



2025

ANNUAL RESEARCH REPORT

Department of Paediatrics and
Adolescent Medicine,
Copenhagen University Hospital -
Rigshospitalet



Rigshospitalet

*“Progress in paediatric healthcare
comes from connecting research,
clinical expertise, and families’ lived
experience”*

EDITORIAL COMMITTEE:

Nadja Hawwa Vissing, MD, Ph.D.

Caroline Sophie Kleis Schmidt, MD.

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Leadership Preface

Paediatrics is a unique medical specialty defined by age rather than by organ systems or disease categories. Spanning all medical subspecialties for children and adolescents aged 0–17 years, it requires a distinct child- and family-centred approach and must also address the specific legal and ethical considerations inherent to paediatric research. As this report demonstrates, these principles are firmly reflected in our research.

In 2025, research remained a central part of our department's work. Across clinical areas, staff contributed to the development of new knowledge through collaborations within the hospital, nationally, and internationally. In a field characterised by small patient populations, complex conditions, and demanding ethical and practical considerations, this depends on strong professional communities and an exceptional level of commitment from our staff.

We are very proud of what our staff continue to accomplish. The breadth, quality, and ambition of the research conducted across the department are remarkable and, in many ways, quite unique. Sustaining this level of activity within a highly specialised paediatric setting is a significant strength of the department and reflects the commitment, expertise, and collaborative spirit of our colleagues.

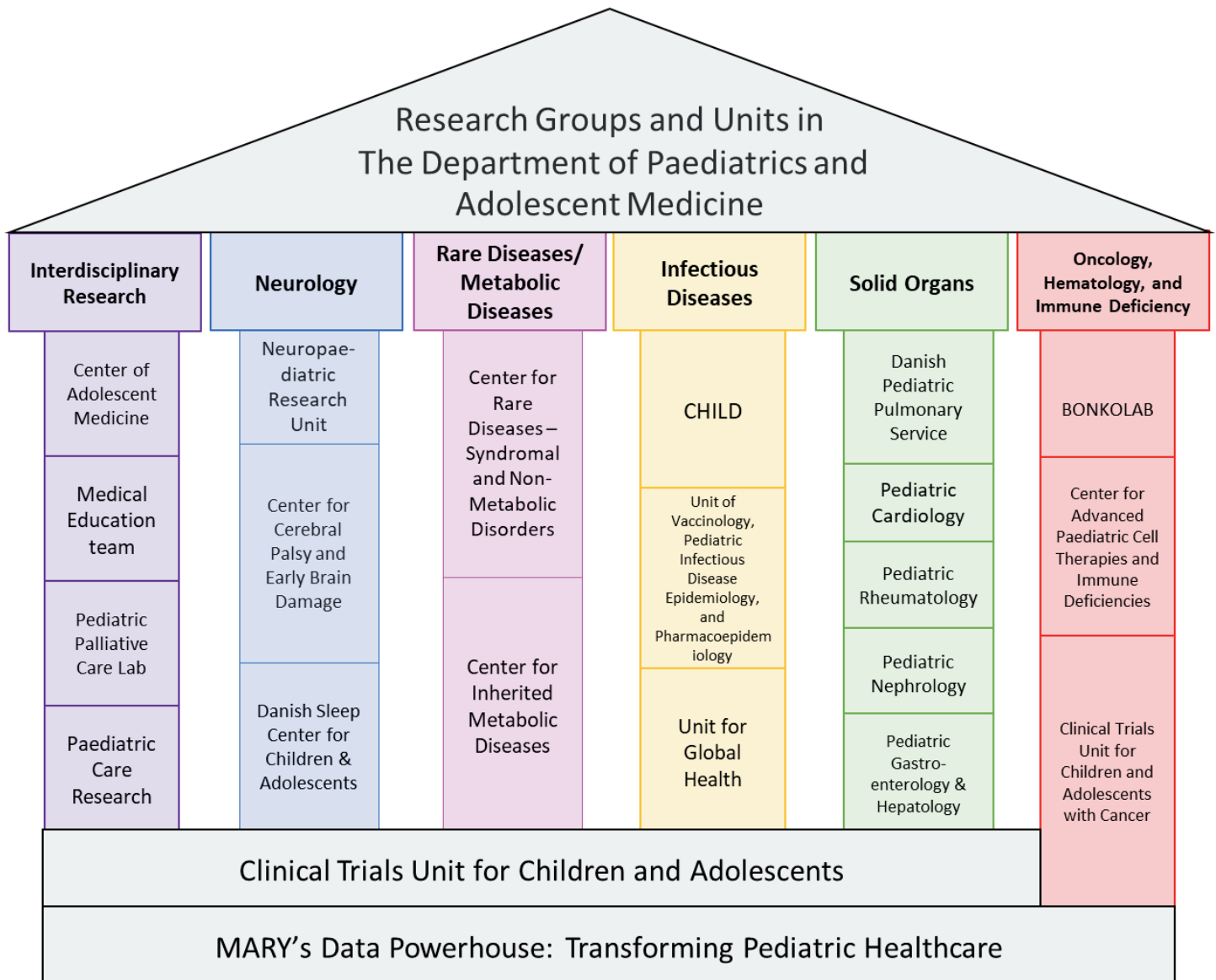
With this years report, we invite you to explore the many achievements and initiatives that reflect our continued commitment to improving paediatric care.

Nadja Hawwa Vissing, MD, PhD
Head of Research



Eva Mosfeldt Jeppesen, MD, DrMedSci
Head of Department

Overview

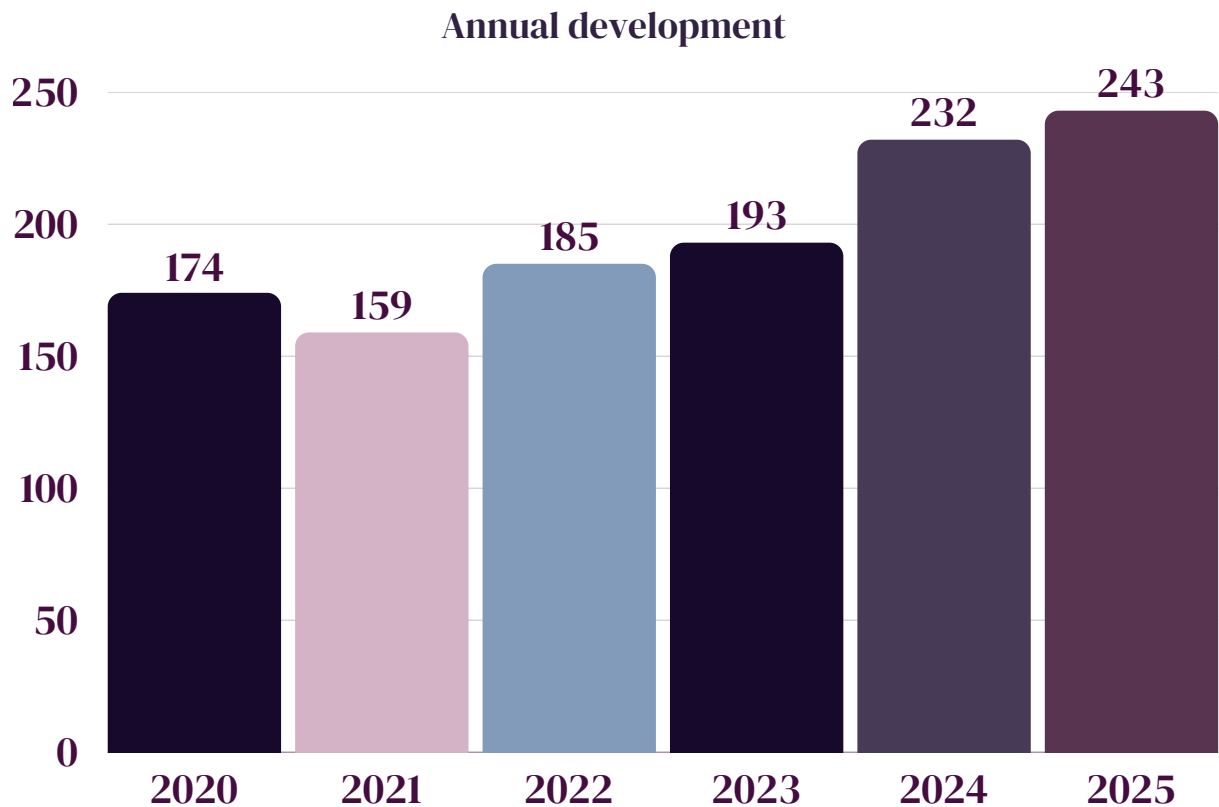


- 22 individual research groups
- 150 full-time equivalent employees in research

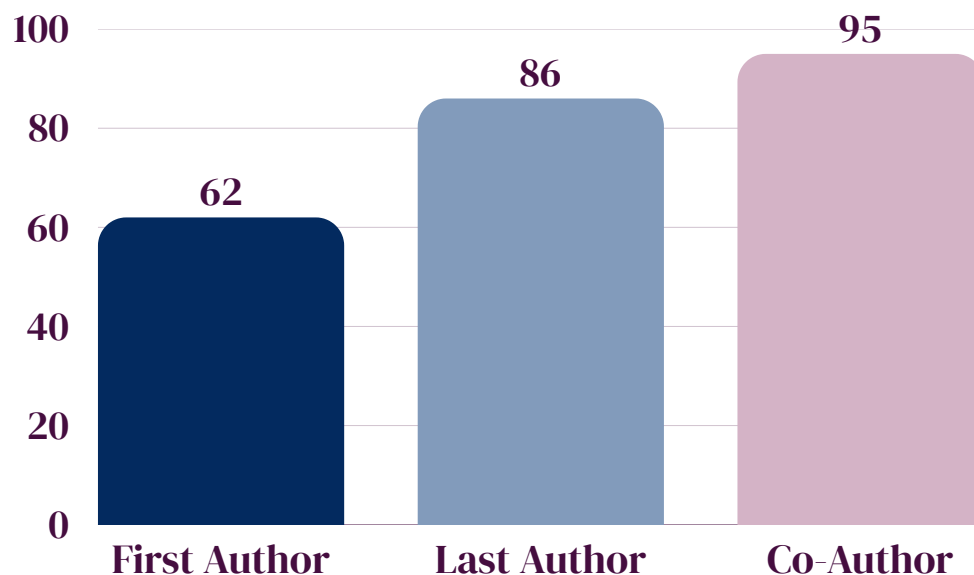
The Achievements



243 peer-reviewed scientific publications in 2025

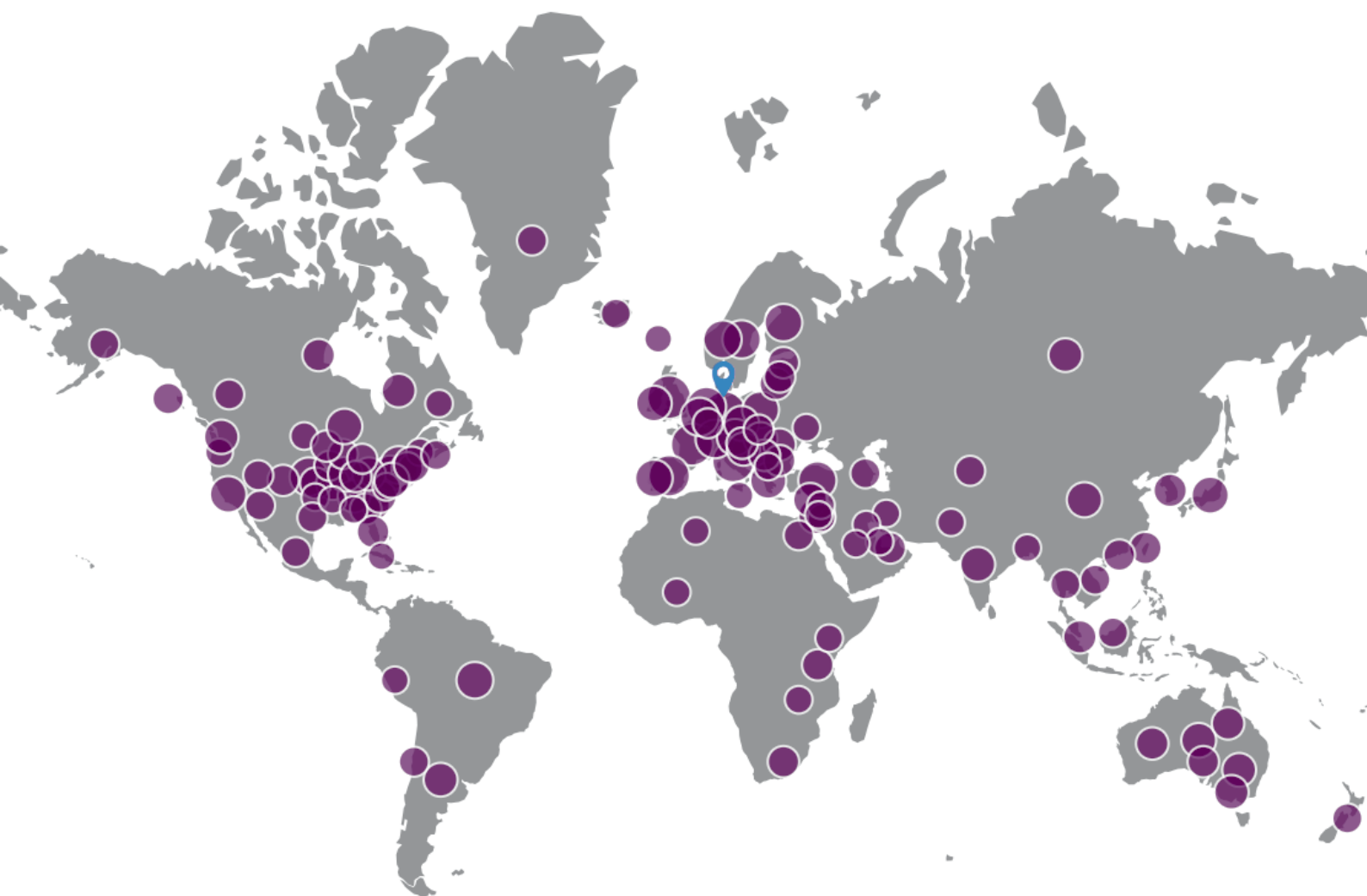


Authorships - 2025



International Partnerships

Collaboration during the past five years



Completed PhDs 2025

Total number: 18

Christine Højte Dahl (MD)

University of Copenhagen
Cystic fibrosis-related liver disease from childhood to adulthood: Insights into diagnosis, progression and the impact of ETI
Danish Pediatric Pulmonary Service

Esben Herborg Henriksen (MD)

University of Copenhagen
Trends in extrapulmonary complications in CF pre and post ETI
Danish Pediatric Pulmonary Service

Eva Rudjord Therkildsen (MD)

University of Copenhagen
From mouse to man: Is it possible to replicate the antispastic effects of the calcium channel blocker Nimodipine in humans?
Center for Cerebral Palsy and Early Brain Damage

Ivana Bardino Novosel (MSc in Physiotherapy)

University of Copenhagen
Development of brain plasticity-based home training for people with cerebral palsy focusing on transition from child to adult
Center for Cerebral Palsy and Early Brain Damage

Jane Lunding Larsen (MSc in Educational Psychology)

University of Copenhagen
Being a parent of an individual with Rett syndrome across different ages and phases of life
Center for Rett's syndrome

Lotte Smedegaard (MD)

University of Copenhagen
Infections in Children with Cancer - CHILD / BONKOLAB

Jesper Kiehn Sørensen (MD)

University of Copenhagen
Genetic associations with measles, mumps, and rubella vaccine response after MMR vaccination at 6 and 15 months of age.
Unit of Vaccinology, Paediatric Infectious Disease Epidemiology, and Pharmacoepidemiology

Jonas Rønne Kronborg (MD)

University of Copenhagen
Fluid management, electrolyte disturbances, acute kidney injury and outcome after congenital heart surgery in infants
Unit for Congenital Heart Disease

Julie Brix Bindslev (MD)

University of Copenhagen
Improving Diagnosis, Acute Treatment and Outcome of Pediatric Stroke
Center for Cerebral Palsy and Early Brain Damage

Julie Løye Hejl (MD)

Aarhus University
My Heart's Mind – Online assessment of mental health disorders and current treatment practices in young patients with a congenital heart disease
Unit for Congenital Heart Disease

Lene Thorup (MD)

University of Copenhagen
Lymphatic function and ventilation
Unit for Congenital Heart Disease

Louise Lindkvist Pedersen (Msc in Clinical Nutrition)

University of Copenhagen
Impact of nutrition on early toxicity and late effects in pediatric cancer treatment
Center for Advanced Pediatric Cell Therapies and Immunodeficiencies / BONKOLAB

Mathias G. Holgersen (MD)

University of Copenhagen
Genotype-phenotype associations in Primary Ciliary Dyskinesia - Lung Function, Mucociliary Clearance, and Pseudomonas aeruginosa Infections in a Danish Cohort
Danish Pediatric Pulmonary Service

Mia Aagaard Doherty (MD)

Aarhus University
Living with Neurofibromatosis Type 1: Cancer Risk, Mortality, and Survival Across the Lifespan
BONKOLAB

Minna Devantier (MSc Anthropology)

University of Copenhagen
Supporting siblings of children with cancer - an interdisciplinary PhD study on care, inclusion, and psychosocial intervention
BONKOLAB

Mona Nourbakhsh (MSc Quantitative Biology)

Technical University of Denmark
Discovery of cancer driver genes for precision medicine in childhood cancers
BONKOLAB

**Sejun Kim (MSc in
Bioinformatics)**

University of Copenhagen

Real-world evidence

approaches to investigating the

long-term safety of post-

marketed biological treatments

for psoriasis: Danish nationwide

register-based studies

Unit of Vaccinology, Paediatric

Infectious Disease

Epidemiology, and

Pharmacoepidemiology

Sonja Riis Meyer (MD)

Aarhus University

Late pulmonary adverse effects

in childhood and adolescent

acute lymphoblastic leukaemia

survivors: The ALL-STAR Lungs

Study

BONKOLAB / Danish Pediatric

Pulmonary Service



Completed Bachelor's & Master's Thesis 2025

Total number: 28

Adam Bøcker Olsen

Master's thesis

The Role of Altitude in Growth and Respiratory Health: A Cross-Sectional Study of Children in Urbanized Low-Altitude and Rural High-Altitude Settings in Kyrgyzstan

Agnes Sønderhave

Bachelor's thesis

Aetiology and antimicrobial resistance patterns in gram-negative neonatal sepsis in sub-Saharan Africa: a rise in resistance?

Amalie Christence Sennels

Master's thesis

The potential of Surfactant protein D as a predictive biomarker for the development of bronchiolitis obliterans after pediatric hematopoietic stem cell transplantation

Amalie Hartman Jacobsen

Master's thesis

Sequencing as part of the neonatal screening for CAH

Andrea Engmann Hildorf

Master's thesis

Malnutrition and spirituality

Anna Absalonsen

Bachelor's thesis

TMAO as a risk factor for atherosclerosis, assessed through a literature review to evaluate the risk associated with carnitine treatment in CTD patients and to propose a potential study of CTD patients in the Faroe Islands

Casper Noah Nilsson

Master's thesis

Lymphatic vascular function in shunted sheep

Cecilie Lindebjerg

Master's thesis

Growth and fibrosis in children with biliary : a retrospective cohort study

Cordelia Lindhardt Schmidt

Master's thesis

Deep phenotyping and genotype-phenotype correlations in patients with GABRB3 loss-of-function variants and epilepsy

Ellen Lawaetz

Bachelor's thesis

Influenza vaccination during pregnancy: Effectiveness against clinical influenza disease in the infant from birth to six months of age

Eva Malling Beck

Master's thesis

The COVID-19 pandemic had only mild health implications for children with cystic fibrosis (CF) followed at CF Center Copenhagen

Ida Handberg

Master's thesis

Altered plasma amino acids in children with acute liver failure

Karoline Grube

Master's thesis

Endothelial damage in paediatric allogeneic stem cell transplantation

Leonora Persson

Master's thesis

The validity of 'Diagnoses of concern' as an indicator of perinatal brain damage

Mariam El-Aoufi

Master's thesis

Nutrition as part of the perioperative care of neonates with congenital heart disease

Marianne Ragnhild Christensen

Master's thesis

Ocular graft-versus-host disease after hematopoietic stem cell transplantation: the significance of Interleukin-7 receptor polymorphisms

Marianne Steen Jensen

Master's thesis

Wound infections in 0-1 years olds after cardiac surgery. A cohort study

Marie Lykke Felby

Master's thesis

Multiple Breath Washout in Children with Esophageal Atresia and Tracheomalacia

Mathilde Primdahl

Bachelor's thesis

Early Detection of Cerebral Palsy in Danish Clinical Practice: A Survey on the Use of Diagnostic Tools and Associated Barriers

Maya Elena Ramirez Schambye

Master's thesis

Patients' Experiences of the Path to Sarcoma Diagnosis: A Qualitative Systematic Review and Thematic Synthesis

**Molly Skov Romdal**

Master's thesis

Exercise Capacity in Children with Esophageal Atresia and Tracheomalacia

Natalie Gelardi Lunde

Bachelor's thesis

MRI findings in children at risk of/with cerebral palsy - a systematic literature review

Nur-Kamila Kandela

Master's thesis

Health Seeking Behavior Among Caregivers of Children Under Five Hospitalized with Fever and/or Diarrhea: A Mixed Methods Study from Pemba Island, Tanzania

Olivia Christiansen

Bachelor's thesis

The Clinical Impact of Early Genetic Evaluation in Cerebral Palsy with Unknown Etiology

Sara Rahim Al-Hamesi

Bachelor's thesis

Incidence, Risk Factors, Neonatal Management, and Outcomes of Low Birth Weight Newborns delivered at a District Hospital in Pemba Island, Tanzania; A Prospective Cohort Study

Sarah Reckeweg Nygaard

Master's thesis

The genetic profile of PCD in Denmark - Genetic and Clinical Characterization of Patients with Primary Ciliary Dyskinesia in Denmark

Simone Maria Lodahl

Bachelor's thesis

Influenza vaccination during pregnancy: Effectiveness against clinical influenza disease in the pregnant individual

Sofie Siemsen

Bachelor's thesis

Influenza vaccination during pregnancy: Safety in the pregnant individual and fetus

Meet Our Professors



ALLAN MELDGAARD LUND

CENTER FOR INHERITED METABOLIC DISEASES

Allan Meldgaard Lund is a clinical professor of paediatrics and a leading expert in paediatric inherited metabolic disorders. His work spans advancements in neonatal screening programs, clinical trials leading to new treatments, and the use of genomic technologies to improve diagnostics. He collaborates internationally, contributing to European networks and multicenter studies. With numerous scientific publications and multiple awards, Professor Lund remains dedicated to transforming care for children with rare diseases.



