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AzurRx BioPharma, Inc. (AZRX - NASDAQ)

FDA Meeting Before Year End

Based on our DCF model and a 15% discount rate, AZRX is valued at approximately $5.00 per share. Our model applies a 15% probability of eventual MS1819 sales for EPI based on historical Phase 2 success ratios. Our valuation includes geographic contributions from both inside outside the US. We do not include any value for the preclinical AZX1103 program.

Current Price (11/19/2018)	$0.80
Valuation	**$5.00**

OUTLOOK

AzurRx employs recombinant protein technology to treat gastrointestinal diseases and microbiome related conditions using oral, non-systemic biologics. It currently has one clinical asset and two preclinical programs in its pipeline.

The company recently completed a Ph2 trial for MS1819 in cystic fibrosis patients and is planning a Phase IIb/III study which will employ higher dosing and/or enteric coated capsules. The drug is an orally delivered, non-systemic, yeast-derived recombinant enzyme. It addresses EPI found in chronic pancreatitis or cystic fibrosis patients. A second compound, AZX1103, is preclinical and is being developed to prevent hospital acquired infections resulting from intravenous antibiotic administration. The discovery stage asset, MTAN, is for treatment for *H. pylori* infections.

Results to date for MS1819 demonstrate safety, efficacy and no need for additional protease in treating EPI for CF patients. We maintain our confidence in eventual approval and commercialization will occur in 2023.

SUMMARY DATA

52-Week High	**3.10**
52-Week Low	**0.42**
One-Year Return (%)	**-63.1**
Beta	**2.1**
Average Daily Volume (sh)	**333,235**
Shares Outstanding (mil)	**26.2**
Market Capitalization ($mil)	**21.0**
Short Interest Ratio (days)	**1.53**
Institutional Ownership (%)	**14.9**
Insider Ownership (%)	**32.8**
Annual Cash Dividend	**$0.00**
Dividend Yield (%)	**0.00**
5-Yr. Historical Growth Rates	
Sales (%)	**N/A**
Earnings Per Share (%)	**N/A**
Dividend (%)	**N/A**
P/E using TTM EPS	**N/A**
P/E using 2019 Estimate	**N/A**
P/E using 2020 Estimate	**N/A**
Zacks Rank	**N/A**

Risk Level	**Above Average**
Type of Stock	**Small-Growth**
Industry	**Med-Biomed/Gene**

ZACKS ESTIMATES

Revenue

(In millions of US$)

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2018	$0.0 A	$0.0 A	$0.0 A	$0.0 A	$0.0 A
2019	$0.0 A	$0.0 A	$0.0 A	$0.0 E	$0.0 E
2020					$0.0 E
2021					$0.0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2018	-$0.29 A	-$0.22 A	-$0.15 A	-$0.23 A	-$0.88 A
2019	-$0.26 A	-$0.25 A	-$0.17 A	-$0.17 E	-$0.82 E
2020					-$0.72 E
2021					-$0.70 E

What's New

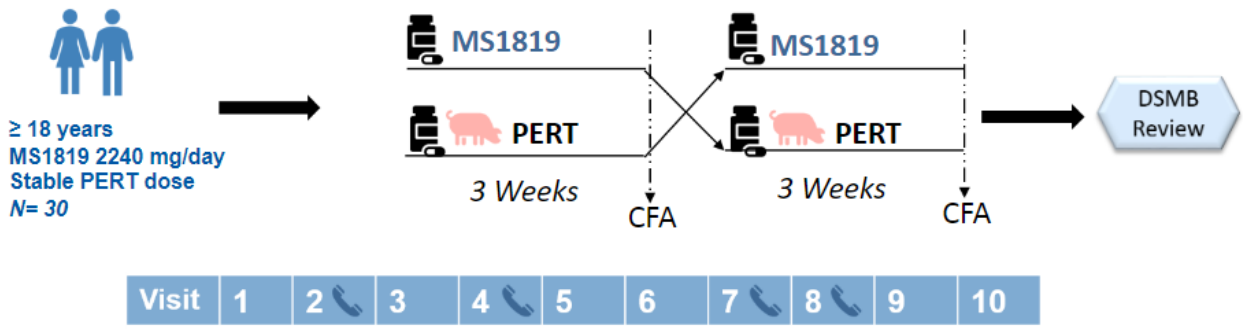
AzurRx BioPharma, Inc. (NASDAQ: AZRX) filed its 2019 third quarter 10-Q with the SEC for the three month period ending September 30, 2019. Highlights for the reporting period and to date include the announcement and first dosing in a new combination trial for patients with severe EPI, the readout from the OPTION trial, the addition of new CEO James Sapirstein and a positive Data Safety Monitoring Board (DSMB) review of the OPTION trial.

