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EQUITY RESEARCH
Company Report

March 28, 2017

PhaseRx (PZRX - $1.51)

Healthcare/Biotechnology

4Q16: 2017 Will Be the Year to Get PRX-OTC Ready for IND Filing in 4Q17 and Phase I/II Trial in 2018

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

Yesterday, PZRX reported 4Q16 financial results with a net loss of ($3.5MM), vs. Laidlaw estimates ($4.3MM). Net loss per share was ($0.30), vs. ($0.37) for Laidlaw. PZRX ended 4Q16 with cash of ~$15.5MM, enough to support its operations into 2018, in our opinion.

- **Development remains on track.** Management indicated that 2017 will be the year to complete all preclinical preparation and enable PZRX to file an IND for PRX-OTC in 4Q17, followed by commencing a Phase I/II trial in 1Q18 for treating ornithine transcarbamylase deficiency (OTCD) patients. PZRX remains on track for completing both GLP toxicology and GMP manufacturing by 3Q17.
- **Proposed initial clinical studies.** We anticipate the initial clinical trial will consist of two clinical POC stages: Phase IIa (single-dose) and Phase IIb (repeat-dose) of PRX-OTC therapy for adults and pediatric patients currently on Ravicti therapy. The first Phase IIa trial will enroll smaller number of adult patients for assessing PK and PD (based on plasma ammonia levels). The subsequent Phase IIb trial of repeat-dosing would enroll larger number of adult and pediatric patients in order to make direct ammonia levels comparisons between Ravicti and PRX-OTC therapy. Additionally, PZRX intends to roll patients over to an extension study for one year treatment. We anticipate the top-line safety and efficacy data readouts for the Phase IIa and Phase IIb portions of the study could be available in 1H18 and 2H18, respectively. We view the two data readouts could be inflection points for PZRX shares.
- **Recent positive preclinical data encouraging.** Earlier reported positive results from the large non-human primate animal study (see our 2016-11-08 note) marked an important step for demonstrating the safety of PZRX's hybrid mRNA platform. A recent presentation at the WORLDSymposium also showed in a hyperammonemia mouse model, PRX-OTC could normalize plasma ammonia by continued expressed hOTC protein with $t_{1/2}$ of >12 days.
- **Action.** We are reiterating our Buy rating and $12 price target. This reflects our positive view of the substantial potential of hybrid mRNA technology driven i-ERT, 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 (3/27/2017)	$1.51
52–Week High (5/27/2016)	$5.77
52–Week Low (11/7/2016)	$0.96
Market Cap. (MM)	$17
Shares Out. (MM)	7.524

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-17E	-0.29	-0.28	-0.30	-0.31	-1.19	NM
FY-16A	-0.42	-1.84	-0.29	-0.30	-2.68	NM
FY-15A	-0.23	-3.29	-3.67	0.00	-1.12	NM
FY-14A	NA	NA	NA	NA	-1.16	NM

