



AXOVANT

THE POWER OF AN OPEN MIND™

Building a leader in innovative CNS therapies

June 2018

FORWARD LOOKING STATEMENTS

Statements made in this presentation contain forward-looking statements, including statements regarding Axovant's expectations about timing of the results for the Phase 1/2 clinical study for AXO-Lenti-PD in Parkinson's disease, expected outcomes of planned clinical development for AXO-Lenti-PD, Axovant's license arrangement with Oxford BioMedica, the expected equity investment from Roivant, and other elements of Axovant's clinical development and regulatory strategy. Forward-looking statements can be identified by the words "believe," "anticipate," "continue," "estimate," "project," "expect," "plan," "potential," "intend," "will," "would," "could," "should" or the negative or plural of these words or other similar expressions that are predictions or indicate future events, trends or prospects. Forward-looking statements are subject to risks and uncertainties that could cause actual results to differ materially and reported results should not be considered as an indication of future performance. These risks and uncertainties include, but are not limited to: risks associated with the success, cost and timing of our product development activities and clinical trials; the approval and commercialization of Axovant's product candidates, including AXO-Lenti-PD; and increased regulatory requirements. These statements are subject to the risk that clinical trial data are subject to differing interpretations, and regulatory agencies, medical and scientific experts and others may not share Axovant's views of the clinical study data. In addition, promising interim results or other preliminary analyses do not in any way ensure that later or final results in a clinical trial or in related or similar clinical trials will replicate those interim results. The product candidates discussed are investigational and not approved and there can be no assurance that the clinical programs including the program for AXO-Lenti-PD will be successful in demonstrating safety and/or efficacy, that Axovant will not encounter problems or delays in clinical development, or that any of Axovant's product candidates will ever receive regulatory approval or be successfully commercialized. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to Axovant's business in general, see the "Risk Factors" section of Axovant's quarterly report on Form 10-Q filed with the Securities and Exchange Commission on February 9, 2018, and other filings that Axovant makes with the SEC from time to time. These forward-looking statements are based on information available to Axovant as of the date of this presentation and speak only as of the date of this presentation. Axovant disclaims any obligation to update these forward-looking statements, except as may be required by law.

Building a leader in innovative CNS therapies

AXO-Lenti-PD: potential best-in-class gene therapy for Parkinson's disease

- Delivery of three genes that encode critical enzymes necessary for endogenous dopamine synthesis in the brain (AADC, TH, and CH1) using lentiviral vector
- Plan to initiate Phase 1/2 study in advanced Parkinson's disease by the end of 2018

Oxford BioMedica will be the clinical & commercial supplier of AXO-Lenti-PD, if approved

- World leader in lentiviral vector product development and manufacturing
- Commercial supplier of lentiviral vector for KYMRIAH® (Novartis), first FDA-approved CAR-T therapy in US

New additions to leadership to bolster gene therapy and CNS expertise

- Fraser Wright, Chief Technology Officer (Co-Founder and former CTO of Spark Therapeutics)
- Michael Hayden, Senior Scientific Advisor and Chairman, Axovant Scientific Advisory Board (former President of Global R&D and Chief Scientific Officer of Teva)
- Gavin Corcoran, EVP Research & Development (CMO of Allergan)

Roivant strategic financing enables continued pipeline expansion

- Roivant committed to invest \$25 million in Axovant
- Funds will be used to support clinical development of AXO-Lenti-PD and additional business development

New additions to leadership team bring decades of gene therapy and CNS expertise



Fraser Wright, Ph.D.

Chief Technology Officer, Gene Therapies

- Co-Founder & Former Chief Technology Officer, Spark Therapeutics
- More than 20 years of experience in gene therapy product development, manufacturing, and quality control testing, including LUXTURNA™ and KYMRIAH®
- Director, Clinical Vector Core Laboratory, Children's Hospital of Philadelphia
- Faculty, University of Pennsylvania Perelman School of Medicine
- Director, Development and Clinical Manufacturing, Avigen



Michael Hayden, MB ChB, Ph.D.

