ASX RELEASE

17 April 2020

CANTRIXIL PHASE I STUDY IN OVARIAN CANCER SHOWS FURTHER POSITIVE DATA; ALL PATIENT FOLLOW-UP NOW COMPLETE

Sydney, 17 April 2020 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to provide an update on its phase I study of Cantrixil (TRX-E-001-2) in ovarian cancer.

Key Points

- Patient follow-up complete; all patients now off study; some data still being finalised
- Preliminary analysis shows one complete response (CR) and two partial responses (PR) in overall dataset, making an overall response rate (ORR) of 15% (n=20). This compares favourably to a figure of 10% for historical controls[1]
- Median progression-free survival (PFS) for Part A was 5.5 months (n=9), versus the historical control of 3.4 months, as previously reported. Kazia expects to report PFS data for the entire data set in 2H CY2020
- Growing body of data provides strong evidence that Cantrixil is clinically active in this very late-stage patient population
- Safety profile consistent with prior experience: most common adverse events were gastrointestinal in nature and low-grade

Australian lead investigator, Associate Professor Jermaine Coward, commented "despite recent progress in ovarian cancer, there remains an urgent need for new therapies. The Cantrixil phase I study was conducted in a very late-stage patient population, with few effective treatment options. In that context, my colleagues and I consider these data to be extremely promising."

The patients recruited to the Cantrixil study all had advanced metastatic ovarian cancer. On average, patients had failed or become resistant to six prior therapies before commencing

[1] Pujade-Lauraine E, Hilpert F, Weber B, et al. Bevacizumab combined with chemotherapy for platinum resistant recurrent ovarian cancer: The AURELIA open label randomized phase III Trial. *J Clin Oncol* 2014; 32 (13): 1302-8.

treatment with Cantrixil. Fifteen patients were Stage IV, the most advanced stage of ovarian cancer, and the remainder were Stage III.

In total, 20 out of 24 patients were evaluable for efficacy (the remaining four did not have enough tumour evaluations to assess response).

Of these, 1 patient experienced a complete response (CR), and 2 patients demonstrated a partial response (PR), using the industry-standard RECIST criteria. This represents an overall response rate (ORR) of 15% (3 out of 20 patients). A 'complete response' means that the patient has no measurable disease after treatment and is effectively in remission, and a partial response means that the tumour has shrunk in size by at least 30%. These responses provide strong evidence that Cantrixil is clinically active and is able to shrink tumours in a proportion of patients.

As reported in September 2019, the median progression-free survival (PFS) for Part A of the study was 5.5 months. For comparison, the historical comparator for a similar cohort of late stage patients provides a median PFS of 3.4 months. While comparison between studies is always imperfect, these data strongly suggest that Cantrixil may be able to slow the progression of disease in advanced ovarian cancer. Kazia expects to report PFS data for the entire study in 2H CY2020.

Toxicities were consistent with prior data, and were principally gastrointestinal in nature (abdominal pain, nausea, and vomiting).

Kazia CEO, Dr James Garner, commented, "patient follow-up for this study is now complete, and we move into a period of finalising and analysing the data. The preliminary results are extremely encouraging. For some patients in this very challenging patient population, Cantrixil has been able to shrink tumours and delay disease progression, demonstrating a clinically meaningful benefit. The outstanding success of the study reflects the enormous commitment and energy of all involved – clinicians, patients, contractors, and Kazia colleagues – and we are grateful for all their efforts."

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 13 patients, all of whom were treated at the MTD, with the goal of seeking exploratory signals of potential clinical efficacy. All 13 patients received at least one dose of Cantrixil in Part B.

The study completed recruitment in August 2019. Preliminary efficacy data from all 9 evaluable patients in Part A was presented in September 2019 at the European Society for Medical Oncology (ESMO) Annual Meeting in Barcelona, Spain.

The last patient visit in connection with the study occurred in March 2020. Kazia will now work closely with sites and investigators to collect and validate all remaining data, and expects to release final data in 2H CY2020.

This data had been accepted for presentation at the annual meeting of the American Association of Cancer Research (AACR) in San Diego, CA from 24 -29 April 2020. However, given the postponement of the AACR meeting due to the COVID-19 outbreak, Kazia has determined to provide an update to investors via this ASX announcement.

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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 multiforme, 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 in April 2020, and further data is expected in 2H 2020. Paxalisib was granted orphan designation for glioblastoma by the US FDA in February 2018.

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 is currently undergoing a phase I clinical trial in Australia and the United States. Interim data was presented at the ESMO Congress in September 2019, and the study remains ongoing. Cantrixil was granted orphan designation for ovarian cancer by the US FDA in April 2015.

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