Nordic NCL Conference 2025

Speaker biographies

Knut Røthe
Parent Advocate | Creative Voice for CLN3 Awareness

Knut Rötne is a devoted parent advocate. As the father of Axel, who lived with CLN3 disease, Knut brings a deep personal perspective to the conference, sharing reflections on love, loss, and the importance of community.
In 2010, Axel and Knut formed Cloudberry Cream and staged a rooftop concert at their Norwegian home—a moving tribute inspired by The Beatles. Six years later, they released their first original song, Life Is Just a Dream, inspired by a dream Axel had that his blindness was only a dream. It featured musicians from around the globe. These performances became a symbol of courage and creativity in the face of childhood dementia, and the song continues to resonate with families across the world.

Jonathan D. Cooper, PhD
Jonathan Cooper leads the Pediatric Storage Disorders Laboratory (PSDL) at Washington University in St. Louis, MO, USA.

Over the last 25 year's the PSDL has been characterizing effects of Batten disease upon the Central Nervous System, describing many of the key features of these diseases and testing experimental therapies to treat these effects. Increasingly, in patient led studies, the lab is also focusing upon the life-limiting effects of disease upon the bowel, the peripheral nervous system, and neuromuscular system. Jon works closely with Batten family associations in the US, Europe and further afield and will look forward to updating the Nordic NCL meeting with latest research developments in the field.

Ineka Whiteman, PhD
Head of Research & Medical Affairs BDSRA Australia & BDSRA Foundation;
Scientific Consultant, Beyond Batten Disease Foundation Australia

Ineka bridges science and advocacy, working closely with families, researchers, clinicians' and industry partners across continents. Her focus is on translating complex research into meaningful support and action. At the conference, Ineka will speak about the evolving global landscape of Batten disease clinical programs and research initiatives.

Angela Schulz, MD
Clinician Scientist Professor for rare pediatric diseases and childhood dementia at the University Medical Center Hamburg-Eppendor (UKE), Germany

As head of the Research Group for Childhood Dementia / NCL, Angela coordinates the international DEM-CHILD NCL patient database and is principal investigator in various clinical trials in the field of neurodegenerative lysosomal diseases. Her mission is to advance early diagnosis and combine clinical care with clinical and translational research to develop innovative therapies and improve care for children affected by childhood dementia.

Miriam Nickel, MD
Pediatrician and Palliative care Specialist (NCL-Specialist) Children's Hospital University Medical Centre Hamburg-Eppendorf (UKE), Germany

Miriam is a dedicated clinician working closely with children affected by rare neurodegenerative conditions. Her expertise lies in pediatric neurology and multidisciplinary care for children with NCL's. At the Nordic NCL Conference, Miriam will contribute clinical perspectives from her work at UKE.

Ingrid B. Helland, MD, PhD
Senior Consultant, Dept. of Clinical Neurosciences (Child Neurology) Oslo University Hospital – Rikshospitalet, Norway

Ingrid has long experience in child neurology and rare disease diagnostics. She leads national efforts in Norway to improve care pathways for children with complex neurological conditions. She is also engaged in raising knowledge about childhood dementia in Norway. At the conference, Ingrid will share insights from Norwegian initiatives, clinical practice, family engagement, and the importance of Nordic collaboration.

Niklas Darin, MD, PhD
Professor; Senior Consultant in Child Neurology Queen Silvia Children's Hospital, Sahlgrenska University Hospital, Gothenburg, Sweden

Niklas is a leading expert in pediatric neurology with a strong focus on inherited metabolic and neurodegenerative diseases. He combines clinical care with research and education, helping shape Sweden's approach to rare disease management. At the Nordic NCL Conference, Niklas will present updates on Swedish clinical initiatives and share strategies for supporting families across the region.

Mette Møller Handrup, MD, PhD
Clinical Associate Professor (Pediatrics); Consultant Centre for Rare Diseases, Aarhus University & Aarhus University Hospital, Denmark

Mette's clinical and research focus includes rare neurogenetic disorders, with expertise in CLN3 disease and autonomic dysfunction. Mette is a child neurologist and researcher with a deep commitment to improving care for children with rare diseases. She works across clinical and academic settings to strengthen diagnostic pathways and support systems for families. At the Nordic NCL Conference, Mette will share insights from Danish initiatives and highlight how national Centre's can collaborate across borders.

Tom Wishart, PhD
Professor of Molecular Anatomy, The Roslin Institute, University of Edinburgh, UK

Tom's research explores the molecular and cellular mechanisms underlying neurodegeneration, with a focus on translational approaches. He is known for his collaborative work across disciplines and his efforts to connect basic science with therapeutic development. At the conference, Tom will present recent findings from preclinical models and discuss how they inform future treatment strategies.

