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Kazia Therapeutics Ltd

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Kazia Therapeutics hits A$1 on grant of Rare Pediatric Disease Designation to paxalisib for DIPG

Kazia Therapeutics Ltd (ASX:KZA) (NASDAQ:KZIA) has surged on being granted Rare Pediatric Disease Designation (RPDD) by the US FDA for its paxalisib for the treatment of DIPG, a rare and highly aggressive childhood brain cancer.

With RPDD granted, Kazia may now be eligible to receive a 'rare pediatric disease priority review voucher' (PRV) if paxalisib is approved for Diffuse Intrinsic Pontine Glioma (DIPG).

A PRV grants the holder an expedited six-month review of a new drug application by FDA.

PRVs can be sold to other companies and have historically commanded prices between US$68 million and US$350 million.

Shares up 78%

Shares have been more than 78% higher in early trade to a new 3.5 year high of A$1.00.

Kazia CEO Dr James Garner said: "Although glioblastoma remains our primary focus for paxalisib, we have been devoting increasing energy to developing the drug in childhood brain cancer as well.

"For patients diagnosed with DIPG, there are currently no FDA-approved drug treatments, and the average survival from diagnosis is around 9.5 months."

"Well-placed to move forward"

Garner said: "The granting of RPDD by the FDA recognises our efforts and achievements so far and leaves us well placed to move paxalisib forward as a potential therapy for DIPG.

"We continue to be inspired by the dedication of our collaborators in this field and are committed to understanding whether paxalisib may be able to help in this enormously challenging paediatric disease."

RPDD has been awarded following positive emerging preclinical data in DIPG and with initial clinical efficacy data expected in the current half-year, positive clinical data may substantially enhance the likelihood of a potential future PRV.

RPDD program

The US Food and Drug Administration's (FDA) RPDD program is intended to advance the development of drugs and biologics for certain serious and life-threatening rare pediatric diseases by providing incentives to industry.

Most significant among these incentives is the potential access of a priority review voucher at the time of a marketing authorisation for the rare pediatric disease.

Price: 0.785

Market Cap: $74.28 m

1 Year Share Price Graph

Share Information

Code:	KZA	
Listing:	ASX	
52 week	**High**	**Low**
	1.18354	**0.354583**
Sector:	**Pharma & Biotech**	
Website:	**www.kaziatherapeutics.com**	

Company Synopsis:

Kazia Therapeutics (ASX: KZA, NASDAQ: KZIA) pipeline includes two clinical-stage drug development candidates.

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RPDD may be granted to drugs in development for diseases which primarily affect children under the age of 18, have an incidence of less than 200,000 new cases per annum in the US and which are serious or life-threatening.

Paxalisib trial

In October 2018, St Jude Children's Research Hospital in Memphis, Tennessee, began a phase I clinical trial of paxalisib in DIPG (NCT03696355).

This study reported favourable top-line safety data in September 2019 and established 27 mg/m2 as the maximum tolerated dose for paediatric use.

The study has completed recruitment and initial efficacy data is anticipated during the second half of 2020.

This data will be used to guide future development of paxalisib (formerly GDC-0084) in this disease.

Extensive research

Dr Matt Dun and colleagues at the University of Newcastle, Australia have conducted extensive laboratory research with paxalisib, focused on phosphoproteomic analysis of its activity in DIPG cell lines.

Phosphoproteomics is a new approach in cancer research that attempts to discern how complex signalling pathways are modified in tumours.

Work at the Dun laboratory has shown paxalisib to be broadly active in DIPG and has identified a number of potential combination strategies which may enhance its activity.

Initial data was presented at the Society for Neuro-Oncology (SNO) Pediatric Neuro-Oncology Basic and Translational Research Conference in San Francisco, CA, in May 2019.

Further ongoing work in animal models is expected to provide additional insight.

In parallel, related laboratory research is underway in the DMG Research Center at the University of Zurich, Switzerland, under the leadership of Dr Javad Nazarian.

Dr Nazarian is also the principal investigator at Center for Genetic Medicine within the Children's National Medical Center, Washington DC with a focus on DIPG.

Laboratory research is also being conducted at St Jude Children's Research Hospital by Dr Chris Tinkle and Dr Suzanne Baker and colleagues, in parallel to the ongoing phase I clinical trial at that centre.

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