CHRISTINA E. HØI-HANSEN

RESEARCH UNIT & CENTER FOR CEREBRAL PALSY AND EARLY BRAIN DAMAGE IN CHILDREN

Christina Engel Høi-Hansen is a professor in paediatric neurology. She focuses her research on neurological disorders in children, including developmental and acquired brain injuries, neurogenetic conditions, and neurometabolic diseases. Her work combines clinical and translational research to improve diagnostic methods, treatment strategies, and rehabilitation outcomes for children with complex neurological conditions. Christina Engel Høi-Hansen is highly regarded for her expertise in neuropaediatrics and her dedication to advancing care for children with neurological challenges.



JAKOB LORENTZEN

RESEARCH UNIT & CENTER FOR CEREBRAL PALSY AND EARLY BRAIN DAMAGE IN CHILDREN

Jakob Lorentzen is a physiotherapist and a professor in paediatrics at Rigshospitalet and the University of Copenhagen. His work focuses on children and adolescents with cerebral palsy (CP), aiming to consolidate and disseminate new knowledge in this field. As part of his role, he will lead the development of a new CP clinic at Mary Elizabeth's Hospital, envisioned as a national center for knowledge, education, and treatment of cerebral palsy. This initiative seeks to establish a central repository of information and a research center to advance early detection methods and effective treatments for children and young people with CP.



KIM GJERUM NIELSEN

DANISH PAEDIATRIC PULMONARY SERVICE

Professor Kim Gjerum Nielsen is a clinical professor of paediatrics and paediatric pulmonology at the Department of Clinical Medicine, University of Copenhagen, and a consultant at the Department of Paediatrics and Adolescents at Rigshospitalet. He was appointed professor in 2018, focusing on research into various critical lung diseases in children. His research areas also include cystic fibrosis and primary ciliary dyskinesia, within which he is also Director of the PCD Clinical Trial Network, which is organised within ERN LUNG (the European Reference Network for rare respiratory diseases). He has contributed to over 240 scientific publications in paediatric pulmonology.



KJELD SCHMIEGELOW

BONKOLAB

Kjeld Schmiegelow is a professor in paediatrics and in oncology. He leads extensive research on childhood cancers, with a primary focus on leukemia. His work spans translational and clinical research, including treatment optimization, late effects of therapy, and pharmacogenomics. He has made significant contributions to understanding individual variability in treatment response, aiming to improve survival rates and reduce long-term side effects for paediatric cancer patients. His research is internationally recognized, and he plays a key role in collaborative efforts to advance paediatric cancer care globally.



KLAUS MÜLLER

COPENHAGEN CENTER FOR ADVANCED PEDIATRIC CELL THERAPIES AND IMMUNE DEFICIENCIES

Professor Klaus Müller is a clinical professor in paediatric oncology at the University of Copenhagen and a consultant at Rigshospitalet's Juliane Marie Centre. His research primarily focuses on paediatric oncology, including hematology and immunology, with a particular interest in late effects following cancer treatment in children.



LONE GRAFF STENSBALLE

1) UNIT OF VACCINOLOGY, PAEDIATRIC INFECTIOUS DISEASE EPIDEMIOLOGY, AND PHARMACOEPIDEMIOLOGY

2) MARYS DATA POWERHOUSE: TRANSFORMING PAEDIATRIC HEALTHCARE

Lone Graff Stensballe is a research leader at Rigshospitalet, heading two highly productive and internationally recognised research environments that span clinical vaccinology, pediatric infectious disease epidemiology, and nationwide register-based research. Together, these units combine randomised clinical trials, advanced laboratory work, and large-scale epidemiological analyses to address key questions in pediatric health, healthcare organisation, and long-term outcomes of disease and treatment.



ULRIKKA NYGAARD

CHILD - CAG HOST INFECTIONS LABORATORY RESEARCH DRUGS

Ulrikka Nygaard is a clinical professor of pediatric infectious diseases at the University of Copenhagen and a consultant at the Department of Pediatrics and Adolescents at Rigshospitalet. She leads 'CHILD,' a nationwide research platform promoting basic, translational, and clinical research in childhood infections. CHILD conducts randomized controlled trials to optimize antibiotic therapy and numerous projects developing innovative diagnostic tools and investigating pathophysiology through omics technologies in prospective real-time nationwide studies.

Project in the spotlight:



Interview with Head of the Department, Eva Mosfeldt Jeppesen, MD, DrMedSci.

Could you describe the background and motivation for establishing the Nordic University Hospital Alliance (NUHA)?

NUHA was established with the aim of strengthening cooperation between the Nordic countries, resulting in a close collaboration between five University Hospitals, one from each country:

The collaboration aims to address the major health challenges of our time in the form of the double demographic pressure with fewer health professionals of working age and more elderly people with more chronic diseases and health challenges

In NUHA, the population is approximately 30 million, which forms a solid basis for research collaboration and strengthens the possibilities for promoting health solutions.

Which institutions and countries are part of NUHA, and how is the collaboration organised in practice?

The association consists of the following five centres:

- Landspítali - Universitetshospitalet, Reykjavik
- Rigshospitalet, København
- Karolinska Universitetssjukhuset, Stockholm
- Oslo Universitetssykehus, Oslo
- Helsinki Universitetssykehus, Reykjavik

The work in NUHA is organized into four work streams:

1. Platform Trials
2. Benchmarking

3. Rare Diseases

4. Future Health (sidetrack with CEOs)

What are the main goals of NUHA?

- To add value to patients and the overall healthcare system
- To address the world's major healthcare challenges
- To strengthen research collaboration
- To promote Nordic healthcare solutions and diplomacy

Which key challenges in paediatric research do you think NUHA can help address?

NUHA expands the population from 6 million in Denmark to 30 million in NUHA. This provides significantly better opportunities for research into rare diseases

All countries in the NUHA collaboration have well-functioning public health care systems.

The population is culturally similar to each other and the opportunities for exchanging patients, laboratory tests, data, research and much more are significantly better compared to a number of other countries with different healthcare systems, cultures, laws, etc.

How do you see NUHA strengthening research in the Department of Paediatrics at Rigshospitalet?

There is already considerable Nordic cooperation in a number of research fields. A good example is NOPHO (Nordic Society of Paediatric Haematology and Oncology) in the oncology field, but the possibilities are endless.

NUHA, for example, will be able to make a difference particularly in relation to data exchange, benchmarking and law issue. Within particularly rare diseases, research could benefit from the larger patient base and cooperation with researchers in other countries.

How do you expect NUHA to influence everyday clinical practice in the department, both for staff and for patients and families?

Nordic cooperation already has clinical significance, for example in relation to new expensive medicine.

The clinical trial unit at the Department of Children and Adolescents receives patients from other Nordic countries, just as the department sends patients out of the country. New expensive treatments such as Advanced Therapy Medicinal Product (ATMP) will have a much better chance of implementation with a larger patient base, and the area could be strengthened considerably.

Another area could be congenital heart disease or organ transplants, which require many special skills that are better supported with a larger patient base.



The five CEOs of the hospitals in the NUHA



Head of Department Eva Mosfeldt Jeppesen

What are the next steps for NUHA over the coming years, and where do you see the Department of Paediatrics contributing most?

- Focus will be on the four work packages: Platform Trials, Benchmarking, Rare Diseases and Future Health.
- In Denmark, a national ATMP strategy is being formed, which can work together with the other Nordic countries. New expensive medicine is an area of interest, not least for purchasing organizations.
- The rare diseases area is working on joint diagnosis registration in Orpha codes and joint identification of variants of uncertain significance.
- Cross-border networks and possible centres of excellence are being explored.
- Platform Trials have been established at RH, especially in the intensive care area, and collaborate Nordic-wide.
- Benchmarking is being developed cross-border.
- CEOs are working on the major health challenges, which are very similar in the Nordic countries with double demographic pressure, resulting in a decreasing number of health care professionals combined with a growing elderly population with more chronic diseases.

Professor in the spotlight:



Interview with Group Leader of CHILD, Ulrikka Nygaard, MD, Dr.Med.

You were appointed Professor in April 2025 – what do you hope your professorship will bring?

I hope my professorship, through leadership of the 'CHILD' nationwide research platform promoting basic, translational, and clinical research in childhood infections, will strengthen the translation of high-quality research into everyday clinical practice at Rigshospitalet, nationally, and internationally. Equally important, I aim to further develop case-based medical education that integrates research, clinical reasoning, and patient safety.

If you had to describe your professional “core” in a sentence, what is the common thread running through your work?

My professional core is improving outcomes for children with infectious diseases by combining rigorous clinical research, nationwide collaboration, and rapid implementation into practice.

Which research projects have you mainly been working on in 2025?

In 2025, my main focus has been on nationwide randomized trials aimed at optimizing antibiotic strategies for childhood infections. This includes finalized trials in children with febrile neutropenia and in neonates with early-onset sepsis, as well as continued work on randomized evidence supporting oral antibiotic strategies in pediatric urinary tract infections and bone and joint infections. Several of these studies have informed or been implemented in clinical practice. In parallel, we have advanced translational diagnostic projects, including studies in children with peripheral facial palsy to reduce the need for lumbar puncture, an invasive procedure often requiring general anesthesia.

How do you work to bridge research and clinical practice in day-to-day life?

I work directly at the interface between ward rounds, guideline development, and clinical trials, ensuring that research questions arise from real clinical needs. Nationwide networks allow rapid implementation once evidence is generated.

In your view, what defines strong research and learning?

Within the CHILD research group, we aim to further consolidate and expand an already strong environment characterized by curiosity, openness, and a robust nationwide network enabling coordinated use of expertise and resources across pediatric departments in Denmark. This requires protected time for research activities, reflection, and structured mentorship. Within our department, research, education, and clinical care are closely interconnected and mutually reinforcing.

Can you point to a concrete initiative from the past year where you feel we have measurably improved the quality of care?

We have implemented new national guidelines based on our large randomized controlled trial demonstrating the non-inferiority of oral therapy in children with uncomplicated bone and joint infections. This novel management strategy reduces the need for intravenous treatment, hospitalization, and healthcare costs.

What is your strategic priorities for 2026?

Looking ahead, we will strengthen the CHILD research network as an international leader in antibiotic stewardship trials and next-generation diagnostics, supporting a “less-is-more” approach that prioritizes evidence-based, targeted care.

Junior Researchers in the spotlight:

Interview with Lene Weber, Msc. & Ivana Novosel, Msc., PhD.



Could you briefly describe how you went from being a physiotherapist in clinical practice to becoming an early-career researcher?

Lene: I started as a paediatric physiotherapist at Rigshospitalet in 2006 and have a particular interest in neuro paediatrics. After completing my MSc in Health Science in 2019, I worked in the Department of Occupational Therapy and Physiotherapy, splitting my time 50% clinical work and 50% on development. Through my clinical work, collaboration with colleagues, and my supervisor, Christina Høi-Hansen, we developed the idea for my PhD as part of the

CP-EDIT (Cerebral Palsy - Early Detection and Intervention Trial).

Ivana: For nearly 16 years, I worked alongside individuals with neurological injuries, supporting motor learning and recovery in ways that shaped their daily lives. During this time, I witnessed, and played a role in, a fundamental shift in neurorehabilitation: from concept-oriented approaches to a more science-based and evidence-driven practice, and I became fascinated by the brain's capacity to change. A fascination that was reinforced every time I saw small but profound breakthroughs in clinical practice.

Name: Lene Weber

Education: Physiotherapist, Master of Science in Health Science

Position: Ph.d student at Marys Center for Cerebral Palsy

Research area: Neuro-paediatrics, focusing on early detection and intervention for infants at risk of cerebral palsy



Name: Ivana Bardino Novosel

Education: PhD in Neuroscience (University of Copenhagen), Master of Science in Physiotherapy.

Position: Postdoctoral Researcher, Clinic for Youth with Cerebral Palsy (CP-SPOT)

Research area: Development of methods to monitor and influence real-world movement behavior in youth with cerebral palsy



Over time, I began to ask more fundamental questions: How can the principles of neuroplasticity be most effectively translated into clinical practice to optimize motor learning and recovery? And to what extent are our assessments and interventions aligned with the real-world challenges, goals, and daily experiences of the individuals we serve? It is this curiosity, this love of the question, that ultimately led me into research.

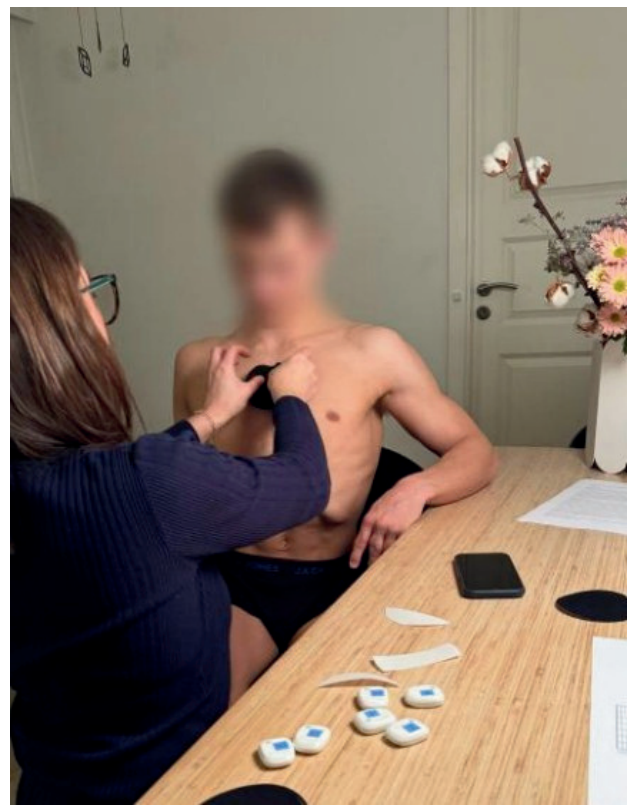
Lene, what is the main focus of your PhD? And what do you hope your upcoming postdoc will allow you to explore that you couldn't before?

The main focus of my PhD is early detection of cerebral palsy across four Danish hospitals. It reflects my key priorities: evidence-based assessment of infants, focus on parent perspectives, and implementation of tests to ensure clinicians are well prepared for the task. In my upcoming postdoc, I will focus on further implementation of my PhD results and on new studies involving infants at high risk of cerebral palsy.

Ivana, what is the main focus of your postdoc, and how does it build on your PhD?

During my PhD, I developed and validated wearable sensors and computational methods to monitor real-world movement behavior and designed home-based motor training programs grounded in neuroscience for adolescents and young adults with cerebral palsy. I did this under the primary supervision of Prof. Jakob Lorentzen, and I'm fortunate to continue this work at the Clinic for Youth with CP in my postdoc. Now the focus is on both monitoring and actively influencing real-world movement behavior.

The "influencing" strand involves a two-arm randomized trial using real-time feedback through technology developed in collaboration with the Elsass Foundation. The "monitoring" strand begins with a large



scoping review of wearable sensors and computational approaches, which guides consensus and informs improvements to our technical infrastructure and future AI training. So, my postdoc builds directly on my PhD, moving from observation to action.

What do you hope your research will ultimately mean for patients and families?

Lene: I hope our research helps ensure that infants at risk of cerebral palsy receive structured, interdisciplinary assessment nationwide, and that this leads to targeted training interventions for the infants and appropriate support for their parents.

Ivana: I hope it will help young people with cerebral palsy maintain and improve their motor skills, stay active, and participate to their fullest in daily life and society as they transition into adulthood. The research will provide personalized, actionable tools, like real-time feedback and wearable monitoring, to help them take charge of their own motor learning and practice, while potentially giving families the confidence to step back and support independence.

How do you work across disciplines, and what can physiotherapy contribute in particular to neuro-paediatric research?

Lene: Assessing for cerebral palsy is like putting together a puzzle of motor and neurological pieces, along with brain imaging. It requires teamwork across all professions to get a full picture of the infant. Paediatricians and physiotherapists play a key role in the motor and neurological assessment, while occupational therapists add fine motor and cognitive testing.

Ivana: I collaborate with several types of clinicians and researchers at Rigshospitalet, as well as engineers and neuroscientists at the University of Copenhagen and the Elsass Foundation. I've also involved, and continue to involve, youth with CP in shaping research questions and selecting outcome measures to ensure the projects address real-world needs. Working across disciplines isn't always easy. We often "speak different languages," and while I've learned a lot from, e.g. engineers, we've also misunderstood each other on more than one occasion. It would be much easier to stay in my own bubble, but I've yet to see anything useful come out of that. We have to work across disciplines in both practice and research because the individuals we meet present inherently interdisciplinary challenges. As a physiotherapist, I contribute a practical, patient-centered understanding of movement, motor learning, and functional goals, ensuring interventions are feasible, motivating, and relevant for adolescents and young adults with cerebral palsy.

If you were to design the ideal research environment for interdisciplinary research in a clinical department, what three things need to be in place to succeed?

Lene: First, collaboration in infant assessments – working hands-on side by side is meaningful for both clinicians and families, second, regular interdisciplinary

meetings for knowledge sharing, and third, interdisciplinary social events to support the work environment.

Ivana: First, there has to be a shared purpose and respect. Everyone needs to understand why the project matters, how each discipline can contribute, and how we can benefit from each other's expertise. Without that, it's easy for people to work in isolation and for ideas to get lost. Second, structured communication and collaboration are key. Regular meetings, clear project plans, and shared protocols keep everyone on the same page. And third, there needs to be openness to learning and adapting. Interdisciplinary work can be messy, but being willing to listen, adjust, and embrace different perspectives is what turns challenges into innovation.

What are you most looking forward to in your research work in 2026?

Lene: I am looking forward to a six-week research stay at the University of Sydney, where I will collaborate with clinical researchers on the early detection of cerebral palsy.

Ivana: I'm excited to get the feedback trial underway and to refine our sensors and AI model. I'm also looking forward to joining consultations with youth with CP in the clinic to better understand their perspectives and needs. Finally, I hope to expand my network with other centers and explore opportunities for future multicenter collaborations and to share insights.



The Research Groups

(in alphabetical order)

BONKOLAB

Head of BONKOLAB: Professor, MD, DMSci Kjeld Schmiegelow.

Employees: More than 130.

Contact: Kjeld.schmiegelow@regionh.dk

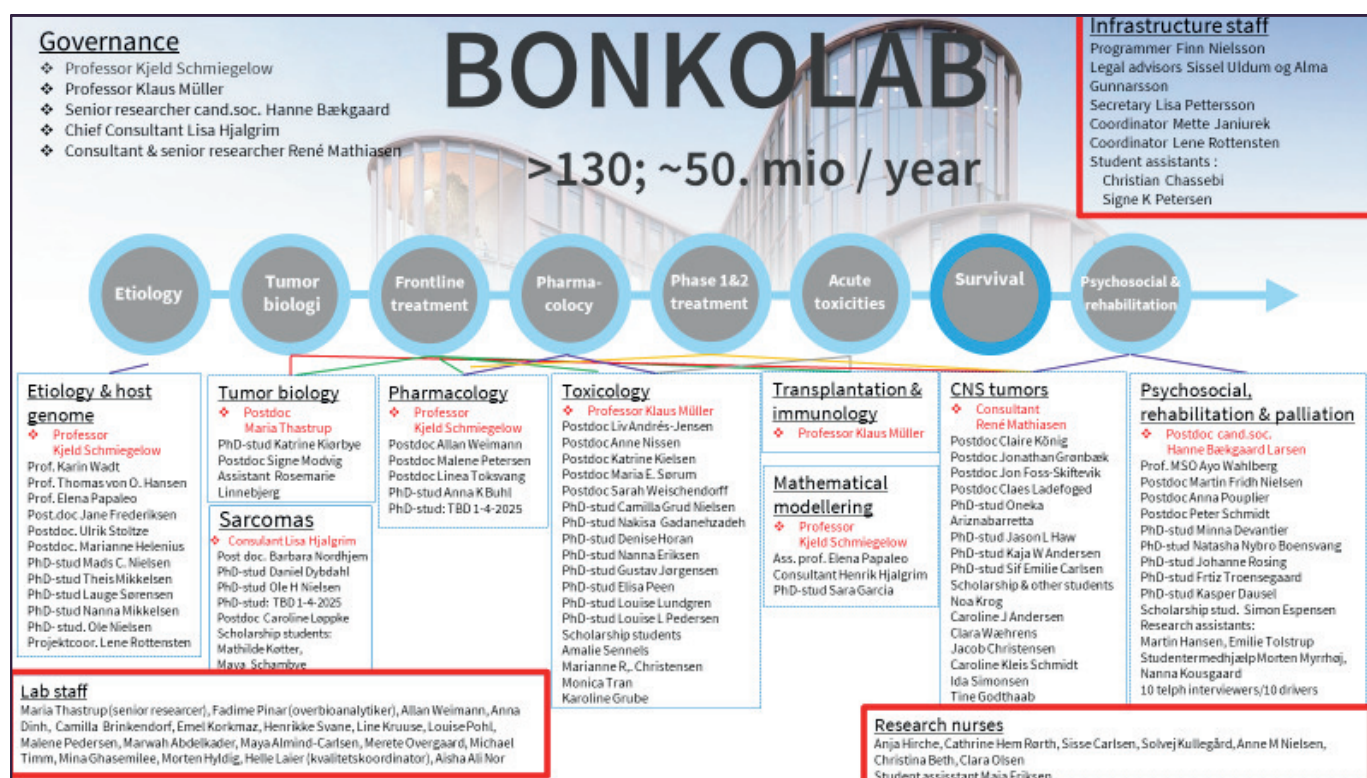
Strategies and Core Areas of Research:

Bonkolab is the largest paediatric oncology research unit in the Nordic/Baltic region with more than 130 employees. The laboratory comprises several specialized research groups addressing the broad spectrum of paediatric cancer research.

The core research areas include cancer etiology (including cancer-predisposition syndromes), host genomic variants influencing treatment efficacy and toxicity, tumor biology (including cancer cell survival and growth within the central nervous system), pharmacology and toxicology of anticancer agents (including immunological aspects), and phase I-III clinical trials of novel anticancer therapies. In addition, Bonkolab conducts extensive research into persistent late effects, psychosocial burden, and rehabilitation among childhood cancer survivors.

Bonkolab conducts the majority of its research within large international collaborative networks, ensuring high scientific quality and broad clinical impact.

Host genome variant group: This group focuses on (i) the use of whole genome sequencing and targeted low-cost sequencing of Danish childhood cancer patients to detect presence of cancer predisposition syndromes among children diagnosed with cancer; and (ii) common host genome variants that influences cancer risk, disease presentation, pharmacology, toxicology, and treatment efficacy.



The latter studies are performed within a large international consortium (“ELEGANT”) including 30 countries from Europe, Australia and South America. Group leaders: Karin Wadt, Professor MD, PhD & Kjeld Schmiegelow, Professor, MD, DMSci.

Pharmacology Group: The pharmacology group conducts research on drug metabolism and therapeutic drug monitoring, with particular focus on thiopurines, methotrexate, busulfan, asparaginase and tyrosine kinase inhibitors, as well as the impact of host genomic variants on drug metabolism and pharmacodynamics. The laboratory carries out several thousand analyses annually.