Sara E. Mole, PhD
Professor of Molecular Cell Biology University College London (UCL), Great Ormond Street Institute of Child Health, UK

Sara Mole is a leading expert in the molecular genetics and biology of Batten disease, with decades of experience uncovering the genetic and cellular mechanisms behind neuronal ceroid lipofuscinoses (NCLs). Her research has been instrumental in shaping global understanding of the genetics and disease pathways and informing potential therapeutic strategies.

At the Nordic NCL Conference, Professor Mole will present "Old and new perspectives on CLN3 disease", exploring how evolving insights into genetic mechanisms contribute to the latest understanding and future directions in treatment.

Yvonne Kruithof, MSc
Educational Psychologist (School for children with a Visual Impairment) Bartiméus, Zeist, Netherlands.
PhD on developing a support module for families of children with CLN3 disease/Batten disease.
VU, Amsterdam, Netherlands

Yvonne specializes in supporting children with visual impairments, including those affected by Batten disease, as well as their parents, siblings, and teachers. Her work focuses on educational strategies, psychological and emotional development, and family-centered approaches. At the Nordic NCL Conference, Yvonne will share insights and practical tools to help families and professionals in talking about a neurodegenerative, life-threatening disease in children

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Michelle L. Hastings, PhD
Dr. Michelle L. Hastings is the Pfizer Upjohn Research Professor of Pharmacology at the University of Michigan Medical School and co-Director of the Center for RNA Biomedicine, directing the Nucleic Acids Therapeutics Core USA

Michelle earned her Ph.D. from Marquette University and completed postdoctoral training at Cold Spring Harbor Laboratory. Her work focuses on RNA-based therapeutics, particularly antisense oligonucleotides (ASOs) that modulate splicing and gene expression to treat disease. Her lab has pioneered candidate therapies for Usher syndrome, Batten disease, cystic fibrosis, Alzheimer's, and Parkinson's disease and developed a personalized ASO medicine, Zebronkysen, that is being used to treat two children with CLN3 Batten disease caused by an ultra-rare variant in the gene.
Dr. Hastings holds many patents for her discoveries and serves on multiple editorial and advisory boards. She contributes extensive experience in RNA therapeutics, translational research, and clinical development to inform strategic decision-making.

Yael Shiloh-Malawsky, MD
Professor of Neurology; Director, Batten Disease Center of Excellence
UNC School of Medicine, Chapel Hill,
USA

Yael leads one of the few dedicated centers for Batten disease, combining clinical care, research, and family support. She specializes in neurogenetic disorders and the compassionate use of individualized therapies. She has established expertise in the clinical implementation of personalized ASO treatments. At the Nordic NCL Conference, Dr. Shiloh-Malawsky will share a one-year update of the clinical trial using Zebronkysen in two patients with CLN3 Batten disease, highlighting insights gained and implications for future ultra-personalized medications.

Michael Strupp, MD
Professor of Neurology & Clinical Neurophysiology LMU Munich, Dept. of Neurology (Vertigo & Balance Center), Germany

Michael is internationally recognized for his work on balance disorders and neurophysiology. His research spans clinical care, diagnostics, and therapeutic innovation, with relevance to neurodegenerative conditions like Batten disease. At the Nordic NCL Conference, Michael will share insights into vestibular dysfunction and its impact on quality of life in pediatric patients.

Diego L. Medina, PhD
Associate Professor of Biology; Investigator & HCS Facility Head University of Naples "Federico II" & Telethon Institute (TIGEM), Pozzuoli, Italy

Diego's research focuses on cellular pathways and lysosomal functions, with direct relevance to NCLs. At TIGEM, he leads efforts to uncover molecular mechanisms and identify therapeutic targets. At the conference, Diego will present recent findings from his lab and discuss how basic science can inform clinical strategies for Batten disease.

Heather R. Adams, PhD
Professor of Neurology and Pediatrics University of Rochester Medical Center (URMC), USA

Heather has been a member of the University of Rochester Batten Center (URBC) since 2003.
As a pediatric neuropsychologist, her research focuses on understanding cognitive symptoms and how they change over time, in NCLs and related conditions that cause childhood dementia. This includes examining the impact of cognitive impairment and interfering behaviors on affected individuals and families, how to best evaluate individuals with sensory and motor impairments and challenging behaviors, and outreach to rare families who are geographically dispersed around the globe. At the Nordic NCL Conference, Heather will present an update on recent research activities of the URBC.

Colin Reilly
Psychologist Queen Silvia Children's Hospital and Associate Professor at the University of Gothenburg, Sweden

Colin's research focusses on psychological aspects of rare neurological conditions in children. At the Nordic NCL Conference, Dr. Reilly will present "Psychosocial aspects of childhood dementia", offering insights into the impact of dementia on psychosocial functioning of affected children and their families.