No revenues were reported. Operating expenses for the July to September period were $4.1 million, up 64% compared to the prior year third quarter. General and administrative expenses were $1.9 million, rising 42% in comparison with 3Q:18 with more funds allocated to investor relations activities and losses recognized due to fraud partially offset by a decrease in stock compensation. Research and development expenses expanded 89% on a year over year basis to $2.2 million. Increases were attributed to the startup of a research and development function in the U.S. including expenses allocated to the OPTION study.

Cash on the balance sheet was $1.6 million and notes payable and convertible debt were held at $1.9 million as of September 30, 2019. Cash burn for the three month period was ($4.2) million which compares to ($2.7) million for 3Q:18. $5.0 million in common stock was raised in a July public offering and on November 14, the company announced a $15 million equity purchase agreement with Lincoln Park Capital.

AzurRx announced Phase II OPTION trial results for MS1819 in cystic fibrosis (CF) patients in late September. Data provided was sparse, but the summary highlighted the positive safety profile for MS1819 and the utility of the drug in comparison with porcine enzyme replacement therapy (PERT). The coefficient of fat absorption (CFA) was 56% for the MS1819 treatment phase and 86% for the PERT treatment phase. The two arms of the trial were comparing fixed doses of MS1819 with variable doses of PERT. The trial demonstrated a high coefficient of nitrogen absorption (CNA) for both arms of the trial, which indicates the ability of the digestive system to break down proteins while undergoing MS1819 therapy. We anticipate AzurRx will share the data from the trial with the FDA and design another Phase II study which will determine an optimal dosing and delivery mechanism. The trial may be of an adaptive design and allow for the Phase II to shift to a Phase III as optimal dosing data becomes available.

Exhibit I – US and EU OPTION Study in Cystic Fibrosis Trial Design[1]



While the full data set was not provided, results from the trial demonstrated "comparable" efficacy of MS1819 to PERT "with approximately 50% of the patients showing CFA high enough to reach non-inferiority with [PERT]." The dosage used in the OPTION trial was 2.2 grams per day, matching the highest level of drug product used in the Phase II Chronic Pancreatitis (CP) study.

CFA for MS1819 was 56% compared to the CFA for PERT of 86%. An important clarification for this data is that the doses used in the PERT control arm varied widely from 2,700 to 9,400 lipase units per kilogram per day, while MS1819 was stable at 2.2 g/d. Clearly the study was not providing a direct comparison between comparable and consistent doses of drug for PERT and MS1819. The rationale for this unequal comparison was a mandate from the FDA requiring the OPTION study to focus on safety rather than efficacy. It is our understanding that the FDA did not allow higher doses to be used at this stage of development. Chief Medical Officer, Dr. Pennington, noted that patients on the highest dose of PERT had the lowest response to MS1819, while the patients administered the lowest dose of PERT had the highest CFA response to MS1819.

[1] AzurRx MS1819-SD OPTION Study Results corporate presentation, September 25, 2019.