Source: Laidlaw & Company estimates

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Anticipated milestones in 2017 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	4Q16	2016	1Q17E	2Q17E	3Q17E	4Q17E	2017E	2018E	2019E	2020E	2021E	2022E	2023E
Revenue																		
Other revenue								0	0	0	0	0	1,000	1,000	2,000	2,000	2,000	2,000
Product revenue								0	0	0	0	0					46,780	140,572
Total revenues	1,200	375	0	0	0	0	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	2,025	6,662	1,883	1,959	2,115	2,179	8,136	9,682	11,521	13,941	16,310	17,452	18,674
General and administrative	1,931	1,299	680	880	1,351	1,242	4,153	1,279	1,241	1,278	1,316	5,115	5,626	6,133	6,623	7,087	7,441	7,813
Marketing and sales																	25,000	26,750
Non-cash financial advising fees			0	7,515	0	0	7,515											
Total operating costs and expenses	6,791	6,182	2,114	9,811	3,138	3,267	18,330	3,163	3,199	3,393	3,495	13,251	15,308	17,654	20,564	23,397	49,893	53,237
Operating Incomes (losses)	(5,591)	(5,807)	(2,114)	(9,811)	(3,138)	(3,267)	(18,330)	(3,163)	(3,199)	(3,393)	(3,495)	(13,251)	(14,308)	(16,654)	(18,564)	(21,397)	(11,534)	62,032
Interest expense	(1,367)	(1,649)	(201)	(1,383)	(233)	(236)	(2,058)	(231)	(169)	(207)	(240)	(847)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	81	110	28	27	61	30	28	40	50	148	163	179	197	217	238	262
Benefit conversion of $4MM convertible loan			(1,052)				(1,052)											
Total other income (expense)	(1,258)	(1,570)	(120)	(1,273)	(205)	(209)	(1,807)	(201)	(141)	(167)	(190)	(699)	(1,437)	(1,420)	(1,401)	(1,380)	(1,391)	(1,399)
Pretax income	(6,849)	(7,377)	(2,234)	(11,084)	(3,343)	(3,476)	(20,137)	(3,364)	(3,340)	(3,560)	(3,685)	(13,950)	(15,745)	(18,074)	(19,965)	(22,778)	(12,924)	60,633
Tax																	0	22,434
Net Income (Loss)	(6,849)	(7,377)	(3,286)	(11,084)	(3,343)	(3,476)	(20,137)	(3,364)	(3,340)	(3,560)	(3,685)	(13,950)	(15,745)	(18,074)	(19,965)	(22,778)	(12,924)	38,199
Basic and diluted net loss per share	($1.16)	($1.12)	($0.42)	($1.84)	($0.29)	($0.30)	($2.68)	($0.29)	($0.28)	($0.30)	($0.31)	($1.19)	($1.24)	($1.15)	($1.07)	($1.21)	($0.54)	$1.60
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,882	6,013	11,690	11,690	7,524	11,710	11,730	11,750	11,770	11,740	12,740	15,740	18,740	18,840	23,840	23,940
Margin Analysis (% of Sales/Revenue)																		
Costs of goods																	18%	18%
R&D	405%	1302%	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	968%	1152%	697%	816%	36%	13%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	563%	613%	331%	354%	15%	5%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-1431%	-1665%	-928%	-1070%	-24%	44%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-1574%	-1807%	-998%	-1139%	-26%	43%
Tax Rate																	37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	NA	NA	NA	NA	-1574%	-1807%	-998%	-1139%	-26%	27%
Financial Indicator Growth Analysis (YoY%)																		
Total Revenue	NA	-69%	-100%	NA	NA	NA	-100%	NA	NA	NA	NA	NA	NA	0%	100%	0%	2339%	192%
R&D	NA	0%	12%	22%	52%	NA	36%	31%	38%	18%	8%	22%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	139%	211%	322%	NA	220%	88%	41%	-5%	6%	23%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	35%	580%	110%	NA	197%	50%	-67%	8%	7%	-28%	16%	15%	16%	14%	113%	7%
Pretax Income	NA	8%	57%	534%	72%	NA	173%	51%	-70%	7%	6%	-31%	13%	15%	10%	14%	-43%	-569%
Net Income	NA	8%	131%	534%	72%	NA	173%	2%	-70%	7%	6%	-31%	13%	15%	10%	14%	-43%	-396%
EPS	NA	-3%	84%	-44%	-92%	NA	139%	-31%	-85%	6%	5%	-56%	4%	-7%	-7%	13%	-55%	-394%

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

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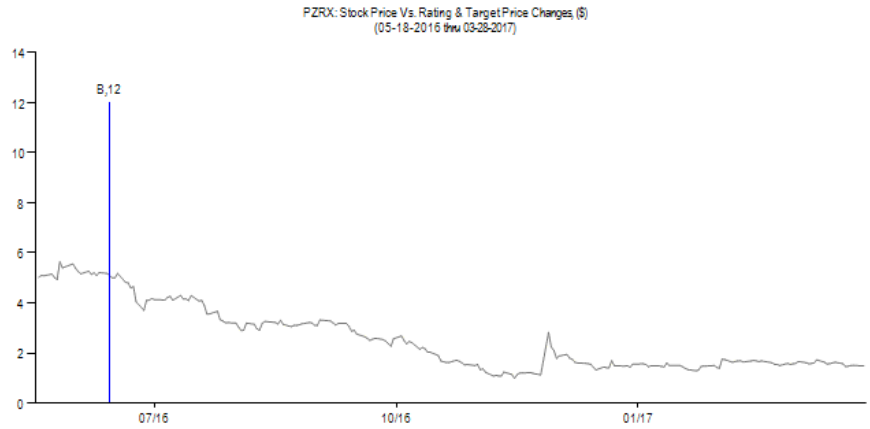
Rating and Price Target Change History



3 Year Rating Change History

Date	Rating	Closing Price ($)
06/14/2...	Buy (B)	5.09

3 Year Price Change History

Date	Target Price ($)	Closing Price, ($)
06/14/2...	12.00	5.09

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

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			Investment Banking	Brokerage
Strong Buy (SB)	Expected to significantly outperform the sector over 12 months.	2.44%	2.44%	0.00%
Buy (B)	Expected to outperform the sector average over 12 months.	63.41%	29.27%	2.44%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	2.44%	0.00%	0.00%
Sell (S)	Returns expected to significantly underperform the sector average over 12 months.	4.88%	0.00%	0.00%

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