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### Strategy: Leveraging Our LentiVector® Delivery Platform

#### Partners' Programmes

Multiple income streams

- Process development fees
- Process development incentives
- Bioprocessing revenues
- Royalties

Process development and bioprocessing

R&D Investment Technical Developments OXB products via spin out or out-licence

- Development milestones
- Royalties
- Bioprocessing revenues

Spin out or out-license

R&D Investment Early Stage/ preclinical

LentiVector® Platform

IP – patents and know-how Facilities
Expertise
Quality systems

# Operational highlights (1 of 2)

- Strong progress from LentiVector® delivery platform and cell therapy partnerships
  - Novartis collaboration progressing well with CTL019 close to launch
  - Strategic alliance with Orchard Therapeutics to develop and supply lentiviral vectors for ex vivo treatments
  - Immune Design collaboration extended, including licence to use lentiviral vector based products for *in vivo* treatments for cancer
  - New R&D collaboration with Green Cross LabCell focused on gene modified natural killer (NK) cell-based therapies
  - 200 litre bioreactor production process established with potential to increase yield and reduce cost per dose
  - Transgene Repression in Vector Production (TRiP) system developed to enhance production titres of a broad range of gene therapy vectors









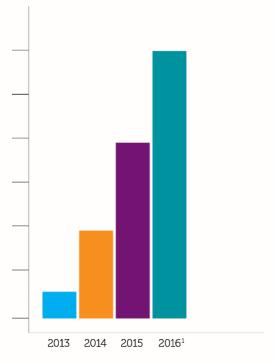
# Operational highlights (2 of 2)

# State-of-the-art bioprocessing and laboratory facilities

- Major capacity expansion completed
- MHRA approval granted for GMP manufacture
- Vector bioprocessing volume increased by 54% compared to 2015

#### Progress with proprietary product development

- Ground breaking long-term results seen from follow-up studies of patients treated with OXB-101 (Parkinson's disease) and OXB-201 (for wet AMD)
- OXB-102 (for Parkinson's disease) and OXB-202 (for corneal graft rejection) ready to start Phase I/II studies following out-licensing/spin-out
- OXB-302 (for solid tumours) pre-clinical proof-ofconcept achieved and ready for further development following out-licensing/spin-out
- SR422459 (licensed to Sanofi for Stargardt disease) in Phase II development



#### **Bioprocessing volumes**

<sup>1</sup> 2016 excludes next generation bioreactor output



### Oxford BioMedica is a leader in lentiviral vector technology

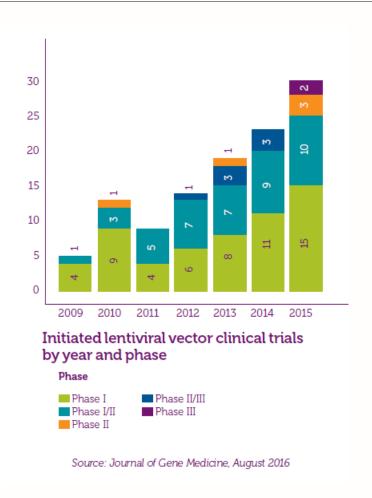
- Gene and cell therapy field set to grow into \$ multi-billion sector over next 5-10 years.
   Several products, particularly ex vivo, likely to launch in next few years
- Lentiviral vectors are preferred choice for ex vivo therapies because they integrate into DNA of target cells with genetic payload replicating when cells divide
- Increasing number of lentivirus clinical studies initiated during 2015 (30% year-on-year)
- Oxford BioMedica has unique combination of patents, know how, expertise and facilities in lentiviral vectors – the LentiVector® platform – leading to partnerships and collaborations



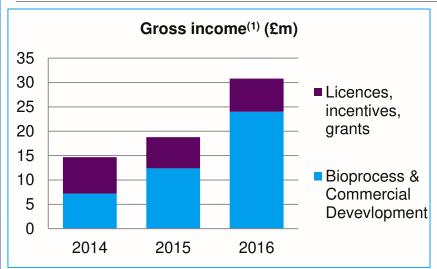


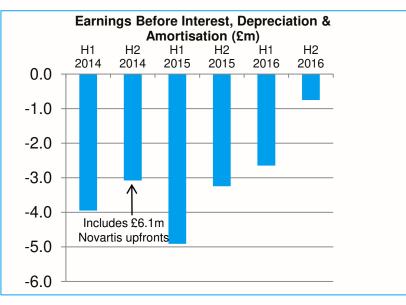


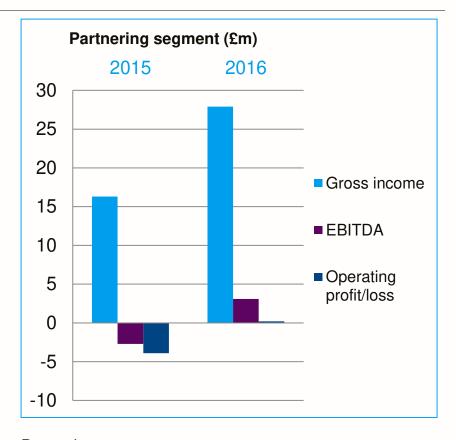
 We expect more such partnerships in the future



