and Prof Frances Platt, University of Oxford.



Tony Somers

Tony Somers is a Professional Counsellor, Life Coach, Trainer and Self Defence Expert. In addition he is a trained Fire Fighter and a 6th Dan Martial Artist. Tony set up Anthony Somers.com in 2007 having spent the previous 17 years as a trained fire fighter working for the West Midlands fire service. Tony is respected by his clients and has built an enviable track record as an accomplished counsellor/coach and self-defence professional.



Dr Marie Vanier MD

Dr. Marie T. Vanier (MD, PhD) received her university degrees and medical training in Lyon, France and Göteborg, Sweden. Alongside her position as Director of Research at the French National Institute of Health and Medical Research (INSERM) (currently emeritus), she was from 1975 till 2009 the Head of a University Hospital laboratory offering diagnostic services (biochemistry and molecular genetics) for neurolipidoses. Her laboratory was one of the earliest in Europe to offer enzymatic assays for Niemann-Pick and Krabbe diseases, and the first to develop Niemann-Pick C testing.



Dr Susan Van Meter MD

Dr Susan Van Meter is Senior Director, Clinical Research, Autoimmune and Rare Diseases Business at Mallinckrodt Pharmaceuticals. Between 1998 to 2003 Susan served as Director on the Mood Disorders Program at Duke University Medical Center, where she developed and lead a multidisciplinary team to provide comprehensive evaluations for outpatients with mood disorders. Additionally, her team provided treatment in a multidisciplinary setting for outpatients with mood disorders.



Dr. Melissa Wasserstein

Dr. Melissa Wasserstein is the Chief of the Division of Pediatric Genetic Medicine at the Children's Hospital at Montefiore and an Associate Professor of Pediatric and Genetics at the Albert Einstein College of Medicine (AECOM). Dr. Wasserstein has published more than 70 articles and chapters.

Melissa is a clinical investigator whose research focuses on evaluating the natural history of Niemann Pick A/B, and has served as Principal Investigator on many clinical trials evaluating novel treatments for rare disease.



Thank you to our video Speakers:

We also want to take the opportunity to thank those who were not able to attend the 26th Annual Family Conference & 10th Interactive Workshop on Niemann-Pick Diseases in person, but still contributed via an informative video presentation.

Andrew Munkacsi PhD

Dr Andrew Munkacsi is Senior Lecturer in the School of Biological Sciences and Director of the Chemical Genetics Laboratory at Victoria University of Wellington. A major focus of his research is Niemann-Pick type C disease, his team uses a suite of genomic, proteomic and lipidomic analyses to identify modifiers of cholesterol and sphingolipid accumulation that are thus candidate targets to modify the onset and progression of Niemann-Pick type C disease.



Felicity Munro Bexspsci (Hons)

Felicity has over 15 years of experience in the sport, health, fitness industries starting early in injury management and prevention as massage therapist and sports trainer. All of her experiences have melded together to create Future Directions Personal Wellbeing and Fitness - a gym and personal training studio in Benalla, North East Victoria. Future Directions Fitness operates with everyone regardless of ability or age, to live their best lives possible.



Sean Van Velsen, AEP MCEP

Sean is an Accredited Exercise Physiologist (AEP) and is cousin to Matthew and Tim Lloyd, who are sufferers of NP-C and as a result he has seen the impact of the disease firsthand. Sean is humbled at the opportunity to present at the NPUK Annual Family Conference & Interactive Workshop and contribute towards the betterment of the treatments available to NP-C sufferers everywhere.



Save the Date!

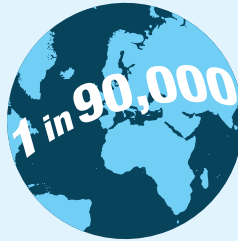
As an advocate of **Niemann-Pick UK (NPUK)** and the wider Niemann-Pick community, we would like to cordially invite you to next year's event, the **27th Annual Family Conference & 11th Interactive Workshop on Niemann-Pick Diseases**, which will be held on the **25th-27th September 2020**. Location...watch this space.

We hope you enjoyed this weekend and found it to be a positive and informative experience - at NPUK we take your feedback seriously, as it helps us prepare and plan future events. Please take the time to fill out a Conference Feedback Form, available on our website, npuk.org. Thank you!

NIEMANN-PICK TYPE C DISEASE (NP-C) IS A

NG RARE PROGRESSIVE IRREVERSIBLE CHRONICLY DEBILITATING LYSOSOMAL STORAGE DISEASE¹⁻³

NP-C affects all ages¹



Incidence of NP-C is
1 in 90,000 live births⁴

Likely an underestimate due
to lack of clinical awareness¹

NP-C takes on average
5 YEARS to diagnose⁵

That's...

