

ASX RELEASE
5 January 2021

KAZIA PRESENTS TO HC WAINWRIGHT & CO BIOCONNECT

Sydney, 5 January 2021 – Kazia Therapeutics Limited (ASX: KZA; NASDAQ: KZIA), an Australian oncology-focused biotechnology company, is pleased to provide a copy of the presentation to be made by our CEO, Dr James Garner, to the HC Wainwright & Co BIOCONNECT conference later today.

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. Seven additional studies are active in various 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.

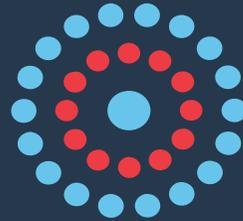
Board of Directors

Mr Iain Ross Chairman, Non-Executive Director

Mr Bryce Carmine Non-Executive Director

Mr Steven Coffey Non-Executive Director

Dr James Garner Chief Executive Officer, Managing Director



KAZIA
THERAPEUTICS



A company developing
innovative, high-impact
drugs for cancer

Presentation to HC Wainwright & Co
BIOCONNECT

Dr James Garner
Chief Executive Officer & Managing Director

5 January 2021

Forward-Looking Statements

This presentation contains “forward-looking statements” within the meaning of the “safe-harbor” provisions of the Private Securities Litigation Reform Act of 1995. Such statements involve known and unknown risks, uncertainties and other factors that could cause the actual results of the Company to differ materially from the results expressed or implied by such statements, including changes from anticipated levels of customer acceptance of existing and new products and services and other factors. Accordingly, although the Company believes that the expectations reflected in such forward-looking statements are reasonable, there can be no assurance that such expectations will prove to be correct. The Company has no obligation to sales, future international, national or regional economic and competitive conditions, changes in relationships with customers, access to capital, difficulties in developing and marketing new products and services, marketing existing products and services update the forward-looking information contained in this presentation.

Corporate Overview



 Company Description	Oncology-focused, late-clinical-stage, small-molecule biotechnology company, headquartered in Sydney, Australia
 Pipeline	Paxalisib – brain-penetrant PI3K / mTOR inhibitor about to enter international phase III for glioblastoma Cantrixil – cancer stem cell-targeting agent in phase I for ovarian cancer
 Financials	Listed on ASX (KZA) and NASDAQ (KZIA) with a market capitalization of ~US\$ 100 million ~US\$ 20M financing round completed October 2020

Investment Rationale

World-Class Asset in Brain Cancer

- Paxalisib developed by Genentech, the world's most successful cancer drug company
- Well-proven mechanism of action, with unique differentiating factor of brain penetration
- Strong scientific rationale for development in brain cancer
- Encouraging clinical data emerging from US-based phase II study
- Potential best-in-class toxicity profile

Clear Path to Commercialisation

- Ongoing FDA-endorsed GBM AGILE study will serve as pivotal study for registration
- US\$ 1.5 billion pa commercial opportunity in glioblastoma, with potential upside in other cancers
- High unmet medical need – existing standard of care ineffective in two-thirds of patients
- 6x additional clinical studies at top tier US hospitals provide multiple shots on goal
- Optimised regulatory position with Orphan, Fast Track, and Rare Paediatric Disease Designations

Strong Corporate Story

- Kazia is a late-clinical-stage company, funded for phase III, with one of the leading assets in the global glioblastoma pipeline, and the potential to address a \$1.5 billion market
- Highly-efficient operating model, with ~80% of expenditure applied directly to R&D
- Lean team of internationally-experienced drug developers
- Good potential for partnering and / or M&A during remaining development of paxalisib

Program Overview

Paxalisib (GDC-0084)

