ASX RELEASE
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TOP-LINE FINAL DATA FROM CANTRIXIL PHASE I STUDY CONFIRMS PRIOR POSITIVE EFFICACY AND SAFETY SIGNALS

Sydney, 9 December 2020 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to share top-line final data from its phase I study of Cantrixil (TRX-E-002-1) in patients with persistent or recurrent ovarian cancer (NCT02903771).

Key Points

- 25 patients with advanced metastatic ovarian cancer received at least one dose of Cantrixil at six sites in the United States and Australia, comprising 11 patients in Part A (dose escalation) and 14 patients in Part B (dose expansion)
- Trial achieved its primary objective, determining the maximum tolerated dose (MTD) of Cantrixil to be 5 mg/kg
- Overall, 16 patients were evaluable for efficacy. One patient demonstrated a complete response (CR) and two patients experienced a partial response (PR), according to industry-standard RECIST criteria, making an overall response rate (ORR) of 19%
- The patient who experienced a complete response remains in remission some three years after her last dose of Cantrixil
- The drug was generally well-tolerated, with primarily gastrointestinal toxicities observed (abdominal pain, vomiting, and nausea)

Australian lead investigator, Associate Professor Jim Coward, commented, “this was a heavily pre-treated population, comprising patients with very advanced disease. Existing treatment options for such patients are limited, and there remains an urgent need for new therapies. My colleagues and I are excited by the potential for Cantrixil to provide benefit here, and we look forward to seeing the drug move forward in its development.”

Kazia expects the full data to be presented at a suitable academic conference and published in a peer-reviewed journal in 1H CY2021. In accordance with common practice, the full data will remain embargoed until they are formally published, in order not to prejudice the appropriate dissemination of the data, and only top-line data are discussed here.

Kazia CEO, Dr James Garner, commented, “we are very pleased to see the Cantrixil phase I study completed. The data unambiguously demonstrates the potential for Cantrixil to provide benefit in this very challenging patient population. With this positive data in hand, our focus now shifts to partnering activity, and we hope to transition Cantrixil to a company which both shares our belief in its potential and is able to apply the necessary resources and expertise to realise that potential over the next chapter of its development.”

Background

The phase I study of Cantrixil in ovarian cancer (NCT02903771) commenced recruitment in December 2016. It was designed in two parts. Part A (dose escalation component) was intended to determine the maximum tolerated dose (MTD) of Cantrixil in women with ovarian cancer. Part B (dose expansion cohort) was intended to seek preliminary evidence of clinical efficacy, as well as providing a deeper understanding of pharmacokinetics and safety of Cantrixil. All patients received two cycles of treatment with Cantrixil monotherapy, followed by up to six cycles in combination with other chemotherapy agents.

Kazia announced completion of Part A in October 2018. At that stage, the study declared 5 mg/kg to be the MTD, and this dose was used for all patients in Part B. The main dose-limiting toxicity (DLT) was abdominal pain. 11 patients received at least one dose of Cantrixil in Part A.

Part B recruited an additional 14 patients, all of whom were treated at the MTD, with the goal of seeking exploratory signals of potential clinical efficacy. All 14 patients received at least one dose of Cantrixil in Part B. In total, 17 patients across Part A and Part B received Cantrixil at the MTD of 5 mg/kg.

The study completed recruitment in August 2019, and last patient last visit occurred in March 2020. Preliminary efficacy data was presented in September 2019 at the European Society for Medical Oncology (ESMO) Annual Meeting in Barcelona, Spain, and at the American Association of Cancer Research (AACR) Virtual Meeting in June 2020.

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. Five additional studies are active 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. 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.