**1,826
DAYS**

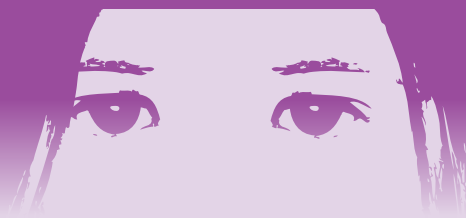
**260
WEEKS**



**43,824
HOURS**

...waiting for an answer,
watching a loved one
get worse

Have you checked for
eye movement
abnormalities?



Vertical supranuclear gaze
palsy (VSGP) is present in
virtually all patients^{1,3}

**THINK AGAIN
THINK NP-C**

THINK AGAIN. THINK NP-C aims to support
healthcare professionals unfamiliar with NP-C
to recognise the key signs and symptoms of
NP-C and reduce the time to diagnosis

Individual symptoms are non-specific to the disease^{1,3}

If you are a: **Paediatrician**

LOOK FOR **ATAXIA, DEVELOPMENTAL DELAY,
HEPATOSPLENOMEGALY**

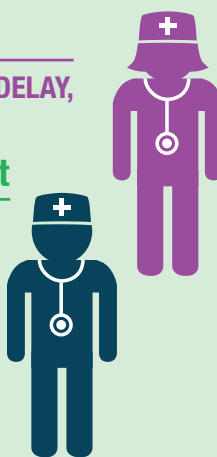


Paediatric hepatologist/neonatologist

LOOK FOR **HEPATO/SPLENOMEGALY,
NEONATAL CHOLESTATIC JAUNDICE,
NEONATAL LIVER DYSFUNCTION**

Adult neurologist/psychiatrist

LOOK FOR **COGNITIVE DECLINE,
ORGANIC PSYCHOSIS, PROGRESSIVE ATAXIA**



References

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3. Wijburg FA, Sedel F, Pineda M, et al. Development of a suspicion index to aid diagnosis of Niemann-Pick disease type C. *Neurology* 2012;78(20):1560–1567.
4. Wassif C, Cross J, Iben J et al. High incidence of unrecognized visceral/neurological late-onset Niemann-Pick disease, type C1, predicted by analysis of massively parallel sequencing data sets. *Genet Med* 2016; 18(1): 41–48.
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“PROGRESS TOGETHER”

International Niemann-Pick Disease Alliance

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go make memories!

"Go Make Memories" is the latest NPUK short film which we hope will help to raise further awareness of Niemann-Pick disease and shine a light on the daily lives of the NPUK Community.

The film was put into development after collaborative conversations with Niemann-Pick patients, NPUK families, and individuals from INPDA member groups, in an attempt to create a narrative which better reflects the experience of those affected by Niemann-Pick disease.

Created by Director & Producer Carl Mason (IMAGINE) and NPUK/INPDA Communications Officer John Lee Taggart, the film will be different from other awareness films you may have watched as it uses a mixture of both live action and animation to better portray the devastating effects of Niemann-Pick and the impact of this deterioration on both the individual and the wider family...

We will première the film this weekend (times below), and are proud to announce it is set to feature at DISORDER: The Rare Disease Film Festival in San Francisco (November 9th-10th). You can view the film at the NPUK Facebook page @NiemannPickUK and the YouTube channel, CineBites, after the last screening at 18:00.

SCREENINGS, 21ST SEPTEMBER
Yew Technology Theatre
13:15-13:30 / 15:30-15:45 / 17:45-18:00



Huge thanks to our Sponsors

Our Annual Family Conference & Interactive Workshop on Niemann-Pick Diseases would not be possible without the generous support of our sponsors - many thanks to each and every sponsor, it is truly appreciated.

The Big Lottery Community Fund has enabled us to expand our community reach thanks to their grant support with the "Shaping Our Future Together" initiative.



BBC Children in Need continue to provide essential grant support to NPUK, enabling access to the vital care and support offered by NPUK Clinical Nurse Specialist, Laura Bell.



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.



The Hollie Foundation has once again provided a grant in support of this year's Children & Young Persons' Activity Programme, and continue to part-fund the essential role of our Senior Families Advocate.



Mallinckrodt 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.



Orphazyme 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.



Sanofi Genzyme 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.



10th Interactive Workshop on Niemann-Pick Diseases
& 26th Annual Family Conference



#NPUKConf19 #NPUKFamily

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