

ASX RELEASE
14 SEPTEMBER 2022

KAZIA TO PRESENT TO HCW BIOCONNECT INVESTOR CONFERENCE

Sydney, 14 September 2022 – Kazia Therapeutics Limited (NASDAQ: KZIA; ASX: KZA), an oncology-focused drug development company is pleased to provide the presentation due to be delivered by the CEO, Dr James Garner, to the H C Wainwright Global Investment Conference in New York, NY on 14 September 2022.

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About Kazia Therapeutics Limited

Kazia Therapeutics Limited (NASDAQ: KZIA; ASX: KZA) is an oncology-focused drug development company, based in Sydney, Australia.

Our lead program is paxalisib, a brain-penetrant 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 commenced recruitment to GBM AGILE, a pivotal study in glioblastoma, in January 2021. 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, and for AT/RT in June 2022.

Kazia is also developing EVT801, a small-molecule inhibitor of VEGFR3, which was licensed from Evotec SE in April 2021. Preclinical data has shown EVT801 to be active against a broad

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

range of tumour types and has provided compelling evidence of synergy with immunology agents. A phase I study commenced recruitment in November 2021.

For more information, please visit www.kaziatherapeutics.com or follow us on Twitter @KaziaTx.

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



KAZIA
THERAPEUTICS



A Diversified Oncology Drug Development Company

Presentation to HC Wainwright
Global Investment Conference

New York, NY
14 September 2022

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 substantial risks and uncertainties, not all of which may be known at the time. All statements contained in this presentation, other than statements of historical fact, including statements regarding our strategy, research and development plans, collaborations, future operations, future financial position, future revenues, projected costs, prospects, plans, and objectives of management, are forward-looking statements. Not all forward-looking statements in this presentation are explicitly identified as such.

Many factors could cause the actual results of the Company to differ materially from the results expressed or implied herein, and you should not place undue reliance on the forward-looking statements. Factors which could change the Company's expected outcomes include, without limitation, our ability to: advance the development of our programs, and to do so within any timelines that may be indicated herein; the safety and efficacy of our drug development candidates; our ability to replicate experimental data; the ongoing validity of patents covering our drug development candidates, and our freedom to operate under third party intellectual property; our ability to obtain necessary regulatory approvals; our ability to enter into and maintain partnerships, collaborations, and other business relationships necessary to the progression of our drug development candidates; the timely availability of necessary capital to pursue our business objectives; and our ability to attract and retain qualified personnel; changes from anticipated levels of customer acceptance of existing and new products and services and other factors.

Although the Company believes that the expectations reflected in such forward-looking statements are reasonable, there can therefore be no assurance that such expectations will prove to be correct. The Company has no obligation as a result of this presentation to clinical trial outcomes, sales, partnerships, 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, or marketing existing products.

In addition, the extent to which the COVID-19 outbreak continues to impact our workforce and our discovery research, supply chain and clinical trial operations activities, and the operations of the third parties on which we rely, will depend on future developments, which are highly uncertain and cannot be predicted with confidence, including the duration and severity of the outbreak, additional or modified government actions, and the actions that may be required to contain the virus or treat its impact.

Any forward-looking statements contained in this presentation speak only as of the date this presentation is made, and we expressly disclaim any obligation to update any forward-looking statements, whether because of new information, future events or otherwise.

Company Overview

A late-clinical-stage oncology drug development company



Paxalisib

Brain-penetrant pan-PI3K / mTOR inhibitor

- Well-validated class with 5x FDA-approved therapies
- Only brain-penetrant PI3K inhibitor in development

Currently in phase III for glioblastoma

- Only FDA-approved drug is ineffective for 2/3 cases
- Orphan indication with very high unmet clinical need

7 other ongoing clinical trials

- Focus on DIPG, Brain Metastases, Glioblastoma
- Collaborations with world-leading cancer centers

Rich commercial opportunity

- Glioblastoma alone sized at US\$ 1.5 billion per annum
- Commercial licensee in place for China

Final Phase III Data: 2H CY2023

EVT801

Selective VEGFR3 inhibitor

- Avoid off-target toxicity of older angiokinase inhibitors
- Primarily targets lymphangiogenesis

Currently in phase I for advanced solid tumors

- Adaptive, biomarker-rich study ongoing at 2 sites in France

Potential use in many solid tumors

- Potential indications include: renal cell carcinoma, liver cancer, colon cancer, thyroid cancer, and sarcoma

Potential combination with immunotherapy

- Strong evidence of synergy in preclinical data opens possibility of monotherapy or combination use

Initial Phase I Data: 2H CY2022 / 1H CY2023

Company is dual-listed on NASDAQ (KZIA) and ASX (KZA) with market cap around US\$ 30 million

Licensing-driven business model, with programs sourced from Genentech (paxalisib) and Sanofi / Evotec (EVT801)

Cash runway to 1Q CY2023, with potential opportunities for non-dilutive income via additional partnering activity

