



CURESEARCH

FOR CHILDREN'S CANCER

Impact Report

Summer 2020

Message From Our CEO

To our CureSearch Community:

In these unprecedented times, it has been inspiring to watch the unwavering focus of our staff, volunteers, and funded researchers in their commitment to accelerate the pace of childhood cancer research. Despite the challenges presented this year, we're proud to share the many innovative ways we're driving new treatment options for the children who are counting on us.

Excitedly, this spring, we announced our largest-ever grant, a \$2.5 million award supporting a potentially groundbreaking, first-in-human clinical trial testing a novel immunotherapy for high-grade glioma, a devastating diagnosis. We committed to two Young Investigator awards supporting innovative treatments for malignant peripheral nerve sheath tumor (MPNST) and neuroblastoma. Each of these projects shows potential in treating additional types of cancer.

Our currently funded researchers are making encouraging advancements even while navigating these uncharted waters. Dr. Avery Posey has completed an important and challenging first step in his CAR-T cell therapy for neuroblastoma, paving the way to begin testing. Dr. Loretta Li's recent results show new promise for patients with a high-risk subtype of B-Cell ALL. We're thrilled to see these projects advance quickly toward the clinic.

Importantly, our strategic collaborations continue to grow as we strive to change the drug development process from within. In June, we announced a new initiative with the Milken Institute FasterCures and Children's Tumor Foundation aimed at revitalizing deprioritized drugs, setting them on a path for pediatric development to help children with tumors. Later this year, we'll host our first Pediatric Early Development Symposium, a unique virtual forum where academia, industry, and regulatory agencies will discuss real-life examples and real-world lessons to increase their ability to meet new FDARA guidelines and thus support early pediatric drug development.

Cancer diagnoses haven't slowed down. Thanks to your support, we continue to push forward at our expedited pace. The need for new, better childhood cancer treatments remains critical, and despite the uncertainty of these times, we, and our volunteer community, remain steadfast in our support of the 43 children diagnosed every day.

With Gratitude,



Kay Koehler

CureSearch for Children's Cancer CEO



Delivering New Treatment Options Today: CureSearch-Funded Clinical Trials Now Enrolling Patients Across the Country



Ranjit Bindra, MD, PhD

Yale Cancer Center

FIVE CLINICAL TRIAL SITES RECRUITING ADOLESCENT & YOUNG ADULT PATIENTS WITH HIGH-GRADE GLIOMA

Dr. Bindra is performing a clinical trial to see if the addition of a new drug to standard brain cancer therapy can reduce the amount of chemotherapy required for treatment. In the last 6 months, Dr. Bindra has opened three new clinical trial sites.

A total of five sites are now recruiting adolescent and young adult patients with high-grade glioma: Dana-Farber Cancer Institute, Johns Hopkins, Oregon Health & Science University, St. Louis Children's Hospital, and the University of California, San Francisco. Two patients are already in treatment.

[Learn More ▶](#)



Crystal Mackall, MD

Stanford University School of Medicine

PROMISING DIPG IMMUNOTHERAPY CLINICAL TRIAL OPENS

Dr. Mackall's early-phase clinical trial will help determine whether CAR T-cell therapy is an option for patients with DIPG, a cancer with a dismal 5-year survival rate of less than 1%.

In early June, Dr. Mackall and colleagues at Stanford University began recruiting patients for this innovative new trial.

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Groundbreaking CureSearch Catapult Project Has Potential to Impact All Solid Tumors



Elias Sayour, MD, PhD

University of Florida

IMMUNOTHERAPY GETS PERSONAL IN HIGH-GRADE GLIOMA

This spring, we committed \$2.5 million to fund a first-in-human, phase I/II clinical trial testing an innovative personalized immunotherapy for pediatric high-grade gliomas, the primary cause of death in children with brain tumors. This CureSearch Catapult Award was granted to Dr. Elias Sayour at the University of Florida who will partner with the

Pacific Pediatric Neuro-Oncology Consortium to enroll patients at 18 different institutions across the U.S.

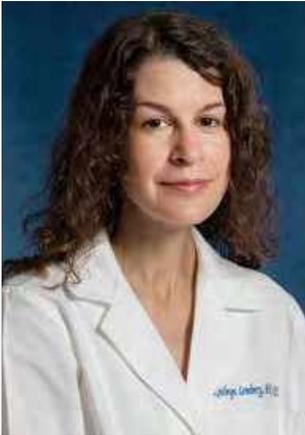
Dr. Sayour will be assessing this therapy in high-grade gliomas, including anaplastic astrocytoma and glioblastoma, which are among the most devastating pediatric cancer diagnoses with a median survival of less than two years. Like a flu vaccine, which takes pieces of the flu virus and activates the immune system against them, Dr. Sayour's technique takes pieces of a tumor's genetic material and activates the immune system to identify and destroy that tumor. If high-grade glioma, a hard-to-treat tumor type, is responsive to this immunotherapy, the treatment will likely translate to other pediatric and adult solid tumors as well.

