



Curtana Pharmaceuticals Announces Breakthrough in Pediatric Brain Cancer Treatment with Dual Studies Published in Nature Communications

Austin, TX – February 4, 2025 – Curtana Pharmaceuticals, a biotechnology company focused on improving outcomes for brain cancer patients, is proud to announce the publication of two pioneering studies in *Nature Communications*. These studies highlight the potential of CT-179, a first-in-class OLIG2 inhibitor, in treating Sonic Hedgehog (SHH) subgroup medulloblastoma, a prevalent form of pediatric brain cancer. The findings represent a significant milestone in the fight against brain cancer and underscore the power of international collaboration.

Global Research Collaboration

The studies were conducted through an international collaboration involving leading scientists from institutions in Canada, Australia, Korea, Sweden, and the United States. The research was led by Peter Dirks, MD, PhD, FRCSC, Professor of Surgery and Molecular Genetics, University of Toronto and Neurosurgeon-in-Chief and Senior Scientist at the Hospital for Sick Children (SickKids); Bryan W. Day, Professor and Group Leader, QIMR Berghofer Medical Research Institute in Brisbane and Co-Director of the Children's Brain Cancer Centre; and Timothy R. Gershon MD, PhD, Pediatric Neurologist, Children's Healthcare of Atlanta, Professor of Pediatrics and Human Genetics and Director, Children's Center for Neurosciences Research, Emory University School of Medicine.

Key Findings

The first study, [*OLIG2 Mediates a Rare Targetable Stem Cell Fate Transition in Sonic Hedgehog Medulloblastoma*](#), by Desai et al. with Dr. Dirks as the senior author explores the role of OLIG2 in regulating stem cell fate transitions within SHH medulloblastoma. The research demonstrates that inhibiting OLIG2 with CT-179 effectively blocks tumor growth and recurrence by preventing cancer stem cells from transitioning to proliferative states. This groundbreaking discovery highlights OLIG2 as a critical driver of tumorigenesis and a promising therapeutic target.

The second study, [*Suppressing Recurrence in Sonic Hedgehog Subgroup Medulloblastoma Using the OLIG2 Inhibitor CT-179*](#), with Dr. Gershon and Prof. Day as the senior authors builds on these findings by evaluating the therapeutic potential of CT-179 in preclinical models. The study shows that CT-179 not only significantly prolongs survival but also enhances the efficacy of radiotherapy when used in combination. These results suggest that CT-179 could play a pivotal role in improving patient outcomes by targeting cancer stem cells that drive tumor recurrence.

Leadership Perspectives

Dr. Gregory Stein, President and CEO of Curtana Pharmaceuticals, stated: "These publications represent a major step forward in our mission to develop transformative therapies for brain cancer. The multiple collaborations with leading brain cancer researchers exemplify our commitment to advancing science through global partnerships."

Dr. Peter Dirks commented: "Our findings offer a novel strategy to target cancer stem cells, providing hope for more effective treatments against aggressive brain tumors."



Professor Bryan Day added: "The ability of CT-179 to cross the blood-brain barrier and target OLIG2-expressing cells opens new avenues for therapy that could significantly improve patient outcomes."

Dr. Tim Gershon commented: "The ability of CT-179 to disrupt OLIG2 function represents a significant advance in our understanding of how to effectively target tumor stem cells. This approach not only holds promise for treating SHH medulloblastoma but also sets a precedent for targeting similar pathways in other cancers."

Implications and Future Directions

The implications of these studies are profound. By targeting OLIG2-expressing cancer stem cells, CT-179 addresses one of the primary challenges in treating SHH medulloblastoma—tumor recurrence. This innovative approach has the potential to transform treatment paradigms for pediatric brain cancer.

Curtana Pharmaceuticals plans to advance CT-179 into clinical trials to evaluate its safety and efficacy in human patients. The company is also exploring combination therapies that could further enhance treatment outcomes.

About Curtana Pharmaceuticals

Curtana Pharmaceuticals, founded in 2013, is a privately held, preclinical-stage biopharmaceutical company headquartered in Austin, Texas. Current investors include Thynk Capital, angelMD, Biosense Global, DEFTA Partners, and other anonymous investors. The company was also awarded a \$7.6 million grant from the Cancer Prevention and Research Institute of Texas (CPRIT). Curtana focuses on the development of novel first-in-class, small molecule therapeutics targeting cancer stem cells in the central nervous system for the treatment of glioblastoma, medulloblastoma, and other brain cancers. For more information, visit <https://www.curtanapharma.com/>.

About The Hospital for Sick Children (SickKids)

The Hospital for Sick Children (SickKids) is recognized as one of the world's foremost pediatric health-care institutions and is Canada's leading center dedicated to advancing children's health through the integration of patient care, research and education. Founded in 1875 and affiliated with the University of Toronto, SickKids is one of Canada's most research-intensive hospitals and has generated discoveries that have helped children globally. Its mission is to provide the best in complex and specialized family-centered care; pioneer scientific and clinical advancements; share expertise; foster an academic environment that nurtures health-care professionals; and champion an accessible, comprehensive and sustainable child health system. For more information, please visit www.sickkids.ca.

About QIMR Berghofer Medical Research Institute

QIMR Berghofer is a world-leading, translational medical research institute based in Brisbane, Australia. Established in 1945, the Institute is home to almost 1,000 scientists, clinician-scientists, support staff, and students working across four key research programs of Cancer Research, Infection and Inflammation, Population Health, and Brain and Mental Health. Its state-of-the-art facilities include Q-Gen Cell Therapeutics, which manufactures cell therapies. QIMR Berghofer seeks to deliver better health and wellbeing through impactful medical research that responds to the foremost health challenges of our time. For more information, visit www.qimrberghofer.edu.au.



About Emory University School of Medicine

The Emory University School of Medicine is a leading institution renowned for its excellence in education, biomedical research, and patient care. Founded in 1854, it consistently ranks among the nation's top medical schools. The school boasts 3,599 full- and part-time faculty members and 994 volunteer faculty. With 605 medical students and 1,414 residents and fellows training in 118 accredited programs, Emory offers a diverse and comprehensive medical education. The school is particularly noted for its work in infectious diseases, brain health, heart disease, cancer, transplantation, orthopedics, pediatrics, renal disease, ophthalmology, and geriatrics. For more information, visit www.med.emory.edu.

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