Trickle Research

Every raging river, every great lake, every deep blue sea starts … with a trickle

Allocation Increase

Report Date: 09/25/19

12- 24 month Price Target: $10.25

Allocation: *8

Closing Stock Price at Initiation (Closing Px: 02/06/18): $2.96

Closing Stock Price at Allocation Increase (Closing Px: 05/02/18): $2.82

Closing Stock Price at Allocation Increase (Closing Px: 02/26/19): $2.11

Closing Stock Price at Allocation Increase (Closing Px: 07/11/19): $1.41

Stock Price at This Update (Closing Px: 09/25/19): $.70

AzurRx BioPharma, Inc.

(Stock Symbol - NASDAQ: AZRX)

http://azurrx.com/

Prepared By:
David L. Lavigne
Senior Analyst, Managing Partner
Trickle Research

AZRX reported results of their MS1819 CF (cystic fibrosis) trial this morning. Below is the full text of that release. We have provided some comments thereafter.

AzurRx BioPharma Announces Positive Results from Phase 2 Trial with MS1819 in Cystic Fibrosis Patients Excellent safety results seen in CF patients, with CFA in line with previous studies and no need for a protease.

NEW YORK, Sept. 25, 2019 (GLOBE NEWSWIRE) – AzurRx BioPharma, Inc. (NASDAQ:AZRX) ("AzurRx" or the "Company"), a company specializing in the development of non-systemic, recombinant therapies for gastrointestinal diseases, today announced positive safety results from its Phase II OPTION Clinical Trial of MS1819 for the treatment of exocrine pancreatic insufficiency in cystic fibrosis. Results showed that the primary efficacy endpoint of coefficient of fat absorption (CFA), was comparable to the CFA in a prior phase 2 study in patients with chronic pancreatitis, while using the same dose of MS1819. The dose used in both studies was 2 gram/day, which was selected in agreement with FDA as a bridging dose. Although the study was not powered for statistical significance, the data also demonstrated meaningful efficacy results, with approximately 50% of the patients showing coefficients of fat absorption (CFA) high enough to reach non-inferiority with porcine enzyme replacement therapies (PERT).

Coefficient of nitrogen absorption (CNA) was comparable between the MS1819 and PERT arms, 93% vs. 97%, respectively, in the OPTION trial. This important finding confirms that protease supplementation is not likely to be required with MS1819 treatment.

"We are thrilled to have seen such favorable safety and meaningful efficacy data in this Phase II study. Importantly, the data were consistent and confirm results seen in prior clinical studies. We are grateful to all the investigators who worked diligently to bring this study to completion on time and also want to thank the patients and their caregivers for taking the time to participate in the trial," said Jim Pennington, M.D., Chief Medical Officer of AzurRx. "We are eager to move forward with what we consider a logical and promising next trial to increase the dose for CF patients." "The search for a non-porcine based pancreatic enzyme replacement for patients with cystic fibrosis has been a challenging but important endeavor," said Dr. Michael Konstan, Professor of Pediatrics at Case Western Reserve University School of Medicine, and principal investigator in the OPTION trial. "With these data showing MS1819 to be safe and to have the potential to support fat absorption, we have considerable reason to be optimistic for the next steps in non-porcine enzyme development."

Thijs Spoor, chief executive officer of AzurRx stated, "We look forward to meeting with the FDA to discuss these results and our next steps. Clearly, these data are exciting for patients, who currently face debilitating symptoms, and we look to advancing to market a therapy that has the potential to improve the quality of life in these patients." Phase II OPTION Study Design The Phase II OPTION trial was an open-label, crossover study, conducted in 14 sites in the U.S. and Europe. Patients were first randomized to either the MS1819 arm, where they received a 2 gram daily oral dose of MS1819 for three weeks; or to the porcine enzyme replacement (PERT) arm, where they received their pre-study dose of PERT for three weeks. After three weeks, stools were collected for analysis, and patients

were then crossed over to another three weeks of the alternative treatment. After three weeks of crossover therapy, stools were collected again for analysis. Patients were then followed for an

additional two weeks for post study safety observation. A total of 32 patients, ages 18 years or older, completed the study. Topline data compared the CFA of the MS1819 treatment phase, 56%, to the CFA of the PERT treatment phase, 86%. In addition, coefficients of nitrogen absorption (CNA) were 93% in the MS1819 group, compared to 97% in the PERT group. Of note, MS1819 contains no protease. Safety in the OPTION trial was excellent, with no severe adverse events (SAEs) and few overall adverse events.