[Learn More ▶](#)

“ Pediatric cancers are unique entities that mandate new research avenues and development of novel targeted therapies. While funding exists for new discoveries, little to no funding infrastructure is in place for catalysis and development of these discoveries into first-in-human clinical trials. I can think of no other foundation primarily focused in funding the developmental work necessary to bring forward new discoveries into groundbreaking clinical trials for the heroic children battling cancer. ”

– Dr. Elias Sayour

New Research Funding Supports Promising Treatments With Potential to Impact Multiple Tumor Types



Kathryn Lemberg, MD

Johns Hopkins University

THE MOST WELL-CHARACTERIZED DRUG TO BE ASSESSED BY A CURESEARCH YOUNG INVESTIGATOR

Malignant peripheral nerve sheath tumors (MPNSTs) are aggressive tumors that arise from the cells that support nerve function. MPNSTs are incredibly challenging to treat and don't respond well to chemotherapy and radiation, making surgical removal a mainstay of treatment. However, if the tumor is not completely removed at diagnosis-which happens approximately 50% of the time-survival is only 30%. New treatment options are desperately needed and Dr. Lemberg will use a new drug, developed at Johns Hopkins University, to starve MPNST cells to death. Additionally, this therapy should limit side-effects since it only activates when taken up by the tumor cells.

Through this project, Dr. Lemberg aspires to set a well-characterized, exciting new drug on the path to human clinical trials in MPNST patients. Additionally, the findings of this study could expand to more than 1,700 kids who are diagnosed in the U.S. each year with pediatric soft tissue sarcomas.

[Learn More ▶](#)

“ Pediatric and young adult patients deserve more effective treatments with fewer side effects than have been classically available for these tumors. As a Young Investigator I look forward to collaborating with the CureSearch community to successfully develop new medicines for sarcoma. ”

– Dr. Kathryn Lemberg

New Research Funding Supports Promising Treatments With Potential to Impact Multiple Tumor Types



Adam Durbin, MD, PhD

Dana-Farber Cancer Institute

A TARGETED THERAPY AIMING TO INCREASE EFFECTIVENESS AND DECREASE LATE-EFFECTS OF HIGH-RISK NEUROBLASTOMA TREATMENT

Dr. Durbin is developing a new treatment for high-risk neuroblastoma. Despite intensive therapy, patients with high-risk neuroblastoma have an overall survival of only 50% and there are currently no effective treatments available for patients with relapsed or progressive disease. Dr. Durbin has identified a targeted therapeutic that is able to kill neuroblastoma cells without impacting normal tissues.

If successful, this project will not only offer an innovative treatment strategy for high-risk neuroblastoma, but has the potential to expand into other pediatric tumors, including high-risk myeloid leukemia and rhabdomyosarcoma.

[Learn More](#) ▶

“ As both a scientist and pediatric oncologist, I am focused on developing new drugs for children with challenging-to-treat cancers. Funding from CureSearch for Children’s Cancer is critical to the early steps of our work to try to deal with these issues by developing less-toxic, more effective therapies for children with cancer. ”

– Dr. Adam Durbin

Current Research Projects Make Advancement Towards the Clinic

CAR optimization complete

At the University of Pennsylvania, CureSearch Young Investigator Dr. Avery Posey is developing a new CAR-T cell therapy for neuroblastoma. In this type of treatment, a patient's T cells are changed in the laboratory so they attack cancer cells. Recently, Dr. Posey completed the challenging and specialized work of identifying an optimal chimeric antigen receptor (CAR) - a significant step forward for his project. With this optimization complete, Dr. Posey will now begin evaluation of his therapy both alone and in combination with other immunotherapies.

[Read More ▶](#)



Avery Posey, MD

University of Pennsylvania

Early indications of overcoming resistance mutations:

Young Investigator Dr. Loretta Li is developing a novel therapy for a high-risk subtype of leukemia that often results in the cancer returning after treatment. The cancer returns because it develops resistance mutations — changes in the cancer that decrease the effectiveness of therapies that were once successful. Early studies of Dr. Li's Type II JAK2 inhibitors indicate that they are able to overcome resistance mutations that develop in response to B-cell acute lymphoblastic leukemia treatment.

