ST. ANNA CHILDREN'S CANCER RESEARCH INSTITUTE

SCIENCE REPORT 2025

St. Anna Kinderkrebsforschung
CHILDREN'S CANCER RESEARCH INSTITUTE

SOUND OF SCIENCE

prymex

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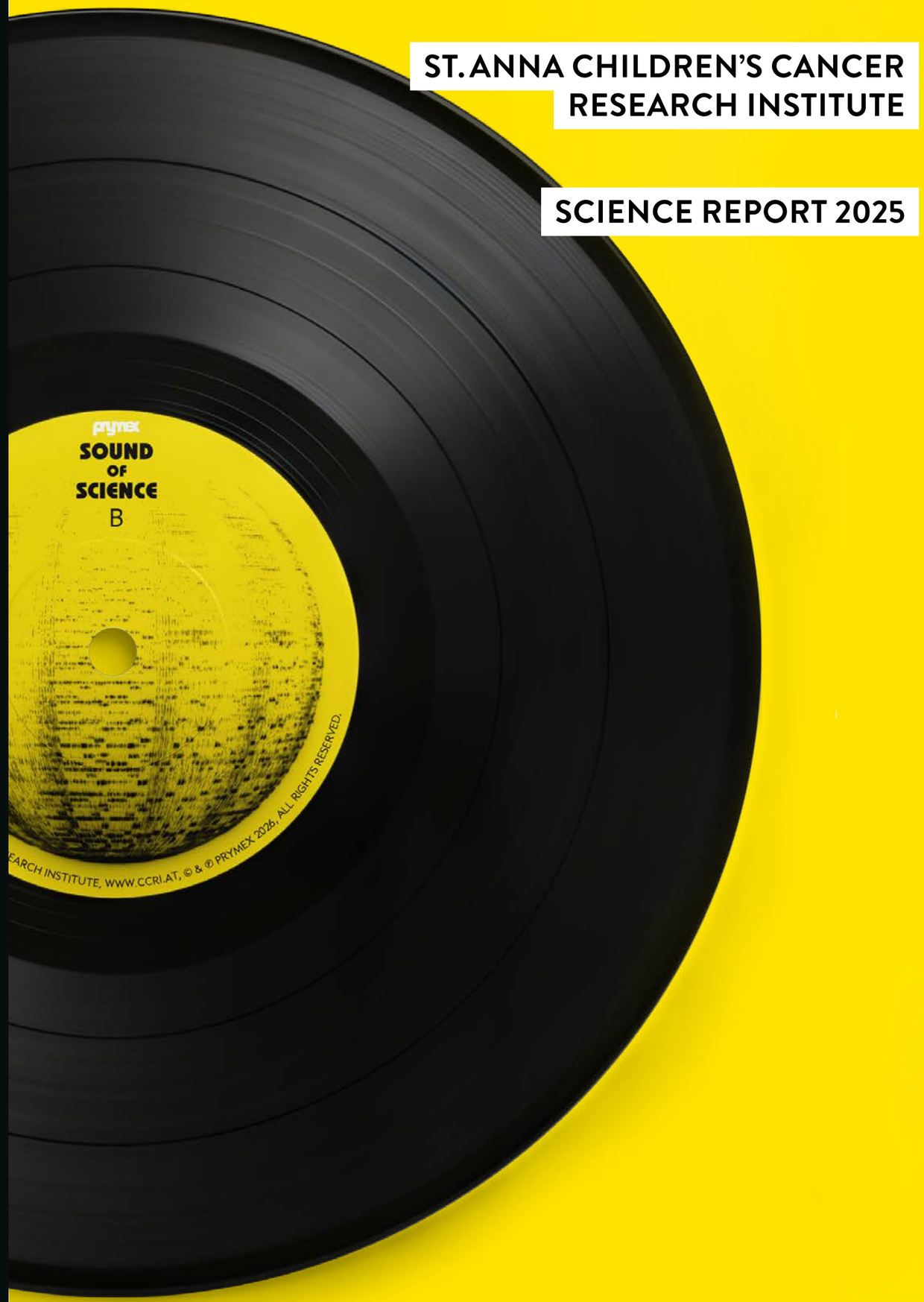

PRYMEX
aka Leo Miedaner
Visual Artist and Composer

A soundscape emerges between sterile laboratory lights and warm synthesiser textures that is as unusual as its source: 'Sound of Science' is an album that makes research audible. The man behind it is PRYMEX, a CGI artist and music producer who seamlessly merges electronic music with scientific reality through his refined sense of sonic aesthetics.

For St. Anna CCRI, PRYMEX developed a concept that transcends traditional composition. Immersing himself in the institute's laboratories, he recorded machine sounds, ambient atmospheres and precise everyday moments, such as the click of pipettes, transforming them into a multi-layered sonic language. These recordings are not merely used as effects; they actively shape the music by modulating synthesizers, defining textures and subtly influencing rhythm.

The result is a sound that feels both precise and atmospheric, driven by rich harmonies, a clear structure and a strong sense of balance. PRYMEX works with tension and release, allowing frequencies to collide and resolve, an approach that gives his tracks depth and movement. While influences from electronic music are evident, they coalesce into a unique and assured artistic voice.

Rather than focusing on the emotional impact of childhood cancer, the project shifts the perspective to the people behind the research. 'Sound of Science' becomes a musical reflection of life in the laboratory, a soundtrack for concentration, precision and progress. It does not seek to illustrate, but to accompany.

The project is released on vinyl, underscoring a commitment to quality and detail. PRYMEX refined his compositions with technical precision, developing existing ideas into a cohesive body of work. The result is a piece that is both artistically sophisticated and meticulously crafted.

With 'Sound of Science', PRYMEX shows how science and music can meet on equal terms, transforming the seemingly clinical into something vivid and unexpected.

Side A
Lab Nights
Emission
Cellular
Horizon
Phase Shift
Echoing Glow
Signal

St. Anna CCRI on Soundcloud.com

Side B
Double Helix
Fluorescence
Trial Phase
Radiance
Inner Guardians
Hidden Pathways
FACS
Catalyst

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We use artificial intelligence (AI) tools for proofreading and translation purposes. All AI-generated output is reviewed and edited by humans before publication.

INTRODUCTION

TRACK "PHASE SHIFT"

FOREWORD

The theme of this year's St. Anna CCRI Science Report is 'Sound of Science'. This is to reflect the way in which scientific progress unfolds, through the interplay of perspectives, disciplines and ideas. As an institute, our role is to create the conditions in which these elements can come together—in a focused and ambitious way, guided by a shared purpose.

Everything we do at our institute is informed by a clear mission: to advance research capable of improving outcomes for children and adolescents with cancer. At St. Anna CCRI, we are driven by the conviction that excellent science must ultimately have a meaningful impact.

Over the past year, the institute has continued to strengthen its position as a world-leading centre for pediatric oncology research. The breadth and depth of our scientific work as well as the progress achieved in 2025 are reflected in the contributions presented in this report.

Such progress is not just the result of scientific excellence, but also relies on a robust and highly professional institutional framework. What enables science at this level is the coordinated, forward-looking, reliable, efficient work of our research groups, core facilities, administrative teams and diagnostic subsidiary, Labdia. This ecosystem allows ideas to mature, collaborations to grow and innovation to take shape.

Similarly, St. Anna CCRI's strength lies in its close alignment with clinical reality. The continuous exchange between research and patient care ensures that our work remains relevant and responsive to clinical needs. This connection is embedded in the way we operate, forming a shared commitment across the institute.

As we move forward, we are building on a strong foundation. The recent transition in scientific leadership marks a continuation with a renewed focus and sense of responsibility. Building on new initiatives and an evolving research landscape, we are creating the conditions that will allow pediatric cancer research to develop further and realize its full potential.

This work has been made possible thanks to the commitment of many people. We are deeply grateful to our donors and sponsors, whose support enables us to continue our research independently. Above all, we would like to acknowledge the dedication of our employees, whose expertise and engagement drive our work forward day by day. We also want to thank our partners, mentors, funding agencies and board members for their trust, guidance and ongoing commitment.

Ultimately, our mission remains unchanged. Since the institute's founding, our purpose has been to improve the lives of young patients with cancer. This purpose continues to guide us, shaping our priorities, decisions and commitment to excellence.

St. Anna CCRI is a diverse and dynamic scientific community. It is this shared commitment—across research, clinical insight and institutional strength—that enables us to move forward together.

Eleni Tomazou
Scientific & Managing Director

Sabine Taschner-Mandl
Scientific & Managing Director

Jörg Bürger
CFO & Managing Director

Caroline Hutter
Head of Institute

THE NEXT STEP IN PEDIATRIC CANCER RESEARCH: SCIENTIFIC DIRECTORS SHARE THEIR VISION FOR THE FUTURE

St. Anna CCRI Scientific Directors Sabine Taschner-Mandl and Eleni Tomazou share insights into their vision for the institute.

© Ela Angerer

You both stepped into the role of Scientific Directors of St. Anna CCRI in 2025. What has this transition meant for you, and how do you understand your roles as Directors?

SABINE TASCHNER-MANDL:
The transition has fundamentally shifted my perspective. As a principal investigator, the focus is naturally on one's own research and team. As a director, the responsibility expands to the entire institute. Our role is to create conditions in which excellent science can flourish. That involves setting a clear strategic direction, supporting people and ensuring a supportive structure so that strong ideas can develop into meaningful impact.

ELENI TOMAZOU:
I fully agree—and what makes this transition particularly powerful is that we lead together. Our joint directorship allows us to bring different perspectives to the same goal. We challenge, complement and support each other, and we are united by a shared vision. This combination strengthens both our decisions and the direction of the institute.

What drives St. Anna CCRI today, and what is the core mission you are building on?

ELENI TOMAZOU:
Our mission is clear: to improve outcomes for children and adolescents with cancer through biomedical research. This mission consistently guides our work and decisions. The institute fosters an environment of true scientific excellence, where researchers are internationally competitive and projects are highly innovative. Our success in securing major grants demonstrates the quality and relevance of our work. Crucially, we are continuously strengthening the structures that allow ideas to progress, enabling sustained development and the creation of impactful projects that will ultimately benefit young patients.

SABINE TASCHNER-MANDL:
Building on this foundation, our mission is to generate new knowledge. Childhood cancers fundamentally differ from adult cancers as they arise from developmental processes, are driven by distinct molecular alterations and often affect growing tissues. Gaining insight into these diseases requires dedicated strong, curiosity-driven basic research. Over the past decades, we have built a broad and integrated research portfolio—from fundamental biology to innovative disease models as well as precision therapeutics approaches such as next-generation CAR T cell therapies and minimally invasive methods for tumor detection and disease monitoring. This scientific depth and breadth is the key strength of the institute.

What is your long-term vision for St. Anna CCRI?

SABINE TASCHNER-MANDL:
Our goal is to further strengthen St. Anna CCRI as a center of excellence that integrates state-of-the-art research, diagnostics and clinical application. The structures we have in place, including our connection with St. Anna Children's Hospital and our clinical diagnostics subsidiary Labdia, position us strongly to achieve this, bringing together leading research programs with a strong clinical trials unit, expert statisticians and dedicated cell processing unit capabilities.

ELENI TOMAZOU:
At the same time, our vision is shaped by the unmet needs. Although survival rates have improved significantly, effective therapies are still lacking for many patients, and survivors often experience long-term consequences. This is why we are developing precision oncology approaches: to enable therapies that are both more effective and less harmful.

A central theme in your vision is translation —how does this focus shape your priorities?

ELENI TOMAZOU:
Translation is about responsibility. It means ensuring that discoveries do not remain in the lab, but reach patients. This requires not only scientific excellence, but also the right infrastructure and a clear strategic focus.

SABINE TASCHNER-MANDL:
We are already seeing how powerful this approach can be. The MONALISA trial, for example, is the world-first study in which a liquid biopsy approach developed by our institute is now being tested to monitor for relapses in pediatric cancer patients. Witnessing our research translate into tangible benefits for patients is incredibly rewarding and underscores the real-world impact of our work.

How are you building the community needed to achieve this vision?

ELENI TOMAZOU:
One key element is education. We are training the next generation of leaders in pediatric cancer research. A major milestone is our physician scientist program, which enables clinical oncologists from St. Anna Children's Hospital to also lead research groups at St. Anna CCRI. This strengthens the scientific-clinical interface and builds a community of experts bridging biology and patient care. We further put emphasis on supporting young scientists—PhD students, postdoctoral fellows and junior faculty—in structured programs.

SABINE TASCHNER-MANDL:
In pediatric cancer research, collaboration is not optional, it is a necessity. Because these diseases are rare, real progress depends on strong networks. We benefit enormously from being part of a very strong and open scientific community in Vienna that brings together complementary expertise to accelerate discoveries and translation. St. Anna CCRI also has an excellent international reputation. We lead major European networks such as the European Reference Network for Pediatric Cancers (ERN-PeadCan) initiative and research consortia. These connections link us with other leaders in pediatric oncology across Europe and beyond.

This year's report is titled "Sound of Science". What role does creativity play in your work?

SABINE TASCHNER-MANDL:
Creativity is essential for progress. Science requires both structure and openness—the ability to recognize patterns, but also to think beyond the established path. That combination is what leads to new discoveries.

ELENI TOMAZOU:
Science is not driven by data alone, it is also driven by ideas. Designing new approaches means imagining solutions that do not yet exist. In that sense, science and art are closely connected: both begin with an idea and shape it into something meaningful.

If we look five years ahead, what will success look like for St. Anna CCRI?

ELENI TOMAZOU:
We want to strengthen our position as a center of excellence for pediatric cancer research worldwide with the long-term ambition that every child with cancer can be cured and live a full life. I also would love to see our young researchers and oncologists grow into internationally recognized experts in their fields.

SABINE TASCHNER-MANDL:
Success will be measured by impact—by clear improvements in patient outcomes that can be directly linked to our work. We want to be able to look back and say: this life-saving innovation was developed at St. Anna CCRI and made a real difference in the lives of young patients.

FACTS AND FIGURES

TRACK "HIDDEN PATHWAYS"

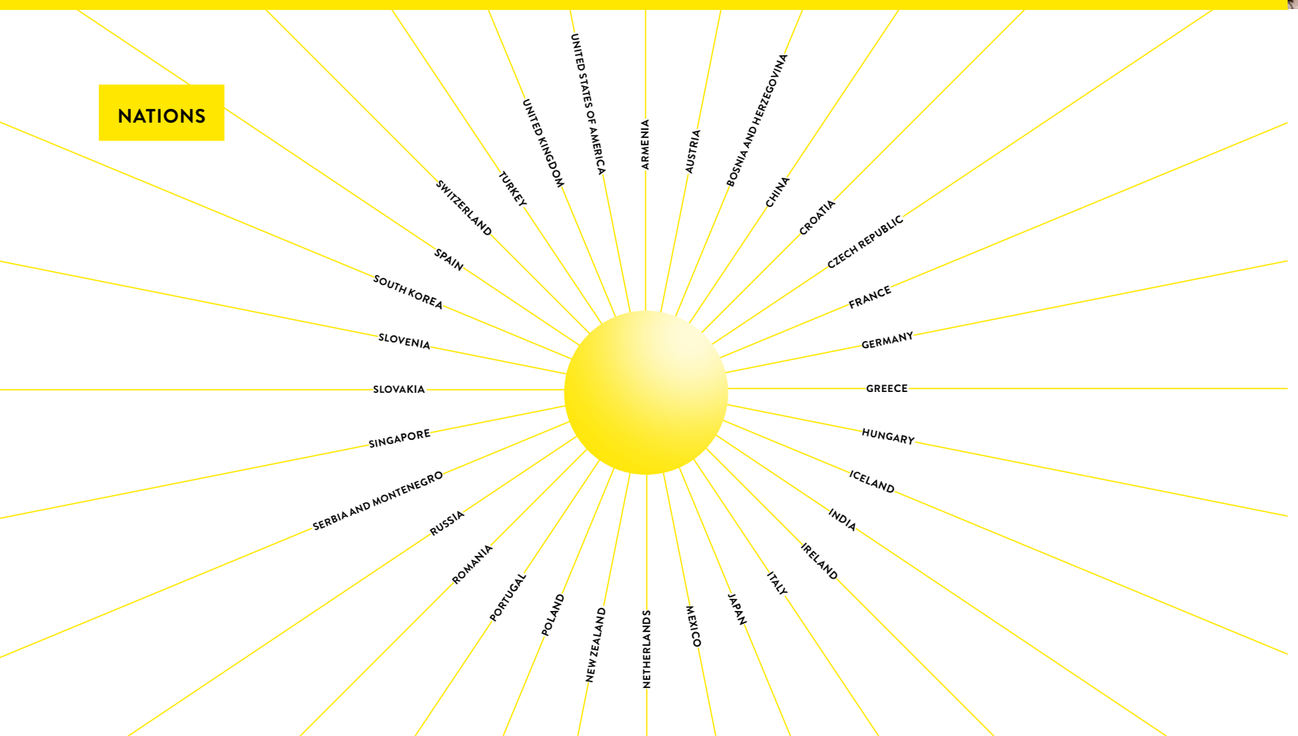
SURVIVAL RATES OF CHILDHOOD CANCER

> 80% of children survive their cancer diagnosis (5-fold compared to 1950s)
However, survival rates vary widely by cancer type.

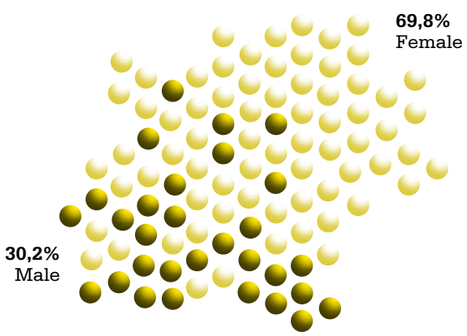
Hodgkin lymphoma
Malignant germ cell tumors
Nephroblastoma
ALL
Non-Hodgkin lymphoma
Neuroblastoma
Brain tumors
Osteosarcoma
AML
Rhabdomyosarcoma
Ewing sarcoma

Burdach et al., Mol Cell Pediatr 2018

NATIONS



STAFF COMPOSITION



WORKING AT ST. ANNA CCRI

The St. Anna Children's Cancer Research Institute (St. Anna CCRI) combines cutting-edge international research with a clear mission: to sustainably improve the treatment of children and adolescents with cancer through innovative diagnostic, prognostic and therapeutic approaches. Close collaboration between clinical practice and research creates an environment in which new ideas can be rapidly translated into meaningful impact—true to our motto "Bringing research from bench to bedside."

Behind our scientific achievements stands a diverse team of dedicated researchers, specialists and support units such as the Research Management Office, Science Communication and General Administration. People from around 35 nations work with us, creating a culturally rich environment that inspires creativity, fosters fresh perspectives, and includes a workforce with over 50% women. Diversity is not just a value—it is part of our everyday reality.

Our 15 research groups thrive on a strong collaborative spirit. Expertise is actively shared, interdisciplinary projects are the norm, and the fact that we are an internationally renowned center for biomedical research located in the heart of Vienna enables numerous partnerships with national and international institutions.

Open communication and active exchange are an integral part of daily life at our institute: regular seminars, internal retreats, institute-wide events and international conferences provide space for discussion, feedback and scientific development. A Scientific Advisory Board composed of leading international experts continuously supports and evaluates our work, ensuring that we meet the highest scientific standards.

It is particularly important to us that new colleagues feel welcome and can grow within our institute. A structured onboarding program facilitates the start, while both personal and professional development is supported by a wide array of internal training opportunities—ranging from scientific training such as scientific writing or fundamentals of statistics to soft skills like communication, self- and time-management or leadership essentials. In this way, we foster talent at all career stages.

We also take part in career fairs and recruiting events where we regularly provide insights into our research and working environment. Occasions like these allow us to showcase the values defining working at St. Anna CCRI: scientific excellence, team spirit and a shared purpose.

The St. Anna CCRI offers a wide range of career opportunities. For more information, please visit our career page at https://ccri.at/working-at-the-ccri/job-openings/. We look forward to meeting you!

NEWS TICKER

TRACK"TRIAL PHASE"

Over 100 attendees from Viennese research institutions joined the first edition of the St. Anna CCRI Symposium. © Lukas Lach / St. Anna CCRI.

1ST ST. ANNA CCRI SYMPOSIUM: CANCER EPIGENETICS

St. Anna CCRI launched the first edition of its annual symposium, which focused on how epigenetics—the molecular switches that turn genes on or off—influences cancer development.

The event, held at the Van Swieten Hall of the Medical University of Vienna, brought together researchers from St. Anna CCRI and other Viennese institutions to hear talks by world-leading experts in the field of cancer epigenetics. Organized by Principal Investigators Davide Seruggia, Eleni Tomazou, Florian Grieben and Kaan Boztug, the meeting aimed to foster international collaborations with the speakers and their institutions.

FROM BENCH TO BEDSIDE AND BACK TO LIFE – CAROLINE HUTTER DELIVERS HER INAUGURAL LECTURE AS PROFESSOR FOR PEDIATRIC HAEMATOLOGY AND ONCOLOGY AT THE MEDUNI VIENNA

St. Anna CCRI's Head of Institute Caroline Hutter delivered an inaugural lecture at the Medical University of Vienna.

The talk focused on the history of St. Anna Children's Hospital, its significance, and the last decades of progress in pediatric hematology and oncology. In her speech, Hutter advocated for the promotion of physician-scientists who can connect the clinical and research worlds, an essential step toward recognizing the clinical relevance of laboratory discoveries.

She also provided insights into the current research areas of St. Anna CCRI, pointing out the importance of collaboration between St. Anna CCRI, St. Anna Children's Hospital, the Medical University of Vienna and other relevant institutions from all over Austria.

Caroline Hutter, group leader as well as Head of Institute at St. Anna CCRI and Medical Director of the St. Anna Children's Hospital, highlighted the strong connection between both institutions. © Medical University of Vienna.

WOMEN IN SCIENCE: RESEARCHERS SHAPING THE FUTURE

For Women in Science Day, we launched a social media campaign highlighting the career experiences of some of the women working at St. Anna CCRI.

Women are essential in science—they shape research, drive innovation, and ensure that discoveries are approached from diverse perspectives. However, stereotypes, structural barriers and a lack of role models still discourage girls from considering a future in science.

At St. Anna CCRI, women—who comprise 67% of the workforce—are key to our mission and success. For this year's Women in Science Day, we shared the stories of five of these women—from Scientific Director Eleni Tomazou to PhD student Martha Zylka and biomedical analyst Mirella Larch—hoping their testimonies will empower more young women to pursue a career in science.

Five St. Anna CCRI employees participated in the campaign. (from left to right): Mirella Larch, Anna Hurt, Martha Zylka, Anna Hakobyan and Eleni Tomazou. © Lukas Lach / St. Anna CCRI.

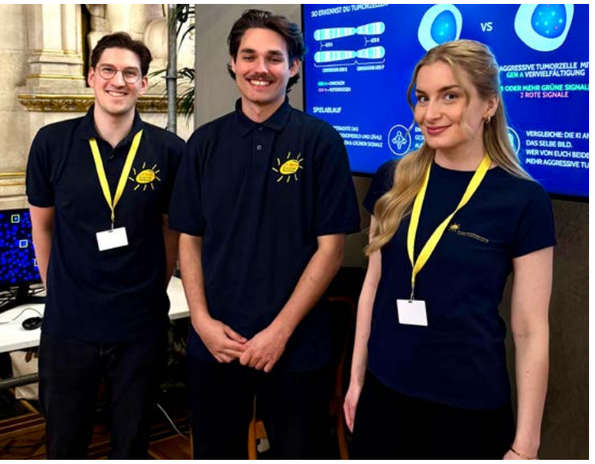
YOUNG AGPHO: NEW NETWORK FOR PEDIATRIC ONCOLOGY

St. Anna CCRI hosted the kick-off meeting of the new initiative aiming to connect early-career Austrian physicians specialized in pediatric hematology, oncology and immunology.

