Zacks Small-Cap Research

Sponsored – Impartial - Comprehensive

April 6, 2020

John D. Vandermosten, CFA
312-265-9588 / jvandermosten@zacks.com

scr.zacks.com **10 S. Riverside Plaza, Suite 1600, Chicago, IL 60606**

AzurRx BioPharma, Inc. (AZRX - NASDAQ)

Tax Credits Augment Cash Flow

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)	$0.55
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	**3.10**
52-Week Low	**0.37**
One-Year Return (%)	**-79.0**
Beta	**2.1**
Average Daily Volume (sh)	**477,496**
Shares Outstanding (mil)	**27.1**
Market Capitalization ($mil)	**14.9**
Short Interest Ratio (days)	**0.84**
Institutional Ownership (%)	**6.0**
Insider Ownership (%)	**18.0**
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 A	$0.0 A
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	-$0.05 A	-$0.68 A
2020					-$0.52 E
2021					-$0.44 E

© Copyright 2020, Zacks Investment Research. All Rights Reserved.

What's New

AzurRx BioPharma, Inc. (NASDAQ: AZRX) filed its 2019 10-K with the SEC for the twelve month period ending December 31, 2019. Highlights for the reporting period and to date include the readout from the OPTION trial, the acquisition of global commercialization rights for MS-1819, the launch of a new combination trial for patients with severe exocrine pancreatic insufficiency (EPI), the addition of a new CEO and CFO, a positive Data Safety Monitoring Board (DSMB) review of the OPTION trial and the receipt of additional capital including $1.8 million in cash R&D tax credits. We expect to see an abstract and academic manuscript available for the OPTION study during 2Q:20.

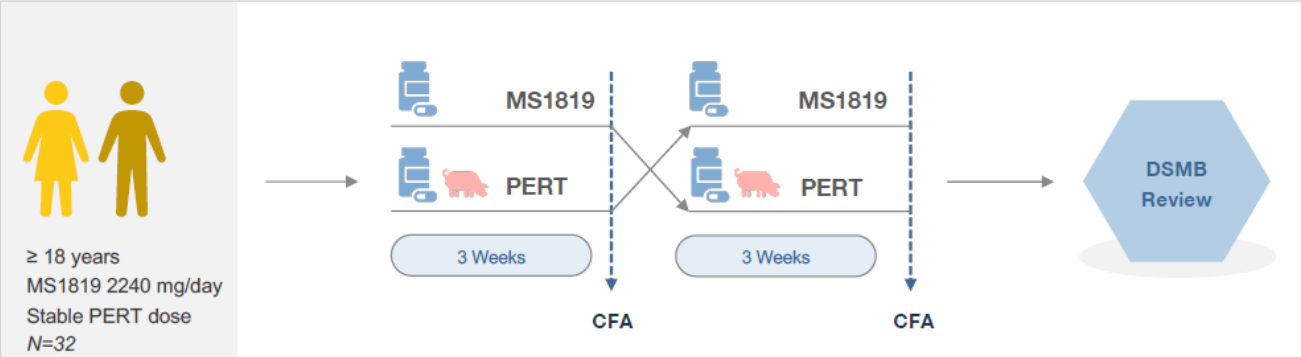
No revenues were reported for either 2019 or 2018 for this development stage company. Operating expenses for the most recent completed year were $14.7 million, up 12% compared to 2018. General and administrative expenses were $6.1 million, down 26% on a year over year basis on lower stock expense. Research and development expenses expanded 74% to $8.7 million. Increases were attributed to the startup of a research and development function in the U.S. including expenses allocated to the OPTION and new combination study.

Cash on the balance sheet was $176,000 and notes payable and convertible debt were held at $1.5 million as of December 31, 2019. Cash burn for the twelve month period was ($14.1) million which compares to ($10.9) million for 2018. Following the end of the reporting period, AzurRx closed a $6.9 million private placement and received $1.77 million from the French research tax credit. The company also secured a $15 million equity purchase agreement with Lincoln Park Capital in November 2019 and drew upon 150,000 shares of the arrangement for proceeds of $144,000.

