MISSION OVERVIEW

RESEARCH REPORT FOR 2025

NATIONAL PEDIATRIC CANCER FOUNDATION®

DEAR FRIENDS,

The National Pediatric Cancer Foundation is headquartered in Tampa, Florida and our mission is dedicated to funding research to eliminate pediatric cancer. We govern a unique research consortium, called the Sunshine Project, consisting of physicians and scientists from forty 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.

David Frazer,
CEO

TESTIMONIALS

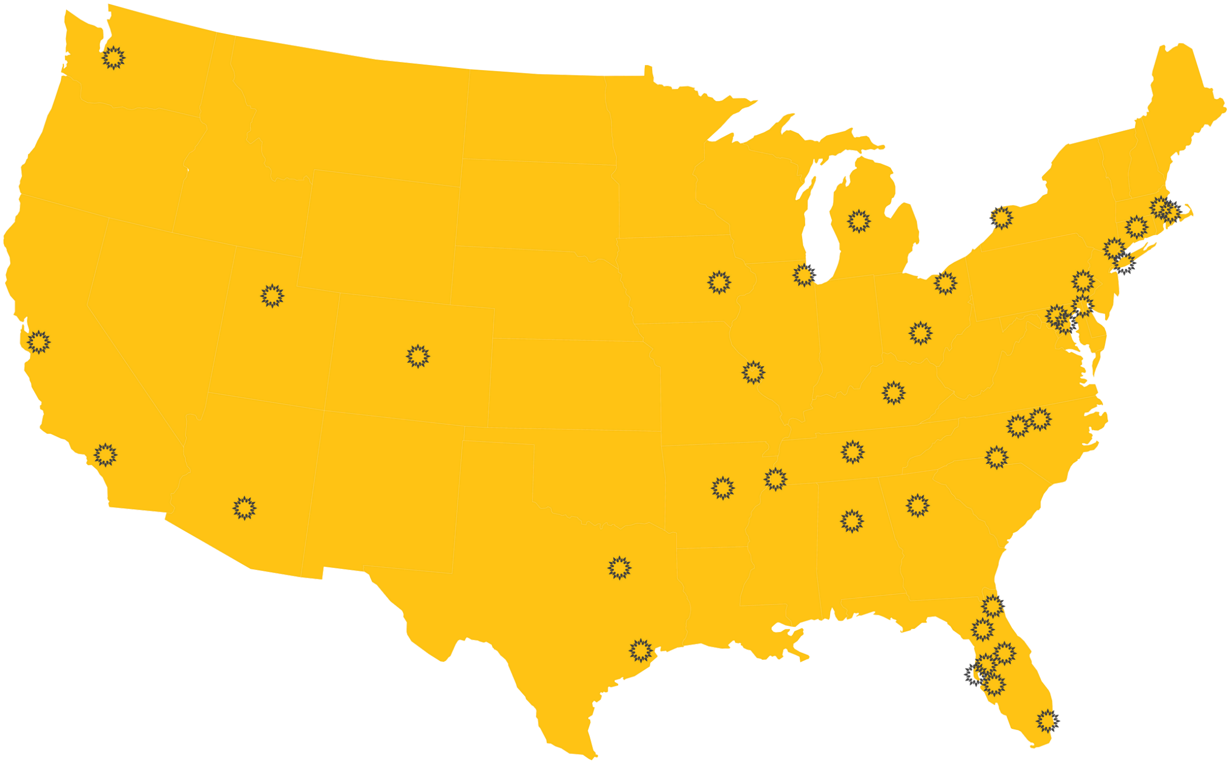
"For a disease like pediatric cancer, no single institution can see enough patients to make a real, tangible difference or impact. We need several institutions working hand in hand with each other. When I see what the Sunshine Project is accomplishing, it's inspiring and makes me feel a part of something much greater."
Elias Sayour, MD, Sunshine Project Site PI, University of Florida

"Curing a child with cancer remains an overwhelming challenge, with some cancers essentially incurable, and many others causing lifelong disabilities. I am so grateful for the existence of NPCF's Sunshine Project and have personally been able to devote my team's time more aggressively towards finding more effective, less toxic cures."
Eugene Hwang, MD, Sunshine Project Central Nervous System Task Force Leader, Children's National Medical Center

ABOUT THE SUNSHINE PROJECT

- 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 40+ 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.