The trial results demonstrated favorable safety with no severe adverse events and few overall adverse events. In animal studies, dosing rose to 100 g/d, and no safety events were observed at this extreme level providing confidence that further dose ranging trials can safely increase doses multiples above the 2.2 g/d level.

A recurring question regarding the comparable efficacy of MS1819 with PERT relates to the enzymes that are present in the two drugs. MS1819 only contains lipases, however, digestion requires lipases, proteases and amylases. While the pancreas secretes all three enzymes, proteases and amylases are also produced in the digestive tract and saliva. A concern with MS1819 has been that there may be insufficient protein breakdown as compared to PERT, which contains all three enzymes. While carbohydrate digestion had not been a concern, it was undetermined whether proteins were being sufficiently digested without protease in the therapy.

Protein digestion is measured by the CNA, a clinical endpoint used to evaluate the level of protein digestion. Normal CNA is 88%[2] according to research conducted by Borowitz et al. In several studies cited for Creon, CNA levels in patients with exocrine pancreatic insufficiency (EPI) due to CF were in the 40% range in the placebo group and in the 80% range for Creon.[3] This compares to the observation of a 93% CNA for MS1819 and a 97% CNA for PERT in the OPTION trial. The much improved CNA is attributed to the slower pace of digestion that takes place when lipases are used as part of therapy which allows the existing proteases time to act. The gastrointestinal (GI) system does produce proteases; however, it is hypothesized that the rapid movement of the food through the GI system in non-treated EPI CF patients does not allow time for these enzymes to act. MS1819 therapy slows the digestion process giving the protease enzymes time to break down proteins, allowing the patient to digest these nutrients. This is a favorable outcome for MS1819 as is suggests that the drug can be used as a monotherapy in patients.

Our original forecast had anticipated that results from the OPTION trial would be sufficient to support a direct move into a Phase III registrational trial. However, the optimal dose has not yet been determined. The company anticipates and we believe that a Phase II dose ranging study will be required before a Phase III trial can be started. Our confidence in MS1819 is not shaken and we see first sales occurring in 2023.

We anticipate that AzurRx will either increase the dose in the forthcoming dose ranging study or they will use an enteric coating for the drug capsule to ensure that more lipase is able to transit the acidic environment of the stomach and reach the duodenum where fat breakdown occurs. We do not anticipate any additional studies will be required to evaluate the enteric coating as it is well characterized and commonly used in a broad variety of other capsules in drug delivery.

Exhibit II – MS1819 Development Milestones[4]

Evaluation	Status	Comment
Animal Safety	✓	Pig and rat data supports high MS1819 doses
Animal Efficacy	✓	Efficacious in Pig EPI model
FLIP 110 Study	✓	Initial safety in humans established
CP Ph 2 Study	✓	2.2g dose safe, and significant improvement of CFA
CF Ph 2 OPTION	✓	Good safety, improvements in CFA comparable to that observed in CP study
Can be used without a Protease	✓	OPTION data in CF patients suggests that a MS1819 lipase only product is sufficient
CF Combination Study	Ongoing	Awaiting further data
Higher dose in CF		Safety data supports increasing the MS18189 dose delivered to the duodenum

While the duration of clinical development now appears to be longer than our original estimates, we maintain our confidence in both MS1819 and the drug development process. AzurRx appears to be leaning toward the use of an enteric coating to address low CFA numbers in EPI patients on higher doses of PERT. While the additional trial may add up to a year to the process, MS1819 has several favorable characteristics that support its continued