Lean virtual pharma model, with ~75% of cashflows applied directly to clinical trials

Pipeline

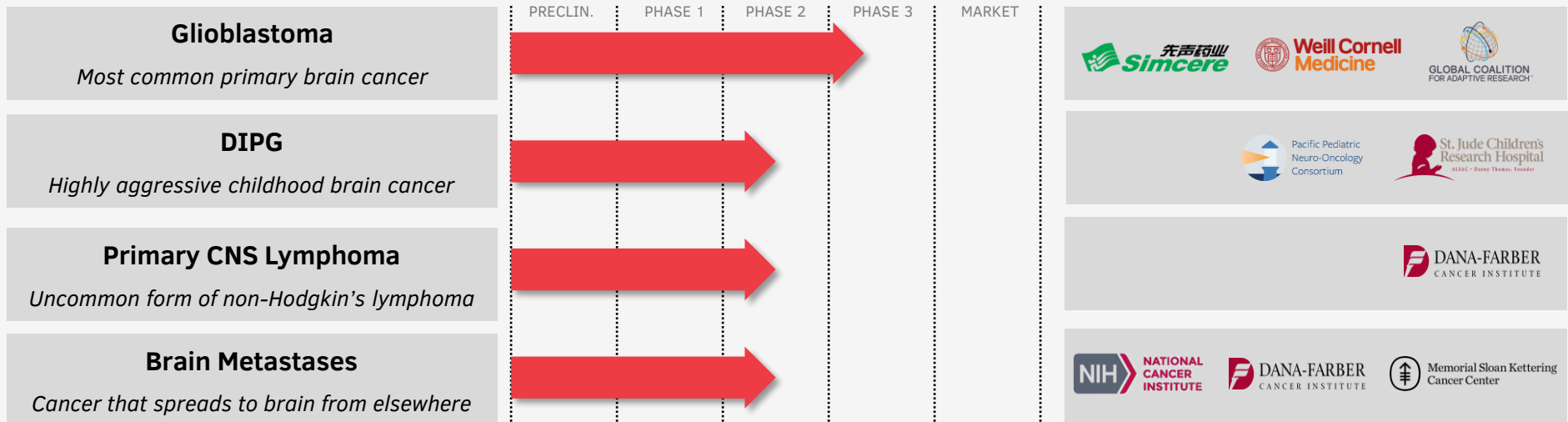
Two world-class assets in clinical trials

Paxalisib (formerly GDC-0084)

Small molecule, highly potent, brain-penetrant inhibitor of PI3K / mTOR

licensed from:

Genentech
IN BUSINESS FOR LIFE



EVT801

Small molecule, highly specific inhibitor of VEGFR3

licensed from:

evotec



Paxalisib – Kazia’s Lead Program

Multiple signals of clinical efficacy in brain tumors

Glioblastoma

**15.7 months
overall survival**

*in newly-diagnosed patients with
unmethylated MGMT promotor*

(vs. 12.7 months for existing
standard-of-care therapy)



International pivotal study in
glioblastoma now ongoing,
with final data expected 2H CY2023

[Wen et al. \(2022\) J Clin Oncol. 40\(16 Suppl\): 2037](#)

Single-arm phase II study of paxalisib in newly-
diagnosed unmethylated glioblastoma (n=30);
comparator figures are from [Hegi et al. \(2005\)](#).

Brain Metastases

**100%
overall response rate**

*in combination with whole brain
radiotherapy (WBRT)*

(vs. 20-50% in comparable studies
of WBRT in brain metastases)



Potential second indication, with
>200,000 patients per annum in
United States alone

[Yang et al. \(2022\) Oral presentation at SNO-ASCO
brain mets conference](#)

Single-arm phase I study of paxalisib in combination
with WBRT for brain mets of any origin (n=9);

DIPG

**“dramatic reductions in tumor
volume and complete resolution of
disease symptoms, extending
overall survival”**

*in case reports from compassionate
use experience*

(vs. 9-11 months median survival
with existing standard-of-care)