OUR PARTNER INSTITUTIONS



A.I. duPont | Nemours Delaware, Wilmington, DE
Arkansas Children's Health | Little Rock, AR
Atrium Health | Levine Cancer Institute Charlotte, NC
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 Medical Center | Dallas, TX
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 Cancer Institute | Durham, NC
Helen Devos Childrens Hospital | Grand Rapids, MI
Johns Hopkins All Children's Hospital | St. Petersburg, FL
Johns Hopkins Comprehensive Cancer Center | Baltimore, MD
Moffitt Cancer Center | Tampa, FL
Montefiore Medical Center | Bronx, NY
National Institues of Health | Bethesda, MD
Nationwide Children's Hospital | Columbus, OH
Nemours Children's Clinic | Jacksonville, FL
Nemours Children's Hospital | Orlando, FL
Phoenix Children's Hospital | Phoenix, AZ
Primary Children's Medical Center | Salt Lake City, UT
Rosewell Park Comprehensive Cancer Center | Buffalo, NY
Seattle Children's Hospital | Seattle, WA
St. Joseph's Hospital | Tampa, FL
Stanford University | Palo Alto, CA
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 Iowa | Iowa City, IA
University of Texas MD Anderson Cancer Center | Houston, TX
Vanderbilt-Ingram Cancer Center | Nashville, TN
Weill-Cornell Medicine | New York City, NY

RESEARCH TRIALS & STUDIES

CURRENT TRIALS

Title: Blood-Based Biomarkers for Minimal Residual Disease Detection in Pediatric Sarcomas
PI: Mas Hayashi, MD – University of Colorado
Launched in 2020 (MCC 20320)

This study is trying to see if testing blood for pieces of DNA (ctDNA) and cancer cells from the tumor (CTCs) floating around in the blood can be used to predict if cancer will come back in patients who seem to be in remission (no signs of cancer).

Title: Evolutionary Inspired Therapy for Newly Diagnosed, Metastatic, Fusion Positive Rhabdomyosarcoma.
PI: Jonathan Metts, MD – Moffitt Cancer Center
Launched in 2020 (MCC 20339/NCT04388839)

Metastatic, fusion-positive rhabdomyosarcoma (RMS) is a type of cancer that usually has a poor outcome, with 94% of patients having the cancer come back by 3 years, even with a great response to treatment. The treatments in this study aim to improve this outcome.

Title: A Multi-Institution Study of TGFβ 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 (TINKS)
PI: Bhuvana Setty, MD – Nationwide Children's Hospital
Launched in 2022 (MCC 21704/NCT05634369)

This trial aims to test whether adding a special type of immune cell (called TGFβ imprinted NK cells) from a universal donor to the chemotherapy drugs gemcitabine and docetaxel is safe for treating sarcomas that have come back or don't respond to other treatments.

Title: Feasibility of Generating Novel Translational and Therapeutic Strategies based on a Multicenter, Pediatric and AYA Evolutionary Tumor Board (pedsETB)
PI: Jonathan Metts, MD – Moffitt Cancer Center
Launched in 2024 (MCC 22170/NCT06423950)

The Pediatric Evolutionary Tumor Board (pedsETB) is a group of doctors and experts from different fields who work together to come up with new ideas for treating children with cancer. They use knowledge from how cancer changes and evolves over time to find better ways to treat it. The goal is to improve outcomes for kids with cancer by using creative, science-based approaches. This team's ideas have helped create some of the newest treatments being tested in clinical trials for children with cancer.

