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## **From a patient's skin cells to neuron replacement therapy: Aspen carves a unique arc in pursuit of durable therapy for Parkinson's disease.**

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**CEOCFO: *Dr. Federoff, what is the vision behind Aspen Neuroscience?***

**Dr. Federoff:** Aspen Neuroscience is dedicated to the development of autologous stem cell-based therapies to address diseases of the nervous system and affected visceral organs starting with the replacement of dopamine neurons in Parkinson's disease. We anticipate that successful dopamine neuron transplants will produce clinical benefit and improve quality of life for people suffering from Parkinson's disease.

**CEOCFO: *Personalized medicine has been around for a while. What are you doing that is different than some of the other approaches or is there a standard approach and you tweaking that model?***

**Dr. Federoff:** We are not taking a standard approach; Aspen is the only company pursuing an autologous approach for Parkinson's disease. That means that we start with a patient who carries the diagnosis of Parkinson's. His/her skin is biopsied and ultimately, after the cells are qualified using predicted genomic assays, we produce a cell population that when transplanted replaces the dopamine neurons lost consequent to Parkinson's. We believe that our approach is the one that has the greatest promise to set the clock back on the disease itself.

**CEOCFO: *Why?***

**Dr. Federoff:** Because the dopamine neurons that are lost produce the motor symptoms of the disease. By placing one's own derived dopamine neurons in the proper brain region they then engraft and wire up correctly, and are anticipated to reconstitute the circuits which support motor movement. Unlike any of the other approaches that are out there, which are, at best, capable of stabilizing the disease or slowing its progression, Aspen's autologous dopamine replacement strategy has the greatest potential to return productive years to patients.

**CEOCFO: *Where are you in the process today?***

**Dr. Federoff:** Aspen Neuroscience has demonstrated that it can take skin cells from Parkinson's patients and produce the types of cells that are necessary to put back in the brain. Within the last ten months or so, Aspen has moved to interact and receive feedback from the FDA so that we can begin the enrollment of what we refer to as our trial ready cohort. Those are the individuals who have Parkinson's and who would meet our inclusion criteria for our subsequent clinical trials. Then, upon the completion of the current non-clinical IND enabling steps that the FDA has requested, we would apply for an IND, with the anticipation that patients in our trial ready cohort, some of whom would be already well characterized, would be available to be transplanted shortly thereafter as part of our clinical trials.

**CEO CFO: *Is there a particular point in Parkinson's where you anticipate the cells being most effective?***

**Dr. Federoff:** The answer is yes. At this time of diagnosis, typically a Parkinson's patient will have lost approximately fifty percent of the particular type of dopamine neurons. As patient's progress they continue to lose additional neurons. We believe that the ideal patient population for Aspen's therapy would be people who have early to moderate or moderate disease. This represents a fraction of all Parkinson's patients, roughly several hundred thousand of the approximately 1 million affected individuals in the US.

**CEO CFO: *Is there any potential downside?***

**Dr. Federoff:** If there is a potential downside for our approach it would be that we do not currently address the non-motor features of Parkinson's (in our lead product) which are important to patients. However, no therapies that are currently available or those we know are in clinical development address modification of the non-motor features. At this point, while limited, Aspen has plans for subsequent clinical programs, one of which may have the potential to address both motor and non-motor features. Presently, our approach robustly addresses the motor complications which are very limiting for people suffering from Parkinson's disease.

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In this second program that Aspen Neuroscience has under development we may begin to address both motor and non-motor complications. Aspen will target the most common familial form of Parkinson's, owing to a mutation in the gene designated GBA. The reason these patients get Parkinson's is likely because the mutation lowers amount of enzyme (glucocerebrosidase) encoded by the GBA gene. The program, Aspen's ANPD002 program, involves a step where we reconstitute the enzyme levels in the autologous dopamine neurons to be transplanted. These are expected to wire up and restore dopamine to the brain, but importantly, they also will produce more enzyme. Whether or not higher level of enzyme can improve functions beyond the transplanted cell population remains to be evaluated in clinical trials.