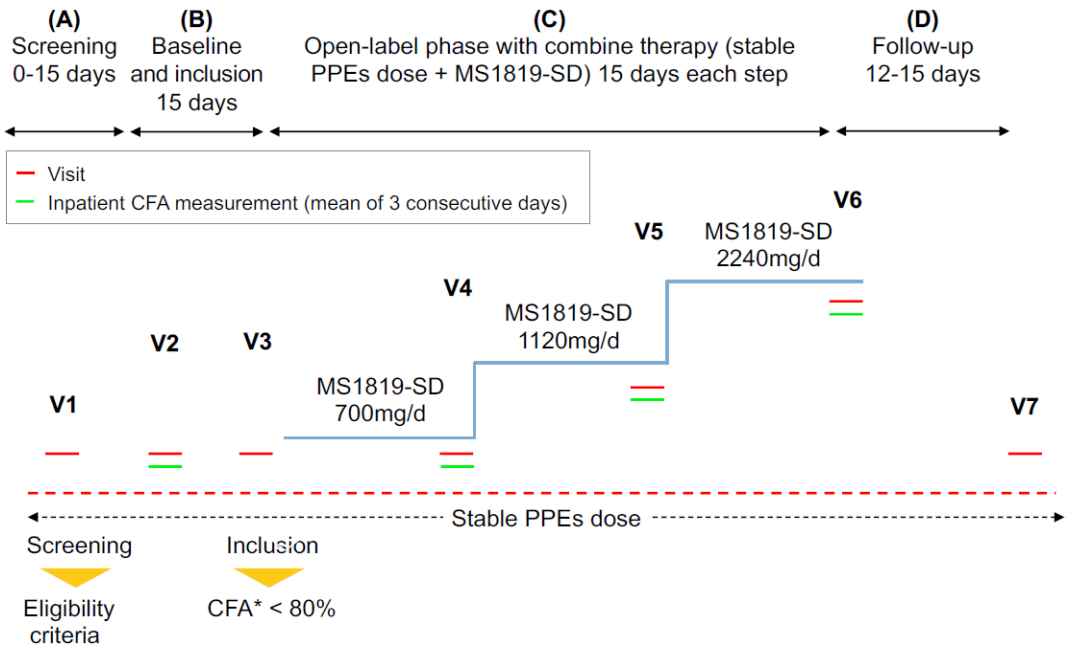
[2] Borowitz D, Konstan MW, O'Rourke A, Cohen M, Hendeles L, Murray FT. Coefficients of fat and nitrogen absorption in healthy subjects and individuals with cystic fibrosis. J Pediatr Pharmacol Ther. 2007;12(1):47-52.
[3] https://www.creon.com/hcp/efficacy
[4] AzurRx MS1819-SD OPTION Study Results corporate presentation, September 25, 2019.

development and likelihood of approval. It is a naturally derived product classified "Generally Recognized as Safe" (GRAS), provides additional consistency compared to animal sourced products, avoids many of the sourcing concerns related to the spread of infectious agents of animal origin and reduces the patient pill burden among other features. Details of our thesis are provided in our initiation.

Severe EPI Trial

In early July, AzurRx launched a Phase II trial investigating MS1819-SD in combination with standard porcine enzyme replacement therapy (PERT) for patients with cystic fibrosis (CF) that suffer from severe exocrine pancreatic insufficiency (EPI). These patients are unable to maintain weight and suffer from fat malabsorption despite taking the maximum dose of PERT. This population with an unmet need may provide an expedited pathway to approval for MS1819-SD. The study will be conducted at six sites in Hungary with an enrollment target of 28 and a primary endpoint of safety and CFA. We discuss AzurRx's new Phase II study in more detail here. In the August corporate presentation the company provides supportive data on why nutrition matters for cystic fibrosis. Greater survival at ideal weight, lung function association with body mass and weight are all supported with cited data and graphics on slide 8. While the company has not discussed the opportunity for an orphan indication for severe EPI, AzurRx estimates about 7,000 to 8,000 patients per year are in this category. This is well below the orphan population threshold of 200,000 and it is an unmet need. However, definitions matter and AzurRx will need the FDA to agree to a definition of an appropriate population before any expedited treatment would be granted to MS1819. Preliminary data is expected in early 2020.