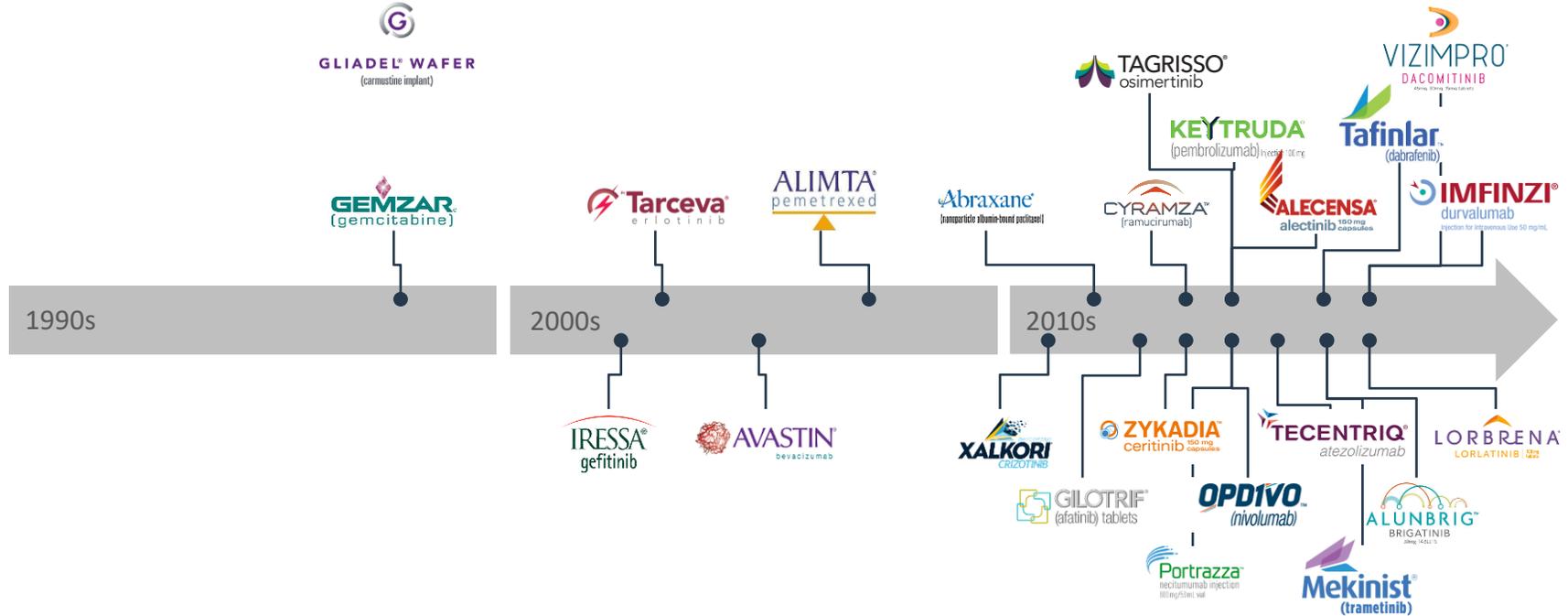
Brain Cancer

Treatment of brain cancer has improved little in recent decades, unlike other cancers

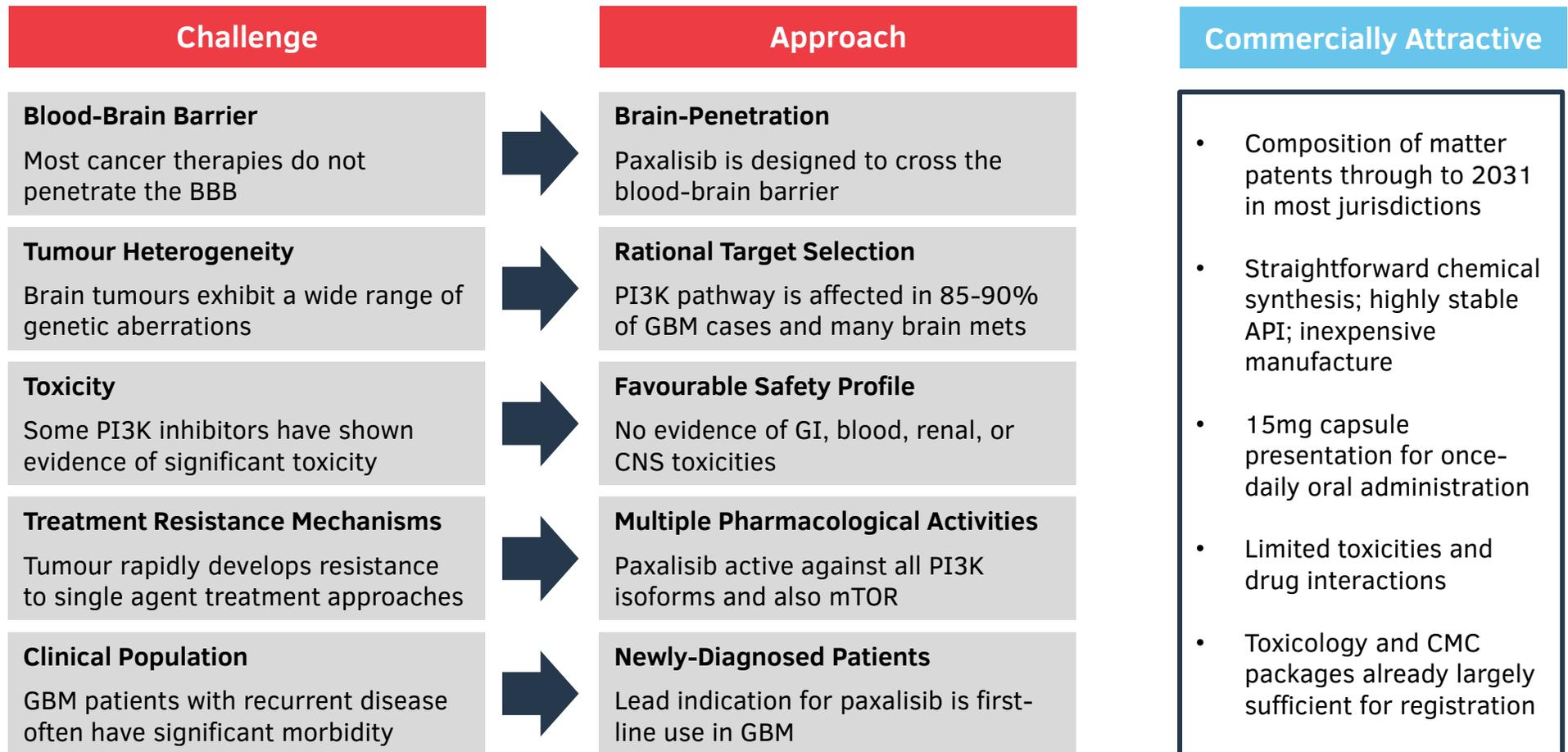
Brain Cancer
(glioblastoma)



