LAIDLAW & COMPANY
Est. 1842
London
New York
San Francisco
Boston

EQUITY RESEARCH
Company Report

May 15, 2017

PhaseRx (PZRX - $1.26)

Healthcare/Biotechnology

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

1Q17: Focus Remains On Completing Manufacturing Activities for PRX-OTC IND Filing and Phase I/II Trial in 1H18

PZRX recently reported 1Q17 financial results with a net loss of ($4.1MM), vs. Laidlaw estimates ($3.7MM). Net loss per share was ($0.35), vs. ($0.29) for Laidlaw. PZRX ended 1Q17 with cash of ~$12MM, enough to support its operations into 2018, in our opinion.

- **Development updates.** PZRX recently received a positive EMA opinion for orphan designation on PRX-OTC for the treatment of ornithine transcarbamylase deficiency (OTCD). Additionally, developmental updates were provided, specifically PRX-OTC scale-up and that both GLP toxicology and GMP manufacturing activities could be completed by 4Q17. Following completion of all preclinical preparations, PZRX guided to file an IND for PRX-OTC, potentially in 1Q18, followed by commencing a Phase I/II trial in OTCD patients, shortly thereafter. We anticipate that PRX-OTC in OTCD Phase I/II single-dose safety and efficacy data, including plasma ammonia levels, could be available in 2H18.
- **Proposed initial PRX-OTC 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 a smaller number of adult patients for assessing PK and PD (based on plasma ammonia levels). The subsequent Phase IIb trial of repeat-dosing could enroll a 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. Should the single-dose safety and efficacy data be available in 2H18, we anticipate the data readout of the repeat-dose could occur in 1H19. We view the two data readouts could be inflection points for PZRX shares.
- **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 (5/12/2017)	$1.26
52–Week High (5/27/2016)	$5.77
52–Week Low (11/7/2016)	$0.96
Market Cap. (MM)	$14
Shares Out. (MM)	7.524

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-17E	-0.35A	-0.35	-0.37	-0.38	-1.46	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

Yale Jen, Ph.D.
Managing Director /
Senior Biotechnology Analyst
(212) 953-4978
yjen@laidlawltd.com

Anticipated milestones in 2017 and beyond

Product	Indication	Event	Timing	Importance
PRX-OTC	Ornithine transcarbamylase deficiency	Potentially complete GMP manufacturing	**4Q17**	***
		Potentially file IND	**1Q18**	***
		Potentially report Phase IIa single-dose safety and efficacy results	**2H18**	****
		Potentially report Phase IIb repeat-dosing safety and efficacy results	**1H19**	****

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

Source: Laidlaw & Company estimates and company presentation.

Yale Jen, Ph.D.
yjen@laidlawltd.com

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.

