ASX RELEASE

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US FDA AWARDS ORPHAN DRUG DESIGNATION (ODD) TO PAXALISIB FOR AT/RT, A RARE FORM OF CHILDHOOD BRAIN CANCER

Sydney, 17 June 2022 – Kazia Therapeutics Limited (NASDAQ: KZIA; ASX: KZA), an oncology-focused drug development company, is pleased to announce that the United States Food and Drug Administration (FDA) has awarded Orphan Drug Designation (ODD) to Kazia's paxalisib for the treatment of atypical rhabdoid / teratoid tumors (AT/RT), a rare and highly-aggressive childhood brain cancer.

Key Points

- Orphan Drug Designation (ODD) is a special status accorded to drugs which are considered promising potential treatments for rare ('orphan') diseases, generally defined as those which affect less than 200,000 cases per annum in the United States.
- ODD can provide drug developers with up to seven years of Orphan Drug Exclusivity (ODE), during which competitors may not rely on Kazia's data to develop generic versions of paxalisib, effectively extending the effective life of a commercial product. It also provides opportunities for grant funding and protocol assistance, and tax credits.
- FDA will waive fees relating to a future regulatory filing in AT/RT, potentially saving more than US$3 million if Kazia seeks approval in this indication.
- Paxalisib has previously been granted ODD in malignant glioma, a category of brain cancer that includes both glioblastoma, an adult brain cancer which is the lead indication for the drug, and diffuse intrinsic pontine glioma (DIPG), a rare childhood brain cancer which is currently the subject of a phase II study led by the Pacific Pediatric Neuro-Oncology Consortium.

Kazia CEO, Dr James Garner, commented, "childhood brain cancer has emerged as an important area of focus for the paxalisib program. We have been working for some years with several world-leading researchers in DIPG, one of the most aggressive childhood cancers. Recent data presented at the AACR conference by Dr Jeffery Rubens and colleagues from Johns Hopkins Medical School has shown the potential of the drug to also add benefit

in AT/RT, another form of childhood brain cancer that is very poorly served by existing treatments. This represents an important new opportunity for paxalisib, and one that we continue to explore enthusiastically with our collaborators and advisors."

Orphan Drug Designation

ODD exists to recognise and encourage the development of a drug for a rare disease, which may affect adults or children. ODD provides an additional period of 7.5 years data exclusivity (for a paediatric disease), which allows companies to better defend their products against competition. It also results in a waiver by FDA of fees for a marketing application, under the Prescription Drug User Fees Act (PDUFA fees), which are over US$ 3 million in FY2022. In addition, drugs with ODD may be eligible for orphan grants by FDA.

Kazia previously received ODD for paxalisib in glioblastoma in February 2018, and for malignant glioma in August 2020.

Next Steps

A phase II clinical trial of multiple drug therapies, including paxalisib, is ongoing, under the sponsorship of the Pacific Pediatric Neuro-Oncology Consortium (PNOC) (NCT05009992). This study combines several investigational drugs in the treatment of patients with diffuse midline gliomas (DMGs), a category which includes DIPG. Initial data from this study is anticipated in CY2023.

A phase I study of paxalisib in DIPG, led by St Jude Children's Research Hospital in Memphis, TN (NCT03696355), is nearing completion, and final data is expected to be submitted for publication by the end of CY2022.

Kazia Therapeutics will participate in a webinar to discuss the company's activities in childhood brain cancer on Wednesday 22nd June 2022. Registration details are available via the company website.

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About Kazia Therapeutics Limited

Kazia Therapeutics Limited (NASDAQ: KZIA; ASX: KZA) is an oncology-focused drug development company, based in Sydney, Australia.

Our lead program is paxalisib, a brain-penetrant 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 commenced recruitment to GBM AGILE, a pivotal study in glioblastoma, in January 2021. 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, and Orphan Designation for AT/RT in June 2022.

Kazia is also developing EVT801, a small-molecule inhibitor of VEGFR3, which was licensed from Evotec SE in April 2021. Preclinical data has shown EVT801 to be active against a broad range of tumour types and has provided compelling evidence of synergy with immuno-oncology agents. A phase I study commenced recruitment in November 2021.

For more information, please visit www.kaziatherapeutics.com or follow us on Twitter @KaziaTx.

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