## ORYZON

2Q-2022



Ticker ORY.MC / ORY:SM Exchange Madrid Stock Exchange Stock Price<sup>1</sup> €2.85 Market Cap<sup>2</sup> €135-215M Cash<sup>3</sup> €32.5M Cash Runway Q1:2023 **Outstanding Shares** 53.06M 52-Week Trading Range (€) 2.63-4.34 Avg. Daily Volume (30-day) 206,588 Headquarters Barcelona, Spain Fiscal Year End December 31st

### Analyst Research Coverage

**Jefferies** Lucy Codrington **Roth Capital** Jonathan Aschoff Ladenburg Thalmann Ahu Demir Kempen René Woulters **Rx Securities** Joseph Hedden Edison Jonas Peciulis **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, Mount Sinai, MGH, Fox-Chase) to optimize development efficiency.

Our expanding Phase II for both molecules builds on a clear demonstration of safety. Over 400 patients treated (300+ 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) and Schizophrenia (SCZ)
- Near-term 3<sup>rd</sup> Phase II personalized medicine trial with registrational potential for Kabuki (neurodevelopmental syndrome)

### ladademstat:

- Finalizing PoC Phase II trial in elderly-unfit acute myeloid leukemia (Unfit AML) - ALICE trial
- Phase Ib and Ib/II studies in 2022 in small cell lung cancer (SCLC) with registrational potential and in R/R-AML (OD designation received)
- Planned Phase II basket trial for treatment of neuroendocrine tumors.

### A Proof Of Concept in AML (ALICE)

ORR CR/CRI





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

Indication	Borderline Personality Disorder
Study	PORTICO
Phase	Phase IIb
Status	Recruiting
Anticipated Milestones	Interim analysis 4Q22

Schizophrenia Negative Symptoms & Cognition
EVOLUTION
Phase IIb
Recruiting
Study updates 2022

Kabuki Syndrome
HOPE
Phase lb/ll
In preparation
IND 1H22/FPI 1H22

	SetD1A Compass related SCZ
Ne	ew Study
Ph	ase lb/II
Un	der study

Aggression in AD
New Study Continuation of REIMAGINE-AD
Under study

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

	Oncology. Idadacinistat
Indication	<b>AML</b> 1L Elderly/Unfit
Study	ALICE (Combo w azacitidine)
Phase	Phase IIa
Status	Ongoing – fully enrolled
Anticipated Milestones	2H22 EHA/ASH Final Data

<b>AML</b> R/R-Flt3mut+
FRIDA (Combo w gilteritinib)
Phase Ib
IND approved
IND 1H22/FPI 1H22

ED-SCLC iL
STELLAR (Combo w ICI)
Phase lb/ll
In preparation
IND 1H22/FPI 2H22

NETs
R/R
NET Basket
(Combo w other agents)
(combo w other agente)
Phase Ib/II
Phase Ib/II In preparation

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

# ORYZON

### Management

Carlos Buesa Arjol, PhD Chief Executive Officer



Saikat Nandi, PhD Global Chief Business 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



Enric Rello, PhD Chief Financial Officer



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### Market Opportunity in CNS Vafidemstat

### Borderline personality disorder

Ongoing Phase IIb

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



Estimated market value ~\$2.5B, expected to grow to \$3B by 2027



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



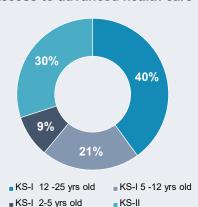
Vafidemstat improves symptoms in pre-clinical models



### Kabuki syndrome

Phase II with registrational potential (to start in 2022)





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

Estimated Market Value ~0.7 Billion

### Market Opportunity in Oncology ladademstat

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



Nature Reviews Drug Discovery 19, 233-234 (2020) Nature Reviews Drug Discovery 19, 507-508 (2020)



### **General Information**

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