EDISON

Oryzon Genomics

Foray into sickle cell disease

Regulatory update

Healthcare

26 August 2025

Oryzon Genomics has announced that the European Medicines Agency (EMA) has provided regulatory clearance for a Phase Ib trial of iadademstat in sickle cell disease. The Phase Ib study (named RESTORE) is expected to enrol 40 adult patients with the condition, and will be based across multiple sites in Spain. The trial is primarily designed to assess the safety and tolerability of the drug candidate, as well as to determine the recommended Phase II dose, with secondary objectives focused on measurements of foetal haemoglobin. While we acknowledge that this is an early-stage programme, we believe it represents a key milestone for Oryzon, since it will be the first investigation of iadademstat in a non-malignant haematological indication.

Year end	Revenue (€m)	PBT (€m)	EPS (€)	DPS (€)	P/E (x)	Yield (%)
12/23	14.2	(6.1)	(0.06)	0.00	N/A	N/A
12/24	7.4	(5.6)	(0.06)	0.00	N/A	N/A
12/25e	8.9	(3.9)	(0.01)	0.00	N/A	N/A
12/26e	48.3	35.9	0.48	0.00	5.8	N/A

Note: PBT and EPS are normalised, excluding intangibles, exceptional items and share-based payments.

According to the announcement, Oryzon has confirmed that its clinical trial application has been accepted by the EMA, enabling the company to conduct its Phase Ib trial in sickle cell disease. This news swiftly follows Oryzon's Q225 results, where the company first communicated its plans to explore the potential expanded application of iadademstat in non-malignant haematological indications, based on encouraging preclinical research and the role of LSD1 (the target of iadademstat) in sickle cell disease. It was noted that an additional indication that may be explored is essential thrombocythemia.

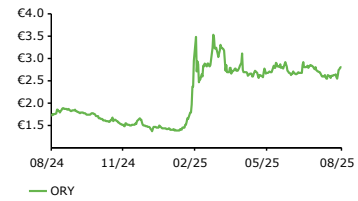
Sickle cell disease is an inherited blood disorder characterised by a mutation in the β-globin gene, leading to the production of haemoglobin S, as opposed to regular haemoglobin A. In low oxygen conditions, haemoglobin S can polymerise, giving rise to the sickle shape in red blood cells, making them rigid and fragile, ultimately leading to microvascular occlusion, haemolysis and chronic inflammation. This has the potential to lead to acute and progressive organ damage, reduced quality of life and premature mortality. According to Fortune Business Insights, the treatment market size is expected to reach $9.8bn by 2030, with the US dominating the market with a share of c 64% (in 2022), as it is the most common inherited blood disorder in the region. The field has evolved significantly in recent years with the approval of gene therapies for the condition. However, given the large price tags of these gene therapies, the opportunity remains for effective treatments that may come in at a more accessible price point, in our view.

Iadademstat is also being actively investigated in a multitude of oncology trials. The lead programme is FRIDA, evaluating iadademstat in combination with gilteritinib in patients with relapsed/refractory acute myeloid leukaemia harbouring the FLT3 mutation. The next update is due to be presented at the American Society of Hematology meeting in December 2025, potentially representing an upcoming catalyst for Oryzon.

For a more detailed overview of Oryzon's current clinical activities, we direct readers to our prior update note.

Price	**€2.82**
Market cap	**€221m**
Pro forma net cash/(debt) at 30 June 2025 (including the €13.3m grant income received in July 2025)	€26.3m
Shares in issue	78.5m
Free float	82.0%
Code	ORY
Primary exchange	MADRID
Secondary exchange	N/A

Share price performance



Business description

Spanish biotech Oryzon Genomics is focused on epigenetics. Iadademstat is being explored for acute leukaemias, small-cell lung cancer and additional indications. Central nervous system asset vafidemstat has completed several Phase IIa trials and a Phase IIb trial in borderline personality disorder (Phase III clinical trial protocol submitted to the FDA). It is also currently involved in a Phase IIb trial for schizophrenia, and management is preparing for an additional Phase II trial in autism spectrum disorder.

Analysts

Jyoti Prakash, CFA +44 (0)20 3077 5700
Arron Aatkar, PhD +44 (0)20 3077 5700

healthcare@edisongroup.com
Edison profile page

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London | New York | Frankfurt
20 Red Lion Street
London, WC1R 4PS
United Kingdom