Group leader: Kjeld Schmiegelow, Professor, MD, DMSci

CNS Group: The CNS Group is a multidisciplinary research network dedicated to improving the diagnosis, treatment, and long-term follow-up of children with central nervous system tumors. The group combines translational and clinical research, including development of novel therapies with national and international clinical studies. Research efforts focus on earlier diagnosis through symptom characterization, improved molecular and imaging-based diagnostics, and treatment optimization using targeted therapies and advanced surgical strategies. In addition, the group addresses survivorship by studying treatment-related toxicity, late effects, and functional outcomes to improve quality of life for children and adolescents with CNS tumors.

Group leader: René Mathiasen, MD PhD, Assoc. Professor

Stem cell transplantation and immunotherapy: High-dose chemotherapy and stem cell transplantation have significantly improved survival rates in children with high-risk leukemia, but are associated with severe acute toxicities and long-term complications, and with treatment-related mortality of approximately 10%. Research in this area aims to characterize the immunological, cellular, and molecular mechanisms underlying these toxic reactions, with the goal of enabling individualized treatment through the use of new biomarkers and identification of molecular signaling pathways that can be targeted prophylactically. This includes multi-omics studies of genetics, the microbiome, metabolism, and immunology. Intensive cancer treatment during childhood results in long-term complications for the majority of survivors, with many survivors exhibiting features of premature ageing already in early adulthood. Ongoing studies investigate the significance of cellular aging and its relationship with treatment modalities and individual risk factors. Furthermore, cancer immunotherapy represents a breakthrough in treatment, leveraging the immune system’s ability to recognize and destroy cancer cells. A promising new strategy for generating an effective anti-cancer immune response is the use of chimeric antigen receptor (CAR) T cells. Bonkolab and the TXID clinicians have established a platform for in-house production of CAR T cells. Ongoing studies seek to enhance the efficacy of this treatment by gaining a better understanding of the mechanisms underlying treatment failure and toxicity. Additionally, efforts are being made to apply this technology to other paediatric cancer types.

Group leader: Klaus Müller, Professor, MD

Sarcoma group: In 2025 the sarcoma group was more formally established with several new PhD students joining the group, and with the development and work on the international innovative treatment protocol RESIST, which is a completely new innovative maintenance strategy for high sarcomas.

The overall goal of the sarcoma group is to improve the overall survival of children, adolescents and young adults with bone- and soft tissue sarcomas in Denmark through national and international research across various disciplines combining epidemiology, clinical and translational research. The research spans the whole trajectory of the diseases from etiology to late effects.

Group leader: Leading consultant Lisa Lyngsie Hjalgrim, MD

Tumour Biology Group: The Tumor Biology Group focuses on deciphering cancer cell biology in childhood hematological cancers and solid tumors and to translate these insights into improved staging and treatment strategies.

Key research areas include:

- High sensitivity methods for detecting central nervous system involvement in acute lymphoblastic leukemia and non-Hodgkin lymphoma using flow cytometry, quantitative mass spectrometry, and circulating tumor DNA profiling of cerebrospinal fluid. The group coordinates several international, multicenter CSF flow studies.
- Advanced in vitro models of acute lymphoblastic leukemia, including the developments of patient-derived 3D in vitro models of the bone marrow and the central nervous system that incorporate important aspects of the tumor microenvironment.

Group leader: Maria Thastrup, Laboratory leader, MSc, PhD

Psychosocial Group: The psychosocial rehabilitation research at Bonkolab focuses on supporting children, adolescents, and young adults live as normal a life as possible during and after cancer treatment. A core approach is early, cross-sectoral, and interprofessional intervention, grounded in a child-centered perspective. Intervention studies are carried out using qualitative and quantitative methods, physical testing, and cohort designs.

Group leader: Hanne Bækgaard Larsen, Assoc. Professor

Cumulated Funding Received in 2025:

Combined the researchers at Bonkolab a total of 42.7 mio DKK in 2025.



Highlights of 2025:

- A cohort of 1127 whole genome sequences Danish childhood cancer patients were published revealing a high frequency of cancer predisposition and based on the pattern of loss-of-function mutations in constrained genes indicated that a much higher proportion of childhood cancer patients may have developed their cancer due to rare underlying genetic cancer predisposition.
- Children with cancer are interested in attending school through telepresence robots. The use of robots has the potential to enhance children's social connection to school and support their learning. However, the implementation of robots takes place in a complex context where multiple factors can influence whether their use is successful.
- Children with cancer who have significantly reduced physical function at diagnosis (compared to healthy children) and participate in physical activity during treatment show significant improvements in their physical function both during and after treatment.[4]
- Siblings to children with cancer highly accept and value a school-based nursing support program.
- Preclinical mouse models have demonstrated that tumor responses towards immune checkpoint inhibitors can be significantly enhanced by co-administration of thiopurines to increase the tumor mutational burden.
- Establishment of a 3D bone marrow organoid culture model and successful engraftment of leukemic patient cells
- Multicolor flow cytometry of spinal fluid identifies malignant cells in the CNS in 30% of patients with T lymphoblastic lymphoma that is not detected by conventional diagnosis methods
- Sarcoma patients are more likely than the background population to have contacts in the primary healthcare sector, hospital outpatient clinics, and emergency rooms, and to fill prescriptions for pain- and antimicrobial medication for up to 24 consecutive months before diagnosis. Patients with metastatic sarcoma at the time of diagnosis were more likely than non-metastatic patients to use healthcare services in the last 2-4 months prior to diagnosis.
- In recent decades in Denmark, improvements were observed in reducing relapse incidence and increasing survival after relapse in children with haematological cancers and solid tumours. However, the persistent survival gap between children who relapse and those who do not across all childhood cancers underlines the need for intensified and highly targeted treatments for children at high risk of relapse.

Key Publications in 2025:

1. Raja RA, Als-Nielsen B, Lund AM, Vilstrup H, Dalhoff KP, Schmiegelow K. Asparaginase-associated hyperammonemia. *Haematologica*. 2025 Aug 1;110(8):1702-1709. doi: 10.3324/haematol.2025.287301. Epub 2025 Apr 24. PMID: 40270200; PMCID: PMC12358781.
2. Stoltze U, Junk SV, Byrjalsen A, Cavé H, Cazzaniga G, Elitzur S, Fronkova E, Hjalgrim LL, Kuiper RP, Lundgren L, Mescher M, Mikkelsen T, Pastoreczak A, Strullu M, Trka J, Wadt K, Izraeli S, Borkhardt A, Schmiegelow K. Overt and covert genetic causes of pediatric acute lymphoblastic leukemia. *Leukemia*. 2025 May;39(5):1031-1045. doi: 10.1038/s41375-025-02535-4. Epub 2025 Mar 24. PMID: 40128563.



3. Toksvang LN, Brigitha LJ, van der Sluis IM, Brivio E, Raja R, Pontoppidan P, Buhl Rasmussen AS, Andres-Jensen L, Uhlving HH, Kielsen K, Als-Nielsen B, Elitzur S, Dalhoff K, Schmiegelow K, Rank CU. Therapeutic drug monitoring in acute lymphoblastic leukemia-a deep dive into pharmacokinetics, -dynamics, and -genetics of antileukemic drugs. *Expert Rev Clin Pharmacol*. 2025 Mar;18(3):131-149. doi: 10.1080/17512433.2025.2465426. Epub 2025 Mar 3. PMID: 39949259.
4. Schäfer Hackenhaar F, Refhagen N, Hagleitner M, van Leeuwen F, Marquart HV, Madsen HO, Landfors M, Osterman P, Schmiegelow K, Flaegstad T, Jónsson Ó, Kanerva J, Abrahamsson J, Heyman M, Norén Nyström U, Hultdin M, Degerman S. CpG island methylator phenotype classification improves risk assessment in pediatric T-cell acute lymphoblastic leukemia. *Blood*. 2025 May 8;145(19):2161-2178. doi: 10.1182/blood.2024026027. PMID: 39841000; PMCID: PMC12105724.
5. Mikkelsen T, Helenius M, Ampatzidou M, Attarbaschi A, Andres-Jensen L, Borkhardt A, Conde Cuevas N, Escherich G, Hagleitner MM, Halsey C, Josephs- Spaulding J, Lundgren L, Pehn S, Polychronopoulou S, Stoltze U, Toksvang LN, Wahlberg A, Junk SV, Schmiegelow K. Role of common host genome variants in Childhood Acute Lymphoblastic Leukemia. *Leukemia*. 2026 Jan;40(1):3-24. doi: 10.1038/s41375-025-02804-2. Epub 2025 Dec 8. PMID: 41361660; PMCID: PMC12789036.
6. Stoltze UK, Hansen TVO, Foss-Skiftesvik J, Byrjalsen A, Henriksen KA, Otamendi Laspiur A, Gerdes AM, Ostrowski SR,..., Mathiasen R, Brok J, Sehested A, Gorissen BL, Rasmussen S, Karczewski KJ, Hjalgrim LL, Wadt KAW, Schmiegelow K. Childhood Cancer Predisposition and Evolutionary Constraints: Novel Lessons from Germline Genomes from 1,127 Children with Cancer. *Clin Cancer Res*. 2025 Nov 3;31(21):4495-4509. doi: 10.1158/1078-0432.CCR-25-0153. PMID: 40815213.
7. Nazerai L, Tsiavou C, Vardouli L, Schmiegelow K, De Zio D. Thiopurine therapy enhances immune checkpoint inhibitor efficacy in low-mutational burden melanoma: A promising anticancer approach. *Proc Natl Acad Sci U S A*. 2025 Mar 4;122(9):e2423246122. doi: 10.1073/pnas.2423246122. Epub 2025 Feb 24. PMID: 39993201; PMCID: PMC11892593.
8. Koch MR, Buhl Rasmussen AS, Als-Nielsen B, Duarte X, Escherich G, Heyman M, Lepik K, Malmros J, Nersting J, Johannsdottir I, Niinimäki R, Petersen MJ, Segers H, van der Sluis IM, Thastrup M, Vaitkeviciene G, Schmiegelow K, Toksvang LN. DNA-incorporated thioguanine to detect potential non-adherence to maintenance therapy in acute lymphoblastic leukemia. *Cancer Chemother Pharmacol*. 2025 Jul 16;95(1):76. doi: 10.1007/s00280-025-04784-7. PMID: 40668416; PMCID: PMC12267380.
9. Laustsen AF, Grønbaek JK, Frič R, Avula S, Mallucci C, Nilsson P, Nyman P, Hauser P, Mudra K, Kiudeliene R, Ročka S, Hjort MA, Brandsma R, Hoving E, Carai A, Beneš V, Táborská J, Dorfer C, Jacobs S, Pavon-Mengual M, Skjøth-Rasmussen J, Schmiegelow K, Sehested A, Mathiasen R, Juhler M. Postoperative speech impairment and cranial nerve deficits in children undergoing posterior fossa tumor surgery with intraoperative MRI - a prospective multinational study. *Acta Neurochir (Wien)*. 2025 Sep 22;167(1):252. doi: 10.1007/s00701-025-06669-3. PMID: 40982141; PMCID: PMC12454580.

10. Petersen MJ, Weimann A, Thastrup M, Toksvang LN, Schmiegelow K. LC-MS/MS quantification of 6-methylthio-2'-deoxyguanosine and 6-thio-2'-deoxyguanosine in genomic DNA with enzymatically synthesized isotope-labelled internal standards. *Anal Bioanal Chem*. 2025 Nov 22. doi: 10.1007/s00216-025-06240-0. Epub ahead of print. PMID: 41275025.

11. Fridh MK, Schmidt-Andersen P, Andrés-Jensen L, Thorsteinsson T, Wehner PS, Hasle H, Schmiegelow K, Larsen HB. Children with cancer and their cardiorespiratory fitness and physical function-the long-term effects of a physical activity program during treatment: a multicenter non-randomized controlled trial. *J Cancer Surviv*. 2025 Apr;19(2):672-684. doi: 10.1007/s11764-023-01499-7. Epub 2023 Dec 6. PMID: 38057671; PMCID: PMC11926049.

Key Partnerships in 2025:

- Pediatric oncology and Bonkolab is member of a European clinical & research network named Ped-One (chaired by Kjeld Schmiegelow) that includes Barcelona, Copenhagen, Düsseldorf, Prague, Vienna and Zürich that aims at developing innovative diagnostics and therapeutics for children with cancer.

Looking Ahead to 2026:

- In 2026, significant focus will be on:
 - Anticancer immunotherapy
 - Novel treatments for high-risk cancer (specifically sarcomas and brain tumors)
 - Toxicology with implementation of international strategies
 - Improvement of quality of life through launching the ChildLife project to support children aged 0–6 years diagnosed with cancer in safely returning to daycare during treatment.



The Clinical Trials Unit for Children and Adolescents with Cancer

Group Leader: Karsten Nysom, Senior Consultant, MD, PhD.

Team:

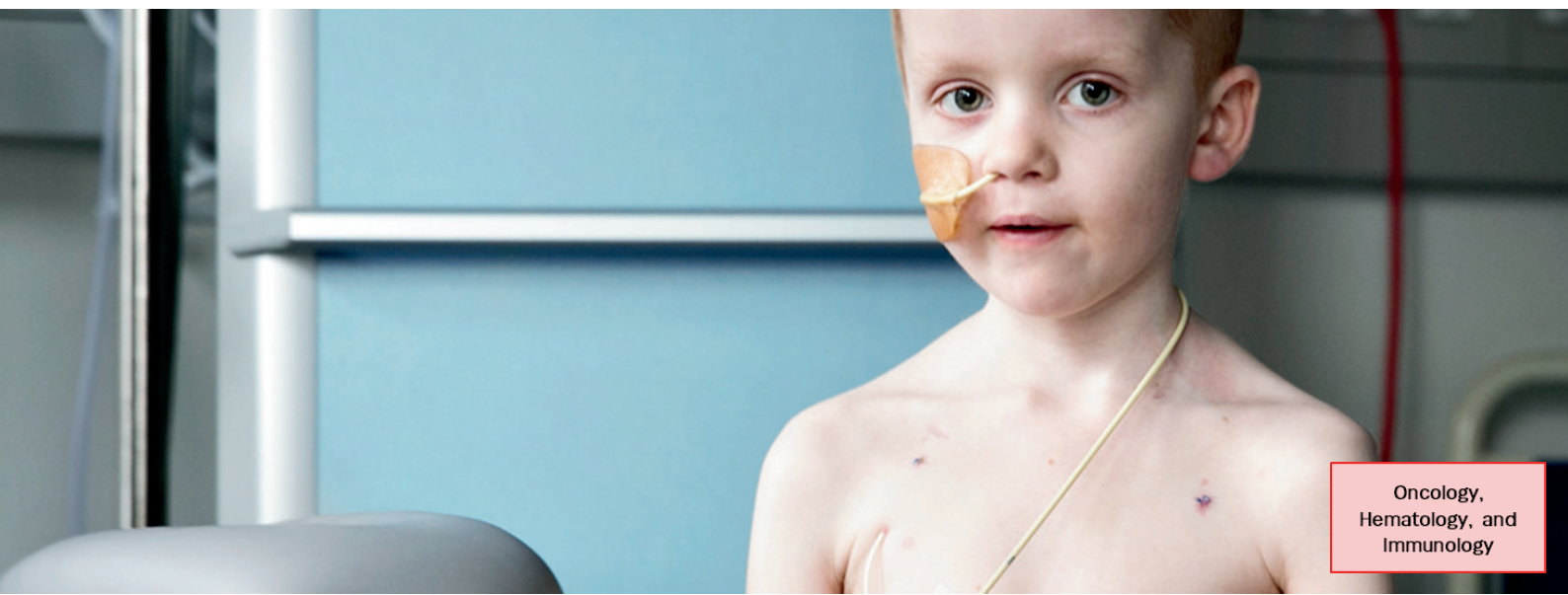
- **Medical:** 2,5 senior consultants (subspecialists in paediatric haematology and oncology)
- **Nursing:** 10 study nurses who also work as study coordinators, including one lead study nurse.
- **Regulatory:** 2 regulatory specialists
- **Administrative:** 1 research coordinator and 1 research secretary

Contact: Karsten.Nysom@regionh.dk

Strategies and Core Areas of Research:

The unit has three closely related activities:

- 1) The Danish National phase 1-2 Clinical Trials Unit for Children and Adolescents with Cancer, which is a member of the Innovative Therapies for Children and Adolescents with Cancer (ITCC) consortium and has ITCC Early Phase Accreditation;
- 2) The Danish National Clinical Trials Unit for Children and Adolescents with cancer, which supports opening, conducting, updating and closing of all clinical trials for newly diagnosed children with cancer in Denmark, supported by a generous grant from the Danish Childhood Cancer Foundation to the Danish multidisciplinary working group for children and adolescents with cancer (DAPHO)
- 3) The Clinical Trials Unit supports the conduct of all clinical trials for children and adolescents with cancer at Department of Paediatrics and Adolescent Medicine at Rigshospitalet.



Highlights of 2025:

- Initiated 4 new phase 1-2 trials and 2 phase 3 trials, of which 2 and 0 were sponsored by the pharmaceutical industry.
- Had 46 actively recruiting clinical trials, of which 13 were phase 1, 14 were phase 2 and 19 were phase 3.
- Enrolled 14 new children in phase 1-2 trials (including 8 referred from abroad), 40 in phase 3 trials and 20 in international registries.
- A total of 47 children participated in phase 1-2 trials during 2024, 177 in phase 3 trials and 58 in international registries.
- Participated in two inspections from the Danish Medicines Agency (DKMA) and one inspection from the European Medicines Agency (EMA).
- Obtained approx. 200.000 € in grants for investigator-initiated clinical trials or international registries

Key Publications in 2025:

- Gray JC et al., *Dinutuximab Beta Added to Temozolomide-Based Chemotherapy for Children With Relapsed and Refractory Neuroblastoma: Results of the ITCC-SIOPEN BEACON Immuno Phase II Trial*. J Clin Oncol. 2026 Jan 20;44(3):176-187. doi: 10.1200/JCO-25-01868.
- Nysom K et al.. *Response to letter from Dr. Avery*. Neuro Oncol. 2025 Dec 8;noaf273. doi: 10.1093/neuonc/noaf273.
- DuBois SG et al., *Paediatric Strategy Forum for medicinal product development of agents targeting GD2 ganglioside in children and adolescents with cancer*. Eur J Cancer. 2025 Dec 9;231:116093. doi: 10.1016/j.ejca.2025.116093.
- DuBois SG et al., *Paediatric Strategy Forum for medicinal product development of agents targeting GD2 ganglioside in children and adolescents with cancer*. Eur J Cancer. 2025 Dec 9;231:116093. doi: 10.1016/j.ejca.2025.116093.



- Baruchel A et al. *Isatuximab in combination with chemotherapy for pediatric patients with relapsed/refractory acute lymphoblastic leukemia or acute myeloid leukemia: The ISAKIDS study*. *Hemasphere*. 2025 Oct 30;9(10):e70245. doi: 10.1002/hem3.70245.
- Schönung M et al. *Assessment of peripheral blood DNA methylation signatures as pharmacodynamic and predictive biomarkers during azacitidine therapy in juvenile myelomonocytic leukaemia: Results of the EWOG-MESRAT study*. *Br J Haematol*. 2025 Oct;207(4):1271-1278. doi: 10.1111/bjh.70046.
- Pearson AD et al. *Paediatric strategy forum for medicinal product development of cyclin-dependent kinase inhibitors in children and adolescents ACCELERATE in collaboration with the European Medicines Agency With participation of the Food and Drug Administration*. *Eur J Cancer*. 2025 Aug 26;226:115629. doi: 10.1016/j.ejca.2025.115629.
- Sarup R et al., *Glucocorticoid use in paediatric posterior fossa tumour surgery and the occurrence of postoperative speech impairment*. *Childs Nerv Syst*. 2025 Jul 11;41(1):231. doi: 10.1007/s00381-025-06850-0.
- Pearson ADJ et al., *New models for the development of and access to CAR T-cell therapies for children and adolescents with cancer: an ACCELERATE multistakeholder analysis*. *Lancet Oncol*. 2025 Apr;26(4):e214-e224. doi: 10.1016/S1470-2045(24)00736-8.
- Mora J et al., *The anti-GD2 monoclonal antibody naxitamab plus GM-CSF for relapsed or refractory high-risk neuroblastoma: a phase 2 clinical trial*. *Nat Commun*. 2025 Feb 14;16(1):1636. doi: 10.1038/s41467-025-56619-x.
- Pearson AD et al., *Paediatric strategy forum for medicinal product development in diffuse midline gliomas in children and adolescents. ACCELERATE in collaboration with the European Medicines Agency with participation of the Food and Drug Administration*. *Eur J Cancer*. 2025 Feb 25;217:115230. doi: 10.1016/j.ejca.2025.115230



Center for Inherited Metabolic Diseases

Group Leader: Allan Meldgaard Lund, Professor <https://orcid.org/0000-0002-6091-7879>

Clinical Team:

- Mette Cathrine Ørngreen, MD, DMSc <https://orcid.org/0000-0002-2991-6309>
- Sabine Weller Grønborg, MD <https://orcid.org/0000-0002-2113-9023>
- Merete Ljungberg, MD, <https://orcid.org/0000-0003-1998-6415>
- Olivia Fjellbirkeland, MD, PhD student <https://orcid.org/0000-0003-1983-1456>
- Cecilie Videbæk, MD, PhD student <https://orcid.org/0000-0002-3487-2849>
- Alberte Lundquist, MD, PhD student <https://orcid.org/0000-0002-8574-9824>
- Ida Handberg, BSc, scholarship student
- Marselía Bríet Borgarsdóttir, BSc, scholarship student
- Camilla Sindesen, Bachelor student
- Kirsten Ahring, cand.scient. PhD, senior researcher (dietician) <https://orcid.org/0000-0002-6518-2226>
- Christine Jørgensen, cand.scient. Clin.Nutr.
- Camilla Carøe, cand.scient, Clin.Nutr.

Contact: allan.lund@regionh.dk

Strategies and Core Areas of Research:

In Center for Inherited Metabolic Diseases, we are dedicated to advancing the understanding of metabolic diseases from diagnosis via newborn screening to treatment and follow-up. Our research spans across various populations, with studies of diagnostic paths, including newborn screening, treatment and pathophysiological investigations, including basal metabolic investigations of fat, glucose and protein metabolism in acutely and chronically ill children, healthy children, and individuals of all ages with inborn errors of metabolism.

Our mission is to uncover the complexities of metabolic processes and their impact on health and disease. Through rigorous clinical trials and innovative research, we aim to develop new strategies for diagnosis (including newborn screening), treatment, clinical and biochemical follow-up, and prevention of metabolic disorders.

Join us as we strive to improve the lives of those affected by metabolic conditions and contribute to the broader field of metabolic health.

