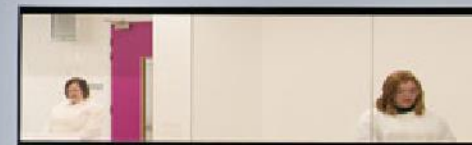


# The LentiVector® Platform Company

A leader in gene and cell therapy

## Oxford BioMedica and Axovant Worldwide Exclusive Licence Agreement for OXB-102 for Parkinson's disease

06 June 2018



## Forward-looking statements

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# A world leading gene and cell therapy company



## Growing Market



Oxford BioMedica is at the centre of the gene and cell therapy revolution – a growing multi-billion \$ market<sup>1</sup>. Three therapies approved in six months and seven more over the next few years.

01

## Profitable Platform



Partnerships with Novartis, Bioverativ, Orchard Therapeutics, GC LabCell and Immune Design.

02

## Developing Products



Developing a pipeline of our own assets in key therapeutic areas - to be either spun out or out-licensed. Products and patents licensed to Sanofi and Axovant.

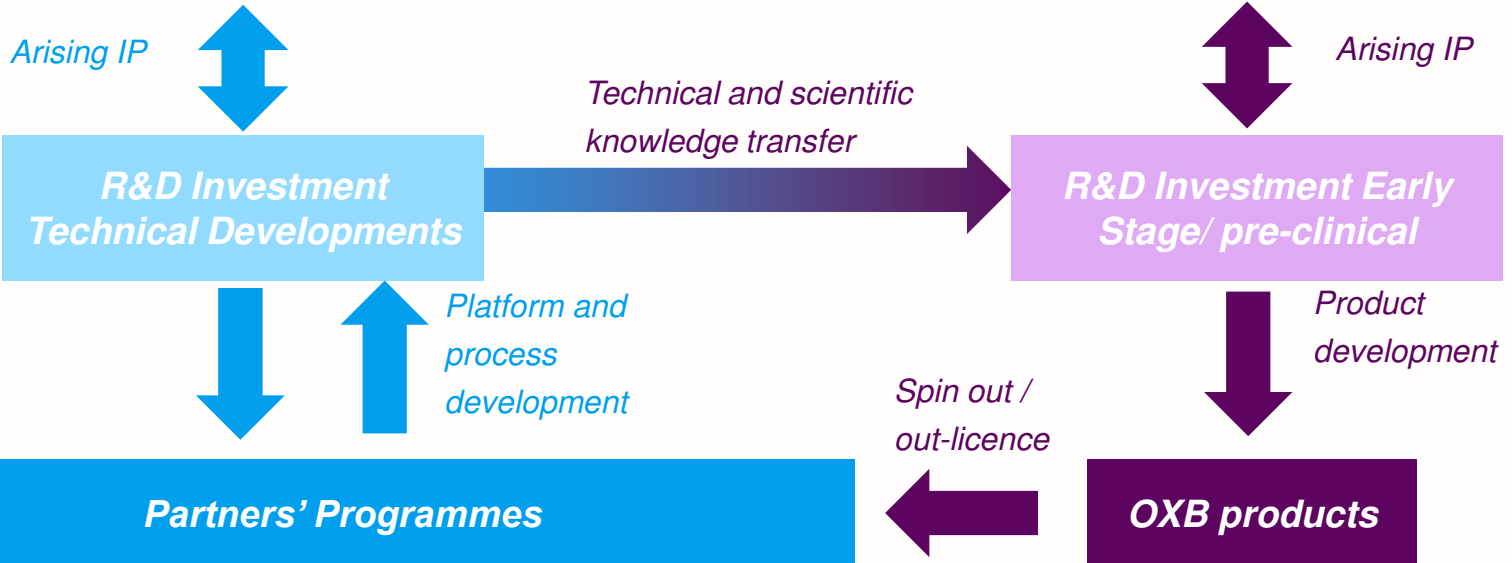
03

<sup>1</sup>Clive Glover, GE Healthcare "Sales of cell and gene therapy will reach \$10 billion by 2021", October 2015.