The Young AGPHO initiative is a newly established platform within the Working Group for Pediatric Hematology and Oncology (AGPHO) of the Austrian Society for Pediatrics and Adolescent Medicine (ÖGKJ). It seeks to foster clinical and scientific exchange and build a strong network for early-career physicians in Austria, offering training opportunities and integrating young investigators into national studies and registries.

The aims of the initiative also include promoting research collaboration among Austria's pediatric oncology centers and increasing visibility for those issues affecting children and adolescents with hematologic and oncologic diseases.

Participants in the kick-off meeting of the Young AGPHO initiative. © Lukas Lach / St. Anna CCRI.



St. Anna CCRI researchers (f.l.t.r) Peter Zöscher, Simon Gutwein and Theresa Michls shared their research with visitors big and small.
© Lukas Lach / St. Anna CCRI.

VIENNA RESEARCH FESTIVAL

St. Anna CCRI was one of the exhibitors at the Vienna Science Festival 2025.

Visitors of all ages were welcomed by our researchers to explore childhood cancer research up close, receiving insight into how AI can improve tumor detection and how personalized medicine is transforming cancer treatment.

The weekend-long event was attended by more than 12.000 visitors who could learn about the research performed by various Viennese scientific institutions. The St. Anna CCRI's booth, presented by our researchers and communication team, offered entertaining games and simulations with a view to connecting with the general public and giving a picture of the institute's work and mission.

VIENNA CITY MARATHON 2025

The St. Anna CCRI Mixed Relay Team took first place in their category—and second total—among all relay teams in the Vienna City Marathon.

A total of 40 St. Anna CCRI members came together for a shared purpose, demonstrating that the key to success lies in endurance, unity and team spirit. The institute's participation in the Vienna City Marathon has become an established tradition, with the number of our scientists and staff who join this fantastic event growing every year.

St. Anna CCRI researchers participated in the Vienna City Marathon.
© CeMM.

BUILDING A RESEARCH AND INNOVATION ECOSYSTEM FOR RARE DISEASES

ERN representatives at the European Parliament. © Ruth Ladenstein.

St. Anna CCRI group leader Ruth Ladenstein participated in a meeting at the European Parliament to discuss how to better support research, innovation and care for patients with rare diseases.

Research on rare diseases is often overlooked for funding and support due to the lack of commercial interest. However, given that millions of people worldwide suffer from such diseases, finding a cure for them is imperative. The "Building a Research and Innovation Ecosystem for Rare Diseases" meeting, which took place at the European Parliament in Brussels, brought together experts, policy-makers and stakeholders from across Europe to discuss new strategies to increase support for research, innovation and care in this field.

Professor Ladenstein was invited to share her expertise on the urgent need for sustainable infrastructure, robust clinical trial networks and a closer alignment between science and policy. Her testimony—tightly linked to her experience at St. Anna CCRI and her close collaboration with the St. Anna Children's Hospital— underscored the importance of long-term investment and EU-wide coordination to accelerate progress for the millions of people affected by rare diseases.

TWO ABSTRACTS HONORED AT THE OEGHO ANNUAL MEETING

Two abstracts submitted by St. Anna CCRI researchers Chantal Lucini as the first and Thomas Lion as the senior author received awards at the annual meeting of the Austrian Society for Hematology and Medical Oncology (OeGHO).

Chantal Lucini´s abstract "Differential *in vitro* sensitivity of BCR::ABL1 kinase domain mutations to tyrosine kinase inhibitors depending on the p190 or p210 background" received a Best Poster Award and her second abstract "Triple drug treatment options for highly resistant compound BCR::ABL1 mutation T315I&F359V in Ph-positive leukemia" received a Best Abstract Award. Chantal presented her work in the prestigious Best Abstract Session.

The annual meeting of the Austrian Society for Hematology and Medical Oncology brings together Austrian researchers and doctors in the fields of hematology and oncology to exchange ideas and share the progress of their research. This year's recognitions highlight the impact and excellence of St. Anna CCRI's work in pediatric oncology and hematology research.

Chantal Lucini receives a Best Abstract Award. © St. Anna CCRI.

ST. ANNA CCRI PARTICIPATED IN PINT OF SCIENCE

Marie Bernkopf shared her research on neuroblastoma diagnostics at Pint of Science Austria. © St. Anna CCRI.

Speaking at Pint of Science Austria, St. Anna CCRI scientist Marie Bernkopf presented her work on neuroblastoma and expounded on how liquid biopsy allows better disease monitoring.

Even after a successful treatment, what keeps patients and their families on edge for years is the risk of relapse. Will cancer return?
And will the doctors find it in time? Constant monitoring can help keep watch but requires invasive tissue biopsies.

Liquid biopsy is an innovative method that relies on simple blood samples to check for genetic markers of cancer. This less invasive method makes monitoring far more tolerable for young patients. In addition, liquid biopsy has a higher sensitivity, allowing doctors to detect relapses at an earlier stage and to personalize treatment strategies.

At Pint of Science Austria, Marie Bernkopf presented the MONALISA project, which aims to establish liquid biopsy as a new post-cancer monitoring tool across Europe.

SIOP EUROPE 2025 IN BUDAPEST

The 6th Annual Meeting of the European Society for Paediatric Oncology was held in Budapest, Hungary.

At the event, St. Anna CCRI Scientific Director Sabine Taschner-Mandl presented the latest milestones achieved by the MONALISA project, including progress on its clinical trial, harmonized liquid biopsy lab protocols, retrospective data collection, a clinical database & support tool and a web-based patient-reported outcome application.

St. Anna CCRI Scientific Director Sabine Taschner-Mandl presented the latest progress of the international MONALISA project. © St. Anna CCRI.

LIFE SCIENCES CAREER FAIR

St. Anna CCRI researchers participated in the Vienna Life Sciences Career Fair, where they interacted with students and young professionals considering to pursue a scientific career.

The Life Sciences Career Fair is an annual event providing jobseekers and potential employers an opportunity to meet and network. St. Anna CCRI had set up a booth where our researchers discussed their career experiences in pediatric cancer research and advised talented students and young professionals on their future career steps.

Our team (Christina Schendl and Theresa Michls) shared their career experiences with students and young professionals interested in science. © St. Anna CCRI.

Ruth Ladenstein shared insights on pediatric cancer care. © St. Anna CCRI.

PRECISION DIAGNOSTICS FOR PERSONALIZED CANCER CARE AT THE EUROPEAN PARLIAMENT

The event showcased the achievements of the INSTAND-NGS4P project in terms of improving patient access to innovation across Europe, with key contributions coming from Kaan Boztug and Ruth Ladenstein.

The meeting brought together policymakers, EU Commission officials, researchers, clinicians and patient advocates. Ruth Ladenstein chaired a session on the “Medical need for NGS and precision diagnostics in cancer care,” which highlighted the urgent need to integrate Next Generation Sequencing (NGS) technology into routine cancer diagnostics in order to improve treatment outcomes across Europe.

FIRST WORKSHOP FOR AUSTRIA'S NATIONAL MIRROR GROUP

Representatives of Austrian research institutions, clinical care, funding bodies and the Ministry of Health have developed a 3-year strategic roadmap.

Under the European Rare Diseases Research Alliance, the National Mirror Group—initiated and led by Kaan Boztug—aims to strengthen national coordination, foster collaboration among Austrian institutions and contribute to a unified European ecosystem for research on rare diseases.

The Workshop brought together 14 key stakeholders from Vienna, Graz, Innsbruck and Salzburg to develop a 3-year strategic roadmap tackling the question of how to improve clinical care, research, health policy and financing, outreach and advocacy.

Participants in the National Mirror Group met to coordinate their efforts toward improving research and clinical care for rare diseases
© Lukas Lach / St. Anna CCRI.

SIOP-RTSG: ADVANCING RESEARCH ON RENAL PEDIATRIC CANCER

St. Anna CCRI researchers presented their work on renal cancer at the annual meeting of the International Society of Pediatric Oncology's Renal Tumor Study Group.

Researchers Florian Halbritter, George Cresswell, Leo Kager, Anna Hakobyan, Marie Cebula and Maud Plaschka represented the St. Anna CCRI at the meeting held in Liverpool.
The highlights included Florian Halbritter taking part in the Biology panel discussion, and Maud Plaschka presenting her work on single-cell analysis aiming to discover new biomarkers of Wilms tumor, a rare type of pediatric renal cancer. Marie Cebula showcased a poster on ß-catenin's role in Wilms tumor.

During a visit to the Vienna Children's University at the MedUni Vienna, St. Anna CCRI transformed complex medical research into an exciting detective game.

Under the title "The Hunt for the Tumor Cell—A Case for C&E," Caroline Hutter and Eleni Tomazou guided over 100 curious children—from 7 to 12 years old—through the fascinating world of cell biology and cancer research. Using an interactive format, they explained what a cell actually is, and how healthy cells can become cancerous.

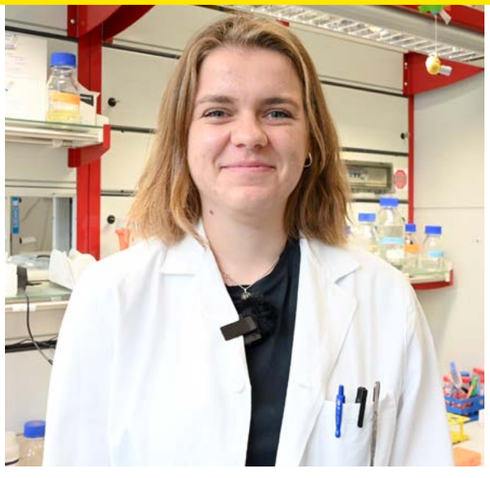
The experts illustrated how "errors" in the DNA's instructions cause cells to multiply uncontrollably and ignore any stop signals, leading to cancer development. Also, the children learned that every cancer is unique, which is why it requires tailored treatment.

The hands-on experience was a vivid demonstration of how complex yet fragile our genetic system is, and will hopefully educate and inspire young students to be curious about science.

THE HUNT FOR THE TUMOR CELL: ILLUMINATING PEDIATRIC CANCER RESEARCH TO CHILDREN

© Lukas Lach / St. Anna CCRI.

DOC FELLOWSHIP: SEEKING NEW WEAK POINTS IN BLOOD CANCER



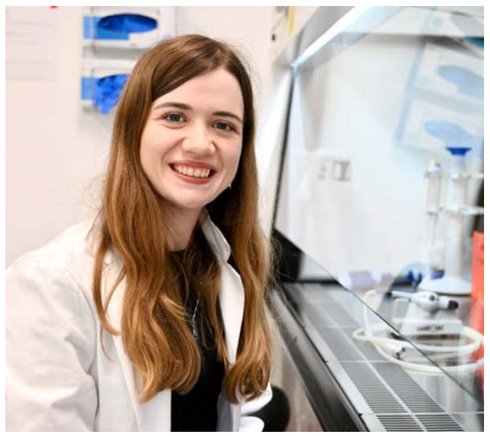
Christina Horstmann received a fellowship to support her research on acute myeloid leukemia. © Lukas Lach / St. Anna CCRI.

Christina Horstmann, PhD student in Florian Grebien's group, received a PhD fellowship from the Austrian Academy of Sciences (ÖAW).

Christina's project investigates the molecular causes behind relapses in patients with acute myeloid leukemia (AML)—a major issue affecting up to 30% of patients. Studying how AML cells develop and progress is limited because these cells often contain multiple mutated genes involved in different regulatory processes. Christina's research focuses on zinc finger proteins, a large protein family involved in cellular processes such as protein degradation, signal transduction, and epigenetic regulation.

Christina's innovative project aims to screen specific domains—small protein regions with a particular function—within zinc finger proteins in order to discover how they contribute to cancer development. Her findings could point to new molecular targets for precise therapies against AML.

BIF FELLOWSHIP: BUILDING EWING SARCOMA MODELS FROM SCRATCH



Hana Bernhardova received a fellowship to support her PhD project studying Ewing sarcoma. © Lukas Lach / St. Anna CCRI.

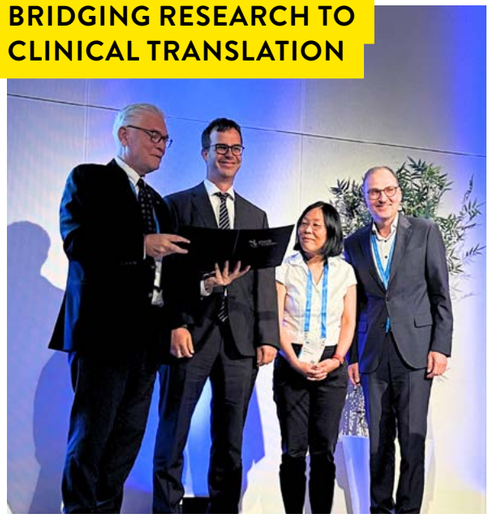
Hana Bernhardova, PhD student in Eleni Tomazou's group, received a PhD fellowship from the Boehringer Ingelheim Fonds for her research on why certain oncogenic drivers only cause cancer in specific cellular contexts.

Ewing sarcoma is among the most aggressive pediatric tumors, affecting bone and soft tissue. However, researchers are still struggling to figure out the cellular origin of the disease, a missing piece that hinders the development of more effective therapies.

In her project, Hana is using a *"build-it-to-understand-it"* approach aimed at modeling the entire course of the disease, from the earliest precancerous stages to the formation of metastasis. Employing pluripotent stem cells, Hana seeks to generate different potential cells of origin and study how they respond to carrying the fusion oncogene EWSR1::FLI1, the main driver of Ewing sarcoma.

Hana's research could establish a much-needed cellular model of this disease that can accurately mimic its progression and be used to test new therapeutic approaches.

BRIDGING RESEARCH TO CLINICAL TRANSLATION



Kaan Boztug was recognized for his work bridging research and clinical practice. © St. Anna CCRI.

Kaan Boztug was awarded the Novartis Prize for Therapy-Relevant Immunological Research 2025 for his work bridging research and clinical practice to understand inborn errors of immunity.

Boztug's group investigates the molecular mechanisms underlying immune (dys)regulation, including autoimmunity and cancer predisposition in childhood. Boztug has played a leading role in the initial description and molecular characterization of more than 20 previously unknown diseases.

Due to his dual role as a clinician and a scientist, Boztug is particularly committed to using new scientific findings to develop new treatment options, translating molecular insights directly into the care of young patients. This continued effort was recognized by the German Society for Immunology and the Novartis Foundation for Therapeutic Research. Botztug shared the award with Professor Evelyn Ullrich from the Medical University of Frankfurt.

Sara Wernig-Zorc, shared postdoctoral researcher in the labs of Sabine Taschner-Mandl and Florian Halbritter, was awarded for her talk on "Immune evasion and therapy resistance in neuroblastoma bone marrow metastasis."

Cancer cells are masters of evolution, quickly adopting new survival strategies that help them evade the immune system and escape even the most advanced therapies. Understanding the molecular mechanisms behind cancer adaptation is essential to develop better treatments and avoid relapses.

At the 2025 SIOPEN Advances in Neuroblastoma Research meeting held in Washington, Sara Wernig-Zorc presented the results of her recent study on how therapy-resistant neuroblastoma cells adapt and survive in the bone marrow—the most common site of metastasis and relapse. Her inspiring talk was selected as the Best Oral Presentation in the "Basic Science" category.

SARA WERNIG-ZORC WINS BEST ORAL PRESENTATION AT SIOPEN ANR2025

Sara Wernig-Zorc's talk was selected as the Best Oral Presentation in the "Basic Science" category © St. Anna CCRI.

ÖGAI – FRITZ UND URSULA MELCHERS DISSERTATION PRIZE FOR JANA BLOCK

Jana Block, PhD student in Kaan Boztug's group, identified a novel immune dysregulation disorder.

Jana's thesis demonstrated that germline loss-of-function mutations affecting the DOCK11 gene impact blood cell formation and T-cell homeostasis, disrupting both hematopoiesis and immune regulation. Her findings uncovered a previously unknown molecular basis for a rare immunological disorder, expanding our understanding of inborn errors of immunity.

Jana's work was recognized by the Austrian Society of Allergology & Immunology at its Scientific Symposium held in Vienna.

Jana Block's outstanding PhD dissertation received an award from the Austrian Society of Allergology & Immunology. © St. Anna CCRI.

FOUR ST. ANNA CCRI RESEARCHERS HONORED FOR HIGH-IMPACT PEDIATRIC ONCOLOGY STUDIES

St. Anna CCRI researchers Kaan Boztug, Chantal Lucini, Florian Halbritter and Leo Kager were honored for their groundbreaking contributions to pediatric research.

The 63rd Annual Meeting of the Austrian Society of Pediatrics and Adolescent Medicine (ÖGKJ) brought together leading researchers under the theme "Together Into the Future." During this prestigious gathering—held during Childhood Cancer Awareness Month—four distinguished scientists from St. Anna CCRI were honored for their groundbreaking contributions to pediatric research.

Leo Krager's publication "A single-center cohort study of patients with hereditary spherocytosis in Central Europe reveals a high frequency of novel disease-causing genotypes" was selected as the best clinical work.

Chantal Lucini's publication "Prevalence of fungal DNAemia mediated by putatively non-pathogenic fungi in immunocompromised patients with febrile neutrpenia: a prospective cohort study" was selected as the best onco-clinical work. Kaan Boztug's publication "LTβR deficiency causes lymph node aplasia and impaired B cell differentiation" was selected as best experimental work. Florian Halbritter's publication "A human neural crest model reveals the developmental impact of neuroblastoma-associated chromosomal aberrations" was selected as the best oncologic-experimental work.

This recognition underscores the scientific relevance and international visibility of our research efforts, demonstrating our institute's commitment to advancing pediatric medicine through rigorous scientific investigation and clinical excellence.

The work of our researchers was recognized by the Austrian Society of Pediatrics and Adolescent Medicine. (from left to right): Florian Halbritter, Kaan Boztug, Chantal Lucini, Thomas Lion and Leo Krager. © St. Anna CCRI.

Within our activities to mark Childhood Cancer Awareness Month, our researchers showed their work to a number of political leaders in Austria. © St. Anna CCRI.

INSTITUTIONAL VISITS TO ST. ANNA CCRI

District Chairwoman Saya Ahmad, Federal Minister Korinna Schumann and Vienna's Health Councilor Peter Hacker honored our activities during Childhood Cancer Awareness Month with visits to our institute.

The annual Childhood Cancer Awareness Month is an important occasion for us to put childhood cancer center stage. As part of our initiatives to highlight our work on pediatric cancer, we invited Austrian political leaders to visit our institute and witness first-hand our work and its impact.

AMBASSADOR CIRCLE

St. Anna CCRI Ambassadors raise awareness for Childhood Cancer Research.

In 2025, a major milestone was reached with the charity gala, 'KIDS FUTURE UNLOCKED', which was organised by the recently established St. Anna CCRI Ambassador Circle and took place at Vienna City Hall. The event reflected a shared vision of giving children with severe diagnoses new hope and better prospects for the future.

Established in December 2024, the St. Anna CCRI Ambassador Circle bring togehter the founder and chairman of Bitpanda Eric Demuth, Daniel Jelitzka, co-owner of JP Immobilien; Michael Höllerer, CEO of Raiffeisen-Holding NÖ-Wien and Raiffeisenlandesbank NÖ-Wien; and Dominic Thiem, entrepreneur and former professional tennis player.

Through their strong personal commitment and influential networks, they support St. Anna CCRI in raising awareness of childhood cancer research and mobilising new supporters. Through their dedication, the ambassadors are making a significant contribution to the long-term work of St. Anna CCRI.

St. Anna Children's Cancer Research Ambassador Circle (from left to right): Daniel Jelitzka, Eric Demuth. Dominic Thiem and Michael Höllerer
© Kids Future Unlocked 2025

GUEST LECTURES

Every year, we invite outstanding researchers from all around the world to share their work with us in our Guest Lecture series. These lectures are a fantastic opportunity to learn about other research fields, get new ideas and establish fruitful collaborations. In 2025, our scientists enjoyed a fantastic lineup of guest speakers.

KATLEEN DE PRETER

Katleen De Preter, Co-Founder of the Cancer Research Institute Ghent and research group leader at the Center for Medical Biotechnology (VIB-UGent), shared how epi-genomic analysis of cell-free DNA can help monitor and diagnose cancer metastasis.

Cancer can spread from its tissue of origin to other bodily structures. This phenomenon, called metastasis, can affect many crucial body functions and is one of the main causes of cancer-related death.

In her talk, De Preter highlighted how analyzing cell-free DNA, small DNA fragments found in blood, urine and other bodily fluids, can be used as better and less-invasive method to detect and monitor metastasis in cancer patients.

Katleen De Preter shared her research on diagnosis diagnosis and monitor metastasis in cancer patients © VIB-UGent Center for Medical Biotechnology

Sander Lambo, postdoctoral fellow at the Dana-Farber Cancer Institute, shared new advances in studying genomic and epigenomic evolution in cancer cells and how they contribute to relapse in pediatric cancer.

Even after a successful treatment, cancer can return. Relapsed cancers are often more aggressive and respond less to treatment, making their prognosis worse. Understanding how cancer cells can adapt to survive the initial treatment is essential to develop more effective therapies that improve outcomes for pediatric cancer patients.

In his talk, Lambo shared his work studying how alterations in the DNA and in gene regulation can help cells better adapt and survive to treatment, and how understanding these alterations can inform new therapies that tackle relapse.

SANDER LAMBO

Sander Lambo shared his research on how pediatric cancer cells adapt and evolve to return after treatment. © Dana-Farber Cancer Institute

LENNART KESTER

Lennart Kester shared his research in improving cancer diagnostics using genomics technology. © Lukas Lach / St. Anna CCRI.

Lennart Kester, associate research group leader at the Princess Máxima Center for Pediatric Oncology, Utrecht, shared how advances in genomics can be translated into routine diagnostics.

Treatment of any pediatric cancer starts with acquiring the correct diagnosis. Kester's group combines state-of-the-art sequencing techniques with artificial intelligence models to develop novel molecular diagnostic techniques that improve pediatric cancer diagnostics, make it faster and more accurate.

In his talk, Kester highlighted how new advances in genomics science can increase diagnostic precision and result in timely, personalized treatment and improved outcomes for children with cancer.

JAN ŠKODA

Jan Škoda, Associate Professor at Masaryk University and group leader at the International Clinical Research Center at St. Anne's University Hospital Brno, Czech Republic, shared his research on repurposing mitochondria-targeted drugs to treat MYC-driven pediatric tumors.

MYC-driven pediatric tumors are fueled by abnormal activity of the MYC gene, a powerful regulator of cell growth and division. When MYC is overactive, it pushes cells to multiply rapidly and ignore normal control signals, contributing to aggressive, fast-growing tumors that are difficult to treat. MYC itself is hard to target, making these tumors "undruggable."

In his talk Jan Škoda shared how mitochondria-targeted drugs can be repurposed to target MYC-driven tumors, an original and promising approach to tackle these aggressive and resistant pediatric cancers.

Jan Škoda shared his work on repurposing existing drugs to treat pediatric tumors. © Lukas Lach / St. Anna CCRI.

JOSE TUBIO

Jose Tubio, director of the Mobile Genomes Group associate researcher and group leader at the Center for Research in Molecular Medicine and Chronic Diseases (CiMUS), shared how mobile DNA can impact human disease, including cancer.