Ongoing PhD-studies:

- **LNNA treatment in phenylketonuria**, by Olivia Welle Fjellbirkeland, ongoing PhD project, Supervisor: Allan Meldgaard Lund
- **BabyPrecision - How to decrease the number of false-positive newborns while increasing performance and adaptability of newborn screening (NBS) – use of targeted next generation sequencing (NGS) in NBS**, by Alberte Aspaas Lundquist, ongoing PhD project. Supervisor: Allan Meldgaard Lund
- **Danish XALD study**, by Cecilie Videbæk, ongoing PhD project. Supervisor: Allan Meldgaard Lund

Key Publications in 2025:

- **Zhao Q, Naume MM, de Winter BCM, et al. Paracetamol and its metabolites in children and adults with spinal muscular atrophy - a population pharmacokinetic model.** Br J Clin Pharmacol. 2025;91(7):2045-2056. doi:10.1002/bcp.70028
- **Sloth TB, Ørngreen MC, Ek J, Bache I, Wibrand F, Lund AM. Human D-Lactate Dehydrogenase Deficiency: A Case Report in a Young Boy.** JIMD Rep. 2025;66(4):e70039. Published 2025 Jul 17. doi:10.1002/jimd2.70039
- **Lee A, Bliksrud YT, Onali M, et al. Palliative Care for Children and Adults With Inherited Metabolic Disease in Europe: An Underutilised Service for Supportive Treatment and Care.** J Inherit Metab Dis. 2025;48(6):e70095. doi:10.1002/jimd.70095
- **Bahtiri L, Jørgensen C, Ahring K, et al. Clinical assessment of growth patterns, weight, and dietary management in children and adults with phenylketonuria - a retrospective study.** Orphanet J Rare Dis. 2025;20(1):621. Published 2025 Dec 13. doi:10.1186/s13023-025-04136-x
- **Videbæk CS, Dunø M, Lindquist SG, et al. Nationwide Study of the Epidemiology and Clinical Features of X-Linked Adrenoleukodystrophy in Denmark.** Mol Genet Metab. 2025;146(4):109265. doi:10.1016/j.ymgme.2025.109265
- **Šáhó R, Formánková R, Eisengart JB, et al. Outcome of Haemopoietic Stem Cell Transplantation in 21 Patients With Alpha-Mannosidosis.** J Inherit Metab Dis. 2025;48(4):e70047. doi:10.1002/jimd.70047
- **Kuypers AM, Das AM, Maiorana A, Heiner-Fokkema MR, van Spronsen FJ; TT1 MetabERN Professional Collaboration Group. Overview of European Practices for Management of Tyrosinemia Type 1: Towards European Guidelines.** J Inherit Metab Dis. 2025;48(5):e70089. doi:10.1002/jimd.70089



- Stepien KM, Thomas S, Hennermann JB, et al. Evolution of mobility, pain/discomfort, self-care, and mental health in patients with alpha-mannosidosis: an international caregiver and patient survey. *Orphanet J Rare Dis.* 2025;20(1):217. Published 2025 May 7. doi:10.1186/s13023-025-03694-4
- Raja RA, Als-Nielsen B, Lund AM, Vilstrup H, Dalhoff KP, Schmiegelow K. Asparaginase-associated hyperammonemia. *Haematologica.* 2025;110(8):1702-1709. doi:10.3324/haematol.2025.287301
- Caroe CDB, Lund AM. Does p-lactate increase in patients with GSD1 after ingesting a meal with common-size sources of fructose and galactose? Observations from a prospective, non-blinded, crossover pilot study. *JIMD Rep.* 2025;66(1):e12457. Published 2025 Jan 16. doi:10.1002/jmd2.12457

Featured Studies:

- Astrid Høj reported a case study of an adult-onset patient with MADD, illustrating that the diagnosis of inborn errors of metabolism (IEM) in adults remains challenging: 2-^[18F] FDG PET/CT in Rapid Late-Onset Multiple Acyl-CoA Dehydrogenase Deficiency: A Case Report. Høj A, Holm-Yildiz S, Krag T, Dejanovic D, van Overeem Hansen T, Dunø M, Ørngreen MC, Vissing J, Løkken N. *JIMD Rep.* 2025;12;66(2).

Highlights of 2025:

- In 2025 Ida Handberg presented her results from the study of: *Altered plasma amino acid profile in children with acute liver failure* at the yearly ESPHGAN meeting in Helsinki, Finland.

Fundings received in 2025:

- Rigshospitalets Forskningspuljer:
 - *Metabolism in children during acute and chronic illness.*
 - Three-year salary funding for 20% research time to Mette Cathrine Ørngreen.
- Børnecancerfonden:
 - *Exploring Metabolic Outcomes in Children with CNS Tumor: Efficacy of Attentin and GLP-1 Treatments.*
 - One-year scholarstipendiate to Ida Handberg

Clinical trials:

See <https://www.rigshospitalet.dk/afdelinger-og-klinikker/julianemarie/boern-og-unge/sjaeldne-sygdomme-center-5004/CMS/klinisk-forsoesenhed-for-sjaeldne-sygdomme/Sider/default.aspx>

Collaborations in 2025:

Our most important national collaborators include Department of Clinical Genetics, Statens Serum Institute, Research Unit for Molecular Medicine, Aarhus, and internationally most of our collaborators are within the European References Network for Hereditary Metabolic Diseases, European Registry and Network for Intoxication Type Metabolic Diseases, Beatrix Children's Hospital, Section Metabolic Diseases, Groeningen, Netherlands as well as the MLD initiative (<https://www.mldinitiative.com/>).



Center for Rare Diseases – Syndromal and Non-Metabolic Disorders

Team: Stense Farholt, Anne Marie Bisgaard, Jane Lunding Larsen, Mette Gyhrs, and Hanne Hove. One team member hold part-time research funding positions: Hanne Hove (30%).

Contact: Hanne Hove, hanne.buciek.hove@regionh.dk

Strategies and Core Areas of Research:

The Center for Rare Diseases is an outpatient clinic with a primary focus on diagnosing and providing follow-up care for complex, multidisciplinary diseases. We specialize in a broad range of rare conditions, such as syndromal craniofacial disorders, skeletal dysplasias, tuberous sclerosis, neurofibromatosis type 1 (NF1), Rubinstein-Taybi syndrome, Prader-Willi syndrome, and Rett syndrome.

Research and clinical work are closely related at the Danish Center for Rett syndrome. We aim to contribute to timely and optimized prevention and treatment. The research is conducted in collaboration with both national and international researchers. We participate actively in national and international conferences and meetings.

External Funding Received in 2025:

We successfully secured external funding of DKK 60.000, which enabled child-friendly wall paintings and decorative artwork in our examination rooms, waiting area, and hallway.

Photo from “The Rare Danes”. Photo Credit: TV2 Danmark



Highlights of 2025:

1. Mia Aagaard Doherty successfully defended her PhD thesis entitled *Living with neurofibromatosis 1: Cancer risk, mortality, and survival across the lifespan*.

- The PhD project was a collaborative effort between the two Centres for Rare Diseases in Copenhagen and Aarhus and the Danish Cancer Institute at the Danish Cancer Society. Two of the three PhD papers have been published: Neoplasm risk in individuals with neurofibromatosis 1 – A Danish nationwide cohort study with long-term follow-up (British Journal of Cancer, 2025 and Non-neoplastic causes of death in neurofibromatosis 1: A cohort study with long-term follow-up (Genetics in Medicine, 2025).
- The PhD is part of a large national research program called “Life with NF1” based on information from national registries, questionnaires, and cognitive tests, which constitutes a powerful instrument for assessing different aspects of living with NF1. We have shown that NF1 is characterized by an extraordinarily high somatic and psychiatric disease burden and cognitive impairments with far-reaching consequences on educational attainment, including school grades, income and employment, founding a family, pregnancy outcomes, and quality of life.

2. Research Collaboration on Achondroplasia:

- A research project was initiated as part of the master's thesis by Louise Hove Buciek, titled Sleep-Disordered Breathing in Children with Achondroplasia Assessed by Polysomnography.
- Louise conducted several months of research at MCRI under the supervision of Professor Ravi Savarirayan (Melbourne) and Professor Christian von Buchwald (Rigshospitalet, Denmark).
- The results are published: Buciek LH, Jacobsen JR, Raj S, Adams A-M, Vandeleur M, Hove HB, von Buchwald C, Kiaer EK, Griffiths A & Savarirayan R. Sleep-disordered breathing in children with achondroplasia assessed by polysomnography: a retrospective chart review. Archives of Disease in Childhood (E-pub ahead of print, 17 July 2025).

3. Center for Rett syndrome

- Jane Lunding Larsen successfully defended her PhD thesis: *Being a parent of an individual with Rett syndrome across different ages and phases of life*. The PhD project focused on the wellbeing in parents of individuals with Rett syndrome, which causes multiple disabilities. More specifically it focused on parents' wellbeing and how and by which factors it can be affected across different ages and phases of life
- Two of the three papers from the study have been published. The first paper, *Psychological aspects of being a parent of an individual with Rett syndrome: A scoping review* was published in January 2024 in Journal of Applied Research in Intellectual Disabilities. The second paper *Being a parent of an individual with Rett syndrome – An explorative study of parental experiences across different ages and phases of life* was published in October 2025 in Journal of Developmental and Physical Disabilities. The third paper are expected to be published in 2026.

- The study Longitudinal trajectory of gross motor skills in school-aged children with Rett syndrome has been accepted for publication in *Frontiers of Neurology*. In collaboration with researchers from The Kids Research Institute Australia, University of Western Australia, Perth, Western Australia, Australia we identified decline in gross motor skills during childhood. This novel natural history data can assist with interpretation of changes following the administration of new therapeutics.

Key publications in 2025:

- Doherty MA, Grell K, Hove H, et al. *Neoplasm risk in individuals with neurofibromatosis 1 - a Danish nationwide cohort study with long-term follow-up*. *Br J Cancer*. 2026;134(4):618-626. doi:10.1038/s41416-025-03316-7
- Doherty MA, Grell K, Hove H, et al. *Non-neoplastic causes of death in neurofibromatosis 1: A cohort study with long-term follow-up*. *Genet Med*. Published online November 25, 2025. doi:10.1016/j.gim.2025.101654

Looking Ahead to 2026:

We have successfully contributed to implement an Adult Centre for Rare Diseases, which is hosted within the Department of Clinical Genetics at Rigshospitalet. We maintain a close and well-integrated collaboration with this center. Many, although not all, adult patients with rare syndromic disease have been transitioned to the Adult Centre for Rare Diseases. This includes patients with neurofibromatosis type 1 (NF1), Prader-Willi syndrome, Williams syndrome, and craniofacial conditions, among others.

Photo from “The Rare Danes”. Photo Credit: TV2 Danmark



Center of Adolescent Medicine

Group Leader: Kirsten Boisen

Employees: 2 part-time employees.

Contact: kirsten.arntz.boisen@regionh.dk

Strategies and Core Areas of Research:

We are a small research unit dedicated to improving conditions for young people with chronic and serious illnesses in Denmark /at Rigshospitalet. In 2019, we collaborated with patients and stakeholders across Denmark to develop the National Guideline for a Youth-Friendly Healthcare System via a Delphi study. All our research aligns with the scope of this guideline and consistently involves young patients and/or their families. As a small unit, we strive to collaborate with other research units and clinics that share an interest in adolescent medicine.

Highlights of 2025:

- Working on the implementation of transitional care in paediatrics together with healthcare professionals from sub-specialties.
- Co-creating transitional care for young adults in cooperation with healthcare professionals, young adults, and parents and presenting the work at the 5th European Symposium on Transition in Paris.
- All data, both qualitative and quantitative, from ParTnerSTEPS has been collected, and analysis can now begin.
- Evaluating and publishing the public and patient involvement (PPI) processes in the development of the transfer program ParTnerSTEPS.
- Co-creation of a digital mental-cognitive coach in a private-public cooperation.



- Analyzing data and writing a report on a nationwide questionnaire regarding young people's priorities in healthcare in association with the Danish Association of Youth Panels
(<https://www.regionh.dk/ungepanel/nyheder/Sider/Nyheder/2025/resultaterne-er-klar.aspx>)
- Keynote speaker at the 5th European Symposium on Transition in Paris presenting: Parents in Transition: Their Role, Challenges, and Strategies for Support during Healthcare Transition.

Key Publication in 2025:

- Vanden Wyngaert K, Suykens J, Kelly D, Goncalves I, Thomsen EL, Willemse J, Mariño Z, Madaleno J, Day J, Samyn M, Ali M, Jørgensen MH, Goossens E, De Munter J, Staa AV, Wray J, Protopapa P, Lohse A, De Bruyne R. *Pediatric to adult care transfer in rare liver diseases: Recommendations based on insights from a European omnistakeholder event*. JHEP Rep. 2025 Mar 26;7(6):101403. doi: 10.1016/j.jhepr.2025.101403.
- Jensen DA, Hansen LM, Johnsen MD, Dengsø K, Thomsen EL. 'You Have to Be Well, to Be Sick'-A Qualitative Study of Young Adults' Experiences of Undergoing Liver Transplantation. Scand J Caring Sci. 2025 Mar;39(1):e70014. doi: 10.1111/scs.70014
- Lunding Larsen, J., Emdal Navne, L., Stahlhut, M., Bisgaard, A., Thomsen, EL. & Hansson H. *Being a Parent of an Individual with Rett syndrome – An Explorative Study of Parental Experiences Across Different Ages and Phases of Life*. J Dev Phys Disabil (2025).
<https://doi.org/10.1007/s10882-025-10031-x>

New Partnerships in 2025:

Member of the educational committee at the International Association for Adolescent Health (IAAH), representing Europe and Euteach (European Training in Effective Adolescent Care and Health).

Looking Ahead to 2026:

Evaluating ParTNERSTEPS RCT study and submitting results for publication.

Working on implementation of transitional care in paediatrics and adult care together with healthcare professionals from sub-specialties.



CHILD: CAG Host Infections Laboratory research Drugs

Group Leader: Professor Ulrikka Nygaard & co-leader Nadja Vissing

Team: Ass. professor Mette Holm, Skejby; consultant Ulla Hartling, Odense; Ass. professor Maren Rytter, Slagelse; consultant Jonathan Glenthøj, Hillerød; Ass. Professor Lisbeth Samsøe, Herlev; Ass. professor Allan Bybeck Nielsen, Hvidovre; Ass. professor Emma Carlsen, Rigshospitalet; post. doc. Kia Dungu, RH/SSI; Anders Husby, RH.

PhD students: 7

Contact: Ulrikka.Nygaard@regionh.dk

Strategies and Core Areas of Research:

CHILD (CAG Host Infections Laboratory research Drugs) is a nationwide research platform promoting basic, translational, and clinical research in childhood infections. CHILD conducts randomized controlled trials to optimize antibiotic therapy and numerous projects developing innovative diagnostic tools and investigating pathophysiology through omics technologies in prospective real-time nationwide studies.

Highlights of 2025:

- Finalized two nationwide randomized controlled trials aiming at optimizing antibiotic therapy in children; one in children with febrile neutropenia, to be published in Lancet Child and Adolescents Health in 2026 (principal investigator Ass Professor Nadja Vissing) and one in neonates with early onset sepsis (principal investigator Ass Professor Emma Carlsen).
- The randomized controlled trial on individualized antibiotic therapy for febrile urinary tract infections, published in Lancet Infectious Diseases in 2025 was selected among the top 10 trials with practice-changing potential at the annual US Infectious Disease Week

Photo from "Colorbox"



- Finalized a clinical study on peripheral facial palsy in children showing that serum *Borrelia* antibody testing can guide diagnostic decision-making and reduce the need for lumbar puncture, supporting implementation of a “less-is-more” diagnostic strategy in children with facial palsy.
- Advanced a nationwide prospective real-world effectiveness study investigating oral therapy to children with bone and joint infections based on the non-inferior findings of oral treatment in our nationwide RCT published in 2024.
- Contributed to the ongoing research debate on antibiotic stewardship using oral treatment strategies in pediatric infections via original research, and through invited reviews e.g. on doxycycline treatment in young children with neuroborreliosis and the implementation of oral antibiotics for uncomplicated bone and joint infections.
- Initiated collaborative basic science research studies using proteomics and cell-free bacterial DNA analyses in plasma to improve diagnostics and provide novel insights into disease pathogenesis in children with facial palsy, bone and joint infections, and febrile neutropenia.

Looking ahead to 2026:

- One nationwide randomized trial will be finalized. This is the first trial of hydrochloric acid vs. placebo to prevent bacteremia in children with central venous catheters.
- Initiate a new multi-omics research project in children with infectious diseases, funded by Novo Nordisk Foundation with DKK 10 million.
- Initiated a nationwide randomized trial on early oral switch in children with bloodstream infections, funded by Rigshospitalets Forskningsfond and Innovation Fund Denmark.

Key Publications in 2025:

- Sethi NJ, Carlsen ELM, Tabassum A, et al. Efficacy and safety of individualised versus standard 10-day antibiotic treatment in children with febrile urinary tract infection (INDI-UTI): a pragmatic, open-label, multicentre, randomised, controlled, non-inferiority trial in Denmark. *Lancet Infect Dis.* 2025;25(8):925-935.
- Bloch J, Schmidt L, Vissing N, et al. Oral Doxycycline Was a Safe and Feasible Treatment in Young Children With Neuroborreliosis. *Acta Paediatr.* 2025;114(8):2061-2065.
- Smedegaard LM, Dungu KHS, Guo Y, et al. Fever in Children with Cancer: Pathophysiological Insights Using Blood Transcriptomics. *Int J Mol Sci.* 2025;26(15):7126.
- Nielsen AB, Holm M, Blanche P, Nygaard T, Nygaard U, Danish CHILd study group. 1-year follow-up of oral versus intravenous antibiotics in children and adolescents with uncomplicated bone and joint infections: a nationwide, randomised, controlled, non-inferiority trial in Denmark. *Lancet Child Adolesc Health.* 2025;9(4):e10-e11.
- Andersen NL, Antsupova V, Boel JB, Christensen MM, Nygaard U, Schmidt LS. Urinary Tract Infection in Children and the Potential Role of Pivmecillinam as a Treatment Option. *Pediatr Infect Dis J.* 2025;44(1):e1-e3.
- Nygaard U, Holm M, Alcobendas R, Nielsen AB. Oral Antibiotics for Children and Adolescents With Uncomplicated Bone and Joint Infections. *Pediatr Infect Dis J.* 2025;44(5):e166-e169.
- Dungu KHS, Malchau Carlsen EL, Petersen OB, Vissing NH, Nygaard U. Herpes simplex virus - state of the art of prevention and treatment. *Semin Fetal Neonatal Med.* 2025;30(4):101664.

Copenhagen Center for Advanced Paediatric Cell Therapies and Immune Deficiencies

Group Leader and team members: Professor Klaus Müller (MD, DrMedSc, PhD), Senior consultants Marianne Ifversen (MD, PhD), Tania Nicole Masmias (MD, PhD, team leader), and Hilde Hylland Uhlving (MD, PhD), Consultant Stine Nygaard Nielsen (MD, PhD), Post docs Katrine Kielsen (MD, PhD) and Maria Ebbesen Sørum (MD, PhD)

Number of employees: 23

Contact: klaus.mueller@regionh.dk

Strategies and Core Areas of Research:

Cellular treatment and severe primary immunodeficiency.

- Focus
 - Application of CAR-T treatment outside approved conditions
 - Early detection of relapse after paediatric hematopoietic stem cell transplantation (HSCT)
 - Toxicity and immune reconstitution early post HSCT
 - Late effects post HSCT
 - Screening, diagnostics, and treatment in primary immunodeficiencies (PID) and bone marrow failures
- Strategies
 - Prospective clinical database
 - Systematic prospective biobanking
 - Participation in international treatment protocols and registries
 - Academical Chimeric antigen receptor (CAR) T-cell therapy and gene therapy studies
 - Cross-sectional and prospective follow-up studies
 - Active recruitment and talent program



New Partnerships in 2025:

1. Establishment of steering committee on new academic CAR-T trial CARS4ALL-01
2. Network for the Advancement of Neutropenia Research and Patient Support (Neutro-NARPS)

Highlights of 2025:

- Initiation of the MindMe – study (Monitoring Neurocognitive Deficit, Metabolic state and Exercise) – a cross-sectional study evaluating late-effect in paediatric HSCT survivors at 2-15 years follow-up.

