

## PhaseRx (PZRX - \$ 1.15)

### 3Q16: Financial Reporting Uneventful While Reporting of the PRX-OTC Large Animal Tolerability Preclinical Study Results by YE16 Remain On Track

This morning, PZRX reported 3Q16 financial results with a net loss of (\$3.3MM), vs. Laidlaw estimates (\$3.5MM). Net loss per share was (\$0.29), vs. (\$0.28) for Laidlaw. PZRX ended 3Q16 with cash of ~\$18.6MM, enough to support its operations into 3Q17, in our opinion.

- Development on track.** PRX-OTC, the lead candidate product for potential treatment in ornithine transcarbamylase deficiency (OTCD), a urea cycle disorder (UCD), remains on track to complete large animal tolerability preclinical study by year-end 2016. We anticipate the study results could be available around a similar time frame (YE16) – an important near-term milestone in our opinion. Additionally, a PRX-OTC IND filing is scheduled in 4Q17 with Phase IIa (single-dose) and Phase IIb (repeat-dose) clinical POC studies to start in early 2018 and potential data reporting in 1H18 and 2H18, respectively. For the second product candidate, PRX-ASL as a potential treatment of argininosuccinate lyase deficiency (ASLD) another UCD, PZRX reported positive POC preclinical data earlier this year (June 21). Preclinical data of PRX-ASL and PRX-OTC showed statistically significant reductions in plasma ammonia levels – a possible approvable endpoint for clinical studies. In addition to current therapeutic development in UCDs, we believe PZRX could explore potential collaborations with prospective partners by leveraging its i-ERT platform in other liver-specific orphan indications and could be additional upsides for PZRX shares.
- PZRX is over-sold.** Despite the recent overall negative sentiment on biotech stocks with a particular hard hit on the early stage small cap names, we believe PZRX shares have been substantially over-sold given the potential upside of the mRNA therapy platform and the greater enthusiasm of privately owned Moderna Therapeutics. As such, we believe it is a buying opportunity for PZRX given its low valuation.
- 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.

*Healthcare/Biotechnology*

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

#### Trading Data:

Last Price (11/07/2016)	\$ 1.15
52-Week High (5/27/2016)	\$ 5.77
52-Week Low (11/7/2016)	\$ 0.96
Market Cap. (MM)	\$ 13
Shares Out. (MM)	12

#### Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
<b>FY-16E</b>	-0.42A	-1.84A	-0.29A	-0.37	-2.91	NM
<b>FY-15A</b>	-0.23	-3.29	-3.67	0.00	-1.12	NM
<b>FY-14A</b>	NA	NA	NA	NA	-1.16	NM
<b>FY-13A</b>	NA	NA	NA	NA	NA	NM

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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.

## Major risks

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**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.

