



CURESEARCH
FOR CHILDREN'S CANCER

Impact Report

October 2022

Powered by hope, fueled by research.

A message from our CEO

Dear CureSearch Supporters,

I'm thrilled to share our latest Impact Report with you.

Our success is not only measured by the number of projects we fund or the total dollars we pledge, but ultimately by how quickly new treatments reach the children who need them most.

In this report, I'm excited to introduce several new research projects to which we awarded \$4.3 million – our largest funding commitment in recent history. You made this possible.

Supporters throughout the country continue to go above and beyond to raise critical funds. The Ultimate Hike program raised a record-breaking million dollars in 2021. Jakefest events also reached incredible heights, raising more than \$5 million dollars to date for lifesaving research.

There is a dire need to support more translational research – moving preclinical projects into late phase clinical trials, and ultimately new treatments for children with cancer. We're laser-focused on closing that funding gap and advancing research with the highest potential, quickly. In fact, 60% of our preclinical projects have moved into clinical trials, compared to a less than 8% average rate of translation for drugs into clinical cancer trials.

Our projects are 7x more likely to advance.

Collaboration is key for translational research success. We host unique events that bring together key stakeholders to exchange ideas in one setting. Our annual Pediatric Early Development Symposium (PEDS) brings together international thought leaders to discuss clinical trials for limited patient populations. Experts at this year's PEDS explored ways that big data and technology will craft the future of pediatric clinical trials.

In addition, our annual CureSearch Summit brought together thought leaders from academia, the pharmaceutical and biotech industries, regulatory entities, patient families and advocacy groups who will continue to collaborate and expand community knowledge of extant resources to promote tissue donation.

**The following is a reflection of our work together.
Thank you for helping save lives faster and bringing us one step closer to curing childhood cancers.**



Kay Koehler

President & CEO, CureSearch for Children's Cancer

Acceleration Initiative project updates | Progress from bench to bedside

Our Acceleration Initiative addresses the largest obstacles in children's cancer research, driving innovative translational research to the clinic – ready to reach the clinic quickly, within three years.



Dr. Mary Beckerle
Huntsman Cancer Institute

Ewing sarcoma treatment moves to Phase 1 clinical trial

Dr. Beckerle identified a novel targeted treatment for Ewing sarcoma, the most common bone cancer in children. The research demonstrated so much potential for its anti-tumor effects, it was licensed to Salarius Pharmaceuticals who is now conducting a Phase 1 trial in Ewing sarcoma.

This trial is [recruiting](#) up to 50 patients at clinical sites across the country including Children's Hospital Los Angeles, Johns Hopkins, Moffitt Cancer Center, Dana-Farber Cancer Institute and MD Anderson Cancer Center.



Dr. Richard Gilbertson
The University of Cambridge

New therapies reach children with brain cancers

Dr. Gilbertson developed new therapies for medulloblastoma, ependymoma and choroid plexus carcinoma (CPC). This critical research provided a new roadmap to identify therapeutic regimens based on tumor subtypes.

A discovery in CPC has resulted in the Gilbertson lab partnering with AstraZeneca to move a new drug into clinical trials. Importantly, preclinical trials with the drug Ribociclib, in combination with the drug Gemcitabine, have supported the development of a Phase I clinical trial that opened at St. Jude in February of 2018.



Dr. Andrew Kung
Memorial Sloan Kettering
Cancer Center

Pediatric sarcoma trial to begin enrolling patients this year

Research conducted by Dr. Kung is now supporting a Phase 2 study of the drug Selinexor in the pediatric kidney cancer Wilms tumor and malignant rhabdoid tumor.

The trial will begin enrolling patients at Memorial Sloan Kettering Cancer Center within the next few months, and Dr. Kung and colleagues are in the process of opening the trial at other national sites.

Catapult Award Project Updates | Propelling research out of the lab

Our Catapult Awards propel high-potential research out of the lab, into the clinic, and ultimately to the kids who need it most. These awards uniquely fund cutting-edge Phase 1 or Phase 2 clinical trials that begin enrolling patients in less than a year. Every research applicant must provide a development plan that demonstrates their ability to move into later-phase clinical trials if successful.



Dr. Crystal Mackall
Stanford University

CAR-T cell therapy shows promise for DIPG/DMG patients

Dr. Mackall is leading a Phase 1 clinical trial to treat diffuse intrinsic pontine glioma (DIPG) and diffuse midline glioma (DMG), which are universally fatal with no current treatments that extend life beyond a median of nine months. In February of 2022, the team published promising early results, demonstrating an ability to treat the tumors, significantly reduce symptoms and extend patients' lives in a way not seen before in DIPG.

