

ASX RELEASE

15 September 2020

KAZIA PRESENTS TO H C WAINWRIGHT CONFERENCE

Sydney, 15 September 2020 – 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 H C Wainwright conference at 7am AEST on 16 September 2020.

[ENDS]

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 a phase II clinical trial in 2018. Interim data was reported most recently at AACR in June 2020, and further data is expected in 2H 2020. Four additional studies are ongoing 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 with the final data expected in the second half of calendar 2020. Interim data was presented most recently at the AACR conference in June 2020. Cantrixil was granted orphan designation for ovarian cancer by the US FDA in April 2015.

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

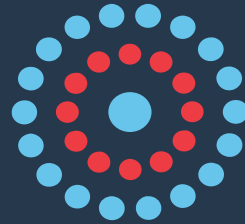
Board of Directors

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
22nd Annual Investment Conference

Dr James Garner
Chief Executive Officer & Managing Director




15 September 2020

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, mid-clinical-stage, small-molecule biotechnology company, headquartered in Sydney, Australia
 Pipeline	Paxalisib – brain-penetrant PI3K / mTOR inhibitor about to enter 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\$ 75 million Current assets at 30 Jun 2020 of ~US\$ 7.7 million

Investment Rationale

Glioblastoma (GBM) is a disease with very high unmet medical need

- 1 GBM is the most common and most aggressive form of brain cancer
- 2 Only existing drug, temozolomide, is ineffective in two-thirds of cases
- 3 Commercial opportunity is ~US\$ 1.5 billion per annum

Kazia's paxalisib (GDC-0084) has a very promising rationale for GBM

- 4 Member of PI3K inhibitor class, which has yielded four FDA-approved cancer drugs
- 5 85-90% of GBM patients have a disordered PI3K pathway
- 6 Only PI3K inhibitor in global pipeline able to cross the blood-brain barrier

Highly encouraging data places paxalisib on a clear path to commercialisation

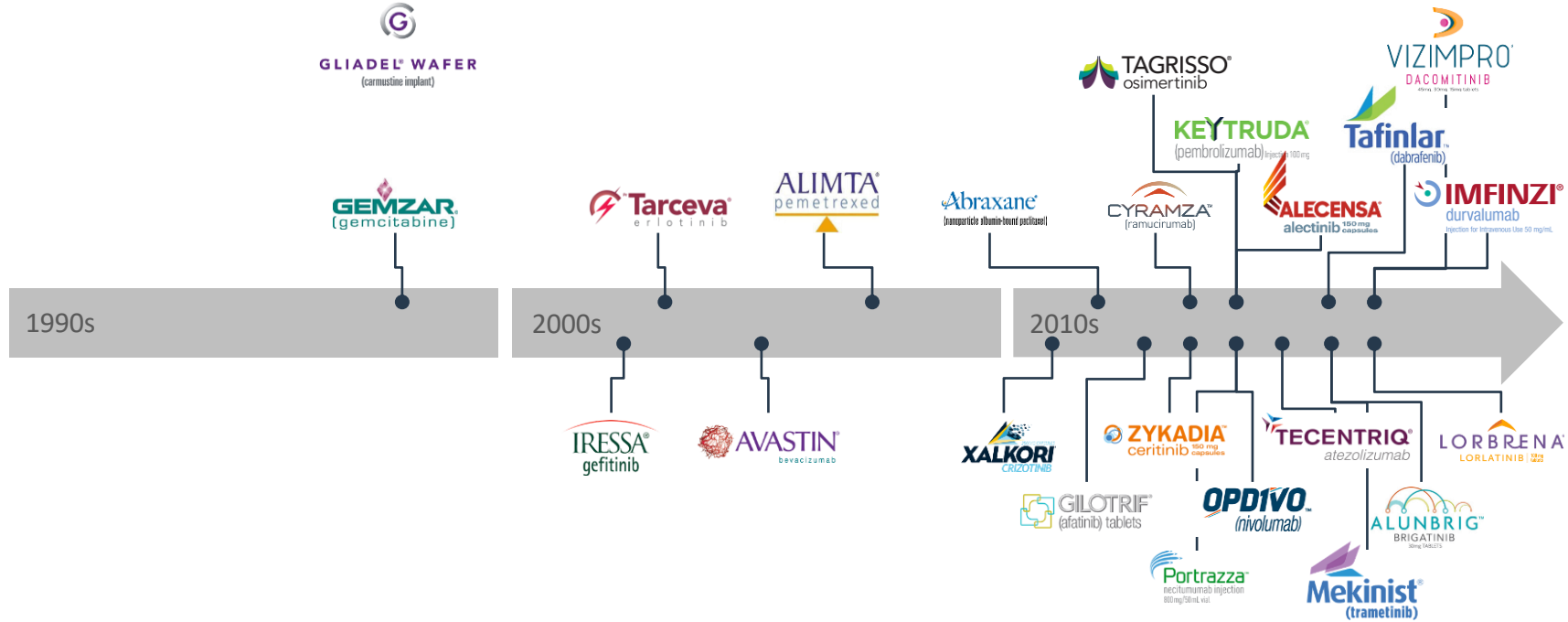
- 7 Strongly encouraging data from ongoing phase II study in GBM
- 8 Clear path-to-market, with international phase III due to start in 2H CY2020
- 9 4x ongoing studies in other forms of brain cancer provide multiple shots on goal

