

## Focus Pick

## Healthcare: Biotechnology

# Retrophin, Inc. | RTRX - \$15.36 - NASDAQ | Buy

### Focus List Update

Estimates Changed

#### Stock Data

52-Week Low - High	\$4.50 - \$24.25
Shares Out. (mil)	24.26
Mkt. Cap.(mil)	\$372.7
3-Mo. Avg. Vol.	514,243
12-Mo.Price Target	\$51.00
Cash (mil)	\$55.0
Tot. Debt (mil)	\$0.0

Cash (mil): Proforma cash on January 2014 equity financing

RTRX trading began on December 17, 2012

#### EPS \$

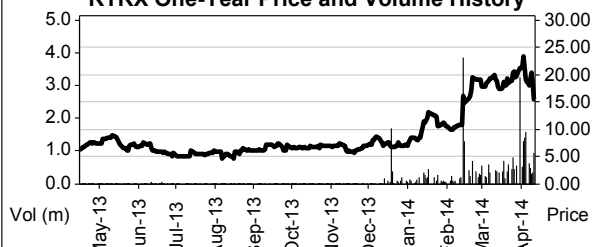
Yr Dec	—2013—	—2014E—		—2015E—	
		Curr	Prev	Curr	Prev
1Q	(0.44)A	(0.44)E	(0.24)E	-	-
2Q	(0.41)A	(0.38)E	(0.22)E	-	-
3Q	(0.71)A	(0.28)E	(0.20)E	-	-
4Q	(0.85)A	(0.17)E	(0.10)E	-	-
YEAR	(2.38)A	(1.27)E	(0.75)E	(0.95)E	(0.71)E
P/E	NM	NM	NM	NM	NM

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	0.8E	0.0E	-	-
2Q	0.0A	2.9E	1.1E	-	-
3Q	0.0A	6.3E	2.9E	-	-
4Q	0.0A	10.1E	7.1E	-	-
YEAR	0.0A	20.0E	11.0E	36.0E	20.0E

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



## RTRX: Revenue Guidance Upped; Pipeline Updated; Reiterate Focus Pick

Based on a favorable reimbursement landscape and recent significant price increases for Chenodal, Retrophin is raising its revenue guidance for 2014 and 2015. RE-024 has also experienced what we consider to be a minor setback as the company will file its own IND for the drug following the FDA disallowance of investigator-sponsored INDs to run the PKAN clinical program. We reiterate our Focus Pick and \$51 price target.

### Event

RTRX provided financial and pipeline updates today. The company is raising its revenue guidance for 2014 and 2015 from \$10-12 million to \$19-21 million for 2014 and from \$19-21 million to \$35-40 million for 2015. This new guidance is based both on the recent major price increase for Chenodal and a favorable reimbursement landscape in CTX patients, since Chenodal is the only approved drug to treat this disorder. The company provided an update for RE-024 for PKAN patients. The FDA disallowed the clinical program to move forward under investigator-sponsored IND filings and is requiring Retrophin to file a company sponsored IND. The company projects this IND filing in the May/June 2014 timeframe, with the Phase I initiation slated for June/July 2014.

### Impact

We are impressed with the increased revenue guidance and continued positive reimbursement landscape in CTX patients for the drug. Driving the increased guidance is also the significant recent price increase of Chenodal from \$9,640 to \$47,300 for 100 250 mg tablets as we believe the drug was significantly underpriced. Recall that Chenodal is the only drug approved for CTX. Regarding the RE-024 update, this, to us, is more of a perception negative for the company as they are delayed only ~6 months while the company IND is being prepared. Once the Phase I is initiated, we believe we could get answers quite quickly from this study as clinical effects in PKAN patients could potentially be seen quite rapidly. The ongoing DUET Phase II using sparsentan in FSGS patients is expected to complete enrollment late 2014/early 2015. RE-034 is expected to enter Phase III studies in 3Q14, including infantile spasms and membranous nephropathy.

### Action

We reiterate our Buy rating, Focus Pick and \$51 price target. We believe that this increase in price target is conservative as we only added Chenodal for CTX in our valuation and did not include any contribution from PBC or Vecamyl contribution. We believe this further highlights the upside potential from Retrophin's portfolio, in our belief.

