

# ANNUAL REPORT

MISSION, NEWS, PROJECTS, PARTNERS, & TRIALS



NATIONAL  
PEDIATRIC CANCER  
FOUNDATION®



**Highest Rated  
National Health  
Organization**



[www.NationalPCF.org](http://www.NationalPCF.org)

EIN # 59-3097333 CFC # 43259



Dear Friends,

The National Pediatric Cancer Foundation® is headquartered in Tampa, Florida, and our mission is dedicated to funding research to eliminate pediatric cancer. According to the watch group, Charity Navigator, we are one of the highest-rated cancer health charities in the country.

We govern a unique research consortium (called the Sunshine Research Project™) consisting of physicians and scientists from 34 of the top hospitals in the nation. Our model is highly efficient, nimble, and based on quality, outcome-based research.

For this reason, we ask you to join our effort. The fastest way to better therapies is to leverage NPCF™'s infrastructure to review, fund, and conduct research and transition the promising research to clinical trials without unacceptable roadblocks. Children with cancer need these new therapies immediately.



David Frazer, CEO  
National Pediatric Cancer Foundation®

## SUNSHINE RESEARCH PROJECT IMPACT

- The leading research consortium with national reach addressing multiple types of high-risk pediatric cancers because this problem is greater than just one hospital.
- Top doctors and scientists in 34 pediatric cancer research institutions collaborating to advance the best ideas and science.
- Fast, nimble, non-bureaucratic, out-of-the box, streamlined research with focused funding.
- National network of patient accruals versus one market/region.
- No pharmaceutical or government funding.
- One of the highest-rated cancer charities in the nation according to Charity Navigator.
- \$37 million in research funding over the past 11 years with success in multiple drug combinations therapies & identification.
- 16 current clinical trials and 30+ translational studies underway.



## OUR PARTNER INSTITUTIONS



- Atrium Health Levine Cancer Institute Charlotte, NC
- Children's Healthcare of Atlanta Atlanta, GA
- Children's Hospital Colorado Aurora, CO
- Children's Hospital of Los Angeles Los Angeles, CA
- Children's Hospital of Philadelphia Philadelphia, PA
- Children's National Medical Center Washington D.C.
- Cleveland Clinic Children's Hospital Cleveland, OH
- Connecticut Children's Medical Center Hartford, CT
- Dana-Farber Cancer Institute Boston, MA
- David Geffen School of Medicine at UCLA Los Angeles, CA
- Duke Children's Hospital Durham, NC
- Intermountain Primary Children's Hospital Salt Lake City, UT
- Johns Hopkins All Children's Hospital St. Petersburg, FL
- Johns Hopkins Sidney Kimmel Comprehensive Cancer Center Baltimore, MD
- MD Anderson Cancer Center Houston, TX
- Moffitt Cancer Center Tampa, FL
- Montefiore Medical Center Bronx, NY
- Nationwide Children's Hospital Columbus, OH
- Nemours Children's Health Jacksonville, FL
- Nemours Children's Hospital Wilmington, DE
- Nemours Children's Hospital Orlando, FL
- Phoenix Children's Hospital Phoenix, AZ
- Roswell Park Comprehensive Cancer Center Buffalo, NY
- St Joseph's Children's Hospital Tampa, FL
- St. Louis Children's Hospital St. Louis, MO
- Sylvester Comprehensive Cancer Center Miami, FL
- UAB Medicine Birmingham, AL
- UK Healthcare Lexington, KY
- UNC Lineberger Comprehensive Cancer Center Chapel Hill, NC
- University of Chicago Chicago, IL
- University of Florida/UF Health Shands Hospital Gainesville, FL
- UT Southwestern Medical Center Dallas, TX
- Vanderbilt University Medical Center Nashville, TN
- Weill-Cornell Medicine New York City, NY

## RESEARCH PROJECTS AND TRIALS

### **1. Phase II Study of nab-Paclitaxel in Combination with Gemcitabine for Treatment of Recurrent/Refractory Sarcoma in Teenagers and Young Adults**

This trial, led by Levine Children's Medical Center in North Carolina, will look at this combination of nab-paclitaxel and gemcitabine in its ability to prevent the formation or growth of tumors in teenagers and young adults with: relapsed or refractory osteosarcoma, Ewing sarcoma, rhabdomyosarcoma, and other soft tissue sarcoma. The trial will also look at the length of time during and after treatment that the disease does not get worse and determine if nab-paclitaxel combined with gemcitabine is safe and tolerable.

### **2. A Phase Ib/II Drug Combination Study to Evaluate the Safety with Azacitidine in Patients with Recurrent, Resectable Osteosarcoma**

This trial is led by the University of North Carolina's Children's Hospital to treat Osteosarcoma (BMS).

