ASX RELEASE

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US FDA AWARDS ORPHAN DRUG DESIGNATION (ODD) TO PAXALISIB FOR MALIGNANT GLIOMA, INCLUDING DIPG

Sydney, 24 August 2020 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to announce that the United States Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to Kazia's paxalisib (formerly GDC-0084) for the treatment of malignant glioma, which includes Diffuse Intrinsic Pontine Glioma (DIPG), 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), extending the effective life of a commercial product. It also provides opportunities for grant funding, protocol assistance, and financial benefits, such as a waiver of New Drug Application fees, and tax credits
- Receipt of ODD follows award of Rare Pediatric Disease Designation (RPDD) for DIPG on 7 August 2020

Kazia CEO, Dr James Garner, commented, "Taken together, RPDD and ODD provide a powerful suite of incentives, opportunities, and protections for the development of paxalisib in DIPG. We look forward to seeing initial data from the ongoing phase I study in DIPG at St Jude Children's Research Hospital during the second half of calendar 2020. In parallel, we are working closely with collaborators, advisors, and researchers to determine the best path forward for paxalisib in this devastating disease."

He added, "This award of ODD concludes a program of regulatory optimisation that Kazia has initiated for paxalisib over the past six months. As we orient paxalisib towards commercialization, these special designations from FDA will allow us to move forward in the swiftest and most effective way possible."

Orphan Drug Designation

ODD exists to recognise 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 just under US$ 3 million in FY2020. In addition, drugs with ODD may be eligible for orphan grants by FDA.

Kazia previously received ODD for paxalisib in glioblastoma in February 2018.

Summary of Paxalisib Regulatory Status

	Glioblastoma *Most common and most aggressive adult brain cancer*	**DIPG** *Highly aggressive childhood brain cancer*
Orphan Designation	February 2018	August 2020
Fast Track Designation	August 2020	
Rare Pediatric Disease Designation	n/a	August 2020

Next Steps

Kazia expects to present further data from its ongoing phase II study of paxalisib in glioblastoma at the Society for Neuro-Oncology (SNO) Annual Meeting in November 2020.

Initial efficacy data from the ongoing phase I study of paxalisib in DIPG at St Jude Children's Research Hospital is expected during 2H CY2020. Precise timing remains uncertain due to pandemic-related disruption in conference schedules, but Kazia expects to provide an update to investors at the earliest opportunity.

Paxalisib has been selected to join the international GBM AGILE pivotal study in glioblastoma, and recruitment is expected to begin in 2H CY2020.

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 by the US FDA 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.