# Strategy: Leveraging our LentiVector<sup>Enabled</sup> delivery platform

## LentiVector<sup>®</sup> Platform

IP – patents and know-how | Facilities | Expertise | Quality systems



Partners' Programmes

- Multiple income streams
- Process development fees
- Process development incentives
- Bio-processing revenues
- Royalties

Orchard therapeutics | IMMUNE DESIGN | Bioverativ | NOVARTIS

OXB products

- Upfront & milestones
- Royalties
- Development funding

SANOFI | AXOVANT | GC LabCell

# Worldwide Exclusive Licence Agreement for OXB-102 (AXO-Lenti-PD)



# Worldwide exclusive licence agreement with AXOVANT

## Licence agreement overview

- Worldwide exclusive licensing agreement to develop and commercialise OXB-102 (now named AXO-Lenti-PD) for Parkinson's disease (includes all indications)
- Licence to Oxford BioMedica's LentiVector Enabled technology
- Access to Oxford BioMedica's industrial-scale manufacturing technology
- Intend on putting in place agreements for clinical and commercial supply
- Axovant will be able to harness the full Roivant drug development platform to ensure its rapid development
- Phase I/II study in advanced Parkinson's disease expected to initiate by the end of 2018

## Key Terms

- Headline value \$842.5 million
- \$30 million upfront (including \$5m pre-payment for manufacturing services)
- In addition achieve payment of:
  - \$55 million for specified development milestones
  - \$757.5 million for specified regulatory and sales-related milestones
- 7-10% tiered royalty payable on net sales

# Axovant

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- Axovant Sciences (NASDAQ: AXON) is a US listed clinical-stage biopharmaceutical company dedicated to advancing treatments for patients with life-altering neurologic conditions
- Their mission is to transform promising therapies into solutions for patients
- Axovant expertise and focus on neurological disorders (including Parkinson's disease) makes them an ideal development and commercialisation partner for OXB-102 (now known as AXO-Lenti-PD)
- Axovant has strong support from its parent Roivant and is perfectly positioned to bring AXO-Lenti-PD to the market as quickly as possible to treat patients with Parkinson's disease

**AXOVANT**



# Products






# Product pipeline

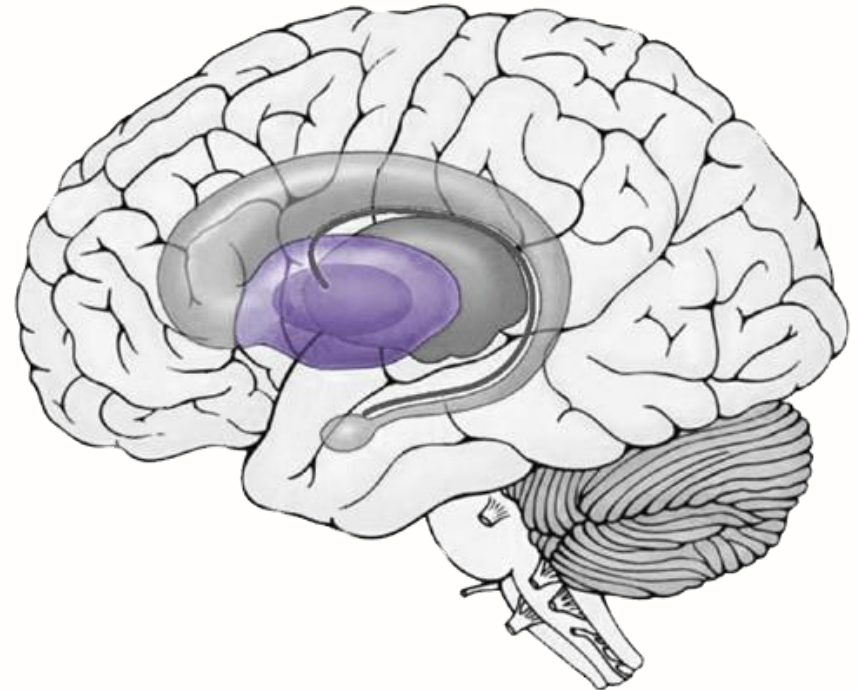
Product	Indication	Pre-Clinical	Phase I	Phase I/II	Phase II	Phase III	Approval
<b>OXB Proprietary Products</b>							
OXB-202	Corneal graft rejection	▶					} To be spun out or out-licensed
OXB-302	Cancer, multiple	▶					
OXB-201	Wet AMD	▶					
<b>OXB Partnered Products</b>							
AXO-Lenti-PD	Parkinson's disease	▶					} Development milestones and royalties
SAR422459	Stargardt disease	▶					
SAR421869	Usher syndrome 1B	▶					