Yale Jen, Ph.D.
yjen@laidlawltd.com

Figure 1: Income Statement

PhaseRx – Income Statement

($'000)	2014	2015	2016	1Q17	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	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	6,662	2,326	2,419	2,613	2,691	10,049	11,958	14,230	17,218	20,145	21,555	23,064
General and administrative	1,931	1,299	4,153	1,589	1,541	1,588	1,635	6,353	6,988	7,617	8,227	8,803	9,243	9,705
Marketing and sales													25,000	26,750
Non-cash financial advising fees			7,515											
Total operating costs and expenses	6,791	6,182	18,330	3,915	3,960	4,200	4,326	16,402	18,946	21,847	25,445	28,948	55,798	59,519
Operating Incomes (losses)	(5,591)	(5,807)	(18,330)	(3,915)	(3,960)	(4,200)	(4,326)	(16,402)	(17,946)	(20,847)	(23,445)	(26,948)	(17,438)	55,750
Interest expense	(1,367)	(1,649)	(2,058)	(235)	(169)	(207)	(240)	(851)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	61	25	28	40	50	143	157	173	190	209	230	253
Benefit conversion of $4MM convertible loan			(1,052)											
Total other income (expense)	(1,258)	(1,570)	(1,807)	(210)	(141)	(167)	(190)	(708)	(1,443)	(1,426)	(1,408)	(1,388)	(1,399)	(1,408)
Pretax income	(6,849)	(7,377)	(20,137)	(4,125)	(4,101)	(4,367)	(4,516)	(17,110)	(19,389)	(22,273)	(24,852)	(28,335)	(18,837)	54,342
Tax													0	20,106
Net Income (Loss)	(6,849)	(7,377)	(20,137)	(4,125)	(4,101)	(4,367)	(4,516)	(17,110)	(19,389)	(22,273)	(24,852)	(28,335)	(18,837)	34,235
Basic and diluted net loss per share	($1.16)	($1.12)	($2.68)	($0.35)	($0.35)	($0.37)	($0.38)	($1.46)	($1.52)	($1.42)	($1.33)	($1.51)	($0.79)	$1.43
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,524	11,690	11,710	11,730	11,750	11,720	12,720	15,720	18,720	18,820	23,820	23,920
Margin Analysis (% of Sales/Revenue)														
Costs of goods													18%	18%
R&D	405%	1302%	NA	NA	NA	NA	NA	NA	1196%	1423%	861%	1007%	44%	16%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	699%	762%	411%	440%	19%	7%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	-1795%	-2085%	-1172%	-1347%	-36%	39%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1939%	-2227%	-1243%	-1417%	-39%	38%
Tax Rate													37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1939%	-2227%	-1243%	-1417%	-39%	24%
Financial Indicator Growth Analysis (YoY%)														
Total Revenue	NA	-69%	-100%	NA	NA	NA	NA	NA	NA	0%	100%	0%	2339%	192%
R&D	NA	0%	36%	62%	71%	46%	33%	51%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	220%	134%	75%	18%	32%	53%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	197%	85%	-60%	34%	32%	-11%	16%	15%	16%	14%	93%	7%
Pretax Income	NA	8%	173%	85%	-63%	31%	30%	-15%	13%	15%	12%	14%	-34%	-388%
Net Income	NA	8%	173%	26%	-63%	31%	30%	-15%	13%	15%	12%	14%	-34%	-282%
EPS	NA	-3%	139%	-15%	-81%	30%	29%	-45%	4%	-7%	-6%	13%	-47%	-281%

Yale Jen, Ph.D. 212-953-4978

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

Yale Jen, Ph.D.
yjen@laidlawltd.com

DISCLOSURES:

ANALYST CERTIFICATION

The analyst responsible for the content of this report hereby certifies that the views expressed regarding the company or companies and their securities accurately represent his personal views and that no direct or indirect compensation is to be received by the analyst for any specific recommendation or views contained in this report. Neither the author of this report nor any member of his immediate family or household maintains a position in the securities mentioned in this report.

EQUITY DISCLOSURES

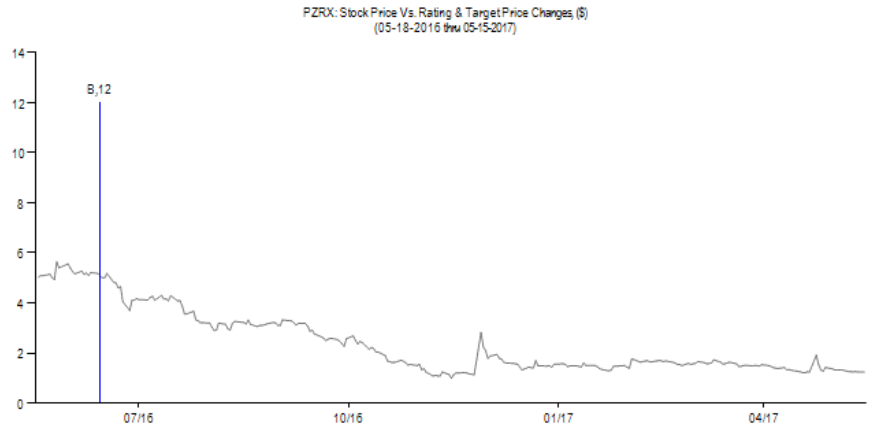
For the purpose of ratings distributions, regulatory rules require the firm to assign ratings to one of three rating categories (i.e. Strong Buy/Buy-Overweight, Hold, or Underweight/Sell) regardless of a firm's own rating categories. Although the firm's ratings of Buy/Overweight, Hold, or Underweight/Sell most closely correspond to Buy, Hold and Sell, respectively, the meanings are not the same because our ratings are determined on a relative basis against the analyst sector universe of stocks. An analyst's coverage sector is comprised of companies that are engaged in similar business or share similar operating characteristics as the subject company. The analysis sector universe is a sub-sector to the analyst's coverage sector, and is compiled to assist the analyst in determining relative valuations of subject companies. The composition of an analyst's sector universe is subject to change over time as various factors, including changing market conditions occur. Accordingly, the rating assigned to a particular stock represents solely the analyst's view of how that stock will perform over the next 12-months relative to the analyst's sector universe.