Senior Scientific Advisor and Chairman, Axovant Scientific Advisory Board

- Former President, Global R&D, and Chief Scientific Officer, Teva
- GLYBERA®, first-ever approved gene therapy, was initially conceived in Hayden's lab
- Killam Professor of Medical Genetics, University of British Columbia; Canada Research Chair in Human Genetics and Molecular Medicine
- Program Director, Translational Laboratory in Genetic Medicine, National University of Singapore



Gavin Corcoran, MB ChB, FACP

Executive Vice President, Research and Development

- Currently Chief Medical Officer at Allergan plc
- Previously served as Chief Medical Officer of Actavis and Executive Vice President for Global Medicines Development at Forest Laboratories
- Head of Late Stage Clinical Development for Inflammation and Immunology at Celgene



AXOVANT



OxfordBioMedica

Extensive drug development
experience in neurodegeneration

Track record of rapid clinical
execution in neurology

Global leader in gene therapy
with 20+ years of experience

Demonstrated manufacturing
capabilities for commercially
available gene therapy products

Parkinson's disease remains an area of high unmet medical need

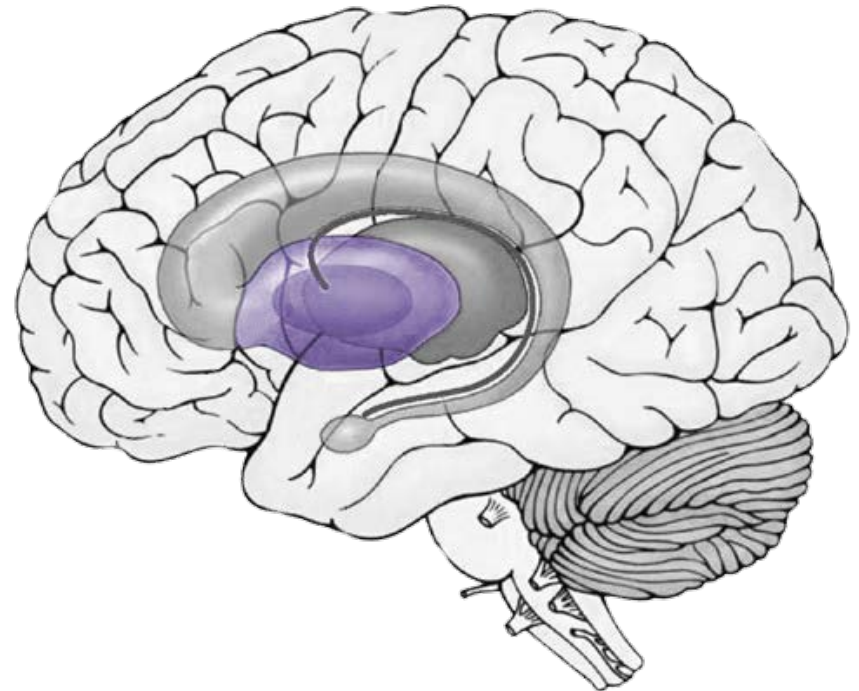
Parkinson's disease (PD) is a progressive neurodegenerative disorder resulting in the loss of dopamine in the striatum

Motor symptoms can include tremor, rigidity, and bradykinesia

PD affects approximately 1% of adults over the age of 60, or 7-10 million patients worldwide

Current standard of care is primarily oral L-dopa. However, significant unmet need exists in treated patients:

- Waning efficacy over time
- Fluctuations between ON and OFF states
- Dyskinesias

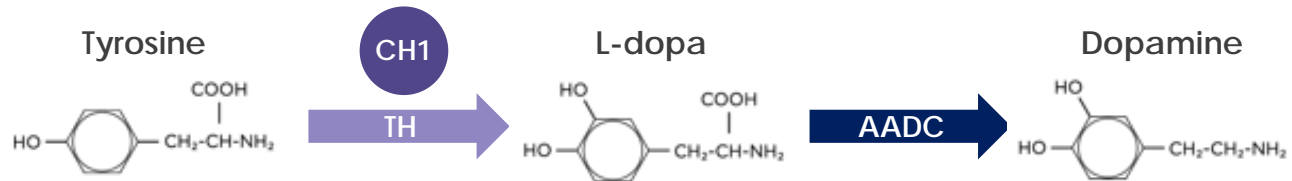


AXO-Lenti-PD: a novel gene therapy for Parkinson's disease



AXO-Lenti-PD contains three genes that encode the critical enzymes required for endogenous dopamine synthesis

