ROTH Capital Partners

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Healthcare: Biotechnology

Retrophin, Inc. | RTRX - $7.00 - OTC | *Buy*

Company Update

Stock Data	
52-Week Low - High	$2.50 - $9.99
Shares Out. (mil)	18.38
Mkt. Cap.(mil)	$128.7
3-Mo. Avg. Vol.	6,657
12-Mo.Price Target	$15.00
Cash (mil)	$16.4
Tot. Debt (mil)	$0.0

RTRX trading began on December 17, 2012

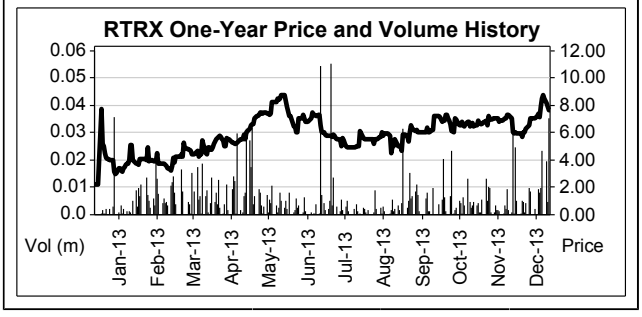
EPS $

Yr Dec	—2013E—	—2014E—	—2015E—
		Curr	Curr
1Q	(0.44)A	(0.29)E	-
2Q	(0.41)A	(0.30)E	-
3Q	(0.71)A	(0.33)E	-
4Q	(0.40)E	(0.35)E	-
YEAR	(1.69)E	(1.27)E	(1.34)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	—2013E—	—2014E—	—2015E—
		Curr	Curr
1Q	0.0A	0.0E	-
2Q	0.0A	0.0E	-
3Q	0.0A	0.0E	-
4Q	0.0E	0.0E	-
YEAR	0.0E	0.0E	0.0E



RTRX: In-licensing, Acquisition Build CNS Pipeline; Near-term Revenue is Bonus

RTRX announced in-licensing of Syntocinon Nasal Spray (synthetic oxytocin) from Novartis (NVS-NC). RTRX will reintroduce the drug for postpartum milk letdown (for which it was approved in 1960 but discontinued for commercial reasons) and will also develop it for schizophrenia and autism. Additionally, RTRX will co-sponsor a clinical trial of intranasal oxytocin for schizophrenia with UCSD and acquire Kyalin Biosciences (private), owner of an intranasal synthetic oxytocin. Reiterate Buy.

Event

RTRX announced the in-licensing of Syntocinon Nasal Spray (synthetic oxytocin) from Novartis for an upfront payment of $5 million plus 20% royalties and undisclosed milestones. Additionally, RTRX will co-sponsor a clinical trial of intranasal oxytocin for schizophrenia with UCSD and acquire Kyalin Biosciences (private), owner of an intranasal synthetic oxytocin.

Impact

Syntocinon was FDA approved for postpartum milk ejection in 1960. Though discontinued by Novartis for commercial reasons, 79% of physicians polled said they would use the drug if available again, as there is currently no FDA approved drug for milk letdown. RTRX plans to reintroduce Syntocinon for this purpose in 2Q14. With only 40-80 sales reps needed and with the former manufacturing infrastructure still in place, we see a nice, de-risked source of revenue here ($50-125 million opportunity depending on market share). RTRX plans to develop the drug for schizophrenia and autism based on 3 proof-of-concept studies showing significant benefit in symptom reduction in schizophrenia patients. The Kyalin acquisition brings the lead product, an intranasal carbetocin (synthetic oxytocin), and also the expertise of Kyalin founder Dr. Srinivas Rao to RTRX, which should nicely complement the Syntocinon in-licensing and lend strength to the CNS pipeline. RTRX also announced an agreement to co-sponsor a Phase II trial of intranasal oxytocin for schizophrenia in collaboration with UCSD. The study is expected to read-out in 3Q14 and will become part of RTRX's Syntocinon development program. RTRX plans to enroll the first patient in the Phase II study of RE-021 in FSGS this month. The FDA has indicated the trial could be a path to accelerated approval. In Jan '14, RTRX plans begin enrolling patients in their Phase I emergency and compassionate use trial of RE-024 in PKAN. RTRX also announced today that one major new pipeline program will be announced by end of 2013.