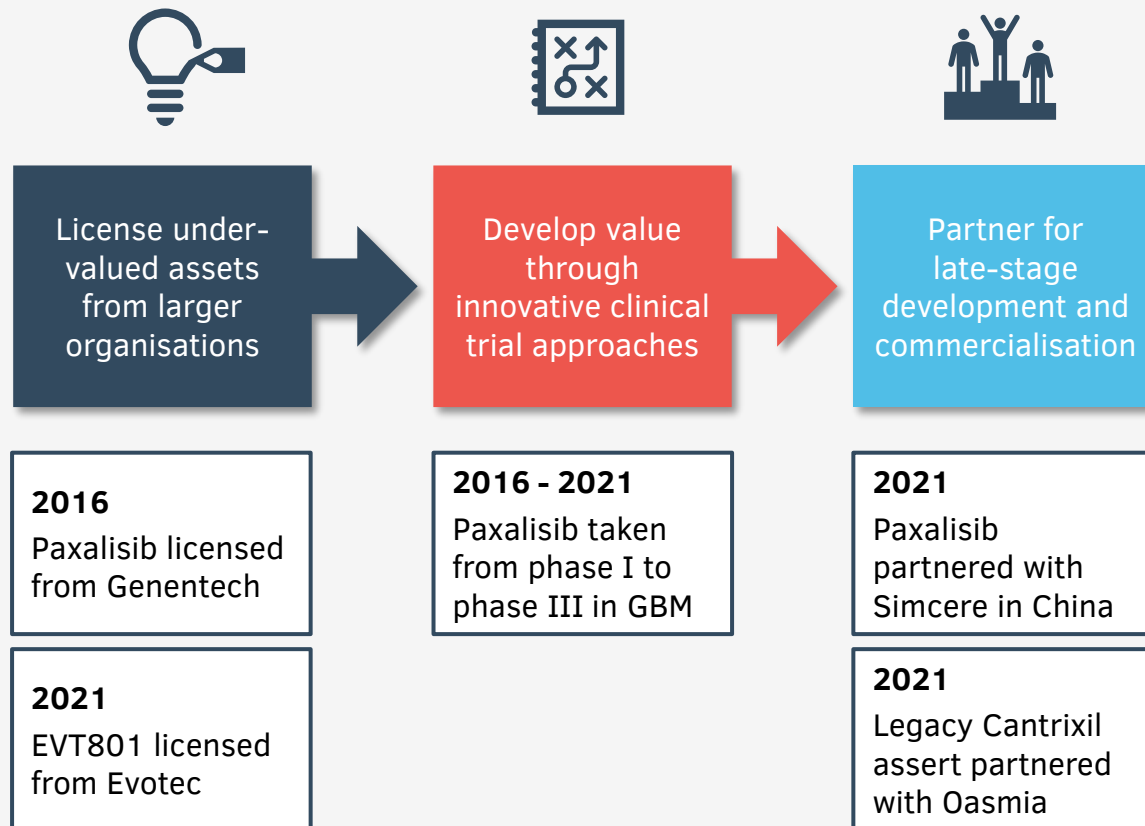
Potential to secure a pediatric
priority review voucher (pPRV) if
initially approved in DIPG

[Dun et al. \(2022\) Presentation at ISPNO conference](#)

Compassionate experience in 2 children (16yo & 6yo)
treated with pax+ONC201; second patient remains
ongoing

Operating Model

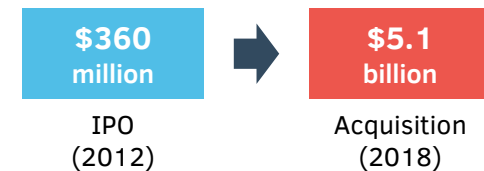
In-licensing advanced assets drives earlier value realization



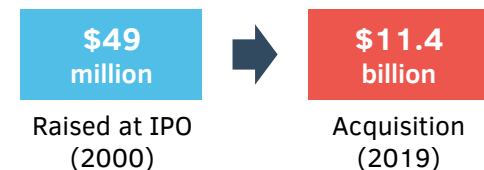
A Proven Strategy



Jun 2010 – licensed niraparib from Merck
Mar 2017 – Zejula® (niraparib) approved by FDA
Dec 2018 – Tesaro acquired by GSK



Dec 2014 – reclaims binimetinib from Novartis
Sep 2017 – licenses encorafenib from Novartis
Jun 2018 – combination approved by FDA



Leadership

160+ years of international drug development experience

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



Management Team



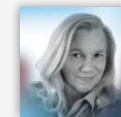
Dr James Garner
Chief Executive Officer
& Executive Director

Physician / MBA; Extensive drug development experience



Dr John Friend
Chief Medical Officer

Industry physician with >25 years experience in oncology drug development



Karen Krumeich
Chief Financial Officer

Accountant with >20 years experience as a biotech CFO in public and private companies



Kate Hill
Company Secretary

Former audit partner at Deloitte and experienced Board director for multiple public companies



Scientific Advisory Board

World-leading experts in brain cancer



Priscilla K Brastianos, MD

Associate Professor of Medicine
Harvard Medical School

Assistant Physician in Medicine,
Hematology/Oncology
Massachusetts General Hospital



John de Groot, MD

Division Chief, Neuro-Oncology
UCSF

formerly
Director of Clinical Research
MD Anderson Cancer Center



Alan Olivero, PhD

Drug Development Consultant

formerly
Senior Director, Discovery
Chemistry & Head of Research
Operations
Genentech, Inc



