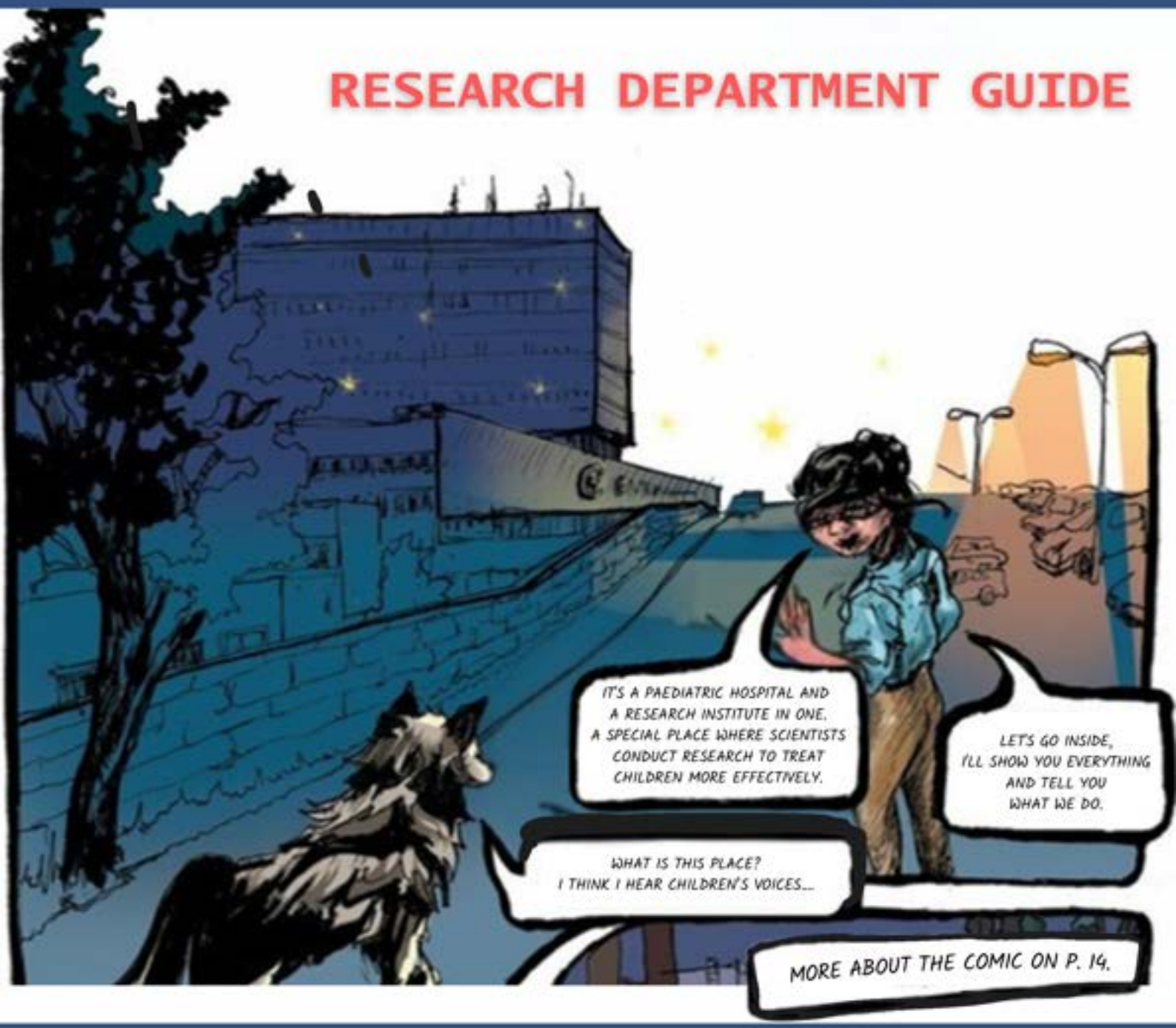
THE CHILDREN'S MEMORIAL HEALTH INSTITUTE

