ASX RELEASE
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KAZIA TO COLLABORATE WITH PACIFIC PEDIATRIC NEURO-ONCOLOGY CONSORTIUM (PNOC) FOR NEW PAXALISIB COMBINATION STUDY IN DIPG

Sydney, 10 December 2020 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to announce that it has executed a Letter of Intent with the Pacific Pediatric Neuro-Oncology Consortium (PNOC) to launch a clinical trial of multiple therapies, including Kazia's investigational new drug, paxalisib (formerly GDC-0084), in diffuse midline gliomas including diffuse intrinsic pontine glioma (DIPG).

The new clinical trial, PNOC022, will employ an adaptive trial design to test several therapies in different combinations and in different subsets of patients. In addition to paxalisib, the other therapies involved will initially include ONC201, manufactured by Oncoceutics, Inc, and panobinostat, manufactured by SecuraBio, Inc. The study is expected to open initially in the United States and will then expand to other countries during CY2021.

Key Points

- PNOC022 uses cutting edge clinical trial design to efficiently and rapidly evaluate combination therapies in DIPG, under the leadership of world experts in the field
- Lead investigator is Professor Sabine Mueller, a leading paediatric neuro-oncologist and co-founder of PNOC
- Study is guided by Australian research at University of Newcastle, under leadership of Associate Professor Matt Dun, who serves as a scientific advisor
- Combination approach builds on recent positive data from St Jude SJPI3K study (NCT03696355) with paxalisib as single agent in DIPG, and brings together several of the most promising candidates in the global pipeline for DIPG
- Kazia will provide paxalisib investigational product; study is fully funded by PNOC

Kazia CEO, Dr James Garner, commented, "DIPG and diffuse midline gliomas have emerged as an exciting second front in the development of paxalisib as a brain cancer therapy. Work by Dr Chris Tinkle and colleagues at St Jude Children's Research Hospital has taught us a great deal about how to use this drug in a paediatric population. In parallel, extensive laboratory research by Associate Professor Matt Dun and colleagues has generated a rich and comprehensive data set to inform combination use. We are delighted to now have the

opportunity to work with the PNOC team to bring these insights together and to take paxalisib into the next chapter of its development as a potential therapy for DIPG."

Clinical Trial Design

PNOC022 will enrol children and young adults with diffuse midline gliomas, a category of brain tumours that includes DIPG. The study will include separate cohorts comprising patients with newly diagnosed disease, patients who have completed initial radiotherapy, and patients who have experienced disease progression after treatment.

At the outset, all patients will be treated with ONC201, combined with either paxalisib or panobinostat. The study employs an adaptive design, in which different arms will opened and closed based on emerging preclinical and clinical data. The primary endpoint will be the proportion of patients progression-free at six months (PFS6) for newly diagnosed patients, and overall survival (OS) for recurrent patients.

The lead investigator will be Professor Sabine Mueller, a board-certified neurologist and paediatric neuro-oncologist whose research focuses on novel therapies in childhood brain cancer. Professor Mueller holds an academic appointment in the Department of Neurology, Neurosurgery and Pediatrics at the University of California, San Francisco (UCSF) and serves as head of the clinical programme at the DMG Centre at the Children's Hospital of the University of Zurich. She obtained her medical degree from the University of Hamburg, and also holds a PhD in molecular biology.

Professor Mueller commented, "DIPG remains one of the most challenging of childhood cancers. No drug treatment has ever demonstrated meaningful efficacy. The PNOC022 study brings a different approach, uniting the best of preclinical research with novel clinical trial techniques. We look forward to commencing enrolment to the study shortly, and very much hope that we are able to generate new hope for patients and their families."

Commencement of the study remains subject to execution of a definitive contract and is dependent on approval by the US FDA and Institutional Review Boards. It is expected that PNOC022 will initially open in the United States in 1H CY2021, with expansion to other countries taking place in CY2021. Discussions are ongoing regarding the potential inclusion of Australian sites in the study.

Australian Scientific Research

The design of the PNOC022 study has been extensively informed by laboratory research in DIPG, and in particular by research undertaken at the University of Newcastle, Hunter Medical Research Institute (HMRI) by Associate Professor Matt Dun and colleagues. The HMRI team has conducted laboratory research with paxalisib for several years and has generated a powerful body of data combining paxalisib with other investigational drugs. This research has been partly funded by RUN DIPG, a not-for-profit organisation led by Associate Professor Dun, the DIPG Collaborative, Defeat DIPG Michael Moiser Foundation and the McDonald Jones Foundation. The robust mechanistic data is expected to be published in high impact scientific journals in coming months.

Pacific Pediatric Neuro-Oncology Consortium (PNOC)

The Pacific Pediatric Neuro-Oncology Consortium (PNOC) is an international consortium, with study sites in the United States, Canada, Swizterland, Europe, India, Israel, and Australia. PNOC is dedicated to bringing new therapies to children and young adults with brain tumours, using the latest scientific understanding to inform a personalised medicine approach.

PNOC comprises 225 leading specialists in childhood brain cancer and is currently driving sixteen international clinical trials. In Australia, the organisation collaborates closely with the Australia and New Zealand Children's Hematology / Oncology Group (ANZCHOG). PNOC's research is substantially supported by the PNOC Foundation, the Pediatric Brain Tumor Foundation, and other not-for-profit entities.

Paxalisib Clinical Program

The initiation of this trial will bring the number of ongoing clinical studies of paxalisib in brain cancer to eight.

Indication	Phase	Sponsor	Registration
Glioblastoma	II	Kazia Therapeutics	NCT03522298
Glioblastoma	II / III	Global Coalition for Adaptive Research	NCT03970447
DIPG & DMGs	I	St Jude Children's Research Hospital	NCT03696355
DIPG & DMGs	N/A*	Pacific Pediatric Neuro-Oncology Consortium	(TBD)
Breast Cancer Brain Metastases	II	Dana-Farber Cancer Institute	NCT03765983
Brain Metastases	II	Alliance for Clinical Trials in Oncology	NCT03994796
Brain Metastases	I	Memorial Sloan-Kettering Cancer Center	NCT04192981
Primary CNS Lymphoma	II	Dana-Farber Cancer Institute	(TBD)

**Note – the PNOC022 has not adopted a 'phase' designation and is described as an 'adaptive platform study'*

Next Steps

Recruitment to this study is expected to commence in 1H 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 GBM AGILE, a pivotal study in glioblastoma, in October 2020. Seven additional studies are active in various 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. Cantrixil was granted orphan designation for ovarian cancer by the US FDA in April 2015.

For more information, please visit www.kaziatherapeutics.com.

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