



CURESEARCH

FOR CHILDREN'S CANCER

**I M P A C T
R E P O R T**

MARCH 2021

A MESSAGE FROM OUR CEO

Dear CureSearch Community,

At CureSearch, our goal is to ensure that every child diagnosed with cancer has a safe and effective treatment option. Thanks to your support, even in these challenging times we continue to drive the development of promising new therapies and to foster strategic collaborations to bring our kids to the forefront of drug development.



Studies are just beginning to reveal the impact of the COVID-19 pandemic on academic research, but our work has not slowed as we continue to fund cutting-edge research for some of the deadliest forms of pediatric cancer. We are seeing the resilience of the research community as CureSearch-supported investigators continue to hit milestones, and we're proud to share those encouraging updates in this report.

We're also thrilled to share updates on two of our past Acceleration Initiative projects, both of which contributed to the launch of clinical trials enrolling patients in need right now. Our preclinical projects are 5 times more likely than average to advance into clinical trials, and they reach the clinic more than 2 years faster. That speed is critical for families facing cancers with the lowest survival rates and fewest treatment options.

We know that long-term impact will require strategic collaboration among all members of the pediatric cancer ecosystem. Last October, we hosted the inaugural CureSearch Pediatric Early Development Symposium, a unique platform that allowed industry, regulatory, and academic leaders to discuss real world solutions to creating successful pediatric study plans, a timely and critical conversation stemming from the recently implemented RACE for Children Act. This year, we're looking forward to hosting the 2021 CureSearch Summit, a 4-part virtual series focused on the topic of solid tumor biopsies in pediatric cancer research.

Finally, I am proud to share that in 2020, our campaign event participants came together virtually despite the challenges COVID-19 presented to raise more than \$1.5 million in support of our critical mission! Supporters throughout the country formed teams for virtual CureSearch Walks and modified Ultimate Hikes, and created their own DIY Gold events with friends and neighbors.

Thank you for your continued support. Together, we're making a real, measurable impact for the children who are counting on us.

A handwritten signature in black ink that reads "Kay Koehler".

Kay Koehler
President & CEO, CureSearch for Children's Cancer

CURESEARCH-FUNDED CLINICAL TRIALS DELIVER HOPE TO PATIENTS TODAY

High-grade glioma trial now enrolling patients at seven sites: 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.

- Dr. Bindra's clinical trial is now active at the University of California San Francisco, the Dana-Farber Cancer Institute, Washington University St. Louis, Johns Hopkins University, Oregon Health & Sciences University, St. Jude Children's Hospital and Yale University, and he anticipates up to 3 additional sites in the next 6 months.

- Dr. Bindra has begun receiving patient specimens for analysis at Yale as part of a correlative (biomarker) study that will allow him to monitor their response to treatment more effectively.



Ranjit Bindra, MD, PhD
Yale University

High-grade glioma treatment inches closer to trial: Dr. Sayour is testing an innovative personalized immunotherapy for pediatric high-grade gliomas, the primary cause of death in children with brain tumors. **Dr. Sayour's group plans to begin enrolling patients in March 2021.**

As part of CureSearch's growing co-funding model, this \$2.5 million Catapult Award project is supported in part by the Norcross Foundation, the Resonance Foundation for Children's Health co-founded by Sheri Sobrato Brisson and Eric Brisson, and by the Rally Foundation for Childhood Cancer Research.



Elias Sayour, MD, PhD
University of Florida

“Our family believes that the work Dr. Sayour is doing has the potential to be a real game changer in the progress and treatment of these cancers. We look forward to seeing the impact that this project will have on so many children and are proud to support this innovative work.”

- Gary Norcross, President, Norcross Foundation

CURESEARCH-FUNDED CLINICAL TRIALS DELIVER HOPE TO PATIENTS TODAY

Innovative DIPG treatment hits milestone paving the way for patient trials to begin: 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%.

- Dr. Mackall's team opened the trial for enrollment in the summer of 2020 and initiated treatment of patients with DIPG.
- Dr. Mackall has successfully manufactured CAR T-cells for these patients, generating adequate cell doses that met all release criteria and can be used in patients.



