Pathways neuro pharma

Corporate Overview

INVESTOR PRESENTATION February 25, 2025

CAUTIONARY NOTE ON FORWARD LOOKING STATEMENTS AND DISCLAIMERS

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.

All references to dollar amounts in the offering summary or to use of proceeds are subject to change pending offering documents.

This presentation highlights basic information about us and the offering. Because it is a summary, it does not contain all of the information that you should consider before investing. This offering may only be made by means of offering documents

PROPRIETARY INFORMATION

PATHWAYS NEURO PHARMA

Pathways Neuro Pharma is developing transformative therapies that directly target brain pathways regulating the root causes of Juvenile Parkinson’s and other rare pediatric neurological disorders

EXECUTIVE LEADERSHIP

Anthony P Mack, MBA
Chief Executive Officer

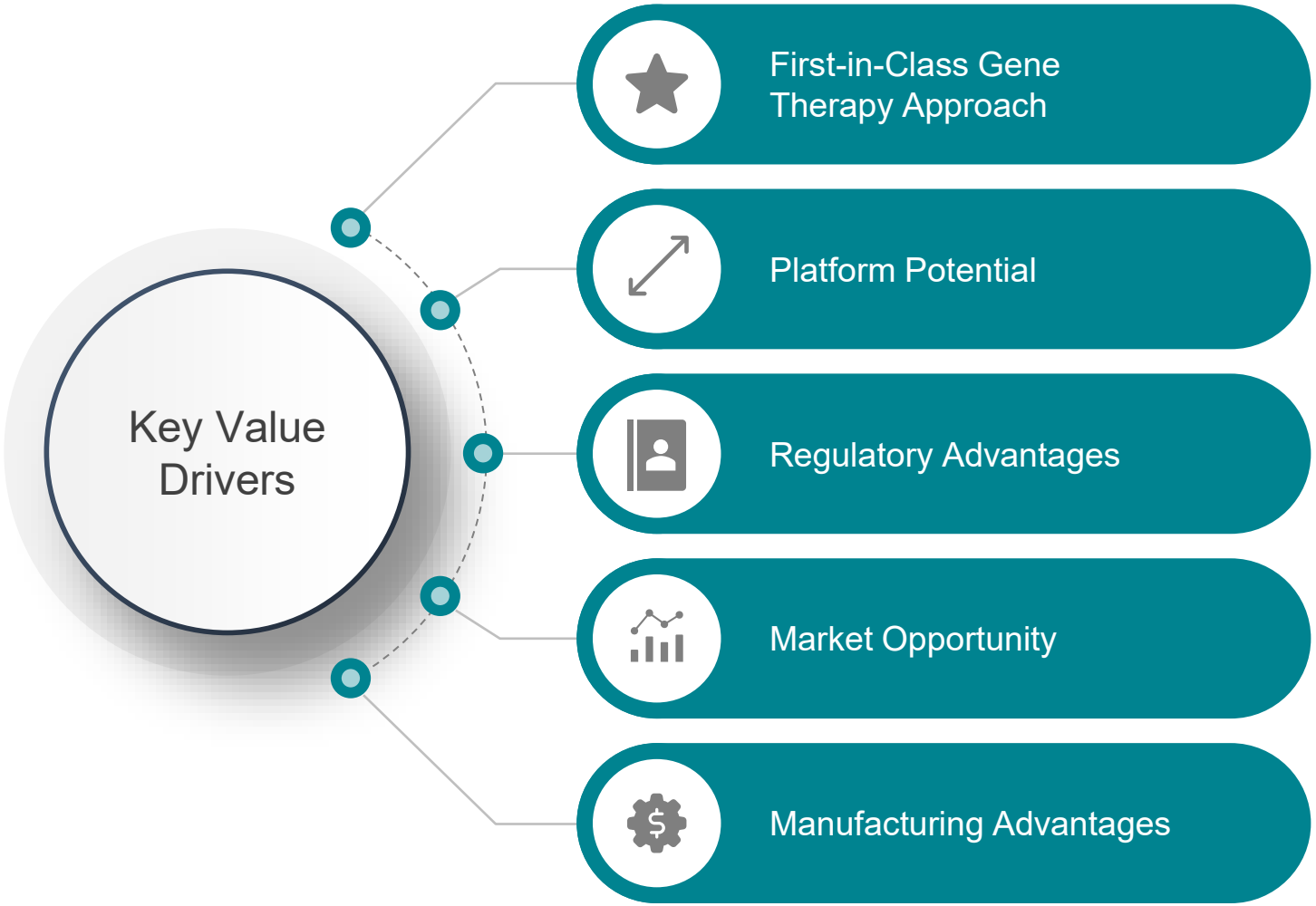
Anthony Mack is a distinguished pharmaceutical and biotech entrepreneur with 35 years in the industry. As CEO, he formerly led Scilex Pharmaceuticals (Nasdaq: SCLX) and founded ProSolus Inc, later acquired by Mission Pharmacal. His notable achievement as CEO includes taking Virpax Pharmaceuticals Inc (Nasdaq: VRPX) public in February 2021, highlighting his expertise in advancing innovative healthcare companies in a competitive landscape.

