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## Investment Proposition – a single investment in the success of a broad range of gene and cell therapy products from multiple companies

Oxford BioMedica is a gene and cell therapy company with the leading lentiviral vector delivery platform (LentiVector®)



- Several ex vivo products likely to reach the market within next 2-3 years
- Multiple players in ex vivo cell therapy CAR-T, TCR, Stem Cells, NK cells, etc.
- Many in vivo clinical studies, particularly in ophthalmology and CNS

#### Lentiviral vectors have advantages over other vector types

- Ex vivo cell therapies require integrating vectors lentiviral vectors are the preferred choice
- Lentiviral vectors beginning to demonstrate long-term efficacy which supports the "one-off" treatment hypothesis

## OXB's highly sought-after LentiVector<sup>®</sup> gene delivery platform

- Can be used for both in vivo and ex vivo lentiviral vector products
- Founded on 20 years' experience of delivering lentiviruses in vivo
- Integrated combination of our IP, employees' expertise and bioprocessing and laboratory facilities

#### OXB's product interests

- Two in-house products to enter Phase I/II clinical studies in next 12 months and a CAR-T pre-clinical program targeting solid tumours
- Economic interest in partners' products: Sanofi (SAR422459/SAR421869); Novartis (CTL-019 and other undisclosed CAR-T programme); Immune Design (LV305) and GSK (two undisclosed rare orphan products)

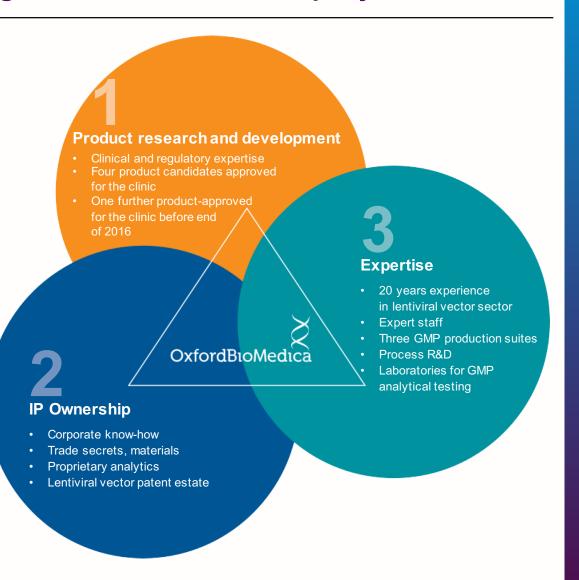




# Oxford BioMedica, <u>the</u> integrated LentiVector® Company

Our USP is based on a unique combination of:

- intellectual property including patents and integrated know-how,
- expert staff
- bioprocessing and laboratory facilities
- product development experience
- clinical & regulatory expertise



# Oxford BioMedica, <u>the</u> LentiVector® Company - at a glance

- 20 years' experience
  - Formed out of Oxford University in 1996 specialising in lentiviral products
  - 1<sup>st</sup> to administer a lentiviral vector *in vivo* (both the brain and the eye)
  - Over 60 patients treated in four Phase I/II studies, with encouraging indications of efficacy lasting up to four years and no significant safety issues
- Integrated LentiVector® gene delivery platform
  - IP extensive IP comprising both patents and know-how
  - Facilities state-of-the-art bioprocessing and laboratory facilities
  - Employees Over 230 staff, many highly qualified and experienced
  - Quality robust quality processes for lentiviral vector production
- In-house products three priority programmes in Parkinson's Disease, corneal graft rejection and a CAR-T approach to solid tumours
- Partnerships/ licences with Novartis, Sanofi, GSK and Immune Design, and ongoing discussions with several other potential partners
- Revenue growth gross income £18.8m in 2015, with £12.4m from bioprocessing and process development up 72% since FY 2014



#### **Products**

Oxford BioMedica has an interest in many gene and cell therapy projects and our integrated platform technology is instrumental in the following wholly-owned and partnered / royalty-bearing programmes



#### **OXB-102 for Parkinson's Disease**

#### **Overview**

# OXB-101 (ProSavin®)/OXB-102 aims to provide dopamine (DA) replacement to patients with Parkinson's disease

- Uses Lentiviral vector technology to deliver genes for 3 enzymes required for DA synthesis
- Administered locally to the striatum, where DA is normally released
- Converts non-dopaminergic cells to replacement of DA
- Evidence of at least 4 year duration emerging from OXB-101 patient follow-up

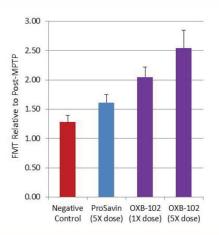
#### **Market size**

# Parkinson's disease affects millions of people worldwide<sup>1</sup>

- Currently 1.7 million adults affected with PD in seven major markets (US, Japan, and EU 5)<sup>1</sup>
- This is expected to rise to 1 million in the US and 880 thousand in the EU by 2022 due to an aging population<sup>1</sup>