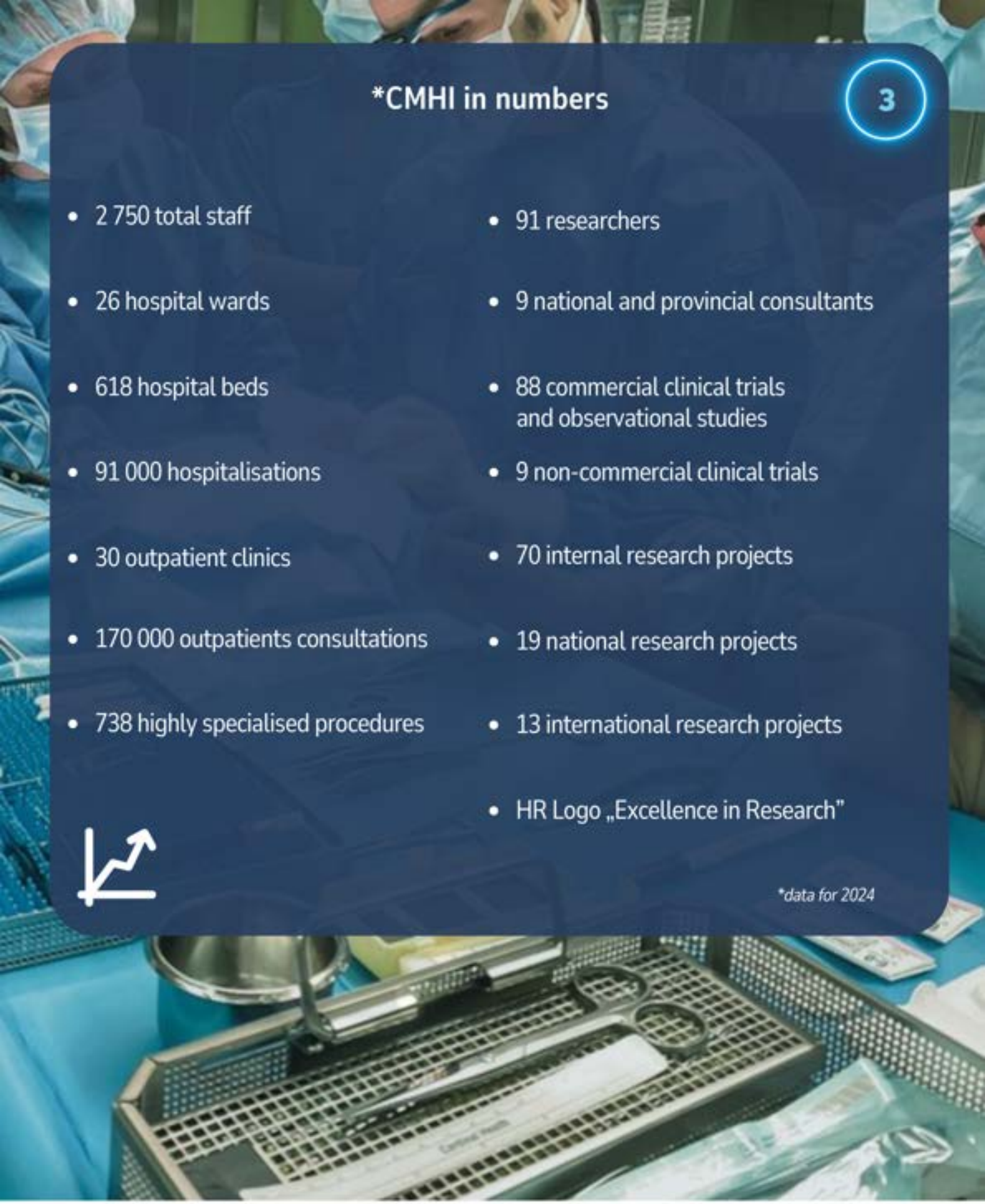
RESEARCH DEPARTMENT GUIDE



PAEDIATRIC HOSPITAL AND RESEARCH INSTITUTE

THE CHILDREN'S MEMORIAL HEALTH INSTITUTE (CMHI) is one of the largest paediatric hospitals in Poland. It has been operating since 1977.