Crystal Mackall, MD
Stanford University of Medicine

One brave young DIPG patient shared his story and the early impact of Dr. Mackall's trial in a recent article in The Kansas City Star:

"His hearing improved. He could walk better. His smile evened. He no longer slurred his words. He could swallow again and use his hands to text. Tests showed that part of his tumor had shrunk by 50%, at least temporarily." - Kansas City Star



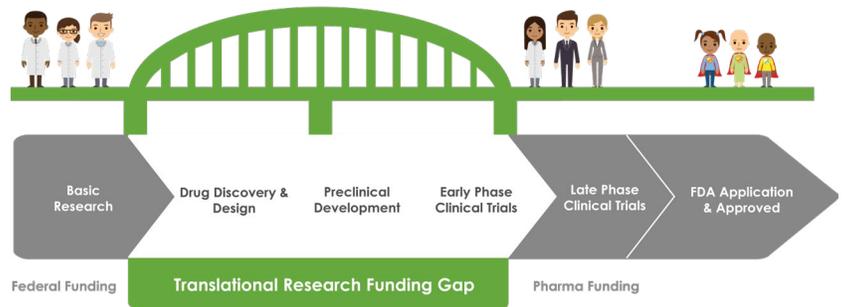
Jace Ward of Wamego, Kansas

Read the full story [here](#).

RESEARCH FUNDING SUCCESS: PROVIDING CRITICAL FUNDS TO DRIVE NEW TREATMENTS INTO CLINICAL TRIALS

Projects funded through our Acceleration Initiative are **5 times more likely than average to advance into clinical trials**, and they reach the clinic more than 2 years faster than average.

CureSearch Research Programs Bridge the Translation Gap



EWING SARCOMA TRIAL NOW ENROLLING PATIENTS NATIONWIDE

A CureSearch Acceleration Initiative award supported the preclinical work of Dr. Mary Beckerle at the Huntsman Cancer Institute at the University of Utah. Dr. Beckerle and her team identified a novel targeted treatment for Ewing sarcoma, a common type of bone cancer in children, providing the preclinical data needed to move this therapy into clinical trials.

After promising results from an FDA-approved single patient compassionate use study of their therapy, Salarius Pharmaceuticals licensed the drug and launched a phase 1/2 clinical trial. **This trial has now determined correct dosing and has demonstrated safety, expanding enrollment to patients with cancers that are similar to Ewing sarcoma by recruiting patients at 8 sites across the country.** Additional information can be found [here](#).

NEW TREATMENT OPTION REACHING MEDULLOBLASTOMA PATIENTS

Dr. Gilbertson received a CureSearch Acceleration Initiative award to discover and develop new therapies for three high-risk pediatric brain cancers: medulloblastoma, ependymoma and choroid plexus carcinoma. Dr. Gilbertson's team tested 1.2 million new and existing drugs and identified several promising therapies to guide immediate clinical testing for pediatric use.

Results of this project contributed to the launch of a phase 1 clinical trial testing combination therapies for malignant brain tumors, with Dr. Gilbertson's research directly impacting patients with recurrent, refractory medulloblastoma. The trial, sponsored by St. Jude Children's Research Hospital, opened in 2019 and is currently enrolling patients. Learn more [here](#).

CURESEARCH YOUNG INVESTIGATORS DRIVE PROMISING NEW TREATMENTS RAPIDLY TOWARD THE CLINIC

MPNST therapy shows promise in stopping tumor growth

- Dr. Lemberg is developing a promising new treatment for malignant peripheral nerve sheath tumor (MPNST), an aggressive tumor that arises from cells that support nerve function.
- Now one year into her project, **Dr. Lemberg has determined that her drug, when used in combination with an additional therapy, is able to stop MPNST growth and prolong animal survival.** This discovery supports the potential to treat MPNST patients.



Kathryn Lemberg, MD
Johns Hopkins University

Neuroblastoma treatment may be effective for other tumor types



Adam Durbin, MD, PhD
St. Jude Children's
Research Hospital

- Dr. Durbin is developing a new, less toxic treatment for neuroblastoma. **Early results suggest that his treatment may also be effective for other pediatric cancers, including rhabdomyosarcoma, T-ALL, AML, and Ewing sarcoma.**
- Over the next 6-12 months, Dr. Durbin will assess potential combination therapies with the aim to increase the effectiveness of treatment while keeping toxicity low.

