Aspen Neuroscience Initiates Cohort 3 in ASPIRO Phase 1/2a Trial, Highlighting Commercial Formulation of ANPD001 and Its Transformative Potential for Parkinson's Disease

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SAN DIEGO, September 25, 2025 /PRNewswire/ — Aspen Neuroscience, Inc., a clinical-stage biotechnology company pioneering autologous regenerative therapies, today announced the initiation of Cohort 3* in its ASPIRO Phase 1/2a clinical trial of ANPD001, the company's personalized dopaminergic neuronal precursor cell (DANPC) therapy for moderate to advanced Parkinson's disease (PD).

This milestone marks the first administration of Aspen's commercial formulation of ANPD001, designed to support scalable, reproducible manufacturing for future clinical and commercial use. The Cohort 3 formulation has been deemed preclinically comparable to the formulation used in the first two Cohorts, ensuring continuity as we advance to this next phase. In earlier cohorts, positive six-month data reported at IAPRD demonstrated strong safety and tolerability, along with clinician- and patient-reported improvements, achieved without the need for immunosuppression.

"Cohort 3 represents an important step toward commercial readiness," said Damien McDevitt, Ph.D., President and CEO of Aspen Neuroscience. "We've optimized our formulation and delivery system to meet the rigorous demands of late-stage development and future market access, while preserving the personalized nature of our autologous approach."

This new formulation allows for the cryopreserved cells to be available for dosing immediately upon arrival to the institution, streamlining the surgical workflow and minimizing the impact on hospital cell processing labs.

Aspen's Three Pillars of Innovation in Autologous Regenerative Cell Therapy
Aspen's commercial formulation of ANPD001 sets a new benchmark for neurodegenerative disease treatment through a proprietary, integrated approach:

1. **Manufacturing Platform.** The proprietary and high-tech manufacturing process converts patient-derived skin cells into DANPCs leveraging machine learning–algorithms that help produce the highest quality personalized cells for the patient.
2. **Therapeutic Platform.** ANPD001 is an autologous induced pluripotent stem cell (iPSC)-derived DANPC therapy. It is a personalized regenerative cell therapy that uses a patient's own cells, avoiding immune rejection and offering tailored treatment.
3. **Proprietary Device for Cell Transplantation Procedure.** Aspen's precision delivery system combines its metered dosing syringe and MRI guidance to ensure sub-millimeter accuracy, minimally invasive surgery, and a streamlined clinical workflow.

"Together, these three pillars form a unified platform that is personalized, precise, and scalable—setting a new standard in autologous iPSC-derived cell therapy," said Lisa Johnson-Pratt, M.D., Executive Vice President, Therapeutic Program Lead. "This commercial formulation is designed not just for clinical success, but for real-world impact. Enabling precision dosing without immunosuppression, ensuring scalable and consistent cell quality, and simplifying surgical workflows reduces the time and burden on hospital cell processing labs which is critical for commercial viability. Our vision is to deliver a commercial-ready solution positioned to redefine how neurodegenerative diseases are treated."

A Competitive Edge in Parkinson's Therapeutics
Aspen's autologous approach (using a patient's own cells) positions ANPD001 uniquely in the PD treatment landscape. Unlike allogeneic therapies, which use foreign cells from donors, ANPD001 avoids immune rejection and the need for prolonged immunosuppression. Its personalized nature allows for tailored treatment, while the commercial formulation ensures consistency and scalability.

With more than one million people living with Parkinson's disease in the U.S. alone, and no disease-modifying therapies currently available, ANPD001 offers a potential first-in-class opportunity to restore lost dopaminergic function with curative intent.

About ANPD001
ANPD001 is the most advanced autologous investigational cell therapy in the United States for treating Parkinson's disease. More information about the Phase 1/2a trial is available at clinicaltrials.gov (NCT06344026).

Aspen's personalized approach means that patients do not require immunosuppressive (IS) drugs to dampen the body's immune response against foreign cells. This approach will eliminate IS-associated adverse events, IS drug-monitoring requirements, and enable dosing for those with contraindications to IS therapies.

Aspen's manufacturing process starts from a small biopsy of the patient's own skin cells, followed by reprogramming to induced pluripotent stem cells (iPSCs) and then differentiation of the iPSCs into DANPCs. These DANPCs are transplanted into the posterior putamen, replacing cells that were lost or damaged due to disease. The quality of each person's cells is assessed at every manufacturing stage using Aspen's proprietary machine learning-based genomics tests.

ANPD001 has received Fast Track designation by the U.S. Food & Drug Administration (FDA).

About the ASPIRO Trial
ASPIRO is the first multi-patient, multi-center clinical trial of an autologous cell therapy for Parkinson's disease. It is evaluating safety, tolerability, and preliminary efficacy of ANPD001 in levodopa-responsive patients aged 50–70. Primary endpoints will be reported at 12 months, with long-term follow-up extending to 15 years.

For more information, visit www.aspenneuroscience.com.

** We are entering testing for the third Cohort, building on funding provided by the California Institute for Regenerative Medicine (CIRM), a state of California agency that supports regenerative medicine, stem cell, and gene therapy research, which enabled Cohorts 1 and 2.*

SOURCE Aspen Neuroscience, Inc.