CEO CFO

ALS BIOPHARMA, LLC

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ALS Biopharma, LLC is focused on Small Molecule Drug Discovery and Development for a Once Daily Oral Dosing Therapeutic for Amyotrophic Lateral Sclerosis (ALS) known as Lou Gehrig's disease where Currently Only One Drug is FDA Approved

Healthcare - Pharma

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Allen B. Reitz
CEO

BIO:
Dr. Reitz has had 31 years of demonstrated accomplishment in the pharmaceutical industry, including nearly 26 years with Johnson & Johnson. For 16 years at the Spring House, Pennsylvania facility of Johnson & Johnson he led the medicinal chemistry research effort in the area of the diseases of the central nervous system for both psychiatry and neurology. He is co-inventor as well as Team Leader, in most cases, for seven compounds that have entered human clinical trials, three of which are currently in the clinic (Phase I and II). He has >135 scientific publications and 48 issued U.S. patents, and is the Editor-in-Chief of the journal *Current Topics in Medicinal Chemistry*. He has extensive experience in project and portfolio management, target validation, hit triage, hit to lead and lead optimization medicinal chemistry, eADME profiling, and preclinical candidate selection. He has starting several ongoing companies including Fox Chase Chemical Diversity Center, Inc. (www.fc-cdci.com) and ALS Biopharma (www.alsbiopharm.com). He is Adjunct Professor at Drexel University, College of Medicine, and the co-founder and President of the Pennsylvania Drug Discovery Institute. He has an Executive Masters in Technology Management from the University of Pennsylvania (Wharton School, Penn Engineering).

About ALS Biopharma, LLC:
ALS Biopharma, LLC is an emerging biotechnology company located in Doylestown, PA, dedicated to the discovery of new therapeutics to treat amyotrophic lateral sclerosis (ALS) and related disorders. ALS Biopharma expects to be in human clinical trials with a new therapeutic agent in 2015, which would have the potential for once daily oral dosing, as compared to twice daily for current therapy, and greater efficacy, with potential utility for other mechanistically-related indications. The company was founded originally in 2009 by an angel investor who had ALS in his family and Dr. Allen Reitz.

Interview by Lynn Fosse, Sr. Editor

CEOCFO: Dr. Reitz, what is the focus at ALS Biopharma?
Dr. Reitz: The focus of ALS Biopharma is on discovering new treatments for the debilitating disorder of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's and motor neuron disease.

CEOCFO: There has certainly been a lot of research in that area. What are you looking at specifically?
Dr. Reitz: Primarily, we focus on small molecule therapeutic drug discovery and development. However, we have conducted research on a protein for a biologic approach, called Heat Shock Protein Hsp70, and have obtained Orphan Drug Designation from the U.S. FDA for that protein. There is only one drug approved currently by the U.S. FDA solely for ALS. This drug is called riluzole (Rilutek®). What we seek to do is to provide additional and more effective treatment options to physicians for this disease.

CEOCFO: Would you give us a little more detail?
Dr. Reitz: ALS is a devastating disease in which there are roughly about thirty thousand patients in the United States. ALS is what is called an orphan indication. Although it is fairly rare, it is extremely devastating for those patients who suffer from it and their caregivers. The problem with is that current therapy riluzole displays variability in effect between patients. The average patient will receive roughly two to three months increase in lifespan after taking the drug for a year and a half, whereas the average time between diagnosis and death is around two to three years. What ALS Biopharma is seeking to accomplish is two things: first, to expand lifespan to a great extent, and an effective agent might be able to do this for six to twelve months or more; and, sec-

ond, to improve quality of life for a greater numbers of patients.

CEOCFO: What is the science? How are you trying to do that, or what are you looking at that may give you the result you want?

Dr. Reitz: We have prepared pro-drugs of riluzole to address the limitations of riluzole in therapy. Currently, riluzole is metabolized very differently by certain individuals, due to variability in expression of the enzyme Cyp1A2. Our new riluzole derivatives are not metabolized by Cyp1A2, and have greater bioavailability upon oral administration and half-life in the body. Even though riluzole is the only drug approved to treat an orphan indication, only roughly 50% of patients take this medicine, so we expect to achieve greater patient penetration and patient compliance. We anticipate once-daily dosing, which is a big competitive advantage relative to riluzole itself which is currently given twice a day. In addition, we are conducting research on a very important protein for ALS called TDP-43. Aggregates of this nucleic acid binding protein are found in the spinal cord motor neurons of >50% ALS patients after death, and are also present in a related condition called frontotemporal lobar degeneration. We have developed the first compound library screen which looks at blocking the binding of nucleic acid substrate to TDP-43, and have gone on to show functional consequences of cell culture. We are currently transitioning to tests in various animal models to see if TDP-43 can be validated as a drug target for the discovery of new therapeutics to treat ALS.

CEOCFO: Will the process be faster because you are using the drug that has already been approved?

Dr. Reitz: It is de-risked to some extent. However, since the pro-drug is a new chemical entity, we will go through the FDA review process as with any new agent prior to giving it to patients. However, the products that will form upon prodrug cleavage are a fairly innocuous amino acid derivative and riluzole itself. In this regard, the pro-drug approach is less risky than most other small molecule therapeutics.

CEOCFO: What is the timetable?

Dr. Reitz: We hope to file an Investigational New Drug (IND) application by the end of next year (2014) for the riluzole pro-drug, and we expect to be in human clinical trials by the end of 2015. Our strategy is to partner with a medium or larger pharmaceutical company for the larger, multi-center clinical trials that will be required for eventual commercialization, and that will hopefully occur in 2015-2016.

CEOCFO: Has the medical and/or investment community been paying attention yet, or is it still too early?

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Dr. Reitz: Yes, we have had numerous meetings with private and public funding institutions and potential collaborators. We post our publications in the peer-reviewed scientific literature on our website, www.alsbiopharma.com. We are funded by grants from the National Institutes of Health, as well as from private foundations. In that regard, our research has undergone near constant peer review providing validation of our overall approach.

CEOCFO: Is ALS in favor these days with investment and medical people?

Dr. Reitz: As an orphan indication, it is more in favor than it used to be. The definition of an orphan indication in the United States is a prevalence of two hundred thousand or lower, at any one time. There is more of an emphasis both from the investment community, and also from the government, in trying to support research for the orphan diseases. Amongst orphan diseases, ALS is widely understood to be poorly treated. There is clear unmet medical need for new treatments for ALS and the potential for substantial commercial return. This type of therapeutic target is not one that the major pharmaceutical companies will spend much time with, but the smaller niche biotechnology companies are very interested in orphan indications such as ALS. Further, it is important to note that ALS, along with other neurological disorders, has undergone a renaissance of understanding in terms of the scientific basis for how the disease progresses; both for the genetic or familial and the sporadic patient populations. This new understanding can be the basis for the discovery of new therapeutics for ALS and related diseases.

CEOCFO: How far will current funding take ALS Biopharma, and will you be looking for more sources?

Dr. Reitz: We can go to the end of next year. Yes, we are looking for new sources of funding. Any interested parties can contact us directly using the appropriate portal on the website.

CEOCFO: Why should ALS Biopharma stand out for investors and people in the business community?

Dr. Reitz: We have a clear path to commercialization for a therapeutic to treat ALS with greater patient penetration and compliance than the existing option. In addition, we have the potential to treat other neurological disorders and may be effective against stage III/IV melanoma, based on research conducted by our collaborators at Rutgers University. We seek to do cutting edge science and to publish our work when appropriate in the peer-reviewed scientific literature. Much of the funding we have received is non-dilutive via granting mechanisms, which provides substantial leverage for any future investment.