ROTH Capital Partners

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Focus Pick

Healthcare: Biotechnology

Retrophin, Inc. | RTRX - $6.80 - OTC | *Buy*

Focus List Update

Estimates Changed, Target Price Changed

Stock Data	
52-Week Low - High	$2.55 - $9.99
Shares Out. (mil)	18.38
Mkt. Cap.(mil)	$125.0
3-Mo. Avg. Vol.	11,685
12-Mo.Price Target	$41.00
Cash (mil)	$16.4
Tot. Debt (mil)	$0.0

RTRX trading began on December 17, 2012

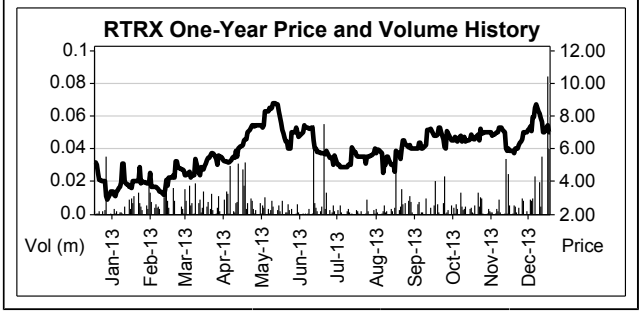
EPS $

Yr Dec	—2013E—	—2014E—		—2015E—	
		Curr	Prev	Curr	Prev
1Q	(0.44)A	(0.24)E	(0.29)E	-	-
2Q	(0.41)A	(0.26)E	(0.30)E	-	-
3Q	(0.71)A	(0.22)E	(0.33)E	-	-
4Q	(0.30)E	(0.20)E	(0.35)E	-	-
YEAR	(1.13)E	(0.91)E	(1.27)E	(1.01)E	(1.34)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	—2013E—	—2014E—		—2015E—	
		Curr	Prev	Curr	Prev
1Q	0.0A	0.0E	0.0E	-	-
2Q	0.0A	0.0E	0.0E	-	-
3Q	0.0A	2.2E	0.0E	-	-
4Q	0.0E	4.0E	0.0E	-	-
YEAR	0.0E	6.2E	0.0E	10.8E	0.0E



RTRX: Compelling 2014 Story; Adding to Focus List; Target to $41

RTRX announced new clinical candidate RE-034, a synthetic peptide of natural hormone ACTH. The company plans to file an IND and initiate a Phase I trial in 1H14, followed closely pivotal trials including infantile spasms and nephrotic syndrome. We are updating our valuation and adding RTRX to the Focus List; and raising our price target to $41 from $15 to accurately reflect (yet modestly, in our belief) the expanding pipeline opportunities.

Event

RTRX announced new clinical candidate RE-034, a synthetic peptide of naturally-occuring adrenocorticotropic hormone (ACTH). RTRX plans to file an IND and initiate a Phase I trial in 1H14, followed closely by pivotal Phase III studies in infantile spasms and nephrotic syndrome. Animal-derived ACTH (Acthar Gel) has shown efficacy in these conditions. RTRX believes the synthetic version will substantially reduce costs, improve consistency of the drug and eventually replace the animal-derived ACTH.

Impact

We see RE-034 as another pipeline boost for RTRX, bolstering both the CNS and nephrology focus. Infantile spasm (West Syndrome) is a catastrophic form of epilepsy which can lead to brain damage if not immediately suppressed. The high cost of animal-derived ACTH has limited patient access. RTRX has met with the FDA and agreed on many aspects of a short, small sample size Phase III study. Nephrotic syndrome is characterized by high amounts of protein in the urine, which constitutes a medical emergency. If not controlled, the condition will necessitate dialysis or transplant. Nephrotic syndrome is induced by various kidney disorders, including FSGS. Aggressive steroid treatment (standard of care) fails many, but a growing body of scientific literature indicates ACTH may drastically reduce proteinuria. RE-034 will be the first long-acting synthetic ACTH and will not include toxic preservative benzyl alcohol, which is standard in animal-derived formulations. RTRX recently in-licensed Syntocinon Nasal Spray from Novartis and acquired Kyalin Biosciences (and their intranasal synthetic oxytocin). These drugs will be developed for autism and schizophrenia. RTRX plans to imminently enroll the first patients in the Phase II study of RE-021 in FSGS (which the FDA has indicated could be a path to accelerated approval) and the Phase II emergency and compassionate use trial of RE-024 for PKAN. In 2Q14 RTRX plans to re-introduce Syntocinon for postpartum milk ejection, which should provide a near-term revenue stream.

