ASX RELEASE

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KAZIA ENTERS CLINICAL COLLABORATION WITH DANA-FARBER CANCER INSTITUTE FOR PRIMARY CNS LYMPHOMA

Sydney, 22 September 2020 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to announce that it has entered into a collaboration with Dana-Farber Cancer Institute (DFCI) in the United States, to investigate the use of Kazia's investigational new drug, paxalisib (formerly GDC-0084), in primary central nervous system (CNS) lymphoma, a potential new indication for the drug.

Key Points

- Lymphoma is a cancer of white blood cells. It occurs in the lymphatic system and can spread almost anywhere in the body; primary CNS lymphoma (PCNSL) occurs exclusively in the brain and central nervous system
- The PI3K inhibitor class is well validated in lymphoma outside the brain; three of the four FDA-approved PI3K inhibitors are treatments for forms of lymphoma, but they are assumed ineffective for PCNSL since they cannot cross the blood-brain barrier
- DFCI will initiate an open-label phase II clinical trial of paxalisib in PCNSL
- The study is expected to recruit up to 25 patients, taking up to 2 years to complete
- Kazia will provide support including study drug and a financial grant
- This study will be the sixth ongoing clinical trial of paxalisib in brain cancer

Dana-Farber Cancer Institute (DFCI) is a world-leading cancer treatment and research centre, based in Boston, Massachusetts. It is a principal teaching affiliate of Harvard Medical School and has been designated a Comprehensive Cancer Center by the US National Cancer Institute. DFCI participates in as many as 600 clinical trials at any given time and has been an important contributor to the development of many important new cancer therapies.

Kazia CEO, Dr James Garner, commented, "this is an exciting new opportunity for the paxalisib program. We are delighted to support the team at Dana-Farber to explore the potential for paxalisib to benefit patients with PCNSL. Dana-Farber is one of the world's leading centres of excellence in this disease, so we are immensely fortunate to be working with them. We are pleased also to see a new and important target added to the broader paxalisib clinical program, and we look forward to seeing the project commence."

Kazia's financial support to the study will use a portion of the funds contributed by shareholders in the Share Purchase Plan (SPP) conducted in May 2020.

Primary CNS Lymphoma

Lymphoma is a haematological malignancy (blood cancer) that originates from lymphocytes, a type of white blood cell involved in the immune system. PCNSL is a specific form of the disease that originates in the brain and central nervous system.

Three of the four PI3K inhibitors approved by the US Food and Drug Administration (FDA) are treatments for various forms of lymphoma, provide a strong validation for PI3K as a target in this disease. Paxalisib is the only PI3K inhibitor in mainstream development with the ability to penetrate the blood-brain barrier, and as such has a unique rationale for development in PCNSL.

PCNSL accounts for approximately 4% of brain tumours, and the incidence is increasing with time. Patients are typically in their 60s or older, and the disease is slightly more common in men.[1] The mainstays of treatment comprise chemotherapy and radiotherapy, but recurrence is common and only approximately 30% of patients remain alive five years after diagnosis.[2] Many of the drugs used to treat lymphoma elsewhere in the body are ineffective in PCNSL due to their inability to cross the blood-brain barrier.

Clinical Trial Design

Dana Farber Cancer Institute will launch a single-arm phase II clinical trial in patients with relapsed or refractory PCNSL, who are resistant to existing treatments. The primary endpoint will be to assess efficacy via overall response rate (ORR), which measures the ability of paxalisib to shrink tumours. Safety and other efficacy endpoints will also be captured. The study will also examine tissue and cerebrospinal fluid samples to identify potential predictors of response.

The principal investigator for the study is Dr Lakshmi Nayak, Director of the CNS Lymphoma Center at Dana-Farber Cancer Institute. Dr Nayak is an Assistant Professor of Neurology at Harvard Medical School and a board-certified neuro-oncologist. Her research interests focus on metastatic brain cancer, glioblastoma, and PCNSL, and she is extensively published in the field of brain cancer. She has been an investigator for multiple clinical trials of experimental drugs in this disease area.