Collaboration offers potential new treatment for high-risk B-cell ALL

- Dr. Li studies a high-risk subtype of B-cell ALL, occurring in about 15% of patients, that is dependent on a protein called JAK2 but is resistant to classic JAK2 inhibitors.
- Building on strong early results, **Dr. Li is now collaborating with a large pharma company to stop tumor growth using a JAK-2 halting therapy that has been successful in treating other cancers.**



Loretta Li, MD
Ann & Robert H. Lurie
Children's Hospital of Chicago

DRIVING CRITICAL COLLABORATIONS: BRINGING CHILDREN TO THE FOREFRONT OF DRUG DEVELOPMENT

CURESEARCH PEDIATRIC EARLY DEVELOPMENT SYMPOSIUM (PEDS): DEVELOPING IPSPS AND PIPS IN AN EVOLVING REGULATORY LANDSCAPE

The 2020 CureSearch PEDS served as a synergistic platform for driving critical stakeholder collaborations to accelerate the pace of pediatric drug development. Specifically, this meeting addressed the challenges in developing initial Pediatric Study Plans (iPSPs) and Paediatric Investigation Plans (PIPs) with consideration to the changing legislature in this space. This symposium was designed to be a practical workshop for iPSP/PIP development and implementation.

Attendees from academia, industry and regulatory agencies discussed real-life examples on how to meet new FDARA guidelines and support early pediatric drug development. When surveyed, 86% of attendees reported that they will return to their company with tactical learnings, and 93% have a better understanding of developing a successful iPSP/PIP.

Derived from the many discussions, questions from attendees, and group conversations over the two-day Symposium, three strategies will be addressed over the next twelve months.

Read the PEDS Impact Statement [here](#).

Strategy

Promote global collaboration across all roles in pediatric drug development

Provide pediatric drug development resources to stakeholders

Address areas of unmet need in clinical development for children with cancer



“CureSearch is a key link facilitating collaboration between industry and academia.”

Brenda Weigel, MSc, MD
University of Minnesota's
Masonic Cancer Center
PEDS Co-Chair



“Success is found when there is both early and close collaboration between industry sponsors, academic experts, and regulatory authorities. CureSearch has worked hard to bring these groups together in the best interests of children with cancer.”

Sam Blackman, MD, PhD
Day One Biopharmaceuticals
PEDS Co-Chair

HOW YOU CAN MAKE AN IMPACT IN 2021

TELL CHILDREN'S CANCER TO TAKE A HIKE!

We've spent a decade taking hundreds of CureSearch supporters on life-changing, lifesaving adventures through our Ultimate Hike program. Together, we've raised over \$6 million to help fund the most promising childhood cancer research!

Here's where we're hiking in 2021:

Lake Georgetown, Texas

Foothills Trail, South Carolina

Shenandoah Valley, Virginia

Superior Hiking Trail, Minnesota

Ozark Highlands Trail, Arkansas

Pacific Crest Trail, Southern California



With distance options ranging from 5 to 30 miles, all skill levels are welcome! Our expert coaches will get you ready for your ultimate adventure with local training teams in select cities or customized virtual support to train from anywhere. [Learn more at ultimatehike.org](#)

JOIN OUR VIRTUAL 2021 CURESEARCH WALK

The CureSearch Walk brings together families, communities and businesses across the country to unite in support of the 16,000 children diagnosed every day.



The 2021 CureSearch Walk will be held virtually on Saturday, September 25, with in-person events being held in 8 US cities across the country throughout the month of September.

Stay updated and learn more about the virtual and in-person walks at [curesearchwalk.org](#).

FREE RESOURCES FOR PATIENTS, FAMILIES AND CAREGIVERS

CURESEARCH EDUCATIONAL RESOURCES

Video Library: From explaining how chemotherapy cancer treatments are developed and administered, to understanding what an echocardiogram is, our library of videos helps parents and caregivers understand some of the common medical procedures related to children's cancer, as well as advice and tips on taking care of a child undergoing cancer treatments. Access our videos [here](#).



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. Access our clinical trial finder [here](#).



BRAVE BARBIE

Through a longstanding partnership with the Mattel Children's Foundation, we're proud to provide a special, Brave 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.

Requests for dolls can be made [here](#).



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



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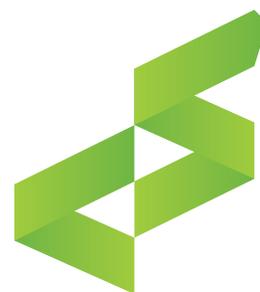
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CureSearch for Children's Cancer

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