Action

We are updating our valuation and adding RTRX to the Focus List; and raising our price target to $41 from $15 to accurately reflect (yet modestly, in our belief) the expanding pipeline opportunities.

Valuation Methodology – Conservative Additions with Meaningful Valuation Outcomes

We are adding shares of Retrophin to the Focus List and increasing our price target from $15 to $41 to more accurately reflect the company's added pipeline opportunities, as well as near term revenue opportunities.

Our original price target on shares of Retrophin was $15. 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. Additionally, while the current shares outstanding is ~18 million, we are assuming a ~7 million share increase as the company needs to raise funds and believe this impact to our NPV model fairly and modestly reflected the opportunities at the time.

This has since chance since the pipeline has greatly expanded by bringing near term revenue opportunities as well as further late stage products

Our prior valuation of Retrophin was based solely on the RE-021 opportunity in FSGS. We project a 55% chance of success, 2016 launch and $375 million in peak sales ($15.27 NPV per share). A level of conservatism in our valuation model comes from our assigned multiple and discount rate for which we apply the historical values for large pharma (17.0x P/E and 15% discount rate) rather than the sometimes inflated non-profitable biotech multiples in the 30-40x range.

Original Retrophin Clinical NPV Valuation Model

Drug name	Indication	Status	Launch	Success	Peak Sales (US$m)	Royalty	Profitability	NPV (US$)
RE-021	FSGS	Phase II	2016	55%	375	100%	30%	15.27
Unknown	Autism	Phase II	2018	0%	500	100%	30%	0.00
Unknown	Schizophrenia	Phase II	2018	0%	500	100%	30%	0.00
RE-024	PKAN	Preclinical	2016	0%	300	100%	30%	0.00
RE-001	DMD	Preclinical	2019	0%	800	100%	30%	0.00
RE-003	SMA	Preclinical	2020	0%	300	100%	30%	0.00
							Total	**15.27**

Source: ROTH Capital Partners estimates

Changes and Additions to NPV

A current overhang on the stock, in our belief, is its current trading on the OTC market. We believe the company is looking to uplist as soon as possible, which should further increase visibility and trading in the name above and beyond the expanding pipeline opportunities, we believe. The changes and additions to our clinical NPV are found in the table below. We believe, based on the market opportunities of each indication (highlighted by unmet medical need), that our changes our modes based both on our projected chances of success and peak sales estimates.

- Changed base year
- Added Syntocinon; 1) milk letdown launch in 2014 with 50% chance of success and $75 million peak sales, 2) schizophrenia with a 2018 launch, 5% chance of success and $500 million peak sales and 3) autism with a 2019 launch, 5% chance of success and $500 million peak sales.
- Added RE-034; 1) infantile spasm launch in 2016 with 30% chance of success and $350 million peak sales and 2) nephrotic syndrome launch in 2017 with 30% chance of success and $350 million peak sales.
- Added RE-024 for PKAN with a 2016 launch, 15% chance of success and $320 million peak sales.

We believe that our projected chances of success are especially conservative for Syntocinon for milk letdown and RE-034 (infantile spasms and nephrotic syndrome) since both drugs have already been validated and we believe the "heavy lifting" has already been done with regard to the regulatory path forward.