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 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 of its arms, which indicates the ability of the digestive system to break down proteins while undergoing 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]



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. The study did not provide 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 emphasize safety over efficacy. The FDA did not allow higher doses 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, March 2020.

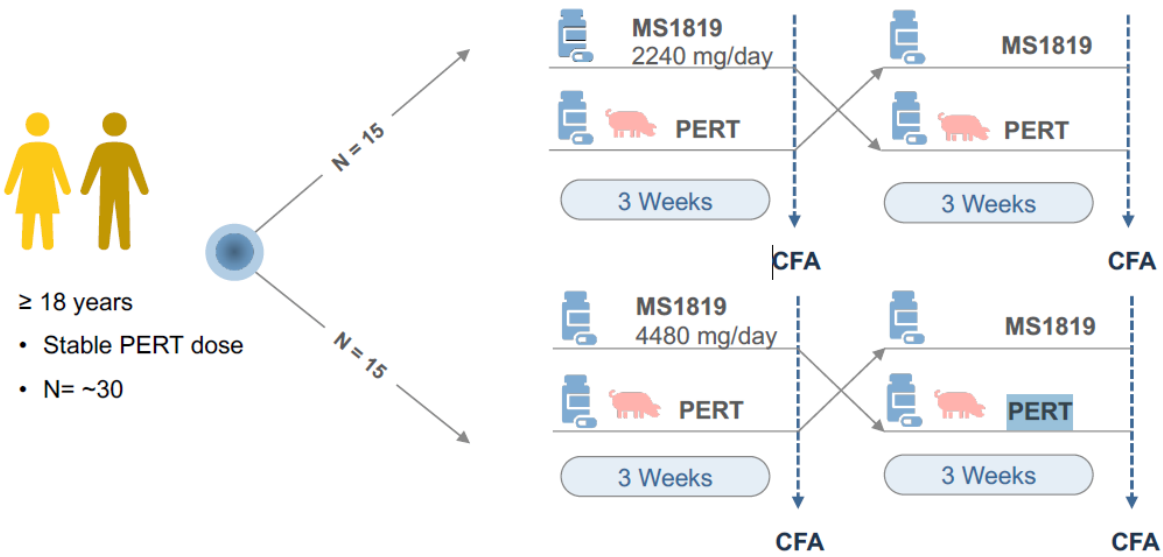
A recurring question regarding the comparable efficacy of MS1819 with PERT relates to the enzymes that are present in the two drugs. MS1819 only contains lipases; however, digestion requires lipases, proteases and amylases. While the pancreas secretes all three enzymes, proteases and amylases are also 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 the protease component in MS1819.

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 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. Note that these numbers are taken from different studies whose populations have different characteristics. 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 break down nutrients. MS1819 therapy slows the digestion process giving the protease and other enzymes time to break down fats, proteins and starches. This is a favorable outcome for MS1819 as is suggests that the drug can be used as a monotherapy in patients. We expect an abstract and academic manuscript will be published based on this data. The original target date for presentation and publication during and following the planned, but now cancelled, Digestive Disease Week which had been scheduled for the first week in May.

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 trial, the scientific team will add the use of enteric capsules to delay release of MS1819 until it reaches the lower gastrointestinal tract. The shift to enteric capsules was sought 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 II – OPTION 2 Trial Design[4]

