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AzurRx BioPharma, Inc. (AZRX - NASDAQ)

1H:21 Readout for OPTION 2 and Combo Trials

Based on our DCF model and a 15% discount rate, AZRX is valued at approximately $4.00 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 (8/21/2020)	$0.86
Valuation	**$4.00**

OUTLOOK

AzurRx employs recombinant protein technology to treat gastrointestinal diseases and microbiome related conditions using oral, non-systemic biologics. It is developing one clinical asset and is running two Phase II clinical trials.

The company recently completed a Ph2 trial for MS1819 in cystic fibrosis patients and has recently launched a Phase IIb study designated OPTION 2 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 cystic fibrosis or chronic pancreatitis patients.

A second trial for severe EPI, which combines MS1819 with PERT is underway. Interim readout for the trial shows an improvement in CFA despite only including five 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	**1.94**
52-Week Low	**0.37**
One-Year Return (%)	**-17.7**
Beta	**2.03**
Average Daily Volume (sh)	**405,843**
Shares Outstanding (mil)	**28.5**
Market Capitalization ($mil)	**24.5**
Short Interest Ratio (days)	**0.15**
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 A	$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	-$0.17 A	-$0.14 E	-$0.14 E	-$0.64 E
2021					-$0.33 E
2022					-$0.24 E

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What's New

On August 14th, 2020, AzurRx BioPharma, Inc. (NASDAQ: AZRX) filed its second quarter 2020 10-Q with the SEC for the three month period ending June 30, 2020. Second quarter and to date highlights include the receipt of tax credits, confirmation of manufacturing and distribution agreements, and IRB approval to start the OPTION 2 trial which was subsequently launched with first patients screened and dosed. Interim results from the combination trial were presented to investors, demonstrating early efficacy and safety for severe EPI patients. Following the end of the reporting period, AzurRx raised gross cash proceeds of $15.2 million and exchanged $6.9 million of notes and accrued interest, through the issuance of Series B Preferred Stock.

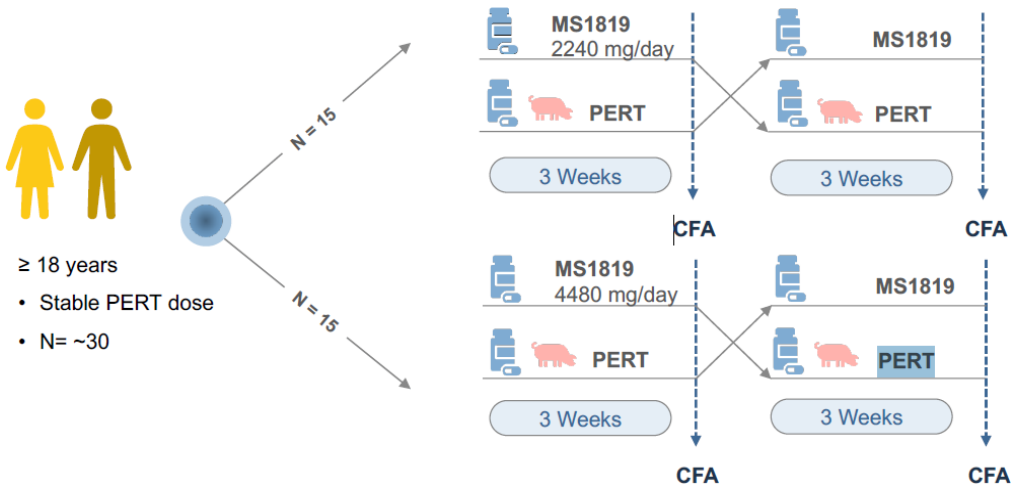
No revenues were reported during the second quarter. Operating expenses for 2Q:20 were $2.4 million, half of prior year levels. General and administrative expenses were $1.3 million, down 41% on a host of lower expense items, including the elimination of bonuses and reductions in communications expenses, directors' fees and travel and entertainment expenditures. These were partially offset by increased insurance, legal and information technology costs. Research and development spend dropped 60% to $1.1 million. The change was attributed to decreased clinical trial costs from the OPTION trial in the prior year period and recruitment delays in the combination study due to COVID-19 in the current year period. Personnel costs were also lower compared to the prior year period.