Line Lindhardt Pedersen
Founder, Bellyfood,
Denmark

Line is a passionate advocate for nutrition and sensory-friendly food experiences, especially for children with special needs. Through Bellyfood, she develops real food tube feeding that gives sensory impressions, that helps the tubefed person to be included in the meal on equal terms with the rest of the family. At the conference, Line will offer practical perspectives on how food can support wellbeing and community — even in clinical settings.

Kathrin Meyer, PhD
Chief Scientific Officer & Head of Research & Development Assistant Professor, Department of Pediatrics, The Ohio State University, Neela Therapeutics, USA

Kathrin leads cutting-edge research in gene therapy and neurodegeneration, with a focus on translational science for pediatric diseases. Her work spans academia and biotech, driving innovation in treatment development. At the Nordic NCL Conference, Kathrin will present updates on therapeutic pipelines and discuss the path from lab to clinic.

Dr Vincent Blomen
Senior Director of Target Discovery Scenic Biotech, The Netherlands

Vincent leads the discovery Science at Scenic Biotech, an Amsterdam-based biotechnology company pioneering modifier therapy approaches. His work centers on identifying modifier genes as therapeutic targets for neurometabolic diseases. At the conference, Vincent will present Scenic's lead program focused on targeting the lysosomal hydrolase PLA2G15, highlighting its potential as a novel treatment strategy for lysosomal diseases, including CLN3 Batten.

Magnar Bjørås, PhD
Professor of Molecular Medicine Department of Clinical and Molecular Medicine (NTNU) & Department of Microbiology, Oslo University Hospital, Norway

Magnar Bjørås is a Professor of Clinical and Molecular Medicine at the Norwegian University of Science and Technology (NTNU) and serves as Principal Investigator at the University of Oslo and Oslo University Hospital. He is internationally recognized for his pioneering research in oxidative DNA damage repair, neurodegeneration, and cancer biology. Bjørås drives innovation at the intersection of molecular medicine, diagnostics, and translational research. He is the founder of three biotechnology companies: Lybe Scientific, Zenit Science, and Nocteva. Zenit Science functions as a contract research organization (CRO) specializing in preclinical testing of drug candidates using human patient-derived organoids. Nocteva is dedicated to developing and testing novel therapeutics for rare genetic diseases, including Childhood dementia CLN3.

Through these ventures, his research group translates fundamental discoveries into impactful clinical applications, reinforcing his commitment to advancing precision medicine. Magnar and his group will present data from their research on patient's derived CLN3 organoids.

Reetta Hinttala, PhD, Professor of Molecular Biology in Medicine Faculty of Medicine, University of Oulu,
Finland

Reetta is a molecular biologist with deep expertise in mitochondrial disorders and neurodegeneration. Her research focuses on the genetic and cellular mechanisms behind rare pediatric diseases, including neuronal ceroid lipofuscinoses (NCLs). She coordinates the Biocenter Oulu Transgenic Core Facility and the Finnish node of the European Mouse Mutant Archive (EMMA), supporting advanced animal models for biomedical research.

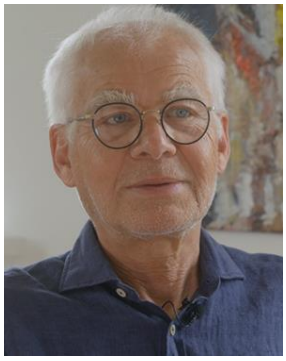
At the Nordic NCL Conference, Reetta will present findings from a novel CLN8 mouse model that recapitulates key hallmarks of Batten disease. Her work offers new insights into disease progression and opens promising avenues for therapeutic development.

Lilli Hinds, RN
Specialized Nurse, Helsinki University Hospital (HUS), Finland

Lilli works at Helsinki University Hospital, where she supports children and families affected by rare neurological conditions. Her clinical experience and compassionate approach make her a valued voice in the care of patients with Batten disease.
At the Nordic NCL Conference, Lilli will contribute to discussions on practical care strategies and share insights from the Finnish healthcare system.

Justiina Salmela Consulting Teacher Valteri Centre for Learning and Consulting, Finland

Justiina is a specialist in inclusive education and lifelong learning for children with complex needs. At the Valteri Centre, she supports schools and families across Finland in navigating educational challenges related to rare neurological conditions including Batten disease. She was a key contributor to the ERASMUS+ project "Juvenile Neuronal Ceroid Lipofuscinosis and Education," which focused on strengthening collaboration between health and education sectors. Justiina has started writing a doctoral dissertation in the field of education covering qualitative research of the role of Finnish schools in supporting the well-being of JNCL children and youth and their families.
At the Nordic NCL Conference, Justiina will share practical strategies for supporting children with NCL in school settings and highlight the importance of cross-disciplinary cooperation.