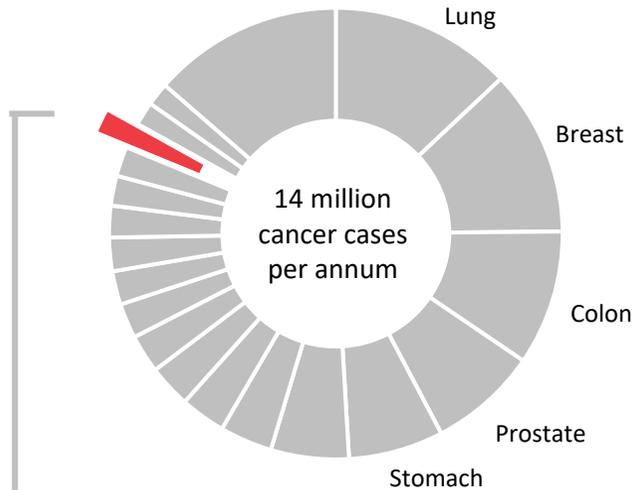
Lung Cancer



Paxalisib was designed specifically to overcome challenges associated with brain cancer treatment



Glioblastoma (GBM) is the most common and most aggressive form of primary brain cancer



Glioblastoma Multiforme

133,000 cases per annum worldwide

Indicative Market Opportunity

US\$ 1.5 billion

No clear cause
or strong risk factors

3-4 months
untreated survival

12-15 months
average survival with treatment

Any age, but most common in
60s

Five-year survival
3 – 5%
(breast cancer: 90%)



Sen. John McCain



Sen. Ted Kennedy



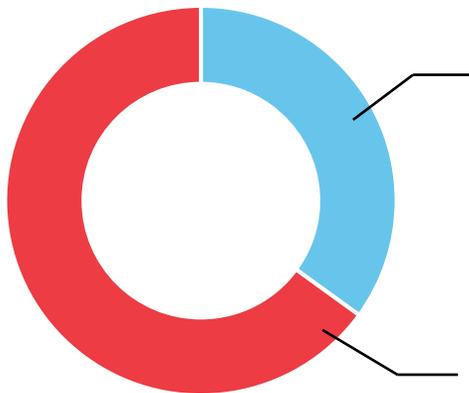
Beau Biden



Dan Case

Temozolomide is only FDA-approved drug for GBM; it is ineffective in ~65% of cases

Standard of Care ('Stupp Regimen')



~35% of patients respond to temozolomide

Extends overall survival from 15 to 22 months

~65% of patients don't respond to temozolomide

Extends overall survival from 12 to 13 months



Paxalisib is being developed for the ~65% of newly-diagnosed GBM patients who will not respond to existing chemotherapy with temozolomide

