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EQUITY RESEARCH
Company Report

June 14, 2016

PhaseRx (PZRX - $ 5.18)

Healthcare/Biotechnology

First Publicly Traded mRNA Therapy Pure Play Potentially Provides Paradigm-Changing Therapeutic Programs

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

We are initiating coverage of PhaseRx with a Buy rating and 12-month price target of $12. By leveraging its novel hybrid mRNA technology, PZRX is developing several urea cycle disorders (UCDs) drugs as part of the novel intracellular enzyme replacement therapy (i-ERT).

- **A leading public mRNA therapy company affords liver-targeted novel treatment with large commercial potential.** PZRX develops proprietary and differentiated hybrid mRNA technology for liver-targeted i-ERT with several UCD therapies as lead products. Given the vast potential of i-ERT and promising PRX-OTC in OTCD POC preclinical data, we believe PZRX's upside could be substantial once they could successfully advance their clinical programs forward.
- **Initial product group could potentially treat >90% urea cycle disorders (UCD), while lead product, PRX-OTC exhibited promising preclinical POC data.** The presumptive lead product, PRX-OTC (as potential ornithine transcarbamylase deficiency treatment) has exhibited promising preclinical POC data with hyperammonemia reduction, prolonged survival and lack of innate immunity activation. Preclinical studies will continue and potential IND filing is scheduled in 4Q17 with two Phase II studies data available in 1H18 and 2H18 – an inflection point for PZRX shares, in our opinion. Successful PRX-OTC could potentially become a paradigm-changing disease-modifying OTCD treatment.
- **Proprietary hybrid mRNA technology well differentiated from peers.** By a two component system that separates transport from cell delivery, hybrid mRNA technology has the potential safety advantage of avoiding activation of innate immunity and hepatocyte-specific delivery. This therapy could be used chronically since it can be dosed repeatedly without compromised expression.
- **Messenger RNA therapy potentially offers a more versatile enzyme replacement therapy (ERT) platform.** i-ERT potentially provides greater benefits over conventional ERT mainly due to its ability for treating diseases if the therapeutic reaction occurs inside the cell and is inaccessible by conventional ERT. As such, i-ERT, if successful, could become a new paradigm or at least expand the treatment scope beyond the conventional ERT.
- **Material upside remains at the current valuation.** With a differentiated hybrid mRNA technology, promising PRX-OTC preclinical results and i-ERT's potential in multiple indications, we believe PZRX shares remain undervalued at current levels. Our 12-month $12 price target is based on peer comparable, probability adjusted DCF and sum-of-the-parts analyses.

Trading Data:

Last Price (06/13/2016)	$ 5.18
52–Week High (5/27/2016)	$ 5.77
52–Week Low (5/18/2016)	$ 4.54
Market Cap. (MM)	$ 60
Shares Out. (MM)	12

Earnings Estimates: ($ per share)

(Dec)	1Q	2Q	3Q	4Q	FY	P/E
FY-16E	-0.42A	-0.17	-0.18	-0.19	-0.96	N.A.
FY-15A	-0.23	0.00	0.00	0.00	-1.12	N.A.
FY-14A	NA	NA	NA	NA	-1.16	N.A.
FY-13A	NA	NA	NA	NA	NA	N.A.

Source: Laidlaw & Company estimates

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Investment Thesis

Our $12 price target is supported by peer comparable, probability adjusted DCF and sum-of-the-parts analyses.

- **We are initiating coverage of PhaseRx (PZRX) with a Buy rating and a 12-month price target of $12.** PhaseRx is an early-clinical stage biotechnology company that leverages its proprietary hybrid mRNA technology to develop multiple intracellular enzyme replacement therapies (i-ERT) with initial focus on treatments of three urea cycle disorders (UCDs) indications: ornithine transcarbamylase deficiency (OTCD), argininosuccinate lyase deficiency (ASLD) and argininosuccinate synthase 1 deficiency (ASS1D). The majority of the positive preclinical results to date are derived from studies of PRX-OTC. Given the vast potential of mRNA therapy for treating multiple diseases and PZRX's differentiated and potentially superior mRNA therapy technology, we believe success in advancing clinical developments of their lead programs could significantly increase PZRX share value. It is also worth mentioning that PhaseRx is the first publicly traded pure play of mRNA therapy.

PZRX's unique mRNA therapy platform, or hybrid mRNA technology, is equipped with a well differentiated dual transport/delivery approach.

Hybrid mRNA technology enables synthesizing proteins specifically in the liver and predominantly in the hepatocyte. As such, this approach represents an mRNA therapy that would be used for many therapies that the key therapeutic active site(s) is located in the liver.

- **Proprietary and differentiated hybrid mRNA technology enables PhaseRx to develop liver-focused intracellular enzyme replacement therapy (i-ERT).** PZRX's unique mRNA therapy platform, or hybrid mRNA technology, is equipped with a well differentiated dual transport/delivery approach: an inert (instead of a charged) lipid nanoparticle (LNP) provides protection during mRNA traveling through circulation, while polymer nanoparticle coupling with GalNAc (N-Acetylgalactosamine) delivers mRNA into the liver and into cytoplasm (so to escape the endosome compartment). As such, the hybrid mRNA technology could have the advantage of not eliciting innate immunity compared to other delivery approaches, given some of the latter have to incorporate immunity-stimulating fusogenic lipids for their transport/cell delivery combined capability. Hybrid mRNA technology enables synthesizing proteins specifically in the liver and predominantly in the hepatocyte (with no apparent expression in Kuppfer cells). As such, this approach represents an mRNA therapy that would be used for many therapies that the key therapeutic active site(s) is located in the liver. PZRX also has demonstrated from preclinical studies that mRNA delivered by hybrid mRNA technology could be dosed repeatedly without losing protein expression. This would be an important attribute for chronically used enzyme replacement therapy. The dosing frequency tested so far is on par or potentially better than the current standard weekly or twice weekly administered enzyme replacement therapies. From a strategic perspective, management indicated that a liver specific mRNA therapy could potentially apply to multiple inherited, single-gene disorders with therapeutic action sites in the liver with relatively low costs and mitigated development risks. The hybrid mRNA technology is protected by broad intellectual property estates with patent expiration extending into 2030 and beyond.

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The first therapeutic target of PZRX's intracellular enzyme replacement therapies (i-ERT) via hybrid mRNA technology are three major indications of urea cycle disorders (UCDs): OTCD, ASLD and ASS1D.

- **PRX-OTC and two other follow-on products, PRX-ASL and PRX-ASS1 could provide improved disease-modifying therapies with significant market potential.** The first therapeutic target of PZRX's intracellular enzyme replacement therapies (i-ERT) via hybrid mRNA technology are three major indications of urea cycle disorders (UCDs): ornithine transcarbamylase deficiency (OTCD), argininosuccinate lyase deficiency (ASLD) and argininosuccinate synthase 1 deficiency (ASS1D). UCDs are a collection of six rare inheritary disorders all due to the mutation-driven malfunction of different genes functioning in the urea cycle leading to hyperammonemia (accumulation of excess ammonina in circulation and tissues). Major symptoms include irritability, vomiting, and lethargy with potential brain damage, coma and death. Although it has not been formally nominated, we view PRX-OTC, which targets OTCD as a presumptive lead product. A majority of the positive preclinical proof of concept (POC) results were generated from studying this product. PZRX expects to formally declare its lead development candidate in 2Q16. In an OTC-*spf*^{ash} mouse model, PRX-OTC has demonstrated potential therapeutic benefits based on multiple criteria. For example, PRX-OTC treated mice demonstrated a statistically significant reduction of plasma ammonia accumulation and returned back to the normal wild-type level vs. control mRNA-treated mice in day 14 and 21. It is noted that a meaningful reduction in ammonia level is the clinical endpoint recognized by the FDA for approving UCDs treatments, such as Ravicti. Additional studies demonstrated that PRX-OTC could reduce the accumulation of orotic acid, an intermediate of urea cycle metabolism; and prolong survival compared to placebo mRNA treatment. PRX-OTC appears to be safe without the elevation of liver enzyme, alanine aminotransferase or ALT, and cytokine IP-10, a biomarker indicating stimulation of the innate immune system.

PRX-OTC has demonstrated potential therapeutic benefits in an OTC mouse model. For example, PRX-OTC could reduce plasma ammonia accumulation. It is noted that a meaningful reduction in ammonia level is the clinical endpoint recognized by the FDA for approving UCDs treatments. PRX-OTC also could prolong survival compared to placebo mRNA treatment. PRX-OTC appears to be safe without the elevation of liver enzyme, alanine aminotransferase or ALT, and cytokine IP-10, a biomarker for stimulation of the innate immune system.

Going forward, PZRX plans to conduct more preclinical studies before filing an IND in 4Q17. The additional works include a large animal tolerability study by year-end 2016 and completion of GMP manufacturing by 3Q17. The planned Phase I/II trials are comprised of two-stage studies for adults and pediatric patients who are currently on Ravicti therapy. The first is a single-dosing Phase IIa trial that will enroll a smaller number of adult patients for the evaluation of PK and PD (based on plasma ammonia levels). The subsequent Phase IIb repeat dosing trial will enroll a larger number of similar patients for a direct comparison of ammonia levels between Ravicti and PZRX's therapeutic candidate. An extension study is also scheduled for rolling over all patients for a one year treatment. PZRX is scheduled to report the Phase IIa and Phase IIb study results in 1H18 and 2H18, respectively – critical binary events for PZRX share value, in our opinion. Given the current standard of care of ammonia scavengers is more palliative in nature; a relatively disease-modifying treatment like PRX-OTC, if successful, could be paridigm changing. We estimate PRX-OTC could potentially reach market in 2022. PZRX also plans to report preclinical POC study results of a second disease model (PRX-ASL or PRX-ASS1) in 2Q16.

PZRX is scheduled to report the Phase IIa and Phase IIb study results in 1H18 and 2H18, respectively – critical binary events for PZRX share value, in our opinion.

- **Intracellular enzyme replacement therapy (i-ERT) could be a new treatment paradigm with large commercial potential.** Beyond UCDs, i-ERT powered by hybrid mRNA technology could potentially have

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I-ERT could have an advantage over the conventional protein-based ERT because i-ERT could synthesize protein directly inside the cell, and therefore, with greater versatility and accessibility for potentially treating more disorders.

utilities in treating multiple inherited, single-gene disorders of metabolism in the liver, such as organic acidemias, porphyria and glycogen storage diseases. i-ERT could have an advantage over the conventional protein-based ERT because i-ERT could synthesize proteins directly inside the cell, and therefore, with greater versatility and accessibility for potentially treating more disorders, while ERT would be effective only if the exogenously generated enzyme can be transported inside the cell or function through the cell surface (i.e. via receptor). i-ERT could also treat disorders with action sites not only in cytosol, but also in different organelles, such as mitochondria. As such, i-ERT, if successful, could become a new treatment paridigm or at least expand the treatment scope beyond the conventional ERT.

- **Valuation is favorable.** We believe PZRX shares are undervalued, 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. Accordingly, our $12 price target is supported by peer comparable, probability adjusted DCF and sum-of-the-parts analyses. We are recommending PZRX shares to long-term oriented investors with high risk tolerance.

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Company Description

PhaseRx is an early-clinical stage biotech company and first publicly traded mRNA therapy pure play that leverages its proprietary hybrid mRNA technology to develop intracellular enzyme replacement therapy (i-ERT) for treating inherited, single-gene disorders with the therapeutic action site located specifically in the liver. Hybrid mRNA technology is well differentiated from other mRNA therapies presumably due to its lack of activation of innate immunity and other positive attributes. PZRX initially targets therapies in urea cycle disorders (UCDs) indications with focus on ornithine transcarbamylase deficiency (OTCD), argininosuccinate lyase deficiency (ASLD) and argininosuccinate synthase 1 deficiency (ASS1D). PZRX has already demonstrated promising preclinical study results in PRX-OTC as a potential treatment for OTCD, and expects to further develop PRX- ASLD and PRX-ASS1D as the other two indications. PZRX plans to conduct more preclinical studies over the next several quarters and is scheduled to potentially file an IND in 4Q17, likely for PRX-OTC for Phase IIa and IIb studies. Top-line results for the two studies are expected in 1H18 and 2H18. In addition to UCDs, PZRX could also employ i-ERT potentially to treat other liver specific orphan indications.

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Anticipated milestones in 2016 and beyond

Product	Indication	Event	Timing	Importance
PRX-OTC	Ornithine transcarbamylase deficiency	Declare preclinical lead development candidate	**2Q16**	***
		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**	****
PRX-ASL	Argininosuccinate lyase deficiency	Potentially report POC in second disease model	**2Q16**	***
PRX-ASS1	Argininosuccinate synthase 1 deficiency	Potentially report POC in second disease model	**2Q16**	***

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

Source: Laidlaw & Company and company presentation.