Cash on the balance sheet was $1.0 million and notes payable and convertible debt were held at $5.4 million as of June 30, 2020. Cash burn for the three month period was ($1.3) million which compares with ($4.0) million for the same quarter last year. Following the end of the reporting period, AzurRx raised gross cash proceeds of $15.2 million from the issuance of Series B Preferred Stock.

OPTION 2 Trial

AzurRx launched its Phase IIb clinical trial of MS1819 in Cystic Fibrosis (CF), designated OPTION 2, in late July which will determine the optimal dose for the Phase III study. OPTION 2 will be an open label, 2x2 crossover design with a target population of CF patients with EPI. 30 CF patients will be enrolled in the United States and Poland and as of mid-August, the first three patients had been dosed and nine of an expected 15 sites, six in the US and three in Poland, were active and recruiting patients. Management has guided towards a 1Q:21 availability of top line data. Based on the results in the completed OPTION 1 trial, the scientific team will add the use of enteric capsules[1] to delay release of MS1819 until it reaches the lower gastrointestinal tract and also include a trial arm with a higher dose of 4.4g. 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.

Exhibit I – OPTION 2 Trial Design[2]

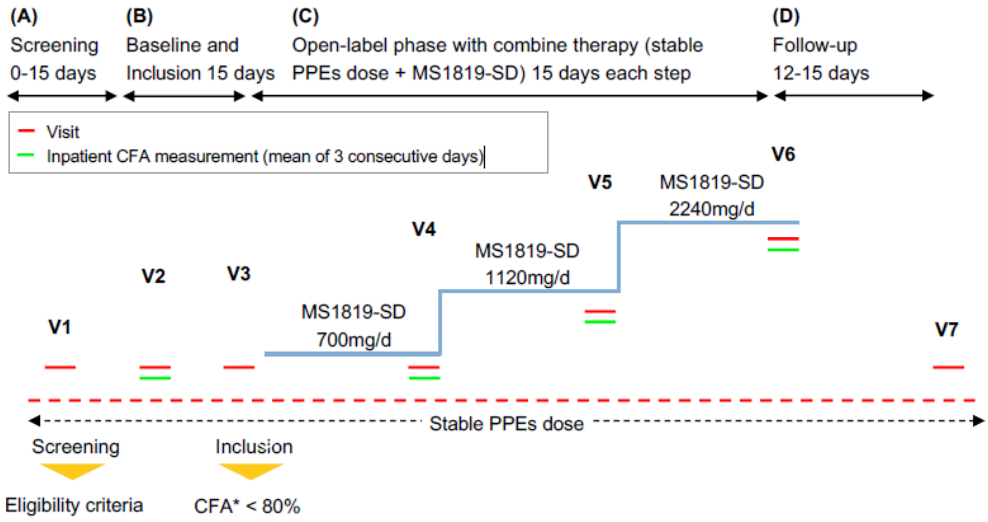


[1] This is in contrast to the hydroxypropyl methylcellulose (HPMC) capsules that were used in previous trials and used in the ongoing combination study for severe EPI patients.
[2] Source: AzurRx Corporate Presentation, May 2020.