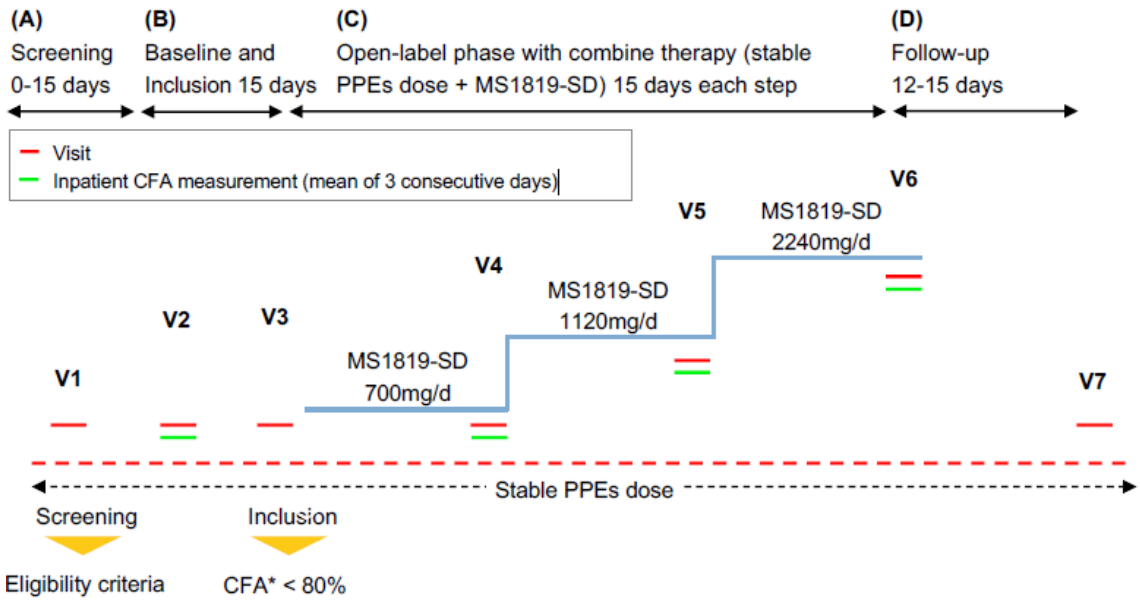


[2] Borowitz D, Konstan MW, O'Rourke A, Cohen M, Hendeles L, Murray FT. Coefficients of fat and nitrogen absorption in healthy subjects and individuals with cystic fibrosis. J Pediatr Pharmacol Ther. 2007;12(1):47-52.
[3] https://www.creon.com/hcp/efficacy
[4] Source: AzurRx Corporate Presentation, March 2020.

Severe EPI Trial

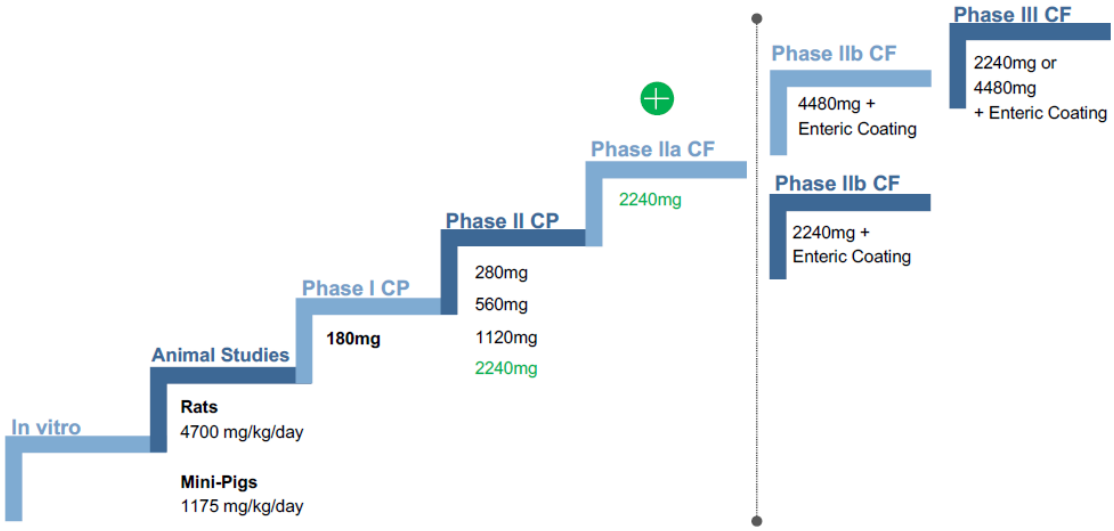
In early July 2019, AzurRx launched a Phase II trial investigating MS1819-SD in combination with standard pancreatic enzyme replacement therapy (PERT) for patients with cystic fibrosis (CF) that suffer from severe exocrine pancreatic insufficiency (EPI). These patients are unable to maintain weight and suffer from fat malabsorption despite taking the maximum dose of PERT. This population with an unmet need may provide 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 III – 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 IV – MS1819 Trial Map[6]