Funding received in 2025:

- BørnerigetFonden/Jaschafonden: 280.000 Euro
- The Danish Childhood Cancer Foundation: 403.540 Euro
- The Danish Cancer Society: 9635 Euro.
- Rigshospitalets Forskningspulje: 133.845 Euro

Key Publications in 2025:

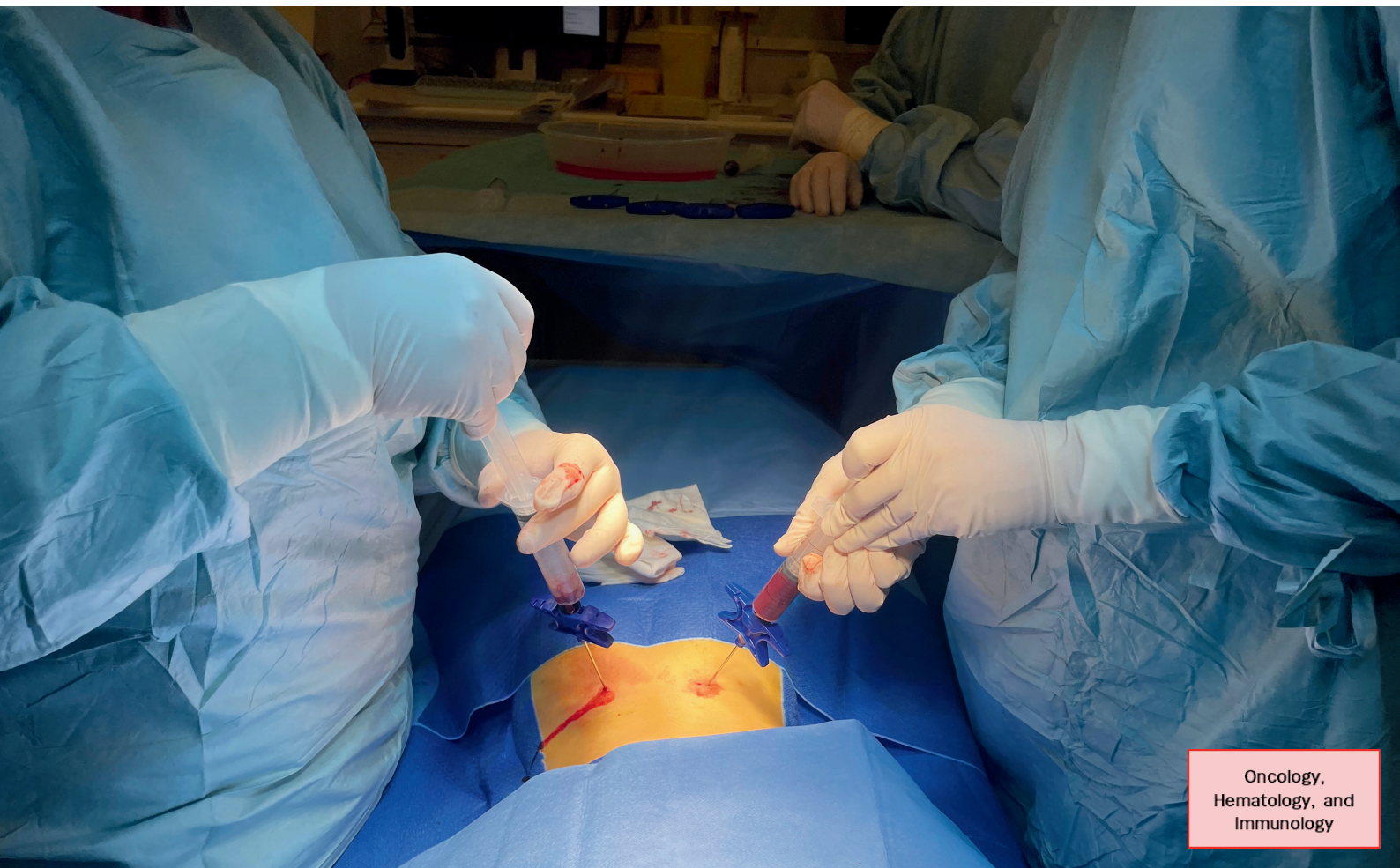
1. Balduzzi A et al: Impact of minimal residual disease on the outcome of hematopoietic stem cell transplantation for childhood acute lymphoblastic leukemia within the forum trial. Hematology Aug 2025 Doi:10.3324/haematol.2025.287456
2. Buechner J et al: Outcome of children with B-cell acute lymphoblastic leukemia with hypodiploidy or BCR::ABL1 fusion undergoing allogeneic HSCT. Blood 2025 DOI: 10.1182/blood.2025030951
3. Kalwak K et al. Comparable outcomes after busulfan- or treosulfan-based conditioning for allo-HSCT in children with ALL: results of FORUM. Blood Adv. 2025 Feb 25;9(4):741-751
4. Pearson ADJ et al. New models for the development of and access to CAR T-cell therapies for children and adolescents with cancer: an ACCELERATE multistakeholder analysis. Lancet Oncol. 2025 Apr;26(4):e214-e224
5. Matt MG et al. Allogeneic haematopoietic stem-cell transplantation for children with refractory systemic juvenile idiopathic arthritis and associated lung disease: outcomes from an international, retrospective cohort study. Lancet Rheumatol. 2025 Apr;7(4):e243-e251
6. Mellor-Heineke S et al. Genetic and clinical characteristics of patients with Shwachman Diamond syndrome with special consideration of treatment with granulocyte-colony stimulating factor. Haematologica. 2025 Sep 1;110(9):2171-2175.
7. Kamari-Kany N et al. Increased Plasma ST2 Levels Precede the Development of Severe Acute GvHD and Chronic GvHD After Pediatric Hematopoietic Stem Cell Transplantation. Pediatr Blood Cancer. 2025 Nov;72(11):e31995
8. Horan DE et al. Syndecan-1 is Associated With Sinusoidal Obstruction Syndrome and Severe Acute Graft-Versus-Host Disease After Pediatric Hematopoietic Stem Cell Transplantation. Pediatr Blood Cancer. 2025 Oct;72(10):e31943

9. Weischendorff S et al. Diagnosing Acute Graft-Versus-Host Disease in Children Undergoing Hematopoietic Stem Cell Transplantation-Comparison of the Modified Glucksberg Criteria Versus the MAGIC Criteria. 2025 Mar;72(3):e31502

10. Maffei M et al: Interplay between Acute Graft-Versus-Host Disease and Graft-Versus-Leukemia effect in Pediatric Acute Myeloid Leukemia Patients Undergoing Allogeneic Hematopoietic Stem Cell Transplantation: Implications for Relapse Incidence and Survival – An EBMT PDWP Retrospective Study. BMT, 2025 Dec 1. doi: 10.1038/s41409-025-02748-2. Online ahead of print. PMID: 41326788

Looking Ahead to 2026:

- Initiation of the NATURE study: Neurocognitive deficit After paediatric Transplantation: Understanding the Role of Environment and physical function: a prospective follow-up cohort, that will evaluate the complex interplay of toxicity, physical activity, parental distress and environmental factors on patient outcome.
- Establishment of RH primary immunodeficiencies consortium.
- Initiation of 5 investigator initiated trials: Forum 2, CARS4ALL_01, VHR CAR CD19, NBL CD2 CART, BuGenes.
- Initiation of new updated European protocol and database for children with severe aplastic anaemia (EWOG-SAA).



Danish Paediatric Pulmonary Service

Group Leader: Professor Kim G Nielsen (MD, Dr.Med.Sci.)

Team: Marianne Skov, chief physician, PhD, Frederik Buchvald, chief physician, PhD, Maria Nøregård Jørgensen, Cand. Scient in Human Physiology, 4 ongoing PhD students, & 8 research year students.

Contact: Kim.G.Nielsen@regionh.dk

Strategies and Core Areas of Research:

The Danish Paediatric Pulmonary Service (Clinic for Children and Adolescents with Pulmonary Diseases) includes the Danish PCD & chILD Centre and CF Centre Copenhagen and is the only centre of its kind in Denmark. It is internationally recognised for treatment outcomes and research in cystic fibrosis (CF) and primary ciliary dyskinesia (PCD) and is a leading centre in Northern Europe for children's interstitial lung diseases (chILD). The clinic integrates the Respiratory Physiology Laboratory 5003, a unique facility providing advanced physiological measurements of lung disease, and contributes actively to specialist training in paediatric pulmonology.

The centre is embedded in international networks, including ERN Lung (CF and PCD) and the ChILD EU Register. Kim Gjerum Nielsen serves as Director of the PCD Clinical Trial Network within ERN Lung, while Marianne Skov is a member of the CF Clinical Trial Network under the European Cystic Fibrosis Society. Frederik Buchvald represents the centre in the PELICAN collaboration (Prematurity's Effects on the Lungs in Children and Adults Network), a European Respiratory Society Clinical Research Collaboration. National collaborations include Odense University Hospital (tracheomalacia and oesophageal atresia) and Aarhus University Hospital (congenital lung malformations and tracheomalacia).

In line with these strategic priorities, the research portfolio focuses on early disease mechanisms, advanced diagnostics, and personalised treatment across the lifespan. The PROPEL programme and the CPH-LUNG cohort address early lung disease in premature and term-born infants using risk stratification and objective lung function assessment with Multiple Breath Washout (MBW), complemented by international benchmarking through PELICAN.

Advanced phenotyping is pursued through the DYNAMI4KIDS and AIRWAY studies, establishing 4D CT as a safe and clinically applicable tool for dynamic airway imaging, and through photon-counting CT to characterise pancreatic and airway structures in children with CF receiving CFTR modulator therapy.



Disease-specific clinical research includes the TRALULALA programme, investigating lung injury and airway dynamics in patients with oesophageal atresia, and the PORTRAIT trial, evaluating whether primary posterior pexia can prevent or reduce tracheal collapse in newborns with oesophageal atresia and tracheomalacia, with long-term follow-up. In CF, studies of CFTR modulator serum concentrations and salt homeostasis address treatment response and systemic physiology in the era of highly effective modulator therapy.

Mechanistic and translational research is conducted through the Spheroid II organoid programme, exploring mRNA-based therapies as part of a personalised medicine strategy, and through genetic and longitudinal lung function studies in PCD linking molecular variation with clinical phenotype and disease progression.

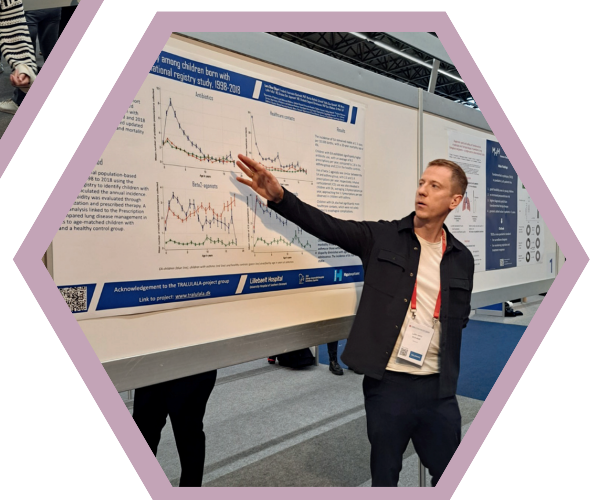
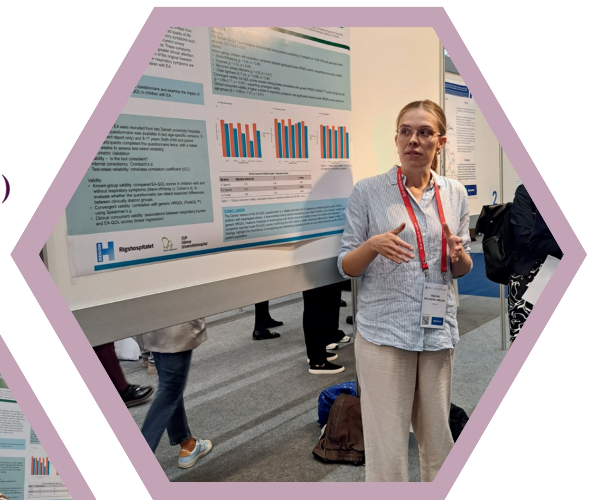
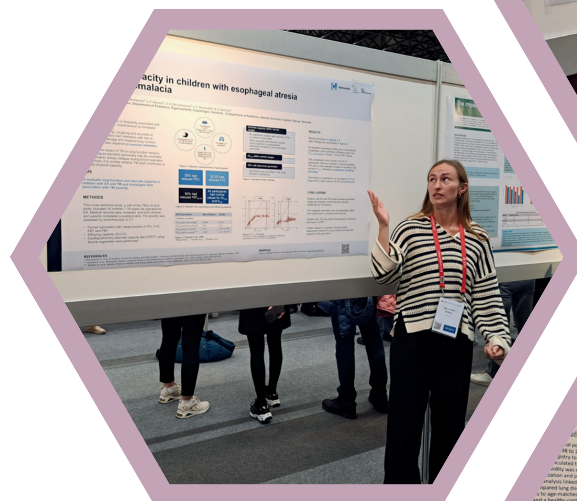
Together, these programmes translate strategic priorities into integrated clinical and translational research, reinforcing the centre's position as a leading European hub for paediatric respiratory medicine.

New Partnerships in 2025:

- Lotte Borgwardt, Consultant Radiologist at the Department of Radiology, and her specialised imaging team became established collaboration partners during 2025. The team provides advanced imaging expertise using 4D CT for high-resolution assessment of the trachea, main bronchi, and adjacent anatomical structures, thereby improving the characterisation of complex airway pathology.
- Katrine Løfgren, Consultant Paediatric Surgeon, together with colleagues from the Department of Paediatric Surgery at Rigshospitalet, became key collaboration partners during 2025 with substantial involvement in the TRALULALA study. In particular, she has played a pivotal role in securing Rigshospitalet's continued and future participation in the PORTRAIT trial (see further details below).
- Lise Aunsholt and Christian Heiring, Consultant Neonatologists, are new collaboration partners within the research programme "PROPEL – Prevention, Risk Stratification, Objective Markers and Prognosis." This programme investigates lung injury and early lung function in extremely, moderately, and late preterm infants as well as term-born children, using Multiple Breath Washout (MBW) and longitudinal follow-up of lung development during the first years of life. Several PhD and Master's projects are currently embedded within this programme.
- Jonathan F. Carlsen and colleagues at the Department of Neuroradiology are strategic collaboration partners in studies addressing the potential development of ventriculomegaly in patients with primary ciliary dyskinesia (PCD). This collaboration builds on their specialised expertise in automated quantitative assessment of the ventricular system on CT imaging.
- Søren Tvorup Christensen and the Cilia Group, University of Copenhagen, are essential collaboration partners in the forthcoming Spheroid II organoid study, which aims to evaluate mRNA-based therapeutic approaches as part of a personalised medicine strategy (see further details below).

Highlights of 2025:

- **Safety And Effect On Mucociliary Clearance Of RCT1100, An Inhaled Lipid Nanoparticle DNAAI mRNA, In Patients With Primary Ciliary Dyskinesia Due to Defect in the DNAAI gene: An Open-label, Multiple-dose, Phase 1b Trial – study completed.**
- **Neutrophil Serine Protease Inhibitor for bronchiectasis patients – including patients with Primary Ciliary Dyskinesia - Airleaf and Clairleaf RCTs – still ongoing.**
- **Reducing outpatient clinic visits for children with CF under cover of ETI modulator therapy. Poster prize at the international European CF Conference (Alexander Rotenberg, Research Year).**
- **Neutrophil Serine Protease Inhibitor (NSPi) for bronchiectasis patients – AIRTIVITY initiated for adults with bronchiectasis – also PCD and CF.**
- **CFTR Modulator therapy (ETI) for 1-2 year olds with CF.**
- **TRALULALA pilot study completed and closed for recruitment. The project is now in the phase of data analysis, preparation of a Master's thesis, and dissemination of results many presentations at both national and international conferences, autumn 2025.**
- **TRALULALA cross-sectional study was initiated in August 2025 and is currently running across all three participating sites (Aarhus University Hospital, Odense University Hospital, and Rigshospitalet). Patient inclusion and data collection are ongoing at all centres.**
- **Enhancing Lung Health in Kids with structural Lung Damage & Malformations: RCT on Azithromycin for Airway Infection Prevention received regulatory approval from the European Medicines Agency (EMA) in November 2025.**



Funding received in 2025:

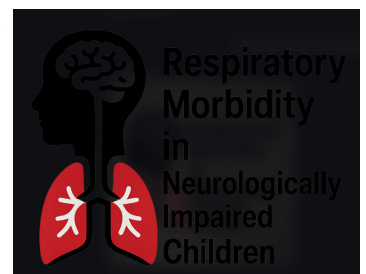
- Rigshospitalet Research Fund, BørneLungeFonden – Children's Lung Foundation, Cystic Fibrosis Foundation & Dagmar Marshall's Fund.

Key Publications in 2025:

- Brustad N, Buchvald F, Jensen SK, et al. Burden of Infections in Early Life and Risk of Infections and Systemic Antibiotics Use in Childhood. JAMA Netw Open. 2025;8(1):e2453284. Published 2025 Jan 2. doi:10.1001/jamanetworkopen.2024.53284
- Schramm A, Raidt J, Riepenhausen S, et al. Limitations of PICADAR as a diagnostic predictive tool for primary ciliary dyskinesia. Front Mol Biosci. 2025;12:1691758. Published 2025 Dec 8. doi:10.3389/fmolb.2025.1691758
- Råket HK, Jensen CB, Wang JN, et al. Impact of elexacaftor/tezacaftor/ivacaftor on utilization of routine therapies in cystic fibrosis: Danish nationwide register study. J Cyst Fibros. 2025;24(1):105-111. doi:10.1016/j.jcf.2024.11.004
- Meyer SIR, Skipper MT, Albertsen BK, et al. Late pulmonary adverse effects in childhood and adolescent acute lymphoblastic leukaemia survivors: a cross-sectional ALL-STAR Lungs study. ERJ Open Res. 2025;11(6):00350-2025. Published 2025 Dec 1. doi:10.1183/23120541.00350-2025
- Ring AM, Vestergaard M, Bøge IS, Buchvald FF, Nielsen KG. Mental Health in a Diverse Group of Children, Adolescents, and Adults Previously Treated for Childhood Interstitial Lung Disease. Pediatr Pulmonol. 2025;60(10):e71356. doi:10.1002/ppul.71356
- Chalmers JD, Mall MA, Nielsen KG, et al. Neutrophil-derived biomarkers in bronchiectasis: identifying a common therapeutic target. Eur Respir J. 2025;66(3):2500081. Published 2025 Sep 17. doi:10.1183/13993003.00081-2025
- Altaraihi S, Marthin JK, Anagnostopoulou P, et al. International consensus statement on routine blood testing in primary ciliary dyskinesia. ERJ Open Res. 2025;11(3):01071-2024. Published 2025 Jun 23. doi:10.1183/23120541.01071-2024
- Henriksen EH, Sandvik RM, Olsen MF, et al. Real-World Improvements of Lung Clearance Index and Ventilation Distribution Efficiency in Children With Cystic Fibrosis After Elexacaftor/Tezacaftor/Ivacaftor Initiation. Pediatr Pulmonol. 2025;60(6):e71166. doi:10.1002/ppul.71166
- Marczak H, Krenke K, Griesse M, et al. Diagnostic Evaluation and Clinical Findings in Children With Persistent Tachypnea of Infancy/Neuroendocrine Cell Hyperplasia of Infancy: A European Multicenter Retrospective Study. Chest. 2025;168(1):171-182. doi:10.1016/j.chest.2025.02.023
- Holgersen MG, Marthin JK, Raidt J, et al. Long-Term Lung Function and Pseudomonas aeruginosa Infection in Genotyped Primary Ciliary Dyskinesia. Ann Am Thorac Soc. 2025;22(2):216-225. doi:10.1513/AnnalsATS.202404-340OC

Looking Ahead to 2026:

- **PROPEL study**
 - Prevention, Risk Stratification, Objective Markers and Prognosis of Early Lung Disease in Premature Infants
- **Initiation of the CPH (Copenhagen Premature Health) project, including establishment of the CPH-LUNG cohort with lung function measurements using Multiple Breath Washout (MBW) in healthy term newborns and preterm infants. In collaboration with the Neonatal Department, Rigshospitalet (Lise Aunsholt and Christian Heiring), and PhD student Emma Therese Bay.**
- **PELICAN (Prematurity's Effects on the Lungs in Children and Adults Network) collaboration.**
 - A preterm model of care: initial international survey of current clinical practices worldwide.
- **BRANCH study (Burden of Respiratory Morbidity among Neurologically Impaired Children)**
 - Medical students: Ida Stampe Frederiksen; Josephine Mølgaard
 - In collaboration with the Department of Paediatric Neurology (Peter Born).
- **PEARL study**
 - Physical Capacity and Exercise Assessment in Respiratory Tract and Lung Diseases
 - Medical students: Katinka Emilie Tang Christensen; Helena Winther Hytoft; and specialised lung physiotherapist Lue Katherine Philipsen).
- **CONNECT study**
 - Rigshospitalet participates in the international CONNECT trial initiated by Erasmus Medical Center, Rotterdam, randomising infants to conservative or surgical management in cases of uncomplicated congenital cystic lung malformations.
- **4D CT studies – DYNAMI4KIDS & AIRWAY**
 - **DYNAMI4KIDS:** Aims to document that 4D CT can be safely and effectively performed in children, producing high-quality diagnostic images of airway dynamics with minimal radiation exposure and limited need for sedation.
 - **AIRWAY:** To quantify dynamic tracheal and central airway collapse during respiration using 4D CT.
 - In collaboration with the Department of Radiology, Rigshospitalet (Lotte Borgwardt; Andreas Leithe; Thomas Christensen; Apameh Khatam-Lashgari).





- **Photon-counting CT study**
 - Assessment of the association between increasing faecal elastase levels and pancreatic structure in paediatric patients with cystic fibrosis receiving triple CFTR modulator therapy (ETI).
 - Medical student: Alexander Rotenberg.
 - In collaboration with the Department of Radiology, Rigshospitalet (Lotte Borgwardt) and Herlev Hospital (Erik Gudman; Steuble Brandt; Michael Brun Andersen).
- **Spheroid II organoid study**
 - Evaluation of mRNA-based therapeutic approaches as part of a personalised medicine strategy.
 - Conducted by PhD student Caroline Nygaard in collaboration with partners listed above.
- **CFTR modulator serum concentration studies**
 - Initiation of studies on serum concentration measurements of CFTR modulators to establish clinical and research applicability.
- **The Cystic Fibrosis Salt Study**
 - Investigation of body salt homeostasis in children with cystic fibrosis in the era of CFTR modulator therapy.
- **TRALULALA study**
 - National cross-sectional and longitudinal studies investigating lung injury, lung function, and dynamic tracheal changes in patients with congenital oesophageal atresia
- **PORTRAIT study**
 - This trial aims to evaluate whether primary posterior pexia can significantly reduce or prevent tracheal collapse in newborns with oesophageal atresia and tracheomalacia. In addition, the trial seeks to determine whether the stabilising effect on tracheal integrity is sustained over time.

Awards and Prizes 2025:

Professor Heymut Omran (University Hospital Münster) has received Rigshospitalet's prestigious international KFJ Prize for his pioneering research in rare lung diseases, particularly primary ciliary dyskinesia (PCD). The award recognises his long-standing and highly productive collaboration with Rigshospitalet, which has advanced diagnostics and enabled the development of mRNA-based therapy, now having completed its first clinical phase. The prize is also granted as an incentive to further strengthen and expand collaboration with the Danish Pediatric Pulmonary Service, supporting continued translational research and the development of new treatments for children with rare respiratory diseases.



Danish Sleep Center for Children & Adolescents

Group Leader and team members:

Nanette Debes, MD, PhD (Group Leader, 50%) & Peter Born, MD, PhD (20%)

Contact: nanette.marinette.monique.debes@regionh.dk

Strategies and Core Areas of Research:

The Danish Sleep Center for Children and Adolescents (DSBU) was established following a donation from the Lundbeck Foundation in September 2025. The center is a collaboration between Herlev and Gentofte Hospital and Rigshospitalet, and aims to serve as a national reference center for pediatric sleep, supporting research as well as health promotion, diagnostics, and treatment across Denmark.



The Center's primary area of research is:

- Longitudinal cohort studies of sleep, cognition and health in children and adolescents with and without chronic disease and healthy control subjects
- Sleep, mental and physical health in a parent/child cohort from birth
- Development and implication of new devices for study of sleep in children from birth
- Prevalence of sleep disturbances in children with disease assessed by a standardized questionnaire
- Development of artificial intelligence methods to evaluate sleep in children and adolescents

Cumulated Funding Received in 2025:

- The Lundbeck Foundation: 2.676.000 Euro
- Rigshospitalets Research Foundation: 33.450 Euro

Highlights of 2025:

In 2025, DSBU was established, and the management team and scientific committee started working.

Looking ahead to 2026:

- Finalizing protocols and regulatory approval for trials.
- Employment of one ph.d. student, one post-doc and one research assistant.
- Start recruitment for the cohort studies.
- Finalizing the "prevalence of sleep disturbance in chronic disease" project.



Global Health Unit

Group Leader: Anja Poulsen

Steering Group Members: Stine Lund, Jesper Kjærgaard, Vibeke Brix Christensen, & Freddy Karup Petersen

Team: Approx. 20 part-time physicians and 10 medical students.

Contact: globalhealth.rigshospitalet@regionh.dk

Website: <https://www.rigshospitalet.dk/afdelinger-og-klinikker/julianemarie/enhed-for-global-sundhed/sider/default.aspx>

Strategies and Core Areas of Research:

1. Research in Low- and Middle-Income Countries

- Implementation research, including the use of new technologies.
- Focus on infectious and respiratory diseases in children under five years old.
- Perinatal and neonatal diseases aimed at improving survival rates.

2. Networking and Education

- Strengthening collaboration in Denmark and with institutions in low- and middle-income countries, including partnerships with the University of Copenhagen.

3. Knowledge Dissemination

- Organizing seminars and courses to share knowledge and raise awareness of global child health issues.