- **Tyrosine hydroxylase (TH):** converts tyrosine to L-dopa
- **Cyclohydrolase 1 (CH1):** rate-limiting enzyme for synthesis of essential cofactor in TH activity
- **Aromatic L-amino acid decarboxylase (AADC):** converts L-dopa to dopamine



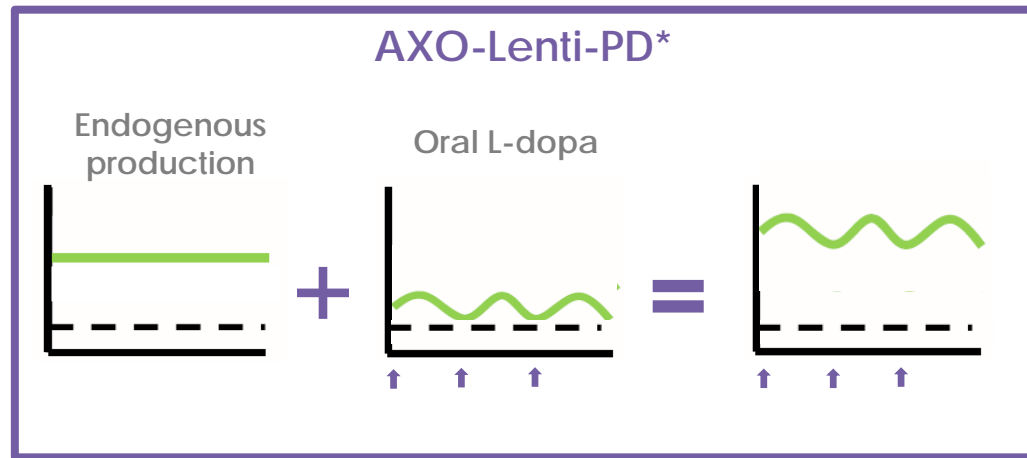
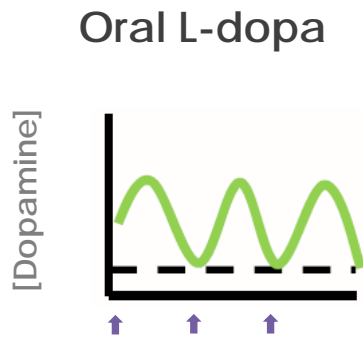
Lentiviral vector system with large gene packaging capacity

- Permits delivery of multiple genes at once



One-time MRI-guided stereotactic delivery into the putamen

AXO-Lenti-PD: designed to reduce motor fluctuations in Parkinson's disease



AXO-Lenti-PD's novel 3-gene therapy approach is designed to (1) increase basal dopamine production and (2) reduce dopamine variability

GOALS OF THERAPY:

- Less troublesome dyskinesia
- Less OFF time
- More ON time
- Lower requirement for exogenous L-dopa

* Theoretical benefits based on postulated mechanism of action (not data from investigational studies)

Why use lentiviral vector?

Lentiviral vector approach may confer unique advantages:

- ✓ **Large vector packaging capacity** enables efficient delivery of multiple genes in a single cassette
- ✓ Lentiviral vectors are capable of integrating into the host genome, which may help **maintain long-term stable transgene expression**
- ✓ To date, **well-tolerated with limited treatment-related adverse events**, based on clinical and commercial experience with lentiviral vectors

ProSavin[®]: proof of concept from earlier vector construct

ProSavin was the predecessor therapy to AXO-Lenti-PD

- Delivery of three genes in the same lentiviral vector as AXO-Lenti-PD, but in a different payload configuration
- One-time MRI-guided stereotactic delivery

Phase 1/2 study of ProSavin in patients with advanced Parkinson's disease (n=15) completed

- Mean UPDRS Part III (motor) "OFF" scores were significantly improved at 6 months and 12 months (p-value=0.0001 at both time points)
- Sustained improvement seen through 4 years of follow-up