Title: Phase II study - Evaluation of Digoxin for Relapsed non-WNT, non-SHH Medulloblastoma
PI: Mike Isakoff, MD - Connecticut Children's & Laura Metrock, MD – University of Alabama-Birmingham
Launched in 2024 (MCC 23221/NCT06701812)

This trial is testing whether the drug digoxin, which is typically used to treat heart problems, can help treat medulloblastoma (a type of brain cancer).

CURRENT TRIALS CONT.

Title: Metastatic Ewing's Trial Testing Schedule Enhancement to Improve Outcomes
PI: Jonathan Metts, MD – Moffitt Cancer Center & Matteo Trucco, MD - Cleveland Clinic
Launched in 2025 (MCC 23281)

This trial will test if giving patients with advanced Ewing sarcoma different chemotherapy treatments in a specific order, called "sequential second strikes," can improve the chances of a cure and prevent the cancer from becoming resistant to treatment.

Title: RNA PRIME – RNA Lipid Particles Targeting Pediatric Recurrent Intracranial Malignancies and Other SystEmic Solid Tumors
PI: John Ligon, MD - University of Florida & Eugene Hwang, MD - Children's National
Launched in 2025 (MCC 23450/NCT05660408)

This study tests the safety, immune effects, and potential effectiveness of RNA-loaded lipid nanoparticles (RNA-LP) vaccines in patients with recurrent pediatric High-Grade Glioma (pHGG), a type of brain cancer, and recurrent Osteosarcoma (OSA), a type of bone cancer. Based on earlier lab research and experience with similar vaccines for kids with brain tumors, we believe this treatment could help trigger immune responses that fight the cancer. This approach may offer benefits for patients with these cancers, where other treatments haven't been able to cure the disease.

Title: Vuja De - Open-label, cohort-sequential dose-escalation and dose-confirmation Phase 1/2 clinical trial to evaluate the safety and efficacy of domatinostat in combination with sirolimus in adolescents and adults with relapsed, refractory sarcoma and osteosarcoma
PI: Amy Armstrong, MD - St. Louis Children's Hospital
Launched in 2025 (TBD)

The Vuja De study is a clinical trial that tests the safety and effectiveness of two drugs, domatinostat and sirolimus, used together in patients with Osteosarcoma and other sarcomas that have come back or don't respond to other treatments. This study will gradually increase the drug doses to find the best and safest amount for patients.

COMPLETED TRIALS

Title: Phase II Study of nab-Paclitaxel in Combination with Gemcitabine for Treatment of Recurrent/Refractory Sarcoma in Teenagers and Young Adults
PI: Javier Oesterheld, MD - Levine Children's Hospital
Launched in 2016 (MCC 18613/NCT02945800)

This trial tested if the combination of nab-paclitaxel and gemcitabine could stop the growth or spread of tumors in teenagers and young adults with osteosarcoma, Ewing sarcoma, rhabdomyosarcoma, and other soft tissue sarcomas that have come back or don't respond to other treatments. The study also checked how long the disease stays under control during and after treatment, and if this combination of drugs is safe and easy for patients to tolerate.

Title: A Phase Ib/II Study to Evaluate the Safety, Feasibility, and Efficacy of Nivolumab in Combination with Azacitidine in Patients with Recurrent, Resectable Osteosarcoma.
PI: Patrick Thompson, MD - UNC Chapel Hill & Mihaela Druta, MD – Moffitt Cancer Center
Launched in 2020 (MCC 19487/NCT03628209)

This was the first time the drugs Nivolumab and Azacitidine were used together to treat osteosarcoma that had come back and can be surgically removed. The goal of this trial was to see if this drug combination helps patients stay cancer-free longer compared to the usual 20% success rate seen in past studies by the Children's Oncology Group.

COMPLETED TRIALS CONT.