4. Internal Collaboration

- Promoting cooperation in Global Health at Copenhagen University Hospital, Rigshospitalet, and the Capital Region.

Key Partnerships

- ICARS (International Center for Antibiotic Resistance Solutions): Zanzibar and Kyrgyzstan projects.
- University of Copenhagen: Collaboration with the Department of General Practice.
- Public Health Laboratory, Ivo de Carneri, Pemba, Zanzibar (PHL).
- Kyrgyz Thoracic Society, Bishkek, Kyrgyzstan.
- The Danish-Vietnamese Friendship Association.
- Catholic University of Health and Allied Sciences (CUHAS), Mwanza University, Tanzania.



Current Research Collaborations

- **Kyrgyzstan:**
 - Focus on point-of-care testing and reducing antibiotic use (follow-up to the EU FRESH-AIR Project).
 - 2025: New study on altitude and child health, investigating lung function in mountain areas; extended study planned in 2026.
 - Two additional qualitative studies on healthcare-seeking behaviour have been conducted.
- **Zanzibar:**
 - Ongoing research on neonatal and child health, including quality of care for low birth weight (<2500g) and sick term newborns, respiratory challenges in children, and healthcare-seeking behaviour.
 - Testing innovative techniques to strengthen healthcare staff knowledge and skills, including Virtual Reality-based neonatal resuscitation training.
 - In 2025, further studies on managing respiratory challenges in children and healthcare-seeking behaviour will commence.
 - Completion of data collection for a randomised controlled trial focusing on Antimicrobiological resistance and point-of-care CRP to reduce antibiotic use in neonates and children at the hospital level; results are being written up by local PhD student Daniel Joshua in collaboration with ICARS, PHL and CUHAS.
- **Tanzania:** Nutrition projects in Mwanza.
- **Vietnam:** Collaborative studies on child health and children's rights.
- **Greenland:** Research on tuberculosis and prophylaxis.

Funding received in 2025

- Dagmar Marshall Foundation
- Holms Memorial Fund
- Beckett Foundation,
- Pregraduate funding from Nordsjællands Hospital x 2



Highlights of 2025

- In January, a week-long course in International Child Health and Refugee Health in Bangladesh, in cooperation with ICDDR,B (the former cholera institute).
- Building Bridges Project: Exchange of 6 medical students and 4 nursing students to Kyrgyzstan and Pemba; 3 medical students have defended their theses.
- September: Workshop arranged by students at Rigshospitalet, presenting their research and experience.
- Collaboration meetings in Tanzania with Stine Lund and Anja Poulsen.
- Extension of VR neonatal training in Pemba, Tanzania, with student Oliver Bonnesen.
- Visits from CEO Dr Tahmeed (Bangladesh) and partners from Kyrgyzstan (Nargiza Osmonbaeva) and Pemba (Farid Said) presenting at DIPS conference.





Key Publications in 2025

- Elvira Isaeva et al., Poulsen A., Kjærgaard J. C-reactive protein testing in primary care and antibiotic use in children with acute respiratory tract infections in Kyrgyzstan: an open-label, individually randomised, controlled trial. *Lancet Reg Health Eur.* 2025;51:101184.
- Lassen ML, Larsen LV, Poulsen A., Lund S. Healthcare providers' knowledge and quality of neonatal care in the first 2 h of life in a district hospital on Pemba Island, Tanzania: a prospective cohort study. 2025.
- Osmonbaeva N. et al., Poulsen A., Kjærgaard J. Microbiological aetiology of paediatric respiratory tract infections in Kyrgyzstan. *BMC Infect Dis.* 2025;25(1):280.
- Daniel Joshua et al., Poulsen A., Lund S. Serial C-Reactive Protein Point-of-Care testing to optimize antibiotic treatment in hospitalized children in Zanzibar: A Feasibility Study. *PLOS Global Public Health*, 2025.
- Daniel Joshua, Jeremiah Seni , Poulsen A., Lund S. Effectiveness of the C-reactive protein point-of-care testing in optimizing antibiotic treatment in neonates and children: an open-label, individual randomized controlled clinical trial protocol. *BMC Pediatrics*, accepted 2025.

Looking Ahead to 2026

- Conduct International Child Health course in Vietnam, in collaboration with Paediatric Hospital No.1, Ho Chi Minh City.
- Finalise Building Bridges exchange program under Globus Fund, including 10 medical and nursing students; evaluation of the program.
- Complete the remaining Master's theses under Building Bridges and publish results.
- Secure funding for a PhD project on expanding VR training in low-income countries; Hanna Rahimi to conduct a study in the Rohingya camp, Bangladesh.
- Publish results of the randomised Zan-Toto CRP study in Zanzibar.
- Compile qualitative studies from the COORDINATE study in Kyrgyzstan.
- Finalise second VR study in Pemba, Tanzania.



Hepatology & Gastroenterology



Group Team: Marianne Hørby, MD PhD, consultant (Hepatology), Bente Utoft-Andreasen, MD PhD senior consultant (Gastroenterology) and Vibeke Brix (Hepatology)
Contact: marianne.hoerby-joergensen@regionh.dk,
bente.utoft.andreassen@regionh.dk, vibeke.brix.christensen@regionh.dk

Strategies and Core Areas of Research:

The group has a long-standing tradition of integrating research with clinical practice and participates in various national and international research collaborations within general hepatology and gastroenterology in paediatrics.

Key research topics include:

- Acute Liver Failure - treatment strategies
- Autoimmune liver diseases
- Liver disease related to Cystic Fibrosis
- Safety in invasive diagnostic procedures
- Infant proctocolitis
- The role of autoantibodies in Type 1 Diabetes
- 'Little Livers' (see next page)

Highlights from 2025

3 significant publications on autoimmune hepatitis, all reflecting the group's active role in both collaborative clinical research and the development of international standards of care:

- Jørgensen MH, Almaas R, Kharrazi G, Urbonas V, Kvistgaard H, Wollen E, Andreassen BU, Casswall T, Fischler B. Various Regimens for Autoimmune Hepatitis in Northern European Children Show Equivalent Outcomes at 1 Year: A Retrospective Study. *J Pediatr.* 2025 Sep;284:114635.
- Cananzi M, Jørgensen MH, Buescher G, De Bruyne R, Samyn M; ESPGHAN Hepatology Interest Group and the ERN RARE-LIVER Autoimmune Hepatitis Working Group. Current practice in the management of paediatric autoimmune liver disease in Europe. *J Pediatr Gastroenterol Nutr.* 2025 Feb;80(2):260-270.
- European Association for the Study of the Liver. EASL Clinical Practice Guidelines on the management of autoimmune hepatitis. *J Hepatol.* 2025 Aug;83(2):453-501. doi: 84.

The group contributed to recommendations on the transition of young people with liver disease:

- Vanden Wyngaert K, Suykens J, Kelly D, Goncalves I, Thomsen EL, Willemse J, Mariño Z, Madaleno J, Day J, Samyn M, Ali M, Jørgensen MH, Goossens E, De Munter, J, Staa AV, Wray J, Protopapa P, Lohse A, De Bruyne R. Pediatric to adult care' transfer in rare liver diseases: Recommendations based on insights from a European omnistakeholder event. *JHEP Rep.* 2025 Mar 26;7(6):101403

The group participated in a European survey study highlighting variability in the management of portal hypertension across European countries:

- Roilidis I, Ackermann O, De Bruyne R, Illhardt T, van der Doef H, Fitzpatrick E, Sotiriadou F, Jørgensen MH, Liccardo D, Mercadal-Hally M, Mtegha M, DiGiorgio A, Grammatikopoulos T. Variability in the management of portal hypertension across European countries: A survey-study by ESPGHAN Portal Hypertension Special Interest Group. *J Pediatr Gastroenterol Nutr*.

Marianne Hørby Jørgensen has supervised the PhD from the Cystic Fibrosis Centre about Cystic Fibrosis related liver disease by Christene Højde (main supervisor: Marianne Skov, Pediatric Pulmonology) with 2-3 publications.

Looking Ahead to 2026

- Starting a PhD project on Phenotype and diagnosis of autoimmune liver disease in combination with inflammatory bowel disease (collaboration with Hvidovre BørneUnge afdeling). Physician Fie Tinging started as a PhD student 1 January 2026.
- An ERN project as co-editor for a textbook titled Rare and complex liver diseases.
- Two projects about the treatment of Autoimmune liver disease.
- Epidemiological studies of pediatric autoimmune liver diseases (Collaboration with Hvidovre).
- Development of a common protocol for high-volume plasma exchange in acute liver failure, along with several projects connected to the ERN Rare Liver database.
- Amino acid profile in children with acute liver failure.



*The European working group on plasma exchange in pediatric acute liver failure
(3rd face-2-face meeting, October 2024)*

Little Livers



Group Leader: Vibeke Brix Christensen

Group Members:

1 Postdoctoral Researcher, 2 Research Year Students, 1 Senior Research Assistant, 1 Master's Thesis Student & 1 Research Assistant.

Contact: Vibeke.Brix.Christensen@regionh.dk

Strategies and Core Areas of Research:

Little Livers focuses on identifying prognostic markers for disease progression and complications in paediatric chronic liver disease (PCLD). Key complications include immune dysfunction, impaired neurocognitive function, disturbances in the liver–gut–brain axis, altered body composition, and reduced quality of life.

Our primary goal is to identify risk factors for liver decompensation and genetic determinants of regenerative potential, supporting individualized, multidisciplinary clinical decision-making, including optimal timing of liver transplantation.

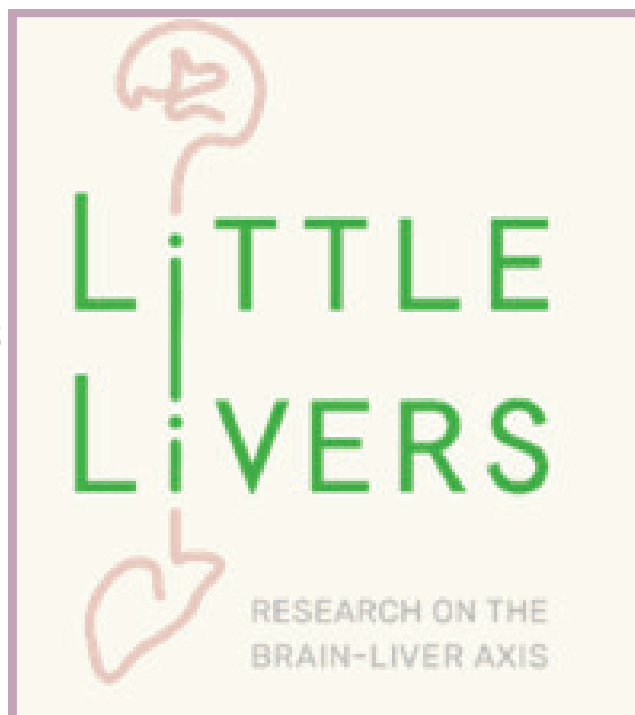
A central ambition is the development of an individualized risk prediction tool to identify infants at high risk of complications. Because paediatric liver care in Denmark is highly specialized and centralized, such a tool has strong translational potential and can be implemented directly in clinical practice.

In addition, the group investigates causes of liver dysfunction—including infectious hepatitis, steatosis, and drug-induced liver injury (e.g., paracetamol overdose)—with a focus on cost-effective and family-friendly monitoring strategies.

Highlights of 2025:

In 2025, the group strengthened its national and international research profile:

- Expanded collaboration with basic scientists to advance research on the liver–gut–brain axis
- Participated in field visits in low-income countries to strengthen global research partnerships including supervision of Hepatitis C treatment in refugee camp
- Completed inclusion of 100 children with PCLD (biliary atresia) and controls in the multidisciplinary BILACO research program and started follow-up.
- Continued scientific collaboration with CVT (Center for Science and Faith), Copenhagen University.





Key Publications in 2025:

- Helt TW, Kurtzhals J, List KK, et al. Hepcidin is low in children with moderate acute malnutrition and asymptomatic malaria: secondary analysis of a 2×2×3 factorial randomised trial in Burkina Faso. *Br J Nutr*. Published online November 17, 2025. doi:10.1017/S0007114525105679
- Winther CL, Helt TW, Fabiansen C, et al. Liver function tests among children with moderate acute malnutrition: A secondary analysis of a randomized trial from Burkina Faso. *J Pediatr Gastroenterol Nutr*. 2025;81(5):1280-1286. doi:10.1002/jpn3.70177
- Hukkinen M, Fischler B, Liliemark U, et al. Multicenter Study on Early Predictors of Biliary Atresia Outcomes. *J Pediatr Surg*. 2025;60(9):162404. doi:10.1016/j.jpedsurg.2025.162404
- Kempf ON, Helt TW, Herrche-Petersen JJ, et al. Paracetamol Overdoses Among Danish Adolescents: A COVID-19 Perspective. *Acta Paediatr*. 2025;114(8):1839-1846. doi:10.1111/apa.70037
- Ficerai-Garland G, Helt TW, Nielsen J, et al. Indocyanine green clearance's association with liver function in paediatric liver disease. *Acta Paediatr*. 2025;114(5):980-985. doi:10.1111/apa.17518
- Lindebjerg C, Helt TW, Christensen VB. Factors Involved in Thymic Atrophy in Severely Malnourished Children: A Systemic Review and Meta-Analysis. *Scand J Immunol*. 2025;101(3):e70014. doi:10.1111/sji.70014
- Helt TW, Cichon B, Fabiansen C, et al. Serum insulin-like growth factor 1 in young children with moderate acute malnutrition: secondary analysis of a randomised trial in Burkina Faso. *Br J Nutr*. 2025;133(4):481-490. doi:10.1017/S0007114525000212
- Grarup KR, Schwartz Sørensen S, Perch M, et al. Adenovirus Infections and Disease in Solid Organ Transplant Recipients: Incidence and Outcomes. *Open Forum Infect Dis*. 2025;12(2):ofaf032. Published 2025 Jan 20. doi:10.1093/ofid/ofaf032
- Esmann FVL, Zahid S, Moestrup KS, et al. Management of Post-transplant Infections in Collaborating Hospitals (MATCH) Programme: a prospective cohort of all transplant recipients at Copenhagen University Hospital-Rigshospitalet, Denmark. *BMJ Open*. 2024;14(11):e089966. Published 2024 Nov 13. doi:10.1136/bmjopen-2024-089966



Looking Ahead to 2026:

In 2026, *Little Livers* will expand its longitudinal, translational, and global research efforts:

- Launch a 10-year follow-up study of 180 children with acute and chronic liver disease, focusing on prognostic markers for long-term progression
- Continue the study of liver steatosis in children with malnutrition in low-income countries
- Advance the BILACO program on biliary atresia and liver transplantation, emphasizing changes in the liver–gut–brain axis
- Continue research on growth and development in children with PCLD to clarify interactions between liver function, nutrition, and overall development
- Follow up on outcomes after hepatitis C treatment with direct-acting antivirals (DAA) in Denmark
- Integrate perspectives on science and faith into selected research initiatives, addressing existential and holistic dimensions of chronic paediatric liver disease, including the MuSICK project on live music for children and families admitted to semi-intensive care unit.



Medical Education & Patient Experiences

Anja Poulsen, MD, PhD, responsible for postgraduate education
Team: Line Klingen Gærde, MD, PhD, Jesper Kjærgaard, MD, PhD, Jette Led Sørensen, MD, Professor, Raheel Raja, MD, PhD, & Jon Nielsen, MD, PhD, Four PhD students, and two master students.
Contact: Anja.Poulsen.01@regionh.dk

Strategies and Core Areas of Research:

The team is responsible for the postgraduate training of paediatric residents at the department and research related to education and patient experiences.

Funding received in 2025:

- XR to Learn – EU Project NEONATAL: VR-based Neonatal Resuscitation Scenario.
 - The project focuses on developing an extended reality (XR)–based training module for neonatal resuscitation. It will be evaluated through a randomized study conducted in Denmark and a parallel randomized study in Bangladesh.
- Total EU funding requested: EUR 300.000, with 60% allocated to development.
- Børneriget Fonden approx. EUR 40.000.



Key Publications in 2025:

- Sohlin AM, Anja Poulsen, Ida Madeline Hoffman, Line Klingen Gjørde, Todd P. Chang, Joy Yeonjoo Lee, Stine Lund, Lone Poulsen, Jette Led Sørensen, Jesper Kjærgaard: *Effects of self-guided versus facilitator-guided debriefing in immersive virtual reality simulation on medical students' teamwork skills: A study protocol for a randomized controlled non-inferiority trial* PLOs One. September 12, 2025
<https://doi.org/10.1371/journal.pone.0332309>
- Sohlin AM, Kjærgaard J, Hoffman IM, Chang TP, Poulsen A, Lee JY, Gjørde LK, Lund S, Paulsen L, Sørensen JL, Overbeck G *Immersive virtual reality training: Addressing challenges and unlocking potentials*. Med Educ. 2025 Jun 15. doi: 10.1111/medu.15748. Online ahead of print.PMID: 40518687
- Thestrup J, Sørensen JL, Bidstrup PE, Rosthøj S, Hybschmann J, Esbjørn BE, Dybdal D, Barslund K, Topperzer MK, Frandsen TL, Gjørde LK. *Effect on healthcare professionals' self-efficacy of a 45-min versus 3-hour educational intervention on communication and play in paediatric clinical practice in Denmark: a randomised controlled trial study protocol*. BMJ Open 2025;15:e101703.

Ongoing PhD projects 2025:

1. Simulation-based training in paediatric emergency medicine – PhD project by Amalie Middelboe Soehlin.
 - a. This project investigates the use of simulation-based training in acute paediatrics to enhance healthcare professionals' ability to manage paediatric emergencies.
 - b. The training addresses both technical skills, such as treatment and resuscitation, and non-technical skills, including communication and teamwork.
 - c. By simulating real-life emergency scenarios, the project aims to improve professionals' responses to acute situations.
 - d. Preliminary results indicate that simulation training enhances skill development and contributes to improved patient safety in paediatric care
2. NEONATAL: Simulated Neonatal Resuscitation Training for Healthcare Workers Using Virtual Reality - PhD project by Hanna Rahimi in cooperation with Department of Paediatrics, Hillerød Hospital
 - a. This project explores the use and dissemination of VR in Denmark and Bangladesh in Neonatal resuscitation
3. A playful approach as a strategy to promote paediatric patients' right to participation in treatment and care - PhD project by Jakob Thestrup Hansen
 - a. This PhD explore whether training healthcare professionals in communication and play can increase their self-efficacy in communicating with children and adolescents
 - b. The project is integrated into the development and testing of the interprofessional educational programme called "Communication and play with children and adolescents in the healthcare setting"
 - c. The research integrates perspectives from both healthcare professionals and children and adolescents
 - d. Evaluation of the educational programme includes a randomized controlled trial comparing a brief (45-minute) intervention with an extended (three-hour) intervention.

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Neuropaediatric Research Unit & Center for Cerebral Palsy and Early Brain Damage



Group Leaders: Christina E. Høi-Hansen, MD, DMSc, Professor & Jakob Lorentzen, MSc, PhD, Professor

Team: The unit encompasses 2 professors, 3 consultant neuropaediatricians, 3 senior researchers/post docs, 12 phd students, 6 scientific staff members, 3 clinical trial unit nurses, one secretary and 3-6 master/bachelor students.

Contact: berit.kirkemann.hansen@regionh.dk

Strategies and Core Areas of Research:

The neuropaediatric research consists of the Neuropaediatric Research Unit, The Clinical Trials Unit for Children and Adolescents and the Cerebral Palsy research unit. There are several research focus areas on the multiple aspects of neurological disorders in children and adolescents.

The current research agenda is dedicated to advancing the understanding and management of paediatric neurological disorders, with a particular emphasis on brain injury and its associated comorbidities. Our investigations are distinguished by a translational approach, bridging fundamental scientific discoveries with clinical application to improve patient outcomes. The primary focus encompasses children and adolescents affected by complex and rare neuropaediatric conditions, notably cerebral palsy, epilepsy, and neuromuscular diseases.



A central tenet of our research is the early identification of at-risk children, aiming to refine diagnostic pathways for disorders that are often challenging to detect in the developing brain. This early intervention paradigm is complemented by efforts to optimize the transition from paediatric to adult care for individuals with neurological disorders, both in clinical practice and within research frameworks.

Our current portfolio also prioritizes the investigation of sleep patterns and disturbances in both neurotypical and neurologically affected paediatric populations. In this context, the establishment of the Danish Sleep Center for Children and Adolescents—realized through collaboration with Herlev and Glostrup hospitals—marks a significant advancement in the field.

In addition, our research unit has a close and long-standing collaboration with the Movement Disorders research group at the Department of Neuroscience, University of Copenhagen. Together, we investigate the pathophysiology, genetics, and treatment of movement disorders, including dystonia, with a particular focus on translational projects that bridge basic neuroscience and clinical neuropaediatrics.

The Clinical Trials Unit for Children, Adolescents, and Adults is actively engaged in pioneering therapeutic trials targeting neuropaediatric disorders. Ongoing studies include novel interventions for Duchenne muscular dystrophy, ataxia telangiectasia, and spinal muscular atrophy, reflecting our commitment to translational innovation.

Furthermore, our research initiatives extend across diagnostic categories, with interdisciplinary projects addressing nutrition, cognitive impairment, and bone health. These efforts are designed to ensure comprehensive, lifespan-oriented care and to foster optimal health trajectories for children with neurological conditions.

Main research areas include:

- **Cerebral palsy:**
 - Early diagnosis and intervention in infants at risk of cerebral palsy (The CP-EDIT trial). Including neuroprognostication in critically ill infants and children and stroke in childhood.
 - Evaluation of cognitive functioning in children with cerebral palsy (The CPCog study).
 - Epidemiological studies regarding features in pregnancy, birth and early life related to risk of subsequent neurodevelopmental disorders.
 - Motor functioning in cerebral palsy, intervention and establishment of home-training programmes.
 - Trial regarding transition from childhood to adulthood (The CP-UNG trial).



- **Epilepsy.** Including treatment response and outcome in severe epilepsy, e.g. infantile spasms and epilepsy surgery.
- **Neuromuscular disorders.** Including assessment of comorbidity, nutrition, bone health and treatment optimization.
- **Sleep as a key factor for mental and physical health in children with neurological disorders**
- **Clinical trial unit.** Industry sponsored and investigator initiated clinical trials including RNS splice modifiers and viral vector gene therapy. Including treatments for spinal muscular atrophy, Duchenne muscle dystrophy, ataxia telangiectasia, arterial ischaemic stroke and dyskinetic movement disorder.

New Partnerships in 2025:

- **Multicenter Natural History Study of Cerebral Palsy-** collaboration with Australia, Italy, Sweden, Norway, USA.
- **Collaboration on GMA and HAI multicenter and automatized evaluation of children at risk of cerebral palsy** with Trondheim, Norway and Karolinska, Sweden.
- **Collaboration on the ENVISAGE tool** with Sick Kids, Toronto, Canada.