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

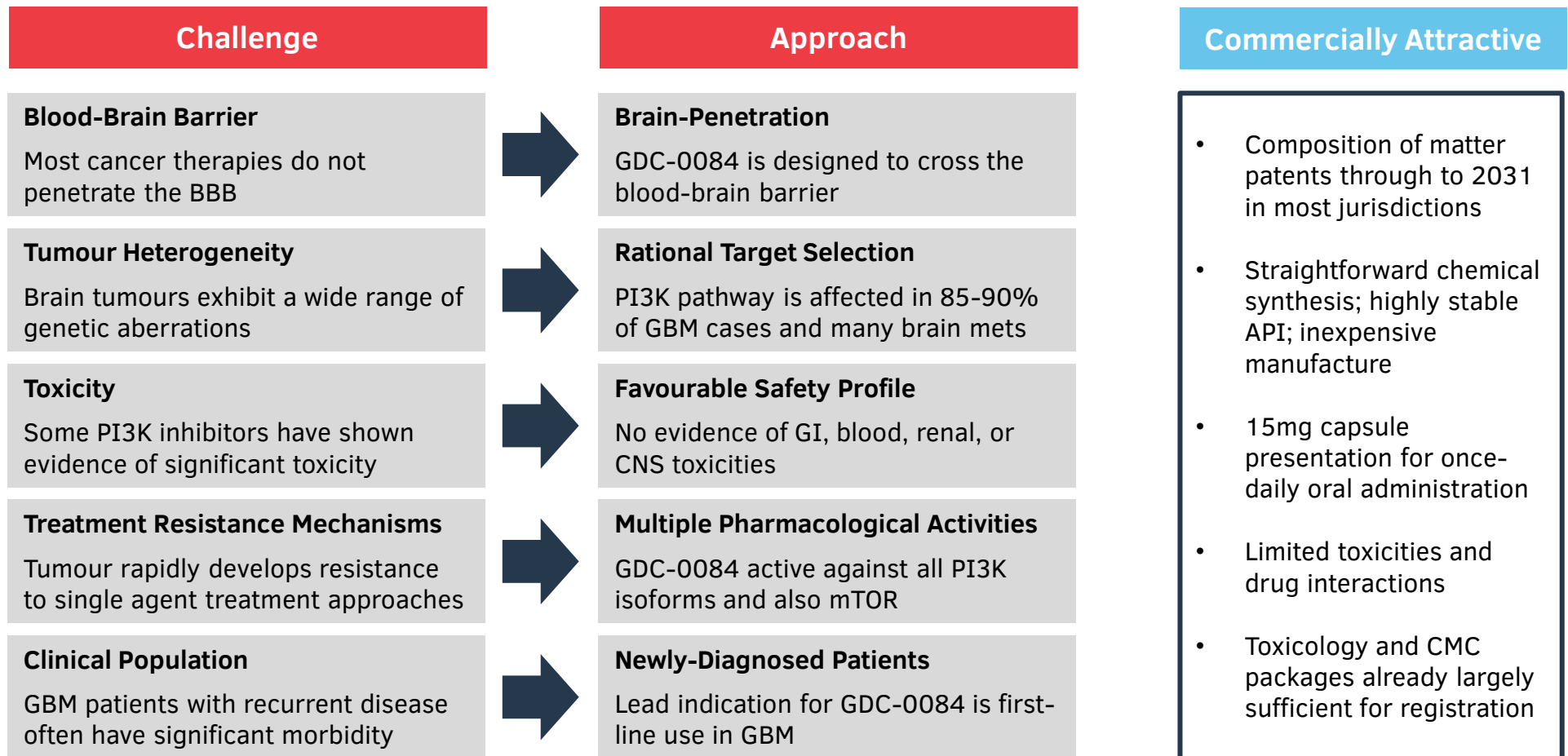
Brain Cancer
(glioblastoma)