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PhaseRx Pipeline

Product	Indication	Preclinical	I	II	III	Comments
PRX-OTC	Ornithine transcarbamylase deficiency (OTC)					Phase I/II study commencement expected in late '17
PRX-ASL	Argininosuccinate lyase deficiency (ASL)					Potentially report POC in second disease model in 2Q16
PRX-ASS1	Argininosuccinate synthase 1 deficiency (ASS1)					Potentially report POC in second disease model in 2Q16

Source: Laidlaw & Company and company presentation

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Presumptive Lead Product, PRX-OTC, Exhibits Promising Preclinical Therapeutic Activities Against OTC Deficiency with More UCD Therapeutic Products to Come

PRX-OTC potentially could become a disease-modifying therapy for OTCD with substantial benefits over current standard of care

PZRX employs its proprietary hybrid mRNA technology to develop mRNA therapies as a novel intracellular enzyme replacement therapy (i-ERT) initially focusing on therapies of urea cycle disorders (UCDs). The presumptive lead product, PRX-OTC, is potentially a disease-modifying treatment for ornithine transcarbamylase deficiency (OTCD). Although PRX-OTC is the most advanced product in its development pipeline, PZRX has not formally nominated it as the lead product. The company guided to formally declare its preclinical lead development candidate in 2Q16. Two other UCDs treatment products in earlier development stages are PRX-ASL and PRX-ASS1, which are potential treatments of argininosuccinate lyase deficiency (ASLD) and argininosuccinate synthase 1deficiency (ASS1D), respectively (Figure 1).

Figure 1: Three PZRX UCD products in development

	OTC	ASL	ASS1
PhaseRx Therapy	PRX-OTC	PRX-ASL	PRX-ASS1
Current Therapy	Liver transplantation	Liver transplantation	Liver transplantation
Drug therapy	Ammonia scavengers w/protein-restricted diet – patients still have crises	Ammonia scavengers w/protein-restricted diet – patients still have crises	Ammonia scavengers w/protein-restricted diet – patients still have crises
PhaseRx Drug	mRNA Synthesized	mRNA Synthesized	mRNA Synthesized
Animal Model	Established	In-house	In-house
Preclinical POC	Established	Pending	Pending

Source: Company presentation

PRX-OTC treated mice demonstrated a statistically significant reduction of plasma ammonia accumulation and returned back to the normal wild-type level (~100 μM) compared to mice treated by control mRNA in day 14 and 21.

PRX-OTC. PZRX has established a proof of concept (POC) preclinical study in a mouse model and demonstrated potential therapeutic benefit of PRX-OTC based on multiple criteria. The system used is the OTC-*spf*^{ash} mouse, which carries a defective OTC gene, and after manipulation by researchers (by gene knock down) to further eliminate OTC enzyme activities, can recapitulate the symptoms, such as hyperammonemia of an OTCD patient. In the study, PRX-OTC treated mice demonstrated a statistically significant reduction of plasma

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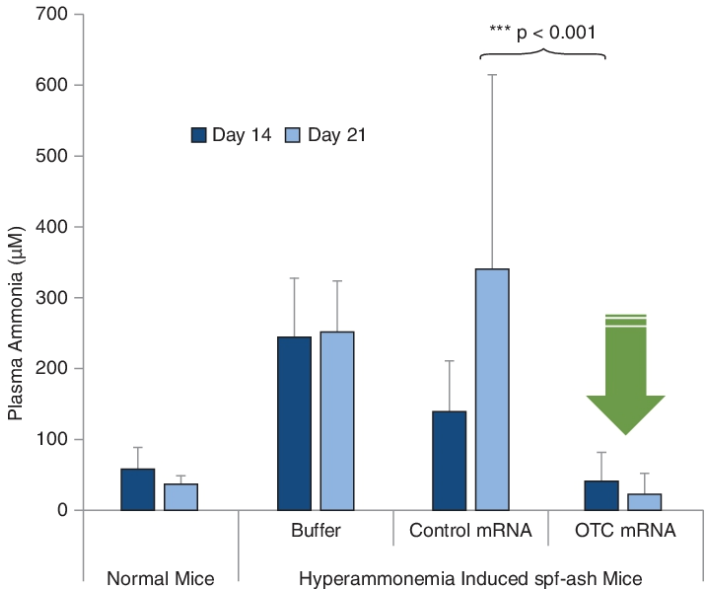
ammonia accumulation and returned back to the normal wild-type level (~100 μM) by compared to mice treated by control mRNA in day 14 and 21 (Figure 2). Mice were treated by PRX-OTC 3 mg/kg twice a week over a three-week period. An additional value of this result is that a meaningful reduction in ammonia level is the clinical endpoint recognized by the FDA for approving UCDs treatments, such as Ravicti from Hyperion (acquired by Horizon Pharma in 1Q15).

Figure 2: PRX-OTC demonstrates ammonia reduction in OTC-spf^{ash} mouse model



Source: Company presentation

To further confirm the reduction of plasma ammonia accumulation results, the researchers examined the pattern of urinary orotic acid under different conditions. Orotic acid is an intermediate in the pyrimidine biosynthetic pathway, and its level is markedly increased in patients with urea cycle and other arginine metabolism disorders. The study results indicated that the orotic acid levels were maintained similar to that in normal mice, while the level increased in buffer or the negative control mRNA-treated mice (Figure 3). In the figure, the majority of negative control mRNA-treated mice were demised after day 12.

The preclinical study also showed that only PRX-OTC-treated mice survived much longer while buffer or control mRNA-treated counterparts did not live beyond days 21 and 27, respectively.

Given fatality is one of the consequences of OTCD, especially in more severe forms of the disease, *OTC-spf^{ash}* mice after hyperammonemia induction also exhibited symptoms of ataxia, significant body weight loss and ultimately death. The preclinical study also demonstrated that only PRX-OTC-treated mice survival was much longer while buffer or control mRNA-treated counterparts did not live beyond days 21 and 27, respectively (Figure 4).

PRX-OTC's major potential benefits over current ammonia scavenger therapies are that patients might not suffer from continued risks of incurring hyperammonemic episodes given the production of ammonia potentially could be controlled within the normal level among each treatment cycle. Additionally,

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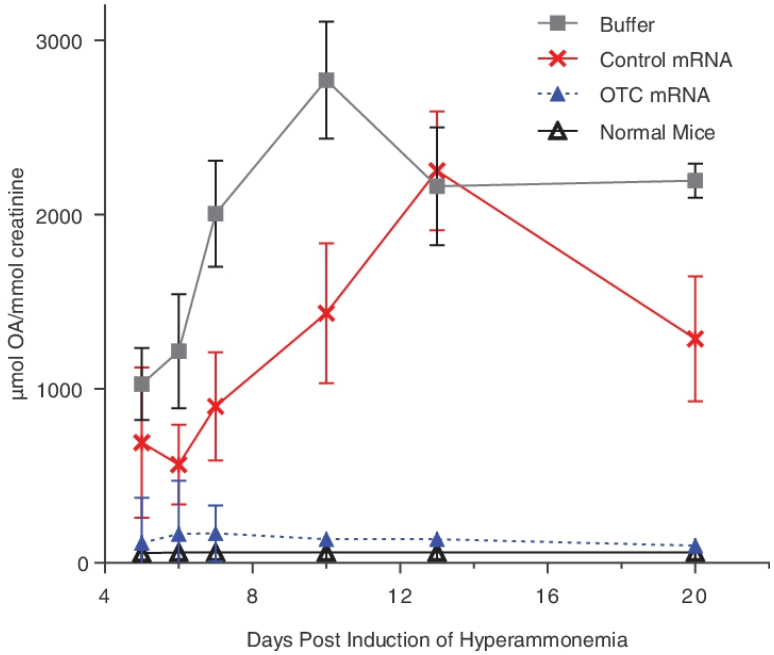
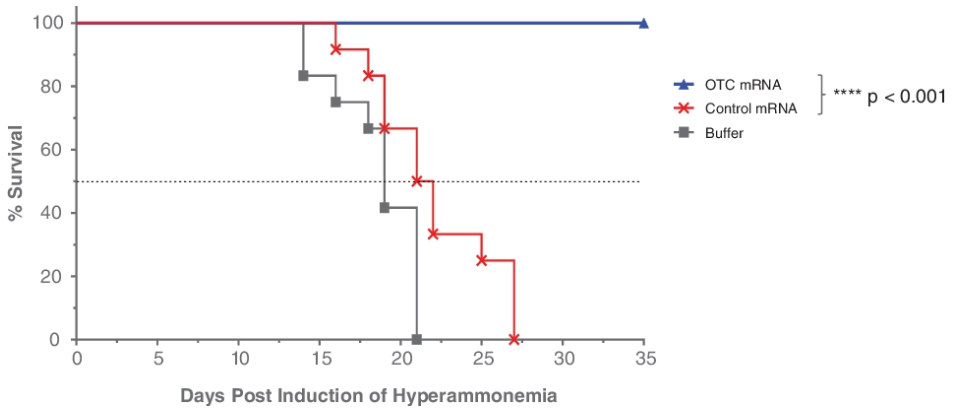
the patient's quality of life could potentially improve substantially and patients could enjoy a more normal life by participating in many regular activities; and might not need or could at least reduce the intensity of restricted diets.

Figure 3: PRX-OTC demonstrates orotic acid level normalization preclinically



Source: Company presentation

Figure 4: PRX-OTC demonstrates complete survival benefits preclinically



Source: Company presentation

PRX-OTC is safe from the animal model study as the drug did not elevate plasma alanine aminotransferase (ALT) levels – an indicator of liver damage.

PZRX also demonstrated PRX-OTC is safe from the animal model study as the drug did not elevate plasma alanine aminotransferase (ALT) levels – an indicator of liver damage – compared to mice treated with buffer or without mRNA 48 hours following dosing (Figure 5, left). In addition, cytokine IP-10 (its elevation suggests a stimulation of the innate immune system) levels were undetectable three hours following dosing under various conditions (Figure 5, right). BLOQ stands for below limit of quantitation.

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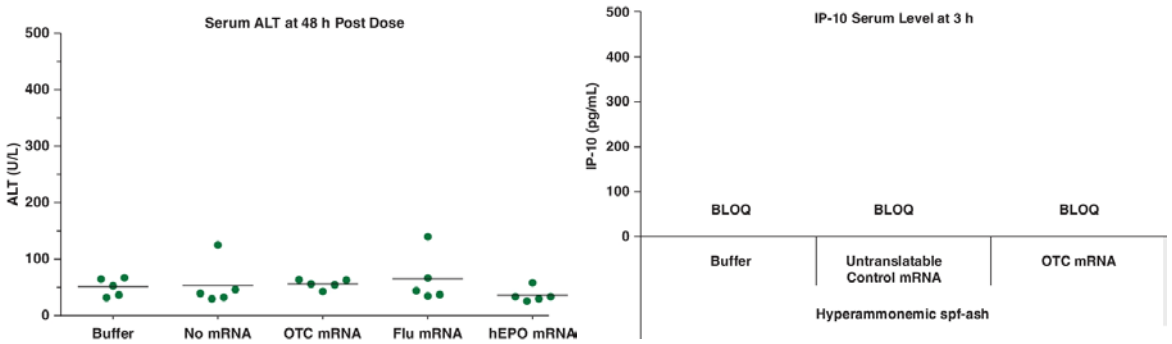
Figure 5: Well tolerated PRX-OTC without ALT elevation (left) and IP-10 changes (right)



Source: Company presentation

Our take *Together, we are encouraged by the positive PRX-OTC in OTCD preclinical results since the drug exhibited activities in multiple fronts for treating this disorder. We believe the mouse model could rather faithfully recapitulate the etiology and symptoms of the human disorder, and the successful reduction of ammonia accumulation is similar to the clinical endpoints that the FDA already used to approve UCDs drugs. The mouse model can survive relying on only 5% of the normal level of ornithine transcarbamylase activities. This is why it needs to reduce more OTC enzyme to create hyperammonemia. Our discussions with management suggested that OTCD patients could enjoy a relatively normal life if their OTC enzyme could increase to the 15 – 20% level (instead of much higher percentages) of a healthy individual. Based on preclinical studies, PRX-OTC has restored OTC activities at the level that was considered as "sufficient" and potentially beyond. As such, the clinical bar for success, in our opinion, could be lower and is in favor for PZRX's treatments.*

Over the next five to six quarters, PZRX is expected to conduct more preclinical studies, including a large animal tolerability study by year-end 2016, completion of GMP manufacturing by 3Q17, and, if successful, file an IND in 4Q17 for starting Phase I/II studies shortly thereafter.

Next Step PZRX anticipates it will formally declare the lead development candidate in 2Q16, and we estimate it could be PRX-OTC in OTCD. Over the next five to six quarters, PZRX is expected to conduct more preclinical studies, including a large animal tolerability study, such as in non-human primate, by year-end 2016, completion of GMP manufacturing by 3Q17, and, if successful, an IND filing in 4Q17 for starting Phase I/II studies shortly thereafter. PZRX plans to develop PRX-OTC therapy as a weekly or even biweekly regimen for patients greater than two years of age.