Funding received in 2025:

- **Lundbeck Foundation 20 mio DKK ‘Danish Sleep Center for Children and Adolescents’**
- **Elsass Foundation 77 mio DKK ‘MARYs Center for Cerebral Palsy’**
- **Dagmar Marshall Foundation 300.000 DKK ‘Neuroprognostication’**
- **Elsass Foundation 1,1 mio DKK ‘AIM NICU: Prediction of CP in neonates’**
- **Elsass Foundation 1.6 mio DKK ‘Cerebral palsy in twins’**

Key Publications in 2025:

- **Esserlind AL, Schmidt LS, Rønde G, Miranda M, Jensen L, Bindslev JB, Born AP, Vissing NH, Møller FT, Møllerup JE, Børresen ML. *Pediatric varicella zoster virus associated arterial ischemic stroke - A post-pandemic rise in incidence in Denmark.* Eur J Paediatr Neurol. 2025 Nov;59:100-106. doi: 10.1016/j.ejpn.2025.10.010.**
- **Fogh MV, Wiingreen R, Ostenfeld A, Clausen TD, Krebs L, Petersen OB, Greisen G, Hoei-Hansen CE, Larsen ML. *School performance of children born to mothers with hyperemesis gravidarum.* AJOG 2025.**
- **Larsen ML, Krebs L, Greisen G, Rackauskaite G, Hoei-Hansen CE. *Prevalence and severity of cerebral palsy in Denmark in children born 2014-2018.* NexRes 2025.**
- **Larsen LH, Kirk H, Lorentzen J. *The feasibility and efficacy of coach-led virtual home-based cycling among individuals with cerebral palsy.* Front Neurol. 2025 Jul 15;16:1604061. doi: 10.3389/fneur.2025.1604061.**
- **Novosel IB, Christensen J, Jensen MK, Lorentzen J. *The effect of extrinsic feedback on improving functioning in people with congenital and acquired brain injury: a systematic review and meta-analysis.* Disabil Rehabil. 2026 Jan;48(1):94-107. doi: 10.1080/09638288.2025.2497461.**

- Pedersen SV, Sørensen JK, Fabricius R, Dunø M, Larsen ML, Hoei-Hansen CE, Østergaard E. *Systematic review of current literature on genetic etiology in children with high risk of CP*. Acta Paediatrica 2025
- Proud CM, Vũ DC, Wilmschurst JM, Sanmaneechai O, Gulati S, Xiong H, Moreno HC, Tay SKH, Thong MK, Born AP, Banzatto Ortega A, Jong YJ, Al-Muhaizea MA, Lee AW, Visootsak J, Tauscher-Wisniewski S, Alecu I, Parlikar R, Finkel RS; STEER Study Group. *Intrathecal onasemnogene abeparvovec in treatment-naïve patients with spinal muscular atrophy: a phase 3, randomized controlled trial*. Nat Med. 2025 Dec 8. doi: 10.1038/s41591-025-04103-w.
- Therkildsen ER, Nielsen JB, Lorentzen J. *The calcium channel blocker nimodipine inhibits spinal reflex pathways in humans*. J Neurophysiol. 2025 Feb 1;133(2):428-439. doi: 10.1152/jn.00585.2024.
- Zhao Q, Naume MM, de Winter BCM, Krag T, Haslund-Krog SS, Revsbech KL, Vissing J, Holst H, Møller MH, Hornsyld TM, Dunø M, Hoei-Hansen CE, Born AP, Jensen PB, Ørngreen MC. *Paracetamol and its metabolites in children and adults with spinal muscular atrophy - a population pharmacokinetic model*. Br J Clin Pharmacol. 2025 Jul;91(7):2045-2056. doi: 10.1002/bcp.70028.

Studies in the Clinical Trials Unit in 2025:

SPECTRUM spinal muskeltrofi type 2, open-label extension of previous study with AAV9 gene terapi, sponsor Novartis

SHIELD Duchenne muskeldystrofi projekt, phase 2b, 8-12 years, satrilizumab, an IL6 inhibitor, sponsor Roche

BRAVE Friedreich ataxia, phase 3, 2-16 years, placebo controlled, omaveloxolone, sponsor Biogen



STELLAR, newborns with screen-positive spinal muscular atrophy, phase 3, salanersen, an intrathecal antisense-oligonucleotide, sponsor Biogen

SPG50 hereditary spastic paraparesis with mutation in the AP4M1-gene, 6-18 years, intrathecal AAV9 gene therapy, investigator initiated trial

New Partnerships in 2025:

- Multicenter Natural History Study of Cerebral Palsy- collaboration with Australia, Italy, Sweden, Norway, USA.
- Collaboration on GMA and HAI multicenter and automatized evaluation of children at risk of cerebral palsy with Trondheim, Norway and Karolinska, Sweden.
- Collaboration on the ENVISAGE tool with Sick Kids, Toronto, Canada.

Highlights of 2025:

- The multicenter CP-EDIT trial in Copenhagen, Herlev, Aalborg and Aarhus: We have included all participants. Last follow-up visit is January 2027. Implementation studies are ongoing, including as part of new PhD study by neonatologist Anna Lewis.
- The clinical trial unit has become well established with increasing number of trials running and a close collaboration in all paediatric subspecialties.
- The Transition clinic for cerebral palsy has been established and now is a valued addition to clinical practice and a new platform for research projects in adolescents with CP.
- Funding has been obtained for establishment of the 'Danish Sleep Center for Children and Adolescents' and the first post doc and PhD student has been enrolled.
- Funding has been obtained for evolvement of the current CP clinic to the MARYs Center for Cerebral Palsy –national and international center of knowledge and treatment within CP. The aim is to create a research-, treatment-, and teaching environment to ensure that the treatment of children and adolescents with CP in Denmark is based on the latest knowledge and the most recognized international principles.

Looking Ahead to 2026:

We are looking forward to increased collaboration, new projects and are preparing for the MARYs Childrens Hospital.

Paediatric Care Research

Group Leader and Team Members: (8 part-time employees)

- Helena Hansson, senior researcher and clinical nurse specialist, associate professor
- Hanne Bækgaard, research leader of the psychosocial group at Bonkolab, assistant professor
- Ena Lindhart Thomsen, clinical nurse specialist, post.doc.
- Louise Roug, clinical nurse specialist, PhD
- Tenna Ruest Haarmark Nielsen, MD, PhD
- Rikke Thenning Michelsen, specialist nurse in home care
- Anne Thomasen, clinical nurse specialist, coming PhD-student
- Maria Nøregård Jørgensen, human physiologist, coming PhD-student

Contact: eva.helena.hansson@regionh.dk

Strategies and Core Areas of Research:

Paediatric Care Research focuses on areas that support the needs for care, prevention and rehabilitation of children and adolescents with acute and chronic conditions, and their families. Special focus is on home care, telehealth, and nurse-led interventions in the hospital setting. A core approach is patient- and family centered care and intradisciplinary collaboration, and the use of complex interventions, implementation research and public and patient involvement (PPI), such as participatory design, to meet the needs of both the families and the healthcare professionals. The mission is to ensure the best possible quality of care.

Cumulated Funding Received in 2025:

165k EUR

Highlights of 2025:

- Two senior research positions were applied
- Jane Lunding Larsen defended her PhD
- Participation at the 57th SIOP conference in Amsterdam with oral presentation and posters
- Keynote at the NOPHO NOBOS Annual meeting in Riga: Home Care for Children and Adolescents with Cancer: What's possible and how?
- Keynote at the 5th European Symposium on Transition in Paris presenting: Parents in Transition: Their Role, Challenges, and Strategies for Support during Healthcare Transition.

Key publications in 2025:

- Lunding Larsen, J., Emdal Navne, L., Stahlhut, M., Bisgaard, A., Thomsen, EL. & Hansson H. *Being a Parent of an Individual with Rett syndrome – An Explorative Study of Parental Experiences Across Different Ages and Phases of Life*. J Dev Phys Disabil (2025). <https://doi.org/10.1007/s10882-025-10031-x>
- Wyngaert KV, Suykens J, Kelly D, Goncalves I, Thomsen EL, Willemse J, Marino Z, Madaleno J, Day J, Samyn M, Ali M, Jørgensen MH, Goossens E, De Munter J, Van Staa A, Wray J, Protopapa P, Lohse A, De Bruyne R. (2025), *Pediatric to Adult Care Transfer in Rare Liver Diseases: Recommendations based on Insights from a European Omnistakeholder Event*. JHEP Reports, 2025, 101403, ISSN 2589-5559, <https://doi.org/10.1016/j.jhepr.2025.101403>.
- Jensen DA, Hansen LM, Johnsen MD, Dengsø K, Thomsen EL. 'You Have to Be Well, to Be Sick'—A Qualitative Study of Young Adults' Experiences of Undergoing Liver Transplantation. Scand J Caring Sci, 39: e70014, 2025. <https://doi.org/10.1111/scs.70014>
- Höök A, Hylén M, Björk M, Nilsson S, Bai J, Berlin H, Hansson H, Kristjansdottir G, Roxner R, Stenström P, Castor C. The electronic Faces Thermometer Scale – construct validity for pain assessment in pediatric postoperative care. Pediatric Anesthesia. September 2025. doi: 10.1111/pan.70056. PMID: 40977637
- Roug L, Topperzer MK, Michelsen RT, Jarden M, Wahlberg A, Hjalmsgrim L, Hansson H. *Feasibility of an intravenous home chemotherapy intervention with low-dose cytarabine administered by parents (INTACTatHome)*. Pediatric, Blood and Cancer. August 2025. 12:e31965. doi: 10.1002/pbc.31965. PMID: 40799017
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New Partnerships in 2025:

- Ena Lindhart Thomsen became part of the board for Dansk Selskab for Ungdomsmedicin.
- Helena Hansson part of research collaboration in 'Precision Health – Towards Person-Centred Paediatric Care' led by Stefan Nilsson, Professor Institute of Health and Care Sciences, Sahlgrenska Academy, University of Gothenburg.
- Helena Hansson part of research collaboration in 'Digital Communication in Pediatric Surgery care' at Department of Health Sciences, Faculty of Medicine, Lund University, Sweden.

Looking ahead to 2026:

- Expanding the Paediatric Care Research group
- New partnerships in paediatric care research e.g., Faith Gibson, professor in Nursing at Great Ormond Street hospital.



Pediatric Palliative Care Lab, Copenhagen

Group Leader: Thomas Nørrelykke Nissen, MD, Ph.D.

Team: Maja Abitz, My Kathrine Hjerneø, Alexandra Calmer Bungum, Maria Stiholt Otto, Ditte Marie Langvad & Caroline Sophie Kleis Schmidt.

Contact: thomas.noerrelykke.nissen.o2@regionh.dk

Strategies and Core Areas of Research:

The research unit focuses on research that directly impacts families and patients in need of paediatric palliative care. The unit is firmly rooted in a multidisciplinary clinical environment, where research is a shared responsibility across the Paediatric Palliative Care Team. All clinicians in the team contribute to scientific work, including doctors, nurses, and social workers, ensuring that research is grounded in everyday clinical practice and the realities of families' lives. They work with novel, comprehensive ideas to improve the quality of life and treatment in PPC. Recently, the focus has mainly been on international collaboration to develop and test new innovative solutions.

Key Partnerships:

- “HOPE4Kids”, 74 partners across 24 European countries, coordinated by Princess Máxima Centre for Pediatric Oncology.
- “PALLIAKID”, 16 partners across 10 European countries, coordinated by Hospital Sant Joan de Déu.
- “HOME-C2P2”. 6 partners across 5 European countries, coordinated by Hospital Sant Joan de Déu.



Interdisciplinary
Research

- Research collaboration with Danish School of Education (DPU, University of Aarhus) exploring collective memory work in PPC.
- Research collaboration with St. Luke's Academy of Palliative Medicine, St. Luke's Foundation.
- Collaboration regarding epidemiological research with the Danish Cancer Society.
- Collaboration with the Respiratory Centre at Rigshospitalet (RCØ).

Highlights of 2025

- In May, PPC Lab hosted the PALLIAKID General Assembly Meeting, a five-day event bringing together more than 40 researchers from across Europe.
- In June, we joined the kickoff meeting in Barcelona to launch HOME-C2P2.
- PPC Lab CPH hosted the Department of Paediatrics' "Yngre Forsker Forum" (Younger Researcher's association) in September, giving us the opportunity to present our research and answer the early-career researchers' questions about paediatric palliative care.
- Kickoff meeting in Amsterdam in October, where HOPE4Kids was launched.
- PhD student Alexandra Calmer Bungum shared what it is like to work in paediatric palliative care through an Instagram takeover on the "Yngre Lægeliv" profile with more than 12.500 followers.



Funding Received in 2025:

- EU4Health, Joint Action, HOPE4Kids. Total budget: 12M Euro (Our budget: 436.000 Euro).
- 60.000 Euro from The Danish Childhood Cancer Foundation to do a national implementation of the HOPE4Kids project.
- Travel grant from the Dagmar Marshall Foundation.

Key Publications in 2025

- Hammer, N. M., Bidstrup, P. E., Olsen, M., Hansson, H., Abitz, M., & Larsen, H. B. (2025). The experiences of grandparents involved in the home-based end-of-life care of their grandchild with cancer: A qualitative secondary data analysis. *Death Studies*, 49(6), 778–788. <https://doi.org/10.1080/07481187.2024.2355252>
- Hammer, N.M., Bidstrup, P.E., Rosthøj, S., Abitz, M., Callesen, M.T., Hjalgrim, L.L., Edslev, P.W., Wolfe, J., Larsen, H.B. and Olsen, M. (2025), Parental Perspectives on Location of Death for Children With Cancer - A Nationwide Cross-sectional Survey of Bereaved Parents. *Pediatr Blood Cancer*, 72: e31878. <https://doi.org/10.1002/pbc.31878>
- Sannerum, K. et al “Advance care planning til børn og unge med livstruende eller livsbegrænsende sygdom” *Ugeskr Læger* 2025;187:V05250390

Looking Ahead to 2026

- Presenting our research at the Grand Round at Rigshospitalet in March.
- Participating in the 20th World Congress of the European Association for Palliative Care, where the research unit has contributed to 12 accepted abstracts.
- Initiation of the PALLIKID RCT during spring.
- Initiation of the PALLIKID Early Detection System Study in the spring of 2026.
- Initiation of the feasibility trial of the home-monitoring model in HOME-C2P2.
- Participating in the 7th Maruzza International Congress on Pediatric Palliative Care in October.



Pediatric Rheumatology

Team: Charlotte Myrup, MD PhD and Helene Ingels, MD PhD

Contact: charlotte.myrup@regionh.dk, helene.andrea.sinclair.ingels.02@regionh.dk

Strategies and Core Areas of Research:

Paediatric Rheumatology or Clinic for Children and Adolescents with Juvenile Idiopathic Arthritis and Complex tissue diseases is one of three large centers in Denmark that treat Children and Adolescents with Juvenile Idiopathic Arthritis, complex tissue diseases and Autoinflammatory diseases.

The clinic has a connection to several international networks including ERN RITA, PReS, PReStAR, CARRA (Childhood Arthritis and Rheumatology Research Alliance), Charlotte Myrup is part of the NoSPeR (Nordic JIA Study Group). Helene Sinclair Ingels is a part of MIWGUC (Multinational Interdisciplinary Working Group for Uveitis in Childhood) and plays a role in IBD Prognosis Study (a prospective, population-based inception cohort study with adolescent and paediatric patients with IBD, collaboration with regional ped. gastroenterologist, adult gastroenterologists)

Key Partnerships:

- A significant portion of our research is based on international collaborations, e.g. as part of the PReStAR (former PReS) network.



Key Publications in 2025

- Gagne SJ, Sivaraman V, Bosman ES, Klammer B, Morishita KA, Huber A, Orjuela A, Eberhard B, Myrup C, Gerstbacher D, Foell D, Al-Abadi E, McErlane F, Cook K, Wagner-Weiner L, Elder M, Moorthy LN, Dancey P, Yeung R, Khubchandani R, Deepak S, Charuvanij S, Tarvin S, Shenoi S, Tanner T, Brown K, Cabral DA; ARChiVe Investigators Network within the PedVas Initiative. Comparing Rituximab and Cyclophosphamide in Induction Therapy for Childhood-Onset Anti-Neutrophil Cytoplasmic Antibody-Associated Vasculitis: An ARChiVe Registry Cohort Study. *Arthritis Care Res (Hoboken)*. 2025 Apr;77(4):504-512. doi: 10.1002/acr.25455. Epub 2024 Dec 10. PMID: 39467015; PMCID: PMC11931354.

Looking Ahead to 2026

- NoSPeR 28-year follow-up planned in 2026.
- CAR-T therapy in Connective Tissue Diseases in children and adolescents
- IBD Prognosis Study – comorbidity studies
- MIWGUC – consensus paper: State-of-art treatment of uveitis related JIA
- Collaboration with local ophthalmologists: screening of JIA patients for uveitis



The Clinical Trial Unit for Children, Adolescents, and Adults at Rigshospitalet

Leadership team:

- Merete Ljungberg, MD, Mette Skov Jensen, Head nurse, clinical trial unit, & Hanne Hove, consultant, and specialist in rare congenital disorders.

Research Coordinator and Economics:

- Augusta Clara Rahbek Birkedal, Health Economist & Research Coordinator

Principal Investigators and Sub-Investigators:

- Allan Meldgaard Lund, PI, Hanne Buciek Hove, PI, Alfred Peter Born, PI, Merete Ljungberg, PI, Malene Landbo Børresen, PI, Christina Høi-Hansen, PI, Ida Maria Schmidt, PI, Stense Farholdt, PI, Anja Poulsen, PI, Marie-Louise von Linstow, SI, Olivia Welle Fjellbirkeland, SI, Line Thousig Sehested, SI, Line Aagaard Nolting, SI (part-time), Mia Aagaard Doherty, SI, Emma Damm Mørch, SI & Maria Hjortgaard Ebbesen, SI.

Study Coordinators & Trial Nurses:

- Andrea Rønde Bjerg, Emilie Skjødt Thyssen, Gertrud Hjarne, Sofie Lemming, Catrine Lykke Carlsen, Agnethe Herskind, Emma Emilie Frydendall (part-time), Catrine Bess (part-time), Laura Linde, Camilla Carøe, Thorkild Matthiesen (part-time), & Rita Møller (Part-time).

Collaborators and National Coordinators:

- Pernille Skovby, National Coordinator, DanPedMed and c4c-stichting National Hub Coordinator
- Hanne Hove – c4c-stichting National Coordinator
- Clinical Trial Units Leadership network at Rigshospitalet

Contact: merete.ljungberg@regionh.dk & Hanne.buciek.hove@regionh.dk

Strategies and Core Areas of Research:

The Clinical Trial Unit (CTU) was consolidated in 2024 with a dedicated physical space to centralize sponsored pharmaceutical research within the Department of Pediatrics and Adolescents with Rare Diseases. The unit encompasses all pediatric subspecialties except for pediatric oncology.

The unit was established to facilitate sponsored research, primarily focusing on interventional studies involving new treatments. In addition, observational registry studies sponsored by external parties are also conducted.

Our vision is founded on the belief that targeted and individualized medicine – including advanced therapies and gene-therapeutic treatments – can offer children with otherwise incurable diseases new opportunities for treatment, improved quality of life and increased survival.

The unit is managed by a departmental leadership team, supported by a research coordinator and trial nurses. The current team comprises of 32 staff members.

Clinical Trials Overview as of 2025:

The CTU oversees 19 active clinical trials sponsored by the pharmaceutical industry across the following subspecialties (with the number of ongoing studies indicated in parentheses)

- Pediatric Nephrology (1)
- Pediatric Neurology (7)
- Metabolic Disorders (5)
- Skeletal Dysplasia (5)
- Paediatric Infectious Disease (1)
- Rare Genetic Disorders (1)

In 2025:

- 7 new studies were initiated
- 5 studies were completed (two transitioned to open-label extensions; one continued as a post-trial access program)
- 68 patients actively participated in clinical trials

Disease registries:

Sanofi Rare Disease Registries is a global observational program designed to track natural history and outcomes of patients with Gaucher, Fabry or Pompe disease. We have entered data in these registries for more than 10 years and are committed to improve disease understanding by contributing and sharing data with our fellow physicians.



Highlights of 2025:

- **External Funding:** We successfully secured DKK 160.000 in external funding. The walls of the trial unit are now decorated with inspiring, child- and family-friendly illustrations. The artwork is designed to create a warm and welcoming atmosphere that helps children and their families feel safe and comfortable. Through gentle colors, playful motifs, and positive imagery, the drawings aim to reduce anxiety and support a calm, reassuring environment for both patients and caregivers.
- **Collaboration with the Murdoch Children's Research Institute, Melbourne:**
 - We established a collaborative partnership with the Murdoch Children's Research Institute (MCRI) in Melbourne, the world's leading clinic for skeletal dysplasia. Notably, the MCRI is conducting the same sponsored studies on Achondroplasia as our center in Copenhagen.
 - The partnership resulted in a JAMA Pediatrics paper to be published in 2026.
- **Scientific Presentation at ESPE 2025**
 - "Effects of Navepegritide on Growth in Children". Results from the Clinical trials of Navepegritide treatment to children with achondroplasia aged 2-11 years of age with Achondroplasia: 52-Week Results from the ApproaCH Clinical Trial.
 - Our CTU is the highest recruiting site worldwide, including participants from Denmark, Norway, Sweden and Austria
- **Holistic Approach to Patient Care in Clinical Trials**
 - We have implemented patient-centered pedagogical principles that prioritize children's involvement and autonomy in the activities required by clinical trials.
 - We believe that fostering participation and shared decision-making leads to better long-term outcomes, happier patients, and greater satisfaction for families, staff, and sponsors.
 - To strengthen this approach
 - One of our coordinators is trained as a youth ambassador
 - One of our coordinators has a MA in Educational psychology
- **FDA-inspections**
 - In September, the Clinical trial unit was visited by the U.S. Food and Drug Administration (FDA), and two of our ongoing trials underwent inspection. Both inspections were completed without any major findings.
 - The inspections confirmed our strong compliance framework and the robustness of our operational and documentation procedures.

Key Collaborators:

- The Clinical Trial Unit has a strong collaboration with c4c and participates in annual meetings through our national coordinator. Additionally, we work closely with DanPedMed, also through our coordinator, Pernille Skovby.
- This year, the Clinical Trial Units Leadership Network at Rigshospitalet was formed. The network brings together leaders from diverse clinical specialties to collaborate, share expertise, and drive excellence in conducting clinical trials.