Patrick Y Wen, MD

Professor of Neurology
Harvard Medical School

Director of the Center for Neuro-
Oncology
Dana-Farber Cancer Institute



>400 peer-
reviewed
academic
publications



>40 patent
inventorships

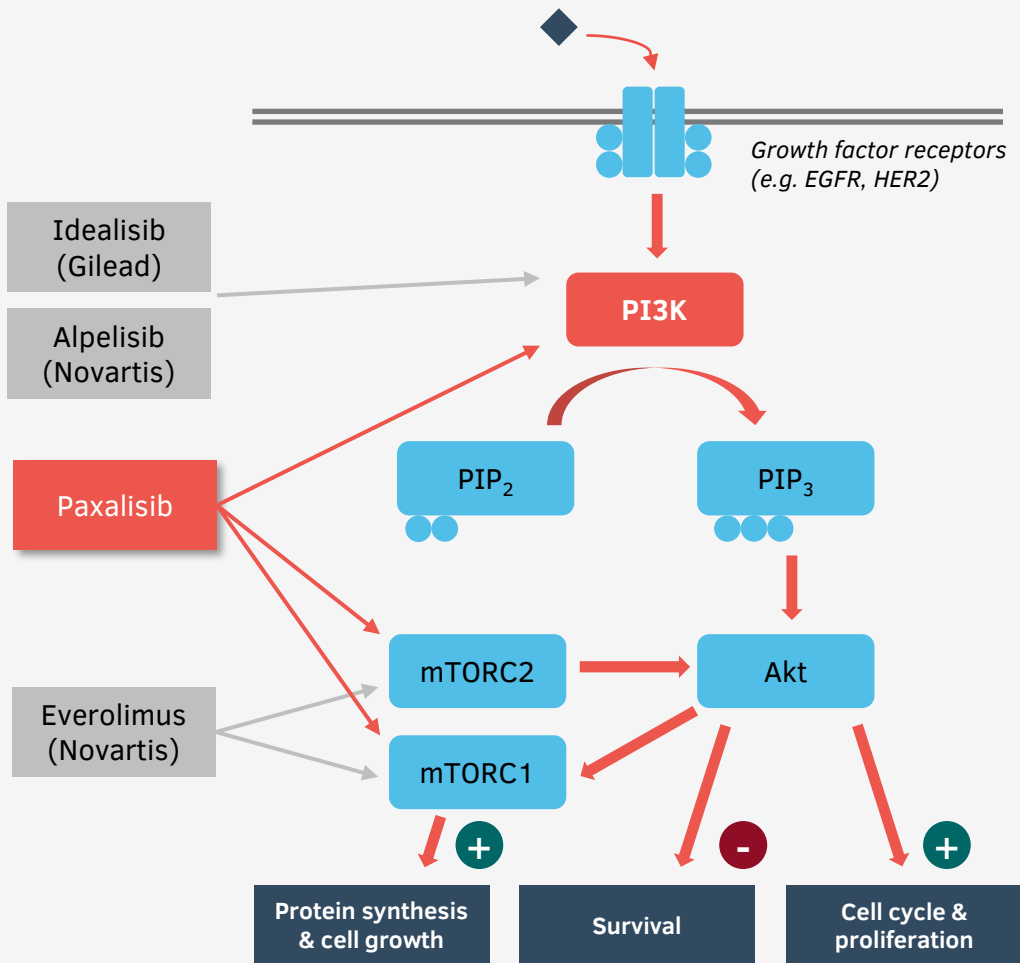


>100 brain
cancer clinical
trials as principal
investigator



Extensive relationships
with NIH, NCI, SNO,
NBTS, and other
organizations

Paxalisib is one of the most broadly potent PI3K inhibitors in the global pipeline



Paxalisib Among Most Potent PI3K Inhibitors

	IC ₅₀ (nM)				
	p110α	p110β	p110γ	p110δ	mTORC 1/2
Paxalisib	2	46	10	3	70
Idelalisib	820	565	89	2.5	>1,000
Alpelisib	5	1200	250	290	>9,100
Buparlisib	52	166	262	116	4,600
Pilaralisib	39	383	23	36	>15,000

Note: lower IC₅₀ implies more potent activity
 Source: HF Zhao et al. (2017) *Molecular Cancer*. 16:100

The PI3K inhibitor 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)



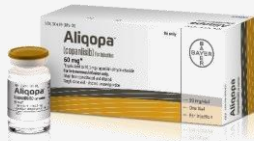
*Crosses
Blood-
Brain
Barrier*

Safety

Potentially fatal
liver toxicity and
diarrhoea



Aliqopa
(copanlisib)



FDA Approved
September 2017
(blood cancers)



Potentially fatal
infections



Copiktra
(duvelisib)



FDA Approved
October 2018
(blood cancers)



Potentially fatal
infections and
diarrhoea



Piqray
(alpelisib)



FDA Approved
May 2019
(breast cancer)



Modest toxicities to
date



Ukoniq
(umbralisib)



FDA Approved
February 2021
(blood cancers)