PZRX is scheduled to report the Phase IIa study results in 1H18 regarding safety and clinical efficacy, and Phase IIb study results in 2H18 – events we view could be inflection points for PZRX shares.

PZRX's initial clinical study is a two-stage trial for adults and pediatric patients who are currently on Ravicti therapy. The first stage single-dosing Phase IIa trial will enroll a smaller number of adult patients for the evaluation of PK and PD (based on plasma ammonia levels). The subsequent Phase IIb repeat dosing trial will enroll a larger number of adults and pediatric patients for a direct comparison of ammonia levels between Ravicti and PZRX's therapeutic candidate. An extension study is scheduled for rolling over all patients for one year treatment. Accordingly, PZRX is scheduled to report the Phase IIa study results in 1H18, and Phase IIb study results in 2H18 regarding safety and clinical efficacy – events we view could be inflection points for PZRX shares.

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Other products in development

PRX-ASL ASLD affects the body's ability to clear the nitrogen already incorporated into the urea cycle as citrulline and argininosuccinate. PRX-ASL development is at an earlier stage as the design and manufacturing of the ASL mRNA is completed, while ASLD animal disease model work is underway. Upcoming tasks include examining ASL mRNA production in normal mice; and administering PRX-ASL into ASL hypomorphic mice suffering from a genetic mutation to examine protein production in liver and reduction of argininosuccinic acid levels in plasma for ASL deficiency.

PRX-ASS1 ASS1D is a deficiency on argininosuccinate synthase 1 enzyme, which is responsible for combining two amino acids, citrulline made by other enzymes in the urea cycle, and aspartate, to form a molecule called argininosuccinic acid. PZRX has completed design and manufacture of ASS1 mRNA and obtained ASS1-deficient mice, while ASS1D animal disease model work is underway. Upcoming tasks include examining ASS1 mRNA production in normal mice, and administration of PRX-ASS1 into ASS1-deficient mice to examine protein production in the liver, reduction of citrulline levels in plasma and presence of argininosuccinic acid levels in plasma.

Next step PZRX is scheduled to report preclinical POC study results of a second disease model, which would be either PRX-ASL or PRX-ASS1.

PZRX is scheduled to report POC study results of a second disease model, which would be either PRX-ASL or PRX-ASS1.

Urea cycle disorders (UCDs). UCDs are a group of hereditary rare disorders due to the deficiencies of enzymes or transporters that are essential for the synthesis of urea from ammonia (NH3 or $NH4^{+}$). UCD patients suffer from the accumulation of toxic levels of ammonia in the blood and brain due to the absence of these enzymes or transporters, resulting in symptoms including irritability, vomiting, and lethargy with potential brain damage, coma and death. The urea cycle is a series of biochemical reactions that remove the toxic byproducts, ammonia (NH_3) and converts it to urea ($(NH_2)_2CO$) (Figure 6). The reactions take place mainly in the liver and to a lesser extent in the kidney.

UCDs are a collection of six different disorders due to the deficiency of a urea cycle enzyme or co-factor. They are:

- Arginase deficiency (ARGD);
- Argininosuccinate lyase deficiency (ASLD);
- Argininosuccinate synthetase deficiency (ASSD);
- Carbamyl phosphate synthetase deficiency (CPSD);
- N-acetylglutamate synthase deficiency (NAGSD), and
- Ornithine transcarbamylase deficiency (OTCD).

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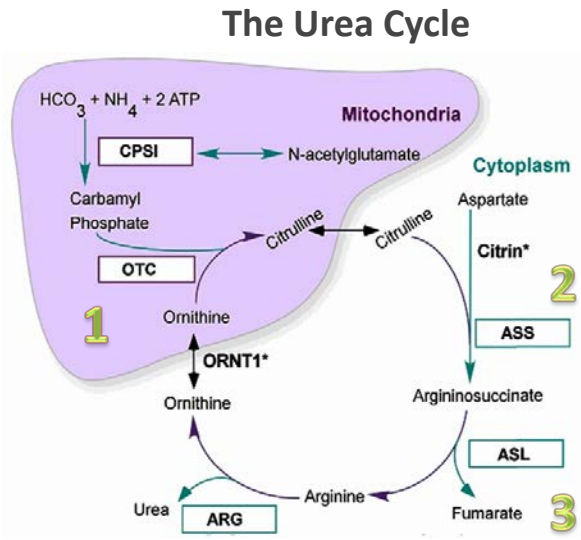
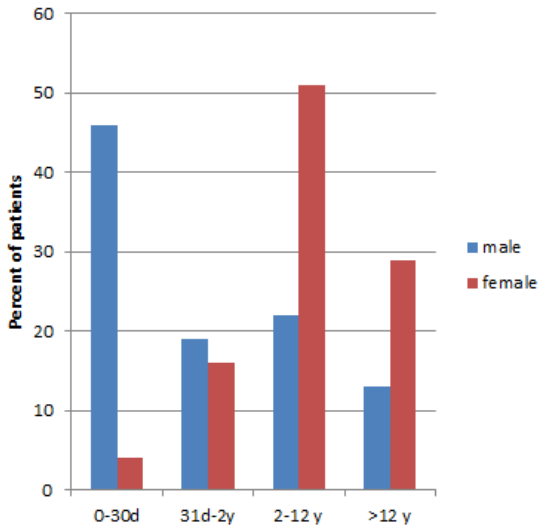
Figure 6: Urea cycle and three PZRX targeted indications



Source: Company presentation

Ornithine transcarbamylase deficiency (OTCD). It is a rare X-linked genetic disorder with complete or partial lack of the OTC enzyme. The severe form of the disorder affects near 25% male OTCD infants shortly after birth (24 hours to a few days), while the milder form (70%) affects both males and females during infancy. If left untreated, severe OTCD patients could fall into coma and potentially develop neurological abnormalities such as intellectual disability, developmental delays, and cerebral palsy. Approximately 45% of male patients have been diagnosed within a month after birth; while a majority of patients of both sexes are diagnosed before age 12 with the presences of hyperammonemic crises (Figure 7). The estimated incidences of OTCD are one in 56,300 live births or ~170 incidences per year in the U.S. and EU combined.

Figure 7: Age at first OTCD diagnosis



Source: Company presentation

Argininosuccinate lyase deficiency (ASLD). ASLD is an autosomal recessive hereditary disorder and affects both sexes equally. The estimated incidences of ASLD are one in between 70,000 and 218,750 live births or ~45 – 130 incidences per year in the U.S. and EU combined.

Argininosuccinate synthase 1 deficiency (ASS1D). The ASS1 enzyme is responsible for the third step of the urea cycle, which combines citrulline and aspartate to form argininosuccinic acid. The estimated incidences of ASS1D are one in 250,000 live births or <40 incidences per year in the U.S. and EU combined.

Summary of UCDs. Patients with early-onset variants, such as in some cases of OTCD, ASSD and CPSD typically have severe disease presentations and a poor prognosis. Patients are seemingly healthy at birth but symptoms, like cerebral edema, lethargy, seizures, and coma, could appear shortly after the intake of protein and trigger severe accumulation of ammonia in the blood. For patients with later-onset of symptoms in childhood, the severity could potentially be milder since patients may still retain low levels of enzyme activities. However, these patients could suffer from rapid plasma ammonia elevation crisis potentially leading to hallucinations, sleep disorders, delusions and vomiting, brain damage and coma. Clinically, a plasma ammonia concentration of $\geq$150 μmol/L is considered for the presence of a UCD. In addition to drug treatment, all UCDs patients need to follow/maintain restricted diets in order to control protein intake to avoid the development of excess ammonia.

The consensus on the U.S. prevalence is less consistent with the range estimated from ~700 to 3,400 and even much higher (6,000) given the difficulty of tracking all patients partially due to the condition being under-diagnosed.

UCDs are rare in-born error orphan indications and the incidence rate of each individual indication differs (Figure 8). The annual incidences in the U.S. are estimated at 115 with OTCD accounting for 62% (n~71). The consensus on the U.S. prevalence figure is less consistent with the range estimated from ~700 to 3,400 and even much higher (6,000) given the difficulty tracking all patients partially due to the condition being under-diagnosed. For example, only about half of OTCD patients have been diagnosed, according to the management of Hyperion Therapeutics. Despite clinical onset often being rapid and devastating in a patient who is genetically affected, one reason for the difficulty in discerning the true prevalence is that the late-onset cases (even older than 40s) may go undetected. Based on an assumption of a similar incidence rate in Europe[1], we estimate that the annual incidences for UCDs in the European Union are ~146. Based on the epidemiological data, we believe the three indications that PZRX initially plans to target could account for **>90%** of UCD patients.

Based on the epidemiological data, we believe the three indications that PZRX initially plan to target could account for >90% of UCDs patients.

Ammonia scavengers are the current standard therapy for UCDs. Given the major causes of UCDs are defective genes; one likely treatment that could correct the defect is liver transplant to restore the missing enzyme or co-factor. Since organ availability is in short supply and therefore limits liver transplants, the most common current treatment is to use various ammonia scavenging

[1] *Summar, M., et. al., Mol Genet Metab. 2013 ; 110: 179–180*

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agents to remove excess plasma ammonia to prevent hyperammonemia as a chronic management for different UCDs. If patients suffer from acute severe hyperammonemia, treatments include dialysis, hemofiltration, arginine hydrochloride via IV and ammonia scavenging agents.

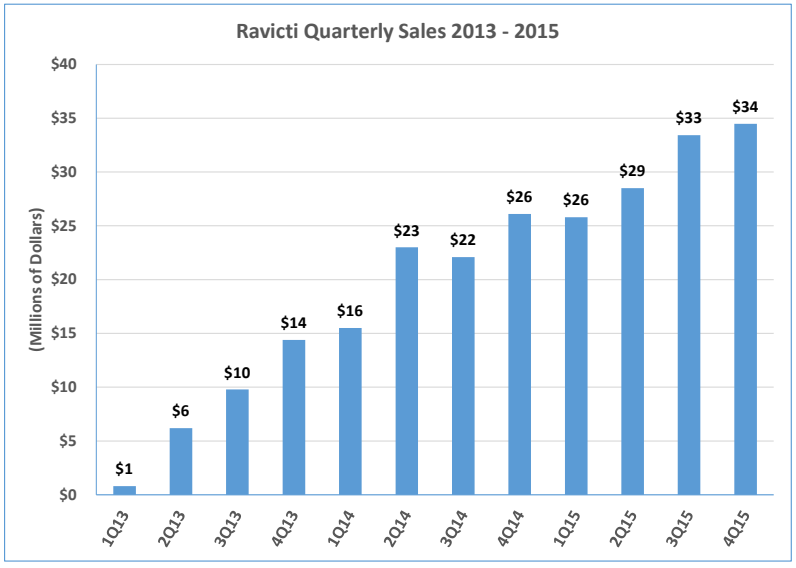
Figure 8: Estimated incidences of UCDs in the U.S. and European Union

Indication	Factor missed	Est. incidence rate	Incidences (US)	Incidences (EU)	Incidences (US+EU)
Urea Cycle Disorders		1:35,000	114	146	260
NAGS deficiency	N-acetyl glutamate synthetase (NAGS)	<1 :2,000,000	2	3	5
CPS1 deficiency	Carbamoylphosphate synthetase I (CPS1)	1:1,300,000	3	4	7
OTC deficiency	Ornithine transcarbamylase (OTC)	1:56,500	**71**	**90**	**161**
ASS1 deficiency	Argininosuccinic acid synthetase (ASS1)	1:250,000	**16**	**20**	**36**
ASL deficiency	Argininosuccinic acid lyase (ASL)	1:218,750	**18**	**23**	**42**
ARG deficiency	Arginase (ARG)	1:950,000	4	5	10

Source: Laidlaw & Company estimates

Three major ammonia scavenging agents have been approved in the U.S. since 3Q 1996: initially with oral Buphenyl (sodium phenylbutyrate), followed by Ammonul (an intravenous sodium phenylbutyrate by Ucyclyd Pharma) in 1Q05 as rescue medication for hyperammonemia crisis. The most recent entrant is Ravicti (glycerol phenylbutyrate) currently marketed by Horizon Pharma. Ravicti was approved in 1Q13 with estimated 2015 sales of ~$120+MM and estimated costs of $420K per patient per year (Figure 9). We estimate currently there are ~300+ UCD patients being treated by Ravicti. A carbamoyl phosphate synthetase 1 (CPS 1) activator, Carbaglu (carglumic acid) is indicated as adjunctive and maintenance therapy for the treatment of hyperammonemia due to one of the smaller UCDs, N-acetylglutamate synthase deficiency (NAGSD). Carbaglu was approved in the U.S. in 2010 and sold by Recordati Pharmaceuticals. Carbaglu costs ~$167K/month or ~$2MM/year, in our estimate.