### **3. Evolutionary-inspired therapy for newly diagnosed, metastatic, Fusion Positive Rhabdomyosarcoma**

This trial, led by Moffitt Cancer Center, examines metastatic, fusion-positive rhabdomyosarcoma (RMS). Metastatic fusion-positive rhabdomyosarcoma (RMS) has a poor outcome, which is worsened with additional risk factors commonly called the Oberlin criteria. Patients that meet all 4 Oberlin criteria have an Event Free Survival (EFS) of less than 20% at 2 years. All therapeutic arms in this study are designed to meet the same primary aim of improving the 3-year EFS from 6% to 35% for these patients.

### **4. Phase 1 trial of the Isd1 inhibitor sp-2577 in patients with relapsed or refractory Ewing sarcoma**

This trial, led by Moffitt Cancer Center, is a targeted treatment for individuals diagnosed with refractory or recurrent Ewing sarcoma—an aggressive, small, round, blue-cell tumor—typically presenting as a primary bone tumor in children and young adults.



## RESEARCH PROJECTS AND TRIALS

### **5. TiNKS: A Multi-Institution Immunology Study of TGF $\beta$ imprinted, *Ex Vivo* Expanded Universal Donor NK Cell Infusions as Adoptive Immunotherapy in Combination with Gemcitabine and Docetaxel in Patients with Relapsed or Refractory Pediatric Bone and Soft Tissue Sarcomas**

This trial, led by Nationwide Children's Hospital, determines the safety of the addition of adoptive transfer of universal donor, TGF $\beta$  imprinted (TGF $\beta$ i), and expanded NK cells to gemcitabine/docetaxel (GEM/DOX) for treatment of relapsed and refractory sarcomas.

### **6. METTSEO: Metastatic Ewing's Trial Testing Schedule Enhancement to Improve Outcomes**

This trial will test our ability to administer frequently changing chemotherapy regimens, called sequential second strikes, to patients with widely metastatic Ewing sarcoma in order to stop development of resistance to chemotherapy and improve cure rates.

### **7. pedsETB: Feasibility of Generating Novel Translational and Therapeutic Strategies based on a Multicenter, Pediatric and AYA Evolutionary Tumor Board**

Pediatric Evolutionary Tumor Board (pedsETB) is a multidisciplinary forum to approach an individual patient's cancer and propose additional ideas for care. The pedsETB will gather together disciplines not often engaged in cancer work and use insights from evolution to optimally model, research, and impact pediatric cancer outcomes. Strategies from pedsETB's predecessor have been instrumental in the design of several of NPCF's most recent clinical trials.

### **8. A Phase I/II Study of RNA-lipid particle (RNA-LP) vaccines for recurrent pulmonary osteosarcoma**

In this study, we will investigate the safety and immunologic activity of RNA-LP vaccines in patients with recurrent pulmonary osteosarcoma.

## RESEARCH PROJECTS AND TRIALS

### **9. ACTION: A study of Adoptive Cellular Therapy following Dose-Intensified Temozolomide in Newly-diagnosed Pediatric High-grade Gliomas**

This trial, led by the University of Florida, examines an innovative immunotherapy for the treatment of high-grade gliomas (HGG) in children.

### **10. Evaluation of Digoxin for Relapsed Non-Wingless Activated (WNT), Non-SHH Medulloblastoma**

This trial will evaluate the efficacy of digoxin in treating patients with relapsed non-SHH, non-WNT medulloblastoma. Medulloblastoma (MB) is the most common pediatric brain tumor, with a culminating incidence among children before the age of five. Unfortunately, disease dissemination is an early event, and as many as 40% of patients carry metastases already at diagnosis with a grim outlook for survival.

### **11. Blood-based biomarkers for minimal residual disease detection in pediatric sarcomas**

The purpose of this study is to see if detecting cell-free plasma tumor DNA (ptDNA) and circulating tumor cells (CTC) can predict recurrence of disease in patients who are in radiographic remission 2-3 weeks after treatment. Plasma tumor DNA (ptDNA) is free floating DNA from the tumor found in the bloodstream and circulating tumor cells.

### **12. Role of myeloid-derived suppressor cells (MDSC) in the development of immune tolerance after allogeneic hematopoietic cell transplantation (alloHCT)**

This is an observational trial with the goal of better understanding the process of developing immune tolerance after blood and marrow transplantation (BMT).

## RESEARCH PROJECTS AND TRIALS

### 13. Pediatric Total Cancer Care

This trial focuses on tissue and blood collection to further personalize medicine for children with cancer and includes:

- Comprehensive molecular profiling of rare pediatric and AYA cancers.
- Development of an infrastructure, methods, and standard operating procedure to collect and procure histology-specific (esthesioneuroblastoma and embryonal sarcoma) tissue resources available throughout the Sunshine Project and associated repositories.
- Comprehensive genetic profiling for pediatric malignancies.
- Assessment of Expanded Tumor Infiltrating NK-Cells Collaborative.
- Development of personalized RNA Loaded Nanoparticles.
- Fusion Proteins by Immunotherapy.