Lung Cancer



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



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



Zydelig (idelalisib)



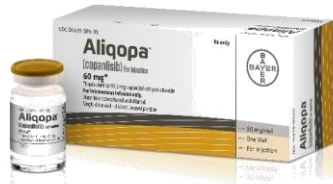
FDA Approved **July 2014** ✓
(blood cancers)
[accelerated approval]

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 ✓



GDC-0084

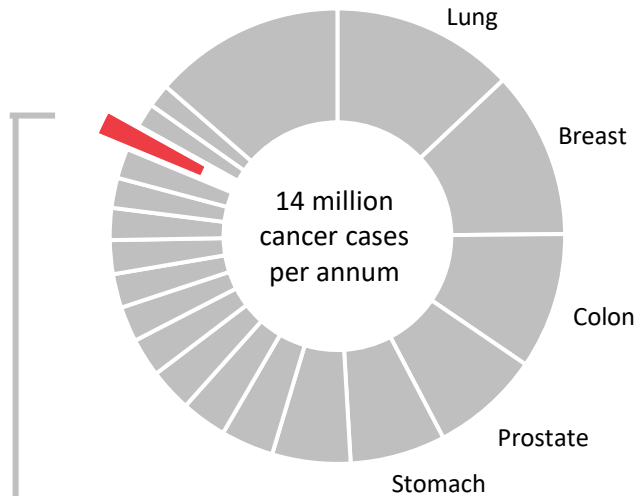


In phase II human trials under US FDA oversight (brain cancer)

Does cross blood-brain barrier ✓

Appears generally safe and well-tolerated thus far ✓

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

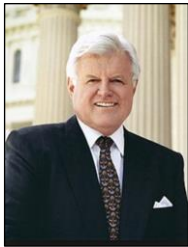
12-15 months
average survival with treatment

Any age, but most common in
60s

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



Sen. John McCain



Sen. Ted Kennedy



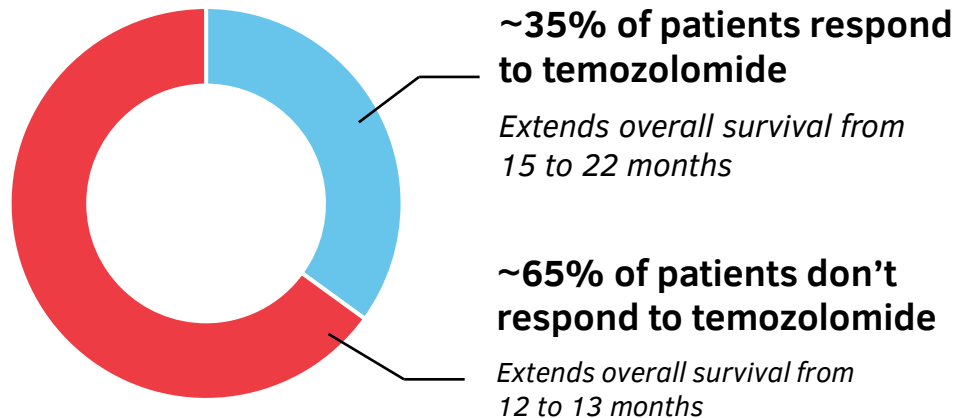
Beau Biden



Dan Case

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

Standard of Care ('Stupp Regimen')