**CEO CFO: *What have you learned so far, as you have been considering this process?***

**Dr. Federoff:** We know that it is possible to go from a skin cell from a biopsy to cells suitable for transplantation in a short period, approximately four months. We also know that Aspen's autologous product does not elicit immune responses and consequently our proposed transplanted patients will not need to be administered powerful immunosuppressive drugs, therefore is safer and less costly. This speaks to the innovations that Dr. Jeanne Loring's lab brought to this field. Formerly of Scripps and now Aspen's co-founder and CSO, Dr. Loring combined her expertise in stem cell biology and genomics to converge on an approach that is unparalleled. This fusion is Aspen's version of a secret sauce.

Lastly, we have made the decision and are fairly far along that Aspen Neuroscience is going to develop a special delivery method to transplant the dopamine stem cells to the brain. We believe that Aspen's approach will shorten the time of the transplant operation and more efficiently deliver the cells to the brain.

**CEO CFO: *Can you tell us more about that or is it somewhat under wraps?***

**Dr. Federoff:** I think I will just leave it there for now.

**CEO CFO: *What is the prevalence of Parkinson's disease?***

**Dr. Federoff:** The total number of people with Parkinson's in the US is approximately one million. Among these individuals, the more common sporadic form accounts for about 90% and the combined familial form about 10%. Where the aging demographic, the 'silver tsunami' is present, the prevalence of Parkinson's will grow as age is the single greatest risk factor.

**CEO CFO: *We came upon Aspen Neuroscience from your recent funding. How far will that take you?***

**Federoff:** The now completed \$70M Series A raise is intended to take us beyond Phase I and provide a runway for our Phase II study of our lead program (ANPD001) for sporadic Parkinson's. We anticipate as well that the funding will also

allow us to advance the ANPD002 program for the most common familial form of Parkinson's in a slightly delayed time frame relative to the lead product.

**CEOCFO: *What has been the reaction from people in the medical community or research community who are aware of what your concept is at Aspen Neuroscience?***

**Dr. Federoff:** I think Dr. Loring has done a great job, as have other stem cell scientists, in making clear that Parkinson's disease is a very good target for stem cell replacement therapy with dopamine neurons. With my clinical background and involvement with translational and clinical research in neurodegeneration, I get to speak to a slightly different group of people, many of whom are close to, or themselves carry a diagnosis or are clinicians who treat neurodegenerative disease patients. It appears that most are looking forward to initiation of clinical trials using the autologous replacement cell approach.

**CEOCFO: *What is the time table? What do you anticipate?***

**Dr. Federoff:** If we did not have COVID 19 the timetable would be a little bit more straightforward. There remain unpredictable business discontinuities beyond Aspen's control. It is too early, given the uncertainty of the pandemic and its evolution to make accurate projections. Aspen continues to develop and refine contingent plans with the anticipation that will allow us to sustain timely operations.

**CEOCFO: *There are many companies to look at in all areas of disease in medicine, without COVID, let alone with COVID. Why pay attention to Aspen Neuroscience? What makes the company so important?***

**Dr. Federoff:** There is no therapy that provides the opportunity to truly set the clock back on the disease process that affects the brain. What we currently offer patients is symptomatic treatments. This autologous program has, in my estimation, the best potential to restore function. This is a new paradigm. It is a matter of moving to the clinic and making those clinical trials count so that we can demonstrate proof of concept and ultimately produce evidence of clinical benefit.

**CEOCFO: *What, if anything, might someone miss when they are looking at Aspen and looking at your approach?***

**Dr. Federoff:** It depends on who is looking. Sophisticated scientists would fully grasp the importance of the robustness of the science Dr. Loring has pioneered, that undergirds what we do at Aspen Neuroscience. Those looking casually from the outside, perhaps some with less scientific sophistication, might believe that we are just like any other stem cell company, which I would assert, "We are not". Most importantly, as more interested parties examine the science and understand just how robust it is, whether or not they are trained as clinicians or scientists, it will be evident that Aspen actually occupies a unique space and has the tools, human capital, resources and plans to potentially change the face of Parkinson's disease.