





Thursday, 1st June 2023 1.00 PM - 5.00 PM

Hosted by the Australian NPC Disease Foundation Learn more at npcd.org.au

REGISTRATION FROM 12.40PM

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Opening Remarks 1pm start

Clinical Updates from 1.10pm 1.5 hours

- Conference Opening and Welcome to Country by Felicity Munro
- National Clinical Guidelines project update by Phillipa Johnston, ANPDF
- Providing Comprehensive Care for Metabolic Patients by Sharmila Kiss, Royal Children's Hospital, VIC
- INPDR Update by Jackie Imrie, INPDR
- Understanding Clinical Trials and Dispelling Myths by Tina Soulis, Alithia Life Sciences

AFTERNOON TEA

Trial Updates
from 3.30pm
2 hours

Clinical & Clinical

- N-acetyl-L-leucine (IB1001) for Niemann-Pick disease type C: Development Overview & Updates by Taylor Fields, IntraBio
- Intravenous Trappsol® Cyclo ™ New horizons: Update on the Pivotal TransportNPC International Clinical Trial by Caroline Hastings, Cyclo Therapeutics
- **Presentation** by Mark Walterfang, Royal Melbourne Hospital
- Azafaros' introduction to the Australian NPC patient community by Laetitia Crapard/Noémie Hurst, Azafaros

DAY ONE CLOSING

Aust. NPC Disease Foundation Inc. www.npcd.org.au info@npcd.org.au



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National Disability Conference Intiative Grant funded by the Australian Government Department of Social Services.







Friday, 2nd June 2023 9.00 AM - 5.00 PM

Hosted by the Australian NPC Disease Foundation

Learn more at npcd.org.au

REGISTRATION FROM 8.40AM

Opening
Remarks &
Research
Updates

9am start 1.5 hours

- Conference Opening and Aust. NPC Disease Foundation Updates by Mandy Whitechurch and Deanna Carpino
- A combination of human and yeast genomics approaches for discovering genetic modifiers of Niemann-Pick C severities and response to treatments by Andres Klein
- The development of gene therapy for Niemann-Pick type C disease by Ahad Rahim
- Optimization and important considerations of gene therapy for Niemann-Pick type C1 disease using a murine model by Cristin Davidson

Research •

Updates from 11am

1.5 hours

MORNING TEA

- Pre-clinical development of a new substrate reduction strategy to treat lysosomal storage disorders by Tony Cook
- mRNA Gene Therapy: A Potential New Treatment Option for Niemann-Pick Disease Type C1 by Ya Hui Hung, Liouba Le Roux & Annmarie Tripodi

Patient Support from 1.30pm

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LUNCH

- NDIS and NP-C by Carly Heath
- Power of Attorneys Types and why they are used by Dickson Ting
- The P Word: Dispelling myths in paediatric palliative care by Molly Williams and Sonya Camilleri

AFTERNOON TEA

Patient
Support
from 3.30pm
1.5 hours

- Childhood Dementia An Introduction by Gail Hlilton
- Childhood Dementia Research by Kris Elvidge
- Does movement matter? Exploring the therapeutic effects of exercise and physical activity by Felicity Munro

FINAL DAY CLOSING

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