For these patients, there is no effective pharmacological treatment currently available

Source: ME Hegi, A-C Diserens, T Gorlia, et al. (2005). *N Engl J Med* 352:997-1003

Note: Temozolomide is only approved therapy for newly-diagnosed patients; Avastin (bevacizumab) is approved for use in recurrent setting

The PI3K class is well-established, but paxalisib is unique in its ability to cross the blood-brain barrier



Zydelig (idelalisib)



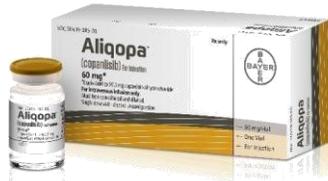
FDA Approved **July 2014** ✓
(blood cancers)
[accelerated approval]

Does not cross blood-brain barrier ✗

Potentially fatal liver toxicity and diarrhoea ✗



Aliqopa (copanlisib)



FDA Approved **September 2017** ✓
(blood cancers)
[accelerated approval]

Does not cross blood-brain barrier ✗

Potentially fatal infections ✗



Copiktra (duvelisib)



FDA Approved **October 2018** ✓
(blood cancers)
[accelerated approval]

Does not cross blood-brain barrier ✗

Potentially fatal infections & diarrhoea ✗



Piqray (alpelisib)



FDA Approved **May 2019** ✓
(breast cancer)
[accelerated approval]

Does not cross blood-brain barrier ✗

Limited toxicities to date ✓



paxalisib



In phase II human trials under US FDA oversight (brain cancer)

Does cross blood-brain barrier ✓

Appears generally safe and well-tolerated thus far ✓

The ongoing phase II study is designed to focus on newly-diagnosed patients, following radiotherapy

Step 1: Dose Optimisation

9 patients
September 2018 – May 2019

Primary objective is to determine the appropriate dose for newly-diagnosed patients (phase 1 was in end-stage patients)

Fully-Recruited



- Top-line safety data: May 2019
- Interim efficacy data: Nov 2019
- Interim survival data: Apr 2020

Step 2: Expansion Cohort

21 patients
June 2019 – February 2020

Primary objective is to generate supportive data for FDA and to provide confirmatory signals of efficacy in newly-diagnosed population

Fully-Recruited



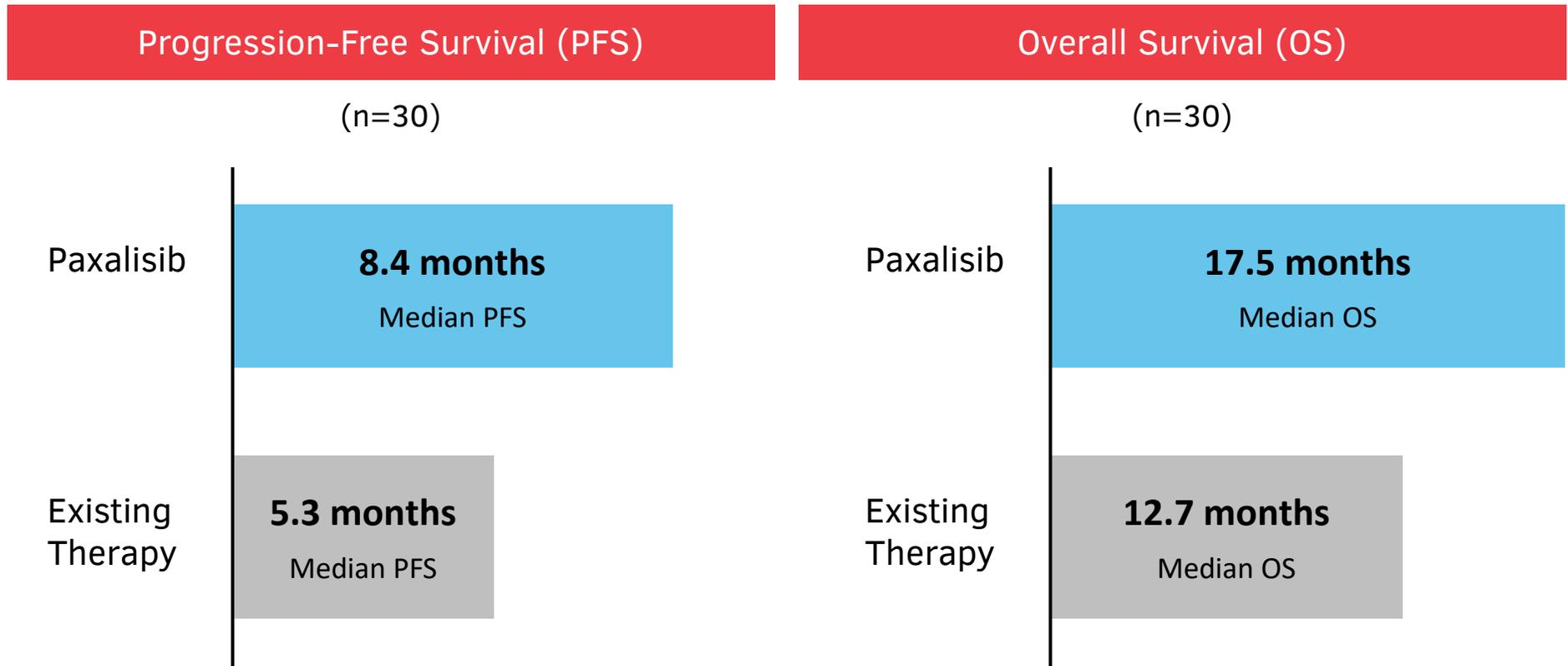
- Interim efficacy data: Apr 2020
- Interim efficacy data: Jun 2020

