

ANNUAL REPORT

MISSION, NEWS, PROJECTS, PARTNERS, & TRIALS



NATIONAL
PEDIATRIC CANCER
FOUNDATION



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 the highest-rated cancer health charity in the country.

We govern a unique research consortium (called the Sunshine Research Project) consisting of physicians and scientists from 32 of the top hospitals in the nation. Our model is highly efficient, nimble, and based on quality, outcome-based research. This year we challenged scientists to compete for \$4.3 million. As a result we received 25 unique and novel proposals for future funding.

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.



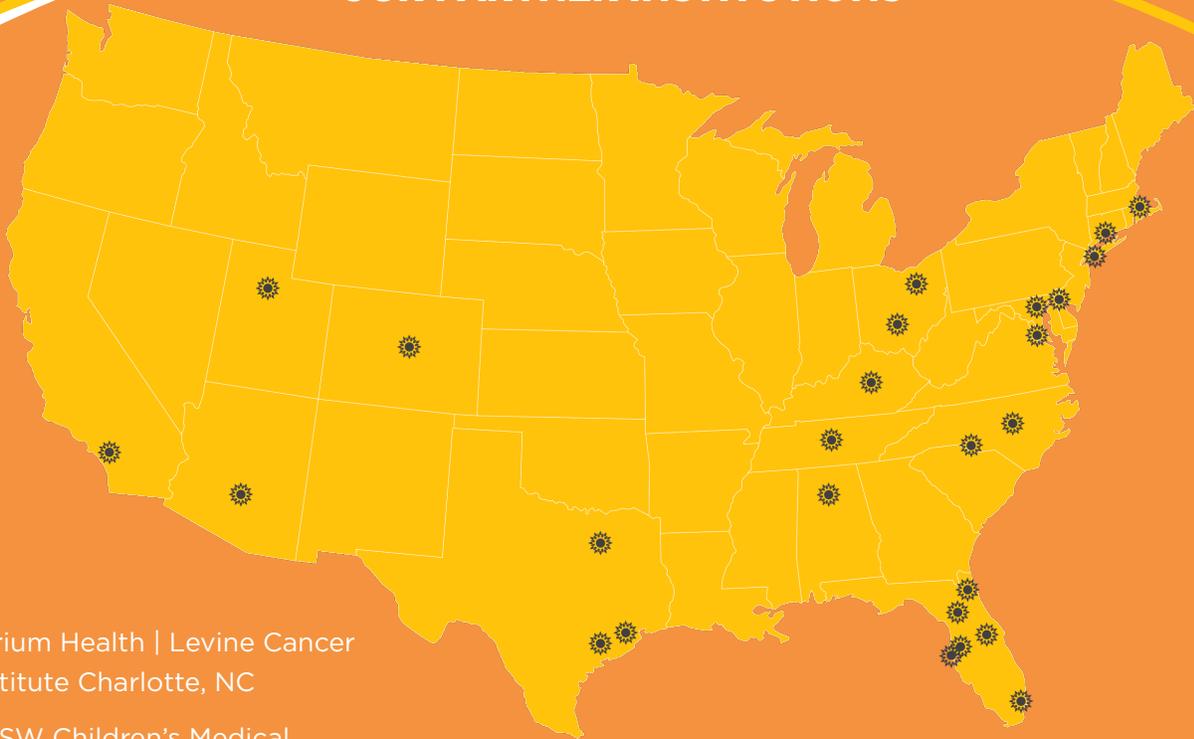
David Frazer, CEO
National Pediatric Cancer Foundation

SUNSHINE RESEARCH PROJECT IMPACT

- The leading research consortium with national reach addresses multiple types of high-risk pediatric cancers versus an organization focused on one institution or type of cancer.
- 100+ top doctors and scientists in 32 pediatric cancer research institutes collaborating to advance the best ideas and science.
- Fast, nimble, non-bureaucratic, out-of-the box, rapid development and streamlined research with focused funding.
- Large national network of patient accruals versus one market/region.
- No pharmaceutical or government funding.
- Highest rated cancer charity in the nation according to Charity Navigator.
- \$35 million in research funding over the past 11 years with success in multiple drug combinations therapies & identification.
- Seven current clinical trials and 30 translational studies underway.



