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AzurRx BioPharma, Inc. (AZRX - NASDAQ)

OPTION Trial Readout

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 (9/25/2018)	$0.70
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 two programs in its pipeline.

The company recently completed a Ph2 trial for MS1819 in cystic fibrosis (CF) patients. The drug is an orally delivered, non-systemic, yeast-derived recombinant enzyme. The drug 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.

In September 2019, AZRX announced topline data from a Ph2 in EPI for MS1819 in CF patients. An additional dose ranging Ph2 will be required before the launch of a registrational Ph3 trial. Based on a favorable safety profile and demonstrated efficacy, we maintain our confidence in eventual approval and commercialization will occur in 2023.

SUMMARY DATA

52-Week High	**3.10**
52-Week Low	**0.64**
One-Year Return (%)	**-76.7**
Beta	**2.2**
Average Daily Volume (sh)	**302,456**
Shares Outstanding (mil)	**26.1**
Market Capitalization ($mil)	**18.3**
Short Interest Ratio (days)	**0.38**
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 2018 Estimate	**N/A**
P/E using 2019 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 E	$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	-$0.11 E	-$0.17 E	-$0.76 E
2020					-$0.67 E
2021					-$0.63 E

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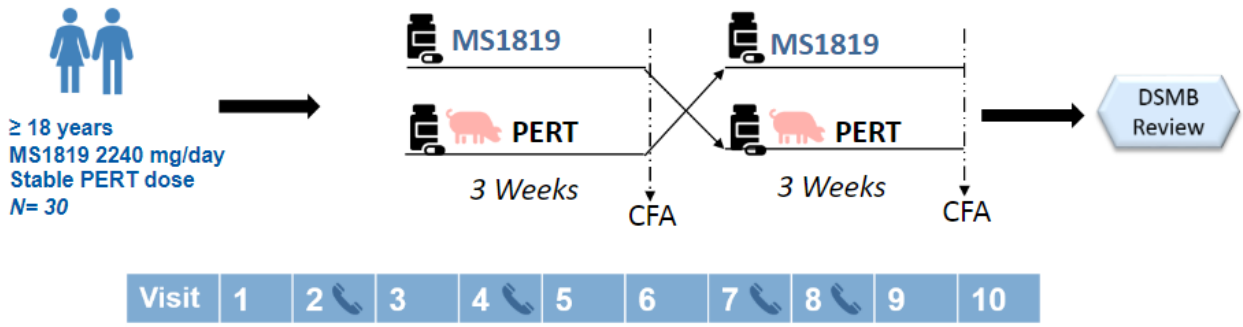
What's New

AzurRx BioPharma, Inc. (NASDAQ: AZRX) announced Phase II OPTION trial results for MS1819 in cystic fibrosis (CF) patients. 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 using 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.

Coefficient of Fat Absorption (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. 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.

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 in this extreme level providing confidence that further dose ranging trials can safely increase doses several multiples above the 2.2 g/d level.

A recurring question relates to the enzymes that are present in MS1819 compared to PERT. 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 is insufficient protein breakdown as compared to PERT, which contains all three enzymes. While carbohydrate digestion has not been a problem, there has been a concern that proteins were not being sufficiently digested without the presence of protease in the therapy.

Protein digestion is measured by the coefficient of nitrogen absorption (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 CNA of

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

93% for MS1819 and CNA of 97% 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. 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; however, we do see the timeline for approval being pushed back by a year and adjust our model according.

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 make it to the duodenum where fat breakdown takes place. We do not anticipate any additional studies will be required to shift to 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. Company management seems to be leaning toward using the enteric coating to address low CFA numbers in EPI patients on high 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 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, reduces the patient pill burden and other factors. Details of our thesis are provided in our initiation.

We adjust our target price to reflect an anticipated one year delay to our launch estimate. We had originally forecast first sales in 2022 and now advance this by one year to 2023. We maintain our original penetration estimates. Our target price moves to $5.00 per share.

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

Summary

Our initial optimistic estimates had anticipated that a Phase III trial would be supported following the OPTION trial. However, the drug development process has functioned as designed and indicated that additional dose ranging studies are necessary, ensuring the safety and efficacy of our drug supply. While this will delay the ultimate launch and approval of MS1819, we see several factors in favor of the product continuing to support its development. The drug addresses shortcomings present in PERT, namely MS1819 is a non-systemic, non-porcine derived lipase enzyme, it provides improved efficacy in acidic environments, and eliminates exposure to contamination risks. The drug can also improve compliance with a sharply reduced pill burden compared to PERT. We anticipate a dose ranging Phase II study will be launched after a December meeting with the FDA. With a strong safety profile and increased confidence on the ability of the drug to support protein digestion, we see few hurdles to continued development after an appropriate dose is found.

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. Due to the anticipated duration of a Phase II dose ranging study we adjust our previous estimates to account for the additional time it will take to perform this analysis. Our target moves to $5.00.