Based upon prior communications with FDA, the company is planning to meet with the agency before year-end to discuss a Phase IIb/III trial design exploring the use of higher doses and/or enteric-coated capsules to ensure higher MS1819 activity in the duodenum.

About MS1819-SD MS1819-SD, supplied as an oral non-systemic biologic capsule, is a recombinant enzyme that is derived from the yarrowia lipolytica lipase, and unlike the standard of care, does not contain any animal products.

About Exocrine Pancreatic Insufficiency Exocrine Pancreatic Insufficiency (EPI) is a condition characterized by deficiency of the exocrine pancreatic enzymes, resulting in the inability to digest food properly, or maldigestion. This deficiency can be responsible for greasy diarrhea, fecal urge and weight loss.

There are approximately 90,000 patients in the U.S. with EPI caused by chronic pancreatitis according to the National Pancreas Foundation, and more than 30,000 patients with EPI caused by cystic fibrosis according to the Cystic Fibrosis Foundation. Patients are currently treated with porcine pancreatic enzyme replacement pills.

Conference Call and Webcast Information Company management will discuss the Phase II OPTION clinical results on a conference call, today, Wednesday, September 25, 2019 at 8:30 am Eastern Time. To participate in the call, dial 877-407-0784 (domestic) or 201-689-8560 (international) fifteen minutes before the conference call begins and reference the conference passcode 13694778. The live conference call can be accessed via audio webcast at http://public.viavid.com/index.php?id=136209. A replay of the call will be available on the Investor Relations section of the Company's website. About AzurRx BioPharma, Inc. AzurRx BioPharma, Inc. (NASDAQ:AZRX) is engaged in the research and development of non-systemic biologics for the treatment of patients with gastrointestinal disorders. MS1819-SD recombinant lipase for EPI is the Company's lead development program, and additional early stage research is being conducted for the prevention of hospital-acquired infections. The Company is headquartered in Brooklyn, NY, with scientific operations based in Langlade, France. Additional information on the Company can be found at www.azurrx.com

Forward-Looking Statements This press release may contain certain statements relating to future results which are forward-looking statements. These statements are not historical facts, but instead represent only the Company's belief regarding future events, many of which, by their nature, are inherently uncertain and outside of the Company's control. It is possible that the Company's actual results and financial condition may differ, possibly materially, from the anticipated results and financial condition indicated in these forward-looking statements. Additional information concerning the Company and its business, including a discussion of factors that could materially affect the Company's financial results, including those related to the clinical development of MS1819-SD and final results of the Phase II OPTION study, are contained in the Company's Annual Report on Form 10-K for the year ended December 31, 2018 under the heading "Risk Factors," as well as the Company's subsequent filings with the Securities and Exchange Commission. All forwardlooking statements included in this press release are made only as of the date of this press

release, and we do not undertake any obligation to publicly update or correct any forward-looking statements to reflect events or circumstances that subsequently occur or of which we hereafter become aware.

For more information:

AzurRx BioPharma, Inc. 760 Parkside Avenue Suite 304 Brooklyn, NY 11226 Phone: (646)-699-7855 info@azurrx.com

Investor Relations contact: LifeSci Advisors, LLC. Hans Vitzthum, Managing Director 250 West 55th Street - Suite 16B New York, NY 10019 Phone: 617-535-7743 www.lifesciadvisors.com hans@lifesciadvisors.com.

Here are a few of our observations.

In this CF study, patient responses were similar to those found in the prior Phase II study of patients with CP (chronic pancreatitis). Specifically; "*Topline data compared the CFA of the MS1819 treatment phase, 56%, to the CFA of the PERT treatment phase, 86%.* Here is what that means as we interpret it. The average CFA ("Coefficient of Fat Absorption)", was 56% for all patients taking MS1819 versus 86% for patients taking the PERT (the pig product and current standard of care). Recognize, while that was the average of all subjects, 50% of the subjects experienced CFA results that were at least as good as PERT. So then, on the face, one could conclude that at the 2-gram dosing ½ of the people will get a good or better response than with the PERT. Granted, it would have been great (although perhaps not *expected* given that the results from the prior trial were similar) if **all** the patients would have had an equal of better response than PERT, but we find it perplexing that the street apparently (given the sell off in the stock) views this negatively. In our view, if this were a commercial product today, we think the fact that it could (presumably) help ½ of the relevant patient population get equal or better results than a porcine based PERT with a significantly lower pill burden, would be enough to capture a meaningful portion of the market…certainly enough to justify a market value many times the current market cap of AZRX. Granted, that is our unsubstantiable speculation, but we don't think that is an unreasonable view, but it is also not the whole story so we will continue.