- CMHI in numbers
- Implementations
- Research projects
- Patients involvement
- Research support
- International cooperation



*CMHI in numbers

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- 2 750 total staff
- 26 hospital wards
- 618 hospital beds
- 91 000 hospitalisations
- 30 outpatient clinics
- 170 000 outpatients consultations
- 738 highly specialised procedures
- 91 researchers
- 9 national and provincial consultants
- 88 commercial clinical trials and observational studies
- 9 non-commercial clinical trials
- 70 internal research projects
- 19 national research projects
- 13 international research projects
- HR Logo „Excellence in Research"

*data for 2024

HR EXCELLENCE IN RESEARCH

Publications 2022-2025

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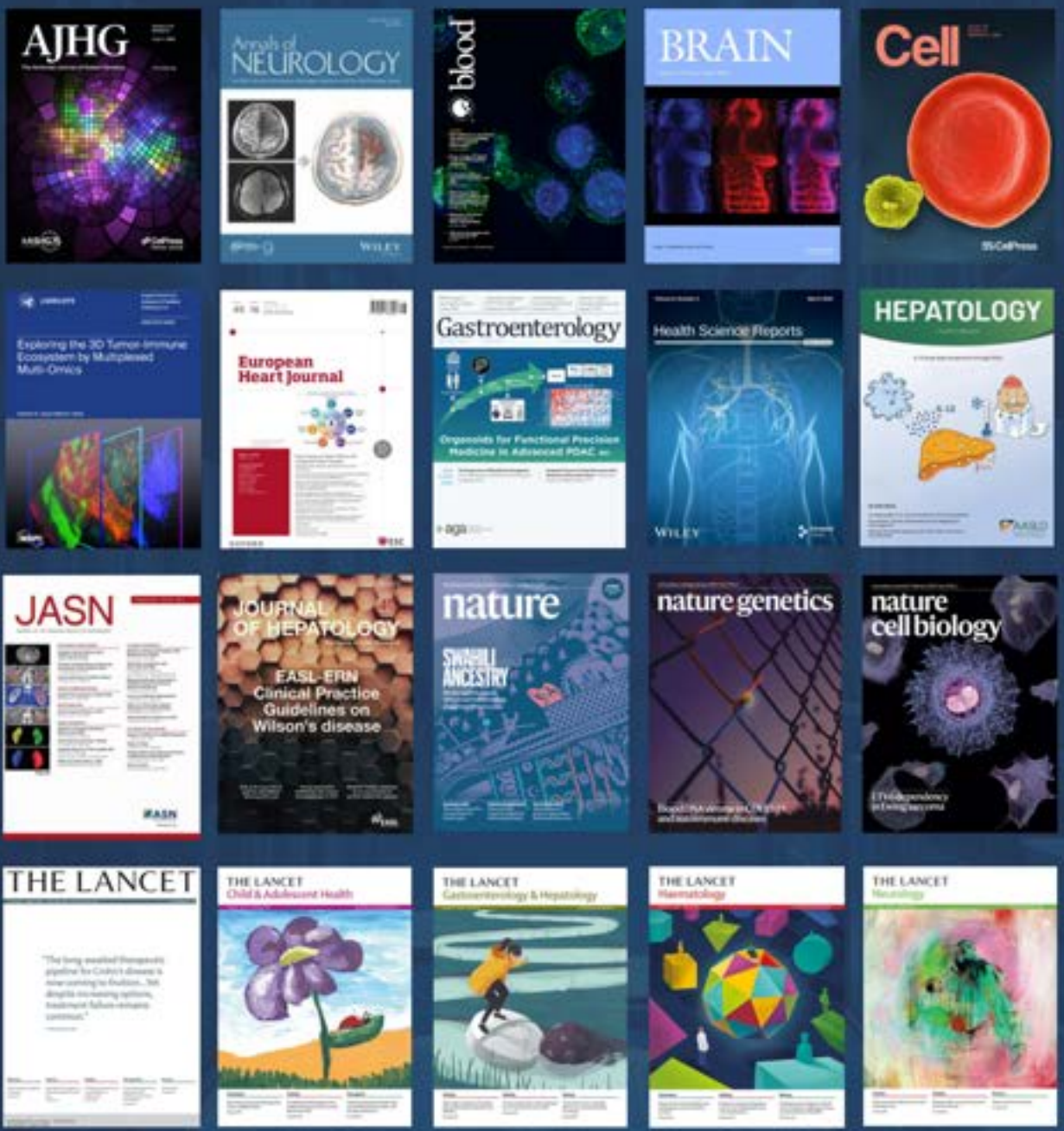
- 572 papers with IF scores
- total IF: 2 938,637

