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AzurRx BioPharma, Inc. (AZRX - NASDAQ)

Next Up: OPTION 2

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.

Current Price (4/3/2020)	$0.82
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 study which will employ enteric capsules, allowing more drug to reach the duodenum. The drug is an orally delivered, non-systemic, yeast-derived recombinant enzyme. It addresses EPI found in chronic pancreatitis or cystic fibrosis patients.

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	**2.71**
52-Week Low	**0.37**
One-Year Return (%)	**-68.2**
Beta	**1.93**
Average Daily Volume (sh)	**277,553**
Shares Outstanding (mil)	**28.2**
Market Capitalization ($mil)	**22.3**
Short Interest Ratio (days)	**0.31**
Institutional Ownership (%)	**5.1**
Insider Ownership (%)	**17.8**
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)
2019	$0.0 A	$0.0 A	$0.0 A	$0.0 A	$0.0 A
2020	$0.0 A	$0.0 E	$0.0 E	$0.0 E	$0.0 E
2021					$0.0 E
2022					$0.0 E

Earnings per Share

	Q1 (Mar)	Q2 (Jun)	Q3 (Sep)	Q4 (Dec)	Year (Dec)
2019	-$0.26 A	-$0.25 A	-$0.17 A	-$0.05 A	-$0.68 A
2020	-$0.19 A	-$0.09 E	-$0.10 E	-$0.11 E	-$0.47 E
2021					-$0.36 E
2022					-$0.24 E

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What's New

On May 15th, 2020, AzurRx BioPharma, Inc. (NASDAQ: AZRX) filed its first quarter 2020 10-Q with the SEC for the three month period ending March 31, 2020. Highlights year to date include the appointment of a new CFO and board member, receipt of ~$3.5 million in French tax credits, confirmation of manufacturing and distribution agreements, and IRB approval to launch the OPTION 2 trial which should begin in the next few weeks. We expect to see an academic manuscript available for the OPTION study this fall at the North American Cystic Fibrosis Conference.

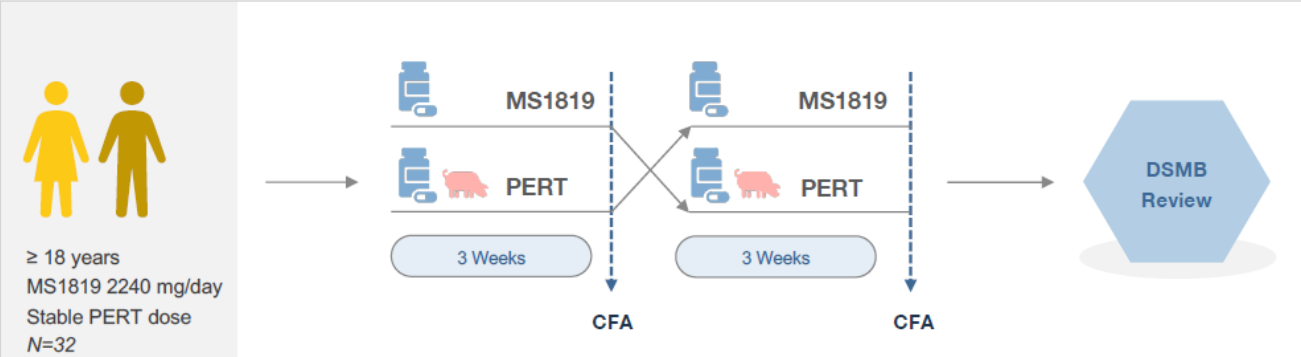
As expected, no revenues were reported during the first quarter of either 2019 or 2020. Operating expenses for the 1Q:20 period were $2.9 million, down 36% compared to the prior year. General and administrative expenses were $1.4 million, down 45% on a host of lower expense items, including stock compensation, legal, director's fees, corporate communications and rent. These declines were offset by increases in other items including consulting, insurance, personnel, travel and entertainment and IT administration. Research and development expenses contracted 27% to $1.4 million. The change was attributed to lower stock based compensation, depreciation and amortization, direct clinical trial costs, consulting and personnel costs.

Cash on the balance sheet was $1.6 million and notes payable and convertible debt were held at $3.3 million as of March 31, 2020. Cash burn for the three month period was ($1.3) million which compares with ($2.6) million for the same quarter last year. Following the end of the reporting period, AzurRx issued 788,464 shares to Lincoln Park for proceeds of $457 thousand and received $1.4 million from the French tax credit.