Intraday: \$15.04, 11:51 ET, 4/14/14

## SUMMARY

### Chenodal

Retrophin previously acquired privately held Manchester Pharmaceuticals for \$62.5 million. RTRX paid \$29.5 million upfront and the remainder will be paid over the course of 2014. RTRX will also pay a royalty on drug sales, which we project could be in the 5-8% range. With the acquisition, the company brings in two FDA approved drugs, Chenodal and Vecamyl, which should generate revenue to the company immediately. Chenodal received FDA approval in 2009 for the treatment of gallstones, but its use has been exclusively in off label use for an orphan disease called CTX, and received Orphan Status in 2010. The company will file for approval for CTX in 2014 and also 1) quickly begin a Phase II/III in primary biliary cirrhosis (PBC) and 2) potentially explore the drug's potential in nonalcoholic steatohepatitis (NASH). We are impressed with the acquisition and believe it fits very nicely with the company's business model targeting orphan and catastrophic diseases

## VALUATION

We reiterate our Buy rating, Focus Pick and \$51 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	20.0	36.0	57.0
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>20.0</b>	<b>36.0</b>	<b>57.0</b>
CoGS	0.0	0.0	0.0	3.0	5.4	8.6
<b>Gross Profit</b>	<b>0.0</b>	<b>0.0</b>	<b>0.0</b>	<b>17.0</b>	<b>30.6</b>	<b>48.5</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	24.5	28.2	32.4
R&D	0.0	0.0	7.1	23.7	27.3	31.4
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>(31.2)</b>	<b>(24.9)</b>	<b>(15.3)</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)	(0.8)	0.1	0.1
Interest expense	0.0	0.1	0.0	0.0	0.0	0.0
<b>EBT</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(33.7)</b>	<b>(32.0)</b>	<b>(24.8)</b>	<b>(15.2)</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	0.0	0.0	0.0
<b>Net Income</b>	<b>(3.3)</b>	<b>(30.3)</b>	<b>(33.7)</b>	<b>(32.0)</b>	<b>(24.8)</b>	<b>(15.2)</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>(32.0)</b>	<b>(24.8)</b>	<b>(15.2)</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>(1.27)</b>	<b>(0.95)</b>	<b>(0.56)</b>
<b>EPS - diluted</b>		<b>(8.29)</b>	<b>(2.38)</b>	<b>(1.27)</b>	<b>(0.95)</b>	<b>(0.56)</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	Q1'14E	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	0.80	2.85	3.65	6.30	9.95	10.05	20.0
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>0.80</b>	<b>2.85</b>	<b>3.65</b>	<b>6.30</b>	<b>9.95</b>	<b>10.05</b>	<b>20.0</b>
CoGS	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.12	0.43	0.55	0.95	1.49	1.51	3.0
<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>0.68</b>	<b>2.42</b>	<b>3.10</b>	<b>5.36</b>	<b>8.46</b>	<b>8.54</b>	<b>17.0</b>
Gross margin	nm	nm	nm	nm	nm	nm	0%	85%	85%	85%	85%	85%	85%	85%
G&A	2.25	5.10	7.35	3.75	11.10	5.78	16.9	5.84	5.98	11.82	6.28	18.10	6.39	24.5
R&D	0.00	0.00	0.00	1.40	1.40	5.68	7.1	5.76	5.87	11.63	5.95	17.58	6.15	23.7
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>(10.9)</b>	<b>(9.4)</b>	<b>(20.3)</b>	<b>(6.9)</b>	<b>(27.2)</b>	<b>(4.0)</b>	<b>(31.2)</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)	(0.20)	(0.20)	(0.40)	(0.20)	(0.60)	(0.20)	(0.8)
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	0.00	0.0
<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>(11.1)</b>	<b>(9.6)</b>	<b>(20.7)</b>	<b>(7.1)</b>	<b>(27.8)</b>	<b>(4.2)</b>	<b>(32.0)</b>
EBT margin							nm							nm
Provision for taxes	0.00	0.00	0.00	0.00	0.00	0.08	0.1	0.00	0.00	0.00	0.00	0.00	0.00	0.0
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>(11.1)</b>	<b>(9.6)</b>	<b>(20.7)</b>	<b>(7.1)</b>	<b>(27.8)</b>	<b>(4.2)</b>	<b>(32.0)</b>
net margin							nm							nm
NoSH	10.7	12.3	11.48	15.37	12.77	15.50	14.21	25.3	25.3	25.30	25.30	25.30	25.30	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>(0.44)</b>	<b>(0.38)</b>	<b>(0.82)</b>	<b>(0.28)</b>	<b>(1.10)</b>	<b>(0.17)</b>	<b>(1.27)</b>

Source: SEC Filings and ROTH Capital Partners estimates

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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.

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Rating	Count	Percent	IB Serv./Past 12 Mos. as of 04/14/14	
			Count	Percent
Buy [B]	192	82.40	109	56.77
Neutral [N]	30	12.88	11	36.67
Sell [S]	1	0.43	0	0
Under Review [UR]	9	3.86	4	44.44

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