Bradley G Thompson, PhD
Chief Technology Officer

Dr Thompson, PhD, co-founder of Kickshaw Ventures, Inc and of Wyvern Pharmaceuticals, Inc, is an experienced biotechnology professional of more than 43 years. Until recently, he served as Director and Chairman of BIOTECanada and as Executive Chairman, CEO, and President of Oncolytics Biotech, Inc. He has served on the boards of directors of numerous public and private companies and is also a member of the Advisory Board of Lifeboat Foundation Biosciences Inc.

COMPANY OVERVIEW

Our approach creates massive value with cost-effective development and near-term milestones



Vital Improvement to SoC

Unlike daily dopamine agonist therapies, our non-agonist solution increases dopamine receptor density at the genomic level, offering enhanced, long-term effect.

Expansive Potential in Neurology

While initially focused on Juvenile Parkinson's Disease, our gene therapy approach can be adapted for other rare pediatric CNS disorders.

Clear Clinical Pathway

Orphan Drug & Rare Pediatric Disease designations provide significant cost reductions and exclusivity benefits, and our team has experience navigating this domain.

Strong M&A Tailwinds

Gene therapies for rare CNS disorders command high reimbursement rates and strong M&A interest.

Cost Reduction

Gene therapies are notoriously expensive to manufacture and Pathways Neuro Pharma has developed methodology to substantially reduce production costs.

AAV6.2FF: KEY VALUE DRIVERS

Improved Delivery
Ensures high-quality viral vectors for effective gene therapy applications.

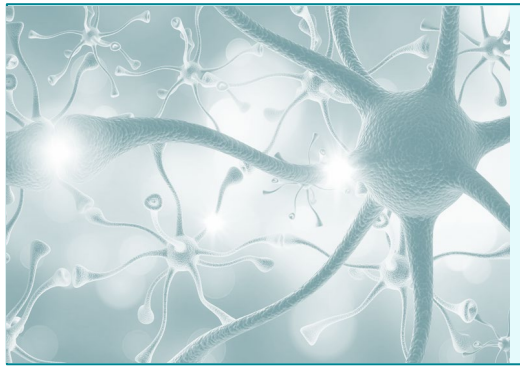
Huge Improvement Over SoC
Shifts treatment from daily dopamine agonist with sever side effects to treatment every 15 months.

Functional Disease Modification
Provides sustained correction of underlying neurochemical dysfunction which is directly linked to disease progression and patient quality of life.

Opportunity to Expand Care
Kids cannot be treated the same way older patients are with dopamine agonists, but we could change that

Juvenile Parkinson’s

UNMET NEEDS IN PEDIATRIC NEUROLOGICAL DISORDERS



Juvenile Parkinson's Disease likely affects approximately 1 in 500,000-1,000,000 individuals under 21 years old[1]

A Rare Pediatric Disease Designation provides expedited regulatory pathways, market exclusivity, and eligibility for a Priority Review Voucher (PRV), accelerating development while enhancing commercial value.