Figure 9: Ravicti quarterly sales from 2013 to 2015



Source: Laidlaw & Company estimates and SEC reporting

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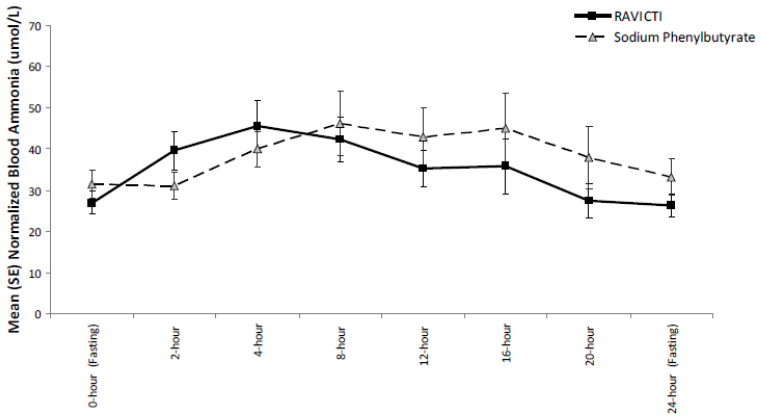
Shortcoming of ammonia scavenging agents. The fundamental value proposition of the ammonia scavenger is to remove excess ammonia in circulation to prevent hyperammonemia. It does not have any disease-modifying attribute. Patients continuously generate ammonia and likely remain at risk of incurring hyperammonemic crisis. As such, patients still cannot really enjoy a relatively "normal" life or participate in many regular activities. For example, ~25% (12/49) of patients experienced hyperammonemic crisis during the one year period during a clinical study[2]. Although Ravicti provides a significant improvement for infrequent dosing and reduction of sodium intake compared to Buphenyl, three times a day administration could still leave room for improvement. In our opinion, a successful disease-modifying therapy in UCDs could afford patient benefits and have commercial outlook analogous to the development of an effective prophylactic, such as Cinryze in hereditary angioedema (HAE) patients. Cinryze enables many patients to conduct and plan multiple activities (such as travelling out long distance and attending social functions) that were prohibitive before.

Ravicti is approved for chronic management of adult and pediatric patients ≥2 years of age with urea cycle disorders (UCDs). The pivotal trial met its primary endpoint of noninferiority to that of sodium phenylbutyrate (Buphenyl) with respect to the 24-hour AUC for venous ammonia on days 14 and 28 (Figure 10).

Figure 10: Ravicti pivotal trial primary endpoint results



Source: Laidlaw & Company estimates and SEC reporting

Competitors. On the competitive side, more than a handful of developments are underway and all are in early stages of development. The technologies employed for therapy cover mRNA therapy, gene therapy, cell therapy and other modalities (Figure 11). Among them, gene therapy and cell therapy also could potentially be a disease-modifying treatment with a long term "cure" prospect if it can provide sufficient and sustainable therapeutic benefits. Some concerns regarding gene therapy are that immune response against viral vectors used for

[2] *Berry, S. A., et. al., 2014, Mol. Genet. Metab. 112:17 - 24*

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delivering genes could diminish the therapeutic effect if patients require repeat administration.

- **SHP637** Shire is developing SHP637 as a potential treatment for UCDs. It is an mRNA therapy and the company did not reveal the specific indication that SHP637 will target. SHP637 is in the preclinical stage. Shire also has a collaboration with Ethris using mRNA replacement therapy to develop MRT ASS1 as a potential treatment for citrullinemia, which is caused by mutations of the argininosuccinate synthetase or ASS1 gene. A preclinical study exhibited ~50% - 100% restoration of liver ASS1 activities and ammonia reduction. The current status of MRT ASS1 is unclear.
- **HepaStem UCD** Promethera Biosciences has developed heterologous human adult liver-derived progenitor cells (HHALPC) called HepaStem as a potential treatment for UCDs. The program is currently undergoing a Phase IIb trial. Promethera recently acquired the key assets from another liver cell therapy company called Cytonet GmbH & Co KG with its Heparesc as the most relevant asset for potential UCD treatment.
- **LUNAR-OTC** Arcturus Therapeutics is developing LUNAR-OTC as a potential OTC treatment; the program is in the preclinical stage. It is an mRNA therapy to be delivered via the company's proprietary lipid-enabled delivery system called LUNAR or Lipid-enabled and Unlocked Nucleic Acid modified RNA delivery technology.
- **AEB1102** Aeglea Biotherapeutics is developing AEB1102, a modified and PEGylated human arginase I as a potential arginase I deficiency treatment. Arginase I deficiency is a UCD with an incidence of 1:350,000 to 1:1,000,000 live births. The company has filed an IND and expects to start Phase I trials in the U.S. in 1H16 and in Europe in 1H17. AEB1102 is also being studied for solid and hematological cancer treatments with a Phase I in solid tumor study ongoing.
- **BB-OTC** Bio Blast Pharma is exploring the development of BB-OTC as a potential treatment for OTCD. It is in the preclinical stage. BB-OTC belongs to the company's mitochondrial protein replacement therapy (mPRT) platform. mPRT is a proprietary mitochondrial fusion protein comprised of a trans-activator of transcription peptide (TAT), a heterologous mitochondrial targeting sequence peptide (MTS), and a fully functional targeted protein. The technology affords an exogenously produced fusion protein to be delivered to, and function within mitochondria.
- **SYNB1010** Synlogic develops a novel class of therapeutics based on its proprietary synthetic biology and microbiome (Synthetic Biotic) technology to augment the innate activities of the microbiome to potentially realize therapeutic effects. Treatments for UCDs and phenylketonuria (PKU) are two leading preclinical programs of its rare inborn errors of metabolism (IEM) platform.

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- **DTX301** Dimension Therapeutics utilizes adeno-associated virus (AAV)-based (mainly of AAV8 and AAVrh10) vectors for developing liver selective gene therapies. DTX301 is the company's product targeting OTCD treatment. Dimension is scheduled to file an IND and start a Phase I study for DTX301 in OTCD in 2H16.
- **OCR-002** Ocera Therapeutics is developing an IV ammonia scavenger, OCR-002 (ornithine phenylacetate) as potential treatment for hepatic encephalopathy. It is currently in Phase II (STOP-HE) development with top-line results potentially available in 2017.

Figure 11: Selected clinical developments of UCD and liver-associated disorder therapies

Product	Developer	Ticker	Indication	Stage	Target	Type	Comments
HepaStem UCD	Promethera Biosciences		Urea Cycle Disorders (OTCD)	Phase IIb		Cell therapy	
SHP637	Shire	SHPG	Urea Cycle Disorders (OTCD)	Preclinical		mRNA therapy	
AEB1102	Aeglea Biotherapeutics	AGLE	Urea Cycle Disorders (AD)	Preclinical	Arginine	Biologics (ERT)	Effective IND
BB-OTC	Bio Blast Pharma	ORPN	Urea Cycle Disorders (OTCD)	Preclinical		Mitochondrial protein replacement therapy	
SYNB1010	Synlogic Therapeutics		Urea Cycle Disorders (OTCD)	Preclinical	Ammonia	Ammonia Scavenger	
LUNAR-OTC	Arcturus Therapeutics		Urea Cycle Disorders (OTCD)	Preclinical		mRNA therapy	
DTX301	Dimension Therapeutics	DMTX	Urea Cycle Disorders (OTCD)	Preclinical		Gene therapy	P1 start 2H16
OCR-002	Ocera Therapeutics	OCRX	Acute hepatic encephalopathy	Phase II	Ammonia	Ammonia Scavenger	
ALXN1540	Alexion Pharma/Moderna	ALXN	Crigler-Najjar Syndrome (CN-1)	Preclinical	UGT1A1	mRNA therapy	P1 start 2016

Source: Laidlaw & Company equity research

We estimate PRX-OTC could potentially reach the market in 2022, with total annual peak sales for PRX-OTC reaching $850+MM by 2030.

PRX-OTC in OTCD revenue model. Our model (Figure 12) projects that PRX-OTC could potentially reach the market in 2022 assuming a pivotal trial is needed after the outcomes of the Phase IIa and IIb trials started in late 2017 or early 2018 are positive. Assuming an annual course of therapy costs of $450k (which is conservative given the current annual costs of Ravicti is ~$420K and the treatment is more palliative) and a conservative assumption of prevalence of ~2,500; we estimate total annual peak sales for PRX-OTC could reach $850+MM by 2030.

Figure 12: PRX-OTC in OTCD revenue model

PRX-OTC in Ornithine Transcarbamylase Deficiency (OTCD) Revenue Model	2021	2022	2023	2024	2025	2026	2027	2028	2029	2030
Total U.S. OTCD prevalence	2,737	2,772	2,808	2,845	2,882	2,919	2,957	2,996	3,034	3,074
% of older than one year	75%	75%	75%	75%	75%	75%	75%	75%	75%	75%
OTCD patient eligible for PRX-OTC therapy	2,052	2,079	2,106	2,134	2,161	2,189	2,218	2,247	2,276	2,305
% receive PRX-OTC treatment		5%	10%	17%	24%	30%	33%	35%	37%	38%
OTCD patients receive PRX-OTC therapy		104	211	363	519	657	732	786	842	876
PRX-OTC annual treatment costs ($)		450,000	463,500	477,405	491,727	506,479	521,673	537,324	553,443	570,047
U.S.PRX-OTC in OTCD sales ($MM)		**47**	**98**	**173**	**255**	**333**	**382**	**423**	**466**	**499**
Total U.S. OTCD prevalence	3,010	3,049	3,089	3,129	3,170	3,211	3,253	3,295	3,338	3,381
% of older than one year	75%	75%	75%	75%	75%	75%	75%	75%	75%	75%
OTCD patient eligible for PRX-OTC therapy	2,258	2,287	2,317	2,347	2,377	2,408	2,440	2,471	2,503	2,536
% receive PRX-OTC treatment			5%	10%	17%	24%	30%	32%	33%	33%
OTCD patients receive PRX-OTC therapy			116	235	404	578	732	791	826	837
PRX-OTC annual treatment costs ($)			370,800	381,924	393,382	405,183	417,339	429,859	442,755	456,037
EU PRX-OTC in OTCD sales ($MM)			**43**	**90**	**159**	**234**	**305**	**340**	**366**	**382**
Global PRX-OTC in OTCD sales ($MM)		**47**	**141**	**263**	**414**	**567**	**687**	**762**	**832**	**881**

Source: Laidlaw & Company estimates

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Hybrid mRNA Technology Could Be One of Best-in-Class mRNA Therapy Platforms with Distinct Advantages

mRNA therapy is an emerging treatment modality and Hybrid mRNA technology is well differentiated from peers

Messenger RNA (mRNA) is the central element that transfers genetic information (DNA of genome via transcription) to the molecules (protein via translation) that execute the biochemical and physiological functionalities (enzyme and other factors). The concept for exploring mRNA as a key therapeutic means for disease treatment started in early 1990, and the realization of this approach is a much more recent phenomenon after several technological advancements.

Major challenges for using mRNA as a direct therapeutic are the drug needs to be delivered (especially systemically) to the site of action (mainly in cytosol of a cell) with minimal damage (both quantitatively and qualitatively).

Major challenges of using mRNA as a direct therapeutic are the drug needs to be delivered (especially systemically) to the site of action (mainly in the cytosol of a cell) with minimal damage (both quantitatively and qualitatively). This is due to the fact that mRNA is highly vulnerable from the degradation by ubiquitously presented nucleases. In addition to mRNA therapy, to deliver RNA safely and effectively is also a critical requirement for other RNA-based therapeutics namely small-interfering RNA (siRNA) and micro-RNA (miRNA). This already has a slightly longer development history. Xue, H.Y. and colleagues have reported that RNA-based therapeutics, especially siRNA has been delivered via several types of materials, including polymers, lipids and inorganic nanomaterials[3]. Among them, the lipid-based lipid nanoparticle (LNP) system so far is considered to have made great progress in clinical development.

LNPs are composed of three to four lipid components: 1) a cationic lipid that contains a cationic head group, a lipophilic tail group, and a connecting linker; 2) a PEGylated lipid; 3) cholesterol; and 4) a helper lipid.

LNPs are solid core, electron-dense nano-carriers with a size $\geq$ 120 nm, and they usually are composed of three to four lipid components: 1) a cationic lipid that contains a cationic head group, a lipophilic tail group, and a connecting linker; 2) a PEGylated lipid; 3) cholesterol; and 4) a helper lipid (Figure 13). From the functionality prospective, cationic lipids could provide the benefits of better interaction with the targeted cells due to the interaction with negatively charged cell surface. PEG could extend LNPs' circulation time in plasma. The addition of certain neutral lipids, so called fusogenic lipids like l-alpha dioleoyl phosphatidyl ethanolamine, could often promote endosomal escape.