John R. Østergaard, DMSci Senior Consultant Department of Communication & Disability, Center for Rehabilitation, Aarhus, Denmark

John Østergaard is a pioneer in the field of rare pediatric neurodegenerative diseases. As former Head of the Center for Rare Diseases at Aarhus University Hospital, he has shaped diagnostic and care strategies for children with neuronal ceroid lipofuscinoses (NCLs) across Denmark and beyond. Now retired from clinical leadership, he continues to contribute as a senior consultant, focusing on children with complex intellectual disabilities in rehabilitation contexts.

At the Nordic NCL Conference, John will present "Acceptance of childhood dementia as a concept – a path paved with cobblestones…", reflecting on the challenges and breakthroughs in framing Batten disease within a broader understanding of cognitive decline in children. His advocacy for the term childhood dementia has helped families and professionals alike find language for a deeply complex experience.

Synnøve Algrøy Fjeldstad, PhD student in Magnar Bjørås group at the Norwegian University of Science and Technology (NTNU).

The group utilizes organoids for modeling rare genetic diseases and uses these models for drug testing. Fjeldstad has a background in chemistry and neuroscience. Her focus is on CLN3 disease, and AAV-based gene therapy testing on patient derived retinal and brain organoids.

María Cámara-Quílez, Ph.D. in Magnar Bjørås group at the Norwegian University of Science and Technology (NTNU).

Joined from the University of A Coruña, where she investigated DNA-repair–linked biomarkers for prostate and ovarian cancer. As a Margarita Salas postdoctoral fellow at NTNU, she studied hypoxia–ischemia with a focus on PARP3 and related DNA-repair pathways in the brain after stroke, followed by a postdoctoral position developing iPSC-derived organoid models of stroke to probe genetic determinants of injury and repair. She also contributes to a glioblastoma program and is an expert in immunohistochemistry, confocal imaging, and image analysis. Maria is heavily involved in the CLN3 project and, through affiliations with Zenit Science AS and Nocteva AS, she is the manager of the Zenit Science AS nuclei isolation team that supports single-nuclei workflows and advanced fluorescence microscopy for translational organoid research, including live imaging techniques.

Jørn-Ove Schjølberg, PhD candidate The Norwegian University of Science and Technology (NTNU), affiliated with Oslo University Hospital.

Jørn-Ove manages the iPSC laboratory in Trondheim within Magnar Bjørås' group, specializing in retinal organoids while also supervising brains and other organoid platforms. With an M.Sc. in Industrial Chemistry and Biotechnology from NTNU, Schjølberg leads the organoid arm of preclinical testing at Zenit Science AS and Nocteva AS, focusing on drug and gene-therapy evaluation in human patient-derived models.

Marie Sebille, MD PhD
Chief Medical Officer at THX Pharma

Marie is an MD by training and holds a PhD in Neurosciences. She has more than 30 years of experience in the pharmaceutical industry, where she has worked in various positions as a physician in Global Medical Affairs and Global Clinical Development. She has contributed to the clinical development of multiple psychotropic drugs in neurology and psychiatry. During her tenure, Marie has held executive positions managing different Clinical Operations Departments in Sanofi R&D. Her teams have supported all major registration files in diabetes & metabolism, I&I, Oncology and Rare Diseases, as well as Life Cycle Management programs. Marie has joined THX Pharma (Theranexus) in May 2021 as Chief Medical Officer to lead the Clinical Development Department to develop drugs in rare neurodegenerative diseases, including the CLN3 Batten disease.

Timo Tulisalo Parent Advocate.
Finland

Timo Tulisalo brings both personal experience and professional insight to the Nordic NCL conference. He will participate in the conference as the chair of the Finnish JNCL Parents' Support Association. The support association has joined forces with professionals to offer families the widest possible range of support.

Timo's professional career includes management positions in children and youth organizations and in the social and health sectors. He is also a member of the board of the Guarantee Foundation, which promotes social support and economic well-being in Finland.

In the session "Energy and support in everyday life with JNCL" Timo joins Justiina Salmela and Lilli Hinds to share experiences of collaboration between parents and professionals in providing comprehensive care and support—practical strategies, emotional perspectives, and reflections on what really supports families living with childhood dementia.

Mette Behnke, Conference Host
Founder of Nordic NCL | Advocate for Children with CLN3, Denmark

Mette Olin Behnke is the conference host for the Nordic NCL Conference and founder of Nordic NCL. As the mother of a child with CLN3 disease, she brings personal insight and commitment to supporting families affected by childhood dementia.

With a background in relationship management, Mette focusses on building connections across borders—linking families, researchers, Clinician's etc. in a shared mission of progress and hope. She is the main driver of the conference, ensuring that the program reflects both scientific advancement and the lived realities of families. Her work is grounded in empathy, precision, and a belief in the power of collaboration.

She also serves on the Knowledge and Development Committee of the Danish NCL Association.