



The INPDR Expands to a New Continent with Site Launch in Melbourne, Australia

15 October 2024 – Tyne & Wear, England.

The International Niemann-Pick Disease Registry (INPDR) is pleased to announce its latest partnership as The Royal Melbourne Hospital in Melbourne, Australia, joins the Registry. This milestone marks the first INPDR site on the Australian continent, further expanding the global reach of our efforts to improve the understanding, diagnosis, and treatment of Niemann-Pick diseases. By establishing our presence in Australia, we are not only expanding our reach but also giving the opportunity to the Niemann-Pick community in Australia to share their experience and contribute to the global understanding of Niemann-Pick diseases.

Importantly, the INPDR belongs to the patients, families and clinicians of Australia. Together, we will work to expand the Registry throughout Australia, fostering collaboration and inclusion, and ensuring that the data collected serves to enhance research, care, and understanding of Niemann-Pick Diseases both locally and globally.

Mandy Whitechurch, founder and president of The Australian NPC Disease Foundation (ANPDF), shared: “I am so proud and thrilled to see the Royal Melbourne Hospital as our first Australian INPDR registered site. Heartfelt thanks to Professor Mark Walterfang and Courtney for all the efforts put in to get this established. So, what's left to do....register...go online at www.inpdr.org and get your loved one's details registered so we can all work together from around the world, to find a cure or at the very least, a better treatment option for those living with Niemann Pick disease.”

INPDR’s Chief Executive Officer, Conan Donnelly stated: “We are deeply grateful to Professor Walterfang, Courtney Harris and their colleagues at The Royal Melbourne Hospital for their dedication and commitment in establishing the INPDR in Australia and we look forward to supporting Professor Walterfang with his research to make a positive impact on Niemann-Pick disease research and care in Australia and beyond.”

“The inclusion of Australia in the INPDR represents another major step towards realizing our ambition to enable patients, clinicians and researchers from around the world to understand more about these rare diseases. This will further empower the whole community to share understanding of both treatment and care of Niemann Pick type C and ASMD Niemann Pick. My congratulations to all who are working so hard to bring us all together” shared Jim Green, INPDR Chairman.

Notes to Editors

About the Australian NPC Disease Foundation

The Australian NPC Disease Foundation (ANPDF) aims to give hope, support and connection to those affected by Niemann-Pick disease type C (NPC) and Acid Sphingomyelinase Deficiency (ASMD) in Australia. We provide the opportunities through conversations, events and our disease-specific conference held in Victoria with an underlying focus on research for better treatments.

Founder and current President Mandy Whitechurch was determined to bring hope to Australia for her two sons, Matthew and Timmy. ANPDF provides practical information, financial and emotional support to both patients and families who choose to reach out.

Learn more about ANPDF here: [Niemann Pick C | ANPDF \(npcd.org.au\)](https://npcd.org.au)

About the INPDR

The INPDR is a web-based disease-specific registry, collecting information about ASMD Niemann-Pick Disease (types A & B), and Niemann-Pick Disease Type C, via, an anonymised Clinician Reported Database (CRD) and a Patient Reported Database (PRD). The PRD enables patients to self-enrol online and to contribute their data through a series of questionnaires including disease impact, health economics and quality of life. The INPDR is actively supported by patients, clinicians, patient advocates and researchers from over 20 countries across five continents.

For more information, visit: www.inpdr.org.

About Niemann-Pick disease

Niemann-Pick Diseases are a group of rare, inherited disorders, affecting both children and adults. They happen because the body can't properly break down certain fats. These diseases are split into two distinct subgroups: Acid Sphingomyelinase Deficiency (ASMD) and Niemann-Pick Disease Type C (NPC).

Acid Sphingomyelinase Deficiency (ASMD) is an extremely rare, progressive genetic disease, previously known as Niemann-Pick Disease Type A (NPA), Type A/B and Type B (NPB). ASMD represents a spectrum of disease, caused by a lack of the enzyme acid sphingomyelinase and resulting in potentially life-limiting illness in children and young adults. NPC is a cellular lipid trafficking disorder, is a significantly life-limiting neurodegenerative disease that can present in different ways. NPC is progressive, with disabling neurological symptoms.

About the The Royal Melbourne Hospital

The Royal Melbourne Hospital (RMH) began in 1848 as Victoria's first public hospital, and today is one of the largest healthcare providers in Australia. The hospital provides a comprehensive range of specialist medical, surgical and mental health services, as well as rehabilitation, aged care, specialist clinics and community programs. It also delivers our services across multiple locations, in people's homes and in the community. The RMH is part of the Parkville Precinct.

Find out more here: [RMH Foundation](https://www.rmhfoundation.org.au).