1: https://pmc.ncbi.nlm.nih.gov/articles/PMC6795374

Juvenile Parkinson's Disease

Mortality Risk in Juvenile Parkinson's:

Patients with juvenile Parkinson's (onset before age 21) exhibit a threefold higher mortality risk compared to the normal population.

Genetic Factors:

Mutations in the PARK2 (PRKN) gene account for approximately 50% of inherited and 15% of sporadic juvenile-onset Parkinson's case, leading to early neurodegeneration and dopamine dysfunction.

Economic Burden:

The total annual economic burden of Parkinson's disease in the U.S. is estimated at $51.9 billion, including direct medical costs and indirect costs like lost income and caregiver expenses.[2]

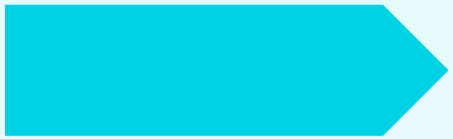
1. hopkinsmedicine.org; 2. pmc.ncbi.nlm.nih.gov

JUVENILE PARKINSON'S: Product Overview

TECHNOLOGY & IP[1]	MARKET OPPORTUNITY[2]	FDA Regulatory Expedited Development Pathways	ACADEMIC RESEARCH	NON-DILUTIVE FUNDING OPPORTUNITIES	COMMERCIAL OPPORTUNITIES
• Route of administration: IV Infusion • Duration and Frequency: Once every 12 to 18 months • "Neuroreceptor Compositions and Methods Of Use" – US Patent Number 11,760,788 – Anticipated Expiration March 2, 2041	• The Global Parkinson's Disease Market Size is Expected to Reach $9.14 Billion by 2033	• Rare Pediatric Disease Designation (RPDD): – Priority Review Voucher • Orphan Drug Designation (ODD): – Tax credits 25% for clinical trials – Waves FDA application fees • Breakthrough Designation (BTD): – Expedited review – Increased access to FDA guidance and enhanced support – Accelerating the approval timeline to address critical unmet needs • Expanded Access /Conditional Approval: For patients with no alternative treatment Pending strong phase 2 data required	• Rebholz H, et al. *Int J Mol Sci.* 2018 • Missale C, et al. *Physiol Rev*. 1998 • S.Sivestri et al. Psychopharmacology, Oct. 2000 • S.Thobois et al. Annals of Neurology, March 2002	• NIH • Michael J Fox Foundation for Parkinson's Research • American Parkinson's Disease Association • Child Neurology Society • Parkinson's Foundation	• Neurologist • Hospitals • Pediatric Movement Disorder Specialist • Pediatric Neurologist • Nurse Practitioners • Physician Assistants (in some regions) • Gene Therapy Research

1. Lau WC, Thompson B, inventors; Pathways Neuro Pharma, assignee. Neuroreceptor compositions and methods of use. US patent 11,760,788. September 19, 2023. 2. https://finance.yahoo.com/news/global-parkinson-disease-treatment-market-190000324.html?guccounter=1

18-MONTH DEVELOPMENT PLAN

	Discovery	Preclinical Efficacy & Tox	First-in-Human Studies	Phase 2
Juvenile Parkinson's		Q2 2025 Anticipated Completion	Q3 2025 Anticipated Commencement	Q2 2026 Anticipated Commencement

*As of Q1 2025.