Option Trial

AzurRx announced Phase II OPTION trial results for MS1819 in cystic fibrosis (CF) patients in late September 2019. Summary data highlighted the positive safety profile for MS1819 and the utility of the drug compared to 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 of its arms, which supports the assertion that the digestive system is able to break down proteins using MS1819 therapy. 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.

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

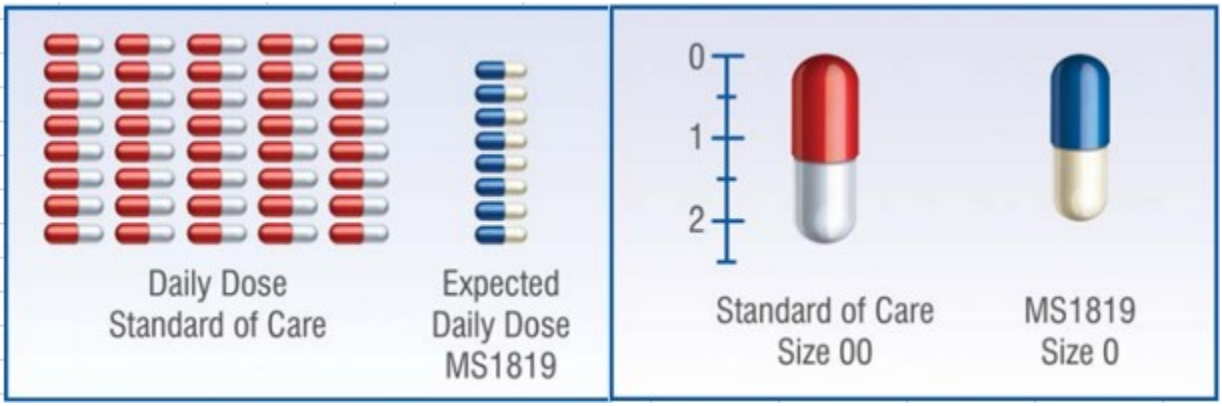


It is important to clarify that the doses used in the PERT control arm varied widely, from 2,700 to 9,400 lipase units per kilogram per day, while doses used in MS1819 were stable at 2.2 g/d. The study did not provide a direct comparison between comparable and consistent doses of drug for PERT and MS1819. The justification for this coarse comparison was a mandate from the FDA requiring the OPTION study to emphasize safety over efficacy. The FDA did not allow higher doses of MS1819 to be used at this stage of development. The 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] Source: AzurRx Corporate Presentation, May 2020.

A concern 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 question regarding MS1819 has been that there may be insufficient protein breakdown compared to PERT, which contains all three enzymes. While carbohydrate digestion had not been a worry, it was not determined whether proteins were being sufficiently digested without the protease component in MS1819.

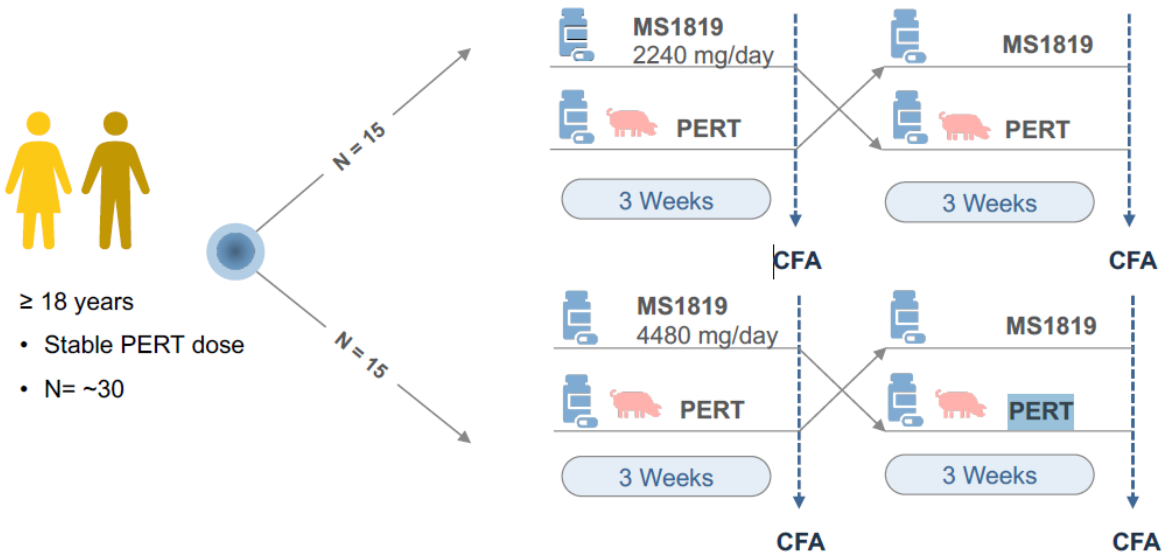
Exhibit II – PERT vs. MS1819 Pill Burden[2]



