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EQUITY RESEARCH
Company Report

August 30, 2016

PhaseRx (PZRX - $ 3.22)

Healthcare/Biotechnology

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

Robust Investor Interest in mRNA Therapeutics Should Bode Well For PZRX Going Forward

According to news reports and its recent SEC filing, privately owned mRNA therapeutics company Moderna Therapeutics has recently raised $451MM of an intended $600MM venture financing for future development.

- **Details.** Moderna reported in late July that by combining financing and partnership payments, the company has ~ one billion dollars (pro forma) cash. Moderna did not reveal details about the financing and we believe the fundraised include a $140MM investment made by AstraZeneca earlier this month. Moderna has 11 products in its pipeline; and has forged several collaborations with Pharma and Biotech companies. They include Alexion (10 rare diseases), Vertex (cystic fibrosis), AstraZeneca (cardiovascular and oncology) and Merck (cancer vaccine). AstraZeneca and Moderna recently have filed a Clinical Trial Application (CTA) of AZD8601 and plan to commence a Phase I trial in Europe. AZD8601 is a VEGF-A targeted mRNA therapeutics with potential for treating heart failure, diabetic wound healing and other ischemic vascular diseases.
- **Implications.** Although Moderna is a competitor to PZRX in the mRNA therapeutics arena; we view the recent great investor enthusiasm could also benefit PZRX and other mRNA therapeutics companies as testimony for the potential of mRNA therapeutics as a valuable emerging treatment modality. We view PZRX has some unique attributes compared to its peers as the only publicly traded company in this space with major focus on developing urea cycle disorders (UCD) therapies in-house. A near-term milestone is the reporting of large animal tolerability preclinical study results of the lead product, PRX-OTC by YE16. 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 late 2017/early 2018 and potential data reporting in 1H18 and 2H18, respectively. PRX-OTC is a potential treatment for ornithine transcarbamylase deficiency (OTCD). PZRX could also leverage its i-ERT platform for collaboration with partners to develop therapies in other liver-specific orphan indications.
- **Action.** We are reiterating our Buy rating and $12 target price to reflect 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 (08/29/2016)	$ 3.22
52–Week High (5/27/2016)	$ 5.77
52–Week Low (8/3/2016)	$ 2.65
Market Cap. (MM)	$ 38
Shares Out. (MM)	12

Earnings Estimates: (per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-16E	-0.42A	-1.84A	-0.28	-0.29	-2.83	NM
FY-15A	-0.23	-3.29	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	2Q16	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	1,416	1,501	1,636	5,987	9,280	11,043	13,141	15,901	18,604	19,906	21,300
General and administrative	1,931	1,299	680	880	906	934	3,400	3,740	4,114	4,484	4,843	5,182	5,441	5,713
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	2,407	2,570	16,902	13,020	15,157	17,625	20,744	23,786	50,347	53,763
Operating Incomes (losses)	(5,591)	(5,807)	(2,114)	(9,811)	(2,407)	(2,570)	(16,902)	(13,020)	(14,157)	(16,625)	(18,744)	(21,786)	(11,988)	61,506
Interest expense	(1,367)	(1,649)	(201)	(1,383)	(1,150)	(1,100)	(3,834)	(1,600)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	81	110	95	90	376	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)	(1,055)	(1,010)	(3,458)	(1,380)	(1,358)	(1,333)	(1,305)	(1,275)	(1,275)	(1,272)
Pretax income	(6,849)	(7,377)	(2,234)	(11,084)	(3,462)	(3,580)	(20,360)	(14,400)	(15,515)	(17,958)	(20,049)	(23,061)	(13,262)	60,234
Tax													0	22,287
Net Income (Loss)	(6,849)	(7,377)	(3,286)	(11,084)	(3,462)	(3,580)	(21,412)	(14,400)	(15,515)	(17,958)	(20,049)	(23,061)	(13,262)	37,948
Basic and diluted net loss per share	($1.16)	($1.12)	($0.42)	($1.84)	($0.28)	($0.29)	($2.83)	($1.16)	($1.15)	($1.09)	($1.03)	($1.18)	($0.54)	$1.54
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,882	6,013	12,255	12,265	9,604	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	1104%	1314%	795%	930%	41%	15%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	411%	448%	242%	259%	11%	4%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	-1416%	-1663%	-937%	-1089%	-25%	43%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1552%	-1796%	-1002%	-1153%	-27%	42%
Tax Rate													37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1552%	-1796%	-1002%	-1153%	-27%	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%	22%	NA	NA	23%	55%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	139%	211%	NA	NA	162%	10%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	35%	580%	NA	NA	173%	-23%	16%	16%	18%	15%	112%	7%
Pretax Income	NA	8%	57%	534%	NA	NA	176%	-29%	8%	16%	12%	15%	-42%	-554%
Net Income	NA	8%	131%	534%	NA	NA	190%	-33%	8%	16%	12%	15%	-42%	-386%
EPS	NA	-3%	84%	-44%	NA	NA	153%	-59%	0%	-5%	-6%	14%	-54%	-385%

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

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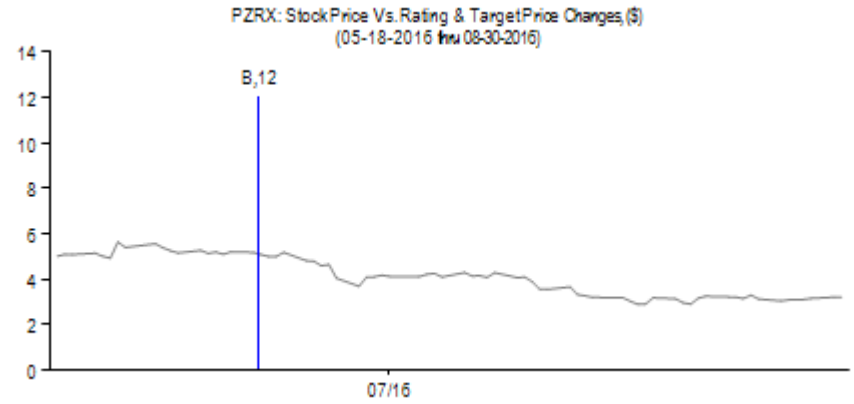
Rating and Price Target Change History



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.	62.16%	27.03%	2.70%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	2.70%	0.00%	0.00%
Sell (S)	Returns expected to significantly underperform the sector average over 12 months.	2.70%	0.00%	0.00%

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AstraZeneca (AZN – Not Rated)

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