Second, the release above references the Coefficient of nitrogen absorption ("CAN"). Specifically, it notes the following: *"Coefficient of nitrogen absorption (CNA) was comparable between the MS1819 and PERT arms, 93% vs. 97%, respectively, in the OPTION trial. This important finding confirms that protease supplementation is not likely to be required with MS1819 treatment".* We're not sure if everyone understands this point, but it is a salient one so we will provide some color.

The three main digestive enzymes are lipase (fat), protease (protein) and amylase (carbohydrates). Pancreatic Enzyme Replacement Therapy ("PERT") includes/provides not only lipase enzymes to breakdown fat, but also protease enzymes that break down proteins. (Amylase, which helps break down carbohydrates like starches and sugars, is provided by the pancreas but is also produced in saliva). That being the case, one of the concerns some have raised about MS1819, has been that while it *may be* able to replace PERTS in terms of replacing lipase to break down fat, it does not contain a protease substitute, thus it followed that an approach substituting MS1819 with PERT would require an additional protease substitute as well. The Company has historically suggested that protease, like amylase is provided by other parts of the digestive system, and as such MS1819 therapy would not require additional protease enzyme supplementation. The paragraph above from the study represents the validation of that notion. That is, the CNA levels (which measure protein absorption) were not substantially different between the PERT and MS1819 therapies. Again, we think that is a highly positive and presumably unexpected revelation given the concern that some had raised periodically prior to the completion of the trial.

Third, while we have argued above that we think the results of this and the prior trial(s) are positive on the face, the Company believes these results can be improved significantly by simply dosing patients with greater amounts of MS1819. That is the primary reason they continue to stress the marked safety results of the trials (no side effects). Recognize, some of the PERT substitute therapies that have been developed and ultimately failed in the past, failed largely because of safety problems that occurred at higher dosing levels, which prohibited those therapies from reaching efficacy levels commensurate with PERTs. In fact, as we have alluded to in the past, even PERT's have dosing limitations in that elevated doses are indicated in a condition known as "fibrosing colonopathy". That by the way, is the basis for their upcoming combination trial. That is, they believe they may be able to first establish commercial penetration by offering MS1819 in combination with PERT to patients that are bumping into the maximum dosing of the latter. In any event, the thesis here is relatively straightforward. Management believes that by (safely) increasing the dosing, they can get a considerable portion of the remaining 50% of the patient population that responded below the PERT efficacy thresholds, to levels more commensurate with PERT success. Consider the Company's analogy in this regard. If we gave 10 patients an aspirin for a headache, some portion of them would likely report relief while the balance would not. However, if we provided 2 aspirin to each patient, a greater number would likely experience relief as those who (for whatever reasons) require more medicine to do the job would now also be included in the relief category. Management believes a higher dosing trial will likely result in similar outcomes and some of their reasoning is related to the minutia of the trial data. For instance, the trial subjects experienced *varying degrees of response* to MS1819 relative to PERT (as opposed to all of them experiencing reasonably similar responses around the 56% average). That suggests that different subjects reacted differently as opposed to all of them hitting some sort of "ceiling" that might suggest the dosing topped out and thus might provide no additional benefit beyond the 2-gram dose. Here again, that is speculation, but we think it is reasonable.

So, here is the summation in our view. We continue to believe these trial results in the context of other issues we see as advantages; pill burden, concerns about porcine based PERTS etc., suggest that AZRX has a viable shot at a commercial drug here. Put another way, at the very least, even if this is as good as MS1819 gets (we don't think that), we believe many patients would try to ascertain if they are part of the favorable 50% that MS1819 works well for in order to try to take advantage of its other benefits. Personally, I realize some (obviously) take exception to that view, but if I ask myself how I would react to providing myself or my CF child with a drug with a 50% chance of working at least as well as a porcine-based high pill burden alternative, there is little doubt in my mind I would give that a try. With that said, we submit, higher doses that could drive an even more robust efficacy profile (relative to PERTS) would certainly be more desirable and looking at the stock price, that is clearly what the street wants to see. However, in our opinion, these results make the end game to our thesis (a transaction with a larger pharma player) more likely not less. Further, while better results from a higher dosing trials would substantially solidify that view, we also think positive results from the combination trial (perhaps for different reasons) could drive that outcome as well. Maybe we have drunk too much of the kool-aid here or there is something we are just missing, but we remain positive on AZRX despite the marked sell-off of the share both today and over the past several weeks.

