



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



## Beat Childhood Cancer Research Consortium opens two brand new trials in 2024

The Research Consortium successfully activated two new trials in 2024 to translate preclinical research directly to the patient, BCC021 and BCC022. BCC021 is a phase I/II study of silmitasertib (CX-4945) in combination with chemotherapy in children and young adults with relapsed refractory neuroblastoma and sarcomas. BCC022 is a phase II trial of tipifarnib and naxitamab for relapsed refractory neuroblastoma. Both trials have enrolled their first patient as of Fall 2024 and are actively opening at hospital sites across the Consortium. 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 and Collaboration with Resonance Health Announced

We're thrilled to announce that the Beat Childhood Cancer Research Consortium, in collaboration with Penn State, has finalized an exciting agreement with Resonance Health. Resonance Health will serve as our International Contract Research Organization, overseeing select BCC trials outside of North America.

The first international partner to join BCC will be Hospital Sant Joan de Déu in Barcelona, Spain, under the leadership of Dr. Jaume Mora. We anticipate that our collaborators in Sao Paulo, Brazil, will soon join as the second country in this expansion. This partnership marks a significant step forward in our mission to combat childhood cancer on a global scale. This collaboration will enable us to leverage diverse expertise and resources, accelerating our efforts to develop innovative treatments and improve outcomes. 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!*

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## 2024 | BY THE NUMBERS

**178** Patients enrolled on treatment studies across 9 clinical trials



Patients enrolled on BCC-BIO-001 biology clinical trial **151**

**54** Cell lines established by POTR Lab



Number of patient samples that came to the POTR Lab in 2024 **1,966**

**50** Molecular Tumor Boards held for patients in 2024



## 2024 | PUBLICATIONS

### BCC publications

**Genome Medicine** – Molecular-guided therapy for the treatment of patients with relapsed and refractory childhood cancers: a Beat Childhood Cancer Research Consortium trial

**Cancer Medicine** – DFMO inhibition of neuroblastoma tumorigenesis

**Cancers** – Panobinostat synergizes with chemotherapeutic agents and improves efficacy of standard of care chemotherapy combinations in Ewing Sarcoma cells

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### A MESSAGE FROM OUR FOUNDER

*We welcome everyone to our 17<sup>th</sup> BCC Annual Consortium Meeting!*

As we reflect on the past year, I am filled with immense pride and gratitude for the incredible strides we have made in our mission to bring hope to families facing the challenges of childhood cancer. Our commitment to pioneering research and identifying innovative therapeutic options, particularly in the realm of precision medicine, has been unwavering. The heart of our success lies in the collaborative spirit that defines the Beat Childhood Cancer family.

Physicians, researchers, nurses, parents, advocates, foundations and partners in the pharmaceutical and biotech industries have come together, united by a singular goal: to enhance the chances of survival for children with cancer through the development of safer and less toxic therapies. Our consortium is not just an alliance of professionals; it's a community fueled by compassion, resilience and a relentless pursuit of better outcomes for young lives.



### Several important goals of the Beat Childhood Cancer Research Consortium will guide our collective work in 2025 and beyond:

- Open trial combining 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).
- Complete Phase 1 portion of the trial utilizing Casein kinase 2 (CK2) inhibitor for relapsed/refractory neuroblastoma and sarcomas.
- Open trial combining DFMO with standard of care therapies for Ewings sarcoma and osteosarcoma.
- Publish more case reports and secondary/exploratory trial endpoints.
- Present abstracts at AACR, ASPHO, SNO, SIOP, APHON and more!
- Develop a Phase I trial using IL13R $\alpha$ 2-Targeting Immunotoxin GB13.
- Further expand our Precision Medicine Program. Including but not limited to new cutting edge trials, utilizing artificial intelligence to improve predictions, epigenetics and proteomics.
- Continue to expand our preclinical work for neuroblastoma, sarcomas and central nervous system tumors.
- Expand upon our precision immunotherapy program to include projects with mRNA vaccines, CART approaches, etc.
- Expansion of our BCC018 upfront Neuroblastoma Trial incorporating Naxitamab during induction across BCC sites internationally.



### 2024 | ACADEMIC POSTERS AND ORAL PRESENTATIONS

#### American Association for Cancer Research (AACR)

Casein Kinase II (CK2) Expression in Pediatric Solid Tumors

#### American Society of Clinical Oncology (ASCO)

Survival Outcomes in High-Risk Neuroblastoma (HRNB) Patients in Remission after Relapsed or Refractory Treatment Receiving Eflornithine (DFMO) Maintenance: A Beat Childhood Cancer Research Consortium Trial.

#### Association of Pediatric Hematology/Oncology Nurses (APHON)

Clinical Administration DFMO (Eflornithine) is Flexible in Pediatric of Patients with HRNB

#### American Society of Pediatric Hematology/Oncology (ASPHO)

Assessment of Household Poverty Exposure in Patients with HRNB Receiving DFMO Maintenance Treatment

End of Induction Response Correlates with Clinical Outcome in High-Risk Neuroblastoma Patients

#### Advancing Precision Medicine (APM)

Oral: Unlocking the Next Quantum Leap in Precision Medicine – A Town Hall Discussion

#### International Society of Paediatric Oncology (SIOP)

Oral: Survival Outcomes in Patients with High-Risk Neuroblastoma (HRNB) in Remission after Relapsed or Refractory Treatment Receiving Eflornithine (DFMO) Maintenance

Oral: IWILFIN – An Oral Maintenance Therapy for High-Risk Neuroblastoma

Oral: Meet the Expert Session: Tumor Board – Metastatic Neuroblastoma

Survival Outcomes in Patients with High-Risk Neuroblastoma (HRNB) Receiving Eflornithine (DFMO) Maintenance Treatment with Matched External Controls: Subgroup Analysis on Geographic Region

Precision Medicine for Patient with Medulloblastoma Identifies Targeted Therapy

Targeting HDAC2 in Ewing sarcoma with Romidepsin Synergizes with Chemotherapy in vitro and in vivo



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