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EQUITY RESEARCH
Company Report

November 8, 2016

PhaseRx (PZRX - $ 1.08)

Healthcare/Biotechnology

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

Positive Preclinical Large Animal Study Results; An Important Step For Demonstrating Hybrid mRNA Platform Safety

This morning, PZRX reported positive results from its preclinical study assessing the safety and tolerability of its novel hybrid mRNA delivery platform in large animals – an important step for advancing the platform and the lead product candidate PRX-OTC (Ornithine Transcarbamylase Deficiency) into clinical studies.

- **Details.** PZRX announced positive results of a single escalating dose response preclinical study in non-human primates for assessing the safety of its proprietary hybrid mRNA technology platform. The data suggested that the platform is safe and well tolerated at all dose levels tested. PZRX used human erythropoietin (hEPO) as a readout and delivered 0.1, 0.3, and 1.0 mg/kg mRNA via its hybrid mRNA technology. The data exhibited dose-dependent increases in hEPO protein while maintaining favorable safety profile. hEPO protein levels were raised at three orders of magnitude above normal physiological levels and were functionally active as reflected in a proportionally robust increase in reticulocyte count in all dose groups. Tolerability was very favorable with no statistically significant dose-related changes in liver enzymes or cytokines, including IL-6, TNF-a, IFN-g, IL-12, or IP-10. We anticipate that during 2017, PZRX will complete IND-enabling studies for PRX-OTC, which include GMP manufacture of sufficient material, with potential to file an IND in 4Q17 and initiation of PRX-OTC in OTCD Phase I study in 2018. We estimate the top-line results of the single dose and the repeated dose studies could be available in 1H18 and 2H18, respectively.
- **Implications.** We view today's news positively as it achieved an important milestone for any drug advancing into clinical study. Although PRX-OTC exhibited efficacy in an OTCD mouse model before, it is critically important to demonstrate safety in a large non-human primate animal model, which most closely resembles human, before advancing PZRX's novel, new platform into a Phase I study.
- **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 (11/08/2016)	$ 1.08
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
FY-16E	-0.42A	-1.84A	-0.29A	-0.37	-2.91	NM
FY-15A	-0.23	-3.29	-3.67	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 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	2Q16	3Q16	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	1,416	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							
Total operating costs and expenses	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
Operating Incomes (losses)	(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
Net Income (Loss)	(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
Margin Analysis (% of Sales/Revenue)														
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%
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%	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 & Company estimates.

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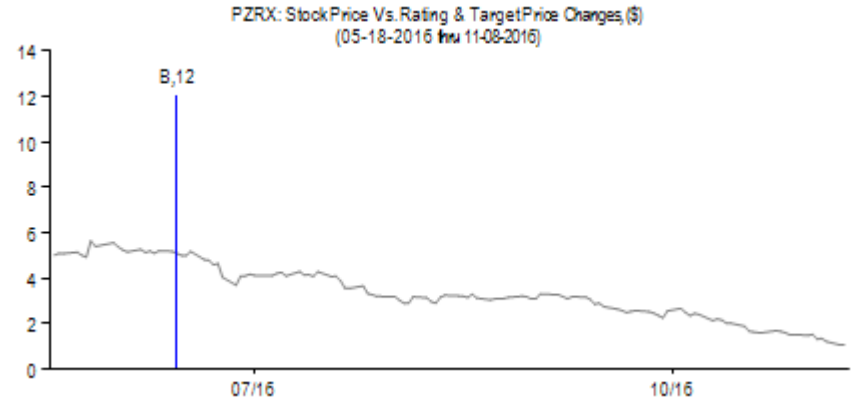
Rating and Price Target Change History



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

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