Our DNA is not a static blueprint. Around 40% of our genome is made up of mobile DNA sequences such as retrotransposons, which can copy and paste themselves into new places in the genome, triggering major structural changes. Tubio's research has shown that retrotransposon insertions can cause cells to lose important tumor suppressor genes or even cause massive chromosomal rearrangements. These changes can help cancer cells evolve and survive, contributing to cancer development and progression.

In his talk, Tubio shared his work using long-read sequencing to map retrotransposon-mediated genetic alterations in cancer, revealing how these "jumping genes" can fuel cancer evolution.

Jose Tubio shared his research on how mobile DNA elements—called retrotransposons—impact cancer. © Lukas Lach / St. Anna CCRI.

PETER KHARCHENKO

Peter Kharchenko, Visiting Professor at the Institute of Science and Technology Austria and the Medical University of Vienna, shared how scientists can use advanced tools to distinguish tumor and non-tumor cells and study how they interact within their microenvironment.

Cancer cells within a tumor continuously interact with their environment, a communication that is essential to hide from the immune system and ensure the tumor's survival. This close relationship makes it difficult to discriminate between tumor and non-tumor cells and study how they interact.

Kharchenko's work focuses on developing high-precision methods—such as single-cell RNA sequencing—that allow scientists to look at cells one by one. Using these approaches, scientists can better study interactions between tumors and their environment.

In his talk, Kharchenko highlighted how transcriptomic data can be used to study the ways duplications or deletions of various DNA regions affect the communication between tumor and non-tumor cells, and how this contributes to cancer progression.

Peter Kharchenko shared his work on single-cell RNA sequencing to study how tumors interact with their environment. © Carina Heinreichsberger / St. Anna CCRI.

Our researchers are an integral part of the Neuroblastoma Research Network. © St. Anna CCRI.

ADVANCING NEUROBLASTOMA RESEARCH: SIOPEN 2025

St. Anna CCRI researchers presented their research in the Annual General Meeting of the Neuroblastoma Research Network (SIOPEN).

SIOPEN, which is part of the European Society for Paediatric Oncology, aims to facilitate clinical, translational and basic research for children and adolescents with neuroblastoma. At this year's Annual General Meeting, held in Copenhagen, our researchers contributed four scientific presentations, including Sören Strohmenger's talk, which received the award for best short presentation.

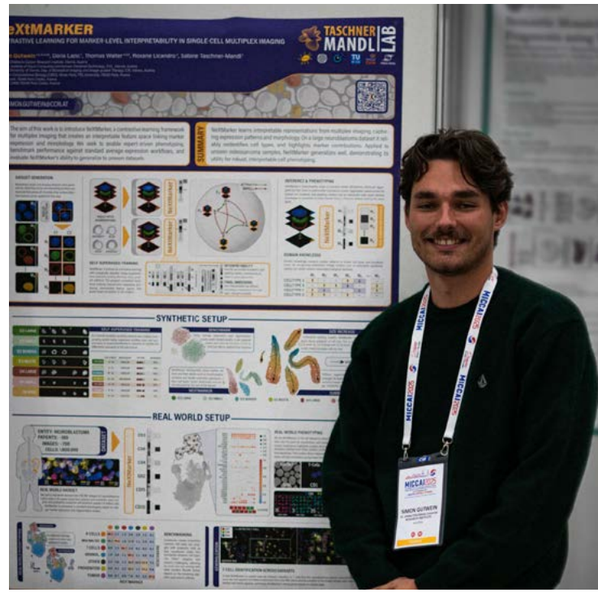
In addition, we held a dedicated meeting for the MONALISA consortium, which brought together nearly 30 members to discuss the project's latest development and plan the months ahead. The meeting featured presentations by the various Work Package leaders, including Sabine Taschner-Mandl and João Frade.

AI-BASED TOOL EMPOWERS CANCER DIAGNOSTICS

Simon Gutwein, PhD student in Sabine Taschner-Mandl's lab, won the Best Poster Award at the International Conference on Medical Image Computing and Computer Assisted Intervention.

Simon's poster—titled "NextMarker: Contrastive Learning for Marker-Level Interpretability in Single-Cell Multiplex tImaging"—portrayed his work developing an AI-based tool to support better childhood cancer diagnostics. It takes mere seconds for this tool to scan tissue images containing millions of cells and flag suspicious-looking cells, making cancer diagnostics faster and more precise and informing better precision treatment for children with cancer.

Simon presented his work at the Computational Pathology and Multimodal Data Workshop (COMPAYL), where he was awarded the Best Poster Award for the second year in a row.



Simon Gutwein was recognized for his work using AI to support better childhood cancer diagnostics. © Simon Gutwein.

ST. ANNA CCRI AT TEDAI

Peter Zöscher joined TEDAI Vienna to explain how artificial intelligence supports childhood cancer research.

Scientists can use imaging techniques like fluorescence in situ hybridization (FISH) to visualize genetic alterations that make cancer cells more dangerous. However, detecting these alterations—which appear as small colored dots—in a sample with up to a million cells can be difficult.

At **TED**AI, Peter Zöscher showcased an AI-based tool developed by Simon Gutwein—a PhD student in Sabine Taschner-Mandl's group—that can scan images in seconds, flag suspicious cells, and make diagnostics faster and more precise. This tool shows how AI can lead to better childhood cancer diagnostic and inform more effective treatment decisions.

Peter Zöscher discusses his work using artificial intelligence to support childhood cancer research. © St. Anna CCRI.

Klaus Fortschegger presented his work on modeling pediatric leukemia. © St. Anna CCRI.

STEM CELL RESEARCH LEADS THE WAY TO NEW TREATMENTS FOR PEDIATRIC CANCER

St. Anna CCRI researchers presented their research at the Annual Meeting of the Austrian Society for Stem Cell Research (ASSCR).

In the poster sessions, Beate Kratschmann and Thomas Eckhardt (Kameneva group) along with Luis Montano-Gutierrez and Katharina Wiener (Halbritter group) presented their projects. Katharina received the 1st prize for her poster.

In addition, Klaus Fortschegger (Strehl group) gave a short talk on the CBFA2T3::GLIS2 leukemia project, a collaborative effort between the Strehl and Halbritter groups that highlights how our research drives new therapeutic approaches to tackle pediatric leukemia.

RESEARCH GROUPS

TRACK "LAB NIGHTS"

BOZTUG GROUP

Immune Deficiency, Cancer Predisposition & Precision Oncology

PRINCIPAL INVESTIGATOR
Kaan Boztug

SENIOR STAFF SCIENTIST
Irinka Castanon Ortega (until 07/2025)

LAB MANAGER
Wojciech Garncarz (until 05/2025)

TECHNICAL ASSISTANT
Alexandra Frohne (jointly with Molecular Human Genetics)
Sarah Giuliani
Raul Jimenez Heredia
Theresa Humer
Claudia Kölbl (jointly with Dworzak Group)
Christina Rashkova
Anna Segarra Roca

SENIOR POSTDOCTORAL FELLOW
Artem Kalinichenko (until 01/2025)
Michael Kraakman
Cheryl van de Wetering

POSTDOCTORAL FELLOW
Juraj Konc (until 05/2025)

PHD STUDENT
Hena Hadzimujagic
Ben Haladik (until 06/2025)
Yirun Miao

RESEARCH ASSISTANT
Ümran Aba (until 01/2025)

VOLUNTEER
Jakob Berner (until 01/2025)
Sören Strohmenger (from 01-07/2025)

The immune system relies on the perfectly coordinated action of many different cell types with specific functions. These cells are in constant communication through complex physical and long-distance interactions. Alterations in these interactions can dysregulate the whole immune system and lead to an increased susceptibility to infections, autoimmune responses and cancer.

Our group studies the molecular basis of innate immune disorders, aiming to decipher the highly complex signaling pathways involved in immunodeficiencies and cancer susceptibility. Using state-of-the-art genomic approaches, including exome sequencing of patient samples, we examine rare immune disorders to identify their genetic and molecular causes. This work allows us not only to gain information about specific disorders, but also to complete our knowledge of how immune dysregulation contributes to autoimmunity and childhood cancer. Our research also focuses on identifying novel therapeutic targets for precision oncology therapeutics. Through better understanding the immune system's molecular blueprint we can pinpoint previously overlooked pathways that can be targeted in tailored treatments for patients resistant to standard therapies.

Our lab also investigates functional approaches to identify targeted treatment options for pediatric cancers including leukemias. Our research topics synergize to gain a clearer understanding of the origin of childhood cancers, the interactions and implications of the immune system therein, and to identify targeted treatments for children with cancer.

CHRISTIAN DOPPLER LABORATORY FOR NEXT GENERATION CAR T CELLS

PROJECT COORDINATOR
Manfred Lehner

RESEARCH ASSISTANT
Dominik Emminger
Hayeon Baik

PHD STUDENT
Sanjana Balaji
Theresa Michls

MASTER STUDENT
Lejla Zijadic (since 05/2025)

VOLUNTEER
Elise Sylvander

EXTERNAL MODULE OF THE CD LABORATORY: BOKU UNIVERSITY

HEAD OF EXTERNAL MODULE
Michael Traxlmayr

TECHNICAL ASSISTANT
Kerstin Holzer
Alexander Mischulnig

PHD STUDENT
Julia Mayer
Delia Sumesgutner

STUDENT ASSISTANT
Mara Fiehn
Sophie Lixl

CAR T cells are engineered immune cells that have revolutionized the treatment of leukemia. Scientists can take a patient's T cells and equip them with a new receptor that allows them to detect and destroy cancer cells with unparalleled efficacy. However, despite its early success, this strategy presents clear limitations: CAR T cells within the patient's body can sometimes overexpand and cause severe side effects or even attack healthy tissue.

Our laboratory tackles these problems by developing advanced CAR T cells whose function can be tightly regulated. Applying advanced protein evolution technology and molecular characterization, we design molecular switches that can be used to precisely tune the activity of CAR T cells during treatment. Through tailored genetic engineering, we also make CAR T cells more efficient at recognizing a tumor and reduce their activity against other tissues.

Overall, our research aims to make CAR T therapy safer and more effective, elevating it from a promise to a reality for pediatric cancer patients.

CLINICAL TRIALS UNIT

HEAD
Andishe Attarbaschi

SCIENTIFIC ASSISTANT
Claudia Zeiner-Koglin

SENIOR OPERATIONS MANAGER
Anke Emberger

TEAM LEAD STATISTICS
Ulrike Pötschger

STATISTICIANS
Helga Björk Arnardóttir
Evgenia Glogova
Paulina Kurzmann

CLINICAL MONITOR
Sibel Kurt
Anni Wu

STUDY DIRECTOR
Milen Minkov (since 09/2025)

CLINICAL TRIAL MANAGER
Lena Brandt (maternity leave)
Theresa Brügmann
Tijana Frank
Kathrin Galistl (since 03/2025)
Susanne Karlhuber
Monika Maronova
Nora Mühlegger
Sonja Neuzil
Marek Nykiel
Karel Pavka
Carina Rajner
Eva Sorz
Elfriede Thiem
Chiara Wertz

Clinical trials are meticulously designed research studies involving human volunteers, aimed at evaluating the safety, effectiveness and side effects of new therapies and diagnostic tools. These studies are an essential part of medical advancement as they allow doctors to test innovative therapies and patients to gain new hope. In rare diseases such as pediatric cancer, clinical trials are often coordinated between several hospitals across Europe.

The Clinical Trials Unit at St. Anna CCRI fosters clinical research in pediatric oncology by coordinating and facilitating national and international clinical trials and registries in line with respective EU directives or regulations. The Unit's experts closely collaborate with St. Anna Children's Hospital where patients involved in clinical trials are treated and monitored.

The Unit's work includes designing, running and analyzing academic clinical trials in the respective childhood cancer target populations. The experts also see to it that standard operating procedures are followed, thus ensuring patient safety as well as accurate data collection and management.

In addition, the Unit's team secures ethical committee approvals and ensures informed consent—key elements for any clinical research. In cooperation with pharmaceutical companies and international consortia, it helps build clinical trials designed to define the future of pediatric cancer therapies in Europe.

CRESSWELL GROUP

Cancer Evolution and Genomics

PRINCIPAL INVESTIGATOR
George Cresswell

POSTDOCTORAL FELLOW
Anna Hakobyan

PHD STUDENT
Béatrice Pichon

MASTER STUDENT
Anna-Laura Mettinger (since 03/2025)

VISITING PHD STUDENT
Lucrezia Valeriani (from 02-05/2025)

VISITING PHD STUDENT
Lucrezia Valeriani (from 02-05/2025)

Pediatric cancers adapt and change as they develop. Cancer cells often accumulate new genetic alterations that help them better survive, reproduce and invade other tissues. Importantly, these cells can also evolve to gain resistance against anti-cancer therapies and evade the immune system. Understanding how cancer cells within a tumor evolve and assume new traits is essential to develop more effective therapeutic approaches for pediatric cancers.

Our group uses advanced genomics and state-of-the-art computational analysis to reconstruct the evolution of cancer cells and uncover how cancers originate and adapt over time—and how they could behave in the future. We focus on identifying the key changes that allow cancer cells to diverge from normal cells and the ongoing alterations that enable cancer evolution. In particular, we study how large chromosomal alterations contribute to cell population adaptability and treatment resistance in aggressive forms of cancer.

Our work also focuses on developing new machine learning-based tools to better detect important genome alterations and other characteristics that can be key to how cancer populations evolve. Our work could identify vulnerabilities in the way cancer cells adapt, and inform new therapeutic strategies to target those weaknesses.

"In data, like in music, there is hidden meaning and beauty in what first appears as noise."

George Cresswell

DWORZAK GROUP

Immunological Diagnostics

PRINCIPAL INVESTIGATOR
Michael Dworzak

STAFF SCIENTIST
Margarita Maurer-Granofszky

TECHNICAL ASSISTANT
Claudia Kölbl (jointly with Boztug Group)
Marion Riebler

PROJECT TEAM MEMBER (PART TIME)
Thomas Heitzinger (since 08/2025)
Michael Reiter
Dagmar Schinnerl (jointly with Strehl Group)
Matthias Wödlinger (from 01-04/2025)

PROJECT TEAM MEMBER (EXTERNAL)
Evdoxia Gounari
Doris Kroiss

Pediatric leukemias and lymphomas represent a majority of childhood cancers, and some of the most deadly. In both cancer types, diagnostic approaches focus on detecting altered cells in the blood and bone marrow using flow cytometry, an imaging technique.

Our group focuses on developing and validating new flow cytometry-based diagnostic methods for pediatric leukemias and lymphomas to improve diagnosis, refine risk stratification, and enable more individualized treatment approaches. In particular, we focus on designing more accurate methods to detect residual cancer cells in pediatric patients—a key indicator of high relapse risk. Our work includes building new machine learning-based tools to automate minimal residual disease detection and quantification in different pediatric leukemia types.

Our efforts also concentrate on developing new models and methods that allow scientists to test novel therapeutic approaches for pediatric leukemia—an important step toward personalized therapies.

In addition, we lead the international standardization and quality control of diagnostic methods—a major stride into improving pediatric cancer care worldwide.

GREBIEN GROUP

Biology of Pediatric Leukemia Oncoproteins

PRINCIPAL INVESTIGATOR
Florian Grebien

PHD STUDENT
Carla Aranda Vallejo (since 09/2025)
Christina Horstmann

Pediatric leukemias are often very aggressive and unresponsive to treatment. Their development is caused by the dysfunction of factors that regulate normal blood development. Most cases are driven by aberrant proteins that gain oncogenic functions as a result of genetic mutations.

Our group studies leukemia-associated oncoproteins using state-of-the-art transcriptomics, proteomics and functional genomics as well as imaging approaches. Our research focuses on how fusion oncoproteins resulting from chromosomal rearrangements interact with normal proteins and the DNA to hijack natural developmental processes and drive pediatric leukemia.

For this purpose, we develop new cellular and animal models of pediatric leukemia and apply cutting-edge proteomics, epigenomics and transcriptomics approaches as well as CRISPR screenings and high-resolution imaging to deeply characterize how oncoproteins function inside cancer cells.

Our main goal is to identify novel vulnerabilities in pediatric leukemia cells that can be targeted therapeutically.

“Music and research both begin with listening—seeking patterns beyond the obvious. In that sense, research has its own sound: a creative process driven by curiosity, turning questions into solutions, especially in pediatric cancer.”

Florian Grebien

HALBRITTER GROUP

Developmental Cancer Genomics

PRINCIPAL INVESTIGATOR
Florian Halbritter

SENIOR POSTDOCTORAL FELLOW
Maud Plaschka
Christoph Hafemeister

POSTDOCTORAL FELLOW
Klemens Tümay Capraz (since 03/2025)

COMPUTATIONAL SCIENTIST
Sara Wernig-Zorc (jointly with Taschner-Mandl Group)

PHD STUDENT
Katharina Wiener

MASTER STUDENT
Hannah Marie Cebula (since 03/2025)

VOLUNTEER
Luis Montano Gutierrez (from 03-08/2025)

INTERN
Dylan Cameron (05/2025)

Childhood cancers are unique because they are not caused by the accumulation of many different mutations over decades, but are instead driven by singular mutations. These alterations throw developing cells off their track, causing them to grow uncontrollably and form a tumor. How this occurs, however, is poorly understood for most cancers.

Our group leverages advanced bioinformatics to study how minimal genetic changes cause catastrophic effects such as cancer. We focus on the molecular changes that occur in cells during normal development and during cancer and use this comparison to pinpoint the specific molecular pathways affected by cancer-causing mutations. For this, we build advanced stem cell-based models of cancer development and advanced data analysis algorithms for functional genomics. Combining these two elements, we can deeply characterize cancer cells at each step in their development to understand the molecular mechanisms involved and identify new therapeutic targets.

Our work includes collaborations with many other groups at the institute so as to facilitate the analysis of complex datasets and shed new light on deep biological questions. Our shared insight into the developmental aspects of pediatric cancer aims to open new avenues for detection, prognosis and treatment.

HUTTER GROUP

LCH Biology

PRINCIPAL INVESTIGATOR
Caroline Hutter

STAFF SCIENTIST
Raphaela Schwentner

TECHNICAL ASSISTANT
Philipp Ben Soussia-Weiss

SENIOR POSTDOCTORAL FELLOW
Giulio Abagnale

POSTDOCTORAL FELLOW
Maki Sakuma (since 04/2025)

PHD STUDENT
Wouter van Midden

MASTER STUDENT
Patrick Say (since 03/2025)

ASSOCIATED CLINICIAN - BIOINFORMATICIAN
Sebastian Eder

Langerhans cell histiocytosis (LCH) is a rare disorder affecting all ages, with the most severe forms occurring in young children. Clinical presentation ranges from self-limited single bone lesions to life-threatening multisystem disease requiring intensive treatment. Although its exact cause remains unclear, LCH is now recognized as a myeloid neoplasm driven by activation of the MAPK signaling pathway.

Our group seeks to understand the molecular basis of LCH as a means to develop new therapeutic options. We use advanced DNA sequencing, flow cytometry and imaging techniques in patient samples to dissect the cellular composition of LCH lesions and understand how different cell types interact. We also analyze the aberrant cells present in the patients' peripheral blood to understand how they contribute to the disperse nature of the disease and to identify new biomarkers that can support the identification of high-risk patients and the evaluation of treatment response in the clinical setting.

Our work also focuses on creating novel in vitro models of LCH and using them to study how LCH develops at the cellular level. Employing these models we perform high-throughput drug screenings to develop new treatment regimens. Our ultimate goal is to fundamentally change diagnosis of LCH and enable optimal treatment of affected children.

KAMENEVA GROUP

Pediatric Tumor Initiation

PRINCIPAL INVESTIGATOR
Polina Kameneva

TECHNICAL ASSISTANT
Anna-Katharina Mautner

PHD STUDENT
Thomas Eckhardt
Bence Fogel (since 09/2025)
Beate Kratschmann (since 03/2025)

Most pediatric cancers begin as small alterations in normal cells during embryonic development. Sporadic mutations and chromosome aberrations can derail cells from their normal developmental trajectory, getting them stuck in a proliferative state that leads to cancer. However, the occurrence of these genetic alterations doesn’t always lead to cancer—the timing and context of their appearance are equally important.

Our group studies how the specific developmental stage of a cell affects whether a genetic alteration will turn it cancerous. For this, we investigate how different genetic alterations impact development at various stages. We dissect their effects on different molecular pathways to see how they influence neuroblastoma development.

Our efforts focus on building novel 2D and 3D neuroblastoma models using human stem cells into which we can introduce mutations at different developmental timepoints. Using genetic engineering, single-cell multiomics and state-of-the-art bioinformatics, we aim to characterize these cells with unprecedented accuracy and study how different genetic alterations affect their development.

We also examine how inherited mutations that predispose children to cancer affect the development of different tissues within the body. Accordingly, we build advanced cellular models to study how these mutations derail the cell fate decisions from a normal path and how some cell types are unaffected by the mutational effects and can continue to develop normally. Our work aims to uncover the mechanisms that cooperate with mutations and lead to cancer initiation, and find a way to exploit them for new targeted therapies.

KOVAR GROUP

Molecular Biology of Solid Tumors

PRINCIPAL INVESTIGATOR
Heinrich Kovar

TECHNICAL ASSISTANT
Karin Mühlbacher

POSTDOCTORAL FELLOW
Utkarsh Kapoor

PHD STUDENT
Veveeyan Suresh
Martha Magdalena Zylka

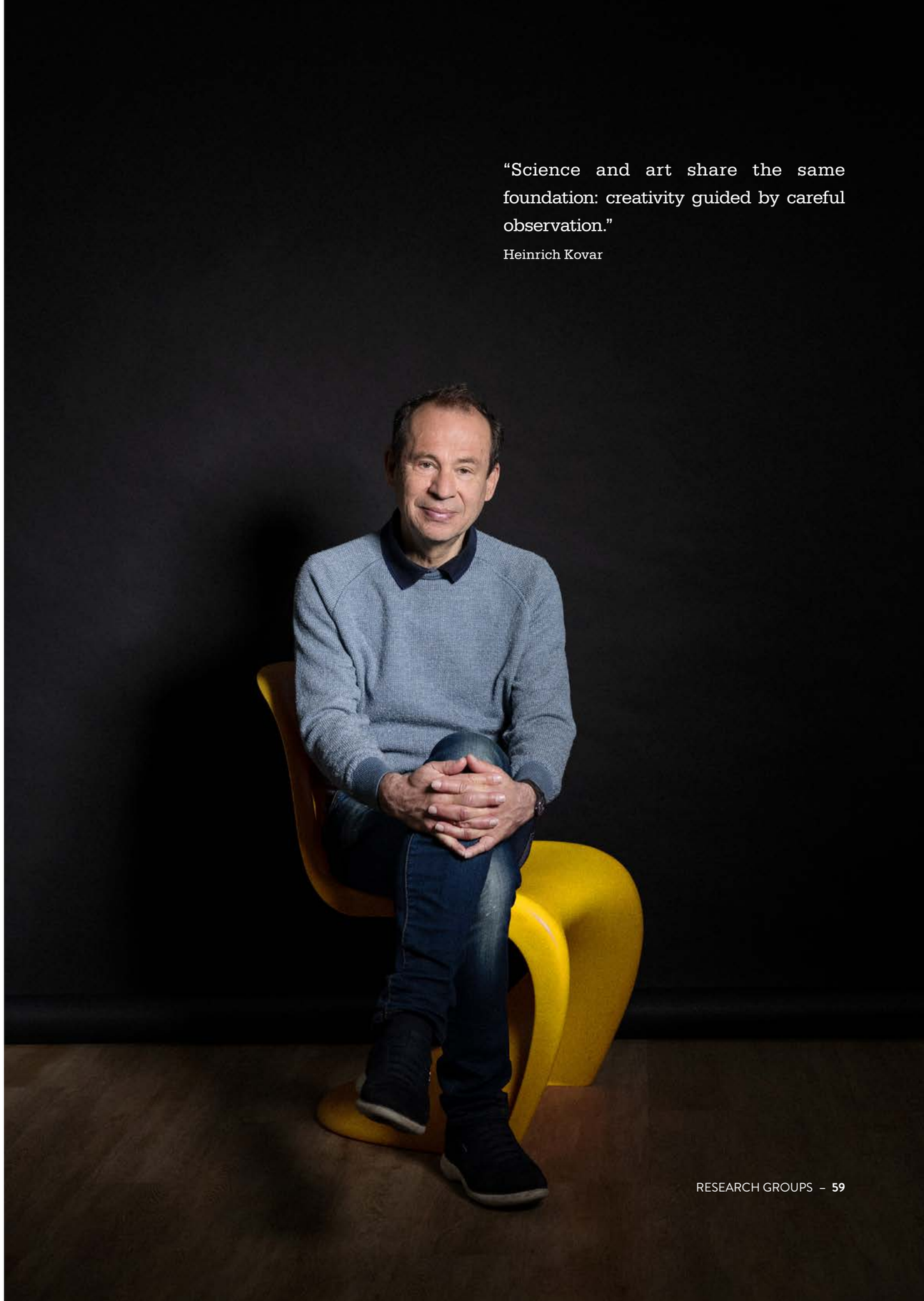
ASSOCIATED POSTDOCTORAL FELLOW
Branka Radic-Sarikas

VOLUNTEER
Sarah Grissenberger (until 03/2025, jointly with ZANDR)

Ewing sarcoma is an aggressive childhood cancer caused by a rearrangement of the genes *EWSR1* and *FLI1*. Despite this genetic simplicity, the molecular mechanisms leading to disease development and progression are still poorly understood.

