

Fast Facts

Ticker	ORY.MC / ORY.SM
Exchange	Madrid Stock Exchange
Stock Price ¹	€2.05
Market Cap ¹	~€120.1M
Cash ²	€13.4M
Outstanding Shares	58.6M
52-Week Trading Range (€)	2.05-2.88
Avg. Daily Volume (200-day)	121,817
Headquarters	Barcelona, Spain
Fiscal Year End	December 31 st

Analyst Research Coverage

Jefferies	Lucy Codrington
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Ladenburg Thalmann	Ahu Demir
Van Lanschot Kempen	S. van Voorthuizen
Rx Securities	Joseph Hedden
Edison	Soo Romanov
Invest Securities	Jamila El Bougrini
Estrategias de Inversión	Maria Mira
El Análisis de:	Luis Navia

Pioneering personalized medicine in epigenetics

ORYZON is a clinical stage biopharmaceutical company and a leader in the development of epigenetics-based therapeutics. We are developing two Phase II small molecule inhibitors of Lysine Specific Demethylase 1 (LSD1): **vafidemstat** and **iadademstat**, in two distinct therapeutic fields – CNS disorders and oncology.

Our experienced trans-Atlantic management team with vast drug development experience leverages key collaborations with prestigious clinical centers (Johns Hopkins, Columbia, Massachusetts General Hospital, Fox Chase Cancer Center) to optimize development efficiency.

Our expanding Phase II for both molecules builds on a clear demonstration of safety. Over 500 subjects treated (430+ vafidemstat; 100+ iadademstat), including elderly and unfit patients.

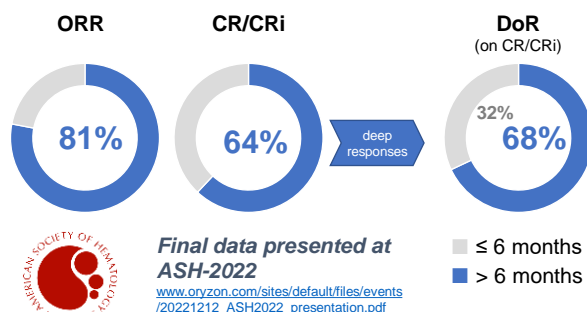
Vafidemstat:

- First and only LSD1 inhibitor in CNS disorders. Currently in two Phase IIb trials in Borderline Personality Disorder (BPD) (topline readout 4Q2023-1Q2024) and Schizophrenia (SCZ)
- New Phase I/II personalized medicine trial for Kabuki syndrome (neurodevelopmental syndrome) to start in 2024

Iadademstat:

- Positive final data in PoC Phase II trial in elderly-unfit acute myeloid leukemia (Unfit AML) - ALICE trial
- Currently in a Phase Ib trial in R/R-AML (OD designation received) and in a collaborative Phase II basket trial with Fox Chase Cancer Center in neuroendocrine tumors
- New Phase Ib/II in 2024 in small cell lung cancer (SCLC) with registrational potential

A Proof Of Concept in AML (ALICE)



CNS: **vafidemstat (ORY-2001)** - CNS optimized LSD1 inhibitor

Indication	Borderline Personality Disorder	Schizophrenia Negative Symptoms & Cognition	Kabuki Syndrome
Study	PORTICO	EVOLUTION	HOPE
Phase	Phase IIb	Phase IIb	Phase Ib/II
Status	Recruitment completed	Recruiting	IND in preparation
Anticipated Milestones	Front line data 4Q23-1Q24	Study updates 2024	IND 2024

Oncology: **iadademstat (ORY-1001)** - Selective LSD1 inhibitor

Indication	AML 1L Elderly/Unfit	AML R/R-Fit3mut+	NETs R/R	ED-SCLC 1L
Study	ALICE (Combo w azacitidine)	FRIDA (Combo w gilteritinib)	NET (Combo w paclitaxel)	STELLAR (Combo w ICI)
Phase	Phase IIa	Phase Ib	Phase II	Phase Ib/II
Status	Completed	Recruiting	Recruiting (Collaborative Study w FCCC)	IND in preparation
Anticipated Milestones	Final positive results presented at ASH2022	ASCO-2024, ASH-2024	Study updates 2H24	IND 2024