"I felt for the first time that we were going to be able to cure this disease someday," said Michelle Monje, MD, PhD, who shares senior authorship of the new research with Mackall.



Patients have also shown a significant amount of functional recovery. The mother of a five-year-old patient sent Dr. Mackall a video of her daughter, who has been treated for over a year, riding a bike.

“***We didn't realize how dramatic the clinical improvement would be for these patients... The findings are a hopeful sign for many types of brain tumors, not just this one.***”

- Dr. Mackall, [article](#) in Stanford Medicine publication: SCOPE

Catapult Award Project Updates | Propelling research out of the lab (cont.)



Dr. Ranjit Bindra
Yale University

Novel drug screening platform leads to high-grade glioma trial

In 2017, supported by a CureSearch Young Investigator Award, Dr. Bindra created an automated screening process for drugs that target a particular gene fusion that leads to rhabdomyosarcoma, a soft tissue cancer. Dr. Bindra is now testing a novel drug in pediatric patients with a specific subtype of chemotherapy-resistant glioma. Now using his CureSearch Catapult Award, Dr. Bindra has treated his first six patients and, over the next six months, his team will recruit additional patients for the second dose cohort of the trial.

"I cannot think of any other pediatric cancer foundation that operates at the level of CureSearch. Their research strategy will benefit children by getting new therapies faster into the clinic."

- Dr. Bindra



Dr. Elias Sayour
University of Florida Health

Sayour lab reaches a milestone in the development of a breakthrough immunotherapy for pediatric high-grade glioma

Dr. Sayour and his colleagues developed a novel treatment platform leveraging the use of nanoparticles (NPs) combined with messenger RNA (mRNA). Previously not thought possible, this novel engineering overcomes previous limitations regarding mRNA packaging into nanoparticles- a remarkable milestone for children with glioma- and opens up opportunities across the cancer spectrum globally. In early September

2022, Dr. Sayour validated a starting phase 1 dose level for pediatric patients, and is now seeking to enroll pediatric glioma patients at the University of Florida. The project is on track to then enroll pediatric patients throughout the country shortly thereafter in collaboration with the Pacific Pediatric Neuro-Oncology Consortium (PNOC).

“The first grant is the most important...and this is the first clinical trial grant we have received; it is the breath of life for all of my clinical work.”

- Dr. Sayour



Next-generation research underway | Our largest funding commitment in recent history

This year, we announced more than \$4.3 million in new translational research funding, supporting investigators in the U.S. and abroad.

YOUNG INVESTIGATOR AWARDS

Our Young Investigator Awards support the next generation of pediatric cancer researchers. These grants are limited to truly transformational science designed to drive innovative treatments toward clinical trials.



Jamie Anastas, PhD
Baylor College of Medicine
Developing a novel therapy for pediatric high-grade glioma by correcting faulty DNA packaging.



Joanna Gell, MD
Connecticut Children's
Collaborating with IBM on the development of a targeted compound for the germ cell tumor marker PRDM14.



Francesca Nazio, PhD
Ospedale Pediatrico Bambino Gesù
Enhancing the immune system's natural ability to fight cancer to treat medulloblastoma.

ACCELERATION INITIATIVE AWARDS



Gregory Friedman, MD
University of Alabama at Birmingham
Developing a combination immunotherapy to improve outcomes in pediatric high-grade glioma.



Andrei Thomas-Tikhonenko, PhD
Children's Hospital of Philadelphia
Discovering new targets for immunotherapy in the most devastating pediatric cancers.



David Langenau, PhD
Massachusetts General Hospital
Developing a novel therapy for a previously undruggable target in rhabdomyosarcoma.

Strategic collaborations | The key to long-term change

We create connections and community among leaders in science, academia, regulatory, funding and industry by providing platforms for engagement. These critical conversations help drive innovation.

The 2021 CureSearch Summit – The State of Solid Tumor Biopsies – addressed the issue of limited or inaccessible patient tissue samples.

Based on engaging panel presentations and attendee discussions, we are working with global stakeholders and volunteer leaders to:

- Encourage tissue donation
- Communicate the availability of pediatric oncology tissue to academic and industry colleagues for research use
- Promote widespread utilization of new technologies for data collection and dissemination



View the 2021 Summit White Papers to learn more:

<https://curesearch.org/publications>



The next Summit will explore how Artificial Intelligence and Machine Learning can impact the field of pediatric oncology.

