EDISON

Oryzon Genomics

Clinical update

New clinical candidate expands CNS portfolio

Pharma and biotech

Oryzon Genomics has nominated a new central nervous system (CNS) clinical development candidate, ORY-4001, for the treatment of rare CNS disorders including Charcot-Marie-Tooth (CMT) disease and amyotrophic lateral sclerosis (ALS). The decision follows positive data in which ORY-4001 demonstrated both an encouraging selectivity and safety profile as well as efficacy signals, triggering a strong anti-inflammatory response in preclinical in vivo models; the compound also showed positive responses in a validated CMT1A peripheral neuropathy in vivo model, which reliably recapitulates many of the symptoms of this condition in humans. In July 2022, Oryzon collaborated with the CMT Research Foundation, which helped fund the preclinical studies for ORY-4001 in CMT and, in our view, highlights the positive impact Oryzon's partnerships can have on expediting development. The company now intends to start investigational new drug enabling studies as it progresses ORY-4001 towards clinical trials, the initiation of which would represent a future catalyst for investor attention.

Year end	Revenue (€m)	PBT* (€m)	EPS* (€)	DPS (€)	P/E (x)	Yield (%)
12/21	10.6	(7.2)	(0.09)	0.0	N/A	N/A
12/22	15.7	(6.6)	(0.07)	0.0	N/A	N/A
12/23e	17.3	(4.2)	(0.03)	0.0	N/A	N/A
12/24e	19.0	(10.0)	(0.14)	0.0	N/A	N/A

Note: *PBT and EPS are normalised, excluding amortisation of acquired intangibles, other income and exceptional items.

CMT is a debilitating, progressive and chronic orphan disease that damages the peripheral nerves in the brain, leading to muscular degeneration and potentially severe mobility issues. CMT affects around one in 2,500 people, which includes approximately 150,000 patients in the United States and around three million people globally. With no therapies approved for the treatment of CMT to date, the disease represents a significant area of unmet need. ALS is another rare and life-threatening CNS indication with no approved treatments. Patients diagnosed with ALS are often met with extremely poor prognosis with median survival from disease onset to death ranging from 20 to 48 months.

While Oryzon's existing clinical candidates, iadademstat and vafidemstat, target the epigenetic modulator lysine-specific demethylase 1, ORY-4001 is a histone deacetylase 6 inhibitor and represents the company's expansion into new epigenetic targets. The positive preclinical results, to date, provide encouraging signs for ORY-4001 and, should the drug progress into clinical studies, it would become Oryzon's second epigenetic programme in CNS indications.

Additionally, investigations into orphan diseases such as CMT and ALS may provide future benefits for the company such as market exclusivity on regulatory approval (if received), exemption of FDA application fees and tax credits for qualified clinical trials.

14 March 2023

Price	**€2.11**
Market cap	**€119m**
Net cash (€m) at end-December 2022	4.0
Shares in issue	56.3m
Free float	80%
Code	ORY
Primary exchange	Madrid Stock Exchange
Secondary exchange	N/A

Share price performance

Business description

Oryzon Genomics is a Spanish biotech focused on epigenetics. Iadademstat is being explored for acute leukaemias, small-cell lung cancer and neuroendocrine tumours. Vafidemstat, its central nervous system (CNS) asset, has completed several Phase IIa trials and a Phase IIb trial in borderline personality disorder is now the lead study, but Oryzon is rapidly expanding its CNS R&D pipeline.

Analysts

Soo Romanoff +44 (0)20 3077 5700

Dr Adam McCarter +44 (0)20 3077 5700

healthcare@edisongroup.com

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