PATHWAYS NEURO PHARMA

A Gene Therapy for the Root Cause of Early-Onset Parkinson's Disease

One-time treatment · Genetically defined patients · Orphan disease · Strategic acquisition opportunity

Important Notice

Forward-Looking Statements

This presentation contains forward-looking statements based on current expectations and assumptions. Actual results may differ materially. All programs are investigational — safety and efficacy have not been established in humans. This presentation does not constitute an offer to sell or solicitation to buy any security.

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No Fairness Opinion

Valuations presented are risk-adjusted estimates based on disclosed assumptions. They do not constitute a fairness opinion. Recipients should conduct independent due diligence and consult qualified legal, financial, and tax advisors.

The Patients This Therapy Is Built For

They are not who most people picture when they think of Parkinson's disease

"Diagnosed in their 20s, 30s, and 40s — often while raising young families. Given the same medications as 60-year-olds with a different disease. There is no approved therapy that addresses why they have this."

Age 17

A high school student notices hand tremors during exams. Genetic testing confirms a PINK1 mutation — one of the youngest known presentations. No approved therapy exists for any of them.

Age 28

A father notices his hand shaking at work. Told it's stress. Diagnosed with Parkinson's six months later. His neurologist has no explanation for why.

Age 34

A mother of two is struggling to button her children's coats. Her doctors offer levodopa — the same drug used since the 1960s.

Age 40

A teacher is told he has early-onset Parkinson's. No genetic test was ever run. No explanation. No path forward beyond managing symptoms for life.

The Problem: Zero Approved Therapies That Address the Cause

0

Approved disease-modifying therapies for PINK1 Parkinson's

Current care is palliative — not curative

Every existing Parkinson's medication manages symptoms of neurons that have already died. None of them slow, stop, or reverse the underlying disease process.

Patients with PINK1 mutations are mismanaged

Most are never genetically tested. They receive the same treatment as 70-year-olds with age-related Parkinson's — a completely different disease with a different cause.

The cause is known — and untreated

For PINK1 patients, the biological reason for their disease is documented in their DNA. A clear, inherited mutation causes their neurons to fail. No approved therapy addresses this known cause.

Understanding the PINK1 Population

Why a subset of Parkinson's patients is a fundamentally different — and better — opportunity

All Parkinson's Disease — 11.7 million patients worldwide | No single genetic cause | Complex, multi-factor

▼

Early-Onset Parkinson's (diagnosed before age 50) — ~10% of all PD = ~1.17M globally | More likely to have a genetic cause

▼

PINK1-Associated Early-Onset Parkinson's Disease

Caused by inherited mutations in the PINK1 gene · 1–9% of early-onset cases globally

~1.17M early-onset PD worldwide × 1–9% PINK1 frequency = 8,500–76,500 global patients · ~35,000 in the U.S. (base case)

Diagnosed in their 20s, 30s, and 40s · Genetically confirmed · Pathways' target population

Why smaller is better: genetically defined patients are identifiable today, qualify for orphan drug designation, and can be enrolled in smaller, faster trials. The precision of the target is a strategic advantage, not a limitation. Anchor narrative: we target genetically defined PINK1 Parkinson's, primarily presenting as early-onset disease, with cases documented in adolescence — creating a potential pathway to Rare Pediatric Disease designation.

[1] Yin & Dieriks, Journal of Parkinson's Disease, 2025 (meta-analysis, 120 studies, 743 cases) · [2] GBD 2021 Parkinson's Disease Collaborators, The Lancet Neurology, 2023

The Market: A Defined Population with Significant Pricing Power

$16B
Parkinson's disease market by 2031

35,000
PINK1 patients in the U.S. (base case)

$3M
Projected one-time therapy price (anchored to comps)

32%
Peak penetration (anchored to Zolgensma)

Why the Sub-Population is the Opportunity

The $16B market is background context — not our addressable market.

Our target is the 35,000 PINK1 patients. This population is:

- ✓ Genetically identified — diagnosable today
- ✓ Orphan drug eligible — faster trials, fewer patients
- ✓ No competition — zero approved disease-modifying therapies
- ✓ Rare disease commercial infrastructure identified

Gene Therapy Pricing — Market Precedent

Zolgensma (SMA)	**$2.1M**	Novartis
Hemgenix (Hemophilia B)	**$3.5M**	CSL Behring
Lenmeldy (MLD)	**$4.25M**	Orchard
PINK1 Gene Therapy	**$3.0M**	Pathways — anchored to comps

[1] Towards Healthcare, Global PD Market 2031. [2] Yin & Dierks 2025. [3] Zolgensma (Novartis), Hemgenix (CSL Behring), Lenmeldy (Orchard) — US list prices at approval

Our Solution: A One-Time Gene Therapy That Addresses the Root Cause

A single injection delivers a working copy of the PINK1 gene directly to the patient's neurons — restoring the biological process that has been failing since birth.