Exhibit III – Phase II Severe EPI Combination Study[5]

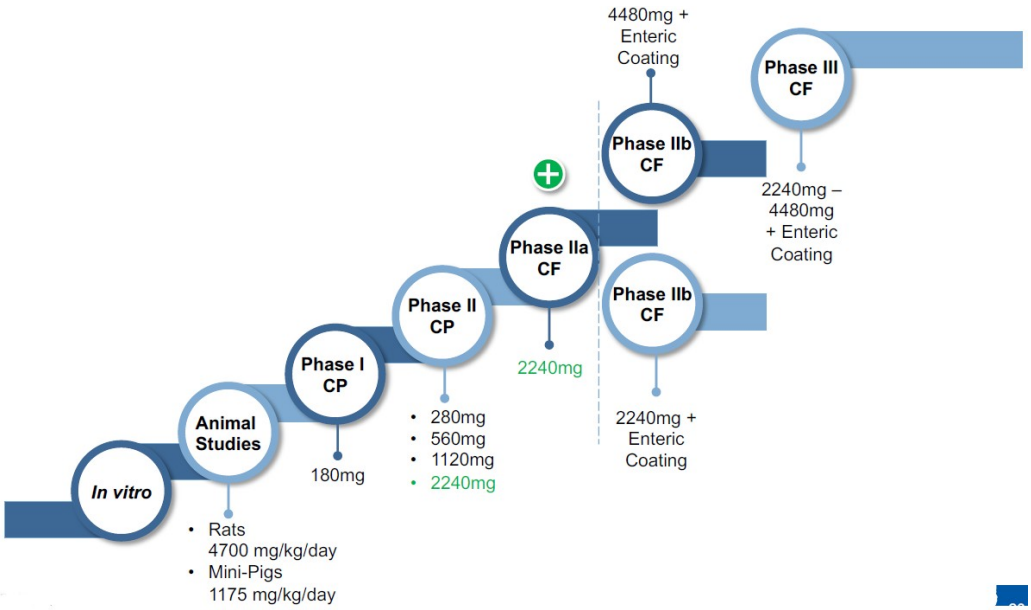


Pathway Forward

To date, AzurRx has completed preclinical, Phase I and two Phase II trials in chronic pancreatitis and cystic fibrosis patients with exocrine pancreatic insufficiency. Data from the most recent Phase II CF trial demonstrated safety, non-inferiority in half of patients compared to varying doses of PERT and a high CNA of 93%, supporting the hypothesis that a protease is not needed for MS1819 therapy. The trial also demonstrated that about half of all patients achieved a CFA sufficient to reach non-inferiority with PERT. Next steps require that an optimal dose be determined and further study be conducted in a Phase IIb or Phase IIb/III study. AzurRx has identified a few mechanisms to increase the activity of its lipase, either by increasing the dose or using enteric coated capsules that will prevent the enzyme from breaking down prior to its reaching the duodenum. The company will meet with the FDA before year end to seek guidance on trial design and to determine the most effective pathway forward. Simultaneous with the work on a registrational trial, AzurRx is also conducting a small study in an orphan population of patients with severe EPI, which could get to market earlier if rare disease status is conferred.

[5] Source: AzurRX Corporate Presentation, November 2019.

Exhibit IV – MS1819 Trial Map[6]



Year to Date Highlights

- MS1819
  - First patient enrolled in CF (OPTION) study – 1Q:19
  - Patent issued in US and Japan for Treatment of *H. Pylori* Infections Using MTAN Inhibitors
  - Public offerings of stock totaling ~$10 MM – April, May & July 2019
  - Presentation of MS1819 in CP – May 2019
  - Complete enrollment of OPTION study – mid-2019
  - Combination PERT study launch – 3Q:19
  - Topline release from OPTION study – September 2019
  - Publication of data for Phase II CP study at conference – mid-2019
  - FDA Meeting for MS1819 regarding CF – 4Q:19
  - Initial results of CF combination study – 1H:20