# Financial impact of growing Partnership activities





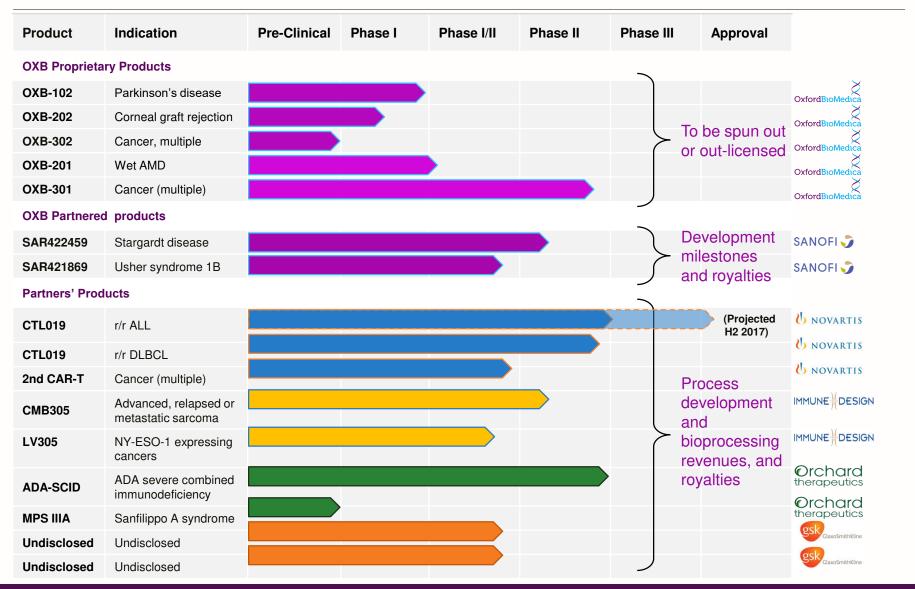


#### Partnering segment

- Gross income received from partnership arrangements
- Now generating cash (2016 EBITDA £3.1m)
- Infrastructure in place to support further growth

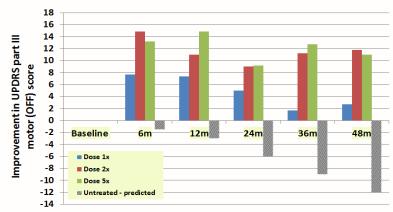


#### **Products Pipeline - Proprietary and Partnered**



# **In-House Programmes**

- OXB-102 (Parkinson's disease)
  - OXB-101 showed encouraging efficacy and long-term duration of benefit
  - OXB-102 in late-stage preparations for Phase I/II clinical study

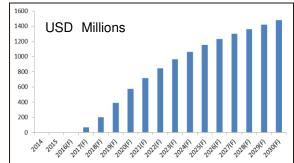


- OXB-202 (prevention of corneal graft rejection)
  - OXB-202 in late-stage preparations for Phase I/II clinical study
  - Tech transfer to clinical site to start once funding secured
- OXB-302 (CAR-T 5T4)
  - Pre-clinical studies completed, demonstrating proof-of-concept
  - Highlights include:
    - CAR-T 5T4 cells can kill tumour cells derived from colorectal cancer and mesothelioma in a "test tube" (in vitro)
    - T cells taken from patients with ovarian cancer can be re-programmed with the 5T4 CAR construct and respond (in vitro) to their own tumour cells, resulting in tumour cell death
    - In industry standard animal model (in vivo) 5T4 CAR-T cells can treat established ovarian cancer
- Out-licensing and spin-out opportunities being explored for priority products

#### Partners' programmes

#### Novartis CTL019

- BLA for r/r ALL accepted by FDA (March 2017), granted priority review
- Novartis plan to file in EU in late 2017
- DLBCL granted FDA Breakthrough Therapy designation
- Submissions for r/r DLBCL in US and EU planned in Q4 2017
- Analyst CTL019 consensus forecasts blockbuster product



#### Novartis 2<sup>nd</sup> CAR-T programme underway

#### Orchard Therapeutics

Development and supply of lentiviral vectors for ADA-SCID underway

#### Immune Design

- LV305 and CMB305 (combination of LV305 and G305 prime boost agent) in Phase I/II studies in cancers expressing NY-ESO-1 antigen
- LV305 activates the immune system against a tumour by generating cytotoxic T cells (CTLs) against specific tumour-associated antigens

#### Green Cross LabCell

• Research collaboration focusing on identifying and developing gene modified natural killer (NK) cell-based therapeutics for treatment of life-threatening diseases such as cancer



### **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
- Gene-modified NK cell therapeutics with Green Cross LabCell for cancer

# Technical developments – continuous improvement of the LentiVector® platform

- Cell and vector engineering projects to improve bioprocessing yield – for example:
  - TRiP system development



- Packaging & producer cell lines
- Analytical methods improvements to improve efficiency and effectiveness of testing
- Scale-up bioprocessing
  - Serum free
  - Suspension
  - 200 L bioreactor

Innovation and optimisation to build long-term value – a key competitive advantage to durably maintain leadership in the field



### Potential catalysts over next 12 months

- Novartis progress
  - Data from adult r/r DLBCL study (expected Q2 2017)
  - Confirmation of OXB commercial supply agreement for CTL019 vector
  - FDA approval of CTL019 for r/r ALL and product launch
  - Submission of DLBCL for approval
- LentiVector® delivery platform
  - Approval to supply lentiviral vector for commercial use
  - Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates
  - Established 200L bioreactor serum-free suspension platform to produce lentiviral vectors at significantly lower cost per dose
- In-house products
  - Spin out / out-license of in-house product candidates

## **Summary: A Leading Gene and Cell Therapy Company**



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



Lentiviral vectors have unique advantages for cell and gene therapy





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



 OXB has world-class bioprocessing facilities and collaboration trackrecord in the field



OXB's product interests include proprietary pipeline assets to be spun out or out-licensed and an economic interest in partners' products

# **Contact Us**

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