OPTION 2 Trial

AzurRx will soon launch its next trial which will determine the optimal dose for the Phase III study. It will be a Phase IIb, open label, 2x2 crossover design designated the OPTION 2 Enteric Dose-Escalation Trial. The target population will be patients with EPI due to CF. Based on the results in the completed OPTION 1 trial, the scientific team will add the use of enteric capsules[3] to delay release of MS1819 until it reaches the lower gastrointestinal tract. The shift to enteric capsules was made after it was determined that approximately half of MS1819 was inactivated in the stomach. The enteric capsule will prevent the drug from degrading until it reaches the duodenum. The FDA is familiar with the enteric capsule and we do not anticipate any difficulties or requirements for additional studies with this change in MS1819's formulation.

Exhibit III – OPTION 2 Trial Design[4]

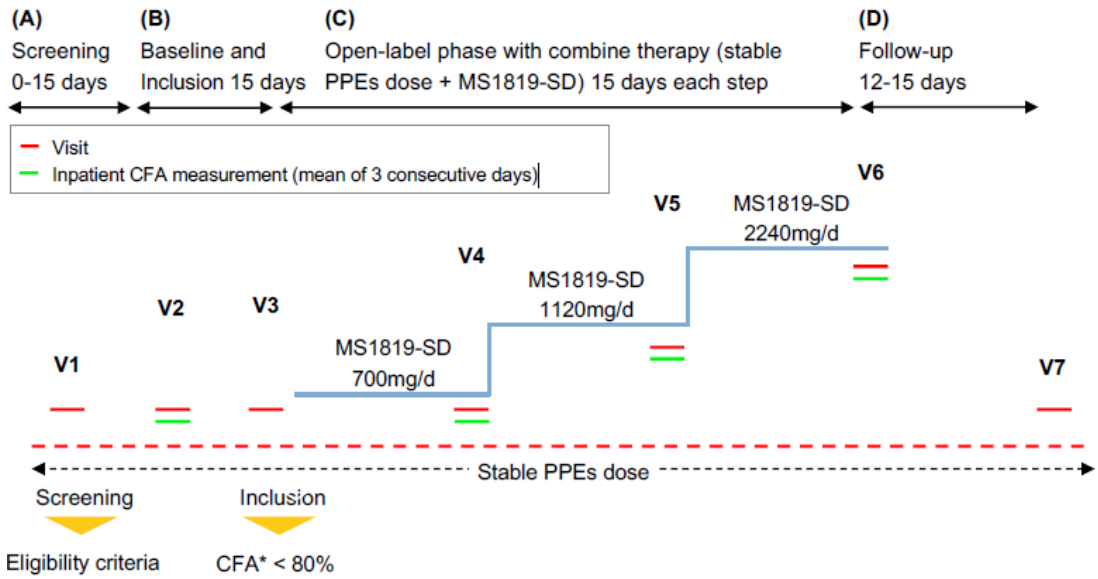


[2] Source: AzurRx Corporate Presentation, May 2020
[3] This is in contrast to the hydroxypropyl methylcellulose (HPMC) capsules that were used in previous trials and used in the ongoing combination study for severe EPI patients.
[4] Source: AzurRx Corporate Presentation, May 2020.