We are aware that some of the other research on AZRX has taken the opportunity to reduce their price targets (despite their viewing the data as positive as well). We submit, at current prices, our target of $10.25 looks aggressive. With a market cap now under $20 million our price target implies a target market cap between $250 million and $300 million. While we might have to reconsider that in the context of the original 12-24 month time frame, we remain committed to the view that if higher dosing leads to a larger portion of the subject population reaching PERT efficacy levels, the resulting valuation should prove to be a lot closer to $10.00 than to $1.00. We admit, the valuation remains quite open ended and lacks visibility. The timing of that sort of inflection probably depends on our end-game thesis coming to fruition (a transaction with a large pharma), and we have no idea if/when that might happen. However, in our view, regardless of the time frame, that event would create an extraordinary bump from current levels. Given the compression in the stock around seemingly good news, we are raising our allocation of AZRX shares (again) from 7 to *8.

General Disclaimer:

Trickle Research LLC produces and publishes independent research, due diligence and analysis for the benefit of it investor base. Our publications are for information purposes only. Readers should review all available information on any company mentioned in our reports or updates, including, but not limited to, the company's annual report, quarterly report, press releases, as well as other regulatory filings. Trickle Research is not registered as a securities broker-dealer or an investment advisor either with the U.S. Securities and Exchange Commission or with any state securities regulatory authority. Readers should consult with their own independent tax, business and financial advisors with respect to any reported company. Trickle Research and/or its officers, investors and employees, and/or members of their families may have long/short positions in the securities mentioned in our research and analysis and may make purchases and/or sales for their own account of those securities. David Lavigne does not hold a position in AzurRx BioPharma, Inc.

Trickle Research has not been compensated directly by AzurRx BioPharma, Inc. for the publication of this report nor has AzurRx compensated Trickle Research for any other services associated with this research report.

Trickle Research has an exclusive content distribution agreement with SMM.Global whereby SMM.Global pays Trickle Research a fee for any Trickle labeled content displayed, hosted or distributed on its site: www.SMM.Global. Per that agreement, SMM.Global may charge issuers to host and distribute licensed research. Issuers may choose to pay SMM.Global for the hosting and distribution of Trickle Research. They are under no obligation to do so. AZRX has paid a hosting and distribution fee to SMM/Global. SMM.Global has paid Trickle a proprietary content license fee for its AZRX research content.

AZRX has paid fees to present at an investor conferences co-sponsored by Trickle Research.

Portions of this publication excerpted from company filings or other sources are noted in *italics* and referenced throughout the report.

Rating System Overview:

There are no letters in the rating system (Buy, Sell Hold), only numbers. The numbers range from 1 to 10, with 1 representing 1 "investment unit" (for my performance purposes, 1 "investment unit" equals $250) and 10 representing 10 investment units or $2,500. Obviously, a rating of 10 would suggest that I favor the stock (at respective/current levels) more than a stock with a rating of 1. As a guideline, here is a suggestion on how to use the allocation system.

Our belief at Trickle is that the best way to participate in the micro-cap/small cap space is by employing a diversified strategy. In simple terms, that means you are generally best off owning a number of issues rather than just two or three. To that point, our goal is to have at least 20 companies under coverage at any point in time, so let's use that as a guideline. Hypothetically, if you think you would like to commit $25,000 to buying micro-cap stocks, that would assume an investment of $1000 per stock (using the diversification approach we just mentioned, and the 20-stock coverage list we suggested and leaving some room to add to positions around allocation upgrades. We generally start initial coverage stocks with an allocation of 4. Thus, at $1000 invested per stock and a typical starting allocation of 4, your "investment unit" would be the same $250 we used in the example above. Thus, if we initiate a stock at a 4, you might consider putting $1000 into the position ($250 * 4). If we later raise the allocation to 6, you might consider adding two additional units or $500 to the position. If we then reduce the allocation from 6 to 4 you might consider selling whatever number of shares you purchased with 2 of the original 4 investment units. Again, this is just a suggestion as to how you might be able to use the allocation system to manage your portfolio.

For those attached to more traditional rating systems (Buy, Sell, Hold) we would submit the following guidelines.

A Trickle rating of 1 thru 3 would best correspond to a "Speculative Buy" although we would caution that a rating in that range should not assume that the stock is necessarily riskier than a stock with a higher rating. It may carry a lower rating because the stock is trading closer to a price target we are unwilling to raise at that point. This by the way applies to all of our ratings.

A Trickle rating of 4 thru 6 might best (although not perfectly) correspond to a standard "Buy" rating.

A Trickle rating of 7 thru 10 would best correspond to a "Strong Buy" however, ratings at the higher end of that range would indicate something that we deem as quite extraordinary..... an "Extreme Buy" if you will. You will not see a lot of these.