Title: Phase 1 Trial of the LSD1 Inhibitor Seclidemstat (SP 2577) With and Without Topotecan and Cyclophosphamide in Patients With Relapsed or Refractory Ewing Sarcoma and Select Sarcomas
PI: Damon Reed, MD – Moffitt Cancer Center
Launched in 2018 (MCC 19365/NCT03600649)

A Phase I clinical trial led by Dr. Damon Reed investigated the safety and optimal dosing of seclidemstat, an experimental drug, both alone and in combination with standard chemotherapy agents—topotecan and cyclophosphamide—in patients with relapsed or treatment-resistant Ewing sarcoma and certain other sarcomas.

Seclidemstat is an experimental oral drug designed to inhibit LSD1, an enzyme that plays a role in cancer cell growth. By blocking LSD1, seclidemstat may help stop or slow the progression of certain cancers.

Title: ACTION Trial: Adoptive Cellular Therapy following Dose-Intensified Temozolomide in Newly diagnosed Pediatric High-grade Gliomas
PI: Duane Mitchell, MD, PhD – University of Florida
Launched in 2018 (MCC 18752/NCT03334305)

This trial tested a type of immunotherapy to see if it is safe for children with High-Grade Gliomas (HGG), a type of brain cancer. It focused on patients who have already received a stronger dose of the chemotherapy drug temozolomide and a special immune treatment using their own immune cells (Dendritic Cells and Autologous Lymphocyte Transfer). Some patients also received their own stem cells as part of the treatment. Researchers also checked if patients could complete the full treatment and track how long they stayed cancer-free and how well they responded overall.

Title: A Phase I Trial of Dose Escalation of Metformin in Combination With Vincristine, Irinotecan, and Temozolomide in Children With Relapsed or Refractory Solid Tumors
PI: Damon Reed, MD – Moffitt Cancer Center
Launched in 2012 (MCC 16962/NCT01528046)

A clinical trial led by Dr. Damon Reed investigated the addition of metformin, a common diabetes medication, to a chemotherapy regimen known as VIT—comprising vincristine, irinotecan, and temozolomide—in treating children with relapsed or treatment-resistant solid and central nervous system (CNS) tumors. The primary goal was to determine the highest dose of metformin that patients could tolerate when combined with VIT, as well as to observe any potential therapeutic benefits.

Title: A Phase I Window, Dose Escalating and Safety Trial of Metformin in Combination With Induction Chemotherapy in Relapsed Refractory Acute Lymphoblastic Leukemia: Metformin With Induction Chemotherapy of Vincristine, Dexamethasone, Doxorubicin, and PEG-asparaginase (VPLD)
PI: John Goldberg, MD – Moffitt Cancer Center
Launched in 2011 (MCC 16601/NCT01324180)

Dr. John Goldberg and his team conducted a Phase I clinical trial to assess the safety and appropriate dosing of metformin when combined with standard induction chemotherapy in children with relapsed or refractory acute lymphoblastic leukemia (ALL). This study aimed to determine if adding metformin could enhance treatment outcomes without introducing significant side effects.

Title: Phase I, Traditional 3+3, Trial of PO Sorafenib and Topotecan in Refractory or Recurrent Pediatric Solid Malignancies
PI: Damon Reed, MD – Moffitt Cancer Center
Launched in 2013 (MCC 16963/NCT01683149)

Dr. Damon Reed conducted a Phase I clinical trial to evaluate the safety and appropriate dosing of combining two drugs—sorafenib and topotecan—in children and adolescents with solid tumors that had either returned after treatment (recurrent) or did not respond to existing therapies (refractory).

The study aimed to determine the highest dose of sorafenib that could be safely administered alongside a standard dose of topotecan.

OTHER RESEARCH

"43 CHALLENGE" AWARD WINNERS (2022)

Title: Engineered Destabilized AU Rich Elements of c-MYC 3'UTR as Therapeutics for C-MYC Driven Pediatric Cancers
PI: Daniel Weiser, MD - 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.