Severe EPI Trial

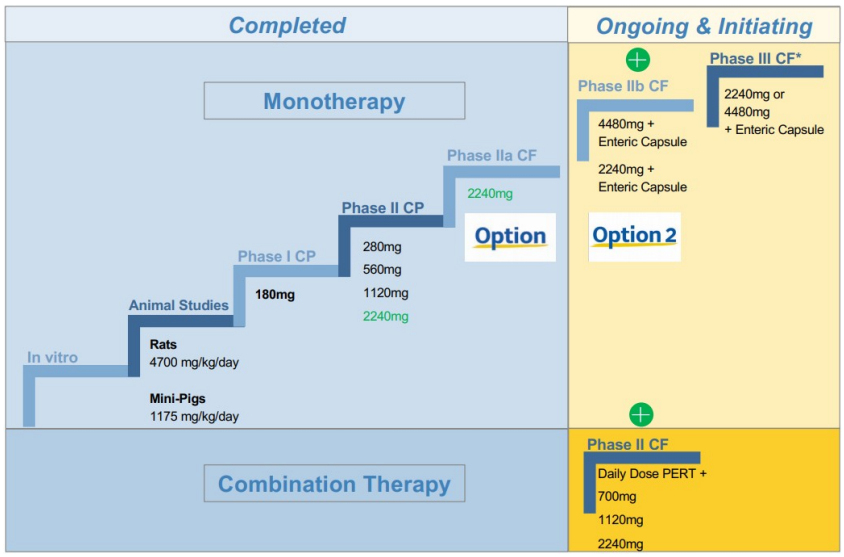
In early July 2019, AzurRx launched a Phase II trial investigating MS1819-SD in combination with standard pancreatic enzyme replacement therapy (PERT) for patients with cystic fibrosis (CF) that suffer from severe exocrine pancreatic insufficiency (EPI). These patients are unable to maintain weight and suffer from fat malabsorption despite taking the maximum dose of PERT. This population with an unmet need may be suitable for an expedited pathway to approval for MS1819-SD. The study will be conducted at multiple European sites including locations in Hungary and Spain with an enrollment target of 24 and a primary endpoint of safety and CFA greater than 80%.

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



The combination therapy is expected to correct nutrient maldigestion, eliminate abdominal symptoms attributable to maldigestion and sustain optimal nutritional status on a normal diet in CF patients with severe EPI. While the company has not discussed the opportunity for an orphan indication for severe EPI, it 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 the characteristics of an appropriate population before any expedited treatment would be granted to MS1819. First patients were dosed in mid-October and preliminary data is expected in 2020. We discuss this study in more detail here.

Exhibit V – MS1819 Trial Map[6]

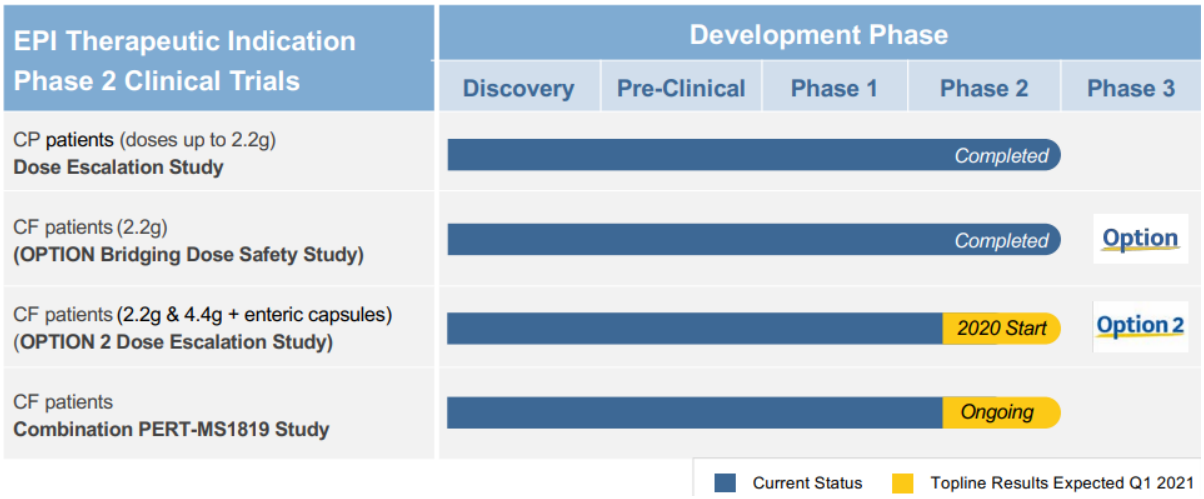


[5] Source: AzurRX Corporate Presentation, May 2020.
[6] Source: AzurRX Corporate Presentation, May 2020