What Every Other Treatment Does	What Our Gene Therapy Does
✗ Manages symptoms of neurons that have already died	✓ Restores PINK1 function before neurons are lost
✗ Replaces dopamine that the brain can no longer produce	✓ Targets the inherited genetic cause — not the downstream symptoms
✗ Requires daily medication — often for life	✓ Single administration — not a daily pill
✗ Does not slow, stop, or reverse disease progression	✓ Designed to modify disease progression, not just manage it
✗ Becomes less effective over time as more neurons are lost	✓ Measurable biological response detectable within months

All programs are investigational. Safety and efficacy have not been established in humans.

The Science in Plain Language

You don't need a biology degree to understand why this works

Think of PINK1 as the recycling system inside each neuron. When it's broken, neurons fill with waste they cannot remove. Over time, the buildup kills them. We deliver a working copy of the PINK1 gene — restoring the recycling system before damage becomes irreversible.

1 The Problem

A patient is born with a broken PINK1 gene. Their neurons cannot clear cellular waste from birth. Damage accumulates silently — until symptoms appear in their 20s or 30s.

2 The Therapy

A single injection delivers a working PINK1 gene directly into the patient's neurons using a safe viral carrier. One treatment. No daily medication.

3 The Result

The PINK1 gene restarts the neuron's recycling system. Waste clears. Neurons survive. We can measure this happening — in months, not years. Recent third-party work has confirmed that PINK1 can be activated pharmacologically, validating the pathway — but only gene replacement can restore function in patients who lack the protein entirely.

FDA's next-generation framework: Safety is now defined by understanding biology — not just observing damage in animals. We are building this program under the FDA's weight-of-evidence framework, using human-derived neurons that are more predictive of patient outcomes than conventional animal models.

FDA Modernization Act 2.0 (2022) authorized non-animal alternatives in IND applications · FDA Roadmap to Reducing Animal Testing, 2026 · Pickrell & Youle, Neuron, 2015

The Team: Executives Who Have Done This Before

Anthony P. Mack

President & CEO · Co-Founder

→ 35 years in pharmaceutical and biotech development

→ CEO: ProSolus — acquired by Mission Pharmacal

→ CEO: Scilex Pharmaceuticals — strategic investment, Itochu Chemical Frontier

→ Founder: Virpax Pharmaceuticals (NASDAQ IPO 2021, >$60M raised)

→ NIH + DoD non-dilutive funding secured

Dr. Bradley G. Thompson, PhD

CTO, Co-Founder & Board Member

→ 43 years in biotechnology

→ Inventor of AAV6.2FF — the delivery vector this program depends on

→ 18 companies co-founded · 9 public listings · 5 acquisitions

→ US Patent 10,806,802 — exclusively licensed to Pathways

→ Co-founder and board member — skin in the game, not a contractor

Partners: Syneos Health, Clinical Development / CRO Partner — GLP toxicology contracted, integrated regulatory and clinical capabilities · Commercial infrastructure partner to be engaged pre-launch · Doug Centilli, Executive Chairman — 20 years US Capitol · Investment bank engagement in process

What Your Investment Does

A clear, five-step path from capital to exit

01

$2.5M Raise

Secures program financing — triggers all downstream milestones

02

IND-Enabling Safety Studies

Rodent and large-animal safety testing required before human trials (6 months)

03

First-In-Patient (FIP)

First patient dosed in Australia — the primary value re-rating event

04

Investment Bank NDR Post-FIP

Non-deal roadshow introduces Pathways to strategic acquirers simultaneously

05

Acquisition at Comparable Valuation

Precedent: AbbVie paid $2.1B at first patient dosed · Astellas paid $2.2B at first patient dosed

Pathway: Program financing → IND-enabling safety studies → First-In-Patient (FIP) → Interim biomarker data → Investment bank NDR → Strategic acquisition · Full analysis available in Investment Summary after CDA

Capstan/AbbVie acquisition: BioPharma Dive / press release, 2023 · AviadoBio/Astellas acquisition: Fierce Biotech / press release, 2024 · All programs investigational

Why First-In-Patient Changes Everything

We are one safety study away from the first patient dosed — and that is not how most drugs work

Most drugs require healthy volunteer trials before reaching patients.