Favorable safety and tolerability profile

- No serious adverse events related to ProSavin or the surgical procedure
- Long-term follow-up ongoing

ProSavin: completed Phase 1/2 study

Phase 1/2 study completed in patients with advanced Parkinson's disease (n=15)

- Study was conducted in UK and France
- Results published in *The Lancet* (Palfi, 2014)

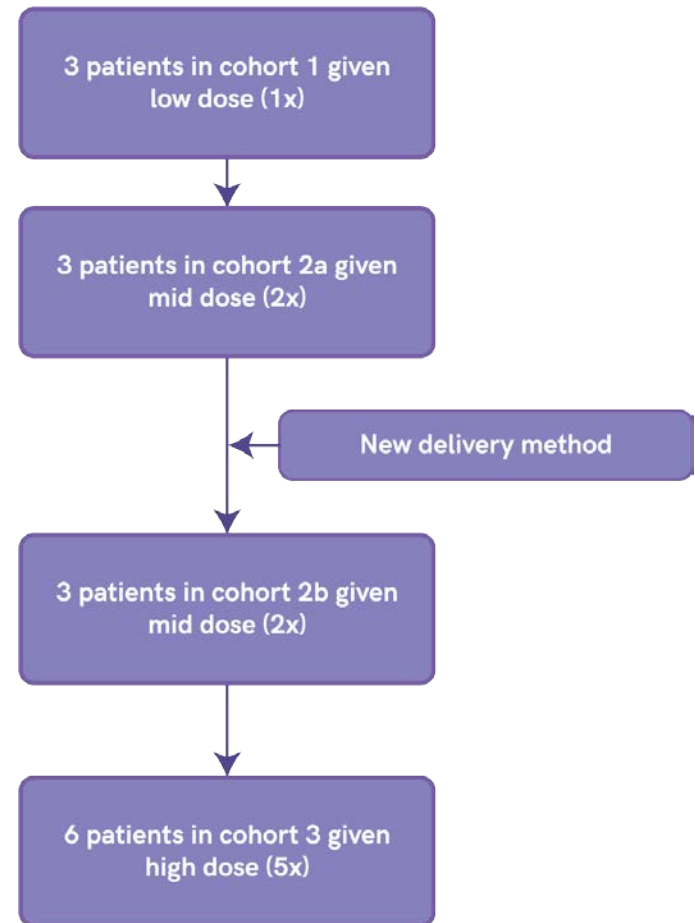
Cohorts in dose-escalation paradigm:

- Cohort 1: Low dose (1.9×10^7 TU)
- Cohort 2a: Mid dose (4.0×10^7 TU)
- Cohort 2b: Mid dose (4.0×10^7 TU) with new delivery method
- Cohort 3: High dose (1.0×10^8 TU)

Primary endpoints in Phase 1/2 study

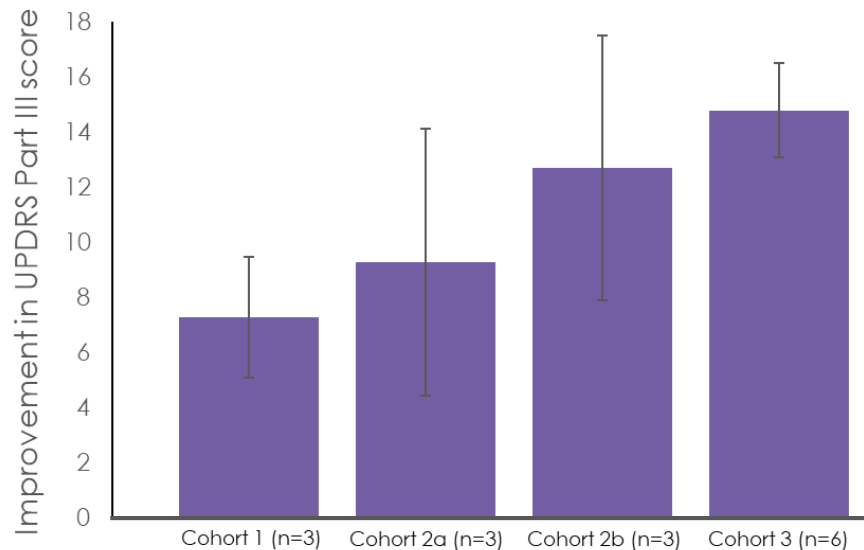
- Number and severity of adverse events
- UPDRS Part III (motor) "OFF" scores at 6 months after vector administration