Agreements

AzurRx extended two agreements to manufacture and package, label and distribute MS1819 for the Phase II trials. In April, Delpharm was selected to act as the contract development manufacturing organization (CDMO) to manufacture drug product supply of MS1819 for the OPTION 2 trial. Delpharm is a French manufacturer of pharmaceutical products with 17 production sites in Europe and Canada. As of April 2020, a preliminary batch was manufactured in preparation for a current good manufacturing practices (cGMP) batch using the new enteric capsule formulation. Despite production work starting during the coronavirus pandemic, there have been no delays to its production and no delays are expected in the effort to launch the OPTION 2 study in 2Q:20. Shared in an early May release, Creapharm was selected to provide packaging, labelling and distribution of enteric drug product for use at Phase II trial sites throughout Europe and the United States. Creapharm is a French company with packaging plants in France and the United States. The partner will send packaged product to AzurRx trial sites in Poland, Hungary, Spain, Turkey and the United States. No pandemic-related delays are expected in Creapharm's efforts.

Exhibit VI – AzurRx Pipeline[7]



Historical and Forward Looking Highlights

- Receipt of French Tax Credits – 1Q:20/2Q:20
- Various capital raises – 1Q:20
- Delpharm Manufacturing Agreement – April 2020
- TDN & IRB Approval to Start OPTION 2 Trial – April 2020
- Creapharm Packaging, Labeling & Distribution Agreement – May 2020
- Abstract Presentation at Digestive Disease Week – May 2020
- Launch of OPTION 2 trial – 2Q:20
- Presentation of Phase II CF study at N. American CF Conference – October 2020
- Initial results of CF combination study – 1Q:21, coronavirus dependent
- Completion of OPTION 2 trial – 1Q:21, coronavirus dependent

[7] Source: AzurRX Corporate Presentation, May 2020

Pathway Forward

To date, AzurRx has completed preclinical Phase I and 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 effective MS1819 therapy. Next steps require that an optimal dose be determined and further study be conducted in the OPTION 2 Phase IIb study which AzurRx expects to launch in 2Q:20. MS1819 will use an enteric capsule to determine the optimal dose for a registrational trial in 2021. Concurrent with the work on the OPTION 2 trial, AzurRx is conducting a small study in a small population of patients with severe EPI. In late April, AzurRx received approval to start the OPTION 2 clinical trial by the Therapeutics Development Network (TDN), which is a key partner in identifying cystic fibrosis patients to populate the company's clinical trials. The sanction by the TDN allows the OPTION 2 trial to access up to 91 accredited US care centers that specialize in CF. Shortly after the TDN announcement, AzurRx also received Institutional Review Board approval to launch the OPTION 2 trial at US sites for CF patients with EPI.

Summary

AzurRx has had a productive 2019 with the completion of the OPTION trial and the launch of the combination study in patients with severe EPI. 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 launch of OPTION 2 imminent, we anticipate another successful trial with results available by 1Q:21. Although AzurRx has been fortunate with no material delays so far related to sourcing of drug product for trials, coronavirus related disruptions could delay the trial and we will update investors as additional information becomes available.

MS1819 is a naturally derived product and received the "Generally Recognized as Safe" (GRAS) designation from the FDA. The lipase 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. We maintain our price target of $5.00 per share.