Severe EPI Trial

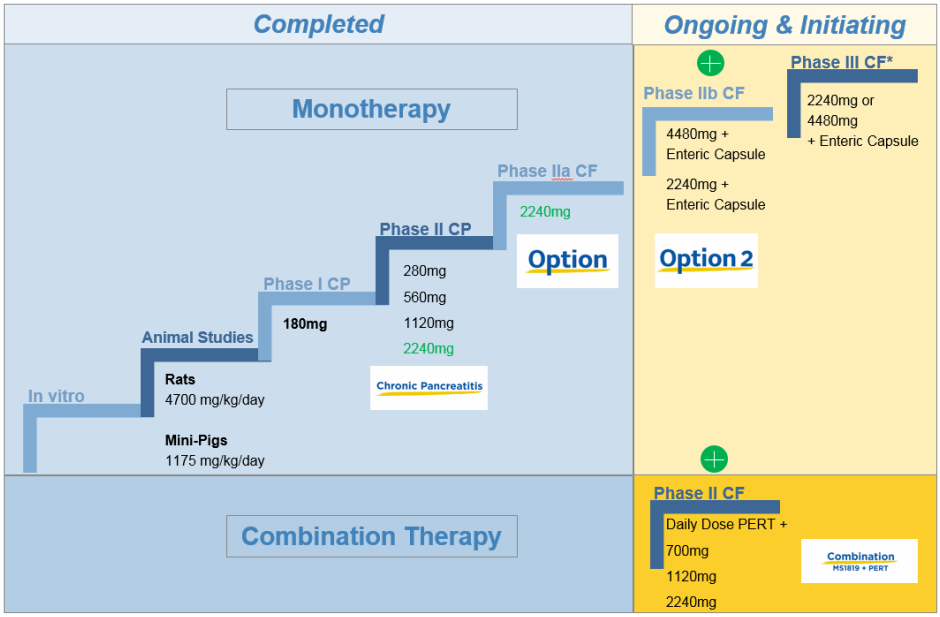
In early July 2019, AzurRx launched a Phase II trial investigating MS1819-SD in combination with standard pancreatic enzyme replacement therapy (PERT) for patients with cystic fibrosis (CF) that suffer from severe exocrine pancreatic insufficiency (EPI). These patients are unable to maintain weight and 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 II – Phase II Severe EPI Combination Study[3]



The combination therapy is expected to correct nutrient maldigestion, eliminate associated abdominal symptoms 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. 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 2019 and topline data is expected in 2Q:21. We discuss this study in more detail here.

Exhibit III – MS1819 Trial Map[4]



[3] Source: AzurRX Corporate Presentation, May 2020.
[4] Source: AzurRX Corporate Presentation, May 2020

On August 11, 2020, AzurRx provided an interim read on the severe EPI combination trial. Despite only including five individuals, the results demonstrated a nine point improvement in the coefficient of fat absorption (CFA) and improvements across the board for stool weight, stools per day and degree of steatorrhea. The primary efficacy endpoint of CFA > 80% was met by all patients across all visits. The first look at results are positive and patients reported that they had less abdominal pain after starting the combination approach adding MS1819 to PERT. Patients experienced an average weight gain of 4 pounds over the 45 days they were taking MS1819. We discuss the interim results in more detail in our note available here.[5]

Exhibit IV – Summary of Severe EPI Interim Data[6]

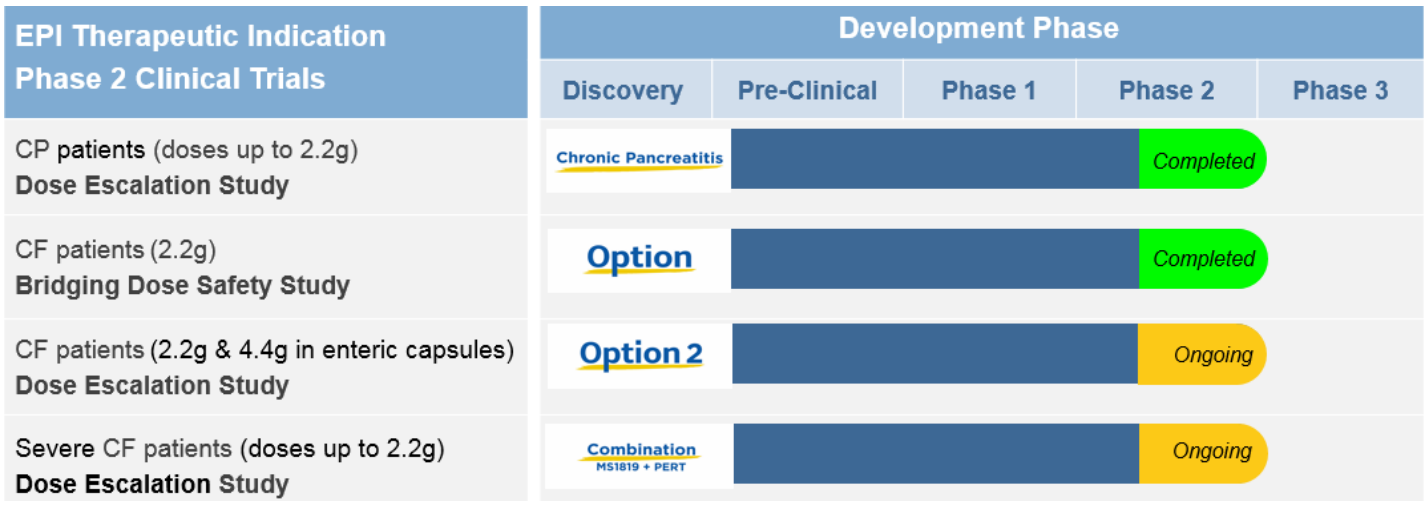
	V2 (Baseline)	AVG V4-V6	Change
CFA (%)	78.4	87.4	9
CNA (%)	96.6	97.7*	1.1
Stool Weight (g/72h)	784.2	552.7	-231.5
Stools/Day	6.8	4.8	-2.0
Steatorrhea (g/24h)	21.0	12.6	-8.5