 In vivo programmes

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

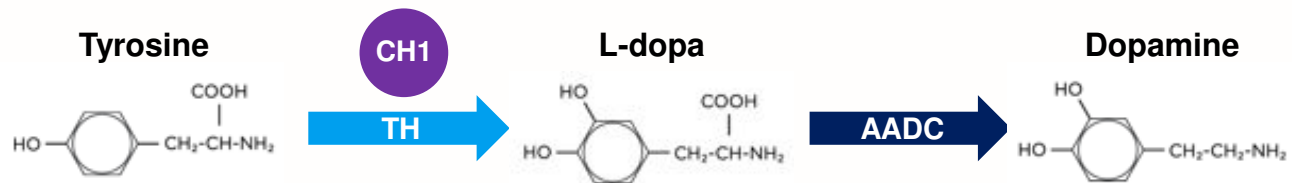
- Parkinson's disease (PD) is a progressive neurodegenerative disorder resulting in the loss of dopamine in the striatum
- Motor symptoms can include tremor, rigidity, and bradykinesia
- PD affects approximately 1% of adults over the age of 60, or 7-10 million patients worldwide
- Current standard of 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 (formerly OXB-102): a novel gene therapy for Parkinson's disease



- AXO-Lenti-PD contains three genes that encode the critical enzymes required for dopamine synthesis
  - **Tyrosine hydroxylase (TH)**: converts tyrosine to L-dopa
  - **Cyclohydrolase 1 (CH1)**: rate-limiting enzyme for synthesis of critical cofactor in TH activity
  - **Aromatic L-amino acid decarboxylase (AADC)**: converts L-dopa to dopamine

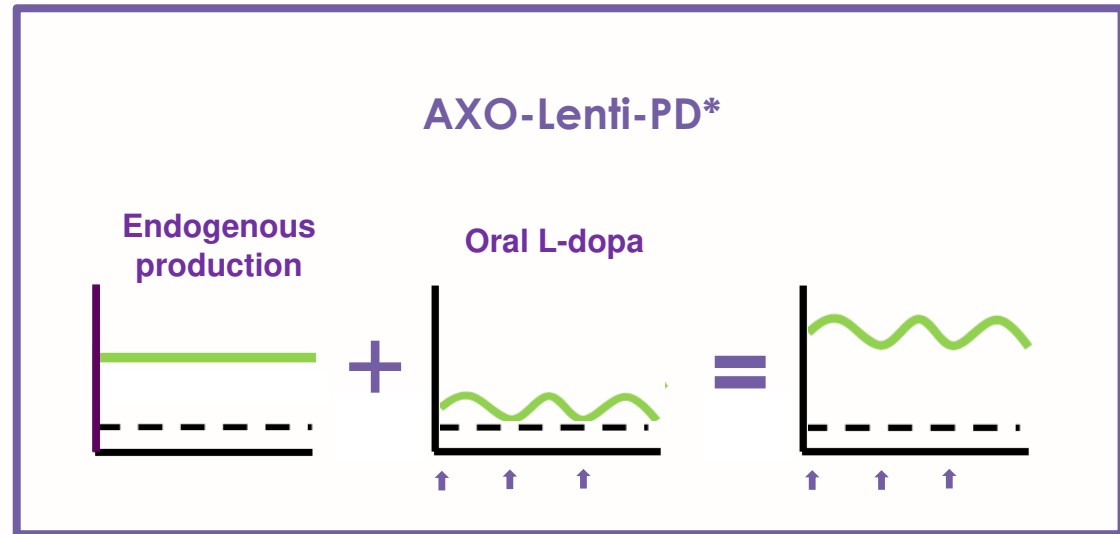
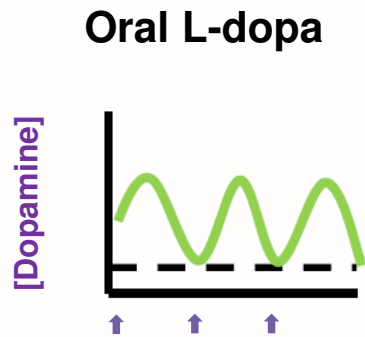