Note: Finalized clinical trials for iadademstat and vafidemstat are not shown. See www.oryzon.com for more details

¹ as of 2023.09.13 / ² as of 2023.06.30

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Management

Carlos Buesa Arjol, PhD
Chief Executive Officer



Douglas Faller MD, PhD
Global Chief Medical Officer



Michael Ropacki, MD, PhD
Chief Medical Officer CNS



Ana Limón, PhD
Sr. VP Clinical Dev & Medical Affairs



Jordi Xaus, PhD
Chief Scientific Officer



Emili Torrell
Chief Business Officer



Sonia Gutierrez, MSci
Director of Clinical Operations



Neus Virgili, EPA
Chief IP Officer



Enric Rello, PhD
Chief Financial Officer



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Market Opportunity in CNS Vafidemstat

Borderline personality disorder

Ongoing Phase IIb –
Top line data 4Q23-1Q24

9M patients in US & EU - No approved drugs to date. Low competition: only 2 Phase II trials ongoing. Patients are given off-label antipsychotics



Estimated market value ~\$2.5B, expected vafidemstat peak sales ~\$3B



2 main types of symptoms: Unstable-extreme interpersonal relationships and aggression & self-aggression



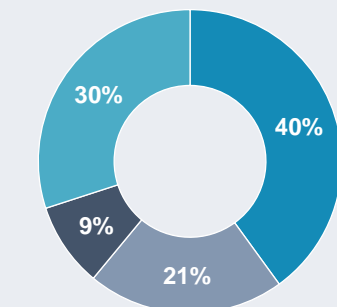
Vafidemstat improves symptoms in BPD patients (REIMAGINE Phase IIa trial) and pre-clinical models



Kabuki syndrome

New Phase Ib/II
(expected start in 2024)

10,000 patients with KS WW with Access to advanced health care



■ KS-I 12 -25 yrs old
■ KS-I 5 -12 yrs old
■ KS-I 2-5 yrs old
■ KS-II

Estimated Market Value ~0.7 Billion

Kabuki syndrome is one of the genetically driven pathologies where LSD1i compensates functional deficits. Opportunity for multiple label extensions

Market Opportunity in Oncology Iadademstat

Significant market opportunity in oncology, particularly where mutations in FLT3 (FLT3mut) increases risk of relapse

Flt3mut occur in 30-40% of AML patients (+6,000 patients per year in US).



US Market potential Flt3mut R/R AML ~\$0.8B by 2028



SCLC is 15% of lung cancer (~50,000 patients in US and EU5; ~100,000 in China)



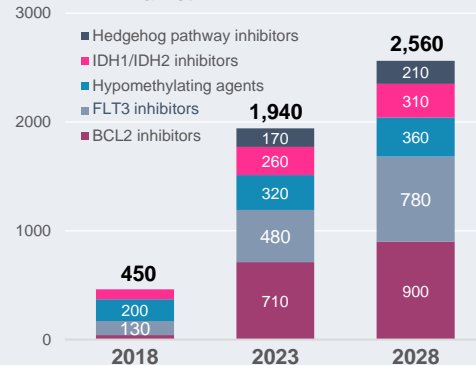
Global market potential first-line SCLC ~\$3.5B by 2027



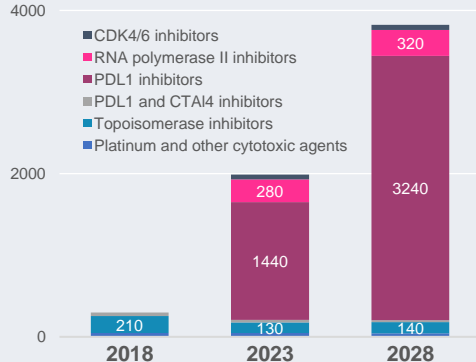
Additional opportunities in neuroendocrine and other solid tumors



AML market



SCLC market



Nature Reviews Drug Discovery 19, 233-234 (2020)

Nature Reviews Drug Discovery 19, 507-508 (2020)

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