Title: Treating Leptomeningeal Spread of Pediatric Medulloblastoma with Quantum Radiobiology
PI: Kai Iwamoto, MD - 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 nuclear bombs on survivors during WWII, he will use quantum mechanics to precisely target specific tumor cells without harming normal cells which will revolutionize 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.

Title: Harnessing the Power of Innate Antibodies for Novel Therapies in Pediatric Cancer
PI: Gregory Sullivan, MD - 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.

OTHER STUDIES

Title: One Carbon Is Not Enough – A Metabolic Attack on Ewing Sarcoma
PI: David Loeb, MD – Montefiore Medical Center
Ewing sarcoma is the second most common bone cancer in children and young adults. While over 75% of patients with localized disease survive, the intense chemotherapy causes serious side effects. Outcomes for those with metastatic disease remain poor. Dr. David Loeb's research focuses on developing safer, more effective treatments by targeting the biology of the disease. Ewing sarcoma relies on a process called One Carbon Metabolism, which uses nutrients like folic acid and serine to help cancer cells grow. His team is testing drugs—including the antidepressant sertraline—to block this process in lab and animal models. If successful, this could lead to clinical trials and better treatment options with fewer side effects.

Title: Targeting Mesoderm-Like Cells in Pediatric Cancer
PI: Anand Patel, MD – St. Jude Children's Research Hospital
Dr. Patel is digging deep to understand what tumor cells are left behind during treatment. We have learned that not all cells that make up a tumor are the same- and even just a few cells that are resistant to therapy can hide in the body- ultimately causing tumors to recur. Dr. Patel has been able to identify these persisting cells and is working on finding additional treatments that will eliminate them.

Title: Preclinical Studies of Nuak Kinase Inhibitors in Fusion Positive RMS
PI: Michael Deel, MD – Cincinnati Children's Hospital
Fusion-positive sarcomas, like fusion-positive rhabdomyosarcoma (FP-RMS), are some of the hardest childhood cancers to treat. This study is testing a new type of drug called NUAK kinase inhibitors to see if they can stop cancer growth. Researchers believe these drugs can block key signals that help cancer cells grow and divide. The goal is to slow or stop tumor growth and make existing treatments work better.

Title: Preclinical Evaluation of M002, a Novel Oncolytic Herpes Simplex Virus in FP Rhabdomyosarcoma
PI: Jamie Aye, MD – University of Alabama – Birmingham
Dr. Aye is taking a bold and innovative approach by using viruses to fight cancer—turning a common enemy into a powerful ally. While viruses are typically known for causing illness, the virus in this study has been carefully engineered to target and destroy cancer cells while leaving healthy cells unharmed. Dr. Aye is currently investigating whether combining this virus with radiation therapy can effectively treat fusion-positive rhabdomyosarcoma (RMS), the most aggressive and hard-to-treat subtype of RMS. Her research offers hope for a smarter, more targeted way to attack this challenging cancer.

Title: Exploring Tertiary Lymphoid Structures in Rms from Promoting Immunotherapy Response
PI: Nico Llosa, MD – Johns Hopkins University
Rhabdomyosarcoma (RMS) is the most common soft tissue sarcoma in pediatric, adolescent and young adult patients. We have discovered that within the tumor beds of RMS, T and B cells interact to form highly organized structures engendering aggregates like follicles in lymph nodes in a pattern that we denominate tertiary lymphoid structures (TLS). We have been able to associate the presence of TLS in RMS with better survival of patients receiving standard therapies. We hypothesize that the formation of TLS in RMS is essential for the generation of intratumorally immune responses. Our proposal focuses on (i) the drivers of TLS formation, (ii) the function and contribution of TLS to the anti-tumor immune response, and (iii) the potential manipulation of TLS as a therapeutic tool in RMS.