“As a community we have a responsibility to educate the next generation of clinician-scientists, through medical school training, through educational initiatives and through symposia like Summit.”

- Dr. Samuel Volchenbom
Associate Chief Research Informatics Officer
The Division of Biological Sciences at the University of Chicago

Learn more and watch our Summit kick-off webinar:

<https://curesearch.org/2023-curesearch-summit>

Pediatric Early Development Symposium: Bringing children to the forefront of drug development

PEDS addresses the challenges of pediatric cancer clinical trial design.

Launched in 2020 in response to the passage of the RACE for Children Act (Title V of the FDA Reauthorization Act), the CureSearch Pediatric Early Development Symposium (PEDS) is a unique opportunity to examine the dynamic nature of clinical trials for children with cancer.

The most effective way to coordinate and implement cutting-edge strategies is to engage leaders across the pediatric oncology space. Using a robust interactive virtual platform, participants discuss challenges and propose solutions to technology integration within their work, and brainstorm opportunities to craft the future of clinical trials..



PEDS 2022 took place virtually this October 12-14 and explored global technological efforts to improve clinical trial design and implementation.

Big data technology is revolutionizing every aspect of our lives and healthcare is no different. Important efforts for improving clinical trials – especially in diseases with small patient populations – include global coordination, integration of innovations like decentralized infrastructure, real-world evidence and external controls.

Learn more at <https://curesearch.org/2022-peds>.

“

When it comes to navigating the human-to-digital divide, we are only starting to scratch the surface of understanding the dynamic real-time-use of data in pediatric oncology clinical data"

- Adam Resnick
Director of Center for D3b
Children's Hospital of Philadelphia

Volunteer impact | Our driving force



“
I think if you're 80 years old, or 75 years old, and if you had cancer, you lived a lot of life, but when you're just 4 or 5 years old, you haven't even gotten started yet. I think that's where we've got to raise money to help these kids have a full life. That's important.

- Frank Gioscia
Ultimate Hike volunteer since 2010

Ultimate Hike raises record-breaking funds

To date, Ultimate Hikers have raised over \$9 million in support of CureSearch's mission to end childhood cancer. Our training program ensures participants of all fitness levels will be prepared for the adventure.

In 2021, the Ultimate Hike program raised more than one million dollars! This was possible because of our dedicated hikers like Frank Gioscia, who goes above and beyond every year to raise money for childhood cancer research. Frank has raised more than \$200,000 since 2010. Join us at ultimatehike.org.

Jakefest events raise \$5M for childhood cancer research

For 15 years, golfers across the country have participated in Jakefest, a series of annual golf tournaments established by Harry and Robin Koenigs in memory of their son, Jake, who died from rhabdomyosarcoma. Kiewit Corporation, Harry's employer and one of North America's largest construction and engineering organizations, turned their annual employee golf outing into Jakefest.

To date, Jakefest has raised more than \$5 million to support groundbreaking childhood cancer research projects conducted by CureSearch-funded investigators, many of which have already made a significant impact. Learn more at jakefest.org.



"The side effects, secondary cancers and lifelong illnesses that are products of pediatric cancer treatments are nothing short of barbaric. That's why this event is so important and impactful for many kids and their families.

- Robin Koenigs
Jake Koenigs' mother

Funding partners | Amplifying the impact on childhood cancer research

Our strong co-funding model allows us to combat duplication of efforts and leverage a combined funding pool.

Rally Foundation drives new treatments for pediatric high-grade gliomas

Outcomes for children with high-grade gliomas are unacceptably poor, and standard therapies are minimally effective and toxic. The Rally Foundation has continued to support innovative research projects, conducted by CureSearch investigators, that aim to develop new treatments for pediatric high-grade gliomas.

In 2020, CureSearch Catapult Award Recipient Dr. Elias Sayour launched a clinical trial to test a new immunotherapy for pediatric high-grade gliomas, supported in part by the Rally Foundation.

Recently, they helped fund a new CureSearch Acceleration Initiative Award recipient, Dr. Gregory Friedman of the University of Alabama at Birmingham, who is developing a combination immunotherapy to improve outcomes in pediatric high-grade gliomas.

“**The Rally Foundation has continued supporting Dr. Friedman's work and have journeyed with him as his research has gone from science at the bench, to a clinical trial with promising results. We are delighted to partner with CureSearch so that our combined funding advances Dr. Friedman's research even further. Together, we are making a difference for kids fighting cancer.**”

- Dean Crowe, CEO
Rally Foundation

SebastianStrong Foundation supports the next-generation of pediatric cancer researchers

The SebastianStrong Foundation has supported two [CureSearch Young Investigators](#) through its Discovery Science Awards, which are granted to researchers applying out-of-the-box approaches in the quest for less-toxic treatments.