ProSavin Phase 1/2 Study Design



ProSavin: multiple doses evaluated in Phase 1/2 study

Mean Improvement in UPDRS Part III (motor) "OFF" score at 12 Months



All patients (n=15):

Mean improvement from baseline of 11.8 points at 12 months (p=0.0001)

Mean reduction in L-dopa equivalent daily dose (LEDD) of 19% at 12 months

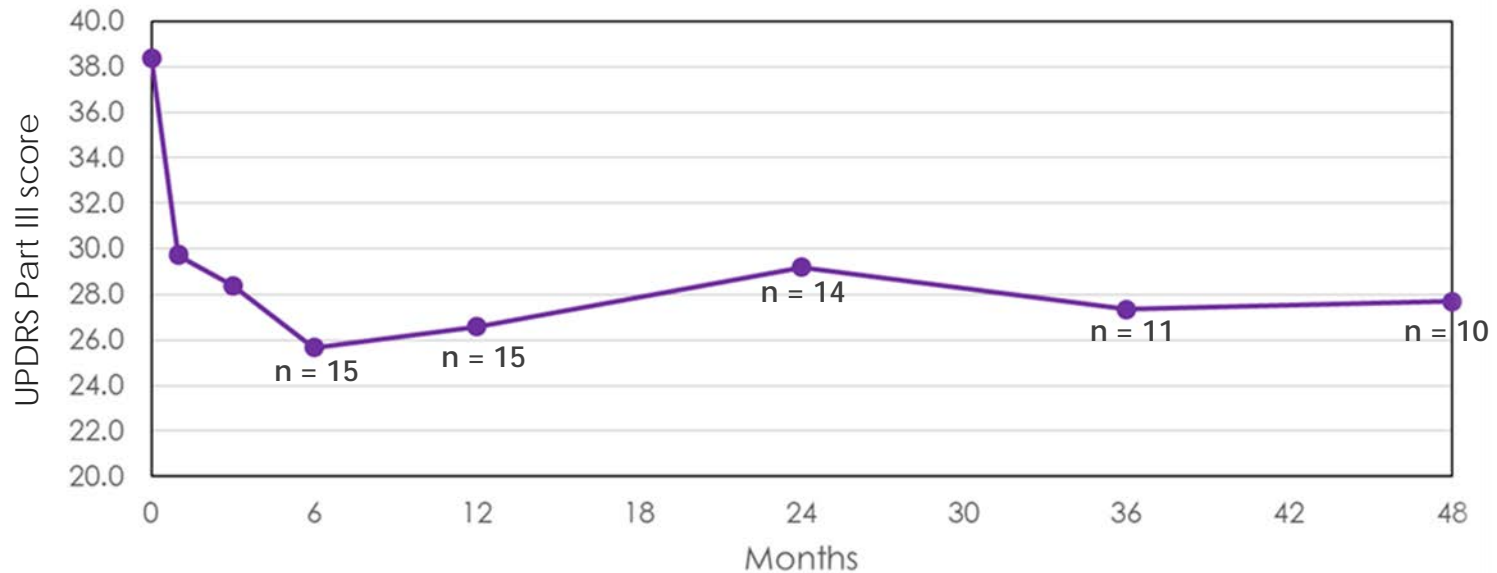
Cohort 1 (low dose): 1.9×10^7 TU

Cohort 2a and 2b (mid dose): 4.0×10^7 TU

Cohort 3 (high dose): 1.0×10^8 TU

ProSavin: sustained response observed several years after administration

Mean UPDRS Part III (motor) "OFF" score



