ROTH Capital Partners

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COMPANY NOTE | EQUITY RESEARCH | October 26, 2020

Healthcare: Biotechnology

Oryzon Genomics SA | ORY.SM - €3.00 - MADRID | *Buy*

Company Update

Estimates Changed

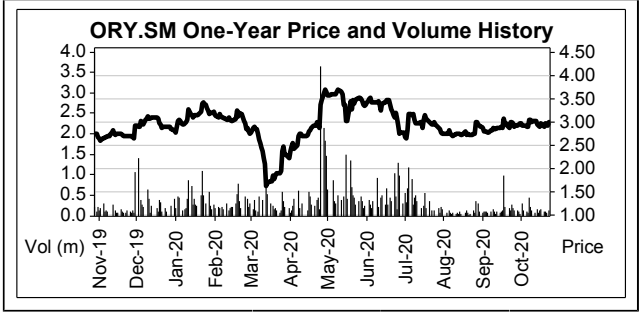
Stock Data	
52-Week Low - High	€1.48 - €3.90
Shares Out. (mil)	53.06
Mkt. Cap.(mil)	€158.92
3-Mo. Avg. Vol.	133,099
12-Mo.Price Target	€15.00
Cash (mil)	$52.2
Tot. Debt (mil)	$13.2

EPS $

Yr Dec	—2019—	—2020E—		—2021E—	
		Curr	Prev	Curr	Prev
1Q	(0.04)A	(0.03)A	(0.03)A	-	-
2Q	(0.02)A	0.00A	0.00A	-	-
3Q	(0.02)A	(0.02)A	(0.07)E	-	-
4Q	(0.02)A	(0.06)E	(0.08)E	-	-
YEAR	(0.10)A	(0.12)E	(0.19)E	(0.35)E	(0.38)E
P/E	NM	NM	NM	NM	NM

Revenue ($ millions)

Yr Dec	—2019—	—2020E—	—2021E—
		Curr	Curr
1Q	0.0A	0.0A	0.0E
2Q	0.0A	0.0A	0.0E
3Q	0.0A	0.0A	0.0E
4Q	0.0A	0.0E	0.0E
YEAR	0.0A	0.0E	0.0E



ORY.SM 3Q20: Cash Runway Into 1H23, Clinical Programs on Track

ORY.SM released 3Q20 results, showing a $52.2 million cash balance that can fund operations into 1H23, and also reviewed its current clinical programs.

- **Iadademstat.** Over 3Q20 at the ESMO conference, ORY.SM released positive Phase 2 trial CLEPSIDRA (n=14; 10 evaluable for efficacy) results in second-line SCLC. The trial evaluated iadademstat plus SOC carboplatin-etoposide therapy. ORR was 40% (4/10; all PRs) and the clinical benefit rate was 60%. The ORR compares favorably with response rates for other drugs approved for second-line SCLC such as topotecan (15-24%) and lurbinectedin (35%), or in third-line such as pembrolizumab (19%). The observed clinical beneft also underscores the potential value of biomarkers in patient selection. The six-cycle triple therapy caused severe hematological toxicity, but additional cycles of single agent iadademstat did not, which inclined the investigators to conclude that these patients cannot tolerate triple therapy and that iadademstat alone should be further investigated in this disease setting. Despite more than 60 weeks of patient monitoring, iadademstat monotherapy did not produce any hematological, neuronal, renal or hepatic toxicity, but the drug still had therapeutic benefit and therefore it has potential in combination with non-hemotoxic drugs. After the close of 3Q20, ORY.SM published its Phase 1 AML data, essentially showing acceptable monotherapy safety and justifying combination therapy in the ongoing Phase 2 ALICE trial. A 28-day course of therapy consisted of dosing for five days of each week and tested doses ranging from five to 220µg/m2/d, with 60µg/m2/d selected for Phase 2 combination therapy with azacitidine. We look forward to updated Phase 2 results at ASH in 4Q20 and emphasize the need for tolerable and effective therapies to treat such a fragile patient population. The ALICE trial was last updated at EHA-2020, demonstrating strong evidence of clinical activity with a 77% ORR (10 of 13 evaluable patients, six of whom had a CR).
- **Vafidemstat.** ORY.SM is collaborating with La Paz University Hospital to evaluate vafidemstat in Phelan-McDermid Syndrome (PMS), which is believed to be one cause of autism spectrum disorder. Despite COVID-19 restrictions, the first patients have been monitored for functional impairment using a set of diverse validated scales. These activities will continue with more genetically characterized PMS patients and should conclude by 1Q21, with the aim being that this cognitive, behavioral and functional baseline assessment of PMS patients will inform a future vafidemstat trial. ORY.SM is also conducting a Phase 2 vadademstat trial (ESCAPE; n=20) in severe COVID-19 patients, which is open-label and randomized into two groups to assess the utility of vafidemstat in combination with SOC to prevent progression to ARDS.