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 current 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



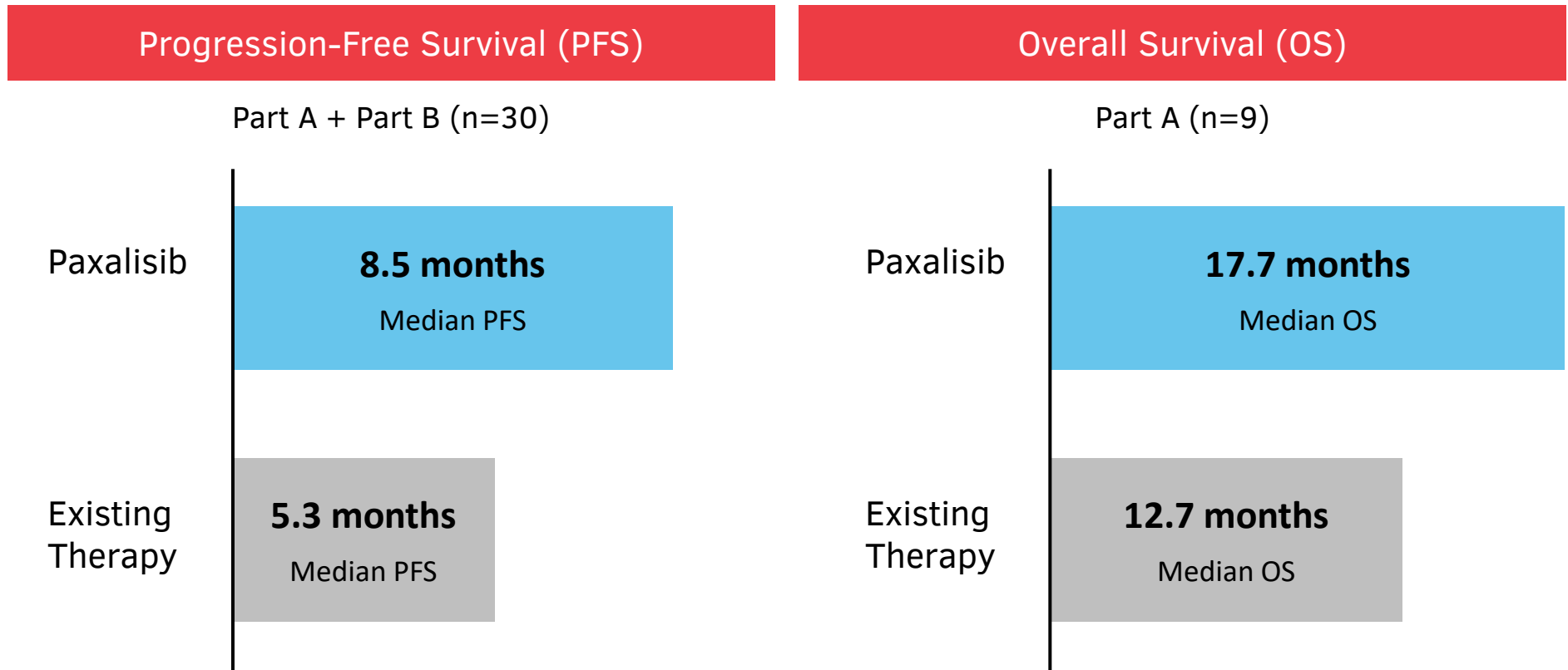
- Interim efficacy data: Apr 2020
- Interim efficacy data: Jun 2020

- 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

New phase II data compares favourably 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)