Title: Harnessing The Body's Immune Defenses to Fight Cancer
PI: Justin Sperlazza, MD – University Of North Carolina
Dr. Justin Sperlazza is working on a way to help the immune system fight childhood cancers more gently than chemo. Some tumors, like Ewing sarcoma and osteosarcoma, don't have enough of a target called GD2 for immune cells to attack. His team found that a drug called Tazemetostat can make these tumors show more GD2, but not for long. By testing different drug combos, they found one that keeps GD2 levels high and helps immune cells (CAR-T therapy) completely wipe out tumors in mice. Now, they're planning a clinical trial to see if this approach can help kids, too.

Title: GD2 CAR-NK Clinical Product Development
PI: Dean Lee, MD – Nationwide Children's Hospital
Dr. Dean Lee's research with The Sunshine Consortium is exploring a new sarcoma treatment using special immune cells called universal-donor NK cells. It's the first study to use these lab-made cells across multiple treatment centers. Adding a "CAR" receptor to the NK cells helps them better find and destroy cancer. Dr. Lee's team developed a way to create these CAR-NK cells efficiently, but they needed funding to produce them at the scale needed for human trials. The National Pediatric Cancer Foundation helped fund key materials, staff, and supplies—allowing the team to meet FDA standards and move closer to making this treatment available for children.

Title: Enhancing Immunotherapy for Pediatric Solid Tumors Through Epigenetic Modulation
PI: Ian Davis, MD, PhD Professor – University of North Carolina
This is a start-up project that explores the effect of small molecule-based chromatin modulation on GD2 tumor cell antigen expression and response to NK (Natural Killer) cell co-culture. The response of other CAR-T cell targeted antigens to epigenetically targeted small molecules will also be tested.

Title: Pre-Clinical Evaluation of Ibrutinib Therapy in Pediatric Glioblastoma
PI: Chris Hubert, MD – Case Western Reserve University
Dr. Chris Hubert's research explores whether a drug called ibrutinib—already used safely in adults and children for other conditions—could help treat aggressive pediatric brain tumors like high-grade glioma (HGG), one of the deadliest cancers in young people. His team found that some brain tumor cells respond well to ibrutinib, especially when a protein called BMX is present. With funding from the National Pediatric Cancer Foundation, the team is now working to gather key data to see if ibrutinib should be tested in clinical trials for children with HGG, and to identify which kids might benefit most.

Title: Overcoming Immunotherapy Challenges to Target Leptomeningeal Disease
PI: Greg Friedman, MD – MD Anderson
Dr. Greg Friedman's research aims to improve treatment for children with brain tumors that have spread to the brain and spine (leptomeningeal disease), a condition with few effective options. His team is testing a promising therapy using a modified herpes virus that kills cancer cells but leaves healthy brain cells unharmed, helping the immune system fight the tumor. While early results are encouraging, current delivery requires brain surgery, limiting repeat treatments. Dr. Friedman's team is now exploring safer ways to deliver the virus into spinal fluid without surgery, including a protective low dose or a drug that boosts natural defenses. These methods show potential in treating leptomeningeal disease. Next, they'll test dosing strategies and combination treatments—like pairing the virus with a tumor vaccine—with support from the National Pediatric Cancer Foundation's Sunshine Project.

Title: Creating The Next Generation Peptide Vaccine for Cytomegalovirus Antigens in Malignant Brain Tumors
PI: Eric Thompson, MD – University of Chicago
Dr. Eric Thompson and his team are developing a new vaccine to treat aggressive pediatric brain tumors like high-grade glioma, diffuse midline glioma, and medulloblastoma. The vaccine targets markers from human cytomegalovirus (hCMV), found in tumor cells but not in healthy brain tissue. Their first version, PEP-CMV, showed promising results—patients tolerated it well, most developed immune responses, and some lived over a year. Now, with support from the National Pediatric Cancer Foundation, they are creating a stronger version that targets multiple hCMV markers. This next-generation vaccine could offer a more effective, less toxic alternative to traditional treatments and improve survival and quality of life for children.