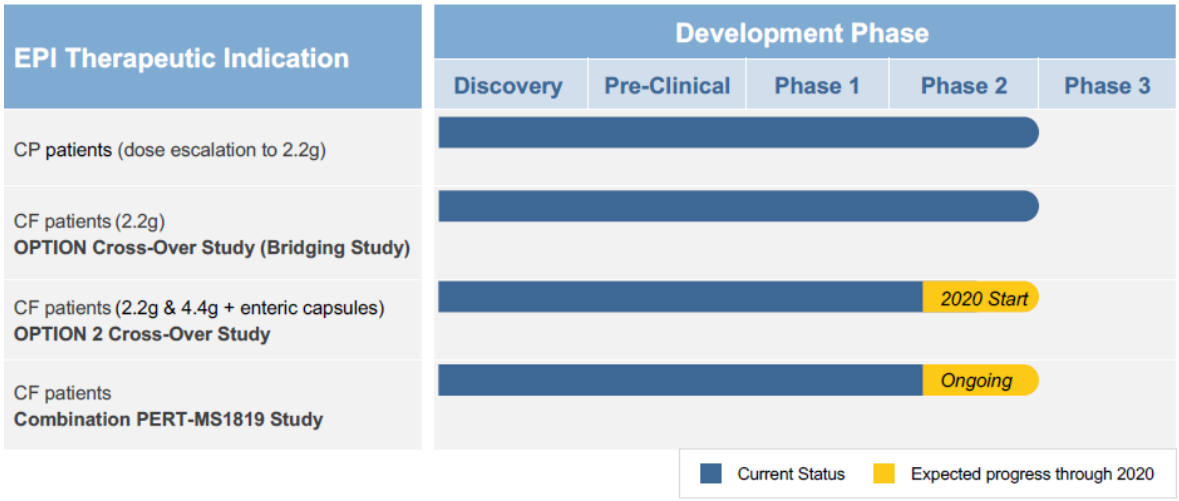


[5] Source: AzurRX Corporate Presentation, March 2020.
[6] Source: AzurRX Corporate Presentation, March 2020

New Management Team

During the third and fourth quarter of 2019 CEO Thijs Spoor[7] and CFO Maged Shenouda resigned respectively to assume positions at other companies. These executive positions were assumed by James Sapirstein as CEO and Daniel Schneiderman as CFO. Mr. Sapirstein was previously CEO of ContraVir Pharmaceuticals, Alliqua Therapeutics and Tobira Therapeutics. Mr. Schneiderman was previously CFO of Biophytis and VP of Finance, Controller and Secretary of MetaStat.

Exhibit V – AzurRx Pipeline[8]



Historical and Forward Looking Highlights

- First patient enrolled in CF (OPTION) study – 1Q:19
- 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
- CF Foundation review of OPTION study finds no safety concerns – October 2019
- First patients dosed in Combination PERT study – October 2019
- FDA Meeting for MS1819 regarding CF – 4Q:19
- Various capital raises – 1Q:20
- Publication of data for Phase II CF study – 2Q:20
- Launch of OPTION 2 trial – 2Q:20
- Initial results of CF combination study – 2H:20, coronavirus dependent
- Completion of OPTION 2 trial – prior to year-end 2020, coronavirus dependent

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 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. Simultaneous with the work on the OPTION 2 trial, AzurRx is conducting a small study in an orphan population of patients with severe EPI, providing a pathway which could provide a faster route to market if rare disease status is conferred.

[7] Thijs Spoor now serves as director on the board of AzurRx and is CEO of KBP Biosciences.
[8] Source: AzurRX Corporate Presentation, March 2020

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 in either 4Q:20 or 1Q:21. We do note that coronavirus issues could delay the trial and we will update investors as additional information is available. MS1819 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. We maintain our price target of $5.00 per share.

