

## Healthcare: Biotechnology

# Retrophin, Inc. | RTRX - \$12.20 - NASDAQ | Buy

### Focus List Update

Target Price Changed

#### Stock Data

52-Week Low - High	\$4.50 - \$24.25
Shares Out. (mil)	24.79
Mkt. Cap.(mil)	\$302.4
3-Mo. Avg. Vol.	580,069
12-Mo.Price Target	\$39.00
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 \$

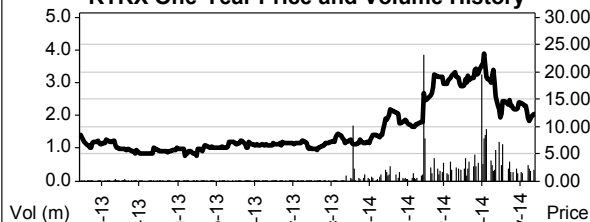
Yr Dec	—2013—	—2014E—	—2015E—
		Curr	Curr
1Q	(0.44)A	(0.44)E	-
2Q	(0.41)A	(0.38)E	-
3Q	(0.71)A	(0.28)E	-
4Q	(0.85)A	(0.17)E	-
YEAR	(2.38)A	(1.27)E	(0.95)E
P/E	NM	NM	NM

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

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



## RTRX: PKAN Moves Forward; Tweaking Projections; Off Focus List; Reiterate Buy

Retrophin announced that a European regulator has allowed for "named patient" compassionate use of RE-024 in up to 15 PKAN patients. We look forward to seeing the impacts from recent price increases for Chenodal and agreed upon guidance from the FDA for a controlled PKAN study in the U.S. We reiterate our Buy rating, though are removing from the Focus List and lowering our target to \$39 from \$51.

### Event

RTRX announced that a European regulator is allowing for "named patient" compassionate use for RE-024 in up to 15 PKAN patients. Enrolling these patients is an important next step for the drug and we believe critical data will be obtained regarding the drug's effectiveness. We also believe that data from the study could also help move discussions forward with the FDA regarding the IND and planned clinical study in the U.S. Recall the FDA disallowed the planned investigator sponsored studies for the clinical program but rather is requiring a controlled company sponsored study. The company projected an IND filing and study initiation for June/July 2014 following potential FDA sign off.

### Impact

Our investment case on Retrophin is wholly unchanged and believe the company is poised for significant growth based on both current and development stage products. The company has entered, in our belief, a critical execution phase and now look toward significant visibility, which could trigger a reassessment of our removal of the name from the Focus list. Recall the company recently increased its financial guidance (based primarily on Chenodal and its associated major price increase for CTX patients). We look toward visibility as to traction of the new drug pricing as well as its successful reimbursement. Regarding RE-024 for PKAN, the company has been in discussions with the FDA and has guided toward a summer Phase I initiation. We believe RE-024 can represent a major contributor to the RTRX investment case and believe investors are anxiously awaiting clarity on the clinical path forward for the drug in the U.S. 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, though are removing from the Focus List and lowering our target to \$39 from \$51. We believe Retrophin continues to execute on its goals and position itself as a leading developer of drugs for catastrophic diseases.

Intraday: \$12.60 at 11:52am ET, 5/13/14

Important Disclosures & Regulation AC Certification(s) are located on page 6 to 7 of this report.

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## VALUATION

We reiterate our Buy rating, though are removing from the Focus List and lowering our target to \$39 from \$51. The primary changes to our valuation model include:

- Change our projected launch timing from sparsentan in FSGS from 2016 to 2017
- Change our projected launch timing form RE-034 from 2016 to 2017 for infantile spasms and from 2017 to 2018 for membranous nephropathy.
- Change our projected launch timing for RE-024 for PKAN from 2016 to 2017.
- Adjusting our overall NPV profitability projections from 30% to 25% based on projected increases in infrastructure and related trial costs.

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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Rating	Count	Percent	IB Serv./Past 12 Mos. as of 05/13/14	
			Count	Percent
Buy [B]	179	80.27	100	55.87
Neutral [N]	23	10.31	8	34.78
Sell [S]	1	0.45	0	0
Under Review [UR]	19	8.52	12	63.16

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