Pathways Neuro Pharma Inc. Announces Strategic Focus on Rare Pediatric Diseases, Led by Juvenile Parkinson's Disease

Pathways Neuro Pharma shifts focus to rare pediatric diseases, advancing a groundbreaking non-agonist gene therapy for Juvenile Parkinson's Disease.

WEST CHESTER, PA, UNITED STATES, February 27, 2025 /EINPresswire.com/ -- Pathways Neuro Pharma Inc., a pioneering biotechnology company, today announced a strategic shift in its development pipeline to prioritize rare pediatric neurological disorders, with Juvenile Parkinson's Disease (JPD) as its lead indication. This move positions the company at the forefront of gene therapy innovation, leveraging its proprietary AAV6.2FF platform to deliver long-lasting therapeutic benefits—offering a transformative alternative to traditional dopamine agonist treatments.

> "Children diagnosed with Juvenile Parkinson's Disease face severe limitations with current treatments, and our approach has the potential to fundamentally alter disease management."
>
> *Anthony Mack, President and CEO*

Pathways Neuro Pharma's investigational non-agonist therapy is designed to restore dopaminergic signaling and enhance neuroprotection in pediatric patients. Unlike existing therapies—primarily L-dopa and dopamine agonists—which provide only temporary symptom relief and lead to severe side effects, this one-time administration therapy is designed to provide sustained therapeutic benefits for up to 15 months.

"This strategic pivot is driven by both scientific opportunity and a compelling market need," said Anthony Mack, President and CEO of Pathways Neuro Pharma Inc. "Current treatments for Juvenile Parkinson's Disease are severely limited, and our approach has the potential to fundamentally change disease management. By pursuing Rare Pediatric Disease Designation, we are positioning ourselves to accelerate development, gain key regulatory incentives, and create a pathway for rapid market entry."

Positioned for Growth: Regulatory and Market Advantages

Pathways Neuro Pharma's focus on rare pediatric diseases aligns with significant regulatory incentives, including the potential for a Rare Pediatric Disease Priority Review Voucher (PRV)—a

highly valuable asset that can be used to expedite FDA approval of another drug or be sold, with past transactions reaching over $100 million.

The shift toward pediatric neurology has already garnered strong interest from investors, scientific collaborators, and regulatory agencies. The company is actively pursuing strategic partnerships and non-dilutive funding through organizations such as the National Institutes of Health (NIH) and the Michael J. Fox Foundation, reinforcing confidence in its development trajectory and commercial potential.

Scientific Progress: Advancing Toward Clinical Development

Pathways Neuro Pharma has initiated animal model studies to evaluate the efficacy and safety of its AAV6.2FF gene therapy, with a focus on dopamine receptor modulation and disease progression. These studies will generate critical preclinical data, informing the next steps toward IND-enabling studies and clinical trials. A successful outcome would position the company for clinical-stage development and significantly de-risk the path to approval.

Expanding the Pipeline Beyond Juvenile Parkinson's Disease

While Juvenile Parkinson's Disease is the initial focus, Pathways Neuro Pharma is exploring additional indications where its gene therapy platform can address other rare pediatric neurological disorders. By prioritizing disease-modifying therapies that eliminate the need for chronic medication, the company is setting a new standard in pediatric neurology.

About Pathways Neuro Pharma Inc.

Pathways Neuro Pharma Inc. is a biotechnology company at the forefront of next-generation gene therapies for neurological disorders. By leveraging precision-engineered viral vectors, the company is developing disease-modifying, long-lasting treatments that eliminate the need for chronic medication. With a core focus on rare pediatric diseases, Pathways Neuro Pharma is dedicated to transforming patient care and redefining treatment paradigms in neurology.

The company's innovative approach integrates advanced AAV-based gene therapy, targeting key neurological pathways to deliver long-term efficacy with a single administration. Its lead program focuses on Juvenile Parkinson's Disease (JPD), a rare and devastating neurodegenerative disorder affecting children. With a commitment to regulatory excellence, scientific rigor, and patient-centered innovation, Pathways Neuro Pharma is actively engaging with key stakeholders, including regulatory agencies, patient advocacy groups, and research institutions, to accelerate the development of life-changing therapies.

Forward-Looking Statements

All statements contained herein other than statements of historical fact, including statements regarding our future results of operations and financial position, our business strategy and plans, and our objectives for future operations, are forward-looking statements. The words “believe,” “may,” “will,” “estimate,” “continue,” “anticipate,” “intend,” “expect,” and similar expressions are intended to identify forward looking statements. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our financial condition, results of operations, business strategy, short-term and long-term business operations and objectives, and financial needs. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including those described in the “Risk Factors” section of the offering documents. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. In light of these risks, uncertainties and assumptions, the future events and trends discussed in offering documents may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements.

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