- Lentiviral vector system with large gene packaging capacity
  - Permits delivery of multiple transgenes at once



- One-time MRI-guided stereotactic delivery into the putamen

# AXO-Lenti-PD (formerly OXB-102) : 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**

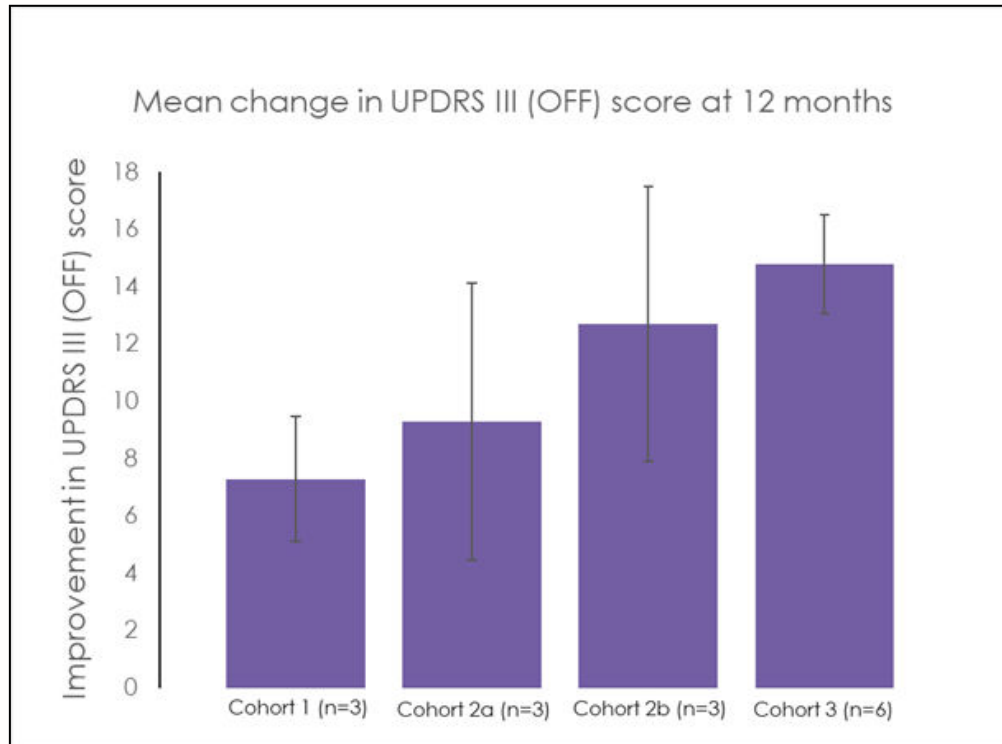
## **GOALS OF THERAPY:**

- 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)

# ProSavin®(OXB-101) multiple doses evaluated in Phase I/II study

## Mean Improvement in UPDRS-III (OFF) Score at 12 Months



- **All patients (N=15): Mean improvement from baseline of 11.8 points at 12 months (p=0.0001)**