Durable effects seen through 4 years after one-time administration of ProSavin

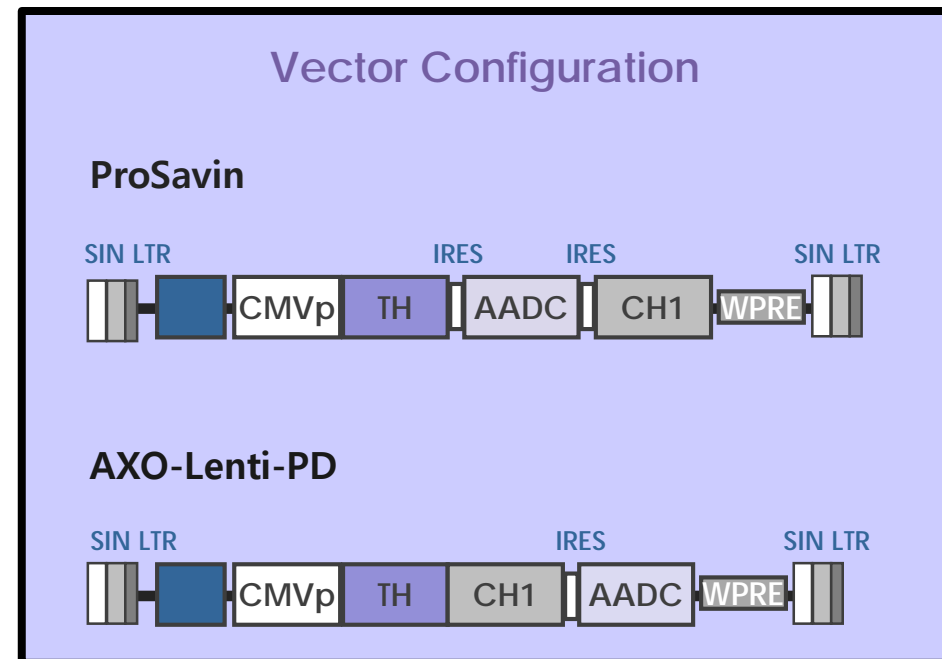
UPDRS Part III (motor) "OFF" scores are typically expected to worsen by 3-4 points/year in this population*

AXO-Lenti-PD: a re-engineered gene therapy product

AXO-Lenti-PD achieves up to 10-fold increases in dopamine + L-dopa production compared to ProSavin, without impacting infusion volume or rate of administration

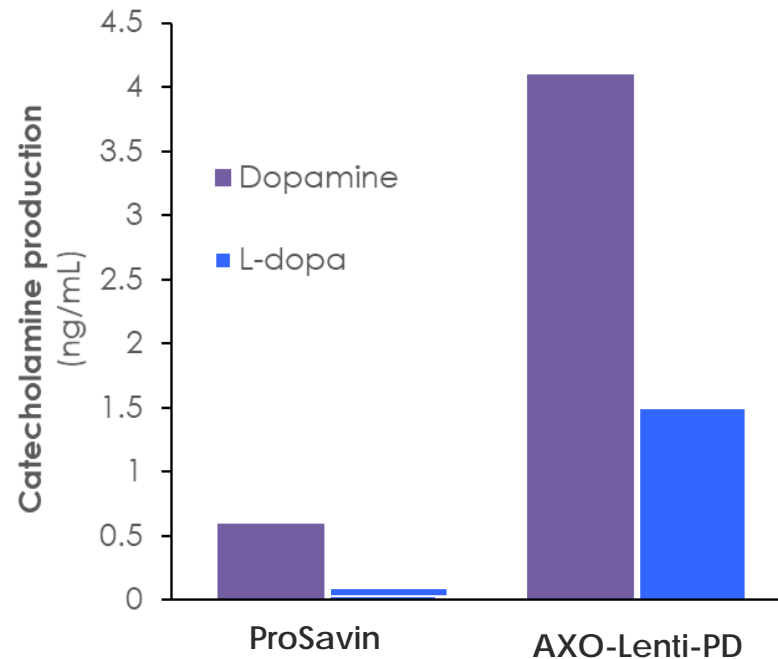
AXO-Lenti-PD was the product of multifactorial experimentation to modify the payload configuration to improve dopamine production