PROJECTED FINANCIALS

AzurRx BioPharma, Inc. - Income Statement

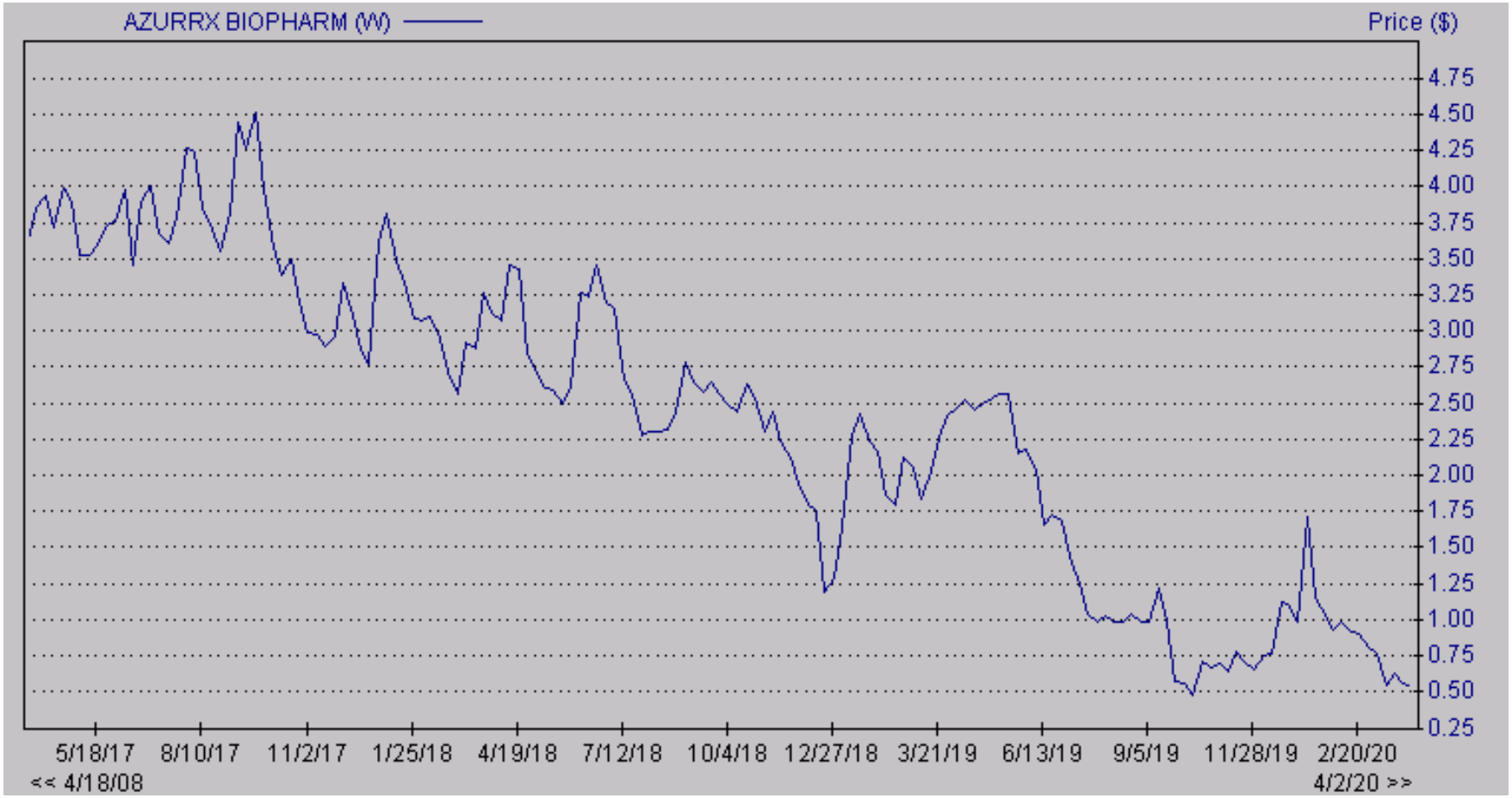
AzurRx Biopharma	2018 A	Q1 A	Q2 A	Q3 A	Q4 A	2019 A	2020 E	2021 E
Total Revenues	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**	**$0.0**
R&D	$5.0	$2.1	$2.7	$2.2	$1.6	$8.7	$9.5	$9.6
G&A	$8.2	$2.5	$2.2	$1.9	($0.5)	$6.1	$9.9	$10.0
Operating Income	($13.2)	($4.6)	($4.9)	($4.1)	($1.1)	($14.7)	($19.4)	($19.6)
Operating Margin	-	-	-	-	-	-	-	-
Interest Expense	($0.1)	($0.1)	($0.1)	($0.1)	($0.2)	($0.4)	($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)	($1.3)	($15.2)	($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)	($1.3)	($15.2)	($19.5)	($19.6)
Reported EPS	**($0.88)**	**($0.26)**	**($0.25)**	**($0.17)**	**($0.05)**	**($0.68)**	**($0.52)**	**($0.44)**
YOY Growth	-	-	-	-	-	-	-	-
Shares Outstanding	15.4	17.7	20.5	25.0	26.5	22.4	37.5	45.0