Our group seeks to answer keyquestions with the goal of identifying new therapeutic targets to treat Ewing sarcoma. Our research focuses on dissecting the molecular pathways driven by the *EWSR1::FLI1* fusion to understand how this protein affects the normal functioning of developing cells, leading to cancer. We employ innovative in vitro and in vivo models recapitulating key stages of the disease. Using gene editing and genetic screenings, we probe *EWSR1::FLI1* functions on tumor phenotype and plasticity.

We also investigate how external factors—such as puberty-associated hormones and other endocrine factors—might trigger Ewing sarcoma development in mutant cells. In addition, we examine how fluctuations in *EWSR1::FLI1* levels influence tumor progression. We furthermore developed an innovative organoid model to study individual steps of bone sarcoma metastasis in vitro. Overall, understanding the mechanisms of disease progression can help us design better treatments and improve outcomes for pediatric cancer patients.



"Research is improvisation in motion, until curiosity, inspiration, and dedication compose a symphony of meaning."

Thomas Lion

LION GROUP

Molecular Microbiology and Leukemia Research

PRINCIPAL INVESTIGATOR
Thomas Lion

TECHNICAL ASSISTANT
Chantal Lucini

VOLUNTEER POSTDOCTORAL SCIENTIST
Anna Zvereva (until 06/2025)

Oncological patients undergoing stem cell transplantation or chemotherapy face a high risk of life-threatening infections caused by viruses, bacteria or fungi. Detecting such infections early is essential for their management and for prevention of dangerous complications.

The group develops sensitive molecular diagnostic tools for pathogenic viruses and fungi that can affect pediatric cancer patients undergoing treatment. These findings help doctors improve treatment strategies against these life-threatening infections in severely immunocompromised patients. Our efforts have also expanded to developing tools to facilitate early diagnosis of graft rejection and other complications of cancer treatment, helping doctors intervene in time and ensuring therapeutic success.

In addition, the work focuses on understanding how bacteria and fungi interact with each other to produce complex infections in immunocompromised patients. A better undrestanding of these interactions can support more informed treatment decisions in patients with complex infections.

Overall, our research is devoted to increasing our understanding of infections that commonly occur in pediatric patients undergoing cancer therapy—with the aim of better diagnosing, treating and managing these infections to ensure optimal treatment.

An additional line of our research addresses resistance mechanisms commonly occurring in patients with specific types of leukemia. We develop tools to detect even complex genetic changes underlying drug resistance and identify effective treatment strategies to improve clinical outcome in drug-resistant patients.

SERUGGIA GROUP

Pediatric Leukemia Biology

PRINCIPAL INVESTIGATOR
Davide Seruggia

TECHNICAL ASSISTANT
Gerlinde Karbon
Sophie Müller

POSTDOCTORAL FELLOW
Paul Daniel Batty
Ana Patricia Kutschat

PHD STUDENT
Hannah Beneder
Freya Jungen (since 09/2025)
Emilia Korczmar
Leonie Lehmayer
Sandra Wittibschlager

MASTER STUDENT
Carlos Roche Arcas (from 02-09/2025)

CEMM PHD ROTATION STUDENT
Patrik Hains (from 11-12/2025)

Leukemias represent roughly 30% of all pediatric cancers, with some forms still having very poor prognosis. Inherited defects in hematopoiesis—the complex developmental process by which all blood cell types are produced—favor the emergence of leukemia. To produce different cell types, different gene sets must be activated or repressed in a controlled manner.

Pediatric leukemia often arises from chromosomal aberrations at genes critical to hematopoiesis, while alterations of their regulatory sequences can predispose to the disease. How specific regulatory sequences contribute to cancer predisposition, however, is poorly understood. Our group studies the way in which these regulatory sequences influence gene expression during hematopoiesis and how their variation can ultimately contribute to leukemia development. Combining advanced CRISPR screening, chromatin profiling and computational approaches, we dissect how non-coding, regulatory sequences such as enhancers contribute to normal hematopoiesis and disease.

This work is complemented by creating innovative cellular models of pediatric leukemia that can accurately recapitulate the development of this disease and serve as a tool for researchers aiming to better understand how pediatric leukemia arises.

Our research aims to expand our understanding of gene regulation during hematopoiesis and uncover how single nucleotide polymorphisms at non-coding regulatory sequences can substantially increase the risk of developing leukemia. These findings might inform new diagnostic and therapeutic approaches to improve the treatment of pediatric leukemias.

"A steady rhythm, a well-coordinated team of professionals—and occasionally the performance of a solo player—are the ingredients of good music and good science."

Davide Seruggia

STREHL GROUP

Genetics of Leukemias

PRINCIPAL INVESTIGATOR
Sabine Strehl

STAFF SCIENTIST
Dagmar Schinnerl (jointly with Dworzak Group)

POSTDOCTORAL FELLOW
Klaus Fortschegger

Leukemia is the most common cancer in children and adolescents. It arises from genetic alterations—such as fusion gene-forming chromosomal rearrangements or mutations—that disrupt hematopoiesis, i.e., the formation of blood cells. Identifying and understanding these genetic lesions allows leukemia to be classified into subtypes with distinct biological and clinical features, which is crucial to optimize treatment and develop targeted therapies.

Our research focuses on evaluating the prognostic impact of leukemia-associated genetic alterations and their combinations to predict treatment outcome. The gained knowledge is intended to establish their value as predictive markers to refine risk stratification and to provide personalized treatment. In close collaboration with oncologists at the St. Anna Children's Hospital and leveraging global networks, we pay particular attention to rare B cell acute lymphoblastic leukemia subtypes, which are still poorly understood in terms of their biological properties and clinical behavior.

Concurrently, we model the early steps of leukemia development in vitro by recapitulating the disease in laboratory dishes, using genetically engineered pluripotent stem cells, which are differentiated into early blood-forming cells. These well-controlled models recreate normal and cancer-promoting blood cell development, enabling the precise dissection of how individual genetic changes such as fusion genes disrupt hematopoiesis and drive malignant transformation.

Ultimately, these complementary approaches are expected to refine personalized treatment strategies and to pinpoint novel therapeutic targets for childhood leukemia.

TASCHNER-MANDL GROUP

Tumor Biology

PRINCIPAL INVESTIGATOR
Sabine Taschner-Mandl

SENIOR STAFF SCIENTIST & TEAM LEAD DIAGNOSTICS
Marie Bernkopf

STAFF SCIENTIST
Eva Bozsaky

COMPUTATIONAL SCIENTIST
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TECHNICAL ASSISTANT
Giada Disconzi

POSTDOCTORAL FELLOW
Snezana Hinic (since 03/2025)

PHD STUDENT
Simon Gutwein
Viktoria Humhal
Tatiana Pashkovskaia (from 02-07/2025)
Magdalena Rados
Eszter Söjtöry (since 09/2025)
Julia Zehenter

SOFTWARE DEVELOPER
Matthias Kellner (until 06/2025)

INTERN
Nuria Martinez-Alarcon (from 06-08/2025)

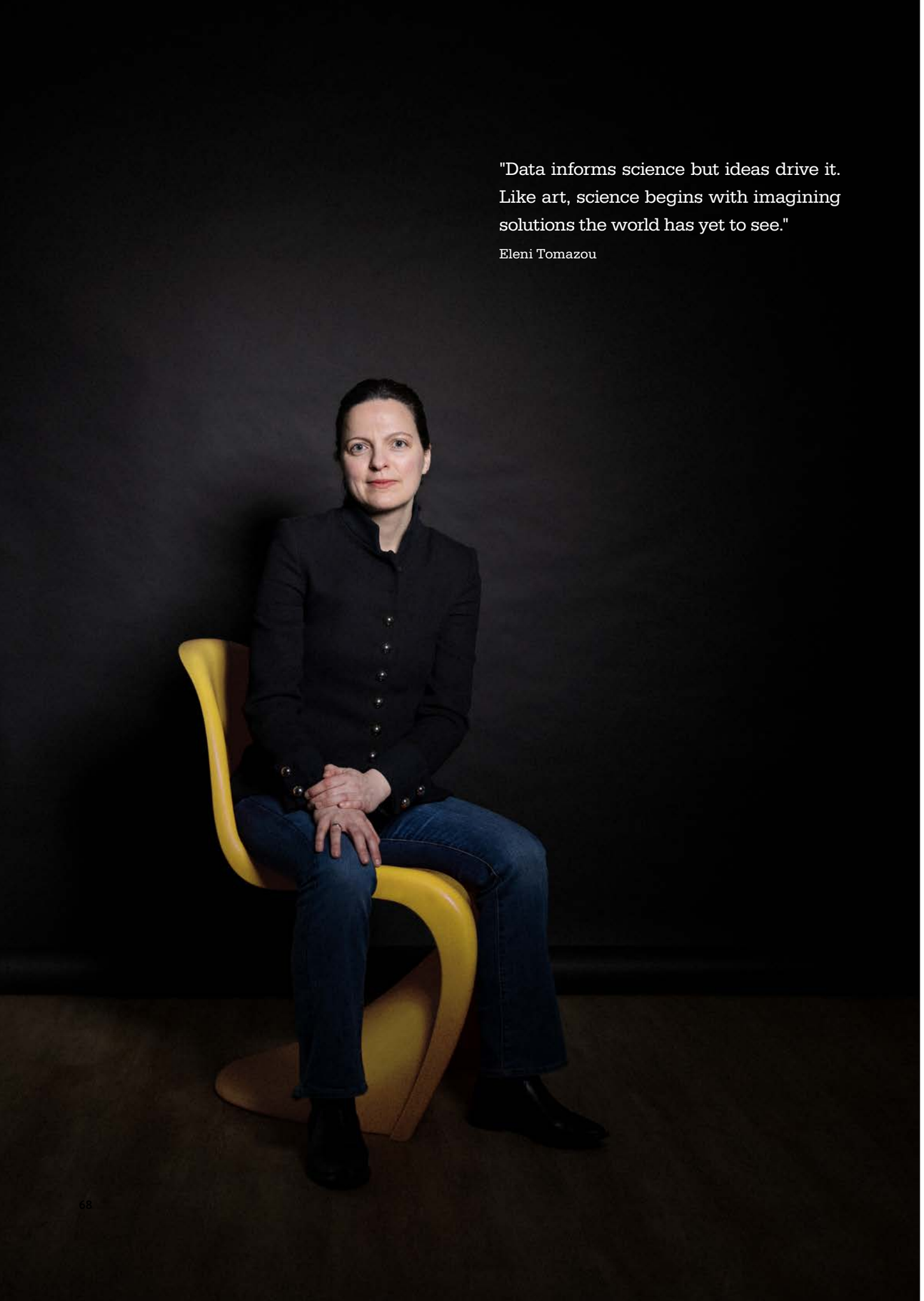
Neuroblastoma is one of the most common—and deadliest—childhood cancers. For high-risk patients, current treatments often fall short, with relapse and metastasis keeping survival rates below 40%.

My group studies the molecular and biological mechanisms of tumor development and progression which are driven by genetic alterations, cell plasticity and tumor—microenvironment interactions. Using cutting-edge tools like single-cell and spatial genomics, epigenomics and tissue imaging together with human stem cell-based models, we investigate how (epi-)genetic alterations drive the disease, how different cell types within a tumor contribute to metastasis and how these molecular interactions can be leveraged to develop more effective treatment approaches.

In addition, our work focuses on creating faster and more precise diagnostic tools. In recent years, we have pioneered the use of liquid biopsy—a less invasive and more sensitive method to monitor for relapses—in pediatric cancer patients. We also co-developed a new combination method that uncovers hidden tumor cells in the bone marrow of high-risk patients. Most recently, we have built an AI-based tool that scans images in seconds, flags suspicious cells and makes diagnostics faster and more precise. Together, these technologies help oncologists diagnose cancer and relapse more accurately and ensure patients receive the right treatment at the right time.

“Creativity is essential for progress. Science requires both structure and openness—the ability to recognize patterns, but also to think beyond the established path.”

Sabine Taschner-Mandl



TOMAZOU GROUP

Epigenome-Based Precision Medicine

PRINCIPAL INVESTIGATOR
Eleni Tomazou

STAFF SCIENTIST
Jason Sims

TECHNICAL ASSISTANT
Aikaterini Maria Fourmouzi (until 05/2025)
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Ornella Urzi

PHD STUDENT
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Matteo Colombo
Peter Peneder (until 03/2025)
Manon Ressaire
Tamina Stelzer (since 02/2025)

MASTER STUDENT
Claudia Pilloni (since 03/2025)

CEMM PHD STUDENT
Daria Romanovskaia (until 12/2025)

FREELANCER
Peter Peneder (from 03-10/2025)

Fusion oncogene-driven sarcomas are aggressive cancers of bone and soft tissue primarily affecting children, adolescents and young adults. Unlike most cancers, they arise from profound transcriptional and post-transcriptional dysregulation rather than from extensive mutational burden. An exemplification of this is Ewing sarcoma, initiated by the pathognomonic *EWSR1::FLI1* fusion oncogene, which disrupts gene regulatory networks and highlights the central role of epigenetic transcriptional/post-transcriptional mechanisms in tumor development.

Our group investigates pediatric sarcomas as developmental cancers driven by transcriptional and post-transcriptional dysregulation. Positioned at the interface of basic biology and translational research, we aim to enable molecularly informed diagnosis and therapy.

Our research program follows three complementary directions. First, we develop human pluripotent stem cell-based models to recapitulate early tumorigenic events, creating versatile systems for mechanistic studies and preclinical drug discovery. Second, we interrogate non-genetic deregulation and cellular heterogeneity in fusion-driven sarcomas, using single-cell epigenomics, functional assays of regulatory elements and analyses of nucleolar biology, ribosome biogenesis and translational control. Third, we develop minimally invasive liquid biopsy approaches, combining next-generation sequencing and machine learning to detect low-abundance tumor signals, classify disease and monitor treatment response.

Together, these efforts seek to uncover shared regulatory principles across sarcoma types and advance precision medicine strategies, ultimately improving outcomes for children and adolescents with these aggressive cancers.

PUBLICATION HIGHLIGHTS

TRACK "HORIZON"

HOW NATURAL KILLER CELLS BOTH FIGHT AND SHAPE LEUKEMIA

IN SIMPLE TERMS:

Cancer and the immune system are locked in a constant battle. Immune cells destroy emerging cancer cells, but if tumor cells survive, a tense standoff follows: The immune system holds the cancer in check while placing it under pressure to change—over time, some cancer cells evolve to escape detection and grow. Scientists at St. Anna CCRI show that natural killer cells are part of both sides: They kill vulnerable cells, but in doing so clear the way for harder-to-kill cells to take over. The findings could guide future immunotherapies that boost natural killer cells and prevent tumors from learning how to hide.

Tumors develop in a dynamic interaction between cancer cells and the immune system—a relationship often described in three phases of cancer immunoediting: elimination, equilibrium, and escape. During elimination, immune cells recognize and kill malignant cells. In equilibrium, tumor cells that survive remain under constant immune pressure. Over time, this selective pressure can drive immune escape, allowing cancer to evade detection and eventually continue growing.

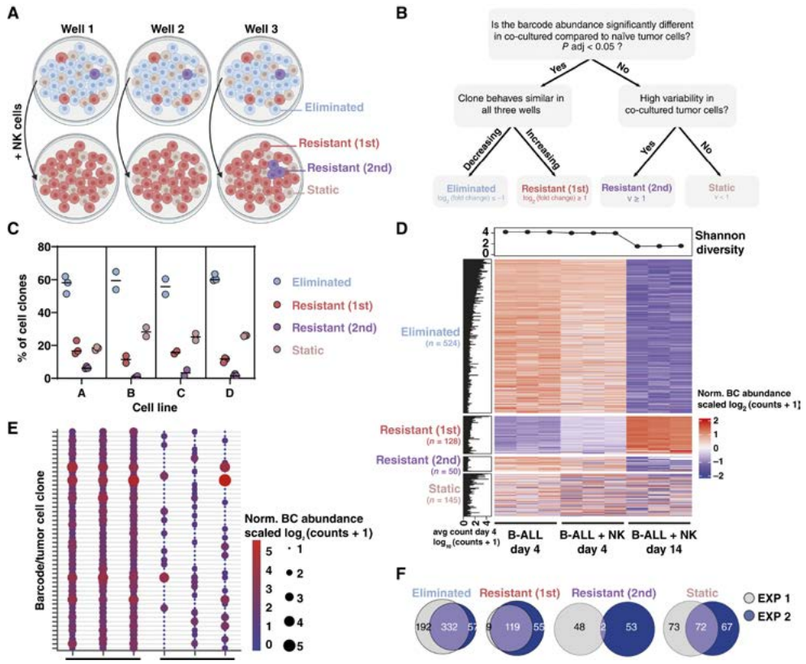
A collaborative project led by PhD student Michelle Buri, who works with former St. Anna CCRI PI Eva König (nee Putz), together with colleagues from the Christian Doppler Laboratory for Next Generation CAR T Cells and the Halbritter group, investigated how natural killer (NK) cells influence this process in B-cell acute lymphoblastic leukemia (B-ALL). NK cells are a type of immune cell that kills cancer cells while sparing healthy tissue, making them attractive candidates for new immunotherapy strategies. However, as the researchers show, the role of NK cells in leukemia is more complex.

Using a co-culture model and cellular DNA barcoding to follow individual leukemia clones over time, the researchers observed how NK cells pruned away cells susceptible to immune attack, thereby creating conditions in which resistant cells could dominate. Two groups of cancer cells emerged as winners: Some leukemia cells already possessed primary resistance and could withstand the attack by NK cells from the outset. Others survived more by "luck of the draw": These initially vulnerable cells acquired secondary resistance during the struggle, suggesting that immune pressure itself can drive new escape routes to emerge.

These findings place NK cells in all phases of cancer immunoediting: as early defenders, as shapers of tumor evolution, and ultimately as contributors to immune escape when cancer eradication is incomplete. This deeper understanding of how leukemia adapts under immune pressure opens the way to new treatment strategies for B-ALL that harness the cancer-killing power of NK cells more effectively, while limiting the routes cancer uses to hide.

PUBLICATION

Buri, M. C., Shoeb, M. R., Bykov, A., Repiscak, P., Baik, H., Dupanovic, A., David, F. O., Kovacic, B., Hall-Glenn, F., Dopa, S., Urbanus, J., Sippl, L., Stofner, S., Emminger, D., Cosgrove, J., Schinnerl, D., Poetsch, A. R., Lehner, M., Koenig, X., Perié, L., … Putz, E. M. (2025). Natural Killer Cell-Mediated Cytotoxicity Shapes the Clonal Evolution of B-cell Leukemia. *Cancer Immunology Research, 13* (3), 430–446. https://doi.org/10.1158/2326-6066.CIR-24-0189



Quantification of NK cell-mediated cancer immunoediting in vitro. (A) Schematic representation of the proposed model. (B) Upon long-term co-culture of B-ALL and NK cells, we hypothesized that each tumor cell clone would fall into one of the following categories: The abundance of a clone can be significantly higher (primary resistant) or lower (eliminated), unchanged (static) or show a high variability (secondary resistant) upon NK cell co-culture. (C) The percentage of the cell clones in each group on day 14 is depicted for cell lines A, B, C and D. (D) The heatmap shows the normalized abundance of barcodes (norm. BC abundance) of cell line A from one representative experiment. (E) Normalized abundance of secondary resistant clones in B-ALL samples (day 4) compared to B-ALL + NK samples (day 14). The x-axis shows the 3 individual wells of each condition, while each row on the y-axis shows an individual tumor cell clone. The size and color of the bubbles indicate the normalized barcode abundance. (F) Comparing 2 independent experiments in cell line A as shown in (D & E), the Euler diagrams highlight a high overlap of eliminated, primary resistant, and static cell clones. In contrast, only 2 secondary resistant clones were shared between both independent experiments.

VERY RARE SUBTYPE OF CHILDHOOD LEUKEMIA CHARACTERIZED

IN SIMPLE TERMS:

An international study led by the group of Sabine Strehl has shed light on a very rare subtype of childhood B-cell acute lymphoblastic leukemia caused by a fusion of the *PAX5* and *AUTS2* genes. By analyzing 50 cases collected from several countries worldwide, the team found that children with this leukemia subtype face a much higher risk of relapse and lower long-term survival than children with more typical forms of the disease. The findings suggest that current standard therapies are often inappropriate for those patients and that alternative treatment options need to be explored.

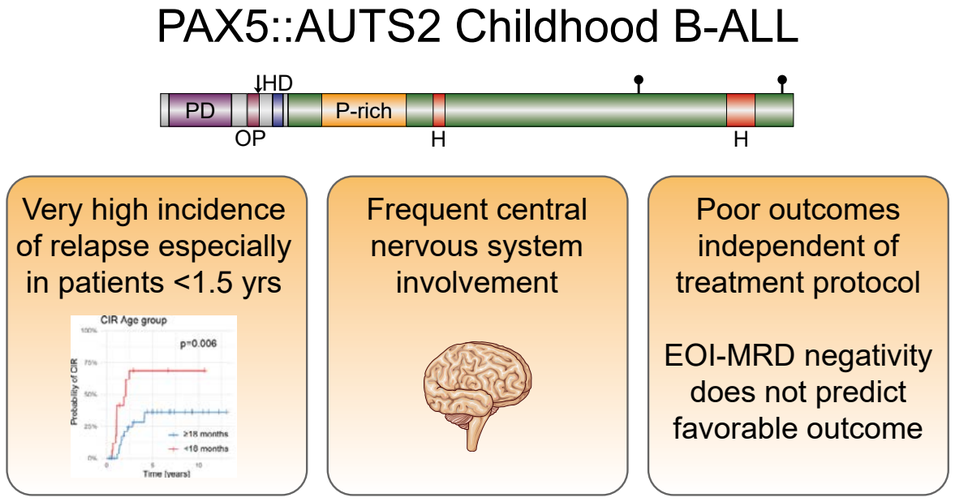
Treating children and adolescents with B-cell acute lymphoblastic leukemia (B-ALL), the most common cancer in this age group, remains a success story of modern medicine, with cure rates approaching 90%. However, about 10–15% of patients still experience a relapse, which dramatically worsens their prognosis. Pinpointing high-risk genetic subtypes is key for improving treatment strategies.