In 2020, the SebastianStrong Foundation, in partnership with CureSearch, funded an exciting project to develop a new treatment approach for DIPG. In 2021, the Sebastian Strong Foundation also funded Young Investigator Dr. Mark Kohler of the University of Colorado Cancer Center. Dr. Kohler is developing two CAR T-cell approaches for acute myeloid leukemia (AML), which will decrease the leukemia cells' ability to avoid treatment and reduce toxicity in children with relapsed AML.

“**Our ultimate goal is to create more potential treatments for kids facing a cancer diagnosis. We want the research that we fund to provide hope so that parents don't have to hear the devastating words that so many parents hear every day: we're out of options.**”

- Oscar Ortiz
Executive Director and
Co-founder of SebastianStrong

Nick Currey Legacy Fund helping to advance Ewing sarcoma research

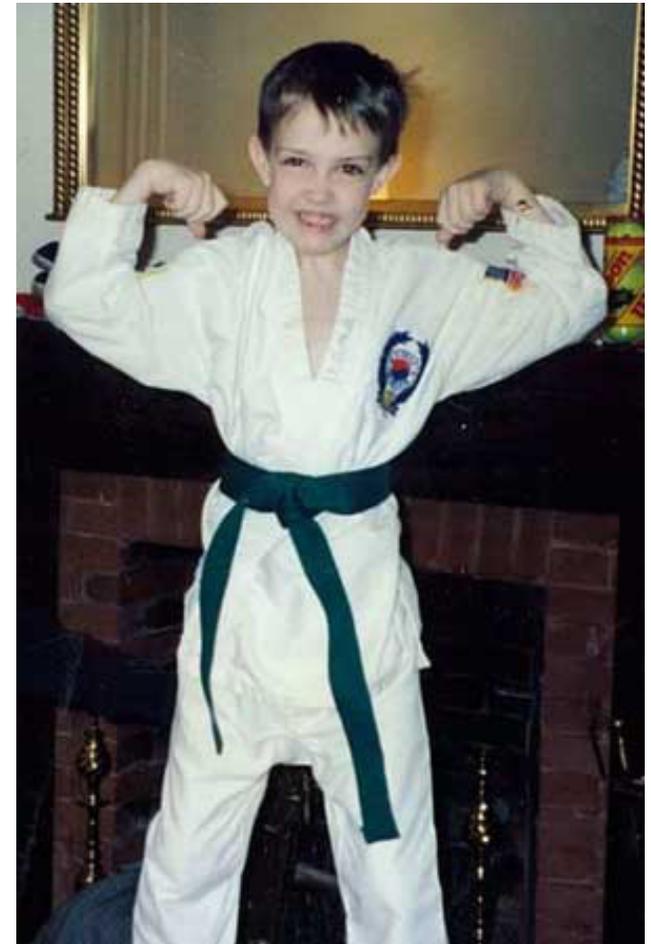
CureSearch Legacy Funds give families the opportunity to honor their child's journey by funding the most promising childhood cancer research. Nancy and Ralph Currey established the Nick Currey Fund to honor their son Nick, who died from Ewing sarcoma.

Established in 2005, the Nick Currey Fund supports CureSearch-funded projects aimed at improving outcomes for children with cancer, including Dr. Mary Beckerle of the University of Utah's Huntsman Cancer Institute and Dr. Andrew Kung of Memorial Sloan Kettering Cancer Center.

Dr. Beckerle's work provided the foundation for a Phase 1 clinical trial that launched in 2018 and Dr. Kung is leading a Phase 2 study of the drug Selinexor in the pediatric kidney cancers Wilms tumor and malignant rhabdoid tumor. The clinical study will begin enrolling patients at Memorial Sloan Kettering Cancer Center within the next few months.

“**We wanted to create a legacy for our son, Nick, who at 19 had just begun his life journey. Our goal is to save the Nicks of the future from the scourge of Ewing sarcoma and other cancers. We are proud to partner with CureSearch in funding projects with the potential to go from the lab, to saving kids as fast as possible.**”

- Nancy and Ralph Currey, Nick's parents



To learn more about creating a legacy fund with CureSearch, please visit: <https://curesearch.org/legacy-funds>

THANK YOU TO OUR CORPORATE PARTNERS

Our impact would not be possible without the extraordinary support of our partners, their generous employees and their customers.



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