* CNA data not available for one patient on V5 and one patient on V6.

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 V – AzurRx Pipeline[7]



[5] https://scr.zacks.com/News/Press-Releases/Press-Release-Details/2020/AZRX-MS1819-Update-Interim-Data/default.aspx
[6] Source: Source: AzurRx Combination Therapy Trial R&D Update Presentation, August 2020
[7] Source: AzurRx Corporate Presentation, July 2020

Historical and Forward Looking Highlights

- Receipt of French Tax Credits – 1Q:20/2Q: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
- Interim readout for combination trial – August 2020
- Launch of & first patients dosed in OPTION 2 trial – 3Q:20
- Presentation of Phase II CF study at N. American CF Conference – October 2020
- Completion of OPTION 2 trial – 1Q:21, coronavirus dependent
- Initial results of CF combination study – 2Q:21, coronavirus dependent

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 OPTION trial demonstrated safety and 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.[8] 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 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.

Valuation

We update our valuation to reflect the favorable data and market expansion opportunity from the interim results related to the severe EPI trial. While the presented data is only from five patients, the results were consistent and a positive first read on an opportunity for success in this subpopulation. We increase our unit sales estimates by 2 percentage points across the board (from 8% to 38% penetration to 10% to 40% penetration during the ramp up to steady state phase). Examining the severe EPI trial data combined with the results from the OPTION 1 trial, we have a greater degree of confidence in the safety and efficacy of MS1819. We also see the raise of sufficient capital to complete studies underway and the start of the OPTION 2 trial as elements that reduce risk justifying our increase for the probability of ultimate commercialization to 25%. Last month, AzurRx raised $15.2 million and issued series B convertible units and warrants, now reflected in our valuation formula. Based on these adjustments our target price is $4.00 per share

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 recent launch of OPTION 2, 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 future efforts and we will update investors as additional information becomes available. We adjust our price target to $4.00 per share to reflect our estimate changes and share balance outstanding.

[8] Some providers voiced concern that a treatment for EPI that only includes lipases may not provide sufficient enzymatic activity to support digestion and that the protease that is present in PERT is necessary for full digestion. The high level of CNA in the OPTION trial demonstrates that additional protease is not necessary.

PROJECTED FINANCIALS

AzurRx BioPharma, Inc. - Income Statement

AzurRx Biopharma	2019 A	Q1 A	Q2 A	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	$1.1	$2.4	$2.6	$7.6	$9.6	$9.5
G&A	$6.1	$1.4	$1.3	$1.8	$1.8	$6.3	$8.5	$9.0
Operating Income	($14.7)	($2.9)	($2.4)	($4.2)	($4.4)	($13.9)	($18.1)	($18.5)
Operating Margin	-	-	-	-	-	-	-	-
Interest Expense	($0.4)	($2.3)	($2.3)	($0.0)	($0.0)	($4.7)	$0.0	$0.0
Pre-Tax Income	($15.2)	($5.1)	($4.9)	($4.2)	($4.4)	($18.6)	($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)	($4.9)	($4.2)	($4.4)	($18.6)	($18.1)	($19.5)
Reported EPS	**($0.68)**	**($0.19)**	**($0.17)**	**($0.14)**	**($0.14)**	**($0.64)**	**($0.33)**	**($0.24)**
YOY Growth	-	-	-	-	-	-	-	-
Shares Outstanding	22.4	26.9	28.0	30.0	32.0	29.2	55.0	80.0