Serious infections,
hepatotoxicity, and
diarrhoea



paxalisib



In pivotal study for
FDA Approval in
glioblastoma

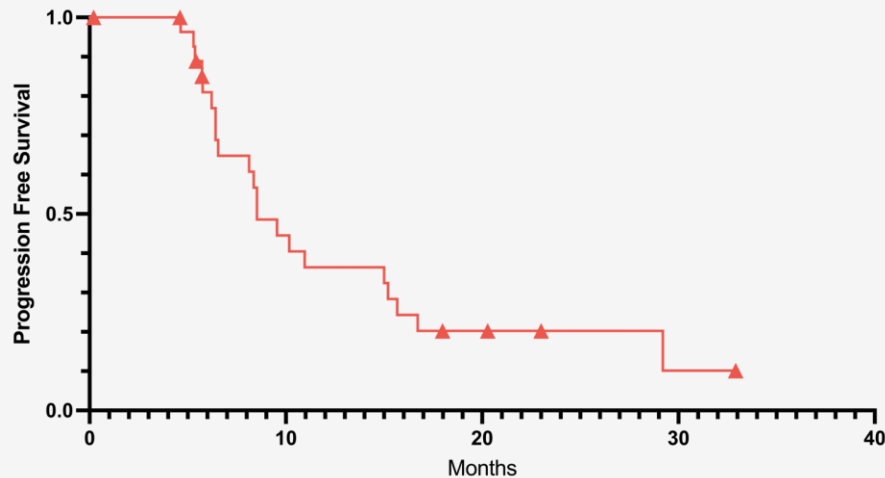


Modest toxicities to
date

Phase II study of paxalisib mono-therapy in newly-diagnosed GBM provides robust signal of clinical efficacy

Progression-Free Survival (PFS)

(n=30)

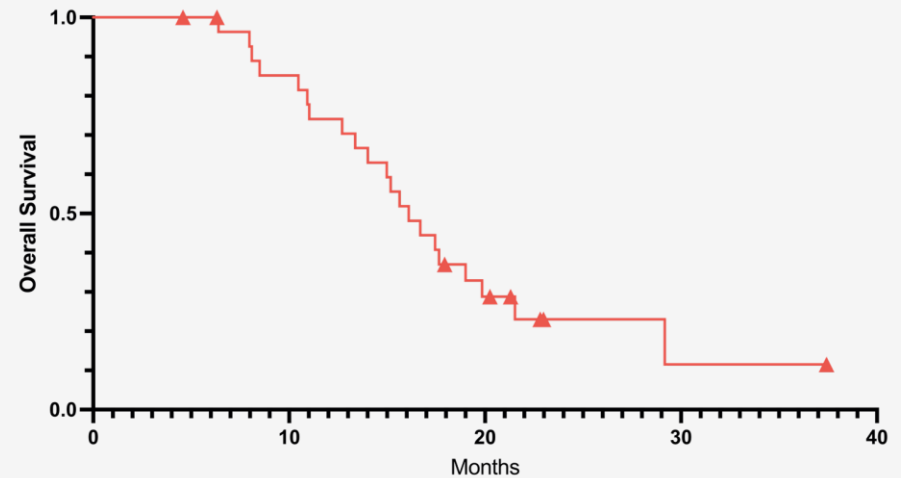


Median PFS: **8.6 months** (6.6-11.0)

Comparator figure for existing therapy: **5.3 months**
(Hegi et al. 2005)

Overall Survival (OS)

(n=30)



Median OS: **15.7 months** (11.1-19.1)

Comparator figure for existing therapy: **12.7 months**
(Hegi et al. 2005)

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

Efficacy signal is generally corroborated by comparison against multiple comparative reference data points

Study	Year	<i>n</i>	OS (95% CI)	Applicability	Comments
Kazia Phase II Study	2022	30	15.7 (11.1-19.1)		
EORTC-NCIC Hegi et al.	2005	60	12.7 (11.6-14.4)	Good	Pivotal study that led to the approval of temozolomide for glioblastoma
Motomora et al.	2011	29	12.5	Moderate	Single-center retrospective study in Japan
RTOG-0525 Gilbert et al.	2013	254	14.6 (13.2-16.5)	Poor	All patients were dosed to 12 cycles of TMZ, an unapproved regimen
RTOG-0825 Gilbert et al.	2014		14.6	Moderate	Some patients were dosed to 12 cycles of TMZ, an unapproved regimen
CORE Nabors et al.	2015	89	13.4 (12.2-14.3)	Good	
Stupp et al.	2017	95	14.7 (9.8-24.8)	Moderate	Large proportion of patients recruited outside US / EU
VERTU Sim et al.	2021	41	12.8 (9.5-15.8)	Good	

Note: all data is for newly-diagnosed unmethylated patient group; applicability based on comparability of patient population and study design to Kazia phase II study

Safety Profile in the phase 2 clinical study in GBM patients is generally mild to moderate, reversible, and manageable