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Loretta Li, MD

Ann & Robert H. Lurie
Children's Hospital of Chicago
and Northwestern University

Current Research Projects Make Advancement Towards the Clinic

A PARADIGM SHIFT IN IDENTIFYING PEDIATRIC SARCOMA TREATMENTS

Dr. Andrew Kung aims to identify therapies that can be applied to a wide range of sarcomas – from Ewing sarcoma and osteosarcoma to rarer tumors, including leiomyosarcoma and infantile fibrosarcoma. Such a treatment strategy, based on tumor drivers called master regulators, has the potential to change how sarcoma clinical trials are conducted, enabling genomic characteristics to determine treatment instead of the cancer's type or subtype.



Andrew Kung, MD, PhD
Memorial Sloan Kettering
Cancer Center

Dr. Kung's final report revealed that analysis of his diverse portfolio of 241 pediatric sarcoma cases has resulted in the identification of 20 prioritized drugs with the potential to treat a wide range of pediatric sarcoma subtypes. Dr. Kung and his team hope that the final data obtained from their studies will inform the development of a master regulator-targeting strategy in pediatric sarcomas within the context of a pediatric clinical trial.

[Read More ▶](#)

“ Dr. Kung has produced a resource that should be invaluable to the broader scientific community and the patients we treat. Congratulations to Dr. Kung, his team and to CureSearch for choosing to support them. ”

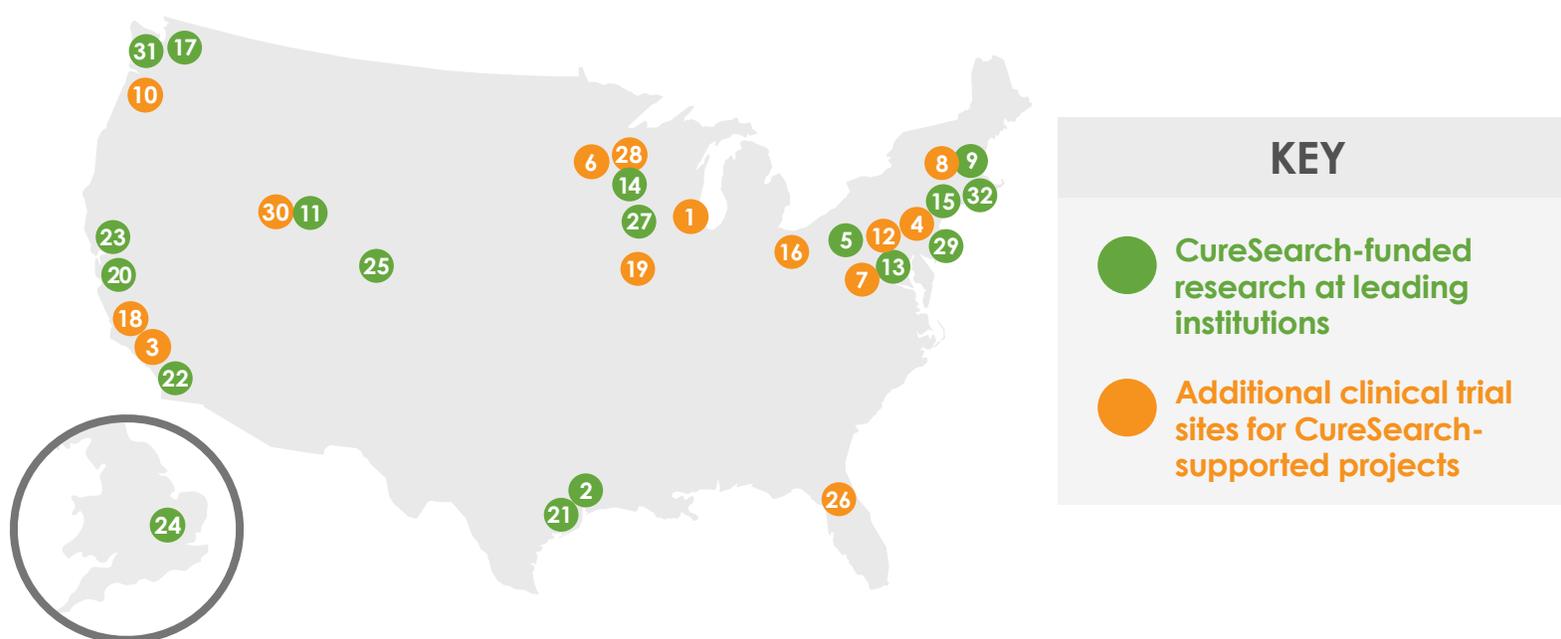
– Malcolm Brenner, MD, PhD

Founding Director of the Center for Cell and Gene Therapy at Baylor College of Medicine

Our Cumulative Impact (2000 - Present)