- Newly-diagnosed patients with the unmethylated MGMT promotor (i.e. resistant to temozolomide)
- Paxalisib administered once daily, orally, as monotherapy in place of temozolomide
- Primary objective is dose determination (Step 1) and signals of efficacy (Step 2)



Note: timelines are estimated and subject to periodic revision based on recruitment performance and treatment effect

Latest phase II data compares well to historical data for temozolomide (existing standard of care)



Note: figures for existing therapy are for temozolomide, per Hegi et al. (2005); comparison between different studies is never perfectly like-for-like

A broad-based clinical program is underway across multiple forms of brain cancer

Paxalisib (GDC-0084)

Primary Brain Cancer (brain cancer that begins in the brain)

Secondary Brain Cancer (brain cancer that spreads from elsewhere in the body)

Glioblastoma

Most common and most aggressive brain tumour

Phase II

[NCT03522298](#)



Glioblastoma

Pivotal study for FDA registration

Phase II / III

[NCT03970447](#)



DIPG

Highly aggressive childhood brain tumour

Phase I

[NCT03696355](#)



Primary CNS Lymphoma

Treatment-resistant brain cancer

Phase II

TBD



Brain Metastases

Cancer that has spread from any primary tumour

Phase II

[NCT03994796](#)



Breast Cancer Brain Mets

(combination with Herceptin®)

Phase II

[NCT03765983](#)



Brain Metastases

(combination with radiotherapy)

Phase I

[NCT04192981](#)



Funded by Kazia

Funded Primarily Through Partnerships and External Funding

GBM AGILE is the pivotal study for paxalisib in glioblastoma

What is GBM AGILE?

- A 'platform study', designed by the leading experts in brain cancer to expedite the approval of new drugs for glioblastoma
- Multiple drugs can be evaluated in parallel, saving time and money; Bayer's Stivarga (regorafenib) is the first drug to participate, and Kazia's paxalisib will be the second
- FDA has provided strong endorsement, saying that positive data from GBM AGILE will be suitable for product registration
- The study is currently active at approximately 28 hospitals in the United States and Canada and recruiting very well; expansion to Europe and China is expected in 1H CY2021
- Cutting-edge 'adaptive design' ensures that the study will only recruit the number of patients needed to reach an answer (up to 200 on paxalisib), avoiding redundancy and ensuring the fastest possible path to market