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

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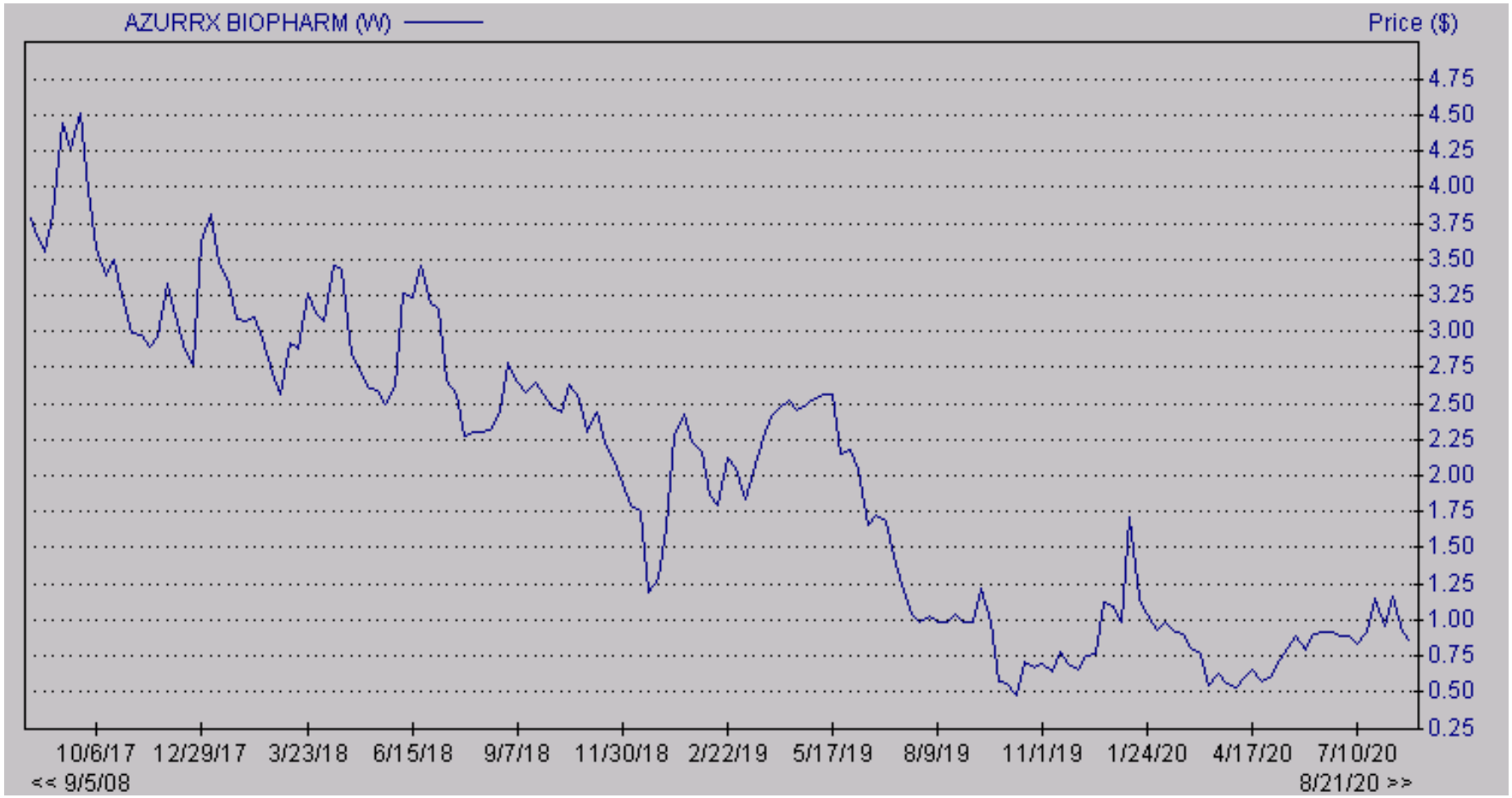
HISTORICAL STOCK PRICE

AzurRx BioPharma, Inc. – Historical Stock Price



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