## 43 CHALLENGE DOCTORS

### 1. Pediatric Oncologist, Dr. Dan Weiser from Montefiore Einstein Cancer Center / Children's Hospital at Montefiore

Dr. Weiser's science has identified a novel technology that can control and degrade a specific c-MYC gene (known as the master regulator) across many types of cancer. Since c-MYC is detected in 74% of human cancers, the ability to degrade and attack this common link has the potential to transform the treatment of cancer.

### 2. Dr. Keisuke Iwamoto from David Geffen School of Medicine at UCLA

Dr. Iwamoto is a renowned expert on radiation and its use in cancer treatment. Using his studies into the radiological effects of nuclear bombs on survivors during WWII, he will use quantum mechanics to precisely target specific tumor cells without harming normal cells, revolutionizing cancer treatment. The method uses conventional clinical procedures in a novel combination within a weak magnetic field, akin to a refrigerator magnet's, such that if successful it could be introduced into any hospital without major infrastructural, financial, or procedural difficulty to treat children and even infants with medulloblastoma, the most common childhood brain cancer.

### 3. Dr. Gregory Sullivan from Paratope Bio, LLC

The work of Dr. Sullivan and Paratope Bio, LLC will leapfrog traditional drug development to produce a smart set of innate antibodies that have limited toxicity and would specifically attack pediatric tumors.



## SUNSHINE PROJECT™ LABORATORY

- The Sunshine Project™ Lab is a system to take the many medications used in the treatment of adult cancers and determine their activity in models of pediatric cancers at levels that we know are safe for child patients.
- We evaluate the best medications in combinations to further increase the chances of having meaningful effects for patients.
- The lab takes potential combinations and determines the top two or three that should be further explored.
- This lab aims for the best strategy to eliminate both cancer cell populations with timing and combinations of therapies.
- Efforts in the Sunshine Project™ Lab are conducted by research physicians and doctors at Moffitt Cancer Center.
- During this past year, the Sunshine Lab has focused on Osteosarcoma heterogeneity by varying the starting amounts of 2 different cell lines, SAOS2 and 143B, grown in spheroids and have attached the proof of the submitted manuscript for the above work.
- We are investigating “second strikes” in both Osteosarcoma and Ewing sarcoma. Second strikes are therapies after the disease has shrunk with initial therapy. Rather than shrink the tumor, we are investigating therapies to eliminate the residual cells better than continuing the initial therapy (first strike). This resulted from the Sunshine Project™ Lab participating in the 9th annual Integrated Mathematical Oncology Workshop at Moffitt Cancer Center.
- We have focused on a new, non-mutated target in Osteosarcoma, the CMG helicase. We have both identified this as a weakness in cancer cells more than normal cells and identified a drug class that holds promise as an eventual therapy. We are exploring this agent alone and in combination to maximize the chance for a successful clinical trial. Early work on the mechanism is submitted and under review in Nature Medicine.
- We are building on prior publications showing the activity of epigenetic drugs like Panobinostat and exploring mechanisms to enhance this therapy in Osteosarcoma.
- We are investigating an underappreciated DNA repair enzyme as an Achilles heel in Ewing sarcoma called PARP16.

## IN FISCAL YEAR 2022 - 2023

Financial Highlights for fiscal year 2022/2023:

- Public Support and Revenue (Net of event direct expenses) = **\$5,175,753**
- Ending Net Assets = **\$5,813,538**
- Program Expenses = **\$4,360,477**
- Management & General = **\$367,965**
- Development & Community Support = **\$222,633**
- Total Operational Expenses = **\$4,951,075**

Specific details & additional accomplishments are provided on our website, to include:

- IRS form 990
- Annual financial statements (income & expense)
- Listing of activities/accomplishments

Our Board Members with service this period: BB Abbott, James Bassil, David Boyington, Frank Capitano, Chris Carrere, Carrie Charles, Dan Doyle Jr., Melissa Dunkel, John Fitzpatrick, Chad Harrod, Ricky Huff, Joseph Lamphier, Jay Langford, Michael Levin, Jeff Maxwell, Mark McHugh, Phillip Minardi, Angela North, Jeremy Persinger, Marco Schnabl, Dawn Siler-Nixon, Al Silva, Alex Sullivan, Joe Taggart, and Michael Weigner.



## NATIONAL PEDIATRIC CANCER FOUNDATION® MISSION

The National Pediatric Cancer Foundation is dedicated to funding research to eliminate childhood cancer. Our focus is to find less toxic, more effective treatments through a unique collaborative research initiative called the Sunshine Project.

**88¢ of every dollar we spend directly supports our mission.** We have been given the highest ratings a nonprofit can receive for financial transparency and accountability.



### NPCF™ RATINGS

Donors can be confident their dollars are spent effectively, as the NPCF has received a perfect 4-star, 100% rating for financial health and transparency.





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NationalPCF.org

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