

## Healthcare: Biotechnology

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

### Company Update

Estimates Changed

#### 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	Prev	Curr	Prev
1Q	(0.44)A	(3.03)A	(3.03)A	-	-
2Q	(0.41)A	(0.59)E	(0.46)E	-	-
3Q	(0.71)A	(0.33)E	(0.37)E	-	-
4Q	(0.85)A	(0.27)E	(0.30)E	-	-
YEAR	(2.38)A	(3.97)E	(3.91)E	(1.03)E	(1.40)E
P/E	NM	NM	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	Prev	Curr	Prev
1Q	0.0A	0.0A	0.0A	-	-
2Q	0.0A	3.1E	2.9E	-	-
3Q	0.0A	11.4E	6.3E	-	-
4Q	0.0A	16.5E	10.8E	-	-
YEAR	0.0A	31.0E	20.0E	63.0E	36.0E

#### RTRX One-Year Price and Volume History



## RTRX: Further Pipeline and Revenue Build Bolstering Company Strategy

Retrophin announced that it has entered into 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 and have significantly raised revenue guidance. The company also announced that they have signed a \$40 million term loan and have issued \$40 million of convertible notes. 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. Thiola was FDA approved in 1988. The company also announced that it has signed a \$40 million term loan and have issued \$40 million of convertible notes. The senior secured term loan matures in 2018 with an interest rate of LIBOR + 10%. The senior convertible notes are due in 2019 (4.5%).

### 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. Due to the new product, Retrophin has increased its revenue guidance for 2014 to \$30-35 million from \$20-22 million and 2015 guidance to \$60-70 from \$36-41 million. We believe RTRX has entered a critical execution phase. Ex-U.S. compassionate use of RE-024 in PKAN patients is ongoing and we believe we can receive answers relatively quickly. Retrophin will have real time access to data and may release raw data through 8-K filings as it becomes available. RTRX expects to file an IND within 2 months, followed shortly by a Phase I trial. RTRX is planning to initiate a patient registry for adolescents diagnosed with bilateral juvenile cataracts with Chenodal, which can be an early sign of CTX. RTRX is aware of off-label use of Vecamyl in rage disorders, autism, and Tourette syndrome and is exploring the possibility of initiating an IST to define effect the drug's effects in these indications. RTRX expects to complete enrollment in the potentially pivotal DUET study of sparsentan in FSGS in 4Q14 or 1Q15. Due to the high risk of FSGS patients losing their kidneys, RTRX believes a six figure price for sparsentan would be supported. RTRX expects to initiate a study of RE-034 in infantile spasms and membranous nephropathy in 3Q14.

### 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.

## 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.

## Retrophin

Mar. 11, 2011 inception through Dec. 31, 2011

(\$ in millions except per share data)

DGTE trading prior to Dec. 17, 2012 - merger

<b>Profit &amp; Loss</b>	<b>2011A</b>	<b>2012A</b>	<b>2013A</b>	<b>2014E</b>	<b>2015E</b>	<b>2016E</b>
Licensing	0.0	0.0	0.0	0.0	0.0	0.0
R&D collaborations	0.0	0.0	0.0	0.0	0.0	0.0
Product and Royalties	0.0	0.0	0.0	31.0	63.0	84.0
Other revenues	0.0	0.0	0.0	0.0	0.0	0.0
<b>Revenues</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>31.0</b>	<b>63.0</b>	<b>84.0</b>
CoGS	0.0	0.0	0.0	4.7	9.5	12.6
<b>Gross Profit</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>26.4</b>	<b>53.6</b>	<b>71.4</b>
<i>Gross margin</i>	<i>0%</i>	<i>0%</i>	<i>0%</i>	<i>85%</i>	<i>85%</i>	<i>85%</i>
G&A	3.3	30.3	16.9	41.4	43.4	45.6
R&D	0.0	0.0	7.1	27.8	29.2	33.6
Other op ex	0.0	0.0	0.0	0.0	0.0	0.0
<b>EBIT</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(24.0)</b>	<b>(42.9)</b>	<b>(19.1)</b>	<b>(7.8)</b>
<i>EBIT margin</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>
Non operating expenses	0.0	0.0	0.0	0.0	0.0	0.0
Net Interest Income/Other	(0.0)	0.0	(9.7)	(55.0)	(4.0)	(8.0)
Interest expense	0.0	0.1	0.0	1.6	1.6	1.6
<b>EBT</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(33.7)</b>	<b>(99.4)</b>	<b>(24.7)</b>	<b>(17.4)</b>
<i>EBT margin</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>
Provision for taxes	0.0	0.0	0.1	1.1	2.0	2.6
<b>Net Income</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(33.7)</b>	<b>(99.4)</b>	<b>(24.7)</b>	<b>(17.4)</b>
Participation of preferred stock	(0.0)	(0.0)	(0.0)	0.0	0.0	0.0
<b>Net Income to common</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(33.8)</b>	<b>(100.5)</b>	<b>(26.7)</b>	<b>(20.0)</b>
<i>net margin</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>	<i>nm</i>
NoSH	2.1	3.7	14.2	25.3	26.0	27.0
<b>EPS - basic</b>	<b>(1.59)</b>	<b>(8.29)</b>	<b>(2.38)</b>	<b>(3.97)</b>	<b>(1.03)</b>	<b>(0.74)</b>
<b>EPS - diluted</b>		<b>(8.29)</b>	<b>(2.38)</b>	<b>(3.97)</b>	<b>(1.03)</b>	<b>(0.74)</b>