Number of Patients at Any Dose (n=30) Experiencing AEs 'Possibly' or 'Likely' Related to Paxalisib (affecting ≥10% of patients)

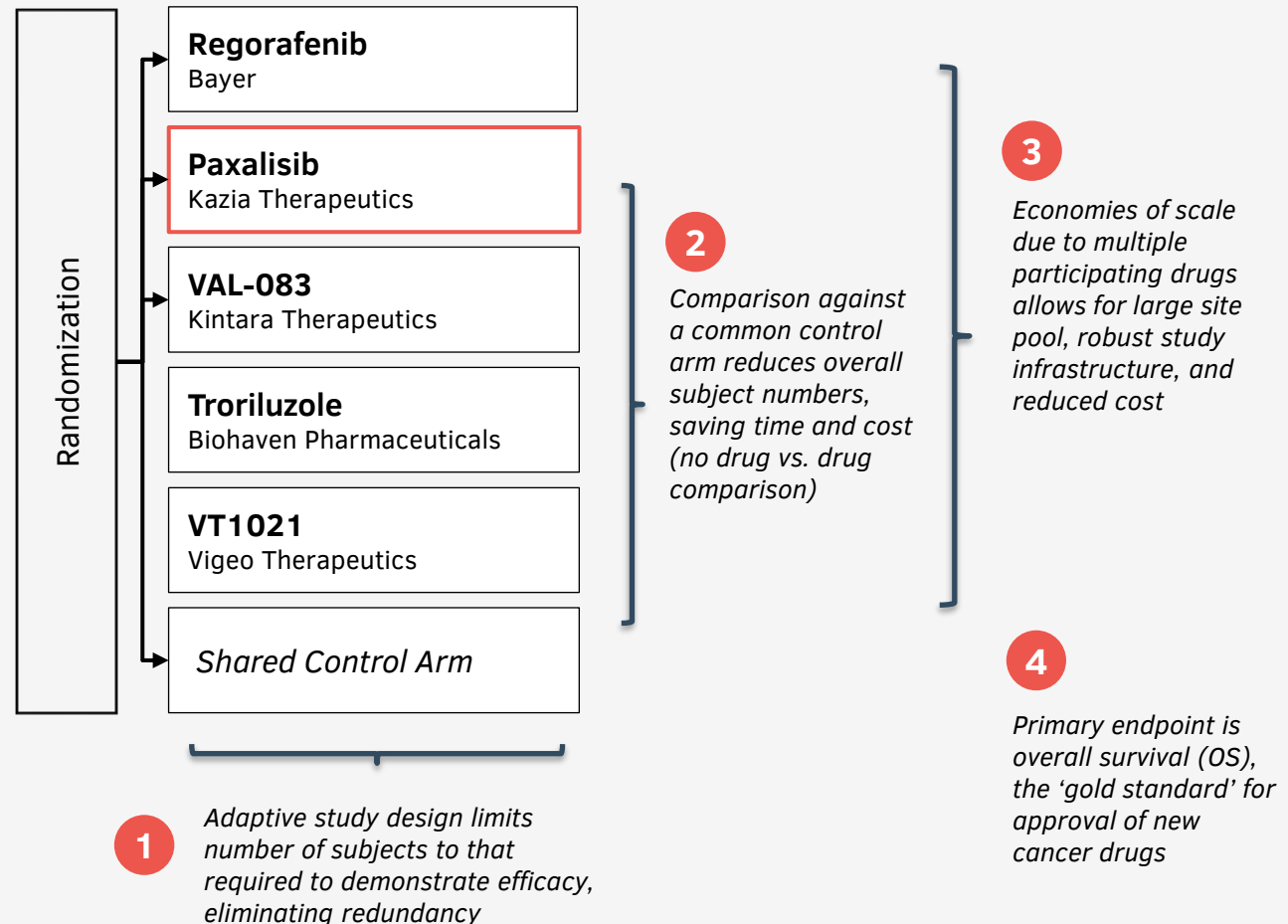
Term	Gr 1	Gr 2	Gr 3	Gr 4	Total (%)
Fatigue	3	13	2		18 (60%)
Stomatitis	4	7	3		14 (47%)
Decreased appetite	6	6	1		13 (43%)
Hyperglycemia	3	1	6	2	12 (40%)
Nausea	4	6	1		11 (37%)
Rash, maculo-popular	1	1	7		9 (30%)
Diarrhea	7	1			8 (27%)
Vomiting	4	2	1		7 (23%)
Rash	2	4	1		7 (23%)
Neutrophils decreased	3	3		1	7 (23%)
Platelets decreased	6	1			7 (23%)
Weight decreased	5	2			7 (23%)
Lymphocytes decreased	2	3			5 (17%)
Dehydration		4	1		5 (17%)
Dysgeusia		4			4 (13%)
Cholesterol increased	4				4 (13%)
ALT increased	1		2		3 (10%)
Triglycerides increased	1	2			3 (10%)
Malaise	2	1			3 (10%)