**Cohort 1** (low dose):  $1.9 \times 10^7$  TU

**Cohort 2a and 2b** (mid dose):  $4.0 \times 10^7$  TU

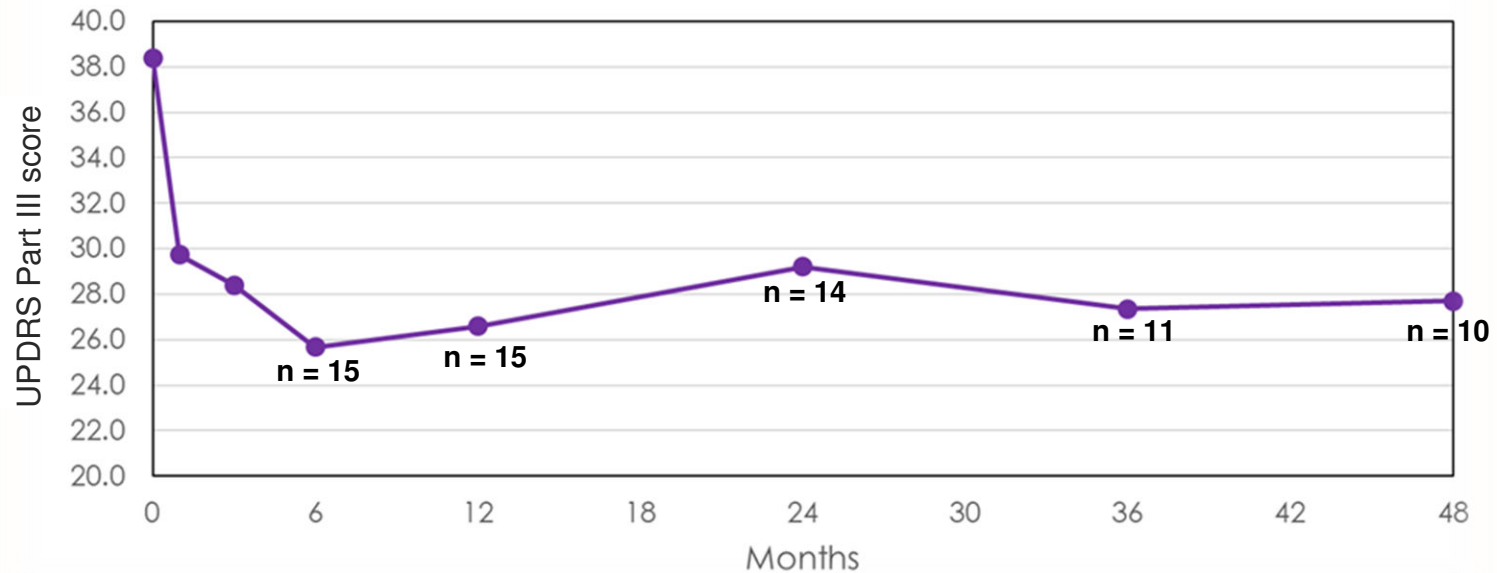
**Cohort 3** (high dose):  $1.0 \times 10^8$  TU

Source: Palfi S, Gurruchaga JM, et al. Long-term safety and tolerability of ProSavin, a lentiviral vector-based gene therapy for Parkinson's disease: a dose escalation, open-label, phase 1/2 trial. The Lancet. January 2014. [http://dx.doi.org/10.1016/S0140-6736\(13\)61939-X](http://dx.doi.org/10.1016/S0140-6736(13)61939-X)

Figure shows mean change in UPDRS III score OFF medication relative to baseline at 12 month for each cohort. Error bars show standard error. UPDRS = Unified Parkinson's Disease Rating Scale. Wilcoxon signed-rank paired test is used to compare the difference of UPDRS scores at 6 or 12 months versus baseline.

# ProSavin®(OXB-101) sustained response observed several years after administration

## Mean UPDRS-III (OFF) Score



- Durable effects seen through 4 years after one-time administration of ProSavin®
- UPDRS-III (OFF) scores are typically expected to worsen by 3-4 points/year\* in this population

Mean UPDRS III (OFF) scores pooled across low, mid, and high cohorts. Number of subjects: 15 (baseline to 24 months), 14 (24 months), 11 (36 and 48 months). Assessments post-deep brain stimulation (DBS) are excluded (n=3)