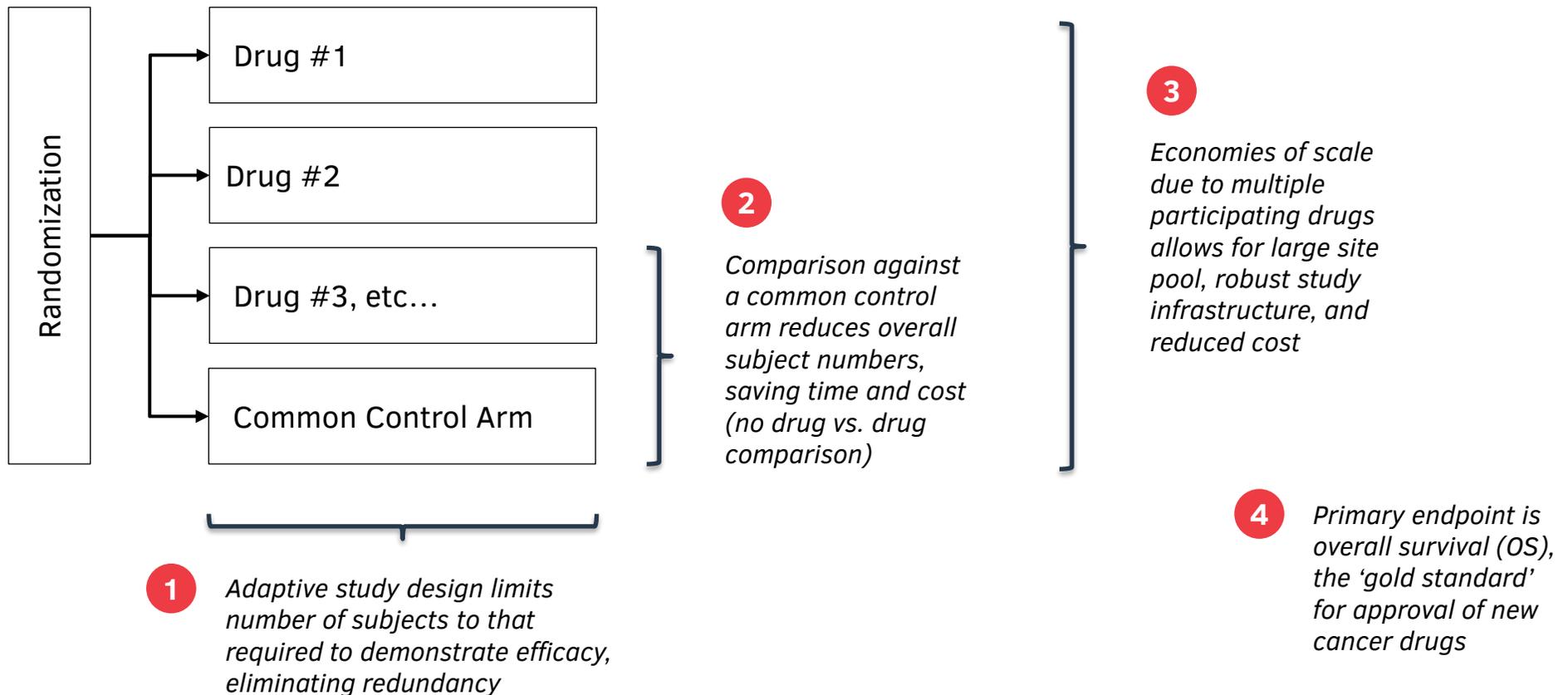
Who is Behind It?

GBM AGILE is sponsored by the Global Coalition for Adaptive Research (GCAR), a not-for-profit entity based in the United States

The study's scientific leadership includes world-leading experts in glioblastoma, among them several clinicians who have participated in clinical trials of paxalisib

GBM AGILE has received substantial grant funding, substantially reducing the cost of participation for companies such as Kazia

GBM AGILE is an adaptive multi-drug registrational study, with strong FDA support



GBM AGILE directly addresses the key challenges faced by small biotechs and their investors

Challenge	Approach	Indicative Parameters
<p>Limited Funding Many biotech companies cannot afford world-class phase III studies</p>	<p>More Cost-Effective Approach AGILE achieves huge efficiencies, and is partly grant-funded</p>	<ul style="list-style-type: none"> • Primary patient population essentially identical to Kazia's successful phase II study • Recruitment of up to 200 patients on paxalisib (but likely fewer due to adaptive design) • Approximately equivalent number of patients in control group, making for a ~400 patient dataset • Approximately 2-3 years to completion • Approximately one-third cost of a comparable company-sponsored study
<p>Long Study Timelines Phase III studies can sometimes take many years to deliver a result</p>	<p>Adaptive Study Design AGILE is an 'adaptive' study, only recruiting the patients needed</p>	
<p>Regulatory Uncertainty Small biotechs can struggle to get regulatory support for study design</p>	<p>Strong FDA Endorsement FDA has provided written backing to the GBM AGILE study design</p>	
<p>Clinician Engagement Competition for top hospitals and clinicians can be intense</p>	<p>Top-Tier Clinical Leadership Many of the world-leading experts in this disease are part of GBM AGILE</p>	
<p>Execution Risk Small companies can struggle to operationalise a complex trial</p>	<p>Live Study GBM AGILE is already underway, recruiting well, and run by IQVIA</p>	