PROJECTED FINANCIALS

AzurRx BioPharma, Inc. - Income Statement

AzurRx Biopharma	2018 A	Q1 A	Q2 A	Q3 E	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	$2.0	$2.0	$8.9	$8.2	$8.3
G&A	$8.2	$2.5	$2.2	$2.4	$2.4	$9.5	$9.9	$9.2
Operating Income	($13.2)	($4.6)	($4.9)	($2.4)	($4.4)	($18.3)	($18.1)	($17.5)
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)	($2.5)	($4.5)	($18.6)	($18.2)	($17.5)
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)	($2.5)	($4.5)	($16.6)	($18.2)	($17.5)
Reported EPS	**($0.88)**	**($0.26)**	**($0.25)**	**($0.11)**	**($0.17)**	**($0.76)**	**($0.67)**	**($0.63)**
YOY Growth	-	-	-	-	-	-	-	-
Shares Outstanding	15.4	17.7	20.5	23.0	26.5	21.9	27.0	28.0

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

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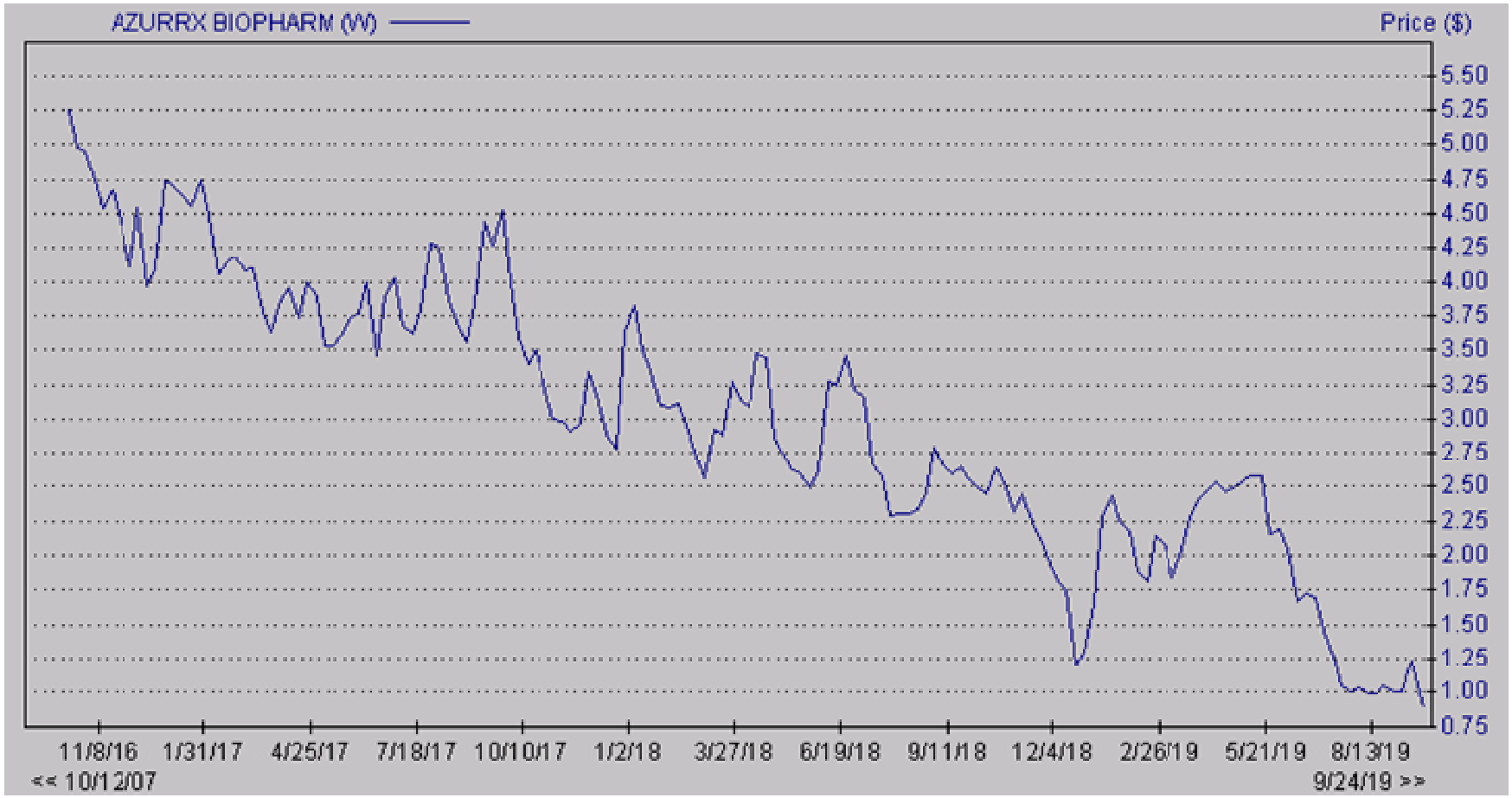
HISTORICAL STOCK PRICE

AzurRx BioPharma, Inc. – Historical Stock Price



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