VALUATION

Our 12-month price target of €15, is based on a DCF analysis using a 35% discount rate that is applied to all cash flows and the terminal value, which is based on a 5x multiple of our projected 2030 operating income of $889 million. We arrive at this valuation by only projecting future revenue from vafidemstat in AD and iadademstat in AML. We view our valuation to be conservative given that it excludes revenue from vafidemstat in ASD, BPD, and ADHD, and from iadademstat in SCLC. Commercial success outside of the two financially modeled indications would serve as upside to our valuation. We believe that ORY.SM has prudently selected areas of unmet need and therefore market demand.

Factors that could impede shares of ORY.SM from achieving our price target include vafidemstat and iadademstat failing to generate statistically significant Phase 3 results in AD and AML, respectively. Also, regulatory agencies could fail to approve these drugs even if both Phase 3 programs are statistical successes, due to the agency viewing the results as not clinically meaningful. Loss of key management personnel could also impede achieving our price target, as could smaller than projected commercial opportunity due to changes in market size, competitive landscape, and drug pricing and reimbursement.

RISKS

- Clinical risk. ORY.SM's clinical staged products could fail to deliver statistically significant results in late-stage clinical trials, substantially reducing the value of ORY.SM's product candidates and therefore our target price.
- Regulatory risk. Even if successful in the clinic, ORY.SM's products could fail to be approved by domestic and/or foreign regulatory bodies, which would reduce ORY.SM’s value and therefore our target price.
- Financing risk. ORY.SM will need additional capital to fund its operations, and such financing may not occur or it could be substantially dilutive to existing investors.
- Competitive risk. For any future approved ORY.SM products, they may not be well adopted in a competitive marketplace, which would adversely affect ORY.SM's value and therefore our target price.
- High stock price volatility. This issue is common among small-cap biotechnology companies with relatively low trading volumes.

COMPANY DESCRIPTION

Founded in 2000 in Barcelona, Spain, Oryzon (ISIN Code: ES0167733015) is a clinical stage biopharmaceutical company considered as a European champion in epigenetics. Oryzon has one of the strongest portfolios in the field. Oryzon’s LSD1 program has rendered clinical stage vafidemstat and iadademstat. In addition, Oryzon has ongoing programs for developing inhibitors against other epigenetic targets. Oryzon has a strong technological platform for biomarker identification and performs biomarker and target validation for a variety of malignant and neurodegenerative diseases. Oryzon has offices in Spain and the United States

Oryzon Genomics SA
Income Statement
Fiscal Year ends December
(in 000, except per share items)

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	2017A	2018A	1Q19	2Q19	3Q19	4Q19	2019A	1Q20A	2Q20A	3Q20A	4Q20E	2020E	2021E
Global iadademstat revenue													
Global vafidemstat revenue													
Collaboration revenue	20												
Total revenue	**20**												
Cost of revenue													
R&D	6,363	8,489	2,610	3,022	3,462	3,553	12,647	4,316	2,731	2,279	2,507	11,833	15,974
G&A	4,502	2,993	876	1,042	742	516	3,176	846	906	733	740	3,225	3,386
Total operating expenses	**10,865**	**11,482**	**3,486**	**4,064**	**4,204**	**4,069**	**15,823**	**5,162**	**3,637**	**3,012**	**3,247**	**15,058**	**19,360**
Operating income	**(10,845)**	**(11,482)**	**(3,486)**	**(4,064)**	**(4,204)**	**(4,069)**	**(15,823)**	**(5,162)**	**(3,637)**	**(3,012)**	**(3,247)**	**(15,058)**	**(19,360)**
Other income (net)	5,659	8,143	2,497	2,516	3,208	3,301	11,522	4,013	2,312	1,787		8,112	
Net income (pretax)	**(5,186)**	**(3,339)**	**(989)**	**(1,548)**	**(996)**	**(768)**	**(4,301)**	**(1,149)**	**(1,324)**	**(1,225)**	**(3,247)**	**(6,945)**	**(19,360)**
Net financial & tax	1,047	(1,991)	368	(924)	73	296	(187)	116	(1,102)	(155)		(1,141)	
Net income	**(6,233)**	**(1,348)**	**(1,357)**	**(624)**	**(1,069)**	**(1,064)**	**(4,114)**	**(1,265)**	**(222)**	**(1,070)**	**(3,247)**	**(5,804)**	**(19,360)**
EPS basic	**(0.20)**	**(0.04)**	**(0.04)**	**(0.02)**	**(0.02)**	**(0.02)**	**(0.10)**	**(0.03)**	**(0.00)**	**(0.02)**	**(0.06)**	**(0.12)**	**(0.35)**
EPS diluted	**(0.20)**	**(0.04)**	**(0.04)**	**(0.02)**	**(0.02)**	**(0.02)**	**(0.10)**	**(0.03)**	**(0.00)**	**(0.02)**	**(0.06)**	**(0.12)**	**(0.35)**
Basic shares outstanding	31,711	34,638	38,455	38,638	43,677	45,489	41,589	45,489	45,808	52,762	53,290	49,337	55,954
Diluted shares outstanding	31,711	34,638	38,455	38,638	43,677	45,489	41,565	45,489	45,808	52,762	53,290	49,337	55,954