Recent regulatory achievements position paxalisib well as it moves towards commercialisation

	Glioblastoma <i>Most common and most aggressive form of brain cancer</i>	DIPG <i>Highly aggressive childhood brain cancer</i>
Orphan Designation	February 2018	August 2020
Rare Pediatric Disease Designation	<i>(not applicable)</i>	August 2020
Fast Track Designation	August 2020	<i>for future consideration</i>
Breakthrough Designation	<i>for future consideration</i>	<i>for future consideration</i>

Advantages to Kazia

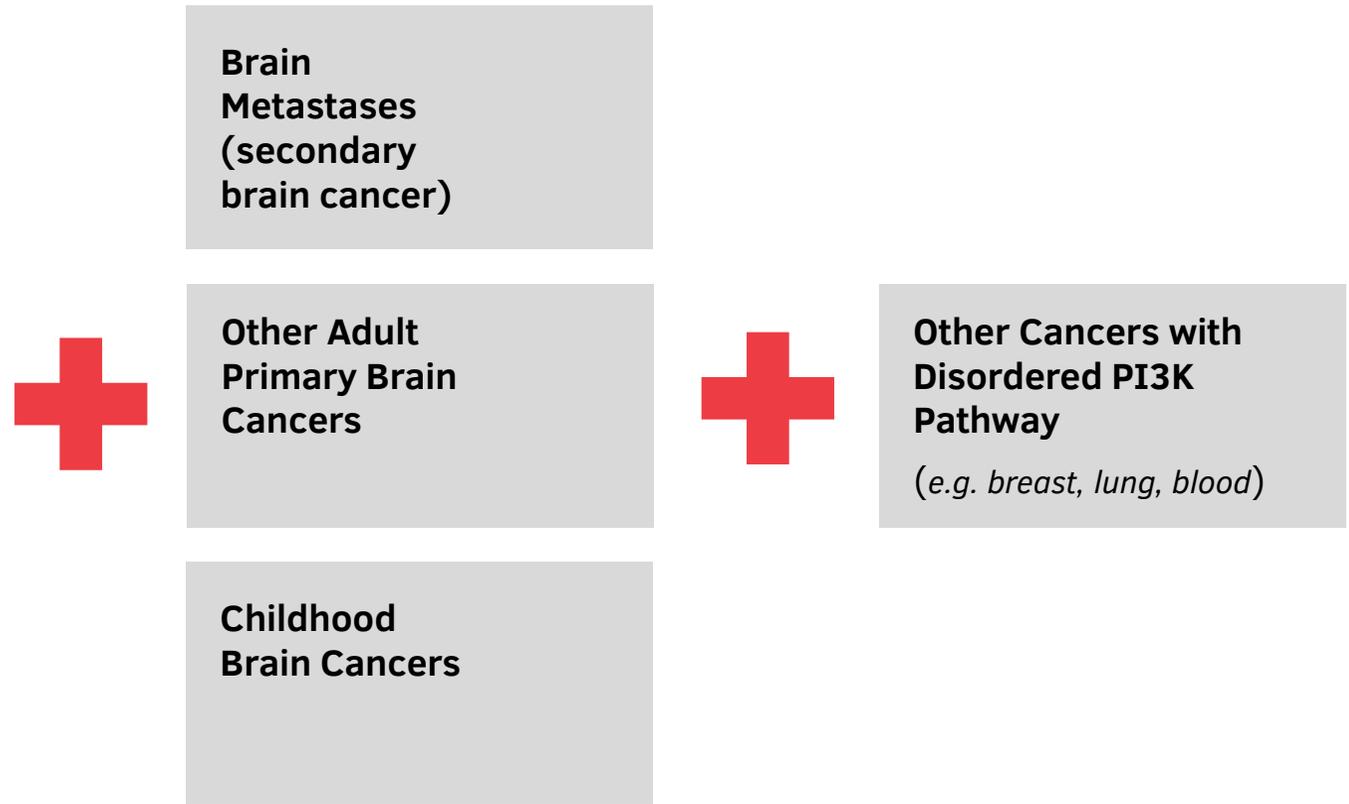
- 'Data exclusivity' provides additional protection against competition beyond granted patents
- Waiver of up to US\$ 6 million in FDA fees at time of filing for marketing authorisations
- Eligibility for orphan grants
- Eligibility for priority review voucher at time of filing for marketing authorisation in DIPG (up to US\$ 350 million in value)
- Enhanced access to FDA, with scope for more frequent and informal meetings
- Ability to submit a 'rolling NDA' in which sections are given to FDA as they are generated, instead of waiting until the end of development