PINK1 gene therapy goes directly to patients — because the patient's own genetics are the basis for the therapy. One IND-enabling safety study separates us from first patient dosing. The Phase 1/2 IS the pivotal trial. That is why strategic acquirers buy at this stage.

Conventional Drug Development

① Preclinical animal studies

② **Phase 1 — healthy volunteers (safety only)**

③ Phase 2 — patients (efficacy)

④ Phase 3 — large pivotal trial

⑤ FDA approval

Typical timeline: 10–15 years

PINK1 Early-Onset PD Pathway

① IND-Enabling Safety Studies

② **First-In-Patient (FIP) ← WE ARE HERE NEXT**

③ **Phase 1/2 = Pivotal Trial**

④ RMAT Accelerated Approval

⑤ Commercial Launch

Pathway: financing → FIP → pivotal data → exit

Why acquirers buy at FIP: After first-in-patient dosing, the next study is the pivotal study — the one that generates approval data. An acquirer who waits until after pivotal data pays 5–10x more. Buying at FIP captures the upside of approval at pre-approval pricing. AbbVie paid $2.1B at first patient dosed. Astellas paid $2.2B at first patient dosed. That is not coincidence — that is strategy.

IND = FDA application required before human trials · FIP = First-In-Patient · RMAT = FDA Regenerative Medicine Advanced Therapy · TGA = Australian Therapeutic Goods Administration

Regulatory Advantage: Three Simultaneous Fast-Track Pathways

Each independently valuable — together they de-risk approval, reinforced by global FDA/EMA regulatory modernization

RMAT Designation (FDA)

Regenerative Medicine Advanced Therapy

FDA's fastest pathway for gene therapies. Allows submitting approval sections as completed — not all-at-once at the end. Otarmeni (AAV gene therapy, OTOF gene, 24 patients) approved in 61 days under RMAT + CNPV on April 23, 2026 — the most direct precedent for PINK1.

Investor benefit: Rolling approval process · Intensive FDA guidance · Potential approval in months, not years

Otarmeni (AAV gene therapy) approved in 61 days under RMAT + CNPV — April 23, 2026[1]

Rare Pediatric Disease Designation (RPD)

Priority Review Voucher (PRV) — Potential Upside

Programs meeting FDA rare pediatric disease criteria may be eligible for a Priority Review Voucher upon approval — a transferable certificate sellable to large pharmaceutical companies. Pathways is evaluating RPD eligibility based on PINK1 disease biology and genetic onset characteristics. PINK1-associated Parkinson's includes early-onset and, in some cases, adolescent presentation — supporting evaluation for Rare Pediatric Disease designation.

Investor benefit: Recent PRV sales: $155M–$210M per voucher · Subject to FDA designation and approval

Subject to FDA designation · Reauthorized through Sept 30, 2029 (CAA 2026)[2]

Australia TGA First-In-Patient

Therapeutic Goods Administration

Australia's regulatory framework allows first-in-patient gene therapy trials to begin faster than in the U.S. Data generated in Australia is accepted by both FDA and EMA — one study, three jurisdictions. FDA and EMA's January 2026 joint AI principles and ongoing global harmonization further support a development package that translates across U.S. and EU markets.

Investor benefit: Pacific Islander PINK1 prevalence: 1 in 1,300 · Data accepted globally

ICH GCP-compliant data accepted by FDA + EMA[3]

[1] Otarmeni approval, FDA press release April 23, 2026 (61 days, RMAT + CNPV) ; [2] CAA 2026 (P.L. 119-75, Section 6604) ; [3] ICH E6(R3) GCP guidelines, TGA effective January 13, 2026

How This Investment Creates Value

Three independent value streams — each meaningful on its own

Pathways generates investor returns through three distinct, independently valuable mechanisms. A detailed risk-adjusted valuation analysis — including scenario modeling and comparable transaction data — is available in the Investment Summary, provided following execution of a Confidentiality and Non-Disclosure Agreement.