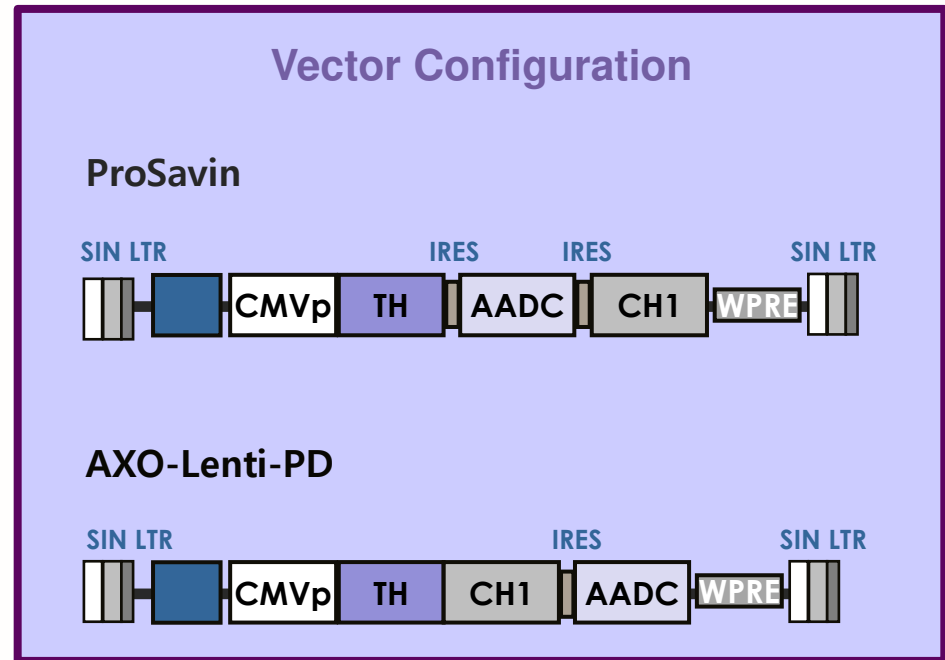
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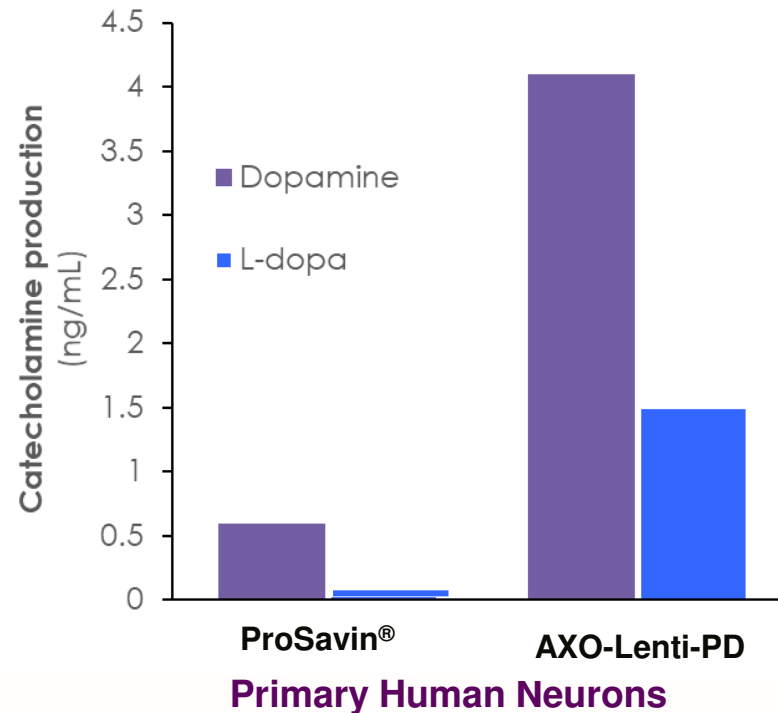
# AXO-Lenti-PD (formerly OXB-102): a re-engineered gene therapy product

**AXO-Lenti-PD achieves up to 10-fold increases in dopamine + L-dopa production compared to ProSavin® (OXB-101), without impacting infusion volume or rate of administration**

- **AXO-Lenti-PD was the product of multifactorial experimentation to modify the genetic payload to improve dopamine production**
  - Different ordering of transgenes
  - Balanced stoichiometry of gene expression to ensure consistent 1:1 production of TH and CH1
  - Fusion of TH and CH1 with flexible linker