Glioblastoma

Most common and most aggressive brain tumour

Phase II

[NCT03522298](https://clinicaltrials.gov/ct2/show/study/NCT03522298)



Glioblastoma

(planned pivotal study for approval [in set-up])

Phase II / III

[NCT03970447](https://clinicaltrials.gov/ct2/show/study/NCT03970447)



DIPG

Highly aggressive childhood brain tumour

Phase I

[NCT03696355](https://clinicaltrials.gov/ct2/show/study/NCT03696355)



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

Brain Metastases

Cancer that has spread from any primary tumour

Phase II

[NCT03994796](https://clinicaltrials.gov/ct2/show/study/NCT03994796)



Breast Cancer Brain Mets

(combination with Herceptin®)

Phase II

[NCT03765983](https://clinicaltrials.gov/ct2/show/study/NCT03765983)

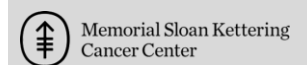


Brain Metastases

(combination with radiotherapy)

Phase I

[NCT04192981](https://clinicaltrials.gov/ct2/show/study/NCT04192981)



Funded by Kazia

Funded Primarily Through Partnerships and External Funding

GBM AGILE is the planned pivotal study for paxalisib in glioblastoma



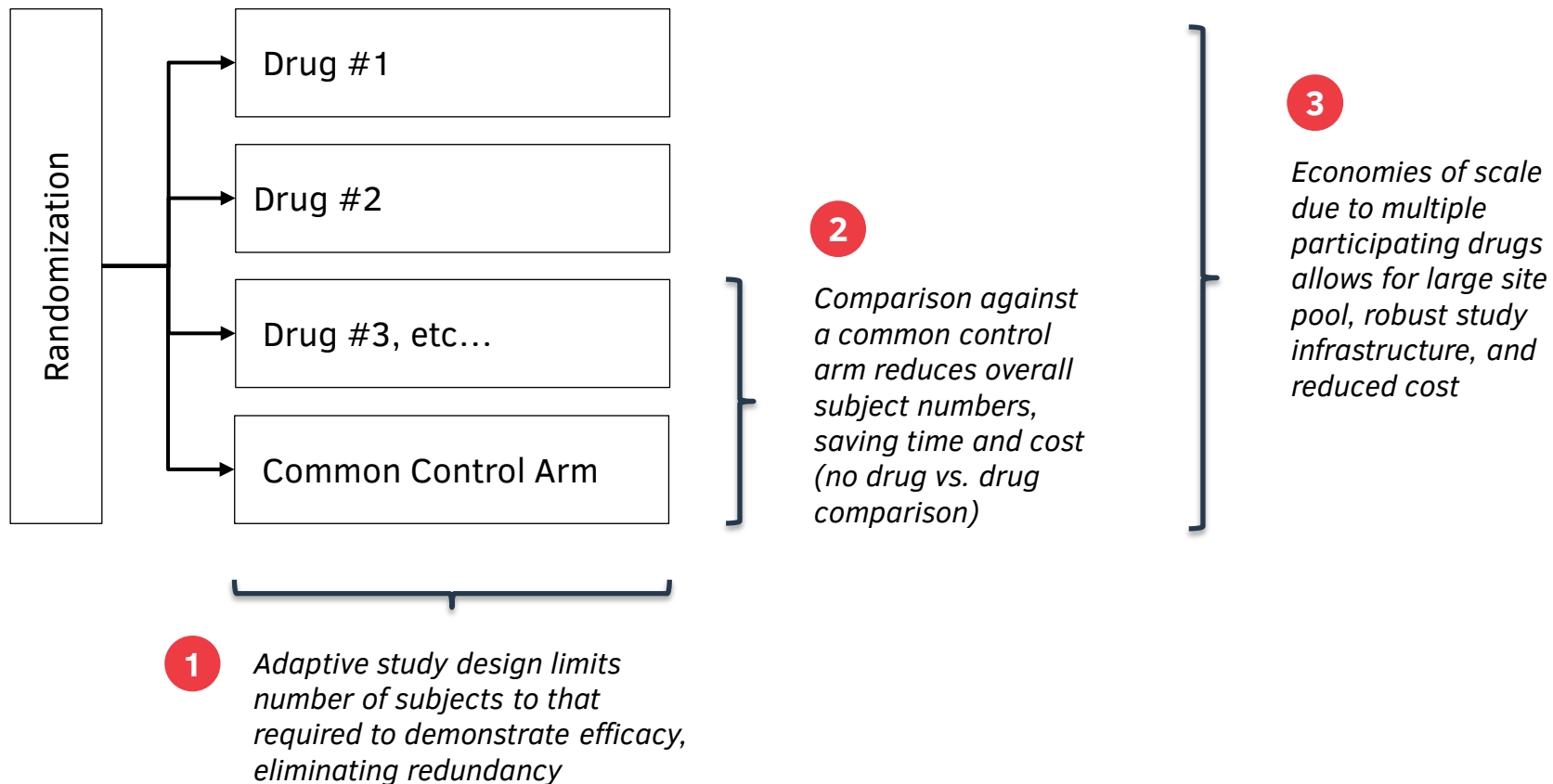
- Primary endpoint of both stages is overall survival (OS); final analysis performed on all patients from both stages, compared to all control patients recruited to date
- Stage 1 is the primary efficacy analysis; Stage 2 is a confirmatory component
- Study is designed to provide definitive data to support product registration if a candidate drug is efficacious
- Anticipated duration: 2 – 3 years