The critical mission for RNA delivery to accomplish is to avoid degradation during blood circulation, enter the target cells efficiently by endocytosis, and escape the endosomal compartment and enter the cytoplasm. One issue is that the large and highly charged molecules typically are taken up into a cellular

[3] *Xue, H.Y., et. al., Nanomedicine, 2014, 9:295-312*

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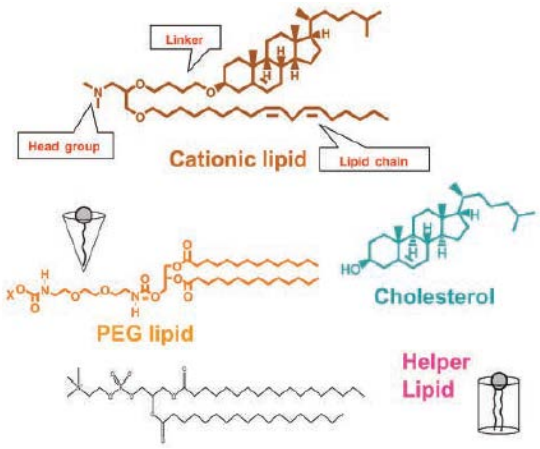
organelle called endosomes, and are subsequently sorted out to the lysosome for degradation or recycled back to the plasma membrane. Given the fact that most molecules enter the endosome are destined for either of the two paths, they are generally unable to cross the endosomal membrane to enter the cytoplasm of the cell. Therefore, it would be critical for a delivery system to avoid such an endosome entrapment.

Figure 13: General compositions of a lipid nanoparticle (LNP)



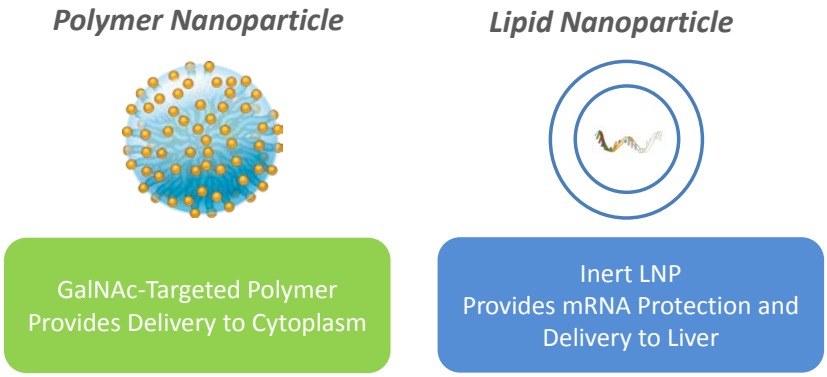
Source: Shi, B., et. al., J Histochemistry & Cytochemistry 2013, 6: 407–420

Although LNPs have been relatively effective, they also have certain associated toxicities that could cause untoward impact clinically. For example, fusogenic lipids could activate the innate immune system and result in dose-limiting toxicities.

PZRX's LNPs do not include fusogenic lipids and it could have potential benefits over other LNP systems given it could be better tolerated than competitors' technology.

Hybrid mRNA technology PhaseRx has developed a proprietary hybrid mRNA technology for its polymer-LNP-based formulation with two components: an inert (instead of a charged) lipid nanoparticle (LNP) that provides protection but without the functionality of intracellular delivery of mRNA; while polymer nanoparticle facilitates mRNA delivery into the liver and into the cytoplasm (Figure 14).

Figure 14: Hybrid mRNA Technology Platform



Source: Company presentation

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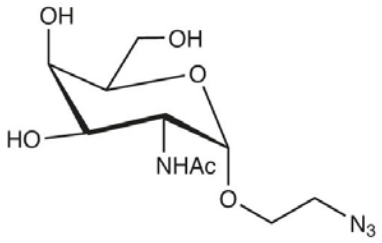
PZRX's LNPs do not include fusogenic lipids and it could have potential benefits over other LNP systems given it could be better tolerated than competitors' technology. The LNP system encapsulates and protects mRNA passing through the blood stream before being taken up into the hepatocytes. PZRX's polymer nanoparticle employs its SMARTT Polymer Technology, which is comprised of a diblock vinyl polymer of two blocks of monomers with distinct delivery functionalities. GalNAc (N-Acetylgalactosamine), an amino sugar, is needed for intercellular communication mainly found in sensory nerve structures (Figure 15). The polymer is decorated with GalNAc moiety in order to target to the asialoglycoprotein receptor on liver hepatocytes. The polymer is comprised of hydrophilic block for imparting water solubility and pH-tunable hydrophobic block that could mediate endosome escape. Polymers can be self-assembled into nanoparticles.

Figure 15: The structure of N-Acetylgalactosamine (GalNAc)



Source: http://www.iris-biotech.de/

PZRX has synthesized proteins specifically in the liver and predominantly in the hepatocyte (with no apparent expression in Kuppfer cells) via its hybrid mRNA technology.

PZRX's polymer is manufactured via the RAFT (reversible addition-fragmentation chain transfer) polymerization process, and it can be scaled up to the hundred kg scale. RAFT polymerization is one of the controlled radical polymerization techniques that utilizes a chain transfer agent, such as thiocarbonylthio compound, to mediate the polymerization via a reversible chain-transfer process. RAFT polymerization is a very versatile method that can be performed under wide ranges of conditions and affords end products of pre-chosen molecular weight or of low dispersity (molecular weight distribution).

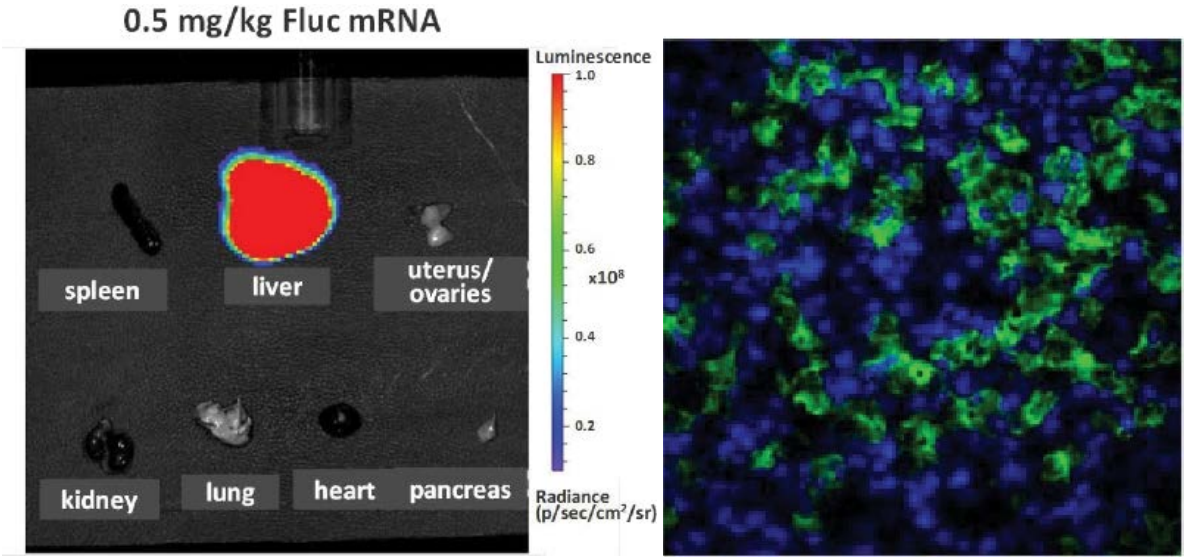
PZRX has synthesized proteins specifically in the liver and predominantly in the hepatocyte (with no apparent expression in Kuppfer cells) via its hybrid mRNA technology (Figure 16). The processes also afford the production of proteins which are located in varieties of compartments of cells, such as cytosolic, mitochondria and the ones that are secreted.

Repeated dosing by hybrid mRNA technology is important given other lipid-based nano-carrier systems, especially some PEGylated ones, could stimulate a phenomenon called accelerated blood clearance (ABC) and rapidly clear the subsequently injected nano-carriers.

In addition, PZRX indicated that repeat dosing can be achieved over a three month period without the loss of expression (luciferase was used as a marker) from a preclinical study. As such, the results suggest this platform could be used for chronic treatments. This is important given other lipid-based nano-carrier systems, especially some PEGylated ones, could stimulate a phenomenon called accelerated blood clearance (ABC) and rapidly clear the subsequently injected nano-carriers. ABC is an immunogenic response caused by the production of anti-PEG IgM (and possibly IgG) and subsequent complement system activation, resulting in accelerated capture of liposomes by Kupffer cells in the

liver. Further, mRNA delivered via the hybrid mRNA technology can provide a rapid and high level of expression of desired proteins.

Figure 16: Hybrid mRNA technology exhibited high liver specific (left) expression in hepatocytes (right)



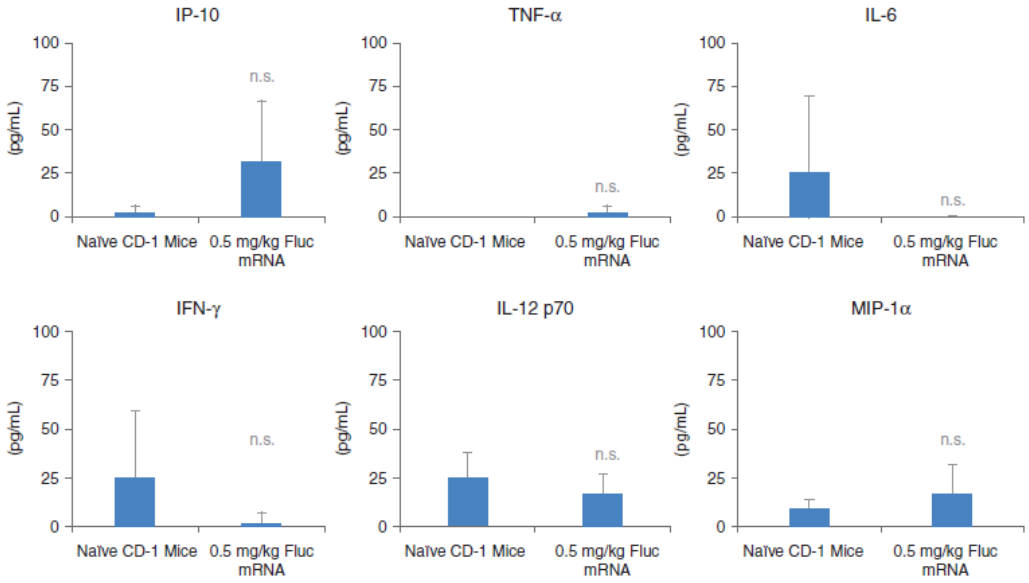
Source: Company presentation

Our discussions with management suggested that the liver focused delivery system could strategically serve the company well with substantial advantages. For example, using the hybrid mRNA technology, the company could easily expand their i-ERT coverage to other liver disorders with very minor adjustments. Further, there are substantial unmet needs for many other liver specific diseases due to the inadequacy or lack of effective treatment. As such, the commercial potential could be very significant should PZRX further develop other liver-targeted treatments beyond their initial focus on the urea cycle disorders indications.

As highlighted earlier that some fusogenic LNPs could create dose-limiting toxicities due to the activated innate immune system with the induction of unwanted cytokines, preclinical studies on luciferase mRNA delivered via hybrid mRNA technology did not meaningfully increase cytokines. Specifically, the levels of various cytokines, such as interferon gamma-induced protein 10 (IP-10), tumor necrosis factor alpha (TNF-α), interleukin-6 (IL-6), interferon gamma (IFN-γ), interleukin 12, (IL-12) and macrophage inflammatory protein alpha (MIP-1α) have not changed to a statistically significant level vs. the background (Figure 17).

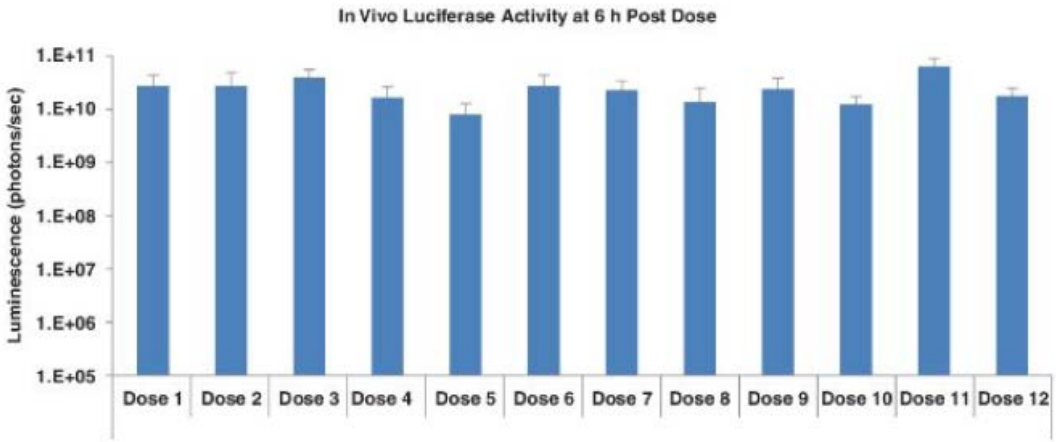
Repeat dosing is an advantage. Messenger RNA therapy delivered by hybrid mRNA technology enables repeat dosing and PZRX has demonstrated a similar level of expression of luciferase activities by weekly administration over a 3-month period (Figure 18). For chronic treatment, such as EPT, repeat dosing without losing potency is very critical. Some RNA-based therapeutics delivered via certain LNP-only formulations have experienced reduced potency after repeat dosing due to accelerated blood clearance (ABC).