Juvenile Parkinson's Treatment Landscape

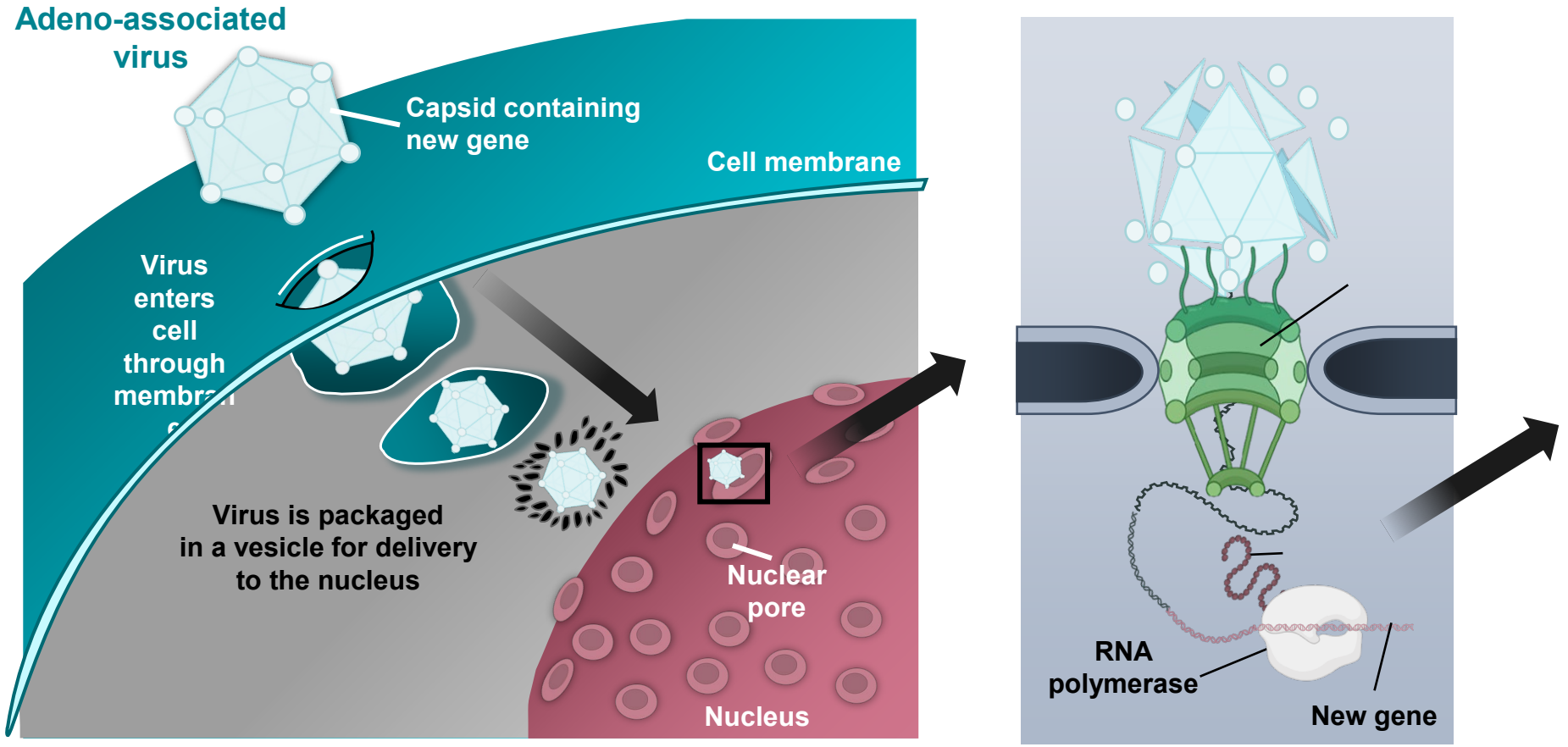
TREATMENT NAME	COMMON SIDE EFFECTS
Levodopa-Carbidopa (Brand Name: Sinemet)	Nausea, dizziness, orthostatic hypotension (drop in blood pressure upon standing), dyskinesias (involuntary movements), and hallucinations.
Dopamine Agonists (e.g., Pramipexole [Brand Name: Mirapex], Ropinirole [Brand Name: Requip]))	Nausea, dizziness, somnolence (drowsiness), hallucinations, and impulse control disorders (e.g., compulsive gambling, hypersexuality)
Monoamine Oxidase B (MAO-B) Inhibitors (e.g., Selegiline [Brand Name: Eldepryl], Rasagiline [Brand Name: Azilect])	Nausea, headache, insomnia, and, rarely, hypertensive reactions.
Amantadine (Brand Name: Symmetrel):	Ankle swelling, livedo reticularis (a mottled skin discoloration), hallucinations, and confusion.
Anticholinergics (e.g., Benztropine [Brand Name: Cogentin], Trihexyphenidyl [Brand Name: Artane]):	Dry mouth, blurred vision, constipation, urinary retention, and cognitive impairment.

