



Learn more about the Beat Childhood Cancer Research Consortium by scanning the QR code with your smartphone camera.



Beat Childhood Cancer Research Consortium opens new trial in 2025

The Research Consortium successfully activated a new clinical trial in 2025 to translate preclinical research directly to the patient, BCC020. BCC020 is a Dose Escalation Study Using Difluoromethylornithine (DFMO) and AMXT-1501 for relapsed/refractory neuroblastoma, sarcomas, atypical teratoid rhabdoid tumor (ATRT), embryonal tumor with multilayer rosettes (ETMR) and newly diagnosed diffuse intrinsic pontine glioma (DIPG). The

first enrollment is expected Q1 2026. We also expect BCC023, a study using DFMO in 4 patient cohorts for Ewings sarcoma and osteosarcoma, to be open Q1 2026! This expansion of additional trials would not be possible without the support of the BCC Foundation, Four Diamonds and parent donors across the country!

International Expansion Launched!

We're thrilled to announce that the Beat Childhood Cancer Research Consortium, in collaboration with Penn State, has finalized agreements to add not one, but two international sites outside of North America! Our first international partner to join BCC is Hospital Sant Joan de Déu (SJD) Children's Hospital in Barcelona, Spain, led by Dr. Jaume Mora. During our October 2025 on-site visit, we were impressed by their comprehensive cancer care and dedicated research spaces. We also warmly welcome GRAACC Children's Hospital in São Paulo, Brazil, under Dr. Natalia Ambar. Visiting GRAACC was equally inspiring, showcasing their exceptional commitment to pediatric cancer treatment and research. These

partnerships mark a significant step forward in our mission to combat childhood cancer on a global scale. These collaborations will enable us to leverage diverse expertise and resources, accelerating our efforts to develop innovative treatments and improve outcomes. Both SJD and GRAACC have submitted BCC018, our clinical trial adding naxitamab to standard upfront HRNB therapy, to their regulatory authorities in Spain and Brazil respectively and we expect them to be open for enrollment very soon! Together, these partnerships represent a monumental stride in our commitment to advancing pediatric oncology research and bringing hope to families around the globe.



We are so excited for patients across the world to have access to cutting-edge clinical trials. We want to thank all the researchers, sites and collaborators that helped make this amazing accomplishment a reality!

WE ARE PENN STATE

2025 | BY THE NUMBERS

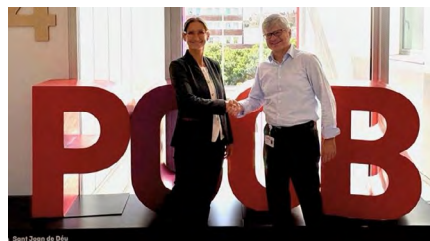
131 Patients enrolled on treatment studies across 9 clinical trials

Patients enrolled on BCC-BIO-001 biology clinical trial **234**

86 Cell lines established by POTR Lab

Number of patient samples that came to the POTR Lab in 2025 **3,031**

73 Molecular Tumor Boards held for patients in 2025



2025 | PUBLICATIONS

BCC publications

Journal of Clinical Medicine – Polyamine Inhibition with DFMO: Shifting the Paradigm in Neuroblastoma Therapy

Journal of Pediatric Hematology/Oncology – Naxitamab - Combination Therapy for the Treatment of Patients With Refractory and/or Relapsed High-Risk Neuroblastoma

Frontiers in Pharmacology – Molecular Guided Therapy Leading to Exceptional Response in Relapsed Osteosarcoma

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A MESSAGE FROM OUR FOUNDER

We welcome everyone to our 18th BCC Annual Consortium Meeting!

As we look back on the past year, I am deeply honored and inspired by the progress we've achieved together in our mission to support families confronting childhood cancer. Our dedication to advancing research and discovering groundbreaking therapeutic solutions, especially in the field of precision medicine and immunotherapy, remains steadfast. The strength of our success lies in the spirit of collaboration that defines the Beat Childhood Cancer community. This consortium brings together physicians, scientists, nurses, parents, advocates, foundations, and partners across the pharmaceutical and biotech sectors, united by one purpose: to improve survival and quality of life for children battling cancer. Through innovation and the pursuit of safer, more effective treatments, we are transforming hope into reality.



