

ASX RELEASE

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ST JUDE STUDY OF GDC-0084 IN DIPG SUCCESSFULLY COMPLETES FIRST STAGE; SECOND PART OF STUDY NOW UNDERWAY

Sydney, 12 September 2019 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to announce that St Jude Children’s Research Hospital has successfully completed the first stage of its ongoing phase I study of Kazia’s investigational new drug, GDC-0084 in diffuse intrinsic pontine glioma (DIPG) and other diffuse midline gliomas, which represent a group of childhood brain cancers with high unmet need. The study has determined an appropriate dose for paediatric use, and will now advance into the second stage of the study, which is designed to explore preliminary signals of efficacy in this disease.

Dr. Christopher Tinkle, co-Lead Investigator on the study, commented, “to date, thirteen patients have received GDC-0084 in our study. We have determined a maximum tolerated dose (MTD) in the pediatric setting of 27 mg/m², which we would expect to be approximately equivalent to the doses currently being explored in adult studies. The safety profile of the drug in children appears to be broadly similar to that in adults, and we have encountered no unexpected findings in this regard. It would be premature to draw conclusions regarding potential efficacy, but we now embark on an expansion cohort to explore the clinical effects of the drug in this patient group. My colleagues and I are very pleased with progress and look forward to reporting further data from the study.”

Key Points

- St Jude Children’s Research Hospital is conducting a phase I human trial of Kazia’s GDC-0084 in diffuse intrinsic pontine glioma (DIPG) and other diffuse midline gliomas. The study is registered on clinicaltrials.gov as NCT03696355.
- The St Jude study is led by Dr. Christopher Tinkle, Assistant Member in the St Jude Department of Radiation Oncology, and Dr. Amar Gajjar, Chair of the St Jude Department of Pediatric Medicine and co-leader of the Brain Tumor Program.
- The study commenced recruitment in November 2018. To date, thirteen patients have received treatment with GDC-0084.
- The first part of the study was designed to determine a maximum tolerated dose (MTD) in the pediatric setting. The study will now proceed into an expansion cohort, designed to seek preliminary signals of efficacy.
- The first part has now completed and has reported a pediatric MTD of 27 mg/m².

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Mr Iain Ross Chairman, Non-Executive Director

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Dr James Garner Chief Executive Officer, Managing Director

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Kazia CEO, Dr James Garner, commented, “DIPG is a devastating illness with a poor prognosis and a paucity of effective treatments. It has been a privilege to support the St Jude team on this incredibly important project. The study has recruited well ahead of our expectations, and we are delighted to have the first part successfully completed. Toxicity can be a significant challenge in the treatment of childhood cancer, so it is very encouraging that GDC-0084 appears to have an acceptable tolerability profile. We are excited to see further data in due course.”

Background

Diffuse midline gliomas, including DIPG, represent one of the most aggressive childhood cancers, and several hundred new cases are diagnosed in the United States each year. Median life expectancy from diagnosis is estimated to be 9-11 months, and there is no approved pharmacological therapy.

The PI3K pathway is frequently activated in these tumours, and so there is a persuasive rationale to explore a brain-penetrant PI3K inhibitor such as GDC-0084. GDC-0084 has shown evidence of activity against a wide range of DIPG cell lines in work undertaken by Professor Matt Dun’s team at the University of Newcastle.

St. Jude Children’s Research Hospital, based in Memphis, Tennessee, is leading the way the world understands, treats and cures childhood cancer and other life-threatening diseases. It is the only National Cancer Institute-designated Comprehensive Cancer Center devoted solely to children. Treatments developed at St. Jude have helped push the overall childhood cancer survival rate from 20 percent to 80 percent since the hospital opened more than 50 years ago. St. Jude freely shares the breakthroughs it makes, and every child saved at St. Jude means doctors and scientists worldwide can use that knowledge to save thousands more children. Families never receive a bill from St. Jude for treatment, travel, housing and food — because all a family should worry about is helping their child live. To learn more, visit stjude.org or follow St. Jude on social media at [@stjuderesearch](https://twitter.com/stjuderesearch).

Growing Focus on DIPG

In the past twelve months, Kazia has devoted increasing resources to investigating GDC-0084 in DIPG. Aside from the phase I clinical trial at St Jude, Kazia has an ongoing collaboration with Professor Matt Dun’s team at the University of Newcastle. Professor Dun and his colleagues are undertaking cutting-edge preclinical research with GDC-0084 in DIPG.

In addition, Kazia has recently been pleased to launch a preclinical collaboration with the University Children’s Hospital in Zurich, under the oversight of Dr. Javad Nazarian, Head of the DIPG Research Institute. Dr. Nazarian’s research will examine precision medicine approaches to the potential use of GDC-0084 in DIPG.

In August 2019, Kazia was proud to sponsor the biannual DIPG Symposium, which was held in Sydney, Australia.

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About Kazia Therapeutics Limited

Kazia Therapeutics Limited (ASX: KZA, NASDAQ: KZIA) is an innovative oncology-focused biotechnology company, based in Sydney, Australia. Our pipeline includes two clinical-stage drug development candidates, and we are working to develop therapies across a range of oncology indications.

Our lead program is GDC-0084, a small molecule inhibitor of the PI3K / AKT / mTOR pathway, which is being developed to treat glioblastoma multiforme, the most common and most aggressive form of primary brain cancer in adults. Licensed from Genentech in late 2016, GDC-0084 entered a phase II clinical trial in 2018. Initial safety data was released in May 2019, and efficacy data is expected in 2H 2019. GDC-0084 was granted orphan designation for glioblastoma by the US FDA in February 2018.

TRX-E-002-1 (Cantrixil), is a third-generation benzopyran molecule with activity against cancer stem cells and is being developed to treat ovarian cancer. TRX-E-002-1 is currently undergoing a phase I clinical trial in Australia and the United States. Initial data was presented at the AACR annual conference in April 2019 and the study remains ongoing. Cantrixil was granted orphan designation for ovarian cancer by the US FDA in April 2015.

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