The team of Sabine Strehl spearheaded an international effort to characterize an exceptionally rare subtype of childhood B-ALL. The study focused on 50 children diagnosed with a leukemia subtype defined by a *PAX5::AUTS2* gene fusion. Because this form of B-ALL is so rare, assembling even this modest number of cases required partnership across ten countries, including France, Italy, Poland, Netherlands, Germany, United Kingdom, Czech Republic, Brazil, India, and Uruguay.

Analysis of clinical outcomes revealed that children with *PAX5::AUTS2* B-ALL have a strikingly high relapse rate—around 48% within five years—and an overall survival rate of less than 80%. Two factors predicted especially poor outcomes: diagnosis of leukemia before 18 months of age and detectable residual disease after initial therapy.

Importantly, even intensive modern frontline protocols failed to improve relapse rates, highlighting that current therapies are inappropriate for this high-risk group of patients. This study emphasizes the need to further explore the biology and drug sensitivity of *PAX5::AUTS2*-driven B-ALL to find alternative treatment options to mitigate the risk of relapse and improve survival.

This collaborative effort also underscores the value of global data sharing and represents a crucial step in ensuring that even the rarest leukemia subtypes are recognized and appropriately treated so as to ultimately increase cure rates for all children affected by B-ALL.



Graphical abstract. B-cell acute lymphoblastic leukemia (B-ALL) driven by the *PAX5::AUTS2* fusion presents with specific symptoms and a poor outcome.

PUBLICATION

Caye-Eude, A., Fazio, G., Pastorczak, A., Boer, J. M., Steinemann, D., Ganguli, D., Sonneveld, E., Haslinger, S., D'Andrea, L., Bradtke, J., Lopes, B. A., Zaliova, M., Escherich, G., König, M., Fortschegger, K., Inthal, A., Stasevich, I., Emerenciano, M., Trka, J., Castillo, L., Parihar, M., Moorman, A.V., Bergmann, A. K., den Boer, M. L., Młynarski, W., Cazzaniga, G., Cavé, H., Nebral, K., Schinnerl, D., Strehl, S. (2025). PAX5::AUTS2 childhood B-ALL: a relapse-prone genetic subtype with frequent central nervous system involvement and a poor outcome. *Leukemia, 39* (2), 482–486. https://doi.org/10.1038/s41375-024-02502-5

ZHORSE: A NEW ZEBRAFISH STRAIN FOR CONTROLLING GENE EXPRESSION WITH LIGHT

IN SIMPLE TERMS:

For genes to be active at the right time and in the right place, they must be tightly regulated. If this control is disrupted, cancer may arise. zHORSE, developed by the Distel and Kovar groups, is a new zebrafish strain allowing gene expression to be activated inside the living organism. Using light, the researchers can switch genes on precisely in the fish, even within just a single cell. zHORSE allows cancer-driving genes to be activated in individual cells, while neighboring cells remain unchanged. By employing this new model, researchers can investigate how cancer begins and how cells with active cancer-driving genes interact with their healthy neighbors.

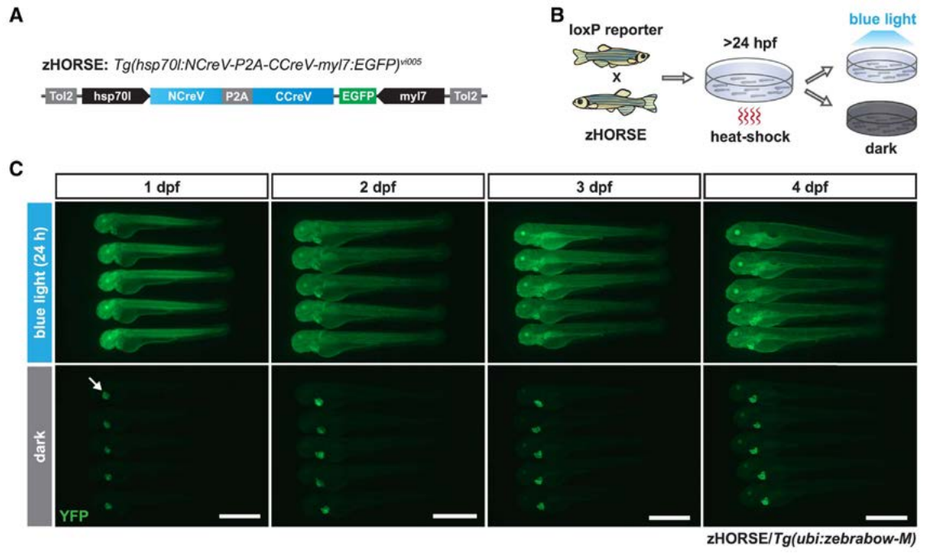
Gene expression is precisely controlled in space and time. When this delicate regulation is disrupted, diseases such as cancer may develop. To understand this complex control, gene activity needs to be precisely manipulated: By switching on gene activity in individual cells, researchers can interrogate the very first events of cells turning cancerous within a surrounding healthy tissue.

The Distel and Kovar groups developed a new zebrafish strain, zHORSE, in which genes can be precisely controlled both in space and time in a two-step activation process combining heat shock and optogenetic control. Optogenetics uses light to switch genes on in specific cells and tissues and thanks to its non-invasiveness can be applied to living organisms.

In the new zHORSE model, heat shock temporarily activates the system's sensitivity, which ensures that cells only respond to light during a specific window, rather than accidentally to ambient light. Once heat-shocked, targeted blue light—such as a laser from a confocal microscope—can trigger gene expression strictly in the illuminated tissue or single cells.

Because zHORSE can be combined with existing zebrafish strains carrying specific target genes, it is a powerful tool for cancer clone modelling. Researchers can use zHORSE to activate cancer-driving oncogenes in just a single cell or in a small cluster of cells. This closely mimics how cancer-driving mutations arise naturally within a healthy tissue environment, leading to the development of the disease.

By targeting the expression of the oncogene *NRASG12D* to individual skin cells, the researchers could observe the very first cellular changes associated with tumorigenesis and cellular senescence. Thus, zHORSE provides a window into understanding how cancer starts.



Schematic of zHORSE transgenesis and light-inducible protein expression. The gene of interest is placed under control of the heat shock promoter hsp70l. Exposure to blue light activates gene expression. The produced proteins are marked with green fluorescence.

PUBLICATION

Varady, A., Grissenberger, S., Fischer, K., Stadler, M. T., Wenninger-Weinzierl, A., Strobl, M., Zila, N., Kovar, H., & Distel, M. (2025). zHORSE as an optogenetic zebrafish strain for precise spatiotemporal control over gene expression during development. *Developmental Cell*, *60* (20), 2825–2839.e4. https://doi.org/10.1016/j.devcel.2025.06.005

AMKL: HOW AN AGGRESSIVE CHILDHOOD LEUKEMIA TAKES HOLD

IN SIMPLE TERMS:

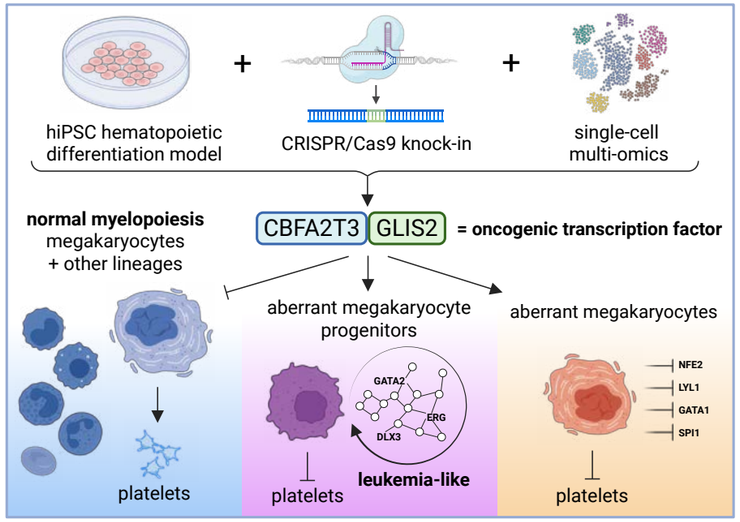
Acute Megakaryoblastic Leukemia (AMKL) is a rare and aggressive form of childhood leukemia with poor outcomes, yet scientists still know little about how it first arises. Researchers from the Strehl and Halbritter groups used stem cells to recreate the earliest stages of the disease, which are impossible to study in patients. They found that a single genetic change pushes developing blood cells down two harmful paths: Some become trapped in a fast-growing, leukemia-like state, while others begin to mature but get stuck halfway. The results reveal a new therapeutic target for AMKL in that they suggest that targeting both states may help effectively treat this aggressive pediatric cancer.

In some patients, aggressive pediatric acute megakaryoblastic leukemia (AMKL), a high-risk malignancy with poor survival rates, is driven by the CBFA2T3::GLIS2 fusion protein. After genetically modifying human induced pluripotent stem cells to express the CG protein, the Halbritter and Strehl groups were able to track the earliest pre-leukemic stages of the disease.

The researchers discovered that the CBFA2T3::GLIS2 fusion protein acts as a rogue transcription factor, rewiring the gene-regulatory landscape of developing blood cells. Rather than generating one uniform leukemic population, it pushes cells into two abnormal fates: One population, aberrant megakaryocyte progenitors, remains locked in a highly proliferative, self-renewing state that closely resembles the leukemia cells seen in patients. A second population, aberrant megakaryocytes, moves partway along the normal maturation pathway but fails to complete differentiation and produce platelets.

These findings deepen our understanding of AMKL. They show that this aggressive pediatric cancer arises as a complex disease made up of distinct abnormal cell states, which helps explain why AMKL is so difficult to treat: Different leukemic populations may not respond equally to therapy. By capturing the earliest stages of transformation, the study also clarifies how the fusion protein drives disease—by sustaining self-renewal programs while shutting down the genetic pathways required for normal blood-cell development.

This has important consequences for treatment strategies. Effective therapies may need to go beyond targeting rapidly dividing cells alone and may also need to address abnormal cell populations that appear more differentiated but still contribute to the disease. The study further shows that leukemic cells remain strongly dependent on the CBFA2T3::GLIS2 fusion protein for survival, underscoring it as a key therapeutic target. The stem-cell model established here provides a powerful platform for testing new drugs and developing treatment approaches aimed at more thoroughly eradicating the disease.



Project workflow. Human induced pluripotent stem cells were used as a model for hematopoietic differentiation. Using CRISPR/Cas9, the CBFA2T3::GLIS2 gene fusion was introduced in these cells. Their development was tracked in detail using single-cell multi-omics. The study showed that the CBFA2T3::GLIS2 fusion protein dysregulates normal megakaryocite development and produces aberrant megakaryocites and progenitor cells.

PUBLICATION

Shoeb, M. R., Schinnerl, D., Shaw, L. E., Farlik, M., Strehl, S., Halbritter, F., & Fortschegger, K. (2025). A stem cell differentiation model reveals two alternative fates in CBFA2T3::GLIS2-driven acute megakaryoblastic leukemia initiation. *Communications Biology, 8* (1), 1289. https://doi.org/10.1038/s42003-025-08730-4

BETTER THERAPY SELECTION FOR CHILDHOOD LEUKEMIA

IN SIMPLE TERMS:

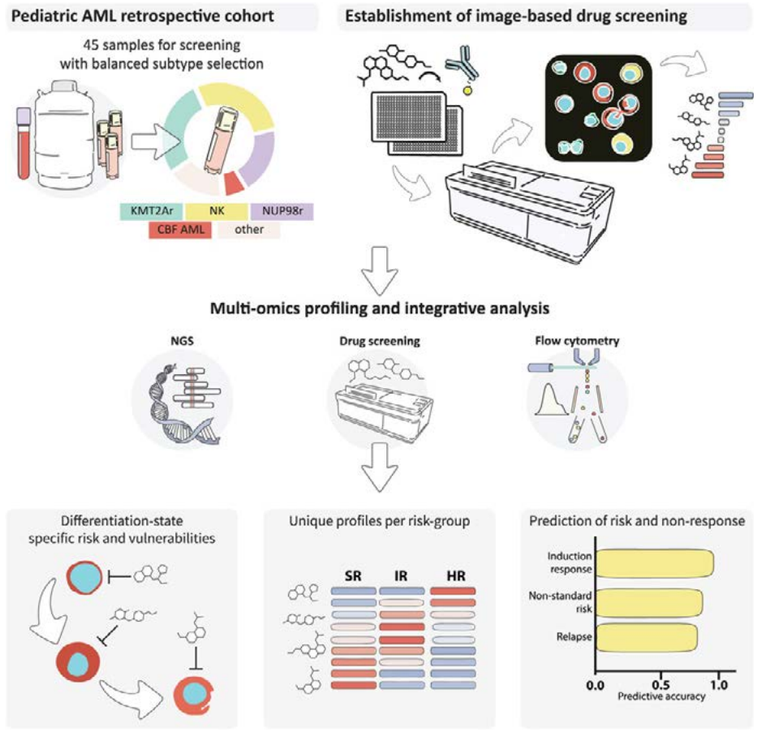
The Boztug and Dworzak groups, together with collaborators, developed a new method to predict how children with acute myeloid leukemia will respond to treatment. Using high-content imaging, molecular profiling, and artificial intelligence, the team found that resistant leukemia cells can be identified already at diagnosis. This will allow doctors to select the most effective therapies earlier and tailor treatment for each patient, potentially improving outcomes for high-risk children whose leukemia does not respond to standard chemotherapy.

Pediatric acute myeloid leukemia (AML) is one of the most aggressive childhood cancers. It arises when immature blood precursor cells in the bone marrow acquire genetic changes that block normal maturation, causing uncontrolled growth of defective cells. This leads to anemia, infections, bleeding, and organ problems. While survival rates have improved thanks to advances in treatment, many children still respond poorly or relapse.

The Boztug and Dworzak groups, in collaboration with Giulio Superti-Furga from the CeMM Research Center for Molecular Medicine, developed a platform combining pharmacoscopy (a high-content imaging method), artificial intelligence, and molecular profiling to detect therapy resistance at diagnosis. Analyzing 45 patient samples, the team generated detailed “chemosensitivity profiles” that show which leukemia cells are resistant or vulnerable to specific drugs.

The study revealed a stem-cell-like leukemia subpopulation insensitive to standard chemotherapy but responsive to combinations of existing drugs such as BCL2 or MDM2 inhibitors. By linking molecular data to functional drug response, clinicians can now identify high-risk patients early and select more effective, individualized therapies.

This functional precision medicine approach complements genetic testing and minimal residual disease monitoring. Providing a direct, practical view of how leukemia cells react to treatment, it offers a new path toward personalized therapy for pediatric AML. As next steps, the method will be applied in real time in prospective clinical studies, and therapy selection will undergo further refinement, potentially improving cure rates for children with high-risk AML.



Workflow of the study. Fresh-frozen samples from 45 patients taken at diagnosis were profiled with flow cytometry and subjected to image-based drug screening and comprehensive next generation sequencing (NGS) characterization.

PUBLICATION

Haladik, B., Maurer-Granofszky, M., Zoescher, P., Jimenez-Heredia, R., Frohne, A., Segarra-Roca, A., Casey, C., Kartnig, F., Giuliani, S., Rashkova, C., Repiscak, P., Dworzak, M. N., Superti-Furga, G., & Boztug, K. (2025). Image-based drug screening combined with molecular profiling identifies signatures and drivers of therapy resistance in pediatric AML. *Cell Reports Medicine, 6* (9), 102304. https://doi.org/10.1016/j.xcrm.2025.102304

IGF-1 AS THE "IGNITION KEY" FOR BONE CANCER: UNRAVELING THE ORIGINS OF EWING SARCOMA

IN SIMPLE TERMS:

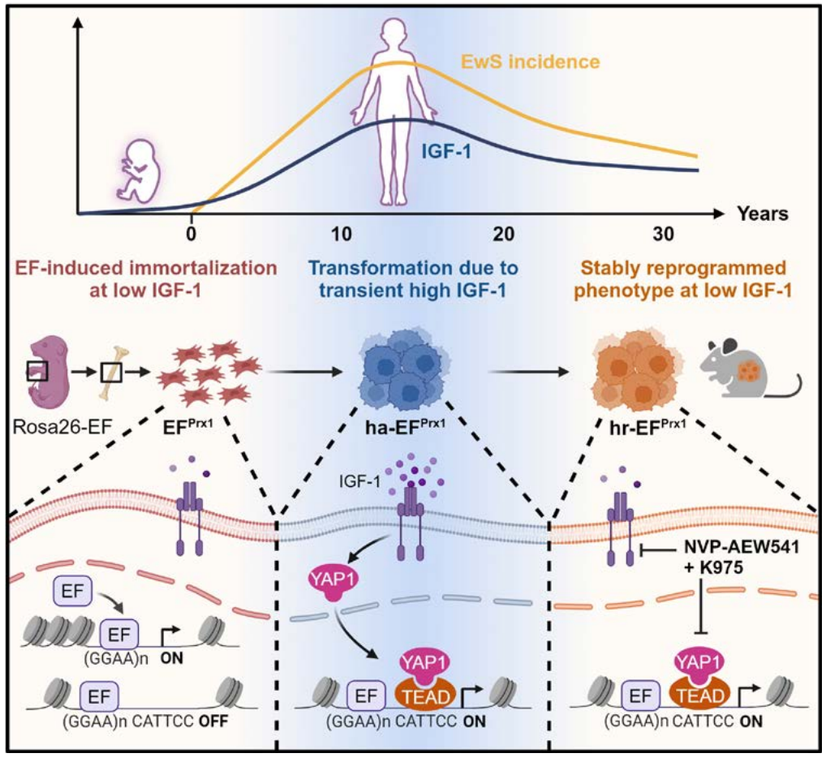
The Kovar and Halbritter groups put forward an explanation of how Ewing sarcoma, a rare and aggressive bone cancer, might be triggered during adolescence. The team found that the disease requires both the cancer-driving *EWS::FLI1* fusion gene and high levels of the growth hormone IGF-1, which surges during puberty. IGF-1 activates a protein called YAP1 that turns on genes allowing tumor cells to grow. Laboratory models showed that blocking both IGF-1 signals and YAP1 activity could reduce tumor growth, thereby pointing toward potential new combination therapies for patients.

Ewing sarcoma is one of the most aggressive bone cancers in adolescents. While the *EWS::FLI1* fusion gene is a hallmark of the disease, it alone is not sufficient to trigger tumor formation. The Kovar and Halbritter groups uncovered a two-step mechanism potentially explaining why the disease arises during puberty: In addition to the *EWS::FLI1* genetic fusion, elevated levels of IGF-1 (Insulin-like Growth Factor 1) activate the protein YAP1, which is essential to switch on genes promoting cancer development.

Using preclinical mouse models, the researchers demonstrated that bone precursor cells carrying the *EWS::FLI1* gene alone did not form tumors. Instead, tumor formation only occurred when these cells were exposed to high IGF-1 levels to mimic the hormonal environment of adolescence. This finding identifies IGF-1 as an "ignition key" activating cells poised to cancer development.

The study also explored therapeutic implications. Laboratory experiments showed that blocking either IGF-1 receptor (IGF-1R) or YAP1 alone had limited effect, but dual inhibition significantly reduced tumor cell viability. This suggests a promising combination strategy for patients resistant to standard treatments.

By revealing a critical interaction between genetic and hormonal signals, the findings provide a deeper understanding of Ewing sarcoma's origins and a potential pathway toward more effective therapies. This insight lays the groundwork for translational research and identifies a highly specific, druggable vulnerability in this aggressive childhood cancer.



Graphical abstract. High IGF-1 levels during adolescence activate the key molecular regulator YAP1 and could be the trigger for malignant transformation in Ewing Sarcoma.

PUBLICATION

Noorizadeh, R., Sax, B., Javaheri, T., Radic-Sarikas, B., Fock, V., Suresh, V., Kauer, M., Bykov, A., Kurija, D., Schlederer, M., Kenner, L., Weber, G., Mikulits, W., Halbritter, F., Moriggl, R., & Kovar, H. (2025). YAP1 is a key regulator of EWS::FLI1-dependent malignant transformation upon IGF-1-mediated reprogramming of bone mesenchymal stem cells. *Cell Reports, 44* (3), 115381. https://doi.org/10.1016/j.celrep.2025.115381

STEM CELL MODEL PAVES THE WAY FOR NEW THERAPIES FOR LANGERHANS CELL HISTIOCYTOSIS

IN SIMPLE TERMS:

A new stem cell-based model allows scientists to better understand Langerhans Cell Histiocytosis (LCH), a rare disease that can affect many organs, including the brain. By engineering the disease-causing BRAFV600E mutation into human cells, the Hutter group showed how this mutation disrupts blood cell development, how it can lead to brain cell damage, and—importantly—how these changes can be reversed with certain drugs. This model may help guide future treatments for LCH.

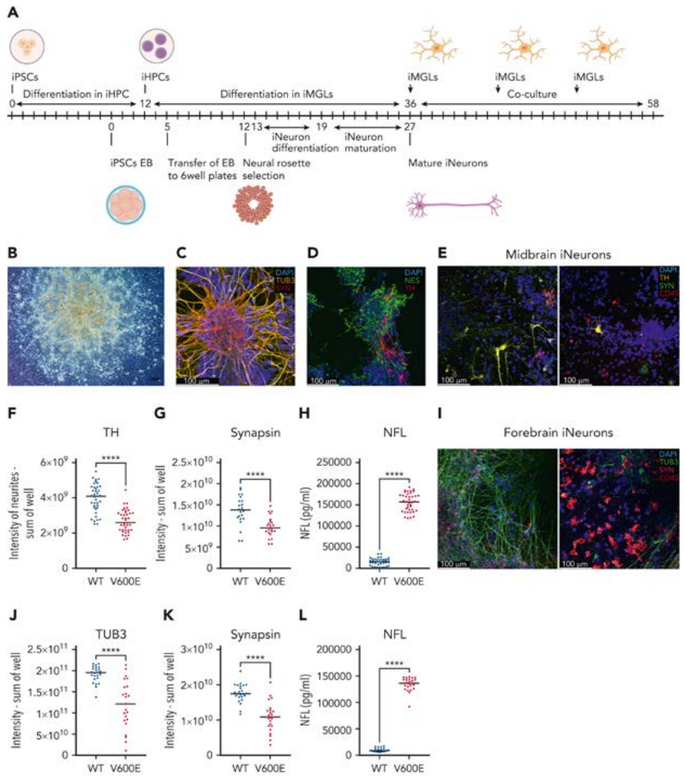
Langerhans Cell Histiocytosis (LCH) is a rare disorder of the blood-forming system that can range from mild, self-healing lesions to severe multi-organ damage and progressive neurodegeneration. A major obstacle in studying LCH has long been the lack of suitable laboratory models that accurately reflect the disease. Researchers in Caroline Hutter's group overcame that barrier by creating the first comprehensive in vitro model of LCH with the use of induced pluripotent stem cells (iPSCs).

The team introduced the BRAFV600E mutation—the most common genetic driver of LCH—into reprogrammed human cells. This mutation alters how genes are regulated during hematopoiesis, pushing early precursor cells toward abnormal developmental paths. As a result, the engineered cells behaved strikingly like the pathological cells found in LCH lesions, allowing researchers to observe disease mechanisms in unprecedented detail.