Updated Retrophin Clinical NPV Valuation Model

Drug name	Indication	Status	Launch	Success	Peak Sales (US$m)	Royalty	Profitability	NPV (US$)
Syntocinon	Milk letdown	Pre-launch	2014	50%	75	100%	30%	3.80
Syntocinon	Schizophrenia	Phase II	2018	5%	500	100%	30%	1.45
Syntocinon	Autism	Phase II	2019	5%	500	100%	30%	1.26
Sparsentan (RE-021)	FSGS	Phase II	2016	55%	375	100%	30%	15.82
RE-034	Infantile spasms	Phase II	2016	30%	350	100%	30%	8.05
RE-034	Nephrotic syndrome	Phase II	2017	30%	350	100%	30%	7.00
RE-024	PKAN	Phase I	2016	15%	320	100%	30%	3.68
							Total	**41.07**

Source: ROTH Capital Partners estimates

Valuation upside potential – Our valuation of Retrophin as mentioned, is now based on what we consider to be modest projections for the totality of the pipeline. We see upside potential on multiple fronts to our valuation, based on increasing the chance of success for a particular indication and increasing the peak sales numbers should any of the products gain more market traction than expected. As additional therapeutic indications are addressed, this could also have a beneficial impact on our valuation methodology. For example, RE-021 has the potential to address multiple other indications which we currently do not include in our valuation. Also, adding other future pipeline programs into our valuation mix should also positively impact our NPV. The company had indicated that they are looking to bring in a revenue producing drug within the next 12-months.

Valuation downside potential – As with the majority of companies in clinical development, there exists the risk of failed or inconclusive clinical trials, which could lead to downward pressure on the stock. We believe that Retrophin helps to mitigate this risk by evaluating therapeutic agents with potential in multiple indications. Cancer is an important example, where failure in one malignancy type and one particular line of treatment does not preclude success in other tumor types.

VALUATION

We reiterate our Buy rating, adding RTRX to the Focus list and raising our price target to $41 from $15. Our changes to our valuation are highlighted above. 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

($ in millions except per share data)

Mar. 11, 2011 inception through Dec. 31, 2011
DGTE trading prior to Dec. 17, 2012 - merger

Profit & Loss	**2011A**	**2012A**	**2013E**	**2014E**	**2015E**	**2016E**
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	6.2	10.8	17.6
Other revenues	0.0	0.0	0.0	0.0	0.0	0.0
Revenues	**0.0**	**0.0**	**0.0**	**6.2**	**10.8**	**17.6**
CoGS	0.0	0.0	0.0	0.9	1.6	2.6
Gross Profit	**0.0**	**0.0**	**0.0**	**5.3**	**9.2**	**15.0**
Gross margin	*0%*	*0%*	*0%*	*85%*	*85%*	*85%*
G&A	3.3	30.3	15.1	18.2	21.4	24.6
R&D	0.0	0.0	3.0	9.5	14.2	19.8
Other op ex	0.0	0.0	0.0	0.0	0.0	0.0
EBIT	**(3.3)**	**(30.3)**	**(18.1)**	**(22.3)**	**(26.4)**	**(29.5)**
EBIT margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*
Non operating expenses	0.0	0.0	0.0	0.0	0.0	0.0
Net Interest Income/Other	(0.0)	0.0	(10.0)	(0.8)	0.1	0.1
Interest expense	0.0	0.1	0.1	0.0	0.0	0.0
EBT	**(3.3)**	**(30.3)**	**(28.2)**	**(23.1)**	**(26.3)**	**(29.4)**
EBT margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*
Provision for taxes	0.0	0.0	0.0	0.0	0.0	0.0
Net Income	**(3.3)**	**(30.3)**	**(28.2)**	**(23.1)**	**(26.3)**	**(29.4)**
Participation of preferred stock	(0.0)	(0.0)	0.0	0.0	0.0	0.0
Net Income to common	**(3.3)**	**(30.3)**	**(28.2)**	**(23.1)**	**(26.3)**	**(29.4)**
net margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*
NoSH	2.1	3.7	25.0	25.3	26.0	27.0
EPS - basic	**(1.59)**	**(8.29)**	**(1.13)**	**(0.91)**	**(1.01)**	**(1.09)**
EPS - diluted		**(8.29)**	**(1.13)**	**(0.91)**	**(1.01)**	**(1.09)**