Figure 17: Multiple cytokines have not changed due to mRNA delivered via hybrid mRNA technology



Source: Company presentation

Figure 18: Hybrid mRNA technology could afford repeat dosing regimen



Source: Company presentation

Robust intellectual properties support hybrid mRNA technology and derived clinical products. PhaseRx owns a robust portfolio of intellectual properties with multiple issued and pending patents covering various aspects of the hybrid mRNA technology and derived therapeutic products (Figure 19). As of May 2, 2016, PZRX owned or has in-licensed 11 issued U.S. patents, 20 issued foreign patents, and over 35 pending U.S. and foreign patent applications. PZRX's initial IP was exclusively licensed from the University of Washington. Major highlights include:

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- For the diblock polymer family, it has issued patents in 6 territories, and 7 pending patents in more territories. Together, they provide exclusivity until at least 2029;
- For the polymer nanoparticle family, it has issued patents in 3 territories, and 4 pending patents in more territories. Together, they provide exclusivity until at least 2029; and
- For the hybrid mRNA technology family, an international PCT patent application is pending and it potentially could provide exclusivity until at least 2036.

Figure 19: Selected patent portfolio of PZRX

Case Family	Issued Patents	Pending Applications by Jurisdiction	Owned or In-licensed
Enhanced Transport	US 6,835,393; US 7,374,778; US 8,003,129; US 8,846,106; EP 1044021; AU 758368; CA 2317549	US	In-licensed
Enhanced Transport	US 7,737,108; US 8,318,816		In-licensed
Temperature and pH-responsive Compositions	US 7,718,193		In-licensed
Diblock Copolymer	EP 2281011; AU 2009246327; CN ZL200980122888.3; IL 209238; MX 316902; SG 166444; ZA 2010/08729	US, AU, CA, JP, BR, CN, IN, KR	Co-owned; UW's rights in-licensed
Micellic Assemblies	EP 2285853; AU 2009246329; JP 5755563; MX 315375	US, AU, CA, JP, KR	Co-owned; UW's rights in-licensed
Polymeric Carrier	US 9,006,193	US	Co-owned; UW's rights in-licensed
Heterogenous Polymeric Micelles	US 9,211,250		Co-owned; UW's rights in-licensed
Bispecific Intracellular Delivery Vehicles	US 8,822,213; 9,220,791	US, EP, CA	Co-owned; UW's rights in-licensed
Hydrophilically Shielded Copolymers		US	Co-owned; UW's rights in-licensed
Multiblock Copolymers	EP 2364330; AU 2009313358; CN ZL200980148153.8; JP 5766611; MX 330456; SG 171100	US, CA, BR, IL, IN, KR, ZA	Co-owned; UW's rights in-licensed
Omega-Functionalized Polymers		US	Co-owned; UW's rights in-licensed
Targeting Monomers		US	Co-owned; UW's rights in-licensed
RNA Targeted to Beta-Catenin		US	Owned
RNA Targeted to c-Met		US	Owned
Block Copolymers		US, AU, EP, CA	Owned
Polymer-LNP Delivery		PCT, US	Owned

Source: Company presentation and Laidlaw & Company equity research

Together, we view hybrid mRNA technology could have several advantages and unique attributes over other mRNA therapy platforms:

1. With separated transport and delivery into two subsystems, hybrid mRNA technology could potentially have greater tolerability and safety over its competitors;
2. High specificity of producing desired proteins in hepatocytes;

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3. Hybrid mRNA technology has a broad patent estate protection;
4. Ability to repeat dose; and
5. Potential for rapid development of other products with relatively low development cost and risks.

The use of mRNA as a therapeutic for directly expressing proteins to correct the disorder could also have major advantages over current standard of care treatment modality, such as protein-based enzyme replacement therapy (ERT) and other emerging treatments, such as gene therapy. Specifically, viral-vector based gene therapy could potentially illicit immunity in patients and possibly reduce efficacy if it requires repeated administration. In addition, if the foreign gene introduced by gene therapy is inserted into an "undesired" spot of the genome, patients could potentially suffer from other untoward consequences, which include tumor formation.

Messenger RNA therapy Competitors. Messenger RNA therapy is an emerging RNA-based treatment technology and has attracted the large and established pharmaceutical and biotech companies as well as the emerging biotech companies to develop therapeutic products via their relatively proprietary approaches either in-house or partnered with developers. Selected examples include Shire, Alexion Pharmaceutical, Moderna Therapeutics, Arcturus Therapeutics, CureVac AG and Eukarÿs (Figure 20). Our scope of mRNA therapy competitors is limited, and primarily to platforms that provide a protein replacement regimen but not on cancer immunology or other immune-stimulating platforms, such as vaccine development.

- **Moderna Therapeutics**. It is a Cambridge, MA-based privately owned company that develops modified mRNA-based therapeutics, which include personalized cancer vaccines and various biologics, such as intra- and extra-cellular and secreted proteins. Moderna has established four proprietary venture companies for developing mRNA-based treatments: Onkaido Therapeutics (in oncology), Valera (in infectious diseases), Elpidera (in rare diseases) and Caperna (in personalized cancer vaccines). Moderna and Alexion Pharmaceuticals forged a collaboration for developing mRNA therapeutics to treat rare diseases with 10 product options. The recently announced program is ALXN 1540, a potential treatment for Crigler-Najjar syndrome (CN-1) and ALXN plans to advance it into clinical trial in 2016. Moderna also forged collaborations with AstraZeneca for developing mRNA therapeutics to treat oncological (a VEGF-A encoding modified mRNA) and cardiovascular diseases (AZD 8601). Merck also has a collaboration for the development of mRNA therapeutics in vaccines (MRK 1777) and passive immunity treatments against viral diseases.
- **Arcturus Therapeutics.** It is a San Diego, CA-based privately owned company that develops RNA-based therapeutics. Arcturus' key technology platforms are unlocked nucleomonomer agent (UNA) and LUNAR nanoparticle delivery. Together, they can be applied toward many types of RNA medicines, such as iRNA, mRNA, antisense,

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miRNA and gene editing therapeutics. The UNA technology can be used to target any gene from various sources for therapeutic purposes. LUNAR utilizes a GMP-ready microfluidic formulation process that yielded small particles (<80nm) with a low polydispersity index (<0.05). Arcturus formed collaborations with Janssen Pharmaceuticals and with Ultragenyx Pharmaceutical for unrevealed rare diseases. Arcturus also has an in-house preclinical OTC deficiency program called LUNAR-OTC, which currently is under development.

- **CureVac AG**. It is a Tübingen, Germany-based privately owned company with operations in Cambridge, MA that develops modified mRNA-based therapeutics.
- **Acuitas Therapeutics**. It is a Vancouver, Canada-based privately owned company that develops modified mRNA-based therapeutics. The platform includes cationic lipid included lipid nanoparticles (LNP), which is under limited license from Arbutus Biopharma. The company published results in 2015 from a collaborative study with CureVac examining the ability of Acuitas' LNP carriers to deliver an mRNA therapy encoding for the protein erythropoietin.
- **Arbutus Biopharma**. A majority of the company's more advanced pipeline products are RNAi (RNA interference) therapeutics. The company also has early stage preclinical research in mRNA and LNPs with possible indications in cancer, cardiovascular or metabolic diseases or infectious diseases.
- **Eukarÿs.** It is a France-based privately owned company that explores its C3P3 technology to develop mRNA therapy. The company indicated that they could potentially incorporate modified nucleosides such as 5'-methylcytidine-5'-triphosphate and pseudouridine-5'-triphosphate to reduce the innate immune response.
- **BioNTech AG**. It is a Mainz, Germany-based privately owned company that develops mRNA therapies. Its mRNA therapy operation focuses on three areas: cancer immunotherapies, infectious disease vaccines and protein replacement.
- **Silence Therapeutics.** The majority of the company's more advanced pipeline products (Phase II of Atu027 in pancreatic cancer) are of siRNA (short interfering RNA) therapeutics. The company also has early stage preclinical research on mRNA and liposome delivery system.
- **Ethris GmbH.** It is a Deutschland, Germany-based privately owned company that explores its SNIM RNA-Technology to develop mRNA therapy. The company indicated that their product could evade the innate immune system due to chemical modifications in RNA building blocks. Ethris has demonstrated in a preclinical study the efficacy of treating Surfactant Protein-B Deficiency, a fatal lung disease.

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Figure 20: Selected mRNA therapy companies

Product/Technology	Developer	Ticker	Partner	Ticker	Indication	Stage	Comments
ALXN1540	Moderna Therapeutics		Alexion Pharmaceuticals	ALXN	Crigler-Najjar Syndrome	Preclinical	Enter clinic in 2016
7 preclinical programs	Moderna Therapeutics		Alexion Pharmaceuticals	ALXN		Preclinical	
mRNA therapy	Moderna Therapeutics		AstraZeneca	AZN		Preclinical	
C3P3	Eukarÿs					Preclinical	
RNArt	Acuitas Therapeutics		CureVac AG			Preclinical	
mRNA therapy	BionTech AG					Preclinical	
mRNA therapy	Arbutus Biopharma	ABUS				Preclinical	
SHP637	Shire	SHPG				Preclinical	
Arc-#1 and Arc-#2	Arcturus Therapeutics		Ultragenyx Pharmaceutical	RARE		Preclinical	
LUNAR-OTC	Arcturus Therapeutics				OTCD	Preclinical	
mRNA therapy	Silence Therapeutics	SLN				Preclinical	
SNIM RNA	Ethris GmbH					Preclinical	

Source: Laidlaw & Company equity research

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Intracellular Enzyme Replacement Therapy (i-ERT) Could Potentially Expand the Therapeutic Coverages As Well As Commercial Opportunities

By developing intracellular enzyme replacement therapy (i-ERT), the hybrid mRNA platform could potentially afford a next generation enzyme replacement therapy

Beyond the mRNA therapy development against major parts of UCD (>90% based on our estimate), the hybrid mRNA technology could also have the potential to develop novel Intracellular Enzyme Replacement Therapy (i-ERT) as a potential next generation enzyme replacement therapy (ERT) to correct many inborn error disorders, especially the ones where corrective actions occur in the liver (specifically in hepatocytes).

Compared to the conventional protein-based ERT, i-ERT could have an important advantage: while ERT would be effective only if the exogenously generated enzyme can be transported into the cell or work through the cell surface (i.e. via receptor), i-ERT could synthesize proteins directly inside the cell, and therefore, with greater versatility and accessibility for potentially treating more disorders. With a proper design, we believe i-ERT could treat disorders with action sites not only in the cytosol, but also in different organelles, such as mitochondria. As such, PZRX focuses its developments on inherited, single-gene disorders of metabolism in the liver that result in deficiency of an intracellular enzyme and thus have been unable to be treated with conventional ERT.

Should the PRX-OTC and other UCD treatments be successful, PZRX believes there are a large number of inherited, single-gene disorders with a therapeutic action site in the liver, which include: organic acidemias; glycogen storage diseases; porphyria; phenylketonuria; hyperoxalurea; tyrosinemia; and Wilson's Disease.

Should the PRX-OTC and other UCD treatments be successful, PZRX believes there are a large number of inherited, single-gene disorders with therapeutic action site in the liver. Examples include:

- Organic acidemias;
- Glycogen storage diseases;
- Porphyria;
- Phenylketonuria;
- Hyperoxalurea;
- Tyrosinemia; and
- Wilson's Disease (Figure 21).

PZRX estimates the potential therapeutic market for these ultra-orphan indications could amount to $4 billion. Figure 22 illustrates the annual incidences in the U.S. and Europe.