Brain cancer represents a significant commercial opportunity for paxalisib, with limited competition

Path to Market



Expansion Opportunities



'Blue Sky' Potential

Corporate Overview



A strong team brings international experience in big pharma and early-stage biotech

Board



Iain Ross
Chairman

Executive and Board roles in pharma and small biotech



Bryce Carmine
Deputy Chairman

36 years executive experience in Eli Lilly



Steven Coffey
Non-Executive Director

Chartered accountant with extensive governance experience



Dr James Garner
Chief Executive Officer
& Executive Director

Physician / MBA; Extensive drug development experience



Scientific Advisory Board



Professor Sir Murray Brennan
Emeritus Chairman of Cancer Surgery at Memorial Sloan Kettering Hospital, New York



Dr Karen Ferrante
Former Chief Medical Officer at Millennium Pharmaceuticals



Professor Peter Gunning
Head of School of Medical Sciences at University of New South Wales



Professor Alex Matter
Former Global Head of Oncology Research at Novartis



Positive newsflow has supported revaluation of Kazia as paxalisib moves towards commercialisation



Note: as at 30 November 2020, unless otherwise noted

Market Capitalisation	~US\$ 125 million										
Shares on Issue	~126 million										
Listing	ASX: KZA NASDAQ: KZIA (1:10 ratio)										
Key Shareholders	<table border="0"> <tbody> <tr> <td>Willoughby Capital</td> <td>16%</td> </tr> <tr> <td>Platinum Asset Mgmt.</td> <td>9%</td> </tr> <tr> <td>Quest Asset Partners</td> <td>9%</td> </tr> <tr> <td>UniSuper</td> <td>6%</td> </tr> <tr> <td>Board & Mgmt.</td> <td>2%</td> </tr> </tbody> </table>	Willoughby Capital	16%	Platinum Asset Mgmt.	9%	Quest Asset Partners	9%	UniSuper	6%	Board & Mgmt.	2%
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Balance Sheet (AU\$) (as at 30 Sept 20)	<table border="0"> <tbody> <tr> <td>Cash:</td> <td>\$6.5 million</td> </tr> <tr> <td>FY20 Spend:</td> <td>\$12.5 million</td> </tr> <tr> <td>Runway:</td> <td>2Q CY2021</td> </tr> <tr> <td>Efficiency:</td> <td>~80% R&D</td> </tr> </tbody> </table>	Cash:	\$6.5 million	FY20 Spend:	\$12.5 million	Runway:	2Q CY2021	Efficiency:	~80% R&D		
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Runway:	2Q CY2021										
Efficiency:	~80% R&D										

Note: AU\$ 25 million financing completed in October 2020

Key Objectives for CY2021



Execute on GBM AGILE study



Drive data from broader paxalisib program



Intensify partnering activity

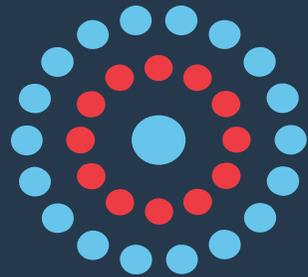


Commence 'rolling NDA' filing activities

Key Milestones and Anticipated Newsflow

Execution of definitive agreement with GCAR for GBM AGILE pivotal study	October 2020	✓
Further interim data from Kazia phase II glioblastoma trial	November 2020	✓
Initial interim data from phase I DIPG trial at St Jude	November 2020	✓
Initial interim data from phase II BCBM trial at Dana-Farber	H1 CY2021	
Commencement of recruitment to GBM AGILE pivotal study in glioblastoma	Q1 CY2021	
Commencement of recruitment to PNOC combination study in DIPG	Q1 CY2021	
Commencement of recruitment to phase II PCNSL study at Dana-Farber	Q1 CY2021	
Half-Year Financial Report	Q1 CY2021	
Initial interim data from phase II brain mets study by Alliance Group	H1 CY2021	
Initial interim data from phase I brain mets study at Sloan-Kettering	H1 CY2021	
Final data from Kazia phase II glioblastoma trial	H1 CY2021	

Note: all guidance is indicative, and subject to amendment in light of changing conference schedules, operational considerations, etc.



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THERAPEUTICS

www.kaziatherapeutics.com