



CURESEARCH

FOR CHILDREN'S CANCER

Impact Report

November 2019



Message From Our CEO

At CureSearch, we're working diligently to accelerate the development of new treatments to ensure children, adolescents and young adults survive cancer now and lead long, healthy lives that are free from debilitating side effects. With your support, our efforts are making an impact. I am proud to share exciting highlights of our progress over the past six months.

In September, we announced a \$1.2 million Catapult Award to a renowned immunotherapy researcher. Thanks to your support, our funding will help launch a phase 1 clinical trial to test a promising new treatment option for DIPG, a devastating brain tumor with a 5-year survival rate of less than 1%. We remain dedicated to funding research that addresses these critical unmet needs in pediatric cancer.

Our Acceleration Initiative grants are laser focused on driving new treatments into clinical trials in just 2–5 years, an accelerated timeframe designed to impact children in treatment today. Recently, two of our Acceleration Initiative projects provided the preclinical data necessary to support the initiation of new phase 1 clinical trials — now offering new treatment options to children across the country.

We know that real progress will require a true paradigm shift to prioritize pediatric cancer drug development. Earlier this year, we hosted the fourth annual CureSearch Summit, bringing together thought leaders from all areas of pediatric drug development to drive critical stakeholder collaborations. As a follow up to that meeting, last spring we launched the game-changing CureSearch IMPACT Series, a first-of-its-kind forum driving early conversations between academics and pharma.

Thank you for helping bring kids to the forefront of cancer research. Together, we are creating a brighter future for children diagnosed with cancer.

Sincerely,



Kay Koehler
CEO, CureSearch for Children's Cancer

Driving Critical Conversations in Childhood Cancer

IMPACT Series

In early 2019, we launched the CureSearch IMPACT Series, a first-of-its-kind approach to accelerating the pace of pediatric drug development by driving earlier collaboration between global academics and pharma.

[Learn More](#)



Catapult Summit

The fourth annual CureSearch Catapult Summit took place in May. The Summit serves as a unique platform for fostering collaborations across sectors that traditionally do not occur, with one end goal in mind: creating a world where children diagnosed with cancer go on to lead long, healthy lives. Drawing on key Summit discussions, ideas and feedback, we have identified three key areas of opportunity to drive collaborative impact. In partnership with stakeholders and volunteer leadership, we will execute on the following strategies:

Strategy	Strategy	Strategy
 <p>Accelerate the pace of drug development via critical stakeholder conversations</p>	 <p>Encourage a cohesive co-funding environment to better support innovative projects</p>	 <p>Expand CureSearch relationships with international investigators</p>

Driving new therapies into clinical trials to save kids TODAY



Across the country, we're driving cutting-edge research quickly into clinical trials, providing new treatment options — and hope — for kids facing some of the deadliest forms of childhood cancer.

In 2013, a team at the University of Utah's Huntsman Cancer Institute received a \$1.73 million grant to test a new treatment for Ewing sarcoma. Successful results provided the foundation for a phase 1 clinical trial that launched in 2018 and will enroll up to 50 Ewing sarcoma patients at trial sites across the country. [Learn More ▶](#)

At Cambridge University, Dr. Richard Gilbertson received a CureSearch Acceleration Initiative Award which funded his project to discover and develop new therapies for high-risk pediatric brain cancers. Results of this project contributed to the launch of a phase 1 clinical trial testing combination therapies for malignant brain tumors. Dr. Gilbertson's research directly impacts patients with recurrent, refractory medulloblastoma. The trial, sponsored by St. Jude Children's Research Hospital, opened in March 2019. [Learn More ▶](#)

Catapult Award Project Updates

In September, we announced our 2019 Catapult Award supporting an innovative new treatment for DIPG



Crystal Mackall, MD

Stanford University

Project: Phase I CAR T-cell immunotherapy trial in DIPG

This \$1.2 million award, co-funded by the Parker Institute for Cancer Immunotherapy, will support a clinical trial testing a CAR T-cell therapy in diffuse intrinsic pontine glioma (DIPG), a highly aggressive pediatric brain tumor with a 5-year survival rate of less than 1%.

[Read The Press Release](#)

Dr. Mackall photo credit: Elena Zhukova — Lucile Packard Foundation for Children's Health

First patients enrolled in promising pediatric glioma trial



Ranjit Bindra, MD, PhD

Yale University

Project: Exploiting mutant IDH1/2-induced DNA repair defects in pediatric glioma

[Learn More](#) ▶

Dr. Bindra enrolled his first patient at the Pacific Pediatric Neuro-Oncology (PNO) site at the University of California, San Francisco in early May 2019. The second site opened in August for patient recruitment at the Dana-Farber/Boston Children's Cancer and Blood Disorders Center. Additional sites are expected to open throughout the coming months.

Young Investigator Project Updates

ALL project receives commercial support

Loretta Li, MD

Dana-Farber Cancer Institute

Project: JAK2 inhibition and degradation in B-Cell Acute Lymphoblastic Leukemia

[Learn More ▶](#)

The 5-year survival rate for children with JAK2-dependent B-cell acute lymphoblastic leukemia (B-ALL) can be as low as 35%. Dr. Li is developing a new family of JAK2 inhibitors that will likely prove more effective in treating this subtype, and the promise of these inhibitors has now been recognized by a start-up that has licensed the therapeutics developed in Dr. Li's lab. Once the optimal structure for these inhibitors has been identified, they will be assessed for effectiveness and safety and, ultimately, should quickly move into clinical trials.