Several important goals of the Beat Childhood Cancer Research Consortium will guide our collective work in 2026 and beyond:

- First enrollment on the BCC023 trial combining DFMO with standard of care therapies for Ewings sarcoma and osteosarcoma.
- Complete Phase 1 portion of the BCC021 trial utilizing Casein kinase 2 (CK2) inhibitor for relapsed/refractory neuroblastoma and sarcomas.
- Complete the preclinical and regulatory work needed to launch a Phase I trial using IL13Ra2-Targeting Immunotoxin GB13 for DIPG.
- Expand upon our precision immunotherapy program to include launching a mRNA vaccine program and a novel CAR-T cell approach.
- First patient enrollment on BCC018 trial utilizing naxitamab during upfront high risk neuroblastoma therapy at international site outside of North America.
- Present research at AACR, ASPHO, SNO, SIOP, APHON and more!
- Further expand our Precision Medicine Program, including international expansion of Molecular Tumor Boards, new cutting edge trials, microbiome work, immune microenvironment, historical control database, imaging and proteomics.
- Complete safety run-in for BCC022 trial utilizing tipifarnib in combination with naxitamab for relapsed/refractory neuroblastoma.
- Publications highlighting study results and secondary and exploratory trial endpoints.
- Continue to expand our preclinical work for neuroblastoma, sarcomas and central nervous system tumors.
- Evaluate and open a pilot trial concept for removing transplant from upfront HRNB therapy.



2025 | ACADEMIC POSTERS AND ORAL PRESENTATIONS

American Association for Cancer Research (AACR)

Targeted therapy with DFMO and immunotherapy equalizes survival in MYCN amplified neuroblastoma: insights from genomic and network analyses

Association of Pediatric Hematology/Oncology Nurses (APHON)

Safety Comparison of Two Different Dosing Regimens for Oral Eflornithine (DFMO) In Pediatric Solid Tumors

American Society of Pediatric Hematology/Oncology (ASPHO)

Safety of Naxitamab+GM-CSF in Combination with Induction Chemotherapy in High-Risk Neuroblastoma

Safety Comparison of Two Different Dosing Regimens for Oral Eflornithine in Pediatric Solid Tumors

Survival Outcome in Patients with HRNB in Remission 7 Years After Starting DFMO Maintenance Therapy

Advancing Neuroblastoma Research (ANR)

End-of-Induction Response as a Surrogate Endpoint for Overall Survival in High-Risk Neuroblastoma

Safety Comparison of Two Different Dosing Regimens for Oral Eflornithine (DFMO) In Pediatric Solid Tumors

Oral: Survival Outcomes in Patients with High-Risk Neuroblastoma on Eflornithine (DFMO) Maintenance: Matched External Control Analysis at 7-Year Follow-Up

Safety of Naxitamab + Granulocyte Macrophage Colony Stimulating (GM-CSF) in Combination with Induction Chemotherapy in High-Risk Neuroblastoma

Safety of ALK Inhibitors in Combination with Eflornithine (DFMO) in High-Risk Neuroblastoma

International Society of Paediatric Oncology (SIOP)

Oral: Novel Combination Therapy Targeting Polyamine Pathway in Neuroblastoma

Oral: Pediatric Consortium Development and Oversight of an International Molecular Tumor Board

Oral: Anti-tumor activity of CK2 inhibitor, CX-4945 in metastatic models of Ewing Sarcoma

Oral: DFMO and Venetoclax Combination Therapy: Targeting Senescent Neuroblastoma Cells to Induce Cell Death

Safety of ALK Inhibitors in Combination with Eflornithine (DFMO) in High-Risk Neuroblastoma (HRNB)

Impact of Eflornithine Maintenance Therapy on Survival in High Risk Neuroblastoma Patients from China, India and Brazil

Safety of Naxitamab+GM-CSF in Combination with Induction Chemotherapy in High-Risk Neuroblastoma

Advancing Precision Medicine (APM)

Oral: Hosted an Expert Panel titled: "Transforming Pediatric Oncology: The Power of Precision Medicine"

Society for Neuro-Oncology (SNO) Pediatric Conference

A case series targeting LIN28 in Embryonal Tumor with Multilayered Rosettes (ETMR) using eflornithine (DFMO)