GBM AGILE international pivotal study is underway

Sponsored by GCAR with support of GBM key opinion leaders

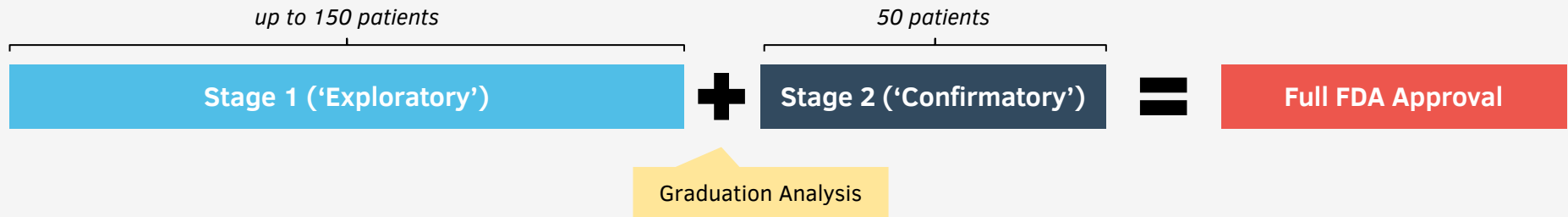
Key Points

- A 'platform study', run independently of individual companies, designed to expedite the approval of new drugs for glioblastoma
- Multiple drugs are evaluated in parallel, saving time and money
- Not a 'winner-takes-all' approach: multiple drugs can succeed
- Cutting-edge 'adaptive design' avoids redundant recruitment, expediting path to market
- FDA acknowledgement that data expected suitable for registration

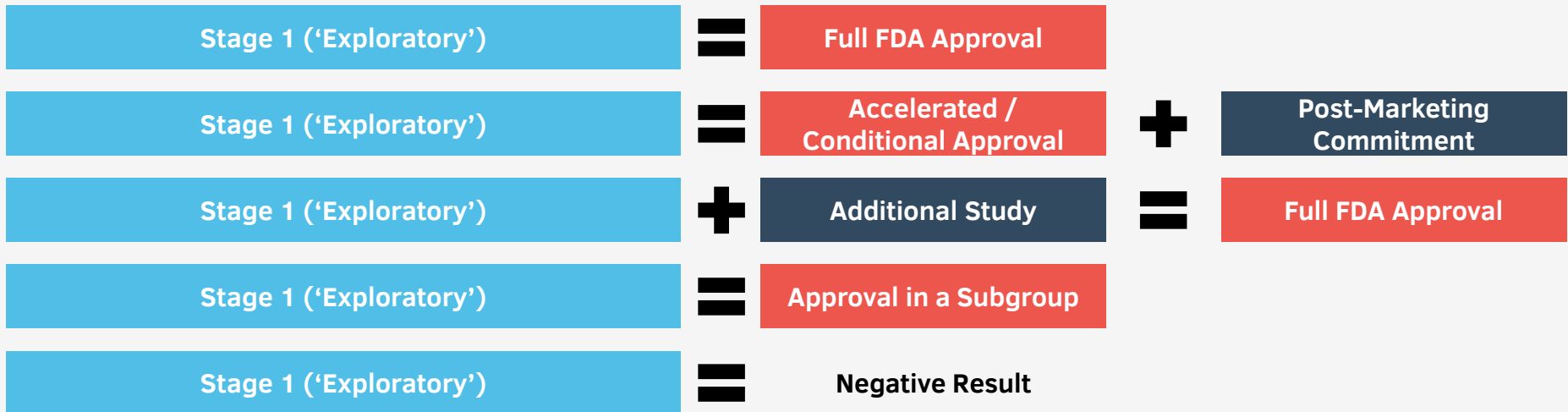


GBM AGILE was designed as a two-stage study; first stage may provide sufficient data for registration