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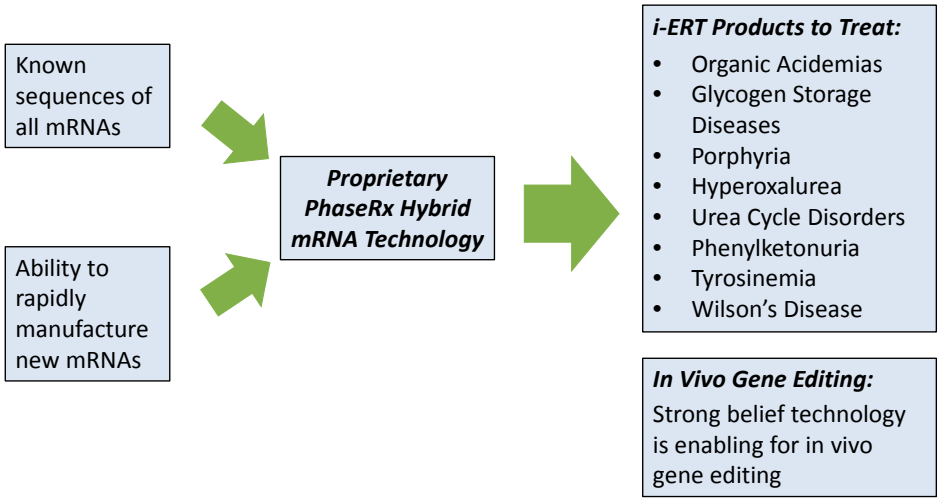
Figure 21: mERT could expand the treatment coverage of enzyme replacement therapy



Source: Company presentation

Figure 22: Annual incidences of selected liver-specific rare disorders

Indication	Incidence Rate (per live births)	Annual Incidence (US)	Annual Incidence (EU)	Incidence Total (US+EU)
Organic Acidemias	1/1000	4,000	5,000	9,000
Glycogen Storage Diseases	1/20,000	200	250	450
Porphyria	1/75,000	53	67	120
Hyperoxalurea	1/175,000	23	29	51
Phenylketonuria	1/15,000	267	333	600
Tyrosinemia (type 1)	1/100,000	40	50	90
Wilson's Disease	1/30,000	133	167	300

Source: Company presentation and Laidlaw and co. equity research

PZRX believes the hybrid mRNA technology could potentially be used as a means for facilitating in vivo gene editing

In addition, PZRX believes the hybrid mRNA technology could potentially be used as a means for facilitating in vivo gene editing. Gene editing or genome editing is an emerging technology that nowadays most frequently uses CRISPR (clustered, regularly interspaced short palindromic repeats) / Cas9 (CRISPR associated protein 9) to potentially achieve precise, directed changes in DNA to correct disease-causing genes. The platform requires CRISPR RNA-guided nuclease Cas9 (a protein-RNA complex) in the targeted cells to make a precise and specific cut in the DNA, ultimately triggering the cell's DNA repair machinery to address the genetic defect (Figure 23). Given the protein/RNA complex is a large molecular weight entity, hybrid mRNA technology could become a viable and potentially effective system transferring CRISPR and Cas9 together, or separately into targeted cells as an alternative delivery method to viral vector systems.

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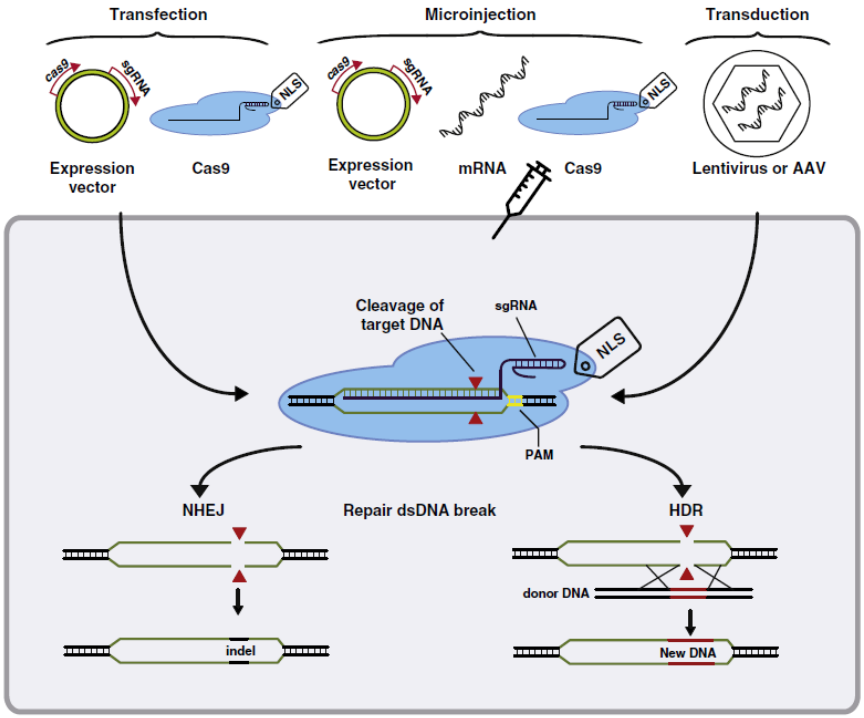
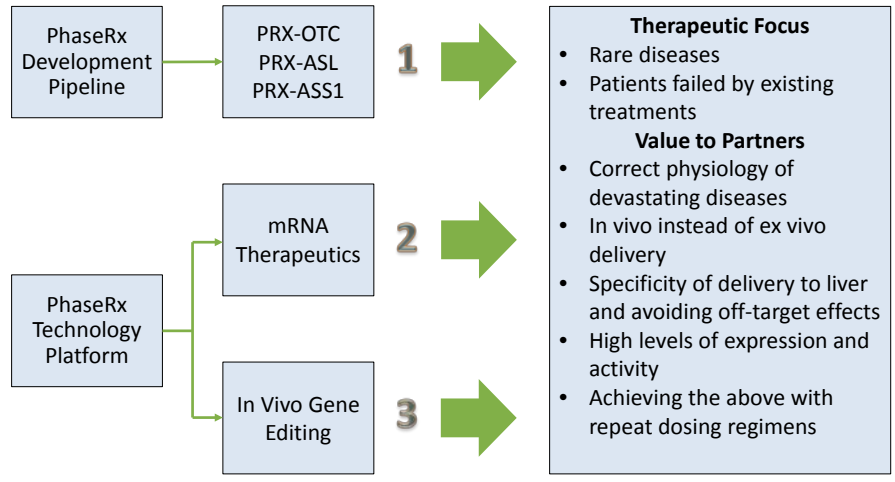
Figure 23: The mechanism of action of the CRISPR/cas9 system



Source: van Erp, P. B., et.al., Curr. Opt.Virol.(2015) 12: 85-90.

Future corporate development directions Given the potential versatility and broad utilities in treating different diseases and gene editing of hybrid mRNA technology, PZRX is contemplating forging potential partnerships with other companies for treating various orphan disorders and as a delivery alternative for different gene editing approaches (Figure 24).

Figure 24: mERT could expand the treatment coverage of enzyme replacement therapy



Source: Company presentation

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Financial Projections and Valuation

After the IPO, PZRX has raised ~$19.5MM cash and the company indicated that the major uses of proceeds include ~ $12.6MM for manufacturing scale up of the lead product candidate; $3.2MM for preclinical activities (including toxicology studies); ~$460K for preclinical proof of concept studies of the second UCD candidate; ~$690K for further development of the selected UCD product candidate; and the remaining for general corporate purposes including working capital requirements. In addition, we project PZRX could enrich its balance sheet further via debt financing, possibly near term.

Our probability-adjusted DCF analysis suggested a one-year target value for PZRX of $11.79 based on cash flow until 2027 with an assumed terminal value multiple of three and a probability adjustment of 34%.

Probability-adjusted DCF analysis

Cash driven NPV	2017	2018	2019	2020	2021	2022	2023	2024	2025	2026	2027
Revenue	0	1,000	1,000	2,000	2,000	48,780	142,572	264,786	416,046	568,848	689,241
R&D	9,441	11,235	13,369	16,177	18,927	20,252	21,670	23,187	24,810	26,546	28,405
SG&A	3,152	3,468	3,780	4,082	4,368	29,586	31,566	33,679	35,935	38,345	40,917
Operating income	(12,594)	(13,703)	(16,149)	(18,259)	(21,295)	(11,479)	62,034	158,619	278,773	399,925	494,216
Net income	(13,974)	(15,061)	(17,482)	(19,564)	(22,570)	(12,753)	38,280	99,132	174,835	251,169	310,582
Period	0.6	1.6	2.6	3.6	4.6	5.6	6.6	7.6	8.6	9.6	10.6
NPV	(12,887)	(12,026)	(12,086)	(11,710)	(11,696)	(5,722)	14,871	33,342	50,912	63,325	67,797

Total DCF	164,119
Terminal value	229,831
Cash (2Q17)	11,000
Total valuation ($ '000)	404,950
Probability adjustment	34%
Value per share	**$11.79**
Shares outstanding (2017)	11,814
Discount rate	16%
Terminal value multiple	3

Source: Laidlaw & Company estimates

Our probability-adjusted-PV-driven, sum-of-the-parts analysis suggests a 12-month target price of $12.06 with value breakdown of PRX-OTC, PRX-ASL /PRX-ASS1 and other opportunities from hybrid mRNA technology accounting for 77%, 10% and 5% of the total value, respectively.

NPV driven sum-of-the-parts analysis

PRX-OTC	**OTCD**		
	Adjusted NPV =	**$116.4**	
	PV per share =	**$9.34**	**77%**
PRX-ASL / PRX-ASS1	**ASLD / ASS1D**		
	Adjusted NPV =	**$15.2**	
	PV per share =	**$1.22**	**10%**
Hybrid mRNA tech.			
	Adjusted NPV =	**$7.7**	
	PV per share =	**$0.62**	**5%**
Cash			
	Adjusted NVP =	**$11.0**	
	NVP per share =	**$0.88**	**7%**
	Total =	**$12.06**	**100%**

Source: Laidlaw & Company estimates

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For the peer comparable analysis, we have chosen a group of early clinical stage (Phase I/II) companies (some in UCDs and RNA-based therapeutics development with several of them went public during the last three years) as comparable peers. As such, our peer comparable analysis suggested a 12-month target price for PZRX of $13.15.

Comparable analysis

Company	Ticker	Rating	Target Price ($)	Price ($) (6/8/16)	Shares Outstanding (MM)	Market Cap ($ MM)	Cash ($ MM)	Debt ($ MM)	Tech Value ($ MM)	Most Advanced Development Stage	Major Indication
Aeglea Biotherapeutics	**AGLE**	NR	NA	7.26	11	83	29	0	54	Phase I	Hyperargininemia
Dimension Therapeutics	**DMTX**	NR	NA	9.94	25	249	117	0	132	Phase I	OTCD / Hemophilia
Voyager Therapeutics	**VYGR**	NR	NA	14.06	27	375	204	0	171	Phase I	Parkinson's, SMA
Mirna Therapeutics	**MIRN**	NR	NA	4.71	21	98	81	0	17	Phase I	Solid tumor (microRNA)
Cidara Therapeutics	**CDTX**	NR	NA	10.63	14	148	97	0	51	Phase I	Fungal infections
Silence Therapeutics	**SLN**	NR	NA	1.29	69	89	52	0	37	Phase II	Pancreatic cancer
Bio Blast Pharma	**ORPN**	NR	NA	2.14	16	35	22	0	13	Phase II	OPMD
Ocera Therapeutics	**OCRX**	NR	NA	2.62	21	56	40	0	16	Phase II	Hepatic encephalopathy
Average						**274**	*86*	*2*	**135**		
PhaseRx	**PZRX**	**Buy**	**12.00**	**5.20**	**12**	**60**	**18**	**0**	**43**	**Preclinical**	**UCDs**

PZRX share fair value matching its Phase I/II oncology peers = $13.15

Source: Company reports and Laidlaw & Company estimates

Together, we assigned our blended 12-month target price for PZRX of **$12.00**.

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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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Management

Robert W. Overell, Ph.D. co-founded and has served as president since 2006 and CEO since 2009. Prior, Dr. Overell was president of Foundation BioVentures, Prior to that from 1996 to 2005, Dr. Overell was a general partner with Frazier Healthcare Ventures. He helped found Targeted Genetics Corp, a spinout from Immunex Corporation's gene therapy, when he was developing gene delivery programs from 1992 to 1996 after he joined in Immunex in 1984. Dr. Overell holds a Ph.D. in biochemistry from the Institute of Cancer Research, University of London, U.K.

Michael Houston, Ph.D. has served as Chief Scientific Officer of PhaseRx since December 2015 and vice president, therapeutics development since 2014. Prior to PhaseRx from 2012 to 2013, Dr. Houston provided consulting services at Solid-Phase Consulting. From 2009 to 2012, He was vice president of chemistry and formulations for Marina Biotech. Prior to Marina, he served as vice president of preclinical chemistry and chemistry, manufacturing and control for Anchor Therapeutics. From 2008 to 2009, he served as vice president, chemistry and formulations at MDRNA. Prior to MDRNA from 2004 to 2008, Dr. Houston served as senior director of chemistry and formulations amongst other leadership positions at Nastech Pharmaceutical. Prior, he served at Cytovax Biotechnologies as director of chemistry and senior scientist. He holds a Ph.D. in bio-organic chemistry from the University of Waterloo, Ontario, Canada.

