

# Niemann Pick India Charitable Trust (NPICT)



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NiemannPick India Charitable Trust continues to work with a singular focus: ensuring that individuals and families affected by Niemann-Pick Disease (NPD) in India are seen, heard, and supported through meaningful access to diagnosis, care, and emerging therapies. Over the past few months, the organization has actively engaged patients, clinicians, researchers, and global partners to strengthen the rare disease ecosystem in India.

A significant milestone was the patient-focused mini symposium held on 16 October at AIIMS, New Delhi, organized to place patients and caregivers at the centre of discussions on Niemann-Pick Disease. Conducted under the guidance of Dr. Pankaj Hari, Head of Department of Paediatrics, and Dr. Neerja Gupta, Head Department of Genetics, the program brought together senior clinicians from leading hospitals across Delhi. Experts addressed diagnosis, disease progression, and current standards of care, while families were given a rare opportunity to ask questions and share lived experiences. Adding an international perspective, Dr. Robert Mark Patterson joined online, highlighting global best practices, the importance of early diagnosis, multidisciplinary care, and international collaboration.

Further strengthening clinician engagement, NiemannPick India organized a Niemann-Pick and Lysosomal Storage Disorders workshop in Kolkata on 20 November in collaboration with Institute of Health and family welfare Kolkata and Sanofi. This workshop brought together doctors from Kolkata and surrounding regions with the objective of improving early recognition, diagnostic pathways, and referral practices for NPD and other LSDs. For families, such capacity-building efforts are critical, as delayed diagnosis remains one of the biggest challenges in rare diseases in India.

On the scientific front, NiemannPick India also presented at the Tata Institute of Genetics Society (TIGS) meeting on 28 November, engaging the broader scientific and genetics community. This interaction highlighted patient-identified gaps, the need for India-specific research, and the importance of aligning scientific priorities with real-world patient needs.

Encouragingly, progress is also being made toward treatment access. Azafaros has identified four clinical trial sites in India for Niemann-Pick Disease Type C, located in Delhi, Mumbai, Jaipur, and Kochi. Enrollment for these NPC clinical trials is currently underway, marking an important step forward for Indian families who previously had little or no access to experimental therapies within the country. For patients, this represents not just participation in research, but renewed hope for timely and equitable access to potential treatments.

Through these initiatives, NiemannPick India brings together patients, clinicians, and researchers across India, while benefitting from the continued support and global collaboration of the International Niemann-Pick Alliance.

