

Healthcare: Biotechnology

Retrophin, Inc. | RTRX - \$12.88 - NASDAQ | Buy

Company Update

Stock Data

52-Week Low - High	\$4.50 - \$24.25
Shares Out. (mil)	25.49
Mkt. Cap.(mil)	\$328.3
3-Mo. Avg. Vol.	504,890
12-Mo.Price Target	\$39.00
Cash (mil)	\$5.0
Tot. Debt (mil)	\$80.0

Tot. Debt (mil): Debt is pro forma on \$40 million term loan and \$40 million convertible note announced May 29, 2014

EPS \$

Yr Dec	—2013—	—2014E—	—2015E—
		Curr	Curr
1Q	(0.44)A	(3.03)A	-
2Q	(0.41)A	(0.59)E	-
3Q	(0.71)A	(0.33)E	-
4Q	(0.85)A	(0.27)E	-
YEAR	(2.38)A	(3.97)E	(1.03)E
P/E	NM	NM	NM

Quarterly EPS may not add to full year based on increases in share count and rounding

Revenue (\$ millions)

Yr Dec	—2013—	—2014E—	—2015E—
		Curr	Curr
1Q	0.0A	0.0A	-
2Q	0.0A	3.1E	-
3Q	0.0A	11.4E	-
4Q	0.0A	16.5E	-
YEAR	0.0A	31.0E	63.0E

RTRX One-Year Price and Volume History



RTRX: Further Thiola Details to Drive Revenue Growth; Reiterate Buy

Retrophin commented at length about their U.S. license agreement with Mission Pharmacal Company for marketing rights to Thiola (tiopronin) for the treatment of cystinuria. RTRX announced its plans regarding Thiola production and marketing. RTRX will likely give update on PKAN in June. Reiterate Buy rating and \$39 target.

Event

Retrophin announced a U.S. license agreement with Mission Pharmacal Company for marketing rights to Thiola (tiopronin) for the treatment of cystinuria, a rare genetic kidney disorder. RTRX announced its plans regarding Thiola production and marketing: RTRX will change Thiola formulation from 100mg to 250mg & 500mg to accommodate patients who need a daily 800-1000mg dose. RTRX plans on developing a long-acting Thiola formulation for a once-daily dose. RTRX will move Thiola into closed distribution to avoid drug shortage. The company will significantly increase Thiola price and will seek ex-US marketing opportunities. RTRX will give an update in June for the first patient dosed in the PKAN study. RTRX also plans on announcing an additional acquisition in the next 90 days.

Impact

This acquisition continues to bolster the company's focus on rare and orphan diseases and continues to build the revenue profile of the company, in our view. RTRX believes that increasing Thiola price will not be met with any issues from prescribing physicians, patients and payors and foresees peak sales of Thiola at \$100 million. The only other FDA-approved product for cystinuria is penicillamine, which has a much higher rate of adverse events and is priced at \$80K-\$140K per year vs. Thiola's current \$4000 per year. RTRX believes this will help support a significant price increase for Thiola. We believe the new Thiola formulations at 250mg and 500mg as well as the closed distribution plans will help strengthen Thiola positioning as a favorable drug for cystinuria treatment. Additionally, future development of a long-acting Thiola formulation will increase compliance and bolster its favorable position. RTRX will also give an update in June on the first PKAN patient treated under European compassionate use in May. We believe RTRX has entered a critical execution phase for its revenue generating product.

Action

We reiterate our Buy rating and \$39 target. We believe Retrophin continues to execute on its goals and position itself as a leading developer of drugs for catastrophic diseases.

Intraday price: \$15.40 at 9:52am ET, 5/30/2014

VALUATION

We reiterate our Buy rating and \$39 price target. Our valuation of Retrophin is based on our probability-weighted clinical net present value (NPV) valuation model. We believe this method is appropriate in capturing the value of the clinical stage pipeline. It allows for the flexing of assumptions based on key factors such as chance of success, peak sales estimates, and year of commercial launch. Factors which could impede shares of RTRX from reaching our price target include negative results from ongoing clinical trials as well as an inability to continually fund operations as a nonprofitable biotechnology company.

RISKS

- **Clinical and regulatory risk.** Drug development company's valuations are highly dependent on the outcomes of clinical trials. When considering Retrophin, the company targets indications with clear unmet medical needs. We believe that one of the continuing perceived risks is the regulatory path for RE-021 in FSGS and the planned primary endpoint of proteinuria. This appears to be a recurring concern for investors, in our belief, though Retrophin has received a letter from the FDA confirming its comfort level with having proteinuria as a primary endpoint. Additionally, while the PKAN program has a potential rapid development pathway, it is only about to enter the clinic, so therefore has increased risk from both a clinical and valuation standpoint.
- **Financing risk.** As with a majority of development-stage biotechnology companies, the ability to maintain sufficient funding is critical to the progress of pipeline candidates. Should Retrophin experience problems raising sufficient capital, its development programs' progress could be significantly impeded, leading to both delays in development timelines as well as potential negative effects on investor confidence. Each of these could have a negative impact on the share price. Retrophin has multiple clinical, in-licensing and potential acquisition plans in play right now. We believe that an important concern of investors is how the company is going to fund all of these initiatives.
- **Perceptions of "accelerated regulatory pathways".** Accelerated paths to potential FDA approval represent an attractive approach for companies that choose the right diseases. While clinical development plans may be clear, Retrophin must be vigilant in its discussions with the FDA to ensure agreement on all points associated with a potential approval path. Should the FDA require additional information or clinical trials, the perception of delays could negatively impact the stock.
- **Manufacturing and operational risks.** We expect Retrophin to continue to outsourcing its manufacturing. Dependence on a third party adds an additional layer of risk and any delays or disruptions in drug supply for trials or commercialization could significantly impact the stock. The stock is currently listed OTC, and we believe this limits the potential investor base and trading liquidity. We believe an uplisting to NASDAQ would help alleviate these issues.

COMPANY DESCRIPTION

Retrophin, Inc., a biopharmaceutical company, engages in the discovery, development, and commercialization of orphan drugs for the treatment of rare and life-threatening diseases. The company develops treatments for Focal Segmental Glomerulosclerosis (FSGS), Pantothenate Kinase-Associated Neurodegeneration (PKAN), Duchenne Muscular Dystrophy and other catastrophic diseases. Its products include RE-021, a small molecule angiotensin receptor blocker and selective endothelin receptor antagonist for the treatment of FSGS; and RE-024, a drug that restores the disruption in the biochemical Coenzyme A pathway caused by the non-functioning PANK2 encoded enzyme. The company also develops RE-001, a recombinant fusion protein that substitutes the dystrophin that is lacking in DMD patients; and RE-003, an investigational agent for spinal muscular atrophy. The company was founded in 2011 and is based in New York, New York.

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On September 28, 2010, ROTH changed its rating system in order to replace the Hold rating with Neutral.

On May 26, 2011, ROTH changed its rating system in order to incorporate coverage that is Under Review.



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Distribution of IB Services Firmwide

Rating	Count	Percent	IB Serv./Past 12 Mos. as of 05/30/14	
			Count	Percent
Buy [B]	184	81.06	101	54.89
Neutral [N]	25	11.01	10	40.00
Sell [S]	1	0.44	0	0
Under Review [UR]	16	7.05	10	62.50

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