Company Assets

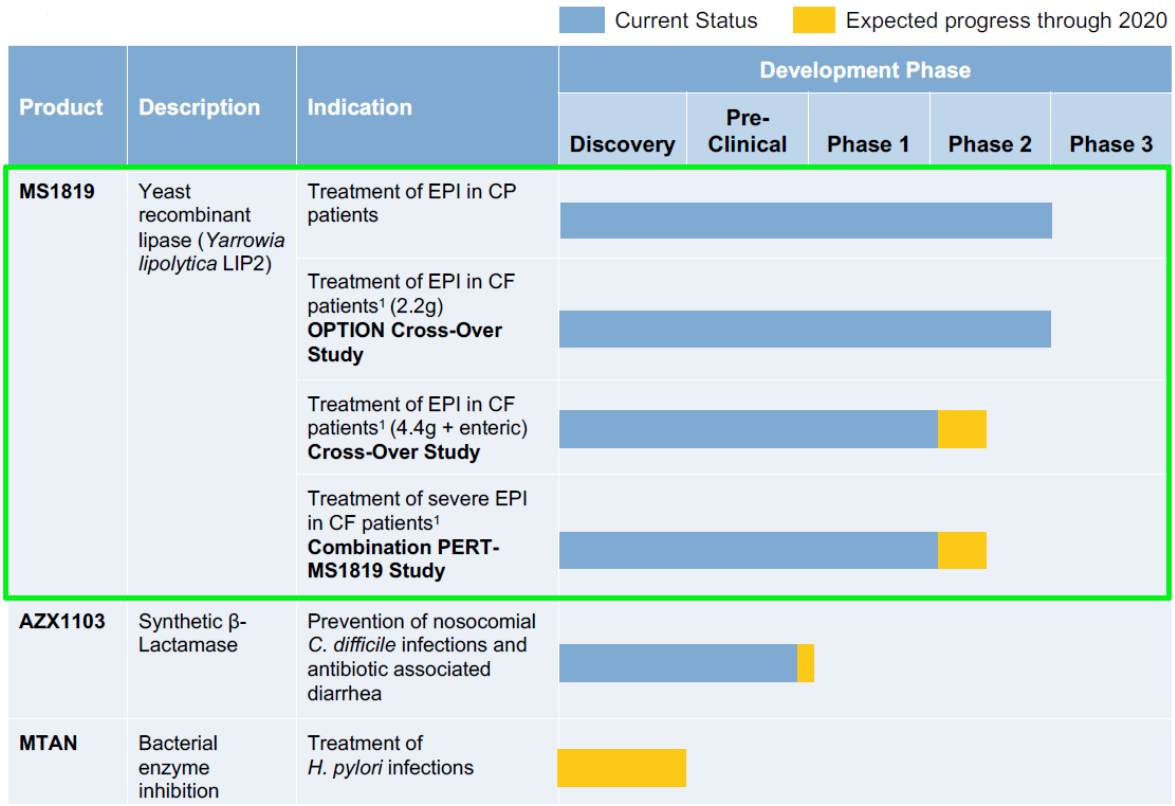
MS1819, is a yeast-derived lipase enzyme used to compensate for exocrine pancreatic insufficiency (EPI). The compound has several superior characteristics compared to standard EPI therapy, demonstrating increased efficacy in low pH environments and derivation from a non-porcine source. Currently MS1819 is being prepared for a second Phase IIb/III trial which we anticipate will launch in 2020.

AZX1103 is AzurRx's second compound in development. This is a recombinant β-lactamase derived from a bacterial source to address hospital-acquired infections acquired as a result of antibiotic use. AZX1103 provides evidence of positive pre-clinical activity and degradation of amoxicillin in the presence of clavulanic acid in the upper gastrointestinal tract in the Gottingen minipig model. The candidate is in pre-clinical development and AzurRx plans to file an investigational new drug (IND) application depending on funding availability. While the market opportunity is substantial, due to the early stage of development we do not attach any value to the β-lactamase program in our analysis.