- Different ordering of genes
- Balanced stoichiometry of transgene expression to ensure consistent 1:1 production of TH and CH1
- Fusion of TH and CH1 with flexible linker



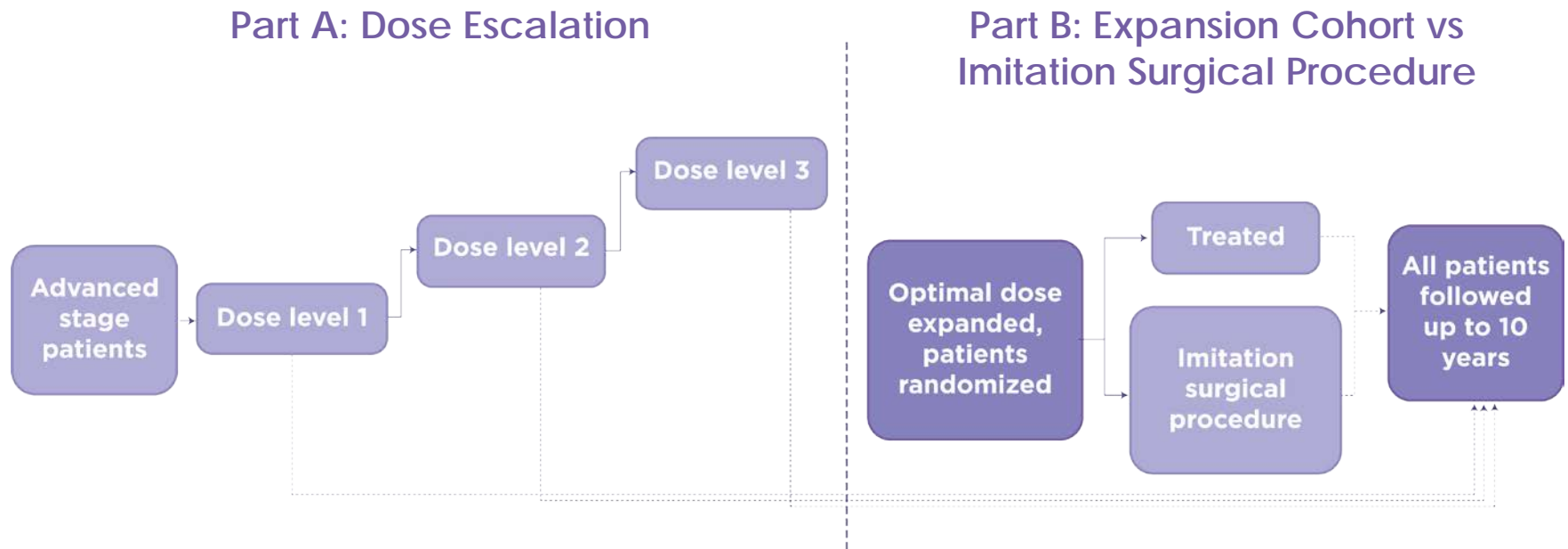
AXO-Lenti-PD: increases catecholamine production compared to ProSavin

Production of catecholamines (L-dopa & Dopamine) in Primary Human Neurons with ProSavin and AXO-Lenti-PD



AXO-Lenti-PD achieved up to 10-fold increase in dopamine + L-Dopa production compared to ProSavin

AXO-Lenti-PD: anticipated phase 1/2 study design



First patient expected to be dosed by end of 2018
Study will capture **sham-controlled** data during Part B

Delivery method: one-time MRI-guided stereotactic delivery with automatic pump

Key assessments: Safety and tolerability, Biomarkers, UPDRS scores and other measures of motor function

Oxford BioMedica: our clinical and commercial manufacturing partner

An industry leader in GMP lentiviral vector manufacturing and commercial production

- Commercial-grade vector manufacturing
- GMP cell banks for adherent and suspension processes
- Certified clean room capacity across three independent suites on two separate sites