The work of our researchers is published in, among others:



Bibliography of Staff

Selected research projects

- **RAMIPS - [SURGERY]** „Comparison of laparoscopic surgery with robotic surgery in selected non-oncological indications in paediatric patients – an open, randomized, medical trial. Robot-Assisted Minimally Invasive Interventions in Pediatric Surgery".
- **NeoWes - [GENETICS]** „Evaluation of effectiveness of whole exome sequencing as the first tier tool of genetic diagnostics in neonates and infants".
- **Anti-Sepsis - [PHARMACOKINETICS]** „Impact of target-directed dosing based on a clinical pharmacokinetics model of antimicrobial drugs on clinical outcome and quality of life in children and neonates - safety, efficacy and cost-effectiveness analysis"
- **LITTLER - [NEUROSURGERY]** „Randomised, two-centre, open-label research experiment comparing the use of laser thermoablation with classical neurosurgical treatment for the treatment of focal intracranial lesions of the eloquent region in paediatric patients".
- **HIT-GLIO - [ONCOLOGY]** „Targeting tumour-host interactions in paediatric malignant gliomas to reinvigorate immunity and improve radio- and immunotherapy efficacy".

Research support - CWpediBK

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CMHI houses the Paediatric Clinical Trials Support Centre (CWpediBK), which aims to support research teams in the implementation of both commercial and non-commercial clinical trials.
Its priorities are:

- increasing the number of commercial and non-commercial clinical trials conducted in the paediatric population in Poland,
- introducing new standards of work in the field of clinical research at the Institute,
- increasing the number of participants in paediatric clinical trials,
- conducting research to improve access to modern therapies for the paediatric population,
- strengthening CMHI's position on the map of paediatric clinical research centres in Poland and worldwide.

CENTRUM WSPARCIA BADAŃ KLINICZNYCH

CWpediBK
Centrum Wsparcia Pediatrycznych Badań Klinicznych

AGENCJA BADAŃ MEDYCZNYCH

Research support - CWpediBK

Non-commercial clinical trials conducted by CMHI in consortia

- **AxGD - [METABOLIC DISEASES]** „Evaluation of the safety and efficacy of ambroxol (ABX) in Polish patients with Gaucher disease, including the neuronopathic form (GD type III, GD3) resulting from the homozygous c.1448T>C mutation (p.Leu483Pro) in the GBA gene, as well as the forms of GD types I and III associated with the carriage of other GBA variants, based on clinical features and multiomic analyses". Leader: CMHI

- **BraimTOR NEURO - [NEUROLOGY]** „Open randomized phase II clinical trial evaluating the safety and efficacy of rapamycin for the treatment of rare and ultra-rare central nervous system diseases associated with mTOR pathway activation in children". Partners: Transition Technologies-Science Sp. z o.o.; National Forum for Rare Disease Therapy – ORPHAN