1 Commercial Revenue

PINK1 Gene Therapy + DRD1A Platform

→ PINK1: one-time administration at gene therapy pricing — anchored to approved commercial comparables

→ DRD1A: annual recurring re-dosing therapy — replaces chronic levodopa for life

→ Commercial partnership provides integrated clinical, regulatory, and commercial infrastructure for rare disease programs

→ Combined addressable population: genetically defined, identifiable, enrollable today

2 Priority Review Vouchers

Non-Dilutive, Transferable Government Certificates

→ Awarded by FDA upon approval of each Rare Pediatric Disease program

→ Two vouchers eligible — PINK1 and DRD1A — each independently valuable

→ Vouchers are transferable and sellable to large pharmaceutical companies

→ Program reauthorized through September 30, 2029 — regulatory window is confirmed open

3 Strategic Acquisition

Five Named Acquirers · Documented M&A Logic

→ Five strategic acquirers identified — each with documented prior investment in this disease area

→ First-In-Patient dosing triggers the investment bank NDR and competitive acquisition process

→ M&A comps anchor the exit range to real, completed transactions in gene therapy and rare neurology

→ Acquiring before approval is how strategic buyers capture the upside — that is the value of FIP timing

Full valuation analysis available in the Investment Summary — provided following execution of CDA and accredited investor attestation · Contact: anthony.mack@pathwaysnp.com

The Exit: Five Named Acquirers, Documented M&A Logic

Each identified acquirer has made a strategic investment in genetic Parkinson's or CNS gene therapy within the past 3–5 years — and they have reason to want ours specifically

AbbVie	Already owns PINK1 biology (acquired Mitokinin, $655M, 2022). Also filed tavapadon NDA (D1 pathway) in 2025. Pathways completes both their gene therapy franchises.	*AbbVie/Capstan: $2.1B at first patient dosed*
Eli Lilly	Bought Prevail Therapeutics (AAV9, GBA1, Parkinson's gene therapy) for $1.04B in 2021. Has the infrastructure and the strategic rationale to complete their genetic PD franchise.	*Lilly/Prevail: $1.04B, gene therapy PD*
Novartis	Owns Zolgensma (world's most expensive drug — one-time AAV gene therapy). Understands rare pediatric disease gene therapy pricing and has commercial infrastructure globally.	*Zolgensma comps: $2.1M/patient*
Biogen	Acquired Reata Pharmaceuticals for $7.3B in 2023 — a rare neurological platform with recurring revenue. The DRD1A annual re-dosing model mirrors exactly what Biogen paid a premium for.	*Biogen/Reata: $7.3B, rare neurology*
Astellas	Acquired AviadoBio (CNS AAV gene therapy, Parkinson's) for up to $2.2B at first patient dosed in 2024. Most direct precedent — same disease area, same development stage, same vector class.	*Astellas/AviadoBio: $2.2B at FIP*

M&A comps: Capstan/AbbVie 2023 · Lilly/Prevail 2021 · Astellas/AviadoBio 2024 · Biogen/Reata 2023 · AbbVie/Mitokinin 2022 · Sources: SEC filings, press releases, BioPharma Dive

Why Invest Now: The Window Is Defined

First-In-Patient dosing is the event that re-rates this company. Before FIP, investors access the asset at pre-clinical pricing. After FIP, the asset trades at a multiple of today's value.

FIP Re-Rates Everything

First human dosing triggers the investment bank NDR and competitive acquisition process. Early investors who come in before FIP access the asset at the most favorable price in the company's history.

Regulatory Window Is Open

FDA's RMAT pathway, the Rare Pediatric Disease PRV program (reauthorized through 2029), and Australia TGA's fast-track capacity are all active today. These windows are not guaranteed to remain open.

PRVs Represent Documented Upside Potential

Programs qualifying for Rare Pediatric Disease designation may receive Priority Review Vouchers upon approval — transferable, sellable assets independent of the M&A exit. Recent PRV sales: $155M–$210M each. Pathways is evaluating RPD eligibility across multiple programs.

The Acquirers Are Already Engaged

AbbVie, Lilly, Novartis, Biogen, and Astellas are not theoretical buyers. Each has made documented investments in genetic Parkinson's programs in the past three years. They are building these franchises now.

PRV comps: Abeona $155M (2025), Jazz $200M (Jan 2026), Cyprium $205M (Feb 2026). CAA 2026, P.L. 119-75, Section 6604.

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The Investment Case — Summary

- ✓ Genetically defined patient population — identifiable and enrollable today
- ✓ One-time gene therapy addressing a known, inherited cause — no treatment like this exists
- ✓ Three simultaneous regulatory fast-tracks: RMAT, RPD, and Australia TGA
- ✓ Potential Priority Review Voucher (PRV) eligibility across multiple programs — subject to designation and approval
- ✓ Five named strategic acquirers with documented activity in this exact disease area
- ✓ Investment bank engagement in process — post-FIP NDR and competitive auction process planned
- ✓ Detailed valuation analysis available in the Investment Summary — request after signing CDA

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