Kazia Therapeutics

Regulatory update

Paxalisib receives ODD for AT/RT

Pharma and biotech

In an encouraging development for Kazia Therapeutics' efforts towards combating pediatric brain cancers, its lead drug paxalisib has received orphan drug designation (ODD) from the FDA for the treatment of atypical teratoid/rhabdoid tumors (AT/RT). AT/RT is a rare and aggressive childhood brain cancer with a five-year survival rate of c 32%. Approximately 600 people are living with the cancer in the United States, with around 60 new cases reported each year. The ODD accords seven years of market exclusivity in the US on approval, in addition to possible grant funding and tax credits. Kazia is undertaking preclinical studies in AT/RT and has recently presented encouraging data from combination studies in xenograft models. As a reminder, paxalisib already has ODD in malignant gliomas, including glioblastoma (GBM) and diffuse intrinsic pontine glioma (DIPG). Our valuation is unchanged at US$294m or US$22.28/ADR. Please see our Deep dive into childhood brain cancer note, published on 24 May.

Year end	Revenue (US$m)	PTP* (US$m)	EPADR (US$)	DPADR (US$)	P/E (x)	Gross yield (%)
12/20	0.8	(7.8)	(1.04)	0.0	N/A	N/A
12/21	11.0	(3.2)	(0.26)	0.0	N/A	N/A
12/22e	0.0	(17.2)	(1.27)	0.0	N/A	N/A
12/23e	0.0	(19.9)	(1.47)	0.0	N/A	N/A

Note: Converted at A$1.38/US$. Dividend yield excludes withholding tax. Investors should consult their tax advisor regarding the application of any domestic and foreign tax laws.

The ODD granted by the US FDA follows recently presented preclinical data on AT/RT at the 20th International Symposium on Pediatric Neuro-Oncology (ISPNO) held on 12–15 June. The data, which was presented by Dr Jeffrey Rubens at Johns Hopkins University, assessed the activity and efficacy of paxalisib in combination with the novel HDAC1/3 inhibitor RG2833 in xenograft models of AT/RT. This built on previously presented data at the American Association for Cancer Research (AACR) meeting in April 2022, which highlighted that paxalisib in combination with RG2833 decreased AT/RT cell growth and increased apoptosis. Both combinations were confirmed to be more effective than paxalisib alone in mouse AT/RT xenograft pilot studies. Kazia will discuss these findings in more detail at the upcoming investor webinar scheduled for 22 June.

AT/RT is one of two rare childhood cancers targeted by Kazia, the other being DIPG, the most aggressive form of childhood brain cancer, which has a very poor prognosis (median survival of eight to 11 months). Both AT/RT and DIPG remain underserved with no currently approved treatments, although several clinical trials are underway. For AT/RT, the current standard of care involves maximum surgical resection followed by adjuvant chemotherapy and radiotherapy, which is often accompanied by severe side effects. Despite this aggressive line of treatment, many patients become refractory to therapy. Given the high unmet need in the space, we expect any approved therapies to gain a sizeable share of the market.

21 June 2022

Price	**US$5.27**
Market cap	**US$71m**
	ADR/Ord conversion ratio 1:10
Net cash (US$m) at 31 December 2021	11.0
ADRs in issue	13.39m
ADR code	KZIA
ADR exchange	NASDAQ
Underlying exchange	ASX
Depository	BNY

ADR share price performance

Business description

Kazia Therapeutics is a late-stage clinical pharmaceutical company with lead asset paxalisib (a PI3K inhibitor that can cross the blood-brain barrier, licensed from Genentech), in a pivotal study for GBM and in early-stage studies in childhood brain cancers, DIPG and AT/RT. The other asset is the Phase I drug EVT801, an inhibitor of VEGFR3, in-licensed from Evotec in April 2021.

Next events

Investor webinar	22 June 2022

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