

FISCAL YEAR 2020-2021

ANNUAL REPORT

MISSION, NEWS, PROJECTS, PARTNERS, & TRIALS



NATIONAL
PEDIATRIC CANCER
FOUNDATION



www.NationalPCF.org

EIN # 59-3097333 CFC # 43259

OUR MISSION

We are dedicated to **raising awareness** and **funds** to research to find a faster cure for pediatric cancer.

This past year, the National Pediatric Cancer Foundation continued aggressively pursuing its mission with robust passion and drive.

We continued to scale our collaborative research and outreach programs nationwide through strategic partnerships, philanthropic, and corporate support.

We are incredibly grateful for the generous donors, volunteers, and partners that helped establish and grow multiple success stories.





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 thirty of the top hospitals in the nation. We collaborate to idealize and aggregate the best scientific ideas and fund innovative research to expedite promising treatment options into clinical trials. 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.



A handwritten signature in black ink, reading "David Frazer".

David Frazer, CEO
National Pediatric Cancer Foundation



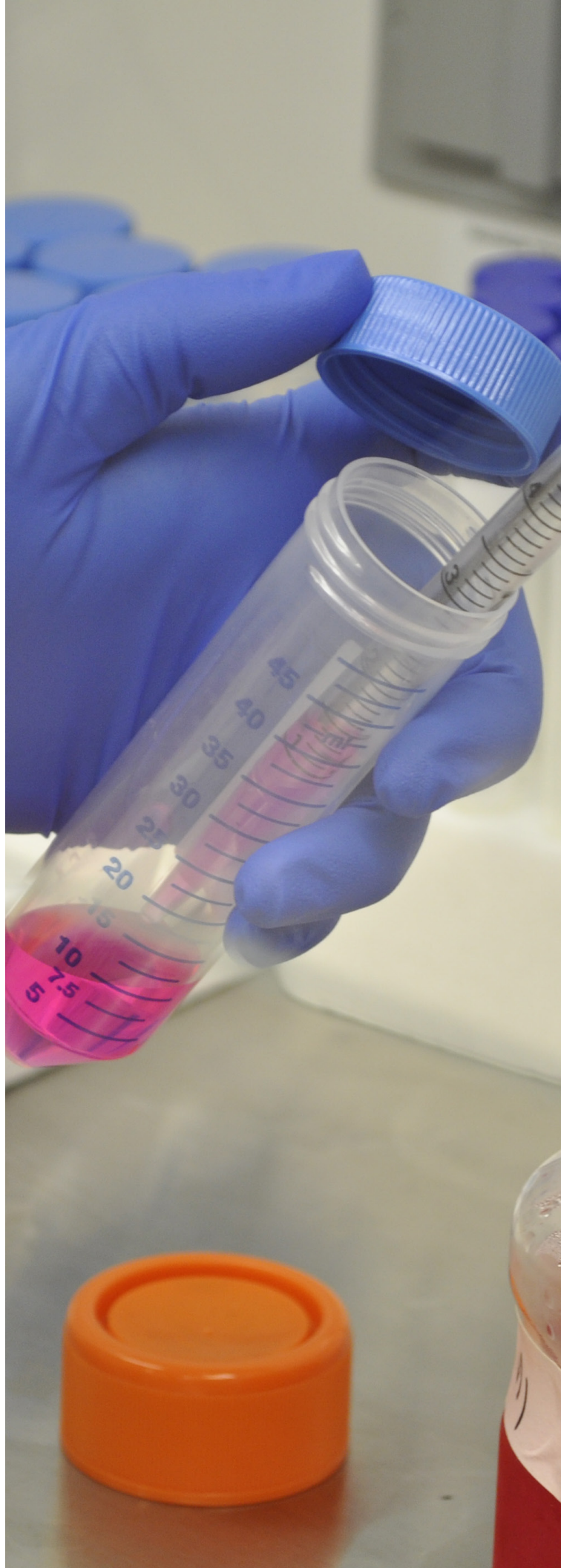
Elias Sayour, MD, PhD
Sunshine Project Site PI
Pediatric Hematology-Oncology
University of Florida

“For a disease like pediatric cancer, no single institution can see enough patients to create a real, tangible difference or impact. We need several institutions working hand in hand with each other. When I see the Sunshine Project, it’s inspiring and makes me feel a part of something much greater.”



Eric Sandler,
Sunshine Project Site PI,
Nemours Children’s Specialty Care, Jacksonville

“The Sunshine Project means the ability to cure more children from cancer. Cancer is still the number one cause of death by disease amongst children. Even though the cure rate has improved to nearly 80% overall, we must still find ways to save the rest.”