MTAN (methylthioadenosine/S-adenosylhomocysteine nucleosidase) is a multifunctional enzyme that has the potential to safely eradicate Helicobacter pylori (*H. pylori*) bacterial infections while sparing the normal microbiome and without precipitating antibiotic resistance in off-target bacterial species. AzurRx has licensed the rights to the patents and presents MTAN inhibitor in its pipeline at the discovery stage.

[6] Source: AzurRX Corporate Presentation, November 2019.

Exhibit V – AzurRx Pipeline[7]



Product	Description	Indication	Development Phase				
			Discovery	Pre-Clinical	Phase 1	Phase 2	Phase 3
MS1819	Yeast recombinant lipase (*Yarrowia lipolytica* LIP2)	Treatment of EPI in CP patients					
		Treatment of EPI in CF patients[1] (2.2g) **OPTION Cross-Over Study**					
		Treatment of EPI in CF patients[1] (4.4g + enteric) **Cross-Over Study**					
		Treatment of severe EPI in CF patients[1] **Combination PERT-MS1819 Study**					
AZX1103	Synthetic β-Lactamase	Prevention of nosocomial *C. difficile* infections and antibiotic associated diarrhea					
MTAN	Bacterial enzyme inhibition	Treatment of *H. pylori* infections					

Summary

AzurRx has had a busy second half of 2019 with a new trial announced and the completion of its OPTION study. With the results demonstrating non-inferiority to half of PERT patients on varying doses of the porcine therapy, the path forward requires the identification of a proper dose and the launch of a registrational trial. Management has advised that there will be a meeting before year end with the FDA to determine next steps which could include a Phase IIb or a Phase IIb/III trial.

We maintain our favorable view on both the success of MS1819 in clinical trials and the need for a more potent and non-porcine source of fat-digesting enzymes. With the OPTION trial now complete and an experienced clinical team led by Dr. Pennington, we anticipate an efficient and uneventful enrollment of the next stage of MS1819 development early in 2020. We maintain our price target of $5.00 per share.