PROJECTED FINANCIALS

AzurRx BioPharma, Inc. - Income Statement

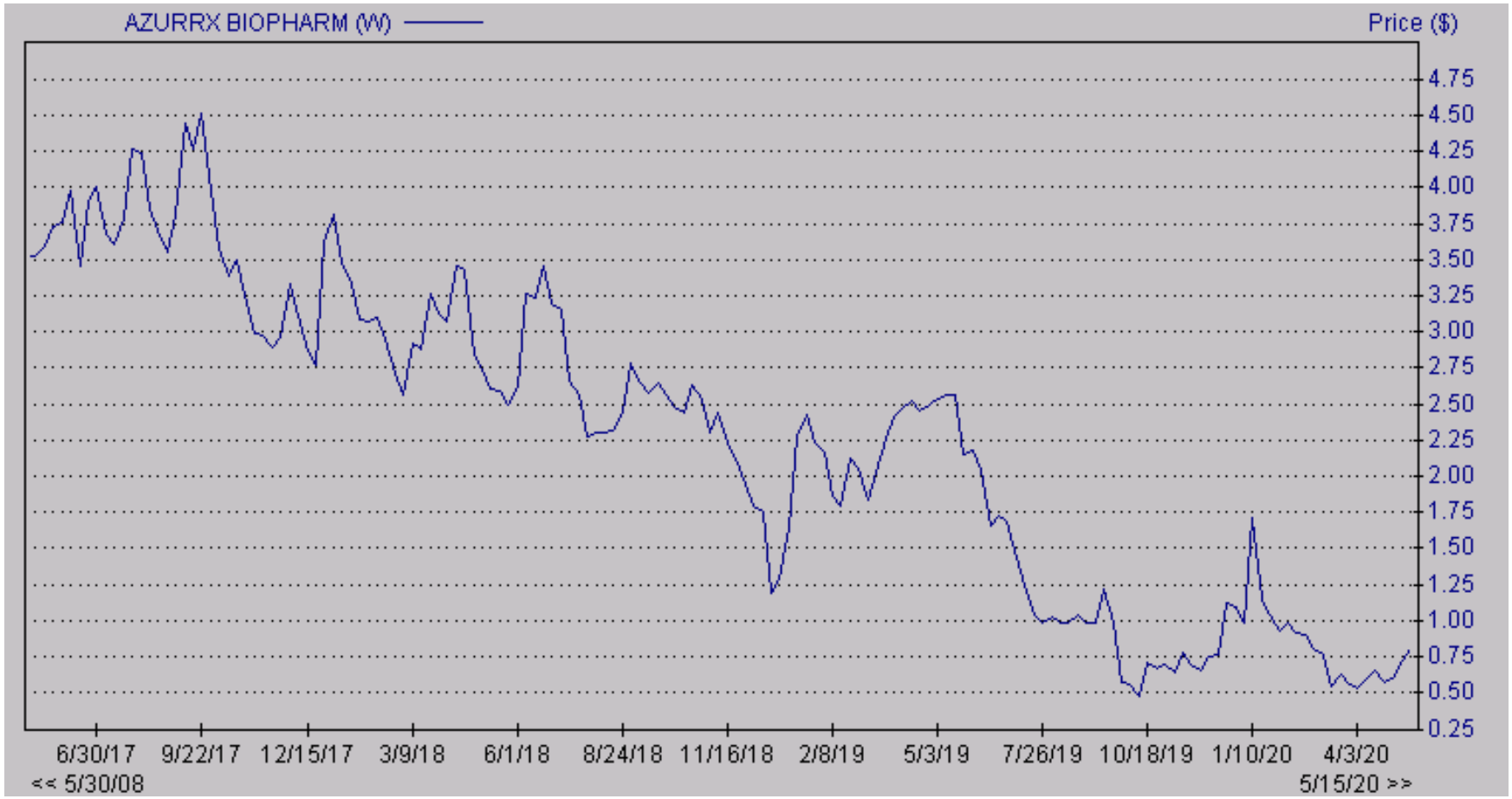
AzurRx Biopharma	2019 A	Q1 A	Q2 E	Q3 E	Q4 E	2020 E	2021 E	2022 E
Total Revenues	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**
R&D	$8.7	$1.6	$2.0	$2.4	$2.6	$8.6	$9.6	$9.5
G&A	$6.1	$1.4	$1.6	$1.8	$1.8	$6.6	$8.5	$9.0
Operating Income	($14.7)	($2.9)	($3.6)	($4.2)	($4.4)	($15.1)	($18.1)	($18.5)
Operating Margin	-	-	-	-	-	-	-	-
Interest Expense	($0.4)	($2.3)	($0.0)	($0.0)	($0.0)	($2.4)	$0.0	$0.0
Pre-Tax Income	($15.2)	($5.1)	($3.6)	($4.2)	($4.4)	($17.5)	($18.1)	($18.5)
Taxes & Other	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$0.0	$1.0
Tax Rate	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*0%*	*100%*
Net Income	($15.2)	($5.1)	($3.6)	($4.2)	($4.4)	($17.5)	($18.1)	($19.5)
Reported EPS	**($0.68)**	**($0.19)**	**($0.09)**	**($0.10)**	**($0.11)**	**($0.47)**	**($0.36)**	**($0.24)**
YOY Growth	-	-	-	-	-	-	-	-
Shares Outstanding	22.4	26.9	40.0	41.0	42.0	37.5	50.0	80.0

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

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HISTORICAL STOCK PRICE

AzurRx BioPharma, Inc. – Historical Stock Price



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