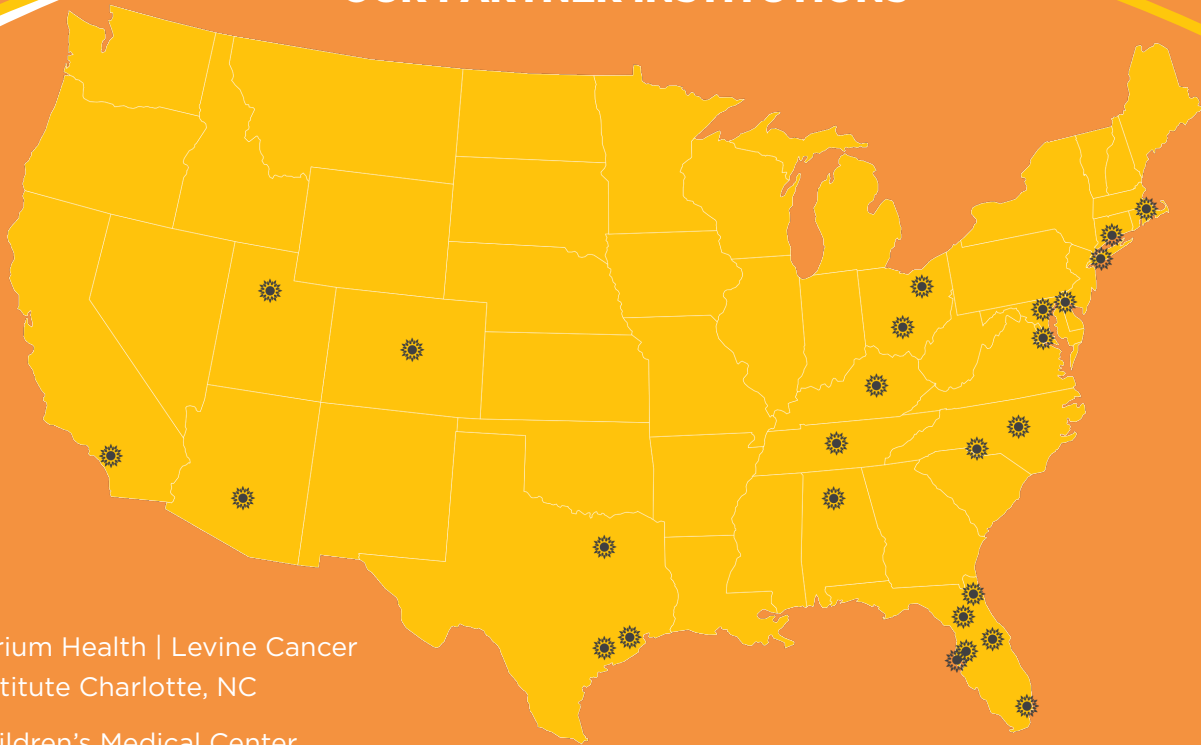


SUNSHINE RESEARCH PROJECT IMPACT

- The leading research consortium with national reach addressing multiple types of high-risk pediatric cancers versus an organization focused on one institution or type of cancer.
- 150+ top doctors and scientists in 30+ 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.
- \$30 million in research funding over the past ten years with success in multiple drug combinations therapies & identification.
- Metformin to treat A.L.L. and establishment of nationwide blood & tissue bank.
- Seven current clinical trials and 30 translational studies underway.



OUR PARTNER INSTITUTIONS



- Atrium Health | Levine Cancer Institute Charlotte, NC
- 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
- Montetiore 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
- Primary Children's Medical Center Salt Lake City, UT
- Rosewell Park Comprehensive Cancer Center Buffalo, NY
- 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
- University of Texas MD Anderson Cancer Center Houston, TX
- Vanderbilt-Ingram Cancer Center Nashville, TN

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 2 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 lsd1 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 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 recurrence of disease in patients who are in radiographic remission 2-3 weeks after treatment. Plasma tumor DNA (ptDNA) is pfree floating DNA from the tumor found in the blood stream 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 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



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 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 2020 - 2021

Made an impact.

Financial Highlights for fiscal year 2020/2021:

Total (in rounded thousands):

- Total Income in past FY = **\$5,251,000**
- Ending Net Assets = **\$6,249,000**
- Program Expenses = **\$3,724,000**
- Management & General = **\$166,000**
- Development & Community Support = **\$178,000**
- Total Operating Expenses = **\$4,068,000**

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, Lisa Companioni-Smith, 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, Rob Webster, Michael Weigner, and Robert White



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 rating for financial health and transparency for the 10th consecutive year. The NPCF is the top rated childhood cancer related nonprofit for this distinction.





**NATIONAL
PEDIATRIC CANCER
FOUNDATION**

NationalPCF.org

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CFC# 43259

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