One of the study's most promising findings is that these harmful molecular changes can be reversed. When the mutant cells were treated with MAPK-pathway inhibitors, their transcriptional abnormalities normalized. This suggests that drugs targeting this pathway—already used in other conditions—could also benefit patients with LCH.

The model also enabled the team to explore why some patients develop neurodegeneration, the most debilitating LCH complication. By generating mutant microglia—immune cells of the brain—the researchers showed how the BRAFV600E-altered microglia damage neurons and release measurable neurodegenerative markers.

Together, these results create a powerful platform for studying LCH at the molecular and cellular levels, opening new avenues for therapeutic development. The Hutter group's stem cell-based system not only reduces the need for animal models but also provides a versatile tool that may accelerate the design of targeted, more effective treatments for patients.



BRAFV600E/WT iPSC-derived microglia (iMGLs) lead to neurodegeneration of iPSC-derived Neurons (iNeurons). (A) Schematic of differentiation of human patient-derived iPSCs into neuronal precursor cells and further forebrain iNeurons is shown, for midbrain iNeurons cells are kept 1 additional week in maturation medium. On day 36 of iMGL differentiation iMGLs were added to iNeurons on day 27 or day 34 of forebrain or midbrain iNeuron differentiation, respectively. (B) At day 8 of differentiation neural rosettes are visible. (C) Mature forebrain iNeurons on day 28 after embryoid body formation. (D) Mature midbrain iNeurons on day 35 after embryoid body formation. (E) Immunofluorescence staining of TH (yellow), synapsin (green), CD45 (red) and DAPI (blue): left panel, midbrain iNeurons with WT patient-derived iMGLs; right panel, with mutant patient iMGLs. (F) Quantification of TH expression in neurite segments of midbrain iNeurons. (G) Quantification of synapsin expression in midbrain iNeurons. (H) Quantification of released NFL in pg/mL from midbrain iNeurons cocultured with either WT or mutated iMGLs. (I) Immunofluorescence staining of coculture of human patient-derived iPSC forebrain iNeurons with iMGLs, synapsin (magenta), TUB3 (green), CD45 (red), and DAPI (blue); left panel, forebrain iNeurons with WT iMGLs; right panel, with mutant iMGLs. (J) Quantification of TUB3 and (K) synapsin expression as sum of intensity of a well in forebrain iNeurons. (L) Quantification of released NFL in pg/mL from forebrain iNeurons in coculture with BRAFV600E/WT or BRAFWT/WT iMGLs.

PUBLICATION

Abagnale, G., Schwentner, R., Ben Soussia-Weiss, P., van Midden, W., Sturtzel, C., Pötschger, U., Rados, M., Taschner-Mandl, S., Simonitsch-Klupp, I., Hafemeister, C., Halbritter, F., Distel, M., Eder, S. K., & Hutter, C. (2025). BRAFV600E induces key features of LCH in iPSCs with cell type-specific phenotypes and drug responses. *Blood, 145* (8), 850–865. https://doi.org/10.1182/blood.2024026066

NEUROBLASTOMA: NEW COMBINATION METHOD RELIABLY DETECTS HIDDEN TUMOR CELLS

IN SIMPLE TERMS:

The Taschner-Mandl group discovered a far more reliable way to find hidden neuroblastoma cells in the bone marrow—cells that often escape routine tests but later cause relapse. By combining three modern diagnostic methods, the team detected many more of these dangerous cells than standard procedures can. The study, which analyzed samples from children in Austria and the Netherlands, also showed that less extensive sampling at diagnosis may be enough so that the burden on pediatric patients can be reduced. The findings could help doctors detect relapse earlier and tailor treatment more precisely for each child.

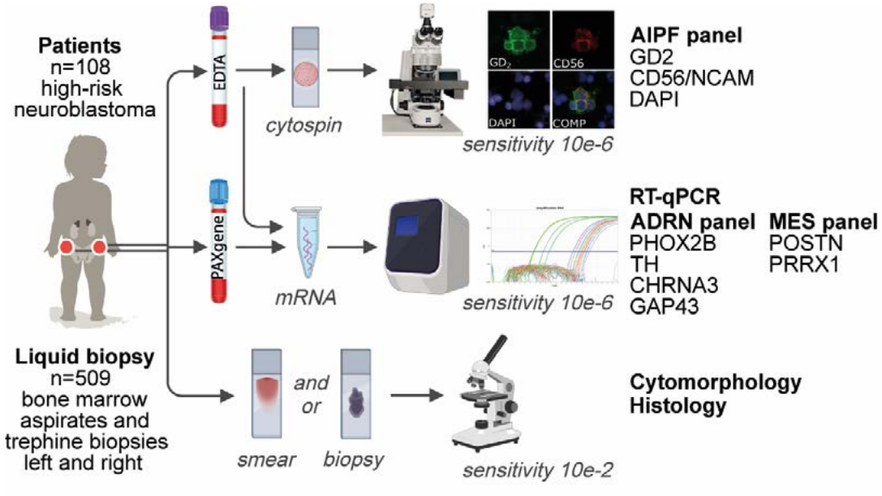
Neuroblastoma is the most common solid tumor outside the brain in children, and in high-risk cases more than 90% of patients have tumor cells in their bone marrow already at diagnosis—cells that are especially dangerous because they often remain undetected and can trigger relapse later, after treatment has been completed. Standard monitoring methods miss about 60% of bone marrow samples containing minimal residual disease, making improved diagnostics crucial.

The Taschner-Mandl group, together with Labdia and international partners at the Princess Máxima Center, analyzed 509 bone marrow samples from 108 high-risk neuroblastoma patients across Austria and the Netherlands. By combining three complementary technologies—automated immunofluorescence plus fluorescence in situ hybridization, PCR-based molecular analysis targeting adrenergic tumor cells, and conventional diagnostic tools—the researchers were able to detect substantially more hidden tumor cells than by using standard methods.

The automated immunofluorescence approach proved especially powerful. Not only did it detect extremely small numbers of tumor cells but also helped identify markers relevant for immunotherapies such as anti-GD2 treatment. This integrated method creates a clearer and more sensitive picture of the disease.

The study also revealed that while the bone marrow often tests positive at diagnosis on both body sides, during treatment and follow-up tumor cells may appear only on one side. This means single-sided sampling, which is significantly less invasive, could be sufficient at diagnosis, reducing the burden on children.

Importantly, the combined methods are feasible in clinical settings, work with very small sample volumes, and can be used across international centers. Detecting tumor changes and a possible relapse earlier will allow physicians to adjust therapy sooner and better understand treatment resistance, offering new hope for children with high-risk neuroblastoma.



Study design, outline of study cohort, and analytical workflows for bone marrow assessment by automated immunofluorescence plus fluorescence in situ hybridization (AIPF), RT-qPCR and cytomorphology/histology. Combined AIPF and RT-qPCR for neuroblastoma-specific adrenergic and mesenchymal mRNA biomarkers allow highly sensitive detection of minimal residual disease and immuno-therapy targets GD2 and NCAM (CD56).

PUBLICATION

Gelineau, N. U., Bozsaky, E., van Zogchel, L. M. J., Rifatbegovic, F., Lazic, D., Ziegler, A., Javadi, A., Zappeij-Kannegieter, L., Pötschger, U., Fiocco, M., Ambros, P. F., Ambros, I. M., Bodenmiller, B., van der Schoot, E. C., Ladenstein, R., Bernkopf, M., Tytgat, G. A. M., & Taschner-Mandl, S. (2025). Sensitive detection of minimal residual disease and immunotherapy targets by multi-modal bone marrow analysis in high-risk neuroblastoma—a multi-center study. *Journal of Experimental & Clinical Cancer Research, 44* (1), 224. https://doi.org/10.1186/s13046-025-03481-w

A LESS BURDENSOME IMMUNOTHERAPY FOR HIGH-RISK NEUROBLASTOMA

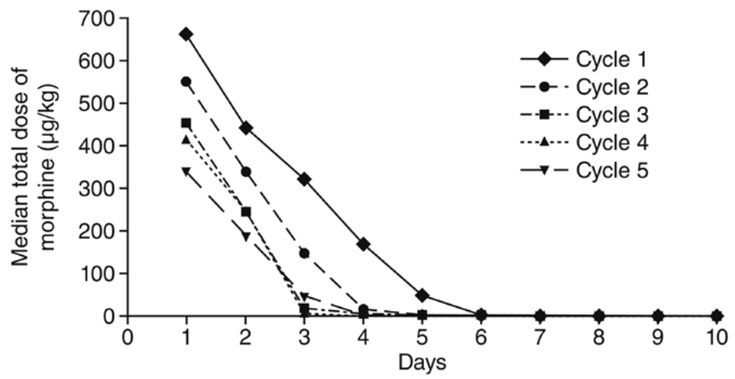
IN SIMPLE TERMS:

High-risk neuroblastoma remains one of the deadliest solid cancers in childhood. Immunotherapy can be lifesaving, but it often comes at the cost of causing severe pain. Ruth Ladenstein and collaborators presented results from a SIOPEN trial showing that administering the immunotherapy agent dinutuximab beta more slowly, as a 10-day infusion, can make treatment much easier to tolerate while preserving strong anti-tumor activity. Children receiving this treatment experienced less neuropathic pain and needed less morphine, and their treatment outcomes remained encouraging. The findings point toward an approach that is not only effective, but also gentler for young patients—an important step toward less painful but still powerful therapy for this aggressive childhood cancer.

High-risk neuroblastoma remains a challenging childhood cancer to treat. Anti-GD2 immunotherapy has improved outcomes, but it is often accompanied by severe neuropathic pain. Ruth Ladenstein and collaborators presented results from a clinical trial coordinated by the SIOPEN Neuroblastoma Research Network. They show that the monoclonal antibody dinutuximab beta can be given as a slower, 10-day long-term infusion in combination with subcutaneous interleukin-2. This approach makes treatment substantially easier to tolerate while preserving strong anti-tumor activity. Children required less intravenous morphine and experienced less treatment-related pain. The clinical outcomes remained encouraging, including a 45% end-of-treatment response rate and 2-year overall survival of 73%.

The study also provides important insight into why this immunotherapy can be effective. Outcomes were strongly influenced by immune factors linked to antibody-dependent cellular cytotoxicity, particularly genetic variations in the immune receptor Fcγ and natural killer cell activity. Children with favorable immune profiles had markedly better outcomes, which shows that the success of anti-GD2 therapy depends not only on the drug itself but also on how effectively the patient's immune system can engage with it. These findings deepen our understanding of how immunotherapy works in neuroblastoma and point to biomarkers that may help refine treatment selection in the future.

The results underline that delivery of a therapy can be improved without reducing its effectiveness, which makes a meaningful difference for patients. By supporting a less painful yet still active immunotherapy regimen, this study helps guide the development of treatment strategies that are both kinder and more precise—an important step toward gentler, still effective approaches for children with high-risk neuroblastoma.



Number of patients that received dinutuximab beta | Cycles | Day 1 | Day 2 | Day 3 | Day 4 | Day 5 | Day 6 | Day 7 | Day 8 | Day 9 | Day 10

122	Cycle 1	662	443	320	168	47	0	0	0	0	0
113	Cycle 2	550	336	142	12	0	0	0	0	0	0
95	Cycle 3	453	243	16	0	0	0	0	0	0	0
91	Cycle 4	414	242	0	0	0	0	0	0	0	0
87	Cycle 5	339	186	44	0	0	0	0	0	0	0

Intravenous morphine use in each dinutuximab beta long-term infusion cycle.

PUBLICATION

Lode, H. N., Siebert, N., Valteau-Couanet, D., Garaventa, A., Canete, A., Anderson, J., Yaniv, I., Ash, S., Gray, J., Klingebiel, T., Loibner, H., Luksch, R., Manzitti, C., Michon, J. M., Owens, C., Pötschger, U., Troschke-Meurer, S., Glogova, E., Ladenstein, R., & SIOP Europe Neuroblastoma Group (SIOPEN) (2025). Fcγ Receptor Polymorphism in Patients with Relapsed/Refractory High-Risk Neuroblastoma Correlates with Outcomes in the SIOPEN Dinutuximab Beta Long-Term Infusion Trial. *Clinical Cancer Research, 31* (17), 3692–3701. https://doi.org/10.1158/1078-0432.CCR-25-0180

STOP THROUGH SPOP: NEW STRATEGY AGAINST AGGRESSIVE BLOOD CANCER

IN SIMPLE TERMS:

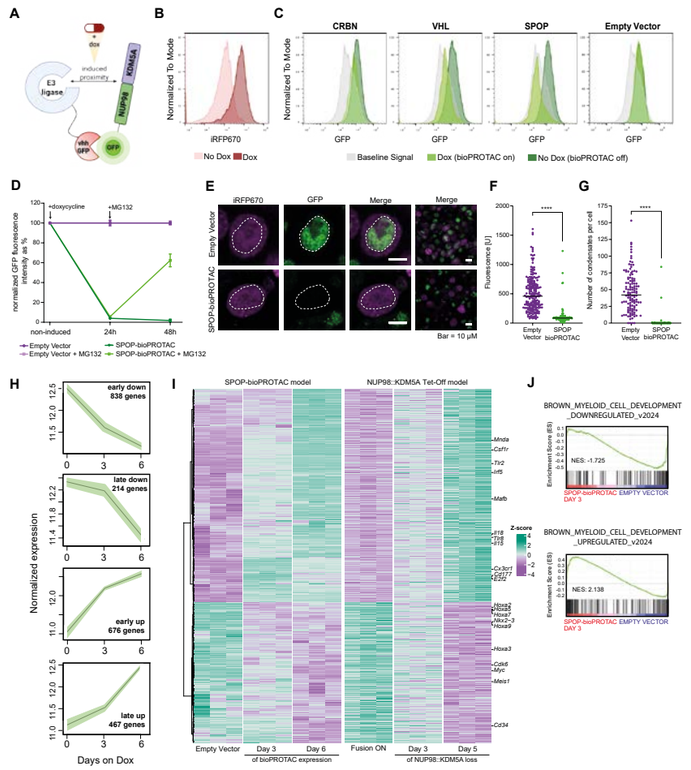
The Grebien group discovered a new way to target a highly aggressive form of childhood leukemia caused by *NUP98* gene fusions. The researchers identified the protein SPOP as a natural regulator able to control and degrade cancer-causing fusion oncoproteins. Using engineered molecules called bioPROTACs, the team succeeded in triggering the destruction of *NUP98* fusion oncoproteins, stopping leukemia cell growth in cell culture and animal models. This breakthrough could guide the development of new, highly targeted therapies for children suffering from AML with NUP98 gene fusions.

Acute myeloid leukemia with *NUP98* fusions (NUP98-r AML) is an aggressive blood cancer in children that is driven by abnormal gene fusions producing oncogenic proteins. These fusion oncoproteins block normal blood cell development and promote uncontrolled proliferation, making standard therapies largely ineffective. Until now, no strategies have been developed to directly degrade these cancer-causing proteins.

When they used CRISPR/Cas9 screening to identify genes controlling the stability of NUP98 fusion proteins, the Grebien group discovered that SPOP, an E3 ligase, functions as a tumor suppressor by naturally promoting the degradation of NUP98 fusion proteins. They observed that low SPOP levels in patients correlated with higher NUP98 fusion protein levels and a more aggressive disease.

Building on this insight, the team developed SPOP-bioPROTACs—engineered molecules that bring SPOP close to NUP98 fusion proteins to trigger their rapid degradation. Within 24 hours, this approach eliminated NUP98 fusion oncoproteins and induced differentiation and death in leukemia cells both in cultured cells and in animal models.

This study establishes SPOP as a novel tumor suppressor in NUP98-r AML and demonstrates a first-of-its-kind strategy to degrade oncogenic fusion proteins by means of proximity-inducing molecules. The findings lay the groundwork for future therapeutic approaches that could specifically target NUP98 fusion proteins and potentially other similar cancer-causing proteins, offering new hope for patients with this challenging childhood leukemia.



SPOP-bioPROTAC degrades NUP98::KDM5A onco-condensates and drives transcriptional programs related to myeloid lineage commitment. (A) Schematic diagram depicting the doxycycline-inducible bioPROTAC system in GFP-NUP98::KDM5A-expressing acute myeloid leukemia cells. (B) Histogram showing changes in iRFP670 median fluorescence intensity (MFI) upon addition of doxycycline, depicting the activation of bioPROTAC system. (C) Histograms showing degradation of NUP98::KDM5A as tracked by GFP MFI upon the activation of the indicated bioPROTACs. (D) Rescue of SPOP-bioPROTAC-mediated degradation of NUP98::KDM5A via the proteasome inhibitor MG132. (E) Confocal images of biomolecular condensates formed by GFP-NUP98::KDM5A upon expression of the SPOP-bioPROTAC or empty vector control. (F and G) Quantification of mean GFP intensity (F) and number of condensates (G) in GFP-NUP98::KDM5A-driven AML cells upon expression of the SPOP-bioPROTAC or empty vector. (H) Time-series plots depicting cluster analysis of differentially regulated genes upon the induction of the SPOP-bioPROTAC or an empty vector control in NUP98::KDM5A AML cells. (I) Heatmaps showing expression of differentially regulated genes that are common in SPOP-bioPROTAC-expressing and NUP98::KDM5A Tet-off cells. (J) Gene set enrichment plots in SPOP-bioPROTAC- and empty-vector-expressing GFP-NUP98::KDM5A cells for genes up- and downregulated in myeloid development.

PUBLICATION

Kirkiz, E., Kaufmann, G., Bergqvist, S., Fernández-Pernas, P., Eder, T., Quell, L., Allram, M., Manhart, G., Walter, W., Haferlach, T., & Grebien, F. (2025). Harnessing the E3 ligase SPOP for targeted degradation of the NUP98::KDM5A fusion oncoprotein. *Cell Reports, 44* (12), 116602. https://doi.org/10.1016/j.celrep.2025.116602

INSIDE OUR CORE UNITS

TRACK "FLUORESCENCE"

ILLUMINATING CHILDHOOD CANCER RESEARCH

At St. Anna CCRI, the Flow Cytometry Core Facility plays a critical but often behind-the-scenes role in both diagnostics and cutting-edge cancer research. Led by Elke Zipperer, the facility supports scientists, clinicians and diagnostic teams both across the institute and the neighboring St. Anna Children's Hospital in their efforts to better understand—and ultimately defeat—childhood cancer.

Equipped with two state-of-the-art research flow cytometers, two diagnostic instruments and a cell-sorter, the Flow Cytometry Core provides essential data that helps scientists uncover how cancer behaves, and clinicians to detect leukemia and immune status in children. "We support anyone who wants to do flow cytometry," says Zipperer. "That means training, helping with experimental setup, troubleshooting, sorting, data analysis—and, of course, keeping all the devices running."

TURNING LIGHT INTO KNOWLEDGE

The human body—and especially our blood—contains various cell types that look very similar at first sight. Thus, just observing cells under the microscope is usually not enough to effectively distinguish them.

"We know that every cell type has characteristic surface structures," Zipperer explains. "Using fluorescently labelled antibodies, we can make these structures visible and decide which type a cell belongs to."

Flow cytometry uses lasers with specific wavelengths to excite these fluorescent antibodies, which serve as "flags" marking proteins in a cell's surface. The resulting emitted light is collected by advanced detectors to analyze each single cell in detail and pinpoint its distinctive traits.

The technology allows researchers to analyze tens of thousands—or even millions—of cells at a time and detect what cell types are present in a sample, their numbers and the molecules they express. This information, crucial for cancer research and diagnostics, can be obtained quickly and reliably using the core unit's advanced machines.

SUPPORTING DIAGNOSTICS AT ST. ANNA CHILDREN'S HOSPITAL

Unlike most research facilities, St. Anna CCRI's Flow Cytometry Core Unit also hosts two diagnostic-grade cytometers used daily by other teams—a unique setup in Vienna.

One instrument, operated exclusively by the leukemia diagnostics group headed by Michael Dworzak, supports minimal residual disease (MRD) detection in children with leukemia. MRD testing helps determine how well a child is responding to therapy by detecting tiny numbers of leukemia cells that remain in the blood after treatment. "This tool is essential to identify leukemia patients at high risk of relapse and improve their treatment outcome," Zipperer explains.

The second diagnostic device is used to monitor the recovery of young patients who undergo stem cell transplantation, a treatment often used for high-risk leukemias.

"Using flow cytometry, we can track how blood cells reappear and increase after transplantation," Zipperer says. "For clinicians, this information is extremely important because it tells them when the new immune system begins to function and protect the patient."

This dual research-diagnostic role makes the facility a central hub connecting laboratory science and clinical care.

FUELING DISCOVERY IN CAR T DEVELOPMENT

On the research side, the Flow Cytometry Core Unit serves all groups across St. Anna CCRI, including the lab of Manfred Lehner, head of the Christian Doppler Laboratory for Next Generation CAR T Cells. His team works on improving CAR T cells, an emerging form of immunotherapy in which a patient's T cells are genetically equipped to fight cancer.

"For us, flow cytometry is essential," Lehner states. "It allows us to characterize our CAR T cells more effectively and precisely."

Beyond routine analysis, Lehner's lab uses the facility for directed evolution experiments, in which millions of protein variants displayed on yeast cells are screened to identify those that bind best to tumor targets.

"We generate millions of mutants that need to be tested," he says. "Flow cytometry allows us to fish out the few clones that do exactly what we want. Without cell sorting, this kind of protein engineering wouldn't be possible."

Cell sorting is an advanced system that allows scientists to sort single cells contained in tiny liquid droplets by applying an electrical charge. This high-precision method makes it possible for scientists to separate a certain cell type out of a mixed sample, providing pure cell populations that can be used for further experiments.

"Thanks to cell sorting, we can keep developing better and better CAR T cells that will hopefully help patients one day," Lehner adds.

THE PEOPLE BEHIND THE MACHINES

Flow cytometry is a powerful technology, but newcomers often find it intimidating.

"Sometimes people see it like a magic box," Zipperer laughs. "You put cells in, and something mysterious happens."

For that reason, the Flow Cytometry Core Unit places great emphasis on training researchers at St. Anna CCRI on how to use the machines and how to best plan their experiments.

The first point of contact is usually a hands-on introduction with the core unit's staff, Johannes Reisecker and Anna-Maria Husa. Building on their combined decades of experience in flow cytometry, they walk new users through how the instruments work, how to prepare their samples and how to plan their experiments to avoid common pitfalls.

"It's always better when we're involved from the beginning," Zipperer emphasizes. "We can help researchers choose the right fluorochrome combinations, avoid unnecessary costs and make sure people get good results straight away."

Lehner agrees that these discussions are essential, and often even inspirational.

"If you sit together and talk with the experts, ideas come up," he says. "Sometimes you learn that an instrument can do more than you had imagined, which opens new, exciting doors for further research."

A SMALL FACILITY WITH A HUGE IMPACT

The Flow Cytometry Core Unit at St. Anna CCRI stands out not only for the breadth of its instruments, but also for its integration with pediatric diagnostics—indeed something rare among comparable research institutes.

By providing expertise, training and advanced technologies, the team empowers scientists to run more accurate experiments, and clinicians to make better-informed decisions for patients.

"Our job is to give researchers everything they need to get the best results," says Zipperer. "Once they understand how flow cytometry works, it turns into a powerful tool they can really trust."

The Flow Cytometry Core Unit has become an essential part of St. Anna CCRI's mission. Their contributions support our researchers in their pursuit of knowledge on childhood cancer, shining light on the invisible world inside every cell.

TURNING DATA INTO DISCOVERY

Modern biomedical research generates enormous amounts of data. From genome sequencing to single-cell analyses, scientists today can measure thousands of biological features at once. However, extracting meaningful insights from these large datasets requires specialized expertise and powerful computational tools. At St. Anna CCRI, this challenge is addressed by the Bioinformatics Core Unit, a team that works closely with researchers across the institute to turn raw data into biological understanding.

