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Drug Development for Rare Ophthalmological Disease Treatment

Albert G. Zamora
CEO

CEOCFO: ***Mr. Zamora, what is the concept behind Bionure?***
Mr. Zamora: Bionure is a late-preclinical drug development company. We are developing a novel first-in-class small molecule that promotes neuroprotection and remyelination for the treatment of two rare ophthalmology diseases: acute optic neuritis and neuromyelitis optica.

CEOCFO: ***What is it that you have figured out that is not currently understood? What is the science?***
Mr. Zamora: Many diseases have neurodegeneration as a common thread, such as Multiple Sclerosis, Alzheimer, Parkinson or Glaucoma. Current therapeutic options are limited and in most cases palliative or only partially effective. So far, no therapies have been approved that successfully address the progressive neuronal degeneration. In the case of acute optic neuritis and neuromyelitis optica, inflammatory damage in the optic nerve leads to axonal and neuronal loss and demyelination, resulting in eye pain, visual impairment and blindness – and in the case of neuromyelitis optica, even death. Our compound BN201 targets the trophic factor pathway downstream, preventing axonal and neuronal damage and promoting myelin repair.

CEOCFO: ***Were you looking for a drug to help in these two diseases or did you have a drug and realized that the indications might be appropriate for those conditions?***
Mr. Zamora: In our case, what came first was the interest in finding a neuroprotective drug, with a special interest in Multiple Sclerosis. After several years of research and from a library of compounds, we found some that were activating the trophic factor pathway as the nerve growth factor (NGF) did, triggering a potential neuroprotective effect. We used the Multiple Sclerosis Experimental Autoimmune Encephalomyelitis (EAE) animal model to assess the activity of these compounds, and after selecting the leading candidate, we tested it in an animal model of glaucoma to confirm its neuroprotective activity. However, once the compound was well characterized, and according to its properties, we identified a much shorter, and affordable, path for development and designed the best clinical and regulatory strategy to obtain a clinical proof of concept in humans – by focusing in these two rare indications in the ophthalmology field.

CEOCFO: ***Is the medical community aware or is it too early?***
Mr. Zamora: The project is starting to be well known. It was under the radar for many years, but during the last year, we have achieved some key milestones, presenting in some conferences and being featured in media. Our partnerships with leading scientific organizations such as the Myelin Repair Foundation and the National Multiple Sclerosis Society reflect the rising awareness on the potential of the project.

CEOCFO: ***Is there skepticism from the science and medical community or is it easy to understand how your drug works and why it works?***
Mr. Zamora: It is not that difficult to understand how it works or why it could be working at the clinical level. The skepticism comes from the past, where some pharmaceutical companies had tried to develop a neuroprotective drug, but ran into some difficulties in proving whether it was working or not at the clinical level (from PK challenges in recombinant proteins to challenges in identifying the appropriate clinical endpoints, lack of tools to measure efficacy, etc). This is why we have chosen to focus in these two orphan, eye diseases: it enables a cost-effective, fast-track development path with

shorter trials; there are precise tools to measure efficacy in the eye (Optical Coherence Tomography, OCT), and imaging and clinical endpoints are well defined and accepted by regulatory agencies. Recently, our CSO published a paper in Annals of Neurology on the correlation between the imaging and the clinical endpoints in acute optic neuritis.

CEOCFO: ***Are you working with the National Multiple Sclerosis Society?***
Mr. Zamora: Yes. Our main goal is Multiple Sclerosis in the mid and long term, but we chose to pursue these other orphan indications first as it enabled a more feasible development path to demonstrate efficacy at the clinical level. Acute optic neuritis is a disease closely related to Multiple Sclerosis, as it is usually one of the first symptoms for MS patients and as much as 75% of patients with an initial episode of acute optic neuritis may eventually develop MS. If we demonstrate that our compound is working in acute optic neuritis, it will serve as a proof of concept for MS. That is the reason why the MS Society became interested in our development. They recently chose us as one of the most promising companies in the pipeline and funded us to complete the preclinical stage and enable IND for Phase 1 trial.

CEOCFO: ***Are you funded for the foreseeable future and what needs do you see coming up?***
Mr. Zamora: We now have the funds to complete the IND enabling studies, positioning the company to start clinical trials by summer. We have just opened an equity round of funding of $15 million to obtain a clinical proof of concept in humans and we expect to close it by May or June.

"With a $15 million investment and a 2-year frame, we will obtain a clinical proof of concept, positioning the company for a big upside in a deal with a big pharmaceutical company."- Albert G. Zamora

CEOCFO: ***Is this an area that is generally in favor with the investment community?***
Mr. Zamora: The intersection of ophthalmology with the orphan space is actually an attractive field for the investment community. We have recently achieved key milestones such as the orphan designation and the agreement with FDA in a pre-IND meeting on study design and primary endpoints – decreasing the risk at the preclinical and regulatory level – which makes the clinical program feasible and affordable. We started talking with investors a couple of weeks ago and the interest is high: with a $15 million investment and a 2-year frame, we will obtain a clinical proof of concept, positioning the company for a big upside in a deal with a big pharmaceutical company.

CEOCFO: ***How do you deal with the frustration of how long it takes to get a potential drug into use?***
Mr. Zamora: We are dealing with that with plenty of perseverance and resilience. Being confident that this is the right way and the right compound, we will be able to prevail at the end. It is not easy, but we have a nice group of private investors right now that are supporting us. We have also built an extraordinary committed team that includes leading authorities in neuro-immunology and neuro-ophthalmology such as Prof. Larry Steinman and Dr. Craig Smith, among others. It is also very helpful that we have been able to raise a great deal of money from grants and the support from renowned institutions such as the National MS Society is exceptional and provides an excellent external validation to the project. Last, the interest shown by big pharmaceutical companies in the project is encouraging.

CEOCFO: ***Put it all together for our readers in healthcare and the investment community. Why pay attention to Bionure?***
Mr. Zamora: In the recent months, we have seen rising interest in drug discovery for remyelinating compounds. However, it is only a part of the solution: if neuronal and axonal damage occurs and axons are not preserved, remyelination can be futile. Bionure is unique as the only company in the pipeline with a neuroprotective and remyelinating, first-in-class candidate. We have positioned BN201 in orphan ophthalmology, an attractive space, and the agreement with FDA on study design and endpoints makes the clinical program feasible and affordable. Having received funding from National MS Society provides external validation by a well-recognized third party, and we have built an outstanding scientific advisory board including Prof. Larry Steinman from Stanford, Prof. Stephen Hauser from UCSF, Dr. Joaquim Trias, co-founder of Tetraphase, Anthera and Vicuron among others, and Dr. Craig Smith, former Genentech and Novartis leader for eye diseases. All this together is what makes Bionure one of the most promising companies in the field.

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