- **BraimTOR ONKO - [ONCOLOGY]** „Open randomized phase II clinical trial evaluating the safety and efficacy of rapamycin for the treatment of rare and ultra-rare central nervous system diseases associated with mTOR pathway activation in children". Partners: Transition Technologies-Science Sp. z o.o.; National Forum for Rare Disease Therapy – ORPHAN

- **DIPGen - [ONCOLOGY]** „Use of targeted therapy in children aged 3 to 18 years with a diagnosis of diffuse intrinsic pontine glioma (DIPG) based on genetic findings". Partners: The Upper Silesian Child Health Center in Katowice, Department of Genetics of The Medical University of Warsaw, GLIOMA Fundation

- **EMPAtia - [METABOLIC DISEASES]** „Evaluation of the efficacy and safety of empagliflozin in the treatment of neutropenia in patients with glycogenosis Ib". Partner: The Medical University of Warsaw (WUM)

Research support - CWpediBK

Non-commercial clinical trials conducted by CMHI in consortia

- **PRIFEN - [DIABETOLOGY]** „Evaluation of effect of fenofibrate on pancreatic beta cell function in children with newly diagnosed type 1 diabetes mellitus". Lider: The Medical University of Warsaw
- **RaRE-TS - [NEUROLOGY]** „Multicenter, randomized, double-blind, placebo controlled, phase III study to assess the efficacy and safety of Rapamycin in drug Resistant Epilepsy associated with TSC".
  Partners: The Medical University of Lodz, Transition Technologies-Science, Sp. z o.o. & Partner-Consultant: Brigham and Women's Hospital, Boston, USA
- **ViRap [NEUROLOGY]** „Randomized placebo-controlled, double-blind, double-sham clinical trial evaluating the efficacy, tolerability, and safety of vigabatrin versus rapamycin treatment as preventive therapy in infants with tuberous sclerosis". Partners: The Medical University of Lodz, Transition Technologies-Science, Sp. z o.o. & Partner-Consultant: Brigham and Women's Hospital, Boston, USA

Non-commercial clinical trials conducted by CMHI independently

- **VEDI-UC - [GASTROLOGY]** „Evaluation of safety and efficacy of induction therapy with vedolizumab compared to standard therapy with infliximab in pediatric patients with ulcerative colitis" (Single-subject contract)

Research support - PeRCMC

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Paediatric Regional Center for Digital Medicine (PeRCMC) supports research in paediatrics through advanced digital technologies. The center integrates and analyzes a various type of medical data (laboratory data, imaging data, omics data, structured EHRs, and unstructured physician notes), ensuring its high quality and security. It operates in compliance with interoperability standards, in line with the implementation of EHDS guidelines.

The center offers comprehensive research support - from acquiring and exchanging medical data, to designing studies and facilitating access to datasets, to analyzing results and supporting the development of artificial intelligence algorithms.

Current projects:

- Intelligent operating theatre planning and optimization system,
- Multi-omic classifier for rare cancer diagnosis,
- Research simulator for designing and optimizing clinical trials,
- Physician assistant for creating structured medical records using large language models,
- Medical software for supporting rare disease diagnosis through medical data integration and analysis.

Research support - Biobank

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Biobank is a repository of biological samples (e.g., blood, urine, saliva, tissues, cells) that are collected, stored and made available for research, medical or diagnostic purposes. Its goal is to create a resource that can be used for analyzing and researching the causes of diseases, evaluating the effectiveness of therapies, developing new drugs, and conducting genetic and basic scientific research.

The Biobank will operate in accordance with EN ISO 20387, which defines quality management requirements for biobanks, ensuring compliance with legal and ethical regulations, and maintaining appropriate standards for the storage of samples and data that guarantee their integrity, security and quality. A biobank certified under this standard must also provide adequate conditions for data management and ensure compliance with applicable privacy and data protection regulations. The Biobank will support the development of innovative research and biotechnological solutions at CMHI.
It will serve as the backbone of research projects, providing a secure repository for storing biological material for research purposes. CMHI's Biobank is scheduled to become fully operational at the beginning of 2026.