Commencement of recruitment to the study is expected in early CY2021, but is subject to receiving necessary approvals from FDA and from institutional review boards. The study will be listed on clinicaltrials.gov closer to the commencement of recruitment.

[1] J Rubenstein et al. (2008) *Leuk Lymphoma* 49(0 1):43-51
[2] MS Shiels et al. (2016) *Br J Haematol*. 174(3):417-424

Dana-Farber Cancer Institute

The CNS Lymphoma Center (CNSLC) at Dana-Farber / Brigham and Women's Cancer Center is the first centre of its kind in the world dedicated to providing comprehensive care and research for patients with primary or secondary CNS lymphoma. With the most advanced treatment options available — including surgery, chemotherapy, stem cell transplant, radiation therapy, immunotherapy, and targeted therapies, and with extensive clinical trial options — CNSLC is uniquely qualified to treat patients with CNS lymphoma and advance the outcome of patients with these tumors.

Other clinicians at Dana-Farber Cancer Institute are currently involved in clinical trials of paxalisib in other forms of brain cancer. Dr Jose Pablo Leone is the principal investigator on a phase II clinical trial of paxalisib in combination with Herceptin (trastuzumab) for breast cancer brain metastases (breast cancer that has spread to the brain). Professor Patrick Wen has been centrally involved in the ongoing phase II study of paxalisib in glioblastoma, and plays a leadership role in the international GBM AGILE study.

Paxalisib Clinical Program

The initiation of this trial in PCNSL brings the number of ongoing clinical studies of paxalisib in brain cancer to six.

Indication	Phase	Sponsor	Registration
Glioblastoma	II	Kazia Therapeutics	NCT03522298
DIPG	I	St Jude Children's Research Hospital	NCT03696355
Breast Cancer Brain Metastases (with trastuzumab)	II	Dana-Farber Cancer Institute	NCT03765983
Brain Metastases	II	Alliance for Clinical Trials in Oncology	NCT03994796
Brain Metastases (with radiotherapy)	I	Memorial Sloan-Kettering Cancer Center	NCT04192981
Primary CNS Lymphoma	II	Dana-Farber Cancer Institute	(TBD)

Next Steps

Recruitment to this study is expected to commence in 1H CY2021.

Kazia completed recruitment to a phase II clinical trial of paxalisib in newly-diagnosed glioblastoma in February 2020, and interim clinical data was presented at the AACR Virtual Annual Meeting II in June 2020. Overall survival was calculated at 17.7 months, which compares favourably to an historical figure of 12.7 for temozolomide, the existing FDA-approved standard of care. Kazia expects to present further data from this study in 2H CY2020, and to conclude the study in early CY2021.

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 paxalisib (formerly GDC-0084), a small molecule inhibitor of the PI3K / AKT / mTOR pathway, which is being developed to treat glioblastoma, the most common and most aggressive form of primary brain cancer in adults. Licensed from Genentech in late 2016, paxalisib entered a phase II clinical trial in 2018. Interim data was reported most recently at AACR in June 2020, and further data is expected in 2H 2020. Four additional studies are ongoing in other forms of brain cancer. Paxalisib was granted Orphan Drug Designation for glioblastoma by the US FDA in February 2018, and Fast Track Designation for glioblastoma by the US FDA in August 2020. In addition, paxalisib was granted Rare Pediatric Disease Designation and Orphan Designation by the US FDA for DIPG in August 2020.

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 has completed a phase I clinical trial in Australia and the United States with the final data expected in the second half of calendar 2020. Interim data was presented most recently at the AACR conference in June 2020. Cantrixil was granted orphan designation for ovarian cancer by the US FDA in April 2015.

This document was authorized for release to the ASX by James Garner, Chief Executive Officer, Managing Director.