The first company to administer an *in vivo* lentiviral vector into patients

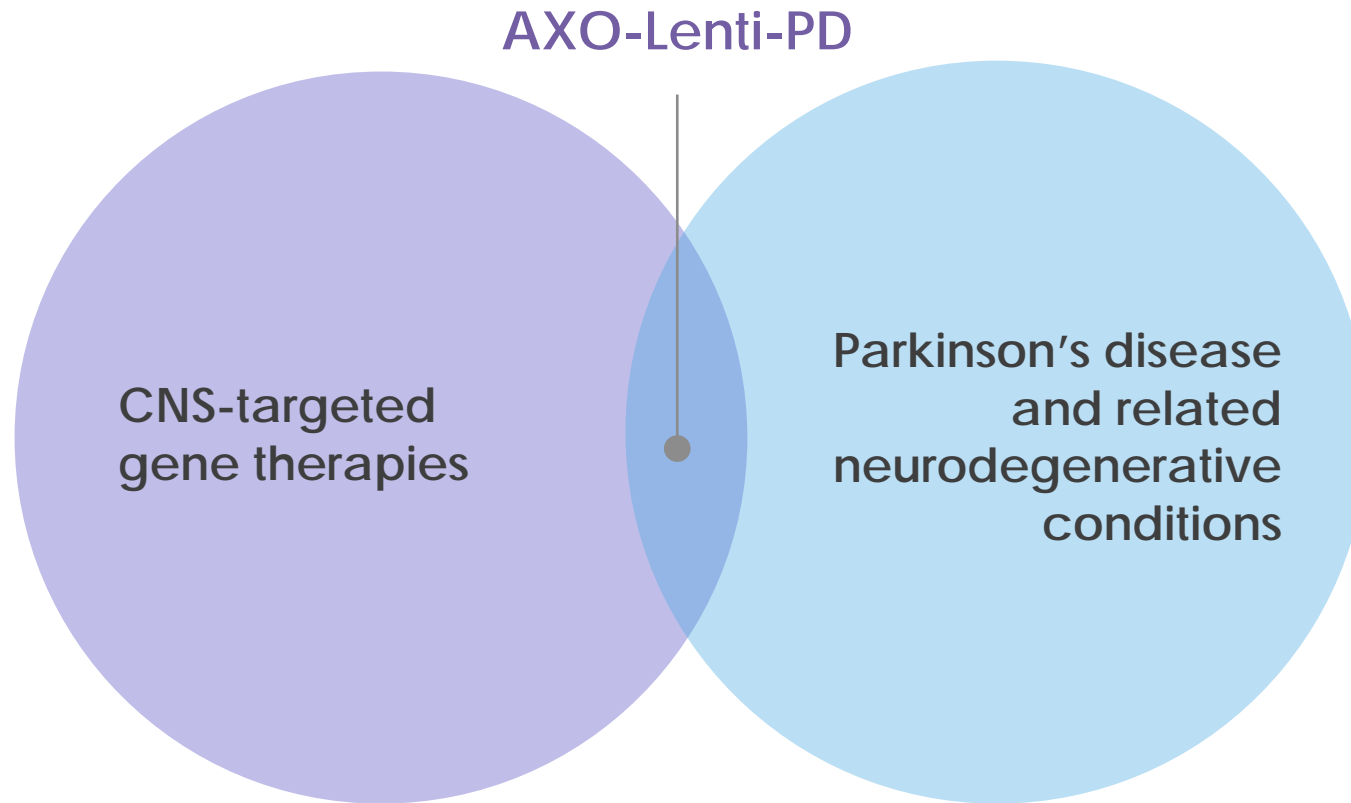
Established strategic partnerships with Novartis (CAR-T), Sanofi/Bioverativ (ophthalmological, hemophilia), Orchard Therapeutics (stem cells)



Financial terms of transaction

Upfront	\$30M in cash Including \$5M pre-payment for manufacturing services
Development Milestones (total)	\$55.0M
Regulatory and Commercial Milestones (total)	\$757.5M
Royalties on Net Sales	7% on annual net sales < \$1B 8% on annual net sales > \$1B and < \$2.5B 9% on annual net sales > \$2.5B and < \$4B 10% on annual net sales > \$4B

Plans for continued pipeline expansion



More pipeline expansion expected along these verticals in FY2018