Adeno-Associated Virus (AAV) Vector Gene Therapy

GENE THERAPY USING AN AAV VECTOR

Modified DNA containing a **new gene** is packaged into an adeno-associated viral vector

Virus disassembles and delivers DNA encoding the **new gene** into the nucleus



Genetic information from the **new gene** is transcribed to create messenger RNA **(mRNA)**, which serves as a blueprint for protein production

How does gene therapy work? NIH. Accessed March 21, 2024. https://medlineplus.gov/genetics/understanding/therapy/procedures/

FDA-APPROVED AAVs IN GENE THERAPY

NAME	COMPANY	DISEASE TREATED	APPROVAL DATE	SEROTYPE*
Luxturna®[1]	Spark Therapeutics, Inc.	**Retinal dystrophy**	**2017**	**AAV2**
Zolgensma®[2]	Novartis Gene Therapies, Inc.	**Spinal muscular atrophy**	**2019**	**AAV9**
Hemgenix®[3]	CSL Behring LLC	**Hemophilia B**	**2022**	**AAV5**
Elevidys[4]	Sarepta Therapeutics, Inc.	**Duchenne muscular dystrophy**	**2023**	**AAVrh74**
Roctavian™[5]	BioMarin Pharmaceutical Inc.	**Hemophilia A**	**2023**	**AAV5**

AAV vectors are considered biosafety level 1[6]

AAVs are not known to cause any diseases in humans[6]

*Serotypes are selected based on their targeted gene therapy applications.

1. Luxturna [package insert]. Philadelphia, PA: Spark Therapeutics, Inc.; 2022. 2. Zolgensma [package insert]. Bannockburn, IL: Novartis Gene Therapies, Inc.; 2023. 3. Hemgenix [package insert]. King of Prussia, PA: CSL Behring LLC; 2022. 4. Elevidys [package insert]. Cambridge, MA: Sarepta Therapeutics, Inc.; 2023. 5.Roctavian [package insert]. Novato, CA: BioMarin Pharmaceutical Inc.; 2023. 6. Collins DE, Reuter JD, Rush HG, Villano JS. Viral Vector Biosafety in Laboratory Animal Research. Comp Med. 2017;67(3):215-221.

OUR PATENTED AAV TECHNOLOGY:
AAV6.2FF DELIVERS HIGH TRANSGENE EXPRESSION

Preclinical Evidence

AAV6.2FF is a triple AAV6 mutant with demonstrated improvements in:

- Kinetics → **101-fold and 49-fold increased** expression in the muscle and lungs **at 24 hours,** respectively
- Transgene expression → **Nearly 10-fold greater** expression in lung vs AAV6 **at day 21**
- **Neutralization resistance** → **10-fold increase in resistance** to pooled immunoglobulin neutralization

→ **Accelerated therapeutic impact**

→ **Extended duration**

→ **Targeted delivery**

van Lieshout LP, et al. *Mol Ther Methods Clin Dev.* 2018;9:323-329.

Preclinical Findings and Rare Pediatric Disease Opportunities

- Pathways Neuro Pharma has demonstrated the ability to increase the expression of transgenes in preclinical models[1]
- This methodology is being applied to increasing the expression of neuroreceptors GPR139, and DRD1, both of which are important therapeutic targets in the brain[2-4]

- Juvenile Huntington's Disease
- Angelman Syndrome
- Rett Syndrome
- Dopa-Responsive Dystonia
- Pitt-Hopkins Syndrome
- Childhood Onset Schizophrenia
- Autism Spectrum Disorders

We are committed to leveraging scientific breakthroughs for the upregulation of neuroreceptors that will enable safer, more effective, and longer-lasting treatments for rare pediatric neurological diseases

1. van Lieshout LP, et al. *Mol Ther Methods Clin Dev.* 2018;9:323-329. 2. Kononoff J, et al. *eNeuro.* 2018;5(3):ENEURO.0153-18.2018. 3. Rebholz H, et al. *Int J Mol Sci.* 2018;19(11):3581. 4. Jones-Tabah J, et al. *Front Cell Neurosci.* 2022;15:806618.

Thank you!

For Information, Please Contact:
Anthony Mack
anthony.mack@pathwaysnp.com

Pathways
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