Action

We reiterate our Buy rating and $15 target. Based on our clinical NPV valuation method, we believe that RTRX share are undervalued based solely on the RE-021 opportunity for FSGS. The maturing pipeline provides significant upside.

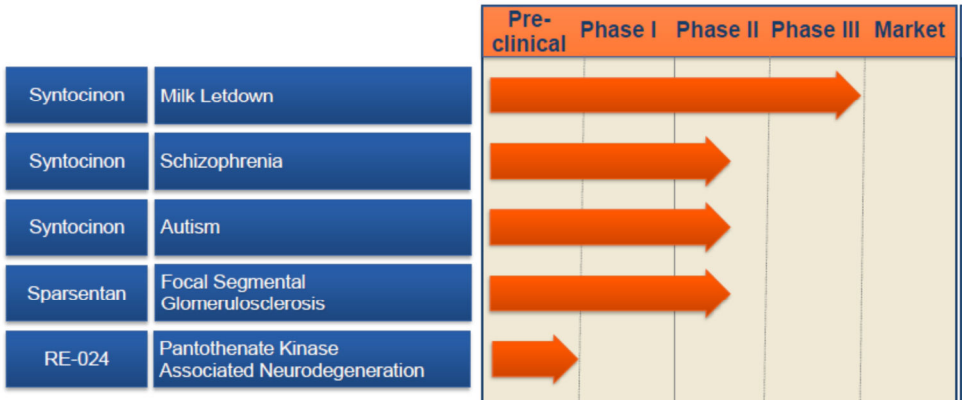
Bringing Products and Development Paths In House – Company is Busy

Below we summarize the updated pipeline for Retrophin and the new licensed product from Novartis, namely Syntocinon (intranasal oxytocin).

All Important Pipeline and Timelines

Before highlighting the upcoming catalysts and new product opportunities for Retrophin, the figure below contains the updated pipeline. Excluded from this slide are two coming additions, according to management; 1) the acquisition of a revenue generating product within the next 12 months and 2) one major new pipeline drug will be announced by year end 2013 (announced yesterday).

Retrophin Pipeline



Source: Retrophin – December 12, 2013 investor presentation

Upcoming catalysts

Syntocinon

- Submit "Re-activation" application to FDA by year end 2013
- 1Q14 – Feedback from FDA
- 2Q14 – Re-launch Syntocinon for milk let-down
- 2Q14 – Initiate Phase II autism study as well as Orphan CNS indication
- 3Q13 – Phase II schizophrenia results expected

Sparsentan

- December 2013 – Initiate FSGS potentially pivotal study

RE-024

- IND and first-patient-in for Phase I study – by year end 2013 or early 2015

Over the next 12 months Retrophin is also expected to inlicense one new revenue generating product and announce the inlicensing of one major pipeline drug by year end.

Syntocinon – Re-Launch Plans

As described above, Retrophin is in the process of resubmitting the "Re-activation application" to the FDA for Syntocinon and the relaunch of the drug without new clinical studies. Supporting this is that the company will use Novartis' existing manufacturing for the packaged drug and a new U.S. distribution network will be established. The company will employ ~40-80 reps including medical science liaisons for medical education. The drug is expected to be relaunched in 2Q14.

Syntocinon – Plans in Schizophrenia

Three randomized, double-blind, placebo-controlled, independent proof-of-concept studies were conducted in schizophrenia patients, each of which showed independent and consistent activity of the oxytocin approach. The validated primary endpoint in these studies and looking forward is the mean change in PANSS total, which has been the basis for approval for multiple antipsychotic medications.

Looking forward, there is currently an ongoing study with a collaborator at UCSD and NIMH to measure the antipsychotic effects of intranasal oxytocin in patients with schizophrenia. ~143 patients will be enrolled into this randomized, multi-dose, placebo-controlled, multicenter study testing 42 IU or 84 IU intranasal oxytocin as an adjunctive therapy to currently prescribed antipsychotics. The primary endpoint of the study is PANSS-Total. Secondary endpoints include: Global Assessment of Functioning (GAF), Clinical Global Impression-Severity of Illness (CGI-S) and Clinical Global Impression-Global Improvement (CGI-I). Results from this study are expected in 3Q14.

