Telocyte Newsletter
Q4 2017

Many biotech firms, particularly those aimed at Alzheimer's disease, find that their major problem is finding the "signal in the noise". They strive to find any statistically positive result over several years of clinical trials and several thousand patients with Alzheimer's disease – so far to no avail, despite the time lost, the money spent, and the patients who fail to benefit. Some large pharmaceutical firms, for example, spend years following thousands of patients (and spending hundreds of millions of dollars), only to find only a marginal, statistically weak result that later proves fruitless and clinically ineffective.

At Telocyte, we have a unique problem.

We expect that our "signal" (cognitive improvement in patients with moderately severe Alzheimer's disease) will be so apparent that we will need only a few months and a dozen patients in order to show unambiguous improvement. Strange as it may appear, that's our problem. Effective clinical results are so unexpected, so far beyond the historical norm, that most people will – predictably – have a difficult time believing those results. Until now, every pharmaceutical company has failed, totally and without exception. When Telocyte succeeds in our phase 1 human trial, it will (and should) prompt questions. How credible are our (incredible) results? We are profoundly concerned not about our ability to achieve results, but our credibility once we achieve such results. Our focus then, is to ensure that when people have questions about what we have done, we have answers. We need to tread carefully and without a misstep as we move into human clinical trials.

There have been far too many claims and, equally, far too many failures.

When a biotechnology company fails to show any effect on Alzheimer's, as every single clinical trial has done to date, no one questions the credibility of that failure: it's an expected outcome. But when a company succeeds, particularly in the face of a history of universal failure, then it can expect doubt and disbelief. At Telocyte, our task is not merely to cure Alzheimer's disease, but to minimize error and maximize our credibility.

To this end, during the last quarter, we have had meetings with the Alzheimer's Association at the global research conference in London, the Alzheimer's Drug Discovery Foundation in their recent New York conference on drug discovery, and with the director of the Dementia Society of America. In every case, we are not seeking support or publicity, but merely that they understand our program and a chance to ensure that they are not blind-sided by our upcoming clinical trials. In addition, we have acquired new internal expertise to be sure that our human trials are done well. To this end, a globally respected expert on statistics and the design of clinical trials, specifically interventional clinical trials in Alzheimer's disease, met with us in London and graciously joined our Scientific Advisory Board to ensure that our clinical trials achieve valid and reliable data. I urge you to look at her biographical notes below, as well as on our website. She is eager to work with us and we, in turn, are more than delighted, and honored, to work with Dr. Hendrix.

As with so many ventures, the path is clear and well-defined at a high-level, but there is a stunning amount of work involved in getting the details right. We continue to move ahead on the completion of full funding for our upcoming animal study and our planned human trial, which is currently scheduled for late 2018. Currently, we are actively and continually reassessing both our animal toxicity protocols and our human protocols, adjusting for new technical advances in diagnosis and delivery systems, optimizing our techniques, and doing everything we can to ensure that we meet with success, both in our meetings with the FDA and, ultimately, in our clinical trials.

It's definitely a labor, but a labor of love.

Meeting our Scientific Advisors:

Over the next several quarters, we'd like to introduce you to the members of our Scientific Advisory Board, members whose expertise underlines our commitment to getting our work done right. This quarter, we introduce the newest member of our SAB, Dr. Suzanne Hendrix.

Suzanne Hendrix, PhD
Scientific Advisory Board

Suzanne Hendrix has worked for the past 26 years as a biostatistician focusing on clinical trial research in many different indications. She has extensive experience designing clinical trials, writing statistical analysis plans, running analyses, writing statistical reports, interacting with the FDA and preparing manuscripts for publication. She is experienced at communicating statistical concepts in an understandable way, and has helped develop software for graphically understanding large complex datasets. For the past 14 years, she has specialized in statistical issues in Alzheimer's disease such as identifying appropriate outcomes, addressing measurement issues, demonstrating disease modification and optimizing clinical trial design and analysis. She has been on multiple advisory boards and expert panels addressing current issues in Alzheimer's disease, and has interacted with the division of Neurology products at the FDA and with the EMA through scientific advice regarding these issues. She is currently president and owner of *Pentara*, a company that provides data management and statistical consultation to the pharmaceutical industry, academic groups and non-profit groups, primarily supporting clinical trial design and optimization in Alzheimer's disease. She has researched methods for discerning disease modification of treatments, and has proposed novel approaches to this problem. She is an active researcher in the Alzheimer's disease field with over 100 publications and presentations.