Our Funding Impact: Cutting-Edge Research at Leading Institutions



1. Ann & Robert H. Lurie Children's Hospital of Chicago
2. Baylor College of Medicine
3. Children's Hospital Los Angeles
4. Children's Hospital of Philadelphia
5. Children's Hospital of Pittsburgh
6. Children's Minnesota
7. Children's National Medical Center
8. Dana-Farber/Boston Children's Cancer and Blood Disorder Center
9. Dana-Farber Cancer Institute
10. Doernbecher Children's Hospital Oregon Health and Science University
11. Huntsman Cancer Institute, University of Utah
12. Johns Hopkins Hospital
13. Johns Hopkins University
14. Mayo Clinic
15. Memorial Sloan Kettering Cancer Center
16. Nationwide Children's Hospital
17. Seattle Children's Hospital
18. St. Jude Children's Research Hospital
19. St. Louis Children's Hospital
20. Stanford University
21. Texas Children's Hospital
22. University of California, San Diego
23. University of California, San Francisco
24. University of Cambridge
25. University of Colorado
26. University of Florida Shands Children's Hospital
27. University of Iowa
28. University of Minnesota/Masonic Cancer Center
29. University of Pennsylvania
30. University of Utah
31. University of Washington
32. Yale University

Changing the Drug Development Landscape From Within By Driving Innovation and Collaboration

REVITALIZING DISCONTINUED DRUGS FOR PEDIATRIC USE

In June, we announced a new partnership with FasterCures, a center of the Milken Institute, and the Children's Tumor Foundation to identify deprioritized drugs that could benefit pediatric patients, and to create a new pediatric-focused pathway for development.

The partnership will utilize CureSearch's relationships with key pediatric advocates within the pharmaceutical industry, including our Industry Advisory Council, to identify previously discontinued drugs as well as those that are likely to be deprioritized soon. These programs have already met certain milestones and are further along the drug development pipeline.

[Learn More ▶](#)

INAUGURAL CURESEARCH PEDIATRIC EARLY DEVELOPMENT SYMPOSIUM: DEVELOPING IPSPS AND PIPS IN AN EVOLVING REGULATORY LANDSCAPE

This unique virtual forum will focus on initial Pediatric Study Plan (iPSP) and Paediatric Investigation Plan (PIP) development and implementation in today's rapidly changing pediatric cancer landscape. Attendees from academia, industry, and regulatory agencies will discuss real-life examples and real-world lessons to meet new FDARA guidelines and support early pediatric drug development.

[Learn More ▶](#)



Get Involved In Your Community

2020 CURESEARCH WALK UNITES SUPPORTERS VIRTUALLY

This year, we're taking the 2020 CureSearch Walk virtual and inviting families, communities and businesses across the country to unite in support of the kids who need us now more than ever. Join a local virtual walk and connect with others in your community, or join our national CureSearch Walk team - either way we walk united.

National Walk Day is September 26, but we invite you to walk on any day that you choose.



ULTIMATE HIKE CELEBRATES 10 YEAR ANNIVERSARY

The world has changed in recent months but our commitment to telling children's cancer to take a hike remains strong. This year we're celebrating the 10 year anniversary of our Ultimate Hike program and, as long as it's safe to do so, we'll be lacing up our boots this fall on some of the country's most beautiful trails.

"Even in the midst of a pandemic, children are still being diagnosed every day, they are fighting every day, they are getting chemo every day. They need our help; they need more funding to find less toxic and more effective treatments! That is why it is vital we continue fundraising and doing what we can to help these children in the fight of their lives!"

- Pediatric nurse and Ultimate Hiker Meaghan Welch, pictured above with her dad Anthony, lost her cousin Jack to cancer on why she plans to lace up again this year

Corporate Sponsorships not only benefit children's cancer research but also support your company's corporate social responsibility, engagement, and health & wellness initiatives.

Learn more about launching a corporate sponsorship in 2020. ▶

Introducing New Resources for Families



Clinical Trial Finder

Approximately 60% of children with cancer participate in a trial, compared to less than 5% of adult patients. The aim of clinical trials is to improve survival rates and/or reduce toxic and often long-term side effects of treatment. A successful trial can lead to FDA approval of new, better treatment options. Our clinical trial finder offers a simple way to identify clinical trials in any location that may benefit both current patients and the entire pediatric cancer community.

[Learn More ▶](#)

New Special Barbie

Through a longstanding partnership with the Mattel Children's Foundation, we released a new, special Barbie free of charge for children affected by cancer. Reflecting the hair loss that many childhood cancer patients experience, the new doll offers interchangeable wigs and other head accessories.

[Learn More ▶](#)



Our Mission: End Children's Cancer

Join the conversation! Follow us on our social media channels and help spread awareness of our critical mission to end children's cancer.



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CureSearch for Children's Cancer

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