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EQUITY RESEARCH
Company Report

June 21, 2016

PhaseRx (PZRX - $ 4.82)

Healthcare/Biotechnology

Ticker:	**PZRX**
Rating:	**Buy**
Price Target:	**$ 12.00**

PRX-OTC Selected As Lead Product Candidate and Positive Preclinical POC Data of PRX-ASL Reported

This morning PZRX formally declared PRX-OTC as lead product candidate for the treatment of Ornithine Transcarbamylase Deficiency (OTCD) and announced positive preclinical POC data for second product candidate, PRX-ASL.

- **Details.** PZRX officially declared PRX-OTC as lead product candidate, as a potential treatment of Ornithine Transcarbamylase Deficiency (OTCD). The company also announced positive POC data for its second product candidate, PRX-ASL, as a potential treatment of Argininosuccinate Lyase Deficiency (ASLD). OTCD and ASLD are indications of urea cycle disorders (UCD) that results in hyperammonemia. Preclinical data in OTC-spf^{ash} mice model showed that PRX-OTC demonstrated a statistically significant reduction of plasma ammonia accumulation and returned plasma ammonia back to the normal wild-type level (~100 μM) when compared to mice treated by control mRNA. Additionally, PZRX announced positive data from preclinical studies of PRX-ASL in ASL-deficient mice showing a statistically significant reduction in ammonia levels following two weeks of treatment. Collectively, our estimate suggests that OTCD and ASLD could account for >75% of UCD patients. PZRX plans to conduct Phase IIa (single-dose) and Phase IIb (repeat-dose) clinical POC studies for PRX-OTC starting in late 2017 or early 2018 with data potentially available in 1H18 and 2H18, respectively. One of the key data of the studies is to measure blood ammonia.
- **Implications.** We view today's news encouragingly as it confirms PZRX is on track with its development timeline. In our opinion, the declared lead product candidate, PRX-OTC, could potentially treat the majority (~62%) of the total UCD patients. Positive preclinical POC results from more than one indication are promising given it could demonstrate the potential versatility of its platform in the treatment of other liver-specific orphan indications. We believe the potential for PZRX's i-ERT platform in other indications could potentially be further reinforced if the results from the large animal tolerability preclinical study of PRX-OTC be positive (potentially available YE16).
- **Action.** We are reiterating our Buy rating and $12 price to reflect our positive view based on the substantial potential of hybrid mRNA technology driven i-ERT and from the promising preclinical POC data from PRX-OTC and potentially from other UCD treatments. Our valuation is based on our peer comparable, probability adjusted DCF and sum-of-the-parts analyses.

Trading Data:

Last Price (06/20/2016)	$ 4.82
52–Week High (5/27/2016)	$ 5.77
52–Week Low (5/18/2016)	$ 4.54
Market Cap. (MM)	$ 56
Shares Out. (MM)	12

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-16E	-0.42A	-0.17	-0.18	-0.19	-0.96	NM
FY-15A	-0.23	0.00	0.00	0.00	-1.12	NM
FY-14A	NA	NA	NA	NA	-1.16	NM
FY-13A	NA	NA	NA	NA	NA	NM

Source: Laidlaw & Company estimates

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Anticipated milestones in 2016 and beyond

Product	Indication	Event	Timing	Importance
PRX-OTC	Ornithine transcarbamylase deficiency	Potentially complete large animal tolerability preclinical study	**YE16**	***
		Potentially complete GMP manufacturing	**3Q17**	***
		Potentially file IND	**4Q17**	***
		Potentially report Phase IIa single-dose safety and efficacy results	**1H18**	****
		Potentially report Phase IIb repeat-dosing safety and efficacy results	**2H18**	****

***** / ***** Major catalyst event that could impact share price very significantly while *** event is more informative*

Source: Laidlaw & Company estimates and company presentation.