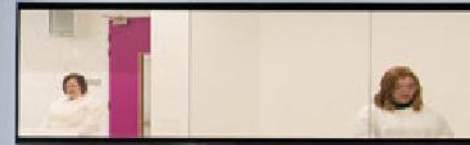


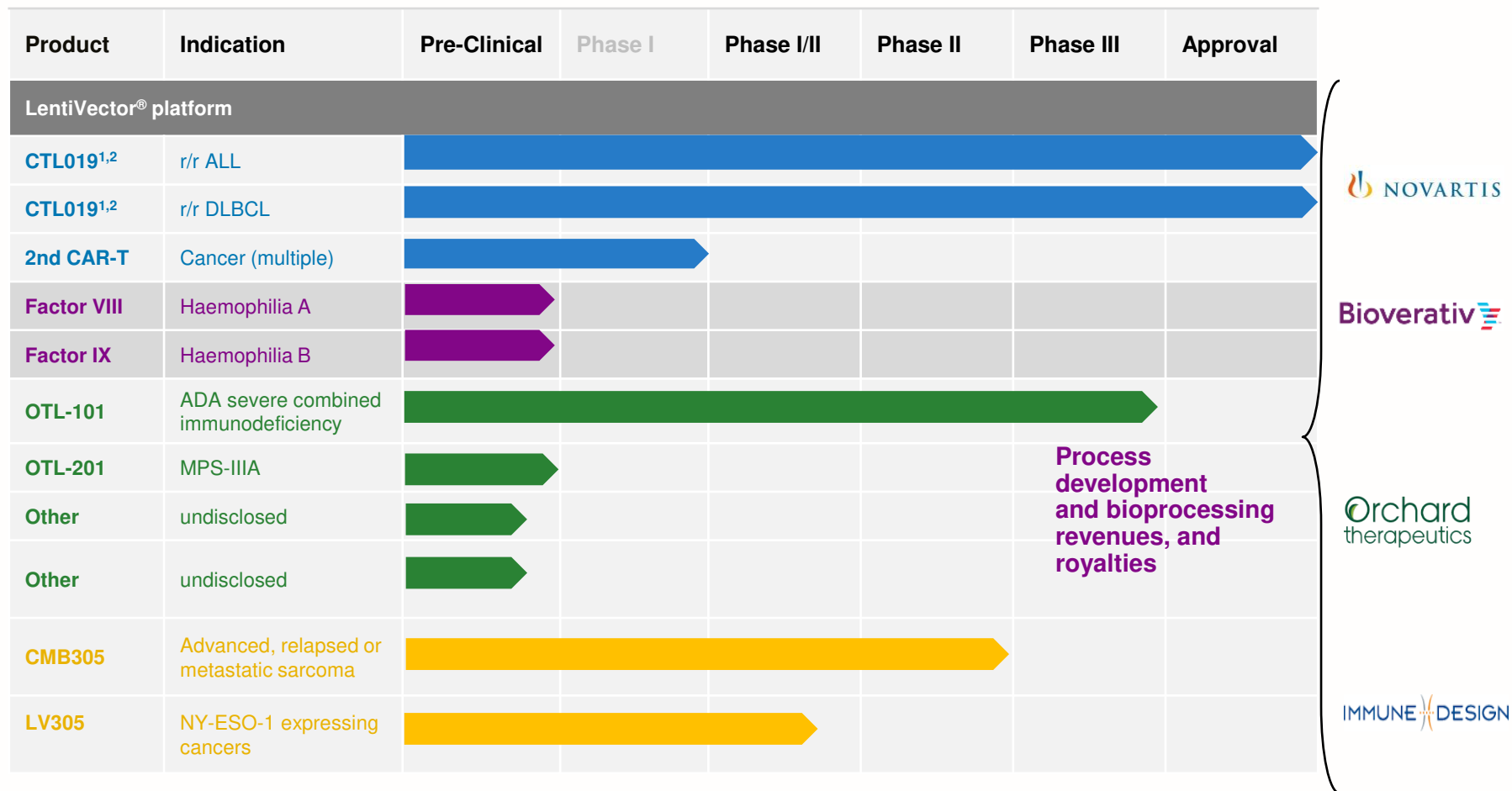
## AXO-Lenti-PD (formerly OXB-102): increases catecholamine production compared to ProSavin® (OXB-101)



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

# Platform





 *In vivo programmes*

<sup>1</sup> USAN name is tisagenlecleucel

<sup>2</sup> Approved in the US only

# Summary



# Expected news flow

## Partners' programmes

### Novartis

2nd CAR-T programme to enter clinic

Royalty stream from Novartis/CTL019<sup>1</sup> increasing in 2018

Expected EMA approval for paediatric r/r ALL and adult r/r DLBCL in EU in H1 2018

### Orchard Therapeutics

Intends to file a BLA for ADA-SCID during H2 2018

### Bioverativ

Bioverativ gene therapy product for haemophilia A & B progressing towards clinical development material by end of 2018

## LentiVector® delivery platform

Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates by end of 2018

Invest in further development of platform to improve the volume and yield from bioprocessing and efficacy of vector transduction of target cells during 2018

## In-house products

Advancement of AXO-Lenti-PD (formerly OXB-102) for Parkinson's disease into clinical development by Axovant - expected before year end 2018

Spin out / out-license of at least one in-house product candidates in H2 2018

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