#### **Programme Status**

- Phase I/II regulatory approval submission underway
  - Study protocol approved by MHRA (UK authority) and submission Q3 2016 for ANSM (French authority)
- Same Cambridge and Paris sites to be used as for OXB-101 Phase I/II study, with potential for an extra site in UK
- 1st patient likely to be dosed during Q3 2016
- Dose escalation over three cohorts of six patients per cohort and dose confirmation cohort of 12 patients



#### PET analysis (with [18F] fluoro-Lm-tyrosine (FMT)

PET imaging indicates that OXB-102 gives rise to higher AADC activity than ProSavin® in the target putamen PET scans

## **OXB-202 for Corneal Graft Rejection**

#### **Overview**

#### OXB-202 is designed to prevent corneal graft rejection

- Despite one of the most successful tissue transplants, a significant number of grafts are rejected due to corneal vascularisation (NV)
- OXB-202 is a human donor cornea genetically modified with the same lentiviral vector as OXB-201 to secrete 2 anti-angiogenesis proteins, endostatin and angiostatin
- This ex vivo treatment of donor corneas prior to transplant inhibits NV and, consequently, graft rejection

# Approximately 100,000 corneal grafts are performed every year worldwide<sup>1</sup>

- This figure, representing only 1% all patients in need of a transplant, will increase significantly as countries develop their own eye banking infrastructure<sup>2</sup>
- Company estimates peak sales range of £120m to £415m

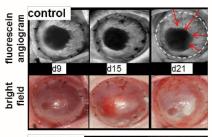
#### **Pre-clinical Data**

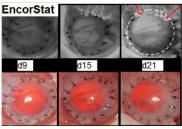
 OXB-202 program supported by extensive OXB-201 data (non-clinical and clinical)

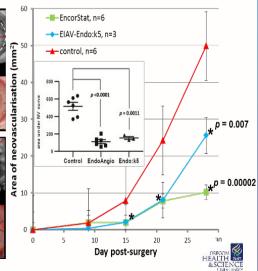
#### **Illustrative Results**

Efficacy in rabbit model of rejection (aggressive)<sup>3</sup>

Reduction in corneal NV, opacity and immune infiltration in a rabbit PK model<sup>5</sup>







#### **Programme Status**

- Submit clinical trial application (CTA) by end of 2016 for Phase I/II clinical study
- Clinical trial may involve up to 40 patients, starting with severe patients and progressing to less severe
- Moorfield Eye Hospital is the UK site, with the potential for a US site

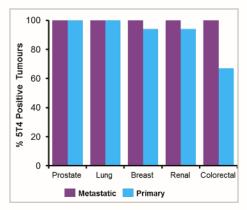
# OXB-302 for Targeting Solid Cancer Tumours (CAR-T 5T4)

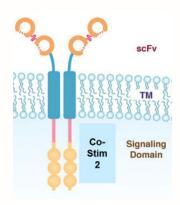
#### **Overview**

- Chimeric Antigen Receptors (CARs) enable the re-direction of a patient's T cells to target cancer cells expressing a specific tumour antigen
- OXB-302 is a combination of our LentiVector® and 5T4
- CAR-T 5T4 targets 5T4, an oncofoetal antigen expressed on the surface of most solid tumours and some haematological malignancies
- The restricted expression profile of 5T4 on normal tissues combined with its broad expression on tumour cells (including cancer stem cells) make 5T4 an attractive target for therapeutic intervention

#### **Illustrative Results**

# Expression of 5T4 on primary and metastatic human tumours:





#### **Pre-clinical Data**

- 2 different OXB-302 Lentiviral based vectors have been produced
- Both OXB-302 vectors transduce human PBMCs
- CAR-5T4 transduced human T cells show good growth kinetics and secrete cytokines in response to "in vitro challenge" with a range of human tumor cell lines
- In vivo testing has demonstrated efficacy in an industry standard tumour challenge model

### **Programme Status**

- End of pre-clinical studies expected by end of 2016
- Following demonstration of pre-clinical proof of concept, clinical planning for OXB-302 will be initiated

# Other proprietary R&D activity

# In-house Product Discovery/Research – providing a flow of new product opportunities

- Several ocular orphan diseases programmes
- CNS orphan disease programme
- Respiratory orphan disease programme