Figure 1: Income Statement

PhaseRx – Income Statement														
(\$'000)	2014	2015	1Q16	2Q16	3Q16	4Q16E	2016E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
<b>Revenue</b>														
Other revenue									1,000	1,000	2,000	2,000	2,000	2,000
Product revenue													46,780	140,572
<b>Total revenues</b>	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	1,416	1,787	1,912	6,549	10,151	12,080	14,375	17,394	20,351	21,775	23,299
General and administrative	1,931	1,299	680	880	1,351	1,392	4,303	4,733	5,206	5,675	6,129	6,558	6,885	7,230
Marketing and sales													25,000	26,750
Non-cash financial advising fees				7,515			7,515							
<b>Total operating costs and expenses</b>	6,791	6,182	2,114	9,811	3,138	3,304	18,367	14,884	17,286	20,050	23,522	26,908	53,661	57,279
<b>Operating Incomes (losses)</b>	(5,591)	(5,807)	(2,114)	(9,811)	(3,138)	(3,304)	(18,367)	(14,884)	(16,286)	(19,050)	(21,522)	(24,908)	(15,301)	57,990
Interest expense	(1,367)	(1,649)	(201)	(1,383)	(233)	(1,100)	(2,917)	(1,600)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	81	110	28	90	309	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)	(1,273)	(205)	(1,010)	(2,608)	(1,380)	(1,358)	(1,333)	(1,305)	(1,275)	(1,275)	(1,272)
Pretax income	(6,849)	(7,377)	(2,234)	(11,084)	(3,343)	(4,314)	(20,975)	(16,264)	(17,644)	(20,382)	(22,827)	(26,183)	(16,576)	56,718
Tax													0	20,986
<b>Net Income (Loss)</b>	(6,849)	(7,377)	(3,286)	(11,084)	(3,343)	(4,314)	(22,027)	(16,264)	(17,644)	(20,382)	(22,827)	(26,183)	(16,576)	35,732
Basic and diluted net loss per share	(\$1.16)	(\$1.12)	(\$0.42)	(\$1.84)	(\$0.29)	(\$0.37)	(\$2.91)	(\$1.37)	(\$1.37)	(\$1.28)	(\$1.21)	(\$1.38)	(\$0.69)	\$1.48
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,882	6,013	11,690	11,700	9,321	11,900	12,900	15,900	18,900	19,000	24,000	24,100
<b>Margin Analysis (% of Sales/Revenue)</b>														
Costs of goods													18%	18%
R&D	405%	1302%	NA	NA	NA	NA	NA	NA	1208%	1437%	870%	1018%	45%	16%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	521%	567%	306%	328%	14%	5%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	-1629%	-1905%	-1076%	-1245%	-31%	41%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1764%	-2038%	-1141%	-1309%	-34%	40%
Tax Rate													37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1764%	-2038%	-1141%	-1309%	-34%	25%
<b>Financial Indicator Growth Analysis (YoY%)</b>														
Total Revenue	NA	-69%	-100%	NA	NA	NA	-100%	NA	NA	0%	100%	0%	2339%	192%
R&D	NA	0%	12%	22%	52%	NA	34%	55%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	139%	211%	322%	NA	231%	10%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	35%	580%	110%	NA	197%	-19%	16%	16%	17%	14%	99%	7%
Pretax Income	NA	8%	57%	534%	72%	NA	184%	-22%	8%	16%	12%	15%	-37%	-442%
Net Income	NA	8%	131%	534%	72%	NA	199%	-26%	8%	16%	12%	15%	-37%	-316%
EPS	NA	-3%	84%	-44%	-92%	NA	160%	-53%	0%	-6%	-6%	14%	-50%	-315%

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

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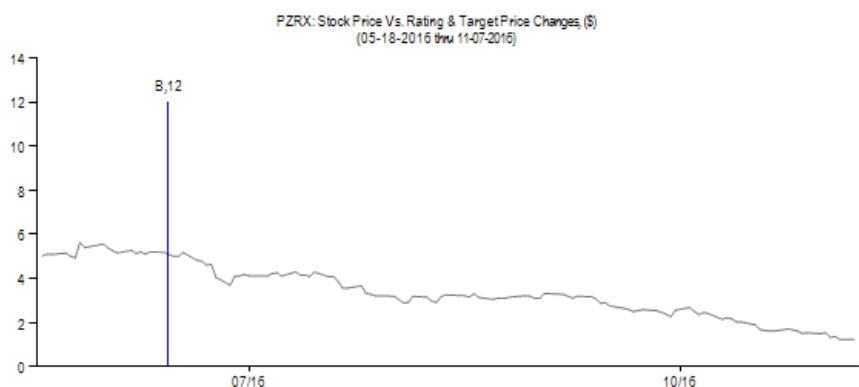
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#### Rating and Price Target Change History



#### 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
<b>Strong Buy (SB)</b>	Expected to significantly outperform the sector over 12 months.	2.56%	2.56%	0.00%
<b>Buy (B)</b>	Expected to outperform the sector average over 12 months.	58.97%	28.21%	2.56%
<b>Hold (H)</b>	Expected returns to be in line with the sector average over 12 months.	5.13%	0.00%	0.00%
<b>Sell (S)</b>	Returns expected to significantly underperform the sector average over 12 months.	2.56%	0.00%	0.00%

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