- Intrathecal onasemnogene abeparvovec in treatment-naïve patients with spinal muscular atrophy: a phase 3, randomized controlled trial. STEER Study Group incl. Born AP. Nat Med. 2025. doi: 10.1038/s41591-025-04103-w.
- Effect of long-term sepiapterin treatment on dietary phenylalanine tolerance in patients with phenylketonuria: interim results from the Phase 3 APHENITY Extension Study. Genet Med, 101683 (2026). van Spronsen, F., Peters, H., Margvelashvili, L., Agladze, D., Schwartz, I.V.D., Gizewska, M., Hamazaki, T., Guilder, L., MacDonald, A., Vijay, S., Inwood, A., Minami, M., Fjellbirkeland, O., Lund, A., Lah, M., Thomas, J.A., Longo, N., Kiykim, E., Ishige, M., Burlina, A., Belanger-Quintana, A., Rutsch, F., Opladen, T., Mungan, H., Ezgu, F., Bratkovic, D., Chakrapani, A., Ito, T., Guimas, A., Zori, R., Tchan, M., Sacharow, S., Oliveira, A., Janeiro, P., Guerra, I.C., Plana, J.C., Yildiz, Y., Canda, E., Khan, A., Vockley, J., Breilyn, M.S., Manti, F., Larkin, A., Hughes, C., Liu, E., Gao, L., Ingalls, K., Smith, N. & Muntau, A.C. Genet Med, 101683 (2026). DOI: [10.1016/j.gim.2026.101683](https://doi.org/10.1016/j.gim.2026.101683)
- Does p-lactate increase in patients with GSDI after ingesting a meal with common-size sources of fructose and galactose? Observations from a prospective, non-blinded, crossover pilot study. Caroe, C.D.B. & Lund, A.M. JIMD Rep 66, e12457 (2025). <https://doi.org/10.1002/jmd2.12457>
- Circulating bone biomarkers as indicators of growth in children with achondroplasia. Rignol, G., Roberts, K., Slade, A., Czech, C. & Ljungberg, M. Dec. 2025: Endocrine and Metabolic Science. 19, 100265



Looking Ahead to 2026:

In 2026, our research unit will continue to expand its clinical research portfolio and strengthen its position as a preferred partner for advanced clinical trials in rare diseases. We aim to deepen our collaborations with pharmaceutical companies, industry partners, and academic institutions to secure a broader and more innovative trial pipeline.

A major milestone for the year will be the initiation of our first investigator-initiated clinical trial. This study will also represent our third gene therapy trial, marking a significant step forward in our expertise with next-generation therapeutic technologies. Conducting an investigator-initiated study will demonstrate our unit's capability not only to operationalize externally sponsored trials but also to generate and lead our own research at an international level.

2026 will also be a pivotal year for the unit as the revised ICH-GCP E6(R3) guideline becomes fully embedded in clinical research operations across Europe. For our unit, adopting ICH-GCP R3 will mean integrating enhanced risk-based processes, strengthening data governance frameworks, and supporting modern trial methodologies. This will not only elevate the quality and compliance of our trial operations but also position us strongly for upcoming partnerships and innovative study designs, including our investigator-initiated research.

Two of our staff members are collaborating with the National Coordinator for DanPedMed to establish a dedicated Network for Project Nurses working with children in Copenhagen in 2026. By contributing to the development of this network, our team is helping to build a stronger, more connected community for specialized research nurses committed to improving healthcare outcome for children.



Lone Graff Stensballe leads the following two collaborating research groups:

1) Unit of Vaccinology, Paediatric Infectious Disease Epidemiology, and Pharmacoepidemiology

Group Leader: Professor Lone Graff Stensballe

Team: Four dedicated researchers.

Location: Rigshospitalet, Tagensvej (Entrance 75)

Contact: lone.graff.stensballe@regionh.dk

Strategies and Core Areas of Research:

Since 2012, the unit has randomised more than 11,000 infants in investigator-initiated vaccine trials, establishing a unique national and international position within pediatric vaccine research. The unit's work is characterised by a strong integration of:

- Randomised clinical trials combined with in-depth laboratory analyses
- Nationwide real-world studies of vaccine effectiveness and safety
- Pharmacoepidemiologic evaluations of long-term safety of biological treatments

This multifaceted approach enables rapid translation from clinical trials to population-level evidence.

Funding Received in 2025:

- 335.350 Euro from the Lundbeck Foundation
- 690.050 Euro from the Independent Research Fund Denmark



Highlights of 2025:

- **PhD Defences:** Our team celebrated two PhD defences:
 - Dr. Jesper Kiehn Sørensen, University of Copenhagen, *Genetic associations with measles, mumps, and rubella vaccine response after MMR vaccination at 6 and 15 months of age*
 - Sejun Kim, University of Copenhagen, *Real-world evidence approaches to investigating the long-term safety of post-marketed biological treatments for psoriasis: Danish nationwide register-based studies*
- **Bachelor Theses:** Our team submitted three Bachelor's theses:
 - Simone Maria Lodahl, University of Copenhagen, *Influenza vaccination during pregnancy: Effectiveness against clinical influenza disease in the pregnant individual.*
 - Sofie Siemsen, University of Copenhagen, *Influenza vaccination during pregnancy: Safety in the pregnant individual and fetus.*
 - Ellen Lawaetz, University of Copenhagen, *Influenza vaccination during pregnancy: Effectiveness against clinical influenza disease in the infant from birth to six months of age.*

Key publications in 2025:

- *Measles-mumps-rubella vaccination at 6 months of age and the risk of atopic disease in the first year of life: Results from a Danish placebo-controlled randomized trial.* J Infect. 2025;90(3):106433.
- *Measles-mumps-rubella vaccination at 6 months of age induces measles-specific T-cell responses: A randomized controlled trial.* Front Immunol. 2025;16:1546253.
- *Safety of pertussis vaccination in pregnancy and effectiveness in infants: A Danish national cohort study 2019–2023.* Clin Microbiol Infect. 2025;31(6):995–1002.
- *Measles vaccines and non-specific effects on mortality or morbidity: A systematic review and meta-analysis.* PLoS One. 2025;20(7):e0321982.
- *Genetic associations with measles PRNT and IgG antibody response to MMR vaccination in 6- and 15-month-old children.* Vaccine. 2025;50:126788.

Looking Ahead to 2026:

Funding obtained in 2025 enables the initiation of two major clinical research projects:

- A randomized controlled trial of early MMR vaccination in premature infants
- Immunogenicity follow-up of participants from the original MMR trial

2) MARYs Data Powerhouse: Transforming Paediatric Healthcare

Group Leader: Lone Graff Stensballe

Team: Eight researchers and data specialists.

Location: Mary Elizabeth Hospital, Building 61, 1st Floor

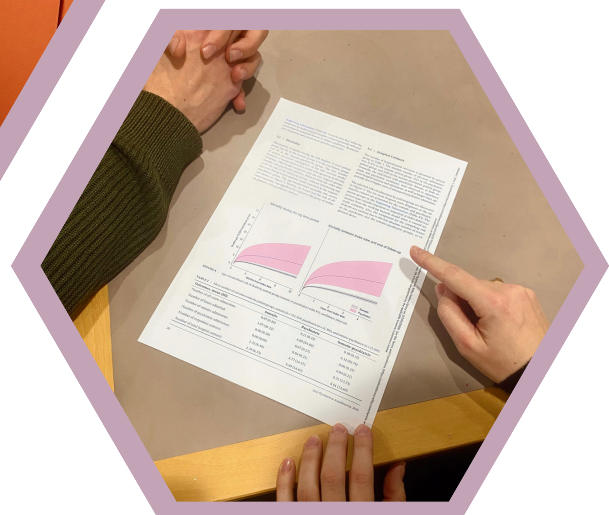
Contact: lone.graff.stensballe@regionh.dk

Strategies and Core Areas of Research:

MARYs Data Powerhouse is a leading environment for applied pediatric epidemiology. Through close collaboration with clinicians, the team conducts nationwide register-based studies examining the long-term somatic, psychiatric, and socioeconomic consequences of severe and chronic pediatric diseases. A central focus is the critical evaluation of how pediatric healthcare is organised and whether current practices deliver optimal value and outcomes for children and adolescents.

Funding Received in 2025:

- 104.580 Euro from the Børneriget Foundation
- 482.145 Euro from the Independent Research Fund Denmark
- 53.545 Euro from the Aase and Ejnar Danielsen's Foundation

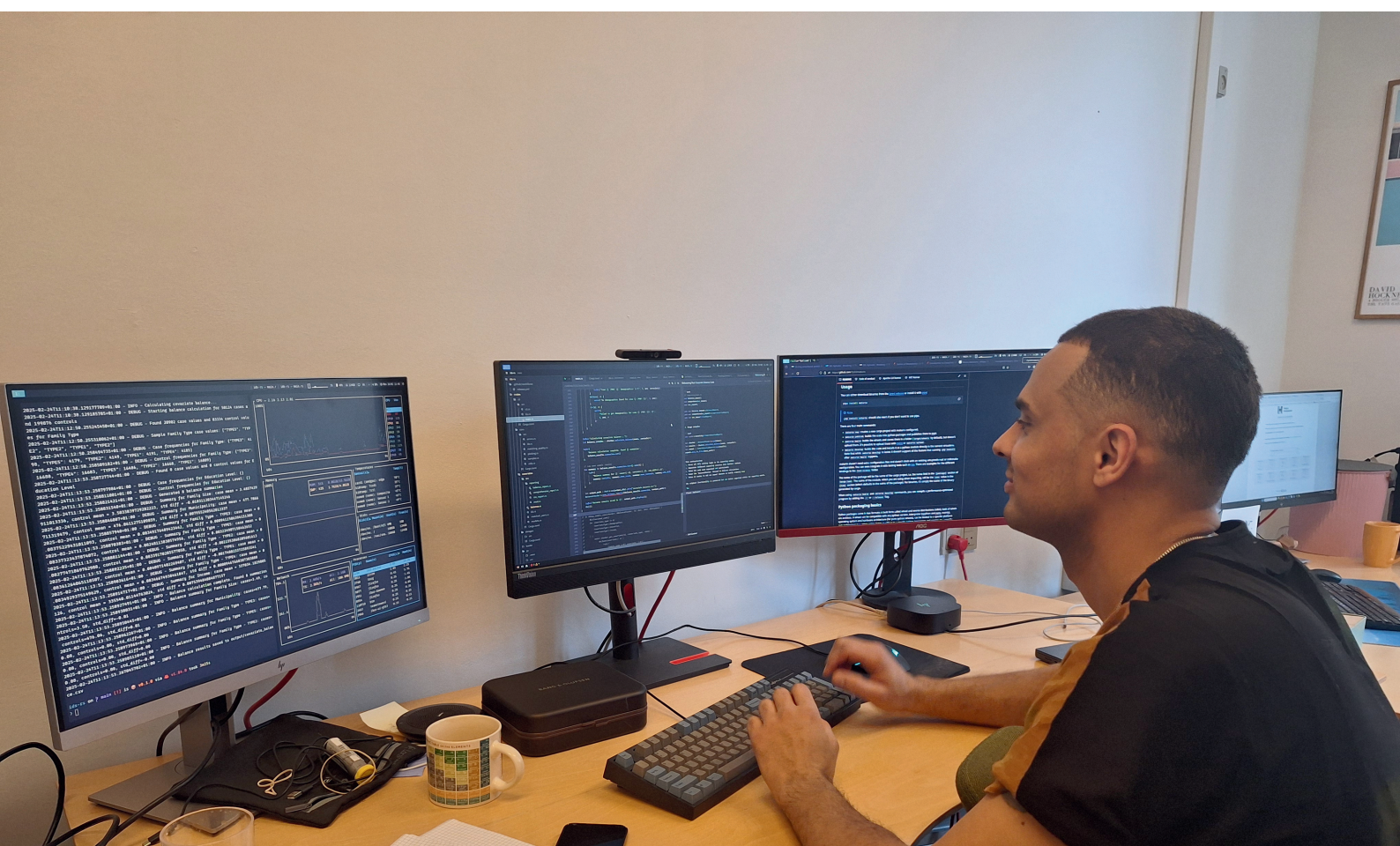


Highlights of 2025:

- **Conference leadership:** Hosting the national conference “Overdiagnosis and Overtreatment” at Rigshospitalet (January 7, 2026), with 187 participants, featuring expert presentations and panel debates on value-based care and healthcare prioritization.
- **Scientific leadership:** Internal summer meeting focused on best practices and ethical standards for co-authorship.
- **International collaboration:** Continued strengthening of collaboration with Karolinska Institutet, particularly within pharmacoepidemiology.
- **Talent development:** Welcoming Alberte Aspaas Lundquist to the team.

Key Publications in 2025:

- *Pediatric somatic and psychiatric hospital contacts in Denmark: A national overview of risk factors, admissions, and mortality.* Acta Psychiatr Scand. 2026;153(1):24–33 (Epub 2025).
- *Impact of COVID-19 on testing, positive cases, patient characteristics, and hospital contacts for respiratory syncytial virus, influenza, and pneumococcus in Danish children.* J Infect Public Health. 2025;18(2):102660.
- *Association between chronic diseases in childhood and subsequent educational achievement: A Danish register-based cohort study.* Eur J Epidemiol. 2025 (online ahead of print).
- *Data-driven algorithms for classification of in- and outpatients in the Danish National Patient Register.* Clin Epidemiol. 2025;17:147–163.
- *Nationwide registry study of long-term survival and comorbidities in total anomalous pulmonary venous connection in Denmark.* Sci Rep. 2025;15:30408.



Strategic Priorities for 2026:

- Longitudinal studies of morbidity, mortality, and healthcare utilization among children and adolescents with psychiatric and somatic conditions.
- Development of consensus algorithms defining complex chronic disease in childhood and adolescence.
- Studies of short- and long-term socioeconomic consequences of severe chronic pediatric disease.
- Expanded national and international collaboration with epidemiologists, statisticians, and health economists.



Unit for Congenital Heart Disease



Staff:

- 1) Paediatric cardiology: 5 senior consultants, 2 junior consultants, 1 resident.
 - Team leader: Lars Idorn.
 - Team members: Jesper Steensberg, Anders Hebert, Charlotte Kruse, Klaus Juul, Beth Kanijo, Cristel Hjortshøj.
- 2) Adults with Congenital Heart Disease (ACHD): 4 consultants.
 - Team leader: Michael Rahbek Schmidt.
 - Team members: Annette Schophuus Jensen, Niels Vejlstrup, Laurence Campens.
- 3) Cardio-thoracic surgery: 3 consultants.
 - Team leader: Morten Holdgaard Smerup.
 - Team members: Morten Smerup, Sabrina Ellesøe, Gustav Thyregod (until 01.01.26), from 01.01.26: Sune Damgaard.
- 4) Cardio-thoracic anaesthesia: 5 consultants.
 - Team leader: Jakob Gjedsted.
 - Team members: Jesper Poulsen, Michael Wancher, Therese Vestergaard, Iben Engelund Luna
- 5) Neonatology: 2 consultants (part-time).
 - Team leader: Simon Trautner.
 - Team members: Emma Louise Malchau Carlsen.
- 6) Research: 1 full-time professor. Vibeke Hjortdal.

UCHD is a clinical and research collaborative unit established more than 20 years ago.

Contributing departments are Paediatric Cardiology, Neonatology, Adult Cardiology, Cardio-thoracic Surgery and Cardio-thoracic Anaesthesia.

Areas of Research:

- Register-based research in somatic and socio-economic outcome after congenital heart disease surgery/intervention.
- Predicting neuro-outcome, neuroprotection after hypoxic/hypo-circulatory events
- Diseases in the lymphatic system

Funding Received in 2025:

- The Danish Heart Foundation. 1.5 mio Dkr. (200k EUR), for the Danish Register of Congenital Heart Disease.

Highlights of 2025:

- Danish Register for Congenital Heart Disease established. Funding secured for 2026-2030.
- Continued work on lymphatic disease research and international collaboration.

Key Publications in 2025:

- van der Have O, Tran-Lundmark K, Wåhlander H, Kaskinen AK, Möller T, Juul K, Jahnukainen T, Weinreich ID, Sarkola T, Nilsson J, Odermarsky M. *Pediatric heart transplantation within the Scandiatransplant region-a multinational observational study spanning 38 years*. J Heart Lung Transplant. 2025 Oct;44(10):1597-1609. doi: 10.1016/j.healun.2025.05.020. Epub 2025 Jun 5. PMID: 40482845.
- Henningsen MB, Bohnstedt C, Vestergaard TR, Smerup MH, Madsen PV, Stensballe LG. *Nationwide registry study of long term survival and comorbidities in total anomalous pulmonary venous connection in Denmark*. Sci Rep. 2025 Aug 19;15(1):30408. doi: 10.1038/s41598-025-15769-0. PMID: 40830188; PMCID: PMC12365164.
- Kronborg JR, Lindhardt RB, Vejlsstrup N, Holst LM, Juul K, Smerup MH, Gjedsted J, Ravn HB. *Impact of acute kidney injury and dysnatremia on length of stay in infants after cardiac surgery*. Pediatr Nephrol. 2025 Oct;40(10):3281-3291. doi: 10.1007/s00467-025-06846-7. Epub 2025 Jun 14. PMID: 40515779 Free PMC article.
- Su J, Schmidt MR, Campens L, Montani D, Kiely DG, Condliffe R, Ersbøll MK *Successfully Treating Pulmonary Arteriovenous Malformation and Pulmonary Arterial Hypertension in a Patient with GDF2 Variant*. Arch Bronconeumol. 2025 Sep 1:S0300-2896(25)00303-5.
- Kelly B, Ravndal M, Nielsen AKM, Krogh E, Hjortdal VE, Idorn L. *30-Year Survival After the Fontan Operation in Denmark*. Pediatr Cardiol. 2025 May 22. doi: 10.1007/s00246-025-03896-4. Online ahead of print. PMID: 40404979
- Kelly B, Ravndal M, Bach P, Telinius N, Bennike TB, Hjortdal VE. *Systemic Changes in Adults With a Fontan Circulation: Insights From the Plasma Proteome*. Am Heart Assoc. 2025 May 20;14(10):e038648. doi: 10.1161/JAHA.124.038648. Epub 2025 May 15. PMID: 40371574 Free PMC article.
- Lau-Jensen SH, Hejl JL, Thorup L, Lauritsen MB, Rask CU, Hjortdal VE. *Psychiatric comorbidities and feasibility of an online psychiatric screening measure in children and adolescents with the Fontan circulation*. Cardiol Young. 2025 Jan;35(1):109-116. doi: 10.1017/S1047951124026738. Epub 2024 Oct 21. PMID: 39429149
- Thorup L, Dehn AM, Heiberg J, Nisja E, Kofod T, Kelly B, Hjortdal VE. *Reduced Lymphatic Activity During Positive Pressure Ventilation and General Anesthesia-A Cause of Postoperative Edema?* Acta Anaesthesiol Scand. 2025 Jul;69(6):e70066. doi: 10.1111/aas.70066. PMID: 40474334 Free PMC article.

- Ramcharan T, Roest A, Voges I, Cantinotti M, Grotenhuis H, Garrido AO, Pasqualin G, Di Salvo G, Krupickova S, Róth G, Jossif A, Salaets T, Brun H, Grant B, Glessgen C, (...) Miller O, Raimondi F, McMahon CJ. *Pediatric Cross-Sectional Cardiac Imaging in Europe: Current Status, Disparities, and Potential Solutions: A Survey from the Association for European Pediatric and Congenital Cardiology (AEPC) Imaging Working Group*. *Pediatr Cardiol*. 2025 Sep 20. doi: 10.1007/s00246-025-04027-9. Online ahead of print. PMID: 40974416
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Key Partnerships in 2025:

National

1. IPSYCH, Roskilde. Genetic involvement in MHS and psychiatric disorders
2. Department of child/adolescent psychiatry, Aarhus University Hospital and Aalborg University Hospital. National study of psychiatric disorders in paediatric patients with heart disease.
3. The Parker Institute, Frederiksberg Hospital. The significance of Vitamin D for the development of MHS.
4. The Institute for Cellular og Molecular Medicine. Panum, KU. The embryogenesis of the heart and congenital defects.
5. Institute of Biomedicine, Panum, KU. The pharmacology of the lymphatic system.
6. Aalborg University, Proteomics in patients after Fontan completion.

International

1. Children's Hospital of Philadelphia, Philadelphia, USA
2. Queen Silvia Children's Hospital, Gothenburg, Sweden
3. Austral University, Pilar, Buenos Aires, Argentina
4. In Cor, Sao Paulo, Brazil



What awaits the department in 2026?

We look forward to...

“...launch the ChildLife project to support children aged 0–6 years diagnosed with cancer in safely returning to daycare during treatment.”

“...initiate 5 investigator initiated trials: Forum 2, CARS4ALL_01, VHR CAR CD19, NBL CD2 CART, BuGenes.”

“...conduct the International Child Health course in Vietnam in collaboration with Paediatric Hospital No.1, Ho Chi Minh City.”

“...quantify dynamic tracheal and central airway collapse during respiration using 4D CT.”

“...Initiate a new multi-omics research project in children with infectious diseases.”

“...To host the “Early Career Symposium on Paediatric Research, Denmark” in collaboration with Young Paediatricians on 17 September.”



... and much more!

Interested in learning more about our department's research? Follow us on LinkedIn!



Afdeling for Børn og Unge, Rigshospitalet

455 følgere
2md. •

Når konflikt rammer, rammer den ofte børnene først. I denne uge satte [Dansk Pædiatrisk Selskab](#) fokus på netop det med symposiet "Child Health in Conflict Settings" på [Rigshospitalet](#). ... mere



Alexandra Calmer Bungum og 39 til

6 genopslag



Afdeling for Børn og Unge, Rigshospitalet

455 følgere
3md. •

Yngre Forsker-Forum i [Afdeling for Børn og Unge, Rigshospitalet](#) – for anden gang! ... mere



43

2 genopslag



Afdeling for Børn og Unge, Rigshospitalet

455 følgere
8md. •

Vores forskningsgruppe CHILD, der beskæftiger sig med infektioner hos børn, har stået i spidsen for dette flotte studie om individualiseret behandling til børn med urinvejsinfektion, som netop er publiceret i The Lancet Infectious Diseases. ... mere



Naqash Sethi • 2.

MD, PhD, Upcoming Pediatrician, Evidence-based Medicine and Methodology
9md. •

★ "PUBLICATION ALERT!" ★

I am thrilled to share that our research article, "Efficacy and safety of ... mere

Vis oversættelse



Efficacy and safety of individualised versus standard 10-day antibiotic treatment in children with febrile urina...
sciencedirect.com

47

3 kommentarer



Synes godt om

Kommenter

Slå op igen

Original Scientific Publications in Peer-Reviewed Journals in 2025

- 1) Ahlgren, L., Pilheden, M., Stureson, H., Song, G., Walsh, M. P., Yang, M., Maillard, M., Zhao, H., Cheng, Z., Singh, V., Castor, A., Pronk, C. J., Marquart, H. V., Lausen, B., Schneider, P., Barbany, G., Pokrovskaja Tamm, K., Abrahamsson, J., Lohi, O., ... Hagström-Andersson, A. K. (2025). The genomic landscape of relapsed infant and childhood KMT2A- rearranged acute leukemia. *Nature Communications*, 16(1), 8964. Artikel 8964. <https://doi.org/10.1038/s41467-025- 64190-8>
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- 7) Andersen, C. C., Kjær, E. K. R., Vase, C. B., Mathiasen, R., Debes, N. M., Jørgensen, N. R., & Jennum, P. J. (2025). Melatonin secretion across puberty: A systematic review and meta-analysis. *Psychoneuroendocrinology*, 173, Artikel 107281. <https://doi.org/10.1016/j.psyneuen.2025.107281>
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