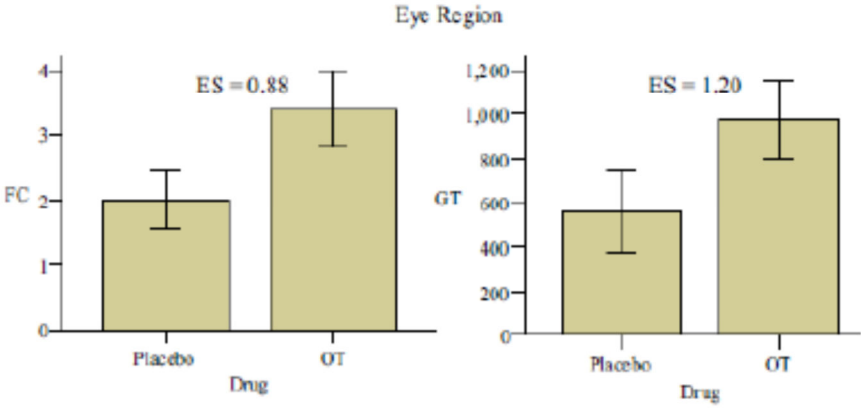
Following potential postive results from this study, Retrophin plans to conduct two Phase III studies. The first Phase III "A" study is targeted to run between January 2015 and June 2016, enroll ~500 patients for 8 weeks of therapy as an add on to existing therapy with a primary endpoint of PANSS-Total. The second Phase III "B" will look to be conducted between March 2015 and August 2016 and be designed essentially identically to study "A".

Syntocinon – Plans in Autism (including past "Social Behavior" clinical studies)

In order to put the plans for autism into perspective, there has been preclinical data generated from over 400 studies that support the beneficial role of oxytocin in social and reproductive behaviors. Importantly, over 20 studies have been performed in healthy volunteers with acute intranasal oxytocin with demonstrated effects in empathy, eye gaze, trust and facial recognition. We present two of those studies below.

One study performed was in "eye gaze". 52 healthy young adult males were treated with oxytocin nasal spray (24 IU) or placebo. The subjects' faces were presented on a computer screen and eye tracking was monitored to quantify "time spent" by looking at the eyes, nose/mouth and forehead/cheeks. As shown in the figure below, subjects receiving oxytocin gazed longer and fixated more on eyes compared to other regions.

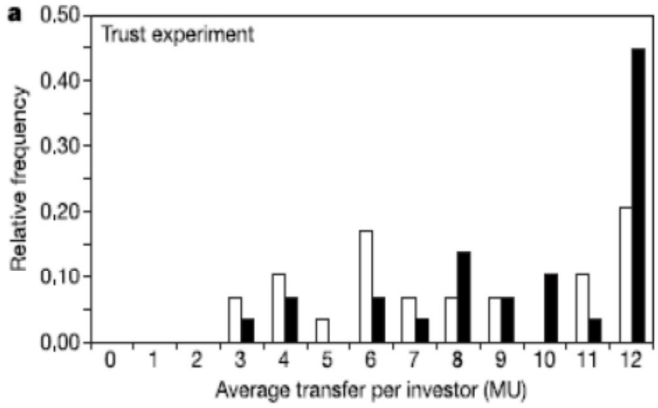
Eye Gaze Study in Young Healthy Adult Males



Source: Guastella et al., 2008, Biol. Psychiat. 63:3-5

Another example was a study of "trust" in which subjects (n=58) were tested for the relative frequency of an investors' average transfers to a trustee while being administered oxytocin (filled bars) or placebo (open bars). It was shown that subjects on oxytocin transfer significantly higher amounts. A subsequent experiment also confirmed that the higher rate of transfer was due to trust and not due to higher risk tolerance.

Social Behavior Experiment – Trust



Source: Kosfeld et al. 2005

Looking forward to the company's clinical plans for autism and another andundisclosed CNS indication they are planning the following.