Research support - RD

The Research Department (RD) supports the Institute's scientific community in conducting research work, clinical trials and development work. It manages and oversees the financial reporting of scientific projects, and facilitates national and international cooperation with research institutes and universities, with the aim of:

- exchanging knowledge and experience,
- participating in collaborative research projects,
- implementing the scientific achievements of the Institute's researchers.

The Research Department actively identifies funding opportunities and assists in the grant application process, including administrative, financial, and reporting support for all research projects carried out at CMHI. Issuing a periodic Newsletter, the department promotes the scientific achievements of staff, provides updates on training opportunities, conferences, current grant calls, and shares news from the international scientific community.

The department also initiates science outreach activities and organizes educational meetings for children and young people, helping them explore the wide range of professions at the Institute and supporting informed career choices.

The following units operate within the Research Department:

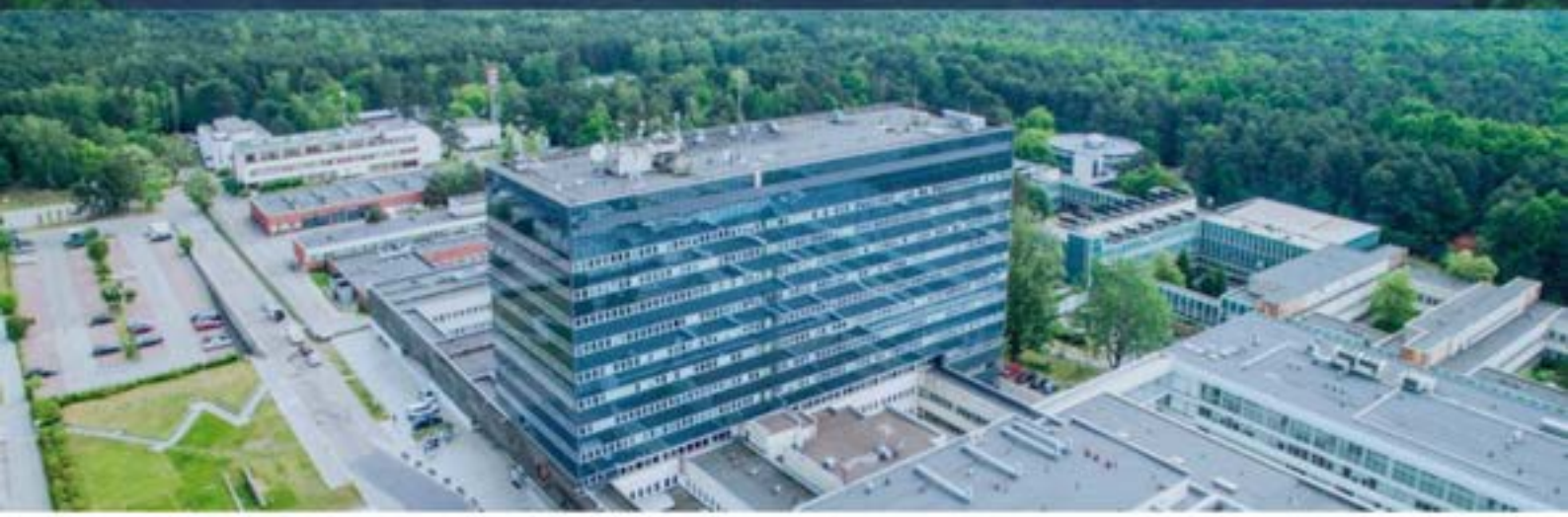
- Paediatric Clinical Research Support Center (CWpediBK),
- Paediatric Regional Center for Digital Medicine (PeRCMC),
- Biobank.

Featured Implementations

Antek app "Mom, Dad, Check if I'm Developing Healthily"
is a mobile application designed for parents. It supports the early detection of physical development disorders in children and adolescents, such as:

- growth deficiency of various origins,
- precocious puberty,
- nutritional disorders, including both malnutrition and overnutrition in children and adolescents.

