

PhaseRx (PZRX - \$ 3.04)

2Q16: Financial Reporting Uneventful While Developments for PRX-OTC Are Full Steam Ahead

Yesterday after market close, PZRX reported 2Q16 financial results with a net loss of (\$11.1MM), vs. Laidlaw estimates (\$2.1MM). Net loss per share was (\$1.84), vs. (\$0.17) for Laidlaw. The discrepancies are mainly due to the differences of \$7.5MM non-cash financial advising fees and a much smaller figure of share outstanding. PZRX ended 2Q16 with cash of ~\$21.1MM, enough to support its operations into 3Q17, in our opinion.

- **Balance sheet is well replenished.** After its IPO and a loan and security agreement, PZRX has collected \$18.5MM and \$6MM, respectively with additional \$2MM loan to come later. With a total of ~\$24.5MM cash expected, we believe the company has sufficient financial resources for advancing the pipeline, mainly PRX-OTC, into clinical studies to demonstrate proof-of-concept results.
- **Pipeline updates.** PZRX recently declared PRX-OTC as lead product candidate as a potential ornithine transcarbamylase deficiency (OTCD) treatment. We anticipate the company will allocate the vast majority of its resources to advance this product forward with an IND filing projected in 4Q17. Phase IIa (single-dose) and Phase IIb (repeat-dose) clinical POC studies are to start in late 2017 or early 2018 with potential data reporting in 1H18 and 2H18, respectively. Additionally, PZRX also reported positive POC data for second product candidate, PRX-ASL, a potential treatment of argininosuccinate lyase deficiency (ASLD) recently. As a reminder, pre-clinical studies of both PRX-OTC and PRX-ASL showed statistically significant reductions in plasma ammonia levels – possible approvable endpoint for clinical studies. Near-term, PZRX plans to report PRX-OTC large animal tolerability preclinical study results potentially by YE16 – an important milestone, in our opinion. In addition to developments of therapies for urea cycle disorders (UCD), potential collaborations with prospective partners by leveraging PZRX's i-ERT platform in other liver-specific orphan indications could be additional upsides for PZRX shares.
- **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.

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

Healthcare/Biotechnology

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

Trading Data:

Last Price (08/02/2016)	\$ 3.04
52-Week High (5/27/2016)	\$ 5.77
52-Week Low (8/2/2016)	\$ 2.87
Market Cap. (MM)	\$ 36
Shares Out. (MM)	12

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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

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	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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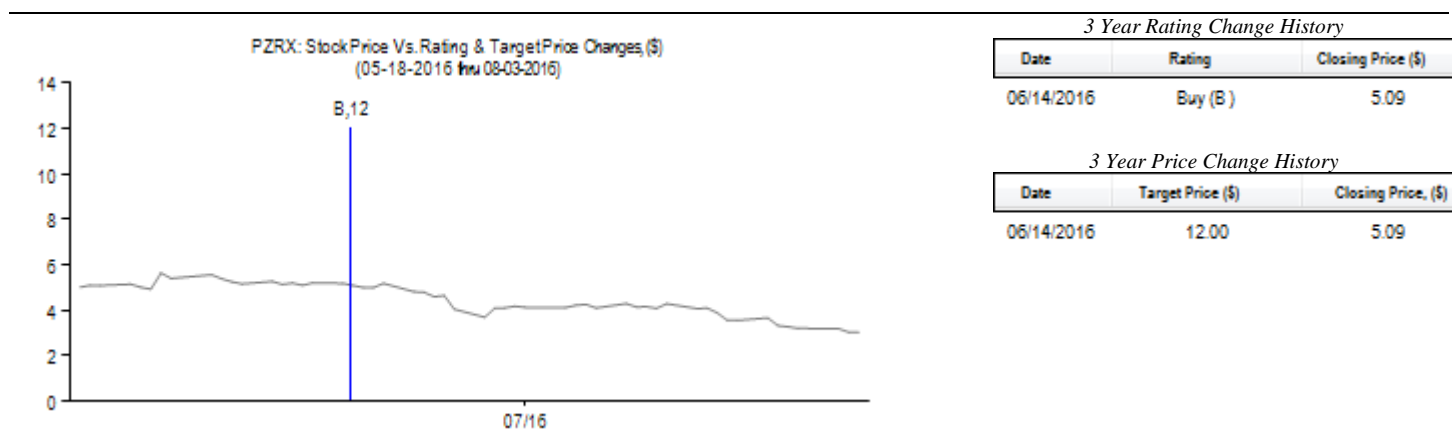
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