OUR PARTNER INSTITUTIONS



- Atrium Health | Levine Cancer Institute Charlotte, NC
- UTSW Children's Medical Center Dallas, TX
- Children's Healthcare of Atlanta Atlanta, GA
- Children's Hospital of 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.
- Children's of Alabama Birmingham, AL
- Cleveland Children's Clinic Cleveland, OH
- Connecticut Children's Medical Center Hartford, CT
- Dana-Farber Cancer Institute Boston, MA
- Duke University Health System/ Duke Cancer Institute Durham, NC
- Johns Hopkins All Children's Hospital St. Petersburg, FL
- Johns Hopkins Sidney Kimmel Comprehensive Cancer Center Baltimore, MD
- Moffitt Cancer Center Tampa, FL
- Montefiore Medical Center Bronx, NY
- Nationwide Children's Hospital Columbus, OH
- Nemours Children's Hospital Orlando, FL
- Nemours Children's Clinic Jacksonville, FL
- Nemours/A.I. duPont Hospital for Children Wilmington, DE
- Phoenix Children's Hospital Phoenix, AZ
- Primary Children's Medical Center Salt Lake City, UT
- Rosewell Park Comprehensive Cancer Center Buffalo, NY
- St Joseph's Hospital Tampa, FL
- St Louis Children's Hospital St. Louis, MO
- Sylvester Comprehensive Cancer Center Miami, FL
- UK Healthcare | Kentucky Children's Hospital Lexington, KY
- UNC Lineberger Comprehensive Cancer Center Chapel Hill, NC
- University of Chicago Chicago, IL
- University of Florida/UF Health Shands Hospital Gainesville, FL
- Vanderbilt-Ingram Cancer Center Nashville, TN
- Weill-Cornell Medicine New York City, NY

SARCOMA TRIALS

(Osteosarcoma, Rhabdomyosarcoma, Ewing Sarcoma, non-rhabdomyosarcoma)

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

This \$300,000 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 \$300,000 trial led by the University of North Carolina's Children's Hospital combination to treat Osteosarcoma.

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

This \$1,000,000 trial led by Dr. Damon Reed, Moffitt Cancer Center examines metastatic, fusion positive rhabdomyosarcoma (RMS). Metastatic, fusion positive rhabdomyosarcoma (RMS) have 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 two years. All therapeutic arms on this study are designed to meet the same primary aim of improving the 3-year event free survival 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 \$1,000,000 valued trial led by Dr. Damon Reed (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.

IMMUNOLOGY TRIALS

(Osteosarcoma, Rhabdomyosarcoma, Ewing Sarcoma, non-rhabdomyosarcoma)

1. TiNKS: A Multi-Institution Immunology Study of TGF β 1 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 Sarcoma

This \$1,000,000 trial led by Dr. Bhuvana Setty, Nationwide Children's Hospital determines the safety of the addition of adoptive transfer of universal donor, TGF β 1 imprinted (TGF β 1), expanded NK cells to gemcitabine/docetaxel (GEM/DOX) for treatment of relapsed and refractory sarcomas.

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

This \$1,000,000 trial led by Dr. Duane Mitchell, University of Florida examines an innovative immunotherapy for the treatment of high-grade gliomas (HGG). An innovative immunotherapy trial valued at \$1,000,000 for the treatment of high-grade gliomas (HGG) in children.

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

This \$300,000 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.

NON-TREATMENT TRIALS

1. 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).

2. Blood based biomarkers for minimal residual disease detection in pediatric sarcomas

The purpose of this \$1,000,000 study is to see if detecting cell-free plasma tumor DNA (ptDNA) and circulating tumor cells (CTC) can predict the 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.

3. Pediatric Total Cancer Care

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

- Comprehensive molecular profiling of rare pediatric and AYA cancers
Development of 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



SUNSHINE PROJECT LABORATORY

- The Sunshine Lab is a system to take the many medications being used in adult cancers and determine their activity in models of pediatric cancers at levels that we know are safe in 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 to figure out 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.
- We have developed a model of these two competing populations, termed heterogeneity, to figure out the best strategy to eliminate both cancer cell populations with timing and combinations of therapies.
- Collaborating with Drs. Andriy Marusyk and Joel Brown, 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 lab participating in the 9th annual Integrated Mathematical Oncology Workshop at Moffitt Cancer Center.
- Collaborating with Dr. Mark Alexandrow 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.
- Collaborating with Dr. Conor Lynch, we are building on prior publications showing the activity of epigenetic drugs like Panobinostat and exploring mechanisms to enhance this therapy in osteosarcoma.
- Collaborating with Dr. Uwe Rix, we are investigating an underappreciated DNA repair enzyme as an Achilles heel in Ewing sarcoma called PARP16.

IN FISCAL YEAR 2021 - 2022

Made an impact.

Financial Highlights for fiscal year 2021/2022:

Total (in rounded thousands):

- Total Income in past FY = **\$5,203,000**
- Ending Net Assests = **\$5,604,000**
- Program Expenses = **\$3,803,000**
- Management & General = **\$308,000**
- Development & Comunity Support = **\$187,000**
- Total Operational Expenses = **\$4,298,000**

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, Frank Capitano, Chris Carrere, Carrie Charles, Dan Doyle Jr., Melissa Dunkel, John Fitzpatrick, Thomas Grossjung, Chad Harrod, Ricky Huff, Joseph Lamphier, Jay Langford, Michael Leven, Jeff Maxwell, Phillip Minardi, Angela North, Jeremy Persinger, 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.

89¢ 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 rating for financial health and transparency for the 11th consecutive year. The NPCF is the top rated childhood cancer related nonprofit for this distinction.





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