The app is based on developmental standards recommended by the Polish Ministry of Health and was developed in collaboration with experts from the Anthropology Lab at CMHI.
Its development was informed by findings from two nationwide research projects: OLAF PL0080 and OLA NR13 0002 06



antek

Patients involvement

CMHI is a founding member of national initiatives aimed at legislative advocacy and shaping pediatric patient-centered health policy.

- **Polish Paediatric Clinical Trials Network (POLPEDNET)** - a network of Polish paediatric hospitals, whose mission is to improve the quality of clinical trials for future medicinal products across all paediatric age groups. The network's activities also include advising on the planning of clinical trials, gathering feasibility data on potential participants, providing training (e.g. for research nurses) also supporting data collection for clinical trials. POLPEDNET is open to cooperation with both the pharmaceutical sector and paediatric patient support or advocacy organizations, including international partners.

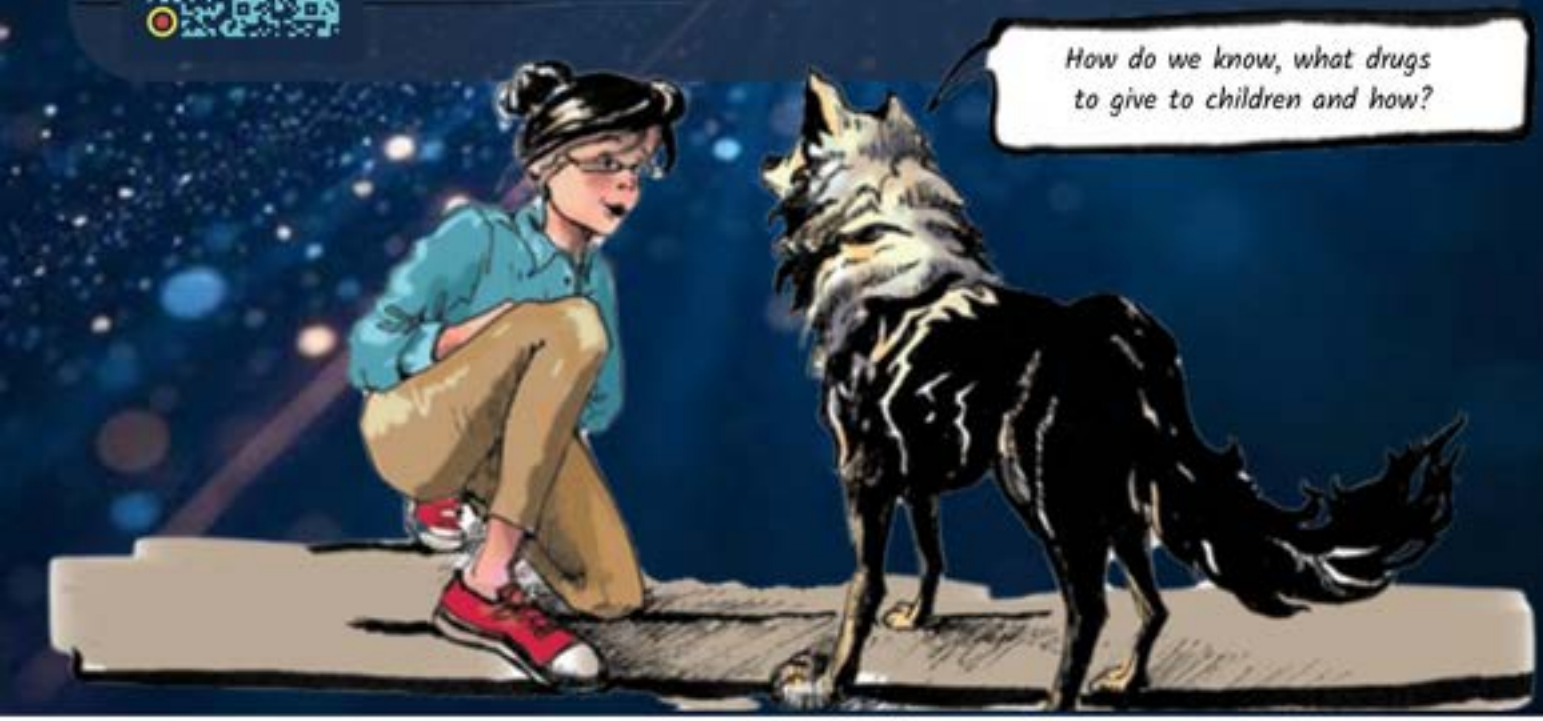
- **Young Person Advisory Group (YPAG) „POLPEDNET Kids" -** an organization comprised of children and young people, which consults on research projects - both in terms of content and how medicinal products are perceived by paediatric patients. The group's main goal is to actively support researchers and clinicians in the design and conduct of pediatric research by incorporating the perspective of young patients. YPAG is part of international youth advisory networks such as iCAN, TEDDY, and eYPAGnet, and collaborates with peers worldwide to share experiences and promote joint initiatives for the benefit of paediatric patients.