Source: SEC Filings and ROTH Capital Partners estimates

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Quarterly P&L

	Q1'13A	Q2'13A	H1'13A	Q3'13A	9M'13A	Q4'13E	FY'13E	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	0.00	0.00	0.00	2.20	2.20	4.00	6.2
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
Revenues	**0.00**	**0.00**	**0.00**	**0.00**	**0.00**	**0.00**	**0.0**	**0.00**	**0.00**	**0.00**	**2.20**	**2.20**	**4.00**	**6.2**
CoGS	0.00	0.00	0.00	0.00	0.00	0.00	0.0	0.00	0.00	0.00	0.33	0.33	0.60	0.9
Gross Profit	**0.00**	**0.00**	**0.00**	**0.00**	**0.00**	**0.00**	**0.0**	**0.00**	**0.00**	**0.00**	**1.87**	**1.87**	**3.40**	**5.3**
Gross margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*	*0%*	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*	*85%*
G&A	2.25	5.10	7.35	3.75	11.10	4.02	15.1	4.19	4.29	8.48	4.48	12.96	5.19	18.2
R&D	0.00	0.00	0.00	1.40	1.40	1.60	3.0	1.66	2.00	3.66	2.68	6.34	3.11	9.5
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
EBITDA	**(2.3)**	**(5.1)**	**(7.4)**	**(5.2)**	**(12.5)**	**(5.6)**	**(18.1)**	**(5.9)**	**(6.3)**	**(12.1)**	**(5.3)**	**(17.4)**	**(4.9)**	**(22.3)**
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)	(1.87)	(10.0)	(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	0.1	0.00	0.00	0.00	0.00	0.00	0.00	0.0
EBT	**(4.7)**	**(5.0)**	**(9.8)**	**(10.9)**	**(20.7)**	**(7.5)**	**(28.2)**	**(6.1)**	**(6.5)**	**(12.5)**	**(5.5)**	**(18.0)**	**(5.1)**	**(23.1)**
EBT margin							*nm*							*nm*
Provision for taxes	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
Participation of preferred stock														
Net income to common	**(4.7)**	**(5.0)**	**(9.8)**	**(10.9)**	**(20.7)**	**(7.5)**	**(28.2)**	**(6.1)**	**(6.5)**	**(12.5)**	**(5.5)**	**(18.0)**	**(5.1)**	**(23.1)**
net margin							*nm*							*nm*
NoSH	10.7	12.3	11.48	15.37	12.77	25.00	25.00	25.3	25.3	25.30	25.30	25.30	25.30	25.30
EPS - diluted	**(0.44)**	**(0.41)**	**(0.85)**	**(0.71)**	**(1.62)**	**(0.30)**	**(1.13)**	**(0.24)**	**(0.26)**	**(0.50)**	**(0.22)**	**(0.71)**	**(0.20)**	**(0.91)**

Source: SEC Filings and ROTH Capital Partners estimates

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ROTH and/or its employees, officers, directors and owners own options, rights or warrants to purchase shares of Retrophin, Inc. stock.

Within the last twelve months, ROTH has received compensation for investment banking services from Retrophin, Inc..

ROTH makes a market in shares of Retrophin, Inc. and Questcor Pharmaceuticals, Inc. and as such, buys and sells from customers on a principal basis.

Shares of Retrophin, Inc. may be subject to the Securities and Exchange Commission's Penny Stock Rules, which may set forth sales practice requirements for certain low-priced securities.

Shares of Retrophin, Inc. may not be eligible for sale in one or more states.

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

			IB Serv./Past 12 Mos. as of 12/18/13	
Rating	**Count**	**Percent**	**Count**	**Percent**
Buy [B]	**164**	**72.57**	**88**	**53.66**
Neutral [N]	**35**	**15.49**	**12**	**34.29**
Sell [S]	**2**	**0.88**	**0**	**0**
Under Review [UR]	**25**	**11.06**	**10**	**40.00**

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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