Source: SEC filings, company press releases, and ROTH Capital Partners

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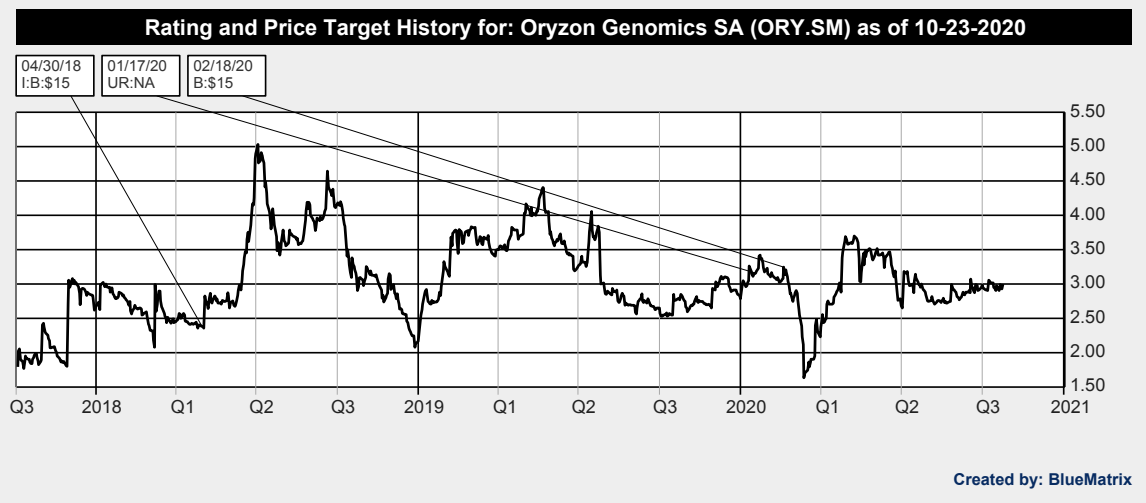
Disclosures:



Each box on the Rating and Price Target History chart above represents a date on which an analyst made a change to a rating or price target, except for the first box, which may only represent the first note written during the past three years. **Distribution Ratings/IB Services** shows the number of companies in each rating category from which Roth or an affiliate received compensation for investment banking services in the past 12 month.

Distribution of IB Services Firmwide

			IB Serv./Past 12 Mos. as of 10/26/20	
Rating	**Count**	**Percent**	**Count**	**Percent**
Buy [B]	**262**	**71.58**	**154**	**58.78**
Neutral [N]	**58**	**15.85**	**20**	**34.48**
Sell [S]	**3**	**0.82**	**2**	**66.67**
Under Review [UR]	**43**	**11.75**	**26**	**60.47**

Our rating system attempts to incorporate industry, company and/or overall market risk and volatility. Consequently, at any given point in time, our investment rating on a stock and its implied price movement may not correspond to the stated 12-month price target.

Ratings System Definitions - ROTH employs a rating system based on the following:

Buy: A rating, which at the time it is instituted and or reiterated, that indicates an expectation of a total return of at least 10% over the next 12 months.

Neutral: A rating, which at the time it is instituted and or reiterated, that indicates an expectation of a total return between negative 10% and 10% over the next 12 months.

Sell: A rating, which at the time it is instituted and or reiterated, that indicates an expectation that the price will depreciate by more than 10% over the next 12 months.

Under Review [UR]: A rating, which at the time it is instituted and or reiterated, indicates the temporary removal of the prior rating, price target and estimates for the security. Prior rating, price target and estimates should no longer be relied upon for UR-rated securities.

Not Covered [NC]: ROTH does not publish research or have an opinion about this security.

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