[7] Source: AZRX August 2019 Corporate Presentation

PROJECTED FINANCIALS

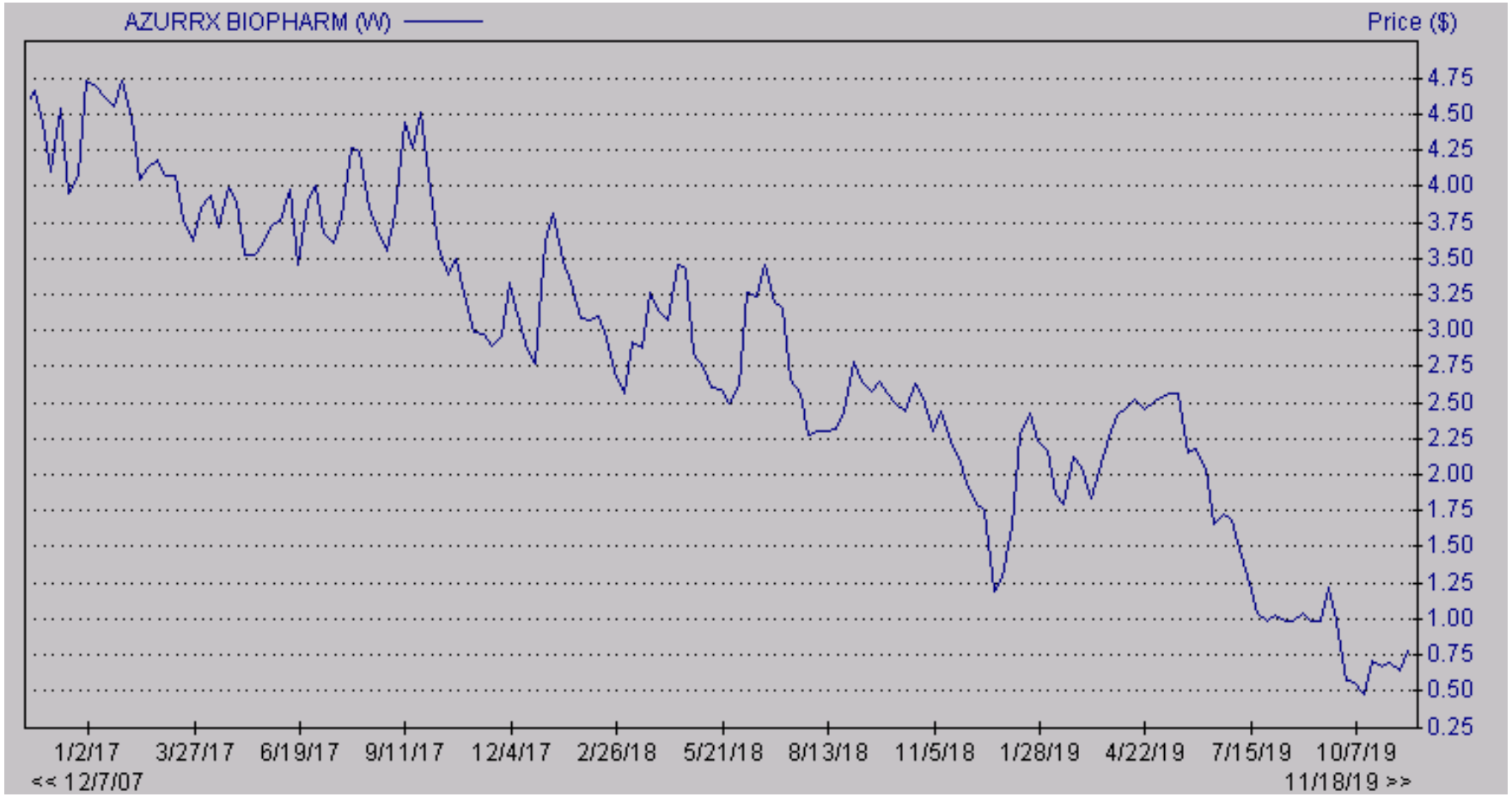
AzurRx BioPharma, Inc. - Income Statement

AzurRx Biopharma	2018 A	Q1 A	Q2 A	Q3 A	Q4 E	2019 E	2020 E	2021 E
Total Revenues	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**
R&D	$5.0	$2.1	$2.7	$2.2	$2.0	$9.1	$9.5	$9.6
G&A	$8.2	$2.5	$2.2	$1.9	$2.4	$9.0	$9.9	$10.0
Operating Income	($13.2)	($4.6)	($4.9)	($4.1)	($4.4)	($18.0)	($19.4)	($19.6)
Operating Margin	-	-	-	-	-	-	-	-
Interest Expense	($0.1)	($0.1)	($0.1)	($0.1)	($0.1)	($0.3)	($0.1)	$0.0
Fair Value Adjustment	($0.2)	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Total Other Income	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Pre-Tax Income	($13.5)	($4.7)	($5.0)	($4.2)	($4.5)	($18.3)	($19.5)	($19.6)
Taxes & Other	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0
Tax Rate	*0%*	*0*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*
Net Income	($13.5)	($4.7)	($5.0)	($4.2)	($4.5)	($18.3)	($19.5)	($19.6)
Reported EPS	**($0.88)**	**($0.26)**	**($0.25)**	**($0.17)**	**($0.17)**	**($0.82)**	**($0.72)**	**($0.70)**
YOY Growth	-	-	-	-	-	-	-	-
Shares Outstanding	15.4	17.7	20.5	25.0	26.5	22.4	27.0	28.0

Source: Company Filing // Zacks Investment Research, Inc. Estir

HISTORICAL STOCK PRICE

AzurRx BioPharma, Inc. – Historical Stock Price



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