Current Status

GBM AGILE: Recruiting in US and Canada; planned to open in EU and China in CY2021
Paxalisib Participation: Enrolment expected to commence in 2H CY2020

- Sponsored by Global Coalition for Adaptive Research (GCAR), a 501(c)(3) non-profit
- Strongly endorsed by FDA and leading brain cancer KOLs
- Paxalisib expected to be second drug to join the study, after Bayer's Stivarga (regorafenib)
- Extensive funding support from National Brain Tumor Society, Cure Brain Cancer Foundation, and other bodies

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



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

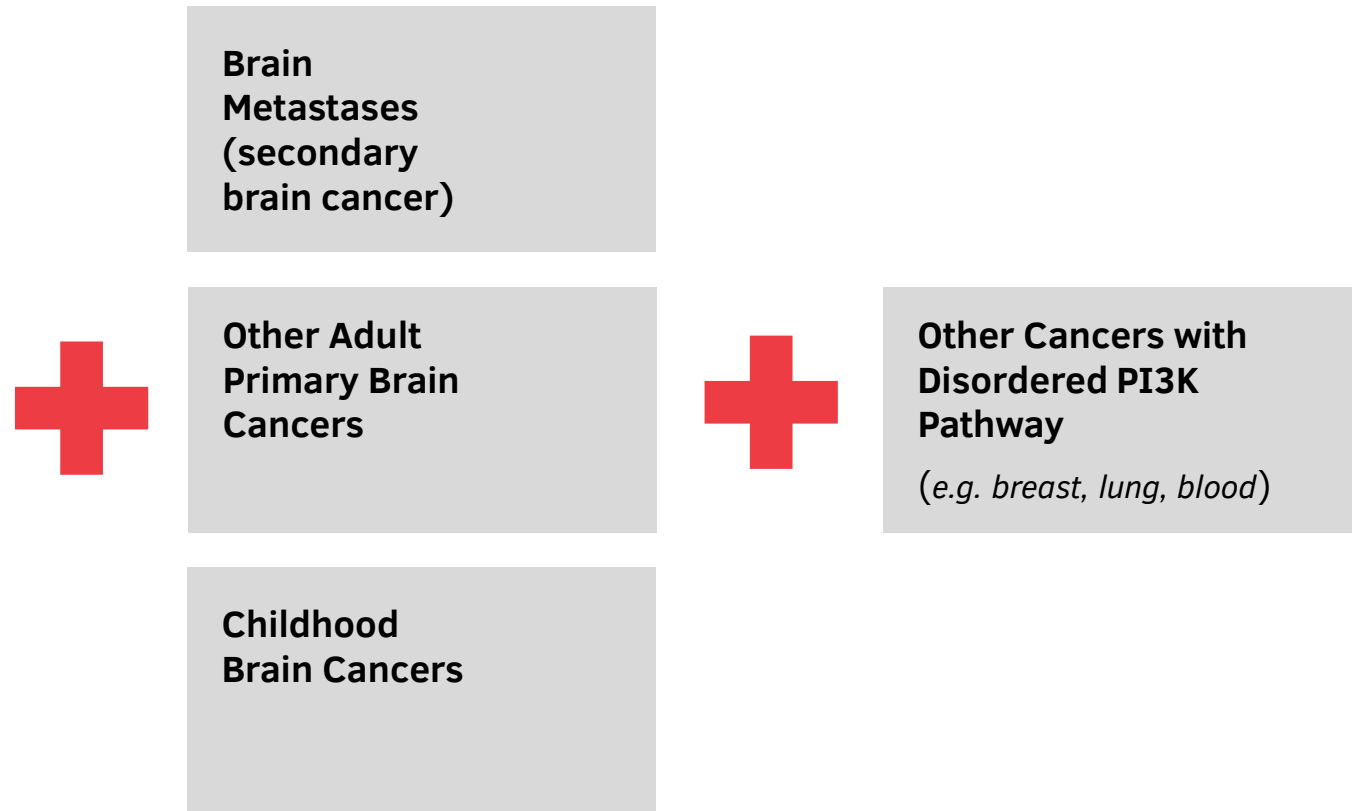
- ‘Data exclusivity’ provides additional protection against competition beyond granted patents
- Waiver of up to US\$ 6 million in FDA fees at time of filing for marketing authorisations
- Eligibility for orphan grants
- Eligibility for priority review voucher at time of filing for marketing authorisation 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