Title: Difluoromethylornithine to Prevent and Treat Osteosarcoma Metastasis
PI: David Loeb, MD, PhD – Children's Hospital at Montefiore
Our laboratory has previously demonstrated that difluoromethylornithine (DFMO), a competitive inhibitor of the metabolic enzymes ornithine decarboxylase and arginase, inhibits the growth of Ewing sarcoma cells in vitro, prevents clonogenic growth of Ewing sarcoma cells in soft agar, prevents Ewing sarcoma sphere formation in vitro, and inhibits Ewing sarcoma metastasis in our orthotopic implantation/amputation model of spontaneous metastasis in mice. Given the in vitro and in vivo activity we see in Ewing sarcoma, we next asked the question whether DFMO has similar effects on osteosarcoma.

Title: B7-H3-CAR iNKT Therapy for High-Risk Medulloblastoma
PI: Barbara Savoldo, MD & David Kram, MD – University of North Carolina, Elias Sayour, MD - University of Florida, Conrad Cruz, MD & Eugene Hwang, MD - Children's National
Researchers from UNC Chapel Hill, the University of Florida, and Children's National are developing a new treatment for medulloblastoma, the most common pediatric brain cancer, which is often fatal when it returns. While CAR T cell therapy has worked well for blood cancers, it hasn't been successful for brain tumors—mainly due to challenges like poor immune cell access, treatment resistance, and severe side effects.

This team is testing a new approach using special immune cells called iNKT cells engineered with a B7-H3 CAR. These cells are designed to better reach brain tumors, reduce toxic side effects, and avoid treatment resistance. Supported by the National Pediatric Cancer Foundation, they are studying how well B7-H3 CAR iNKT cells work on their own and combined with CAR T cells. The ultimate goal is to create an off-the-shelf, effective, and less toxic therapy to improve survival for children with aggressive brain tumors.

Title: Metastatic Osteosarcoma Working Group
PI: Mas Hayashi, MD – University of Colorado, Dan Weiser, MD & David Loeb, MD, PhD - Children's Hospital at Montefiore, Mike Isakoff, MD - Connecticut Children's, Ryan Roberts, MD - Nationwide Children's Hospital
Specific Aim: Evaluate how accurately molecular profiling of circulating cell-free DNA (cfDNA) can detect minimal residual disease (MRD) in osteosarcoma (OS) and predict treatment outcomes.

A major gap in OS research is the lack of reliable methods to measure disease burden, since imaging alone is not sensitive enough and no biomarkers exist to predict response at diagnosis. It's long been known that nearly all OS patients—even those with localized tumors—have micrometastatic disease. Our team has confirmed this by detecting circulating tumor DNA (ctDNA) and tumor cells in patient blood samples.

We aim to develop and test a highly sensitive, non-invasive liquid biopsy assay to track disease over time. We believe that profiling serial blood samples will allow us to measure tumor burden, monitor treatment response, detect early recurrence, and observe how tumors evolve at the molecular level.

Title: Precision Adoptive Cellular Therapy Targeting Refractory Medulloblastoma
PI: Duane Mitchell, MD, PhD – University of Florida
Medulloblastoma (MB) is the most common type of malignant pediatric brain tumor, and despite aggressive multi-modality therapy, up to a third of children with MB will still die from recurrent disease. Tumor-specific immunotherapy, including adoptive cellular therapy (ACT), is a promising treatment modality for children with refractory medulloblastoma that may meet the significant unmet need of safe and effective treatment for this pediatric population.

