

Learn more about the Beat Childhood Cancer Research Consortium by scanning the QR code shown to the right with your smartphone camera:



## PennState College of Medicine

### Beat Childhood Cancer Research Consortium relocates to Penn State College of Medicine

The Research Consortium has moved to Hershey, Pennsylvania! In September, 2023, the Research Consortium was welcomed to its new home—Penn State College of Medicine. Dr. Sholler took on new roles, as the director of pediatric oncology research at the College of Medicine, and division chief of pediatric hematology/oncology at Penn State Health Children's Hospital. The value of housing the consortium within an academic medical center is unmatched! Here, we can combine the pillars of research and care to advance knowledge, drive medical discoveries and change lives!

We have already started leveraging the amazing work from preclinical laboratories at Penn State to translate cutting-edge research into clinical trials for patients in 2024!



### Beat Childhood Cancer Research Consortium research leads to approval from U.S. Food and Drug Administration

#### DFMO/Eflornithine (now iwilfin) approved

Eflornithine (iwilfin) has been granted FDA approval to reduce the risk of relapse in adult and pediatric patients with high-risk neuroblastoma (HRNB) who have had at least a partial response to prior multiagent, multimodality therapy including anti-GD2 immunotherapy. This regulatory decision was based on findings from a controlled trial comparing outcomes from Study 3b (NCT02395666; investigational arm) and Study ANBL0032 (clinical trial-derived external control arm). Previously, in October, 2023, the FDA's Oncologic Drug Advisory Committee (ODAC) voted 14 to 6 that eflornithine shows sufficient evidence to reduce the risk of relapse in pediatric patients with HRNB who are in remission and have completed multi-agent, multi-modality therapy.

**“**  
*We are so excited for patients across the country to have access to this medication and want to thank all the researchers, sites and collaborators that helped make this amazing accomplishment a reality!*  
**”**

## 2023 | BY THE NUMBERS

**120**

Patients enrolled on treatment studies across 7 clinical trials



Patients enrolled on BCC-BIO-001 biology clinical trial

**120**

**50**

Cell lines established by POTR Lab



Number of patient samples that came to the POTR Lab in 2023

**725**



Passed FDA inspections at the Sponsor, Site and Vendor Levels



## 2023 | PUBLICATIONS

### BCC publications

Journal of Clinical Oncology - Eflornithine as Postimmunotherapy Maintenance in High-Risk Neuroblastoma: Externally Controlled, Propensity Score-Matched Survival Outcome Comparisons  
Cancer Therapy and Prevention - A phase II trial of nifurtimox combined with topotecan and cyclophosphamide for refractory or relapsed neuroblastoma and medulloblastoma  
DFMO inhibition of Neuroblastoma Tumorigenesis  
Molecular-guided therapy for the treatment of patients with relapsed and refractory childhood cancers: A Beat Childhood Cancer Research Consortium Trial

WE ARE PENN STATE

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### A MESSAGE FROM OUR FOUNDER

*We welcome everyone to our 16th 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.



Above: Dr. Giselle Saulnier Sholler and team in her lab at Penn State College of Medicine



**Several important goals of the Beat Childhood Cancer Research Consortium will guide our collective work in 2024 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).
- Open trial utilizing Casein kinase 2 (CK2) inhibitor for relapsed/refractory neuroblastoma, sarcomas and other solid tumors.
- Open trial combining Tipifarnib + naxitamab for relapsed/refractory neuroblastoma.
- Present abstracts at AACR, ASPHO, SIOP, APHON and more!
- Continue to expand our Precision Medicine Program.
- Continue to expand our preclinical work for neuroblastoma, sarcomas and central nervous system tumors to include four mouse studies in 2024 in addition to many in vitro experiments.
- Expansion of our BCC018 upfront Neuroblastoma Trial incorporating Naxitamab during induction across BCC sites.



### 2023 | PRESENTATIONS

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

Molecular-guided therapy for the treatment of relapsed neuroblastoma: A Beat Childhood Cancer Research Consortium Trial  
Comparative validation of neuroblastoma cell lines using flow cytometry and CyToF  
HDAC inhibition increases Ewing sarcoma sensitivity to chemotherapy  
The anti-tumor effects of GSK-3B inhibitor (9-ING-41) in ETMR and ATRT pediatric brain tumors

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

EFS and OS in Study 3b Patients with High-Risk Neuroblastoma Receiving Eflornithine (DFMO) Maintenance Treatment with Matched External Controls

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

Sensitivity Analysis of Event Free and Overall Survival in High-Risk Neuroblastoma Patients Receiving DFMO Maintenance Treatment with Matched External Controls Including MYCN

Tolerability and Safety of Eflornithine (DFMO) in High-Risk Neuroblastoma Patients Treated with DFMO Maintenance Therapy  
Mice Sensitivity Analysis of Event Free Survival in High-Risk Neuroblastoma Patients Receiving Eflornithine (DFMO) Maintenance Treatment with Matched External Controls

#### Advances in Neuroblastoma Research (ANR)

Molecular-Guided Therapy for the Treatment of Relapsed/Refractory Neuroblastoma: Results from Beat Childhood Cancer Research Consortium Trial MGT009  
Tolerability and Safety of Eflornithine (DFMO) in High-Risk Neuroblastoma (HRNB) Patients Treated with DFMO Maintenance Therapy  
Subset Analysis of the role of MYCN in DFMO treated patients  
Sensitivity Analyses of Event Free and Overall Survival in High-Risk Neuroblastoma Patients Receiving Eflornithine (DFMO) Maintenance Treatment with Matched External Controls



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