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Major risks

Clinical study failure could have a major impact on PZRX share value. Despite promising pre-clinical results of the company's lead products, PRX-OTC, PRX-ASL and PRX-ASS1, it remains too early to predict the longer term safety and efficacy from the upcoming clinical studies. Given that clinical validation has not been established, it would be critical for these studies to demonstrate efficacy and a positive safety profile in order to increase the assets and shareholder value. Negative results of Phase I and future clinical studies could have a materially negative impact on the shareholder value; especially since the company has a very diverse-limited pipeline profile.

Yet-to-be-validated hybrid mRNA delivery platform for i-ERT could remain uncertain. Although enzyme replacement therapy has been established as a validated treatment modality in enzyme deficiency diseases; currently there is no hybrid mRNA delivery platform that has been approved or is in a late clinical development stage to demonstrate efficacy. As such, clinical risks for hybrid mRNA based i-ERT are higher than similar products generated from other more proven development platforms.

Product may not be approved or reach anticipated sales. Although PZRX's current pipeline products have exhibited the potential to generate positive clinical outcomes from current and future trials; it remains too early to project whether any of these products would be approved by regulatory agencies. Even if the products were to enter the market, sales could be significantly below projections due to the specific product label under approval, physician consensus for prescribing the drug, changes of treatment paradigms, entrance of competitors, and the possible changes in pricing flexibility and payer reimbursement. A revenue outlook below expectations could also negatively affect PZRX shareholder value.

Additional financings could dilute shareholder value. Although the company currently has ~$19.5MM (pro forma) cash after its recent financing, PZRX most likely would need more financial resources going forward if they want to expand and further develop their pipeline. Should the future operational expenses, especially from R&D and COGs, increase significantly, products not receive FDA approval, or product revenue not reach expectations; the company might need to issue new equity to raise additional cash. Under such a scenario, the share value of existing shareholders could be diluted.

Limited trading liquidity limits shareholder options. Given that PZRX shares only entered the public market recently; daily trading volume and name recognition are relatively modest. As such, shareholders wanting to increase or reduce their positions more substantially in a volatile stock market may face constraints.

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Figure 1: Income Statement

PhaseRx – Income Statement

($'000)	2014	2015	1Q16	2Q16E	3Q16E	4Q16E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Revenue														
Other revenue									1,000	1,000	2,000	2,000	2,000	2,000
Product revenue													46,780	140,572
Total revenues	1,200	375	0	0	0	0	0	0	1,000	1,000	2,000	2,000	48,780	142,572
Gross revenue													38,360	115,269
Research and development	4,860	4,883	1,434	1,448	1,535	1,673	6,091	9,441	11,235	13,369	16,177	18,927	20,252	21,670
General and administrative	1,931	1,299	680	707	728	750	2,866	3,152	3,468	3,780	4,082	4,368	4,586	4,816
Marketing and sales													25,000	26,750
Total operating costs and expenses	6,791	6,182	2,114	2,156	2,264	2,424	8,957	12,594	14,703	17,149	20,259	23,295	49,838	53,235
Operating Incomes (losses)	(5,591)	(5,807)	(2,114)	(2,156)	(2,264)	(2,424)	(8,957)	(12,594)	(13,703)	(16,149)	(18,259)	(21,295)	(11,479)	62,034
Interest expense	(1,367)	(1,649)	(201)	0			(201)	(1,600)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	81	50	55	60	246	220	242	266	293	322	354	390
Benefit conversion of $4MM convertible loan			(1,052)				(1,052)							
Total other income (expense)	(1,258)	(1,570)	(120)	50	55	60	45	(1,380)	(1,358)	(1,333)	(1,305)	(1,275)	(1,275)	(1,272)
Pretax income	(6,849)	(7,377)	(2,234)	(2,106)	(2,209)	(2,364)	(8,912)	(13,974)	(15,061)	(17,482)	(19,564)	(22,570)	(12,753)	60,762
Tax													0	22,482
Net Income (Loss)	(6,849)	(7,377)	(3,286)	(2,106)	(2,209)	(2,364)	(9,964)	(13,974)	(15,061)	(17,482)	(19,564)	(22,570)	(12,753)	38,280
Basic and diluted net loss per share	($1.16)	($1.12)	($0.42)	($0.17)	($0.18)	($0.19)	($0.96)	($1.12)	($1.12)	($1.06)	($1.01)	($1.15)	($0.52)	$1.55
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,882	12,245	12,255	12,265	11,162	12,465	13,465	16,465	19,465	19,565	24,565	24,665
Margin Analysis (% of Sales/Revenue)														
Costs of goods													18%	18%
R&D	405%	1302%	NA	NA	NA	NA	NA	NA	1123%	1337%	809%	946%	42%	15%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	347%	378%	204%	218%	9%	3%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	-1370%	-1615%	-913%	-1065%	-24%	44%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1506%	-1748%	-978%	-1128%	-26%	43%
Tax Rate													37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1506%	-1748%	-978%	-1128%	-26%	27%
Financial Indicator Growth Analysis (YoY%)														
Total Revenue	NA	-69%	-100%	NA	NA	NA	-100%	NA	NA	0%	100%	0%	2339%	192%
R&D	NA	0%	12%	NA	NA	NA	25%	55%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	139%	NA	NA	NA	121%	10%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	35%	NA	NA	NA	45%	41%	17%	17%	18%	15%	114%	7%
Pretax Income	NA	8%	57%	NA	NA	NA	21%	57%	8%	16%	12%	15%	-43%	-576%
Net Income	NA	8%	131%	NA	NA	NA	35%	40%	8%	16%	12%	15%	-43%	-400%
EPS	NA	-3%	84%	NA	NA	NA	-14%	17%	0%	-5%	-5%	15%	-55%	-399%