OVERALL STUDY DESIGN

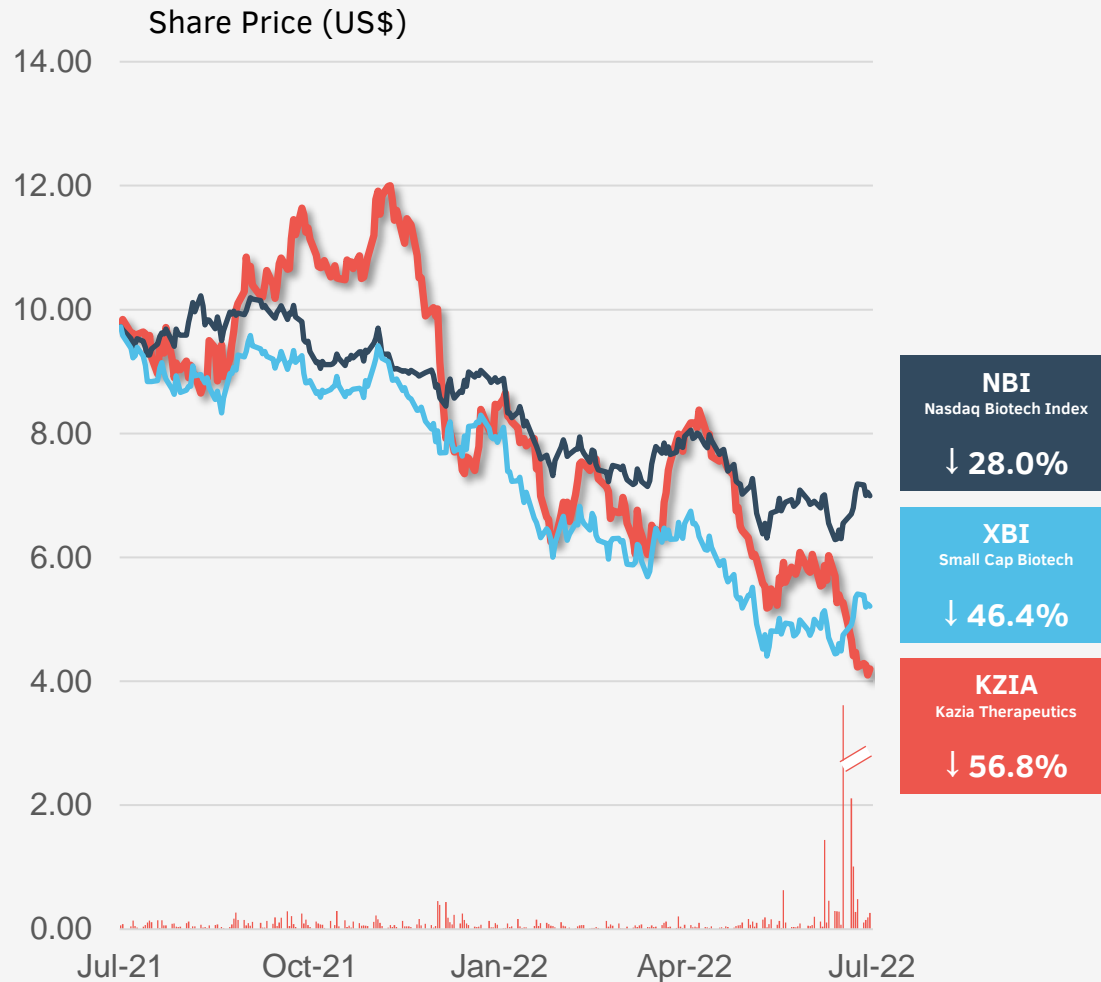


POTENTIAL OUTCOMES FOR PAXALISIB, GIVEN DECISION IN AUGUST 2022 NOT TO GRADUATE TO STAGE 2



Financial Metrics

Lean operating model drives financial efficiency



Market Capitalisation	US\$ 60M
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Listing

ASX (primary)	KZA
NASDAQ (ADSs @ 1:10 ratio)	KZIA
Shares on Issue	138M
Average Daily Volume (12 months)	\$525,000

Balance Sheet	US\$
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Cash (at 30 Jun 22)	\$5M
Monthly Burn Rate	~\$1M

Substantial Shareholders

Willoughby Capital	14%
Quest Asset Partners	8%
Platinum Asset Management	5%
Board and Management	2%

CY2022 Milestones and Newsflow

Multiple catalysts across two clinical programs

Open GBM AGILE paxalisib arm to recruitment in EU	1H CY2022	✓
Commence recruitment to paxalisib phase II GBM study at Weill Cornell	1H CY2022	✓
Preclinical data for paxalisib in AT/RT presented at AACR (April 2022)	1H CY2022	✓
Preclinical data for paxalisib in DIPG presented at ISPNO (June 2022)	1H CY2022	✓
Final data from Kazia's paxalisib phase II study in GBM presented at ASCO (June 2022)	1H CY2022	✓
Initial data from paxalisib phase II brain metastases study with Alliance for Clinical Trials in Oncology	1H CY2022	✓
Initial interim data from paxalisib + radiotherapy phase I brain mets study at Memorial Sloan-Kettering	2H CY2022	✓
Paxalisib granted orphan drug designation in AT/RT by FDA	1H CY2022	✓
Paxalisib granted rare pediatric disease designation in AT/RT by FDA	2H CY2022	✓
<i>Further preclinical data on paxalisib in childhood brain cancer published in peer-reviewed journals</i>	2H CY2022	
Initial interim data from paxalisib phase II PCNSL study at Dana-Farber	1H CY2023	
Initial interim data from Kazia's EVT801 phase I trial	1H CY2023	
<i>Final data from GBM AGILE pivotal study of paxalisib</i>	2H CY2023	

Italics – updated guidance

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



KAZIA

THERAPEUTICS

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