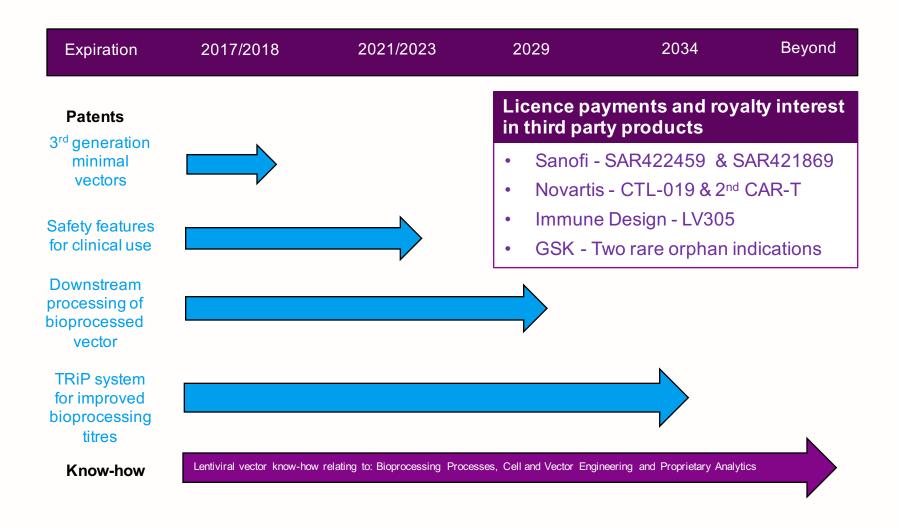
# Technical developments – continuous improvement of the LentiVector® platform

- Cell and vector engineering projects to improve bioprocessing yield – for example:
  - TRAP/TRiP system development
  - Packaging & producer cell lines
- Analytical methods improvements to improve efficiency and effectiveness of testing

Innovation and optimisation to build long-term value



# LentiVector® Platform IP & Key Intellectual Property





## Oxford BioMedica Facilities in the UK









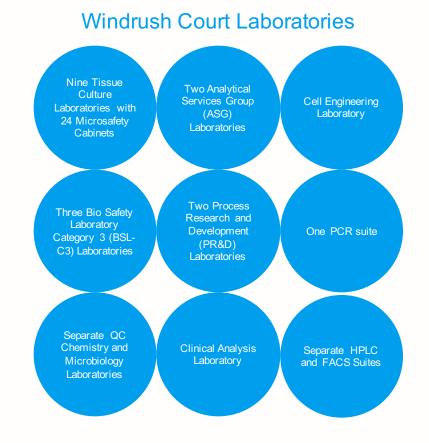
Facilities less than 1 hour from London Heathrow Airport

# Specialist Bioprocessing Facilities (all located in Oxford, UK)

Two separate bioprocessing sites (total clean rooms 1,200m²/12,919ft²)

Laboratories (2,136m<sup>2</sup>/22,992ft<sup>2</sup>)

#### Harrow House **Yarnton** Two independent **GMP2** facility One independent GMP clean room designed for up to GMP clean room suites (GMP1 and two 200L single suite (GMP4) of GMP2) totalling use bioreactors 560m2/6.030ft2 640m2/6,889ft2 Potential for use with 200L single Potential for further expansion use bioreactors





#### 2016/2017 Potential Newsflow

- In-house priority products
  - OXB-102 Phase I/II first patient dosed
  - OXB-202 Phase I/II study CTA filing in H2
  - OXB-302 pre-clinical study results
- Partners' products
  - Novartis CTL-019 study results
  - Novartis CTL-019 BLA submission
- LentiVector® delivery platform
  - Successful development of 200L bioreactor serum-free suspension process to produce lentiviral vectors
  - Further contracts with new partners giving us long-term economic interest in partners' product candidates

# Vision of Oxford BioMedica, <u>the</u> LentiVector® Company – by end 2018

## In-house

#### **OXB-102**

Phase I/II first three cohort data

#### **OXB-202**

Phase I/II first two cohort data

#### **OXB-302**

In Phase I/II clinical study

New product candidates emerging from research/discovery using the LentiVector® platform

# **Partnerships**

#### **Novartis**

- CTL-019 launched
- Oxford BioMedica supplying commercial material
- Royalties from CTL-019
- Second CAR-T product into clinical development
- Further CAR-T programmes assumed

#### Sanofi

 SAR422459 in pivotal trial (Phase IIb/Phase III)

#### Immune Design

• LV305 progressing well in clinical development

Multiple further partnerships giving Oxford BioMedica economic interests in a range of gene and cell therapy products

# **Bioprocessing**

Facilities operating at, or very near capacity

# Summary: a world leading gene and cell therapy company

• Gene and cell therapy is set to grow into a multi-billion US\$ sector over the next 5-10 years

• Lentiviral vectors have advantages over other vector types

OXB's highly sought-after LentiVector® gene delivery platform for both in vivo and ex vivo lentiviral vector products

OXB's product interests include in-house focused clinical and preclinical pipeline and an economic interest in partners' products

OxfordBioMedica

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