Telocyte Newsletter
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For those of you who might like to understand our approach, I suggest you may want to read a paper that will be published on Wednesday January 8th in Alzheimer's & Dementia, the premier global journal on Alzheimer's disease, and published by the Alzheimer's Association.

I gave an invited talk in Washington for the association last year and was then asked to write the paper. In the talk, I pointed out that while we have a number of "targets" (such as beta amyloid and tau tangles) and a number of sophisticated techniques (such as monoclonal antibodies), what we completely lack is an overall "systems" model that explains how the risk factors are related to the clinical findings.

For example, we know that closed head injuries, infections, radiation exposure, toxins, and dozens of genes increase the risk of beta amyloid deposits and tau tangles, but we don't have a unified model that explains WHY those risk factors cause those deposits and tangles, along with the clinical findings of Alzheimer's disease. We need a single, overarching model to explain not only Alzheimer's, but other age-related dementias (including Parkinson's, FTD, EOAD, Lewy body disease, vascular dementia, mixed dementias, and others). Such a model should also explain why animals suffer from cognitive decline as they age. Most importantly, we know that the single major risk factor for Alzheimer's disease is age, but exactly how does aging cause human dementias?

Our model ("A Unified Model of Dementias and Age-Related Neuro-degeneration") explains how and why age-related dementias occur, why animal models fail to work in humans, why all prior human trials have failed, and presents the rationale for the novel target that we will use in our FDA human trials. It also answers ten key questions that have bothered other researchers in this field.

If you'd like to understand why Alzheimer's occurs and why we expect to succeed in our human trials, let us know and we'll send a copy to you. The paper is not for the faint-of-heart: it's not only quite technical, but it's also a long paper (17k words, 269 references, and 8 figures, even before the appendix was added). The paper had eight reviewers who were remarkably complimentary. Most of the reviewers wished the paper were shorter, but then also asked me to add more information about issues that were specific to individual reviewers. Given this interest from the reviewers, the editors requested an appendix, making the paper even longer but giving readers a feel for the dialogue between the eight reviewers and myself.

The editor-in-chief was impressed enough with the model that he joined our Scientific Advisory Board. The paper promises to be a watershed for the Alzheimer's field, as well as offering insight to our investors as they try to understand how

Telocyte will succeed where others have not. We welcome anyone who would like a copy of the paper. Just email us and ask.

Several people have asked about a company that is trying a similar approach, although they are doing so without FDA oversight, in an "offshore" location, and at a substantial financial cost to patients. We can understand the need for haste, but we have grave concerns about their approach. Our ethics require us to go through the FDA, use an academic setting for our trial, get the approval of an institutional review board, and provide our therapy free-of-charge to patients in our phase 1 human trial. Our concerns revolve around three issues that define Telocyte: credibility, safety, and efficacy.

We realize that any trial – regardless of where it is done or under whose auspices – will have a difficult time gaining credibility if there are positive results in treating Alzheimer's patients. There have been more than 400 registered Alzheimer's trials, all of which have, by global consensus, had negative results. Any trial that suggests an effective intervention (or even a marginal delay in disease, as with the Biogen results presented when I was at the recent CTAD conference in San Diego in early December) will automatically generate professional eye-rolling and a degree of understandable disbelief. That disbelief is clear enough when the studies are done by well-known pharmaceutical firms and biotech companies with careful oversight by the FDA, but to expect any scientific or public confidence when a human trial is done in a more lackadaisical manner is impossible.

We are also profoundly concerned about safety and efficacy. I just returned from a major conference on gene therapy in neurological disease (GTxN). While there is enormous potential for the use of gene therapy in medicine, there are also major pitfalls and difficult technical issues whose dangers escape the unwary. At Telocyte, we have been working on our human protocols for three years and yet we still see potential safety risks and uncertainties that have us talking to gene therapy experts, combing the literature, and tweaking our approach to increase safety and ensure efficacy. While there is enormous potential, there are also enormous dangers and difficulties; dangers which we are aware of and which we know how to mitigate, difficulties which we understand and know how to avoid.

We intend to cure Alzheimer's. We do not intend to cure Alzheimer's by taking shortcuts with people's lives or by using an approach that is likely to fail. We are in this not only for the long run, but for the needs of those we hope to cure. We are fully committed to curing Alzheimer' credibly, safely, and effectively.