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# 2016 Operational Highlights (1/2)

- LentiVector® delivery platform
  - Novartis collaboration progressing well with blockbuster potential product CTL019 and second undisclosed CAR-T programme
  - Strategic alliance established with Orchard Therapeutics to develop and supply lentiviral vectors for ex vivo treatments
  - Immune Design collaboration expanded, 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 at commercial scale with potential to substantially increase yield and reduce cost of a patient dose
  - Transgene Repression In Vector Production (TRiP) system developed to enhance the production titres of a broad range of gene therapy vectors

# 2016 Operational Highlights (2/2)

- Bioprocessing and laboratory facilities
  - Major capacity expansion completed
  - MHRA approval granted for GMP vector manufacture
  - Vector production volume increased by 54% compared with 2015 (1st generation process)
- Progress with proprietary product development
  - Ground-breaking long-term results seen from follow-up studies of patients treated with OXB-101 (for 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 cancer tumours) pre-clinical proof-of-concept achieved and ready for further development following out-licensing / spin out
  - SAR422459 (licensed to Sanofi for Stargardt disease) in Phase II development

# 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

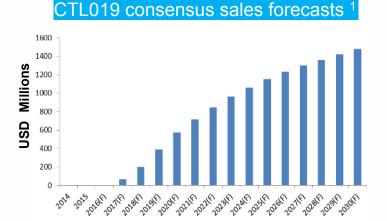
R&D Investment Early Stage/ preclinical

LentiVector® Platform

IP – patents and know-how Facilities
Expertise
Quality systems

## **Delivery of support to Novartis CTL019 programme**

- Delivered batches of LentiVector® for clinical studies
- Input into Biological Licence Application
- Ongoing development of next-generation manufacturing processes



- Novartis R&D update in January 2017 included CTL019 in its list of late stage potential blockbuster products
- Analysts forecast<sup>1,2</sup> at least \$1 billion worldwide peak sales for CTL019
- ELIANA Phase II clinical trial in r/r ALL in paediatric and young adults presented at ASH, Dec 2016
  - Met primary endpoint with strong overall response rate (CR/Cri 82%)
  - Acceptable safety profile with no deaths due to CRS, neurologic toxicities and no cases of cerebral oedema reported
- Novartis plan to file CTL019 for r/r B Cell ALL with the FDA "early 2017" and in the EU "late 2017"
- Pivotal JULIET Phase II trial data for diffuse large B-cell lymphoma (DLBCL) expected in Q2 2017
- DLBCL submissions in US and EU planned in Q4 2017

## Many companies conducting clinical trials with lentiviral vectors

Examples of companies working in clinical development





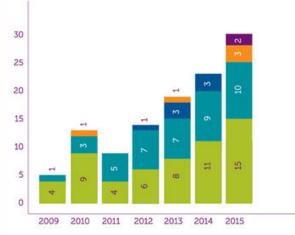














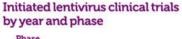














Source: Journal of Gene Medicine, August 2016



bluebirdbio





Examples of companies working in pre-clinical development