"Bioinformatics is essentially about using computers to get more out of biology than you could in the wet lab alone," explains Jan Oppelt, Team Lead of the Bioinformatics Core Unit. "You can explore complex datasets, identify patterns across many experiments and uncover connections that would otherwise be impossible to detect."

A COLLABORATIVE APPROACH TO DATA ANALYSIS

Rather than operating as a simple support service, the Bioinformatics Core Unit collaborates closely with research groups. Scientists approach the team with datasets or biological questions, and together they develop an analysis strategy tailored to the project.

The unit's five specialists bring expertise in different areas, including but not limited to genomics, transcriptomics and single-cell data analysis. Once a collaboration begins, a team member takes ownership of the project and maintains close communication with the research group. This allows results to be discussed and refined throughout the process.

"We don't just run an analysis and send back a result," Oppelt says. "There is a continuous exchange with the scientists by way of sharing intermediate results, discussing whether the analysis is going in the right direction and adjusting the approach if needed."

This collaborative model also helps the team stay at the forefront of rapidly evolving computational methods. Researchers frequently introduce new technologies or experimental approaches, prompting the bioinformatics experts to explore and implement new analytical techniques.

“Technologies like machine learning are revolutionizing the field of biology,” Oppelt comments. “We make sure to keep up to date with new tools that can be useful for our scientists and support them in how to best apply them to their research.”

SUPPORTING SCIENCE ACROSS THE INSTITUTE

The Bioinformatics Core Unit contributes to a wide range of projects at St. Anna CCRI, from routine data analysis to the development of new computational methods. Some tasks involve running established pipelines—for example, analyzing sequencing data to check whether stem cell cultures have developed genomic abnormalities. These standardized analyses allow research groups to obtain reliable results quickly and efficiently.

At the same time, the team is also involved in more exploratory research collaborations. In these cases, the bioinformatics experts work closely with scientists to interpret complex datasets and design new analyses that offer deeper biological insights. For Polina Kameneva, one of the newest research group leaders at St. Anna CCRI, this expertise has been invaluable. Her team studies neuroblastoma initiation using stem cell–based models, generating highly complex datasets that combine multiple types of biological information.

“In one project, we introduced a mutation common in neuroblastoma into our model system and examined what happens in the very early stages after it appears,” Kameneva explains. “The dataset contained multiple layers of information from the same cells, which made the analysis quite challenging.”

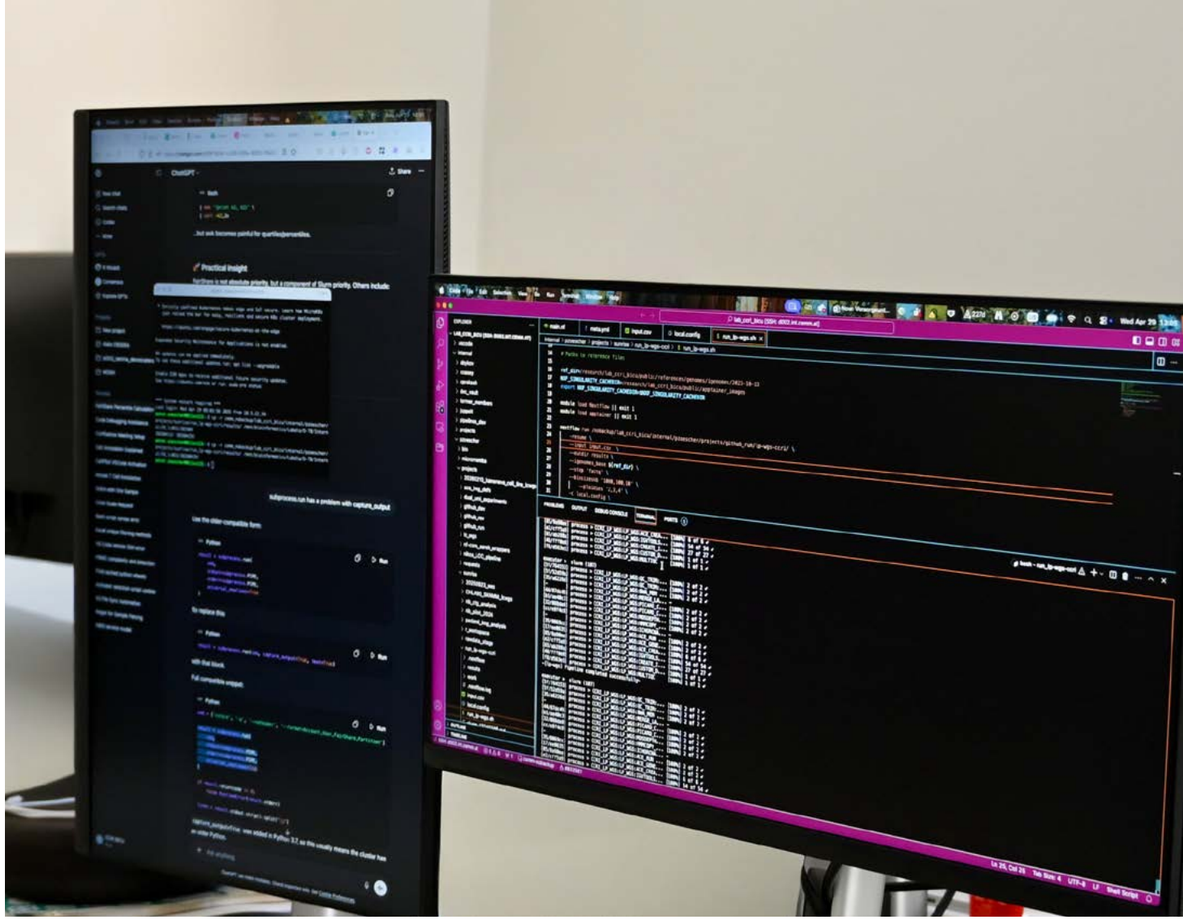
The Bioinformatics Core Unit helped the group process and interpret this complex data using specialized pipelines and computational approaches developed ad hoc.

“Having access to this expertise was crucial,” Kameneva says. “Without it, we might not have been able to analyze the data in the depth we needed. With their help, we could start to see what biological processes might be changing and design the next experiments.”

INFRASTRUCTURE FOR DATA-INTENSIVE RESEARCH

Handling large-scale biological datasets requires substantial computational power. To support this work, St. Anna CCRI collaborates with partners at CeMM Research Center for Molecular Medicine of the Austrian Academy of Sciences to provide access to advanced high-performance computing infrastructure.

This setup allows the Bioinformatics Core Unit to run demanding analyses efficiently while ensuring that research data is processed and stored securely. In addition to analysis, the team also contributes to institute-wide initiatives such as research data management and the development of best practices for computational workflows.



BUILDING BIOINFORMATICS EXPERTISE

Another important goal of the Bioinformatics Core Unit is to share knowledge across the institute. Oppelt and his team offer training opportunities and best-practice guidelines for scientists seeking to strengthen their computational skills.

The aim is to help researchers structure their data analysis projects in a reproducible and well-organized way from the very beginning.

“If people start a project with the right structure and tools, it saves a lot of time later,” Oppelt says. “It also ensures that our scientists have high-quality, reproducible code to build upon in the future.”

AN ESSENTIAL PARTNER FOR MODERN BIOLOGY

As biological experiments continue to generate ever larger and more complex datasets, bioinformatics has become an indispensable component of biomedical research. By combining computational expertise with close collaboration, the Bioinformatics Core Unit enables scientists at St. Anna CCRI to fully harness the potential of their data.

For researchers like Kameneva the impact is clear. “It’s like having several bioinformatics experts as an extension of your team,” she says. “Their knowledge helps us analyze complex data faster and more effectively—and ultimately move our research forward.”

HOW TINY FISH LARVAE FUEL PEDIATRIC CANCER RESEARCH

The Zebrafish Core Unit has become an essential engine behind many of St. Anna CCRI,'s scientific breakthroughs. The unit, which includes a dedicated aquatic facility and is supported by the ZANDR Platform, specializes in generating and applying cutting-edge zebrafish models for the study of pediatric cancers.

Jointly led by Stefanie Kirchberger and Caterina Sturtzel, a team of five supports researchers across the institute with expert advice, advanced techniques, and unique in vivo models that few cancer research institutes can offer.

FROM RESEARCH TO SERVICE

Originally developed from the institute's research efforts, the Zebrafish Core Unit has evolved into a service-oriented entity that enables scientists at St. Anna CCRI to easily translate discoveries from the petri dish to functional, organism-level models.

"Our services are mainly in-house, but we're also open to external collaborations in the field of pediatric cancer," explains Stefanie Kirchberger.

The unit's cornerstone tools are xenografts—human pediatric tumor cells transplanted into zebrafish larvae—and genetic tumor models created by expressing or modifying genes within zebrafish tissues. These two complementary approaches give researchers both speed and depth: Xenografts offer rapid functional testing, while genetic models deliver long-term, stable systems to study cancer development.

WHY ZEBRAFISH?

Zebrafish (*Danio rerio*) are small tropical freshwater fish, typically 3–5 cm long, with distinctive horizontal blue and silver stripes. They are active, social swimmers that live in groups and are relatives of carps and goldfish. These tiny fish—and particularly their embryonic and larval stages—have long been established as a laboratory model in developmental biology because they develop quickly and are easy to maintain—strengths that translate remarkably well into pediatric cancer research.

As Kirchberger notes, "Just 24 hours after fertilisation, the larvae already have a relatively complete set of organs. And they're mostly transparent, so we can easily visualize what's happening inside them." Employing zebrafish larvae and advanced imaging technology, experts at the Zebrafish Core Unit can study tumor behavior, metastasis, immune interactions, and vascular changes in real time.

In addition, zebrafish larvae can easily absorb compounds such as drugs directly from the water—a skill that makes them an essential model for drug studies.

"We can easily test different compounds just by adding them to the water," says Caterina Sturtzel. "This allows us to directly study how different drugs inhibit tumor growth, providing us with a powerful tool for identifying new therapeutic options."

But zebrafish larvae also act as a canary in a coalmine, warning of potential negative effects. "Thanks to our expertise on zebrafish development, we can quickly detect toxicity or effects on development associated to any drug we test," adds Sturtzel.

Combined, these features make zebrafish larvae an ideal intermediate model for researchers working primarily with cell cultures. Using this model, they can validate their findings before moving on to more complex and time-demanding animal models such as mice.

HOW A ZEBRAFISH XENOGRAFT IS MADE

Behind the scenes, xenografting is a delicate, technically demanding process that takes significant skill to perform reproducibly.

The team begins when larvae are barely two days old and just developed enough to have a full body plan. Using ultra-fine glass needles produced in-house, they inject 200–1,000 lab-grown tumor cells into a small cavity near the shrinking yolk or other sites.

"Some types of tumors, like osteosarcoma and Ewing sarcoma, thrive in this environment," Sturtzel explains. "Others, such as neuroblastoma, require substantial optimization."

The injected cells are fluorescently labeled for the team to easily track them within the larvae. Once the xenograft tumor is established, the larvae can be treated with drugs of interest. Comparing images obtained before and after treatment allows the team to determine whether the tumors grew, stalled, regressed, or disappeared entirely.

Such moments can be striking. "We once tested combination therapy and could see the tumor cells completely vanish after two days," Sturtzel recalls. "Moments like these prove to us how powerful this model is."

SUPPORTING RESEARCHERS EVERY STEP OF THE WAY

While imaging and microinjections are among the most visible aspects of the unit's work, much of their impact happens earlier, during experimental planning.

Most scientists approach the team when transitioning from in vitro to in vivo experiments. The unit's expert staff then helps them adapt their cellular models for zebrafish: adjusting culture conditions, establishing staining strategies, choosing drug concentrations, defining controls, and ensuring that the different compounds to test are safe for the larvae.

"It requires a lot of fine-tuning," Kirchberger stresses. "Our job is to support our scientists not only with the procedure itself but with everything leading up to it."

In addition to xenografts, the team also develops genetically engineered tumor models. Although these models require longer production timelines and a more complex process, they can be extremely useful to scientists studying how different genes influence tumor development. Some of the engineered zebrafish lines established by the team in the last years have become a benchmark research model both at the institute and beyond.

A UNIQUE SERVICE, LOCALLY AND INTERNATIONALLY

Few European institutes offer dedicated zebrafish platforms specifically tailored to pediatric oncology, with St. Anna CCRI being one of them. Therein lies the institute's distinctive strength.

"It's a unique strength of our institute," says Kirchberger. "Even within Europe, there are not many places with similar assets."

Through the associated ZANDR Platform and its high-content imaging capabilities, the Zebrafish Core Unit regularly collaborates with external groups from adjacent institutions such as the Medical University of Vienna and international collaborators like the University of Münster.

This very mix of specialization, accessibility, and collaborative spirit makes St. Anna CCRI an attractive destination for researchers seeking to accelerate translational cancer work.

EMPOWERING SCIENCE THROUGH A SMALL BUT MIGHTY MODEL

The Zebrafish Core Unit exemplifies how specialized technological platforms can reshape research possibilities. By giving scientists access to rapid, visual, and flexible in vivo systems, the team affords discoveries that would otherwise take months—or never happen at all.

The unit's expertise continues to push forward the boundaries of pediatric cancer research at St. Anna CCRI—all of which starts with a tiny, transparent fish larva.

FINANCIAL REPORT

TRACK "SIGNAL"

GUIDELINES FOR THE USE OF DONATIONS

For the operation of the research institute more than thirteen million euros are required annually, whereas the association has no basic funding from the public sector. Additional funds are acquired through competitive project grants from recognized national and international funding agencies.

We are committed to our donors to use the funds entrusted economically and efficiently. The annual financial statement is prepared in accordance with the provisions of § 22 of the Federal Act on Associations. As a large association, the financial management as well as the annual financial statements of the association are audited by a public accountant who provides an independent auditor's certificate. Thus, proper and appropriate handling and allocation of the donations in alignment with the statutes can be assured.

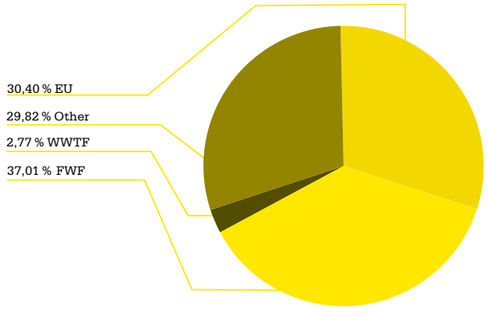
SEAL OF APPROVAL FOR DONATIONS AND TAX DEDUCTIBILITY

Since 2002, St. Anna Children's Cancer Research Institute has been one of the first organizations in Austria to receive the seal of approval for donations from the Chamber of Public Accountants and Tax Advisors. For the annual re-awarding, an auditor carries out an additional audit to the one already provided for the annual accounts, scrutinizing for transparency and proper use of funds in accordance with the strict guidelines of the Donation Quality Certificate. On the basis of a notice (Bescheid) issued by the Federal Ministry of Finance, St. Anna Children's Cancer Research Institute is classed as a tax-privileged group of recipients, so donations are tax-deductible from either private or corporate income tax.

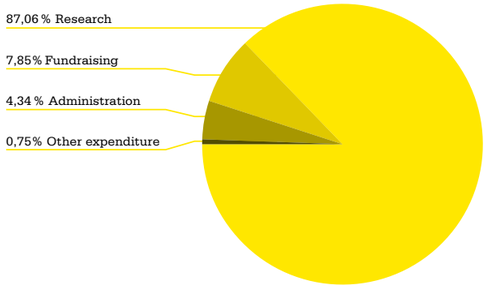
QUALITY ASSURANCE OF SCIENTIFIC WORK

The research institute has a Scientific Advisory Board - a committee of external experts - with the task of continuously evaluating the scientific work and advising the Institute's management. In addition, new scientific projects are regularly submitted to renowned national and international research funding bodies and research results are published in internationally recognized scientific journals. In addition, an objective assessment of the scientific performance by recognized external experts in the field takes place at regular intervals.

COMPETITIVE THIRD-PARTY FUNDS IN 2025 / KOMPETITIVE DRITTMITTEL IM JAHR 2025



ALLOCATION OF FUNDS 2025 / ZUWEISUNG DER GELDMITTEL IM JAHR 2025



SOURCE OF FUNDS / MITTELHERKUNFT

			2024	2025
I.	Donations	Spenden		
	a) undedicated donations	*a) ungewidmete*	€ 0,00	€ 0,00
	b) dedicated donations	*b) gewidmete*	€ 13.644.709,36	€ 16.968.545,21
II.	Membership fees	*Mitgliedsbeiträge*	€ 760,00	€ 640,00
III.	Operating income	*Betriebliche Einnahmen*		
	a) operating income from public funds	*a) betriebliche Einnahmen aus öffentlichen Mitteln*	€ 0,00	€ 0,00
	b) other operating income	*b) sonstige betriebliche Einnahmen*	€ 3.643.615,26	€ 1.886.283,13
IV.	Public subventions and subsidies	*Subventionen und Zuschüsse der öffentlichen Hand*	€ 0,00	€ 0,00
V.	Other income	*Sonstige Einnahmen*		
	a) asset management	*a) Vermögensverwaltung*	€ 11.785,79	€ 5.883,84
	b) other income not included in positions I to IV	*b) sonstige andere Einnahmen sofern nicht in Punkt I. bis IV. enthalten*	€ 0,00	€ 0,00
VI.	Revenue from release of donations and subsidies not yet used for the intended purpose	*Auflösung von Passivposten für noch nicht widmungsgemäß verwendete Spenden bzw. Subventionen*	€ 0,00	€ 0,00
VII.	Release of reserves	*Auflösung von Rücklagen*	€ 0,00	€ 0,00
VIII.	Annual loss	*Jahresverlust*	€ 0,00	€ 0,00
TOTAL			**€ 17.300.870,41**	**€ 18.861.352,18**

USE OF FUNDS / MITTELVERWENDUNG

			2024	2025
I.	Expenditures for statutorily defined purposes	*Leistungen für die statutarisch festgelegten Zwecke*	€ 13.617.539,39	€ 14.688.305,54
II.	Fundraising	*Spendenwerbung*	€ 1.320.494,92	€ 1.324.428,02
III.	Administration	*Verwaltungsausgaben*	€ 560.008,74	€ 731.927,68
IV.	Other expenditures not included in posititons I to III	*Sonstige Ausgaben, sofern nicht unter I. bis III. enthalten*	€ 13.132,61	€ 126.995,66
V.	Donations and subsidies not yet used for the intended purpose (allocation to liabilities)	*Zuführung zu Passivposten für noch nicht widmungsgemäß verwendete Spenden bzw. Subventionen*	€ 1.789.694,75	€ 1.989.695,28
VI.	Allocation of funds to reserves	*Zuführung zu Rücklagen*	€ 0,00	€ 0,00
VII.	Annual profit	*Jahresüberschuss*	€ 0,00	€ 0,00
TOTAL			**€ 17.300.870,41**	**€ 18.861.352,18**

ANNEX

TRACK "FACS"

LABDIA LABORDIAGNOSTIK GMBH

MANAGEMENT
Managing Director
Jörg Bürger
Thomas Lion
Katharina Rötzer-Londgin

Medical Director
Thomas Lion
Milos Hejtman (Deputy)

Commercial Director
Amelie Szalony

Secretariat
Claudia Gras
Victoria Milford

Central Sample Processing
Helga Daxberger
Katharina Mann (since 07/2025)°
Victoria Milford
Pia Riedl (until 03/2025)

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Department Head
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Lab Manager
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Laboratory Staff Members
Marlies Lampe (until 06/2025)*
Svea Pfefferkorn*
Fabian Pucher (since 08/2025)*
Daniela Scharner*
Anna Schmuckenschlager (since 09/2025)*
Dijana Trbojevic*
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Ulrike Kastner
Eva Winkler°
Sven Wohlmacher°

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Deputy Department Head & Staff Scientist
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Laboratory Staff Members
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Marie Christin Meta
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Andrea Inthal
Margit König
Sören Mai
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Laboratory Staff Members
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Larissa Kreuzhuber°
Bernhard Wildom°

* also works in St. Anna CCRI
° works in both groups

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Eleni Tomazou (since 02/2025)

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Caroline Hutter (since 04/2025)

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Executive Assistant
Maqeeta Ahmed

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Zoltán Dobai
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Karolina Valentova (from 01-08/2025)

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Reinhard Orense
Andrea Prantl (until 03/2025)
Christina Rigaud
Elisabeth Tax
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Barbara Umlauft

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Vanessa Kühl
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Sabine Radosavljevic
Tamara Roth (since 05/2025)
Christina Schendl (since 05/2025)
Caroline Schmid

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Senior Legal Counsel: Nina Zobernig-Krejci
Anastasia Kolleti

GIGAX FOUNDATION

Board Member
Ursula Kellner

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Staff Members
Adam Ahmed (until 06/2025)
Marcel Fürst (since 09/2025)
Claudia Hasenöhrl
Klaus Kienzer
Christian Six
Lukas Voglmüller
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Faith Godfrey (since 12/2025)

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Saskia Nemec
Anita Polgári
Yvonne Schnetzinger (until 01/2025)
Julia Weinzettl (until 10/2025)

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Staff Members
Monika Gabrle
Max Mayer

SPECIAL CLINICAL PROJECTS

Staff Member
Ruth Ladenstein

CORE UNITS

CLINICAL BIOLOGY & FACS CORE UNIT

Core Unit Head
Elke Zipperer

Technician
Christine Hoffmann-Freimüller
Marlies Lampe (until 06/2025)
Svea Pfefferkorn
Fabian Pucher (since 08/2025)
Daniela Scharner
Anna Schmuckenschlager (since 09/2025)
Dijana Trbojevic
Julia Westermayer (since 02/2025)

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Laura Senk (until 02/2025)
Johannes Reisecker

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Chloe Anne Lara Casey
Celine Prakash
Peter Zöscher

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Andrea Wenninger-Weinzierl

Aquatic Technician
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John Joseph Cawley
Ekin Dogan (until 02/2025)
Katharina Musey (since 03/2025)

Volunteers
Martin Distel (until 06/2025)
Sarah Grissenberger (until 03/2025, jointly with Kovar Group)

AFFILIATED CLINICIANS

AFFILIATED CLINICIANS FROM ST. ANNA CHILDREN'S HOSPITAL

Andishe Attarbaschi, MD
Edit Bardi, MD
Dorothea Bauer, MD
Heidrun Boztug, MD
Anna Cvrtak, MD
Michael Dworzak, MD
Sebastian Eder, MD
Gernot Engstler, MD
Caroline Hutter, MD
Leo Kager, MD
Anita Lawitschka, MD
Roswitha Lüftinger, MD
Christina Peters, MD
Herbert Pichler, MD
Fiona Poyer, MD
Dominik Reisinger, MD
Leila Ronceray, MD
Hannah von Mersi, MD
Volker Witt, MD

PHD, MASTER'S (DIPLOMA) AND BACHELOR'S THESES

GRADUATED IN 2025

CARLOS ROCHE ARCAS
Proteasomal Degradation of uncomplexed KAT2A mediated by the HECT-type E3 ligase
> Supervised by Davide Seruggia
Master thesis

MATTHIAS KELLNER
Contrastive Representation Learning on Synthetic Imaging Data for Classification of Alternative Lengthening of Telomeres
> Supervised by Sabine Taschner-Mandl
MSc thesis

PETER PENEDER
Integrative Machine Learning Approaches for Multimodal Biomedical Data: From Cell-Free DNA Fragments to Single-Cell Transcriptomes
> Supervised by Eleni Tomazou
PhD Thesis