POLPEDNET KIDS

POLPEDNET
Polska Sieć Badań Klinicznych w Pediatrii

Patients involvement

The educational comic book "A Journey Through Clinical Trials" was created through a collaboration between CMHI and the international network conect4children (Collaborative Network for European Clinical Trials for Children)
This publication aims to popularize knowledge about clinical trials, particularly in the field of paediatrics.
In paediatric care, many medicines are used off-label, meaning 'outside' of their officially approved indications. This results from the limited number of clinical trials involving children. More paediatric research leads to a greater availability of well-studied, safe, and effective therapies for young patients.
It is essential for children and their families to understand the purpose and importance of clinical trials so they can make informed decisions about participation.
The comic book has been positively reviewed by scientific experts as an accessible and helpful tool for explaining this complex topic to young audiences.



International cooperation

CMHI is an active member of several international paediatric research organizations:

- **European Children's Hospitals Organisation** (ECHO) is a multi-country network of paediatric hospitals that brings together researchers, clinicians, and healthcare managers to promote the development and implementation of research-informed policies in children's hospitals across Europe.

- **European Paediatric Translational Research Infrastructure** (EPTRI) is a research infrastructure to accelerate the development of tailored solutions for the paediatric population. EPTRI promotes innovative, collaborative models aimed at reducing barriers to adopting new tools and practices in paediatric research and healthcare.

- conect4children (c4c) – Collaborative Network for European Clinical Trials for Children - focused on facilitating the development of new drugs and therapies for children of all ages. It is a pioneering opportunity to build capacity for the implementation of multinational paediatric clinical trials whilst ensuring the needs of babies, children, young people and their families are met.
  c4c seeks to provide a sustainable, integrated platform for the efficient and timely delivery of high-quality clinical trials in children across all medical conditions and phases of the drug development process. It also aims to introduce innovation at every stage of clinical development by establishing new models for both organization and execution.

International cooperation - ERN

European Reference Networks (ERN) - virtual networks involving healthcare units from across Europe. They include more than 900 highly specialized units from more than 300 hospitals in 26 EU countries. Their goal is to diagnose and treat rare diseases and multifactorial conditions that require highly specialized care and a combination of expertise and resources.

CMHI is a member of 7 Reference Networks:

- **EpiCARE** - European Reference Network on Rare and Complex Epilepsies
- **TRANSPLANTCHILD** - European Reference Network on Transplantation in Children
- **ITHACA** - European Reference Network on Rare Congenital Malformations and Rare Intellectual Disability
- **PaedCan-ERN** - European Reference Network for Paediatric Cancer
- **MetabERN** - European Reference Network for Rare Hereditary Metabolic Disorders
- **RARE-LIVER** - European Reference Network on Rare Hepatological Diseases
- **RITA** - Rare Immunodeficiency, Autoinflammatory and Autoimmune Diseases Network

European Reference Networks

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