PUBLISHED RESEARCH

Published in 2024 - Clinical Cancer Research
Title: Phase II Trial of Gemcitabine and Nab-Paclitaxel for Recurrent Osteosarcoma with Serial Monitoring Using Liquid Biopsy: A Report from the National Pediatric Cancer Foundation
PI: Dhir A, Hayashi M, Bodlak A, Oesterheld J, Loeb DM, Mascarenhas L, Isakoff MS, Sandler ES, Borinstein SC, Trucco M, Lagmay JP, Setty BA, Pratilas CA, Caywood E, Metts J, Yin H, Fridley B, Yin J, Laborde J, Reed DR, Adams DL, Wagner LM

Published in 2024 - American Association for Cancer Research Journal
Title: Identification of ATP-Competitive Human CMG Helicase Inhibitors for Cancer Intervention that Disrupt CMG Replisome Function
PI: Xiang, Welch, Reed

Published in 2024 - Journal of Clinical Oncology
Title: Pediatric Leukemia Roadmaps are a Guide for Positive Metastatic Bone Sarcoma Trials
PI: Reed, Metts, Trucco

Published in 2024 - Cell/Science Direct
Title: RNA Aggregates Harness the Danger Response for Potent Cancer Immunotherapy
PI: Sawyer, Ligon, Mitchell, Sayour

Published in 2023 - Cancer Medicine
Title: VIT & Metformin
PI: Reed, Metts, Trucco, Weiser, Thompson, Sandler, Gorlick, Gill

Published in 2023 - Scientific Reports
Title: Modeling Phenotypic Heterogeneity Towards Evolutionarily Inspired Osteosarcoma Therapy
PI: Welch, Metts, Brohl, Reed

Published in 2023 - Cancer
Title: Consensus Recommendations in the Management of Ewing Sarcoma from the National Ewing Sarcoma Tumor Board
PI: Gupta, Borinstein, Isakoff, Trucco

Published in 2023 - Clinical Cancer Research
Title: Breaking Up Isn't Hard To Do: Isolating Cell-Free DNA Fragments in Osteosarcoma
PI: Weiser, Hayashi

Published in 2022 - Journal of Clinical Oncology
Title: Study of nab-Paclitaxel in Combination with Gemcitabine for Treatment of Recurrent/Refractory Sarcoma in Teenagers and Young Adults
PI: Oesterheld

Published in 2022 - Cancer
Title: A Proposed Trial Design for the Treatment of Widely Metastatic Ewing Sarcoma Inspired by Evolutionary Dynamics
PI: Reed, Trucco, Russell, Metts

Published in 2021 - Journal of Clinical Oncology
Title: Phase I Trial of the LSD1 Inhibitor Seclidemstat (SP-2577) with and without Topotecan and Cyclophosphamide in Patients with Relapsed or Refractory Ewing Sarcoma and Select Sarcomas
PI: Reed, Setty, Metts, Loeb

Published in 2020 - Cancer
Title: An Evolutionary Framework for Treating Pediatric Sarcomas
PI: Reed, Metts, Hayashi, Isakoff, Loeb

Published in 2020 - Cancer
Title: An Evolutionary Framework for Treating Pediatric Sarcomas
PI: Reed, Metts, Hayashi, Isakoff, Loeb

Published in 2018 - Pediatric Blood & Cancer
Title: VPLD & Metformin
PI: J. Barredo, Trucco, Gil, Reed

Published in 2017 - Pediatric Blood & Cancer
Title: Consensus and Controversies Regarding the Treatment of Rhabdomyosarcoma
PI: Borinstein, Hayashi, Loeb, Isakoff, Brohl, Wagner, Reed

Published in 2015 - Scientific Reports
Title: Identification of Synergistic, Clinically Achievable, Combination Therapies for Osteosarcoma
PI: Yu, Sullivan, & Reed

Published in 2015 - Cancer Medicine
Title: Phase I trial - Relapsed Solid Tumors, Sorafenib & Topotecan
PI: Reed, Mascarenhas, Gill, Sandler, Isakoff, Sullivan

National Pediatric Cancer Foundation's 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.

NATIONAL PEDIATRIC CANCER FOUNDATION®

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