Shing-Yin (Helen) Tsui has served as vice president, finance of PhaseRx since December 2015. Prior to PhaseRx from 1999 to 2015, Ms. Tsui held various accounting positions at Dendreon including vice president, corporate controller (2014-2015), senior director, corporate controller (2013-2014), senior director, accounting operations and enterprise applications (2011-2013) and corporate controller (1999-2011). Ms. Tsui holds a B.A. in business administration from the University of Washington.

Mary G. Prieve, Ph.D. has served as director of biology at PhaseRx since February 2008. Prior to PhaseRx from 2004 to 2007, Dr. Prieve was senior research scientist at Nastech Pharmaceutical. Prior to Nastech from 2003 to 2004, Dr. Prieve was a visiting assistant professor at Smith College and a lecturer in cell and general biology at University of Washington and Seattle University. Dr. Prieve holds a Ph.D. in biological sciences from University of California, Irvine.

Sean Monahan, Ph.D. has served as the Director of Chemistry at PhaseRx since July 2009. Prior to PhaseRx from 1998 to 2011, Dr. Monahan served in various capacities (senior scientist I-III) with Roche/Mirus Bio Corporation. Prior from 1996 to 1998, Dr. Monahan worked in medicinal chemistry, new lead discovery at Berlex Biosciences of Schering AG. Dr. Monahan holds a Ph.D. in organic chemistry from University of Wisconsin, Madison.

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PhaseRx – Income Statement

($'000)	2014	2015	1Q16	2Q16E	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	1,448	1,535	1,673	6,091	9,441	11,235	13,369	16,177	18,927	20,252	21,670
General and administrative	1,931	1,299	680	707	728	750	2,866	3,152	3,468	3,780	4,082	4,368	4,586	4,816
Marketing and sales													25,000	26,750
Total operating costs and expenses	6,791	6,182	2,114	2,156	2,264	2,424	8,957	12,594	14,703	17,149	20,259	23,295	49,838	53,235
Operating Incomes (losses)	(5,591)	(5,807)	(2,114)	(2,156)	(2,264)	(2,424)	(8,957)	(12,594)	(13,703)	(16,149)	(18,259)	(21,295)	(11,479)	62,034
Interest expense	(1,367)	(1,649)	(201)	0			(201)	(1,600)	(1,600)	(1,599)	(1,598)	(1,597)	(1,629)	(1,662)
Other income (expense)	109	79	81	50	55	60	246	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)	50	55	60	45	(1,380)	(1,358)	(1,333)	(1,305)	(1,275)	(1,275)	(1,272)
Pretax income	(6,849)	(7,377)	(2,234)	(2,106)	(2,209)	(2,364)	(8,912)	(13,974)	(15,061)	(17,482)	(19,564)	(22,570)	(12,753)	60,762
Tax													0	22,482
Net Income (Loss)	(6,849)	(7,377)	(3,286)	(2,106)	(2,209)	(2,364)	(9,964)	(13,974)	(15,061)	(17,482)	(19,564)	(22,570)	(12,753)	38,280
Basic and diluted net loss per share	($1.16)	($1.12)	($0.42)	($0.17)	($0.18)	($0.19)	($0.96)	($1.12)	($1.12)	($1.06)	($1.01)	($1.15)	($0.52)	$1.55
Shares used to calculate the basic and diluted net loss per share	5,895	6,575	7,882	12,245	12,255	12,265	11,162	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	1123%	1337%	809%	946%	42%	15%
SG&A	161%	346%	NA	NA	NA	NA	NA	NA	347%	378%	204%	218%	9%	3%
Operating Income (loss)	-466%	-1549%	NA	NA	NA	NA	NA	NA	-1370%	-1615%	-913%	-1065%	-24%	44%
Pretax	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1506%	-1748%	-978%	-1128%	-26%	43%
Tax Rate													37%	37%
Net Income	-571%	-1967%	NA	NA	NA	NA	NA	NA	-1506%	-1748%	-978%	-1128%	-26%	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%	NA	NA	NA	25%	55%	19%	19%	21%	17%	7%	7%
SG&A	NA	-33%	139%	NA	NA	NA	121%	10%	10%	9%	8%	7%	5%	5%
Operating Income (Losses)	NA	4%	35%	NA	NA	NA	45%	41%	17%	17%	18%	15%	114%	7%
Pretax Income	NA	8%	57%	NA	NA	NA	21%	57%	8%	16%	12%	15%	-43%	-576%
Net Income	NA	8%	131%	NA	NA	NA	35%	40%	8%	16%	12%	15%	-43%	-400%
EPS	NA	-3%	84%	NA	NA	NA	-14%	17%	0%	-5%	-5%	15%	-55%	-399%

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

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PhaseRx – Balance Sheet

($'000)	2014	2015	1Q16	2Q16E	3Q16E	4Q16E	2016E	2017E
Assets								
Cash and cash equivalents	2,031	3,290	930	24,549	23,421	22,203	22,203	9,735
Short term investments							0	0
Liquid assets	***2,031***	***3,290***	***930***	***24,549***	***23,421***	***22,203***	***22,203***	***9,735***
Receivable								
Prepaid expenses, other current assets, and deferred financing costs	175	388	836	878	904	967	967	1,093
Total Current Assets	2,206	3,678	1,766	25,427	24,325	23,171	23,171	10,828
Property and equipment, net	453	236	317	398	498	464	464	429
Total Assets	**2,659**	**3,914**	**2,083**	**25,825**	**24,822**	**23,634**	**23,634**	**11,257**
Liabilities and Stockholders' Equity								
Accounts payable	197	396	678	1,695	2,882	4,034	4,034	4,526
Accrued liabilities	435	445	416	433	450	468	468	571
Accrued interest	2,055	3,199	0	0	0	0	0	0
Convertible notes, net of debt discount	12,540	19,841	0	0	0	0	0	0
Deferred contract revenue	375	0	0	0	0	0	0	0
Deferred rent	193	47	33	40	42	47	47	49
Total current liabilities	15,795	23,928	1,127	2,168	3,373	4,549	4,549	5,146
Preferred stock warrant liability	2,695	3,163	644	0	0	0	0	0
Total Liabilities	18,490	27,091	1,771	2,168	3,373	4,549	4,549	5,146
Series A, $0.0001 par value	20,205	20,212	-	-	-	-	0	0
Series A-1, $0.0001 par value	5,500	5,500	-	-	-	-	0	0
Common stock	2	2	3	4	4	4	4	4
Additional paid-in capital	428	452	52,938	78,388	78,388	78,388	78,388	79,388
Accumulated deficit	(41,966)	(49,343)	(52,629)	(54,735)	(56,943)	(59,307)	(59,307)	(73,280)
Total Stockholders' Equity	(41,536)	(48,889)	312	23,657	21,449	19,085	19,085	6,112
Total Liabilities and Stockholders' Equity	**2,659**	**3,914**	**2,083**	**25,825**	**24,822**	**23,634**	**23,634**	**11,257**

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

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PhaseRx – Cash Flow Statement

($'000) Cash Flows From Operating Activities:	2014	2015	1Q16	2Q16E	3Q16E	4Q16E	2016E	2017E
Net income (loss)	(6,849)	(7,377)	(2,234)	(2,106)	(2,209)	(2,364)	(8,912)	(13,974)
Adjustments to reconcile net loss to net cash used in operating activities:								
Depreciation and amortization	512	331	61	80	90	110	341	390
Amortization of debt discount	282	505	151	0	0	0	151	0
Stock-based compensation	30	21	19	26	30	45	120	110
Noncash interest expense	1,003	1,144	50	0	0	0	50	0
Deferred contract revenue	375	(375)	0	0	0	0	0	0
Revaluation of preferred stock warrant liability	(109)	(79)	(81)	0	0	0	(81)	0
Changes in operating assets and liabilities:								
Prepaid expenses and other current assets	(99)	(213)	(2)	(42)	(26)	(63)	(133)	(126)
Accounts payable	(22)	199	282	282	1,017	1,187	2,768	491
Accrued liabilities	230	10	(29)	17	17	18	23	103
Deferred rent	(214)	(146)	(14)	7	2	5	0	2
Net Cash provided by (used in) Operating Activities	**(4,861)**	**(5,980)**	**(1,797)**	**(1,736)**	**(1,079)**	**(1,062)**	**(5,674)**	**(13,002)**
Cash flows from investing activities:								
Acquisitions of property and equipment	(53)	(114)	(142)	(70)	(50)	(155)	(417)	(465)
Net Cash provided by (used in) Investing Activities	**(53)**	**(114)**	**(142)**	**(70)**	**(50)**	**(155)**	**(417)**	**(465)**
Cash Flows From Financing Activities:								
Deferred offering costs	0	0	(446)	0	0	0	(446)	0
Proceeds from issuance of convertible notes	0	7,675	0	0	0	0	0	0
Proceeds from issuance of notes			0	6,000	0	0	6,000	0
Proceeds from issuance of redeemable convertible preferred stock	5,500	0	0	0	0	0	0	0
Principal payments on credit facility	(232)	0	0	0	0	0	0	0
Debt issue costs	0	(332)	0	0	0	0	0	0
Proceeds from exercise of stock warrants		10	0	0	0	0	0	1,000
Proceeds from issuance of common stocks			0	19,450	0	0	19,450	0
Net Cash Provided by (used in) Financing Activities	**5,268**	**7,353**	**(446)**	**25,450**	**0**	**0**	**25,004**	**1,000**
Net increase (decrease) in cash	**354**	**1,259**	**(2,385)**	**23,644**	**(1,129)**	**(1,217)**	**18,913**	**(12,468)**
Cash at beginning of period	1,677	2,031	3,290	905	24,549	23,421	3,290	22,203
Cash at end of period	**2,031**	**3,290**	**905**	**24,549**	**23,421**	**22,203**	**22,203**	**9,735**

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Source: Bloomberg LP; Company reports; Laidlaw & Company estimates. 1Q16 Net Income adjusted.

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DISCLOSURES:

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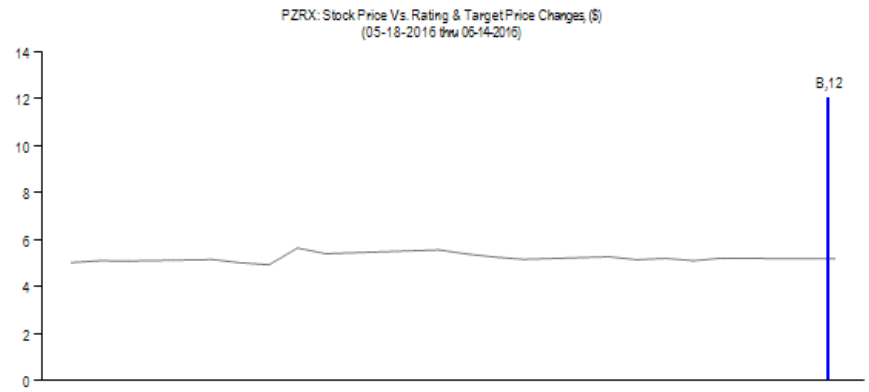
Additional information available upon request.

‡ Laidlaw & Company has received compensation from the subject company for investment banking services in the past 12 months and expects to receive or intends to seek compensation for investment banking services from the company in the next three months.
The member or affiliate managed or co-managed a public offering of securities for the subject company in the past 12 months;

RATINGS INFORMATION

Rating and Price Target Change History



3 Year Rating Change History

Date	Rating	Closing Price ($)
06/14/2016	Buy (B)	5.18*

3 Year Price Change History

Date	Target Price ($)	Closing Price, ($)
06/14/2016	12.00	5.18*

** Previous Close6/13/2016*

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.	67.57%	27.03%	2.70%
Hold (H)	Expected returns to be in line with the sector average over 12 months.	0.00%	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

Horizon Pharma (HZNP – Not Rated)
Recordati Pharmaceuticals (REC-MI – Not Rated)
Shire (SHPG – Not Rated)
Aeglea Biotherapeutics (AGLE – Not Rated)
Bio Blast Pharma (ORPN – Not Rated)
Dimension Therapeutics (DMTX – Not Rated)
Ocera Therapeutics (OCRX – Not Rated)
Alexion Pharma (ALXN – Not Rated)
AstraZeneca (AZN – Not Rated)

Merck (MRK – Not Rated)
Janssen Pharmaceuticals (JNJ – Not Rated)
Ultragenyx Pharmaceutical (RARE – Not Rated)
Arbutus Biopharma (ABUS – Not Rated)
Silence Therapeutics (SLN – Not Rated)
Voyager Therapeutics (VYGR – Not Rated)
Mirna Therapeutics (MIRN – Not Rated)
Cidara Therapeutics (CDTX – Not Rated)

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