Neuroblastoma therapy moving towards preclinical testing

Avery Posey, PhD

University of Pennsylvania

Project: Polysialic acid-specific CAR T-cells for the treatment of neuroblastoma

[Learn More ▶](#)

Nearly 700 patients are diagnosed with neuroblastoma each year in the U.S. and, while the current standard treatment has increased two-year event-free survival by 20%, dosing is limited by adverse side effects such as neuropathic pain. Dr. Posey's goal is to identify a more specific immunotherapy target that could result in better outcomes and fewer side-effects. The optimization and implementation of PolySia immunotherapy has great promise in this arena. To date, Dr. Posey's work has opened up the potential for CAR T-cell therapy in neuroblastoma, demonstrating initial success in cultured cells.



Acceleration Initiative Project Updates

Success contributes to pediatric sarcoma research world



Andrew Kung, MD, PhD

Memorial Sloan Kettering Cancer Center

Project: Integrative analysis to identify new therapies for pediatric sarcoma

[Learn More ▶](#)

Dr. Kung continues to exceed the goals he set upon the initiation of his project. He is contributing invaluable tools and techniques to the pediatric sarcoma research world that will enable the study of a wide range of patient-derived tissues and sequencing data. This contributes to a better understanding of pediatric sarcoma and discovery of potential therapies.

Help us drive critical research for deadly childhood cancers.

Donate Today

Our Cumulative Impact

<p>23 therapies in preclinical testing for:</p>  <ul style="list-style-type: none"> ✓ brain ✓ sarcoma ✓ leukemia ✓ Wilms tumor ✓ neuroblastoma 	<p>300+ potential new drug targets</p>  <p>in 9 types of children's cancer</p>	<p>1 clinical trial for high-grade glioma</p>  <p>in adolescents and young adults enrolling patients at two clinical trial sites</p>
<p>1 novel cell therapy for AML</p>  <p>nearing pediatric clinical trials</p>	<p>1 novel drug for Ewing sarcoma</p>  <p>in pediatric clinical trials</p>	<p>1 new treatment for recurrent, refractory medulloblastoma</p>  <p>in pediatric clinical trials</p>

Tumor types that our investigators have studied:			
5 sarcoma	4 brain	6 leukemia	1 liver
2 kidney	1 lymphoma	3 neuroblastoma	1 rare tumor of the head and neck

Upcoming Research Funding

Our research programs sparked international interest from academic institutions and biotech companies.



Earlier in the year, we invited researchers to apply for funding by submitting a Letter of Intent (LOI) for one of our research programs.



Young Investigator

20 LOIs received from
17 institutions in
2 countries



Acceleration Initiative

23 LOIs received from
20 institutions
across the U.S.



Catapult Awards

11 LOIs received from
10 institutions or small pharma/biotech from
3 different countries

Total: **54** LOIs received from

35 academic institutions or small pharma/biotech companies in

3 different countries (U.S., Canada, Australia)

Fundraising To Make An Impact

This year, our campaign event participants have raised over \$1.4 million and counting! Supporters across the country are making an impact by hitting trails for an Ultimate Hike, forming teams for a local CureSearch Walk, adding meaning to their miles with CureSearch Challenge, or creating their own DIY fundraiser through CureSearch Gold.



BronyCon

Gold event BronyCon had its most successful year yet, raising nearly \$100,000



 **CURESEARCH ULTIMATE HIKE**

Foothills Ultimate Hike breaks records this year, raising over \$268,000



 **CURESEARCH WALK**

Over \$300,000 raised at walks across the country

Learn more or join an event near you by visiting curesearch.org/events. ▶



Announcing our newest fundraising program, Trek for 43!

We're excited to introduce our newest community fundraising event, Trek for 43! During National Take a Hike Day weekend this November 15-17, create or join a Trek for 43 event in your community to honor the 43 kids diagnosed with cancer each day.

Learn more at trekfor43.org. ▶

Expanding Our Expert Volunteer Leadership

We're proud to welcome two new members to our Scientific Advisory Council

Dr. Scott Armstrong, Chairman of the Department of Pediatric Oncology at Dana-Farber Cancer Center and Dr. Brenda Weigel, Director of the Division of Pediatric Hematology/Oncology at the University of Minnesota's Masonic Cancer Center have joined the CureSearch Scientific Advisory Council.

The council includes leading pediatric oncologists and cancer researchers who help to set the academic priorities for CureSearch research initiatives and evaluate projects on scientific merit.



Dr. Scott Armstrong

Chairman of the Department of Pediatric Oncology

Dana-Farber Cancer Center



Dr. Brenda Weigel

Director of the Division of Pediatric Hematology/Oncology

University of Minnesota's Masonic Cancer Center

[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.



facebook.com/curesearch



twitter.com/curesearch



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linkedin.com/company/curesearchforchildrenscancer



youtube.com/curesearchnccf

We're moving! Our new address:

4800 Hampden Lane, Suite 200, PMB 64
Bethesda, MD 20814

Donations can be addressed to:

CureSearch for Children's Cancer
PO Box 45781, Baltimore, MD 21297-5781



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