Additional information available upon request.

Laidlaw & Co (UK) Ltd. has not provided any investment banking services for the company (ies) mentioned in this report over the last 12 months.

RATINGS INFORMATION

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**

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.	2.27%	0.00%	0.00%
Buy (B)	Expected to outperform the sector average over 12 months.	61.36%	29.55%	2.27%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	2.27%	0.00%	0.00%
Sell (S)	Returns expected to significantly underperform the sector average over 12 months.	0.00%	0.00%	0.00%

ADDITIONAL COMPANIES MENTIONED

ADDITIONAL DISCLOSURES

As of the date of this report, neither the author of this report nor any member of his immediate family or household maintains an ownership position in the securities of the company (ies) mentioned in this report.

This report does not provide individually tailored investment advice and has been prepared without regard to the individual financial circumstances and objectives of persons who receive it. Laidlaw & Co (UK), Ltd. recommends that investors independently evaluate particular investments and strategies, and encourages investors to seek the advice of a financial adviser. The appropriateness of a particular investment or strategy will depend on an investor's individual circumstances and objectives. The securities, instruments, or strategies discussed in this report may not be suitable for all investors, and certain investors may not be eligible to purchase or participate

Yale Jen, Ph.D.
yjen@laidlawltd.com

in some or all of them. This report is not an offer to buy or sell or the solicitation of an offer to buy or sell any security/instrument or to participate in any particular trading strategy.

Associated persons of Laidlaw & Co (UK), Ltd not involved in the preparation of this report may have investments in securities/instruments or derivatives of securities/instruments of companies mentioned herein and may trade them in ways different from those discussed in this report. While Laidlaw & Co (UK), Ltd., prohibits analysts from receiving any compensation. Bonus or incentive based on specific recommendations for, or view of, a particular company, investors should be aware that any or all of the foregoing, among other things, may give rise to real or potential conflicts of interest.

With the exception of information regarding Laidlaw & Co (UK), Ltd. this report is based on public information. Laidlaw & Co (UK), Ltd makes every effort to use reliable, comprehensive information, but we make no representation that it is accurate or complete and it should not be relied upon as such. Any opinions expressed are subject to change and Laidlaw & Co (UK), Ltd disclaims any obligation to advise you of changes in opinions or information or any discontinuation of coverage of a subject company. Facts and views presented in this report have not been reviewed by, and may not reflect information known to, professionals in other Laidlaw & Co (UK), Ltd business areas. Laidlaw & Co (UK), Ltd associated persons conduct site visits from time to time but are prohibited from accepting payment or reimbursement by the company of travel expenses for such visits. The value of and income from your investments may vary because of changes in interest rates, foreign exchange rates, default rates, prepayment rates, securities/instruments prices. market indexes, operational or financial conditions of companies or other factors. There may be time limitations on the exercise of options or other rights in securities/instruments transactions. Past performance is not necessarily a guide to future performance. Estimates of future performance are based on assumptions that may not be realized. If provided, and unless otherwise stated, the closing price on the cover page is that of the primary exchange for the subject company's securities/instruments.

Any trademarks and service marks contained in this report are the property of their respective owners. Third-party data providers make no warranties or representations of any kind relating to the accuracy, completeness, or timeliness of the data they provide and shall not have liability for any damages of any kind relating to such data. This report or any portion thereof may not be reprinted, sold or redistributed without the written consent of Laidlaw & Co (UK), Ltd. This report is disseminated and available primarily electronically, and, in some cases, in printed form.

The information and opinions in this report were prepared by Laidlaw & Co (UK), Ltd. For important disclosures, please see Laidlaw & Co (UK), Ltd.'s disclosure website at www.Laidlawltd.com, or contact your investment representative or Laidlaw & Co (UK), Ltd at 546 Fifth Ave, 5th Floor, New York, NY 10036 USA.

Yale Jen, Ph.D.
yjen@laidlawltd.com

NOTES:

Yale Jen, Ph.D.
yjen@laidlawltd.com