Source: SEC Filings and ROTH Capital Partners estimates

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## Quarterly P&amp;L

	Q1'13A	Q2'13A	H1'13A	Q3'13A	9M'13A	Q4'13A	FY'13A	Q1'14A	Q2'14E	H1'14E	Q3'14E	9M'14E	Q4'14E	FY'14E
Licensing	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.00	0.00	0.00	0.0
R&D collaborations	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.00	0.00	0.00	0.0
Product and Royalties	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.03	3.10	3.13	11.36	14.49	16.51	31.0
Other revenues	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.00	0.00	0.00	0.0
<b>Revenues</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.0</b>	<b>0.03</b>	<b>3.10</b>	<b>3.13</b>	<b>11.36</b>	<b>14.49</b>	<b>16.51</b>	<b>31.0</b>
CoGS	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.43	0.43	1.80	2.23	2.42	4.7
<b>Gross Profit</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.00</b>	<b>0.0</b>	<b>0.03</b>	<b>2.67</b>	<b>2.70</b>	<b>9.56</b>	<b>12.26</b>	<b>14.09</b>	<b>26.4</b>
Gross margin	nm	nm	nm	nm	nm	nm	0%	97%	86%	86%	84%	85%	85%	85%
G&A	2.25	5.10	7.35	3.75	11.10	5.78	16.9	10.09	10.30	20.39	10.45	30.84	10.53	41.4
R&D	0.00	0.00	0.00	1.40	1.40	5.68	7.1	6.89	6.93	13.82	6.96	20.78	7.06	27.8
Other op ex	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.00	0.00	0.00	0.0
<b>EBITDA</b>	<b>(2.3)</b>	<b>(5.1)</b>	<b>(7.4)</b>	<b>(5.2)</b>	<b>(12.5)</b>	<b>(11.5)</b>	<b>(24.0)</b>	<b>(17.0)</b>	<b>(14.6)</b>	<b>(31.5)</b>	<b>(7.9)</b>	<b>(39.4)</b>	<b>(3.5)</b>	<b>(42.9)</b>
EBITDA margin							nm							nm
Non operating expenses	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.00	0.00	0.00	0.0
Net Interest Income/Other	(2.45)	0.06	(2.39)	(5.74)	(8.13)	(1.60)	(9.7)	(53.61)	(0.20)	(53.81)	(0.20)	(54.01)	(0.99)	(55.0)
Interest expense	0.04	0.00	0.05	0.00	0.05	0.00	0.0	0.00	0.00	0.00	0.00	0.00	1.55	1.6
<b>EBT</b>	<b>(4.7)</b>	<b>(5.0)</b>	<b>(9.8)</b>	<b>(10.9)</b>	<b>(20.7)</b>	<b>(13.1)</b>	<b>(33.7)</b>	<b>(70.6)</b>	<b>(14.8)</b>	<b>(85.3)</b>	<b>(8.1)</b>	<b>(93.4)</b>	<b>(6.0)</b>	<b>(99.4)</b>
EBT margin							nm							nm
Provision for taxes	0.00	0.00	0.00	0.00	0.00	0.08	0.1	0.07	0.15	0.22	0.30	0.52	0.58	1.1
Participation of preferred stock														
<b>Net Income to common</b>	<b>(4.7)</b>	<b>(5.0)</b>	<b>(9.8)</b>	<b>(10.9)</b>	<b>(20.7)</b>	<b>(13.1)</b>	<b>(33.8)</b>	<b>(70.6)</b>	<b>(14.9)</b>	<b>(85.5)</b>	<b>(8.4)</b>	<b>(93.9)</b>	<b>(6.6)</b>	<b>(100.5)</b>
net margin							nm							nm
NoSH	10.7	12.3	11.48	15.37	12.77	15.50	14.21	23.3	25.3	24.32	25.30	24.64	24.64	25.30
<b>EPS - diluted</b>	<b>(0.44)</b>	<b>(0.41)</b>	<b>(0.85)</b>	<b>(0.71)</b>	<b>(1.62)</b>	<b>(0.85)</b>	<b>(2.38)</b>	<b>(3.03)</b>	<b>(0.59)</b>	<b>(3.52)</b>	<b>(0.33)</b>	<b>(3.81)</b>	<b>(0.27)</b>	<b>(3.97)</b>

Source: SEC Filings and ROTH Capital Partners estimates

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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.



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

Rating	Count	Percent	IB Serv./Past 12 Mos. as of 05/29/14	
			Count	Percent
Buy [B]	185	81.50	102	55.14
Neutral [N]	24	10.57	9	37.50
Sell [S]	1	0.44	0	0
Under Review [UR]	16	7.05	10	62.50

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