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Source: Bloomberg LP; Company reports; Laidlaw & Company estimates.

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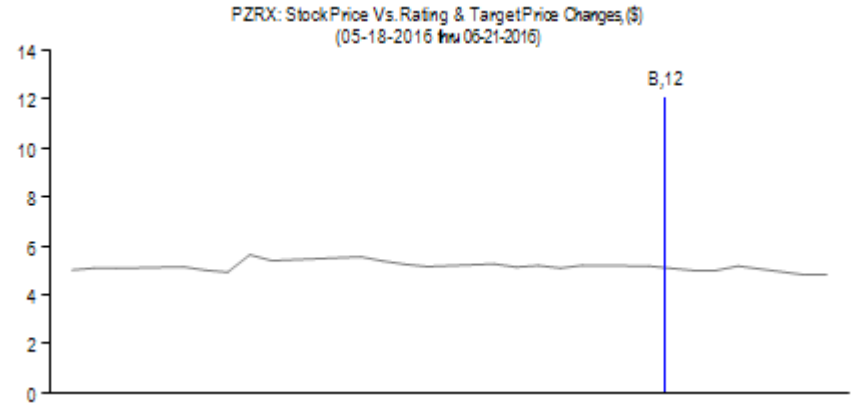
Rating and Price Target Change History



3 Year Rating Change History

Date	Rating	Closing Price ($)
06/14/2016	Buy (B)	5.09

3 Year Price Change History

Date	Target Price ($)	Closing Price, ($)
06/14/2016	12.00	5.09

Source: Laidlaw & Company **Created by: Blue-Compass.net**

Laidlaw & Company Rating System*		% of Companies Under Coverage With This Rating	% of Companies for which Laidlaw & Company has performed services for in the last 12 months	
			Investment Banking	Brokerage
Strong Buy (SB)	Expected to significantly outperform the sector over 12 months.	0.00%	0.00%	0.00%
Buy (B)	Expected to outperform the sector average over 12 months.	67.57%	27.03%	2.70%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	0.00%	0.00%	0.00%
Sell (S)	Returns expected to significantly underperform the sector average over 12 months.	0.00%	0.00%	0.00%

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