Other Cancers with Disordered PI3K Pathway
(e.g. breast, lung, blood)

Kazia has delivered all milestones to date, with multiple data read-outs expected over 6-12 months

2019	2020		2021	
H1	H1	H2	H1	H2
Commence NCI-funded 'Alliance' study in brain metastases	Further efficacy data from phase II GBM study at ASCO & AACR	FDA Fast Track Designation for GBM	Final data from phase II GBM study	Anticipated data from phase I MSKCC radiotherapy study
Commence radiotherapy combination study with Sloan-Kettering		FDA Rare Pediatric Disease Designation for DIPG	Final data from phase I St Jude DIPG study	Anticipated data from phase II Dana-Farber BCBM study
Initial interim efficacy data from phase II GBM study at SNO		Further data from phase II GBM study at SNO (Nov 20)	Anticipated data from phase II Alliance brain mets study	
		Initial data from phase I St Jude DIPG study		
		Initial data from phase II Dana-Farber BCBM study		
		Commence recruitment to GBM AGILE pivotal study		

■ Complete
 Anticipated

Note: forward-looking milestones are forecast and indicative but subject to revision, particularly in light of changing conference schedules

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



Market Capitalisation	~US\$ 75 million								
Shares on Issue	~94 million								
Listing	ASX: KZA NASDAQ: KZIA (1:10 ratio)								
Balance Sheet (as at 30 Jun 20)	Current Assets: US\$ 7.7M FY20 Spend: US\$ 9M Runway: 2Q CY2021 Efficiency: ~80% R&D								
Key Shareholders	<table border="0"> <tbody> <tr> <td>Hyecorp (SYD family office)</td> <td style="text-align: right;">17%</td> </tr> <tr> <td>Platinum Asset Mgmt.</td> <td style="text-align: right;">9%</td> </tr> <tr> <td>Other Institutional</td> <td style="text-align: right;">~10%</td> </tr> <tr> <td>Board & Mgmt.</td> <td style="text-align: right;">2%</td> </tr> </tbody> </table>	Hyecorp (SYD family office)	17%	Platinum Asset Mgmt.	9%	Other Institutional	~10%	Board & Mgmt.	2%
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Note: as at 31 August 2020, unless otherwise noted

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





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