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

© Copyright 2020, Zacks Investment Research. All Rights Reserved.

HISTORICAL STOCK PRICE

AzurRx BioPharma, Inc. – Historical Stock Price



DISCLOSURES

The following disclosures relate to relationships between Zacks Small-Cap Research ("Zacks SCR"), a division of Zacks Investment Research ("ZIR"), and the issuers covered by the Zacks SCR Analysts in the Small-Cap Universe.

ANALYST DISCLOSURES

I, John Vandermosten, hereby certify that the view expressed in this research report accurately reflect my personal views about the subject securities and issuers. I also certify that no part of my compensation was, is, or will be, directly or indirectly, related to the recommendations or views expressed in this research report. I believe the information used for the creation of this report has been obtained from sources I considered to be reliable, but I can neither guarantee nor represent the completeness or accuracy of the information herewith. Such information and the opinions expressed are subject to change without notice.

INVESTMENT BANKING AND FEES FOR SERVICES

Zacks SCR does not provide investment banking services nor has it received compensation for investment banking services from the issuers of the securities covered in this report or article.
Zacks SCR has received compensation from the issuer directly, from an investment manager or from an investor relations consulting firm engaged by the issuer for providing non-investment banking services to this issuer and expects to receive additional compensation for such non-investment banking services provided to this issuer. The non-investment banking services provided to the issuer includes the preparation of this report, investor relations services, investment software, financial database analysis, organization of non-deal road shows, and attendance fees for conferences sponsored or co-sponsored by Zacks SCR. The fees for these services vary on a per-client basis and are subject to the number and types of services contracted. Fees typically range between ten thousand and fifty thousand dollars per annum. Details of fees paid by this issuer are available upon request.

POLICY DISCLOSURES

This report provides an objective valuation of the issuer today and expected valuations of the issuer at various future dates based on applying standard investment valuation methodologies to the revenue and EPS forecasts made by the SCR Analyst of the issuer's business.
SCR Analysts are restricted from holding or trading securities in the issuers that they cover. ZIR and Zacks SCR do not make a market in any security followed by SCR nor do they act as dealers in these securities. Each Zacks SCR Analyst has full discretion over the valuation of the issuer included in this report based on his or her own due diligence. SCR Analysts are paid based on the number of companies they cover. SCR Analyst compensation is not, was not, nor will be, directly or indirectly, related to the specific valuations or views expressed in any report or article.

ADDITIONAL INFORMATION

Additional information is available upon request. Zacks SCR reports and articles are based on data obtained from sources that it believes to be reliable, but are not guaranteed to be accurate nor do they purport to be complete. Because of individual financial or investment objectives and/or financial circumstances, this report or article should not be construed as advice designed to meet the particular investment needs of any investor. Investing involves risk. Any opinions expressed by Zacks SCR Analysts are subject to change without notice. Reports or articles or tweets are not to be construed as an offer or solicitation of an offer to buy or sell the securities herein mentioned.

© Copyright 2020, Zacks Investment Research. All Rights Reserved.