MOHAMED SHOEB
Perturbation of gene regulatory networks in pediatric hematopoietic malignancies
> Supervised by Florian Halbritter
PhD Thesis

ADAM VARADY
Light-mediated control over cancer modelling and treatment in zebrafish
> Supervised by Martin Distel
PhD Thesis

ELLA WEIDL
Validierung der Immunphänotypisierung mittels Durchflusszytometrie bei akuten Leukämien unter Berücksichtigung der ISO 15189
> Supervised by Michael Dworzak
Bachelor Thesis

ACKNOWLEDGEMENTS

- Abbott Österreich GmbH / Abbott Austria GmbH
- Alex's Lemonade Stand Foundation for Childhood Cancer
- Alle der St. Anna Kinderkrebsforschung nahestehenden Institutionen, Verbände und Vereine / All institutions, associations and societies related to St. Anna Children's Cancer Research Institute
- Alle Direktionen und alle Mitarbeiter:innen des St. Anna Kinderspitals / All heads and employees of St. Anna Children's Hospital
- Alle Mitarbeiter:innen der St. Anna Kinderkrebsforschung / All employees of the St. Anna Children's Cancer Research Institute
- Alle Vereins- und Vorstandsmitglieder / All association and board members
- Ambassadors: Eric Demuth, Michael Höllerer, Daniel Jelitzka, Dominic Thiem
- BAUHAUS Depot GmbH
- Bezirksorganisation Alsergrund / District Organisation Alsergrund
- BMW Goup Werk Steyr
- Boehringer Ingelheim Fonds
- Bone Cancer Research Trust
- Bundesministerium für Bildung, Wissenschaft und Forschung (BMBWF) / Federal Ministry of Education, Science and Research (BMBWF)
- Bundesministerium für Frauen, Familie, Integration und Medien/ Federal Ministry for Federal Ministry for Women, Family, Integration and Media
- Bundesministerium für Inneres / Federal Ministry of the Interior
- Bundesministerium für Soziales, Gesundheit, Pflege und Konsumentenschutz / Federal Ministry of Social Affairs, Health, Care and Consumer Protection
- Cancer Research UK
- CeMM Forschungsinstitut für Molekulare Medizin der Österreichischen Akademie der Wissenschaften / CeMM Research Center for Molecular Medicine of the Austrian Academy of Sciences
- Christian Doppler Forschungsgesellschaft / Christian Doppler Research Association (CDG)
- Desireé Treichl-Stürghk
- Deutsches Krebsregister, Universität Mainz / German Cancer Registry, University of Mainz
- EBMT Europäische Gruppe für Blut- und Knochenmarktransplantation / EBMT European Society for Blood and Marrow Transplantation
- Energy3000 Solar GmbH
- Ethikkommission der Medizinischen Universität Wien / Ethics Commission of the Medical University of Vienna
- European Commission / Europäischen Kommission
- European Hematology Association (EHA)
- Eva Angyan
- Fellinger Krebsforschung / Fellinger Cancer Research
- Fonds der Stadt Wien für innovative interdisziplinäre Krebsforschung / City of Vienna Fund for Innovative, Interdisciplinary Cancer Research
- Fonds zur Förderung der wissenschaftlichen Forschung (FWF) / Austrian Science Fund (FWF)
- Forschungsrahmenprogramme der Europäischen Kommission / Research and Innovation Programmes of the European Commission
- Gesellschaft für Pädiatrische Onkologie und Hämatologie (GPOH) / Society for Paediatric Oncology and Haematology
- Gigax Privatstiftung FL | Gigax Private Trust FL
- Handler Holding GmbH
- Herzfeldersche Familienstiftung / Herzfeldersche Family Foundation
- Histiocytosis Association
- Incyte Corporation
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- Innovative Medicines Initiative (IMI)
- Kurt Mann Bäckerei & Konditorei GmbH & Co KG
- Leyrer + Graf Baugesellschaft m.b.H
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- Medizinische Universität Wien / Medical University of Vienna
- Medizinisch-Wissenschaftlicher Fonds des Bürgermeisters der Stadt Wien / Medical-Scientific Fund of the Mayor of the capital city of Vienna
- Mitglieder des Ehrenkomitees der St. Anna Kinderkrebsforschung / Members of the St. Anna Children's Cancer Research Institute Honorary Committee
- Nationale und internationale Medienpartner:innen / National and international media partners
- Omar Sarsam
- Oncopeptides AB
- Österreichische Agentur für Bildung und Internationalisierung (OeAD) / Austria's Agency for Education and Internationalisation (OeAD)
- Österreichische Akademie der Wissenschaften (ÖAW) / Austrian Academy of Sciences (ÖAW)
- Österreichische Forschungsförderungsgesellschaft (FFG) / Austrian Research Promotion Agency (FFG)
- Österreichische Gesellschaft für Hämatologie & Medizinische Onkologie (ÖGHO) / Austrian Society of Haematology and Oncology (ASHO)
- Österreichische Nationalbank (OeNB) / Austrian National Bank (OeNB)
- Österreichischen Gesellschaft für Kinder- und Jugendheilkunde (ÖGKJ) / Austrian Society for Paediatric Medicine (ÖGKJ)
- Österreichisches Rotes Kreuz, Landesverband Wien / Austrian Red Cross, Vienna Regional Association
- Österreichisches Stammzell-Register / Austrian Stem Cell Register
- Paradifference Foundation
- Peter und Traudl Engelhorn Stiftung / Peter and Traudl Engelhorn Foundation
- Polymun Scientific
- Private Förder:innen, Mentor:innen, Vereinsmitglieder und Spender:innen der St. Anna Kinderkrebsforschung / Private sponsors, mentors, association members and donors for the St. Anna Children's Cancer Research Institute
- SIOPEN
- Solving Kids' Cancer UK
- SPAR AG, SPAR Wien, Niederösterreich, nördliches Burgenland
- Stadt Wien / City of Vienna
- Stadtrat für Soziales, Gesundheit und Sport / Senior City Councillor for Social Affairs, Health and Sport
- The Myrovlytis Trust / The Myrovlytis Trust
- Universität für Bodenkultur / University of Natural Resources and Life Science
- Universität Wien / University of Vienna
- Verein für Dermatologie, Wien / Association for Dermatology Vienna
- Wiener Wissenschafts-, Forschungs- und Technologiefonds (WWTF) / Vienna Science and Technology Fund (WWTF)
- Wirtschaftsagentur Wien / Vienna Business Agency

PUBLICATIONS

LEAD PUBLICATIONS

* Shared first authorship, # Shared senior authorship.

1. Kirkiz, E., Kaufmann, G., Bergqvist, S., Fernandez-Pernas, P., Eder, T., Quell, L., Allram, M., Manhart, G., Walter, W., Haferlach, T., & Grebien, F. (2025). Harnessing the E3 ligase SPOP for targeted degradation of the NUP98::KDM5A fusion oncoprotein. Cell Rep, 44(12), 116602, Article PMID: 41307993 https://doi.org/10.1016/j.celrep.2025.116602

2. Kalinichenko, A., Huemer, J., Humer, T., Haimel, M., Svaton, M., Socquet-Juglard, N., Casoni, G. P., Prakash, C., von der Linde, M., Pazmandi, J., van de Wetering, C., Nunez-Fontarnau, J., Kamnev, A., Giuliani, S., Jaeger, M. G., Hahn, E., Dobner, S., Rukavina, A., Sylvander, E., Seigner, J., Rashkova, C., Hoeger, B., Traxlmayr, M. W., Lehner, M., Bryceson, Y. T., Saarela, J., Hannich, T., Castanon, I., Winter, G., Dupre, L., & Boztug, K. (2025). Protein palmitoylation and sphingolipid metabolism control regulated exocytosis in cytotoxic lymphocytes. Sci Immunol, 10(112), eado3825, Article PMID: 41105755 https://doi.org/10.1126/sciimmunol.ado3825

3. Haladik, B., Maurer-Granofszky, M., Zoescher, P., Jimenez-Heredia, R., Frohne, A., Segarra-Roca, A., Casey, C., Kartnig, F., Giuliani, S., Rashkova, C., Repiscak, P., Dworzak, M. N., Superti-Furga, G., & Boztug, K. (2025). Image-based drug screening combined with molecular profiling identifies signatures and drivers of therapy resistance in pediatric AML. Cell Rep Med, 102304, Article PMID: 40840446 https://doi.org/10.1016/j.xcrm.2025.102304

4. Shoeb, M. R., Schinnerl, D., Shaw, L. E., Farlik, M., Strehl, S., Halbritter, F., & Fortschegger, K. (2025). A stem cell differentiation model reveals two alternative fates in CBFA2T3::GLIS2-driven acute megakaryoblastic leukemia initiation. Commun Biol, 8(1), 1289, Article PMID: 40866546 PMC12391542 https://doi.org/10.1038/s42003-025-08730-4

5. Gelineau, N. U., Bozsaky, E., van Zogchel, L. M. J., Rifatbegovic, F., Lazic, D., Ziegler, A., Javadi, A., Zappeij-Kannegieter, L., Potschger, U., Fiocco, M., Ambros, P. F., Ambros, I. M., Bodenmiller, B., van der Schoot, E. C., Ladenstein, R., Bernkopf, M., Tytgat, G. A. M., & Taschner-Mandl, S. (2025). Sensitive detection of minimal residual disease and immunotherapy targets by multi-modal bone marrow analysis in high-risk neuroblastoma - a multi-center study. J Exp Clin Cancer Res, 44(1), 224, Article PMID: 40753395 PMC12317575 https://doi.org/10.1186/s13046-025-03481-w

6. Kager, L., & Boztug, K. (2025). The NUDIX hydrolase NUDT5 influences purine nucleotide metabolism and thiopurine pharmacology. J Clin Invest, 135(14), Article PMID: 40662363 PMC12259244 https://doi.org/10.1172/JCI194434

7. Lode, H. N., Siebert, N., Valteau-Couanet, D., Garaventa, A., Canete, A., Anderson, J., Yaniv, I., Ash, S., Gray, J., Klingebiel, T., Loibner, H., Luksch, R., Manzitti, C., Michon, J. M., Owens, C., Potschger, U., Troschke-Meurer, S., Glogova, E., & Ladenstein, R. (2025). FC gamma receptor polymorphism in relapsed/refractory high-risk neuroblastoma patients correlates with outcomes in the SIOPEN dinutuximab beta long-term infusion trial. Clin Cancer Res, Article PMID: 40627545 https://doi.org/10.1158/1078-0432.CCR-25-0180

8. Andreana, M., Sentosa, R., Sturtzel, C., Pfister, M., Werkmeister, R., Schmitt, A., Traver, D., Leitgeb, R., Drexler, W., Distel, M., & Unterhuber, A. (2025). Multimodal Investigation of Angiogenesis and Its Prevention by Small Compounds in a Zebrafish Cancer Model. Adv Sci (Weinh), e15176, Article PMID: 40557748 https://doi.org/10.1002/advs.202415176

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INTERNATIONAL AND NATIONAL GRANTS 2025

INTERNATIONAL GRANTS 2025

Contribution to the Single cell Pediatric Cancer Atlas
CCRI responsible Project Lead: Sara Wernig-Zorc (Sabine Taschner-Mandl group)
Grant from the Alex's Lemonade Stand Foundation, USA
Duration 01/11/2025 – 01/05/2026

The prognostic value of circulating tumour DNA and RNA and circulating IGF2BP3 protein in recurrent and refractory Ewing sarcoma: biomarker analysis of the rEECur trial
CCRI responsible Principal Investigator: Eleni Tomazou
Coordinator: Martin McCabe (University of Manchester, UK)
Grant from the Bone Cancer Research Trust, UK
Duration: 22/09/2025 – 21/09/2027

In vivo programming of Ewing sarcoma
CCRI responsible Project Lead: Hana Bernhardova (Eleni Tomazou group)
Grant from the Boehringer Ingelheim Fonds (BIF), Germany
Duration 01/09/2025 – 31/08/2027

Genome wide copy number study on patients enrolled in the SIOPEN/ HR-NBL1 trial
CCRI responsible Project Investigator: Sabine Taschner-Mandl
Grant from the SIOPEN Association
Duration 01/06/2025 – 31/05/2027

Contribution to the Single cell Pediatric Cancer Atlas
CCRI responsible Project Lead: Maud Plaschka (Florian Halbritter group)
Grant from the Alex's Lemonade Stand Foundation, USA
Duration 15/12/2024 – 15/06/2025

The European Rare Diseases Research Alliance (ERDERA)
CCRI responsible Principal Investigator: Kaan Boztug
Coordinator: Daria Julkowska (INSERM, France)
Grant from the European Union, HORIZON Work Programme, ID – 101156595
Duration: 01/09/2024 – 31/08/2031

Tumor initiation in familiar SDHb-mutated paraganglioma modeled in human iPSCs-based organoids
CCRI responsible Principal Investigator: Polina Kameneva
Grant from the Paradifference Foundation, UK
Duration 01/09/2024 – 31/08/2026

Building models of fusion-driven sarcomas via cell fate engineering
CCRI responsible Project Lead: Hana Bernhardova (Ornella Urzi group)
Grant from the European Union, Marie Skłodowska-Curie Actions-PF, ID – 101149215
Duration 01/05/2024 – 30/04/2026

A SIOPEN pragmatic clinical trial to MOnitor NeuroblastomA relapse with LIquid biopsy Sensitive Analysis (MONALISA)
CCRI responsible Principal Investigator and Scientific Coordinator: Sabine Taschner-Mandl
Coordinator: SIOPE, Belgium
Grant from the European Commission, HORIZON Work Programme, ID – 101137028
Duration: 01/01/2024 – 31/12/2028

European Reference Network on Paediatric Oncology (ERN-PaedCan)
CCRI responsible Principal Investigator and Coordinator: Ruth Ladenstein
Grant from the European Union, EU4Health Work Programme, ID – 101155946
Duration: 01/10/2023 – 30/09/2027

Developmentally programmed pediatric sarcomas: a versatile platform for drug discovery and molecular precision medicine (SARCOMAkids)
CCRI responsible Principal Investigator: Eleni Tomazou
Grant from the European Union, HORIZON ERC-Consolidator Grant, ID - 101087883
Duration: 01/09/2023 – 31/08/2028

International Study for Treatment of Childhood Relapsed ALL 2020 (IntReALL 2020)
CCRI responsible Principal Investigator: Andishe Attarbaschi
Coordinator: Arend von Stackelberg (Charité, Germany)
Grant from the European Commission, HORIZON Work Programme, ID – 101104582
Duration: 01/05/2023 – 30/04/2028

European Rare Disease Research Coordination and Support Action (ERICA)
CCRI responsible Principal Investigator: Ruth Ladenstein
Coordinator: Alberto Pereira (Leiden University Medical Center, the Netherlands)
Grant from the European Union, H2020 Grant Agreement ID – 964908
Duration: 01/03/2021 to 31/08/2025

PanCare studies of the scale-up and implementation of the digital Survivorship Passport to improve people-centred care for childhood cancer survivors (PanCareSurPass)
CCRI responsible Principal Investigator: Ruth Ladenstein
Coordinator: Desiree Grabow (Universitätsmedizin Mainz, Germany)
Grant from the European Union, H2020, ID – 899999
Duration: 01/03/2021 to 31/08/2025

Tracking Ewing sarcoma origin by developmental and trans-species genomics (ORIGIN)
CCRI responsible Principal Investigator and Coordinator: Heinrich Kovar
Grant from the Alex's Lemonade Stand Foundation, USA, Crazy 8 Initiative Award Program
Duration: 01/03/2021 – 28/02/2026

Functional Interrogation of Non-coding DNA Sequences in leukemia development and drug resistance (FIND-seq)
CCRI responsible Principal Investigator: Davide Seruggia
Grant from the European Union, H2020 ERC-Starting Grant, ID – 947803
Duration: 01/03/2021 – 28/02/2027

Integrated and standardized NGS workflows for Personalised therapy (INSTAND-NGS4P)
CCRI responsible Principal Investigators: Ruth Ladenstein and Kaan Boztug
Coordinator: Kurt Zatloukal (Medical University Graz, Austria)
Pre-Commercial Procurement (PCP) Grant from the European Union, H2020 – Innovation Procurement, ID – 874719
Duration: 01/01/2020 - 31/05/2025

Charting key molecules and mechanisms of human immune Dysregulation (iDysChart)
CCRI responsible Principal Investigator and Awardee: Kaan Boztug
Grant from the European Union, ERC Consolidator Grant, ID - 820074
Duration: 01/06/2019 – 31/05/2025

Comprehensive heatmap for TKI-resistance of mutations in BCR-ABL1 kinase domain
CCRI responsible Principal Investigator: Thomas Lion group
Incyte Corporation - Incyte open calls, ID – 5907191
Duration: 01/01/2019 – 01/04/2027

NATIONAL GRANTS 2025

Resistance evolution in solid paediatric cancers with HRD
CCRI responsible Principal Investigator: George Cresswell
Grant from the Austrian Science Fund (FWF), Principal Investigator Project (PAT)
DOI: 10.55776/PAT2727724
Duration: 01/11/2025 – 31/10/2029

Understanding the role of BRAFV600E in Langerhans Cell Histiocytosis (UnVEiL)
CCRI responsible Principal Investigator: Caroline Hutter
Grant from the Austrian Science Fund (FWF), Principal Investigator Project (PAT)
DOI: 10.55776/PAT6449724
Duration: 01/04/2025 – 31/03/2028

Improving tumor specificity of cellular immunotherapies
Head of CD Laboratory and CCRI responsible Project Lead: Manfred Lehner
Grant from the Austrian Science Fund (FWF), Principal Investigator Project (PAT)
DOI: 10.55776/PAT8789924
Duration: 01/11/2024 – 31/10/2028

EXPLORE-NB
CCRI responsible Principal Investigator: Sabine Taschner-Mandl
Grant from the Austrian Science Fund (FWF), International - Multilateral Initiatives
DOI: 10.55776/PIN2827223
Duration: 01/11/2024 – 31/10/2027

Single cell transcriptomics and epigenomics unravel the role of monocytes in neuroblastoma bone marrow metastasis
CCRI responsible Principal Investigator: Sabine Taschner-Mandl
Grant from the Austrian Society for Pediatrics and Adolescent Medicine (ÖGKJ) – 2024 Science Prize
Duration: 05/10/2024 – 31/12/2027

Modelling pediatric tumor initiation with human stem cells
CCRI responsible Principal Investigator and Awardee: Polina Kameneva
Grant from the Austrian Science Fund (FWF), START Award
DOI: 10.55776/STA193
Duration: 01/10/2024 – 30/09/2029

Devising Advanced TCR-T-cells to eradicate Osteosarcoma (DART2OS)
CCRI responsible Principal Investigator: Sabine Taschner-Mandl
Coordinator: Johannes Zuber (IMP, Austria)
Grant from the Austrian Science Fund (FWF), Emerging Fields Program
DOI: 10.55776/EFP45
Duration: 01/10/2024 – 30/09/2029

Role of enhancers in non-mutational drug resistance and relapse
CCRI responsible researcher and Awardee: Leonie Lehmayer (Supervisor: Davide Seruggia)
Grant from the Austria Academy of Sciences (ÖAW), DOC fellowship, ID – 27138
Duration: 01/09/2024 – 01/09/2026

ß-catenin Roles and Dynamics in Wilms Tumors
CCRI responsible researcher and Awardee: Maud Plaschka (Supervisor: Florian Halbritter)
Grant from the Austrian Science Fund (FWF), ESPRIT Program
DOI: 10.55776/ESP652
Duration: 01/09/2024 – 31/08/2027

Oncogenic aberration of development in childhood cancer
CCRI responsible Principal Investigator: Florian Halbritter
Grant from the Austrian Science Fund (FWF), Principal Investigator Project (PAT)
DOI: 10.55776/PAT1300223
Duration: 01/06/2024 – 31/05/2028

Human Induced Pluripotent Stem Cells as a preclinical trial platform for Langerhans Cell Histiocytosis (HIPSC-LCH)
CCRI responsible Principal Investigator: Caroline Hutter
Grant from the Austrian Science Fund (FWF), Principal Investigator Project (PAT)
DOI: 10.55776/P37332
Duration: 01/11/2024 – 31/10/2028

New Hsp90 Inhibitor-based Therapies for Ewing Sarcoma (HSP90IES)
Head of Facility and CCRI responsible Project Lead: Caterina Sturtzel
Grant from the Austrian Science Fund (FWF), Principal Investigator Projects International
DOI: 10.55776/I6685
Duration: 08/01/2024 – 07/01/2027

Proteostasis, Metabolism and a Novel Immunodeficiency Syndrome (PROMISE)
CCRI responsible Project Lead: Michael Kraakman (Kaan Boztug group)
Grant from the Austrian Science Fund (FWF), Principal Investigator Project
DOI: 10.55776/PAT4663523
Duration: 01/12/2023 – 30/11/2026

Disease-associated variants at ARID5B
CCRI responsible Principal Investigator: Davide Seruggia
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P36302
Duration: 01/09/2023 – 31/08/2026

Artificial intelligence for diagnostics of ALT-positive cancer (AI4CAN)
CCRI responsible Principal Investigator: Sabine Taschner-Mandl
Grant from the Vienna Science and Technology Fund (WWTF), NEXT 2022, ID – NXT22-009
Duration: 01/09/2023 – 30/06/2025

Lost in translation
CCRI responsible Principal Investigator: Kaan Boztug
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P36548
Duration 01/02/2023 – 31/08/2026

RiboPOP
CCRI responsible Project Lead: Irinka Castanon (Kaan Boztug group)
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P36334
Duration 01/02/2023 – 31/01/2027

Harnessing vulnerabilities at SAGA in MYC-driven cancer
CCRI responsible Principal Investigator: Davide Seruggia
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P36069
Duration: 01/01/2023 – 31/12/2026

Linking ex-vivo chemosensitivity, treatment and pathway activations for a deeper understanding of pediatric AML (ExTrAct-AML)
CCRI responsible Principal Investigator and Coordinator: Kaan Boztug
Additional project partners: Giulio Superti-Furga (CeMM) and Michael Dworzak (CCRI)
Grant from the Austrian Science Fund (FWF), Programme Clinical Research
DOI: 10.55776/KLI1056
Duration: 01/10/2022 – 30/09/2025

Exploration of lung metastases in pediatric cancer through single-cell analysis and 3d modelling (MetLung)
CCRI responsible Principal Investigator and Coordinator: Heinrich Kovar
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P35353
Duration: 02/05/2022 – 01/03/2026

Mapping metastatic cancer by multi-modal imaging (MAPMET)
CCRI responsible Principal Investigator: Sabine Taschner-Mandl
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P35841
Duration: 01/05/2022 – 30/04/2026

Interplay of fusion genes and cellular context in sarcoma
CCRI responsible Principal Investigator: Eleni Tomazou
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P34958
Duration: 01/10/2021 – 30/09/2025

Validation of a liquid biopsy based molecular diagnostic toolkit for pediatric sarcomas
CCRI responsible Principal Investigator: Eleni Tomazou
Grant from the Vienna Science and Technology Fund (WWTF), Life Sciences 2020, ID – LS20-045
Duration: 01/09/2021 – 31/08/2025

EWS-FLI1 fluctuation in Ewing sarcoma
CCRI responsible Principal Investigator: Heinrich Kovar
Grant from the Austrian Science Fund (FWF), Stand-Alone Project
DOI: 10.55776/P34341
Duration 01/04/2021 – 31/03/2025

CD Laboratory for "Next generation CAR-T cells"
Head of CD Laboratory and Coordinator: Manfred Lehner
Grant from the Christian Doppler Association, Christian Doppler Lab, ID – 345
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