- Phase II/III for an orphan CNS indication which is expected to run between June 2014 and June 2015 and enroll ~24 patients. Patients will be treated for 8 weeks and will be an add on to an existing therapy. The primary endpoint of the study will be weight gain.
- Phase II study will be conducted between January 2015 and January 2016 and will enroll ~150 patients with 8 weeks of treatment as an add on to existing therapy. The primary endpoint of the study will be ABC-SW vs. RMET.

VALUATION

We reiterate our Buy rating and $15 price target on Retrophin. 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

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	0.0	0.0	4.5
Other revenues	0.0	0.0	0.0	0.0	0.0	0.0
Revenues	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**4.5**
CoGS	0.0	0.0	0.0	0.0	0.0	0.7
Gross Profit	**0.0**	**0.0**	**0.0**	**0.0**	**0.0**	**3.8**
Gross margin	*0%*	*0%*	*0%*	*0%*	*0%*	*85%*
G&A	3.3	30.3	15.1	17.4	18.3	19.2
R&D	0.0	0.0	1.9	6.0	8.7	12.1
Other op ex	0.0	0.0	0.0	0.0	0.0	0.0
EBIT	**(3.3)**	**(30.3)**	**(17.0)**	**(23.4)**	**(26.9)**	**(27.5)**
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)**	**(27.1)**	**(24.2)**	**(26.8)**	**(27.4)**
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)**	**(27.1)**	**(24.2)**	**(26.8)**	**(27.4)**
Participation of preferred stock	(0.0)	(0.0)	0.0	0.0	0.0	0.0
Net Income to common	**(3.3)**	**(30.3)**	**(27.1)**	**(24.2)**	**(26.8)**	**(27.4)**
net margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*
NoSH	2.1	3.7	16.0	19.0	20.0	25.0
EPS - basic	**(1.59)**	**(8.29)**	**(1.69)**	**(1.27)**	**(1.34)**	**(1.10)**
EPS - diluted		**(8.29)**	**(1.69)**	**(1.27)**	**(1.34)**	**(1.10)**

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	0.00	0.00	0.00	0.0
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
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**
CoGS	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
Gross Profit	**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**
Gross margin	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*	*0%*	*nm*	*nm*	*nm*	*nm*	*nm*	*nm*	*0%*
G&A	2.25	5.10	7.35	3.75	11.10	4.02	15.1	4.12	4.29	8.41	4.48	12.89	4.51	17.4
R&D	0.00	0.00	0.00	1.40	1.40	0.50	1.9	1.21	1.29	2.50	1.62	4.12	1.87	6.0
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)**	**(4.5)**	**(17.0)**	**(5.3)**	**(5.6)**	**(10.9)**	**(6.1)**	**(17.0)**	**(6.4)**	**(23.4)**
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)**	**(6.4)**	**(27.1)**	**(5.5)**	**(5.8)**	**(11.3)**	**(6.3)**	**(17.6)**	**(6.6)**	**(24.2)**
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)**	**(6.4)**	**(27.1)**	**(5.5)**	**(5.8)**	**(11.3)**	**(6.3)**	**(17.6)**	**(6.6)**	**(24.2)**
net margin							*nm*							*nm*
NoSH	10.7	12.3	11.48	15.37	12.77	16.00	16.00	19.0	19.0	19.00	19.00	19.00	19.00	19.00
EPS - diluted	**(0.44)**	**(0.41)**	**(0.85)**	**(0.71)**	**(1.62)**	**(0.40)**	**(1.69)**	**(0.29)**	**(0.30)**	**(0.60)**	**(0.33)**	**(0.93)**	**(0.35)**	**(1.27)**

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

			IB Serv./Past 12 Mos. as of 12/12/13	
Rating	**Count**	**Percent**	**Count**	**Percent**
Buy [B]	**163**	**72.44**	**89**	**54.60**
Neutral [N]	**34**	**15.11**	**11**	**32.35**
Sell [S]	**2**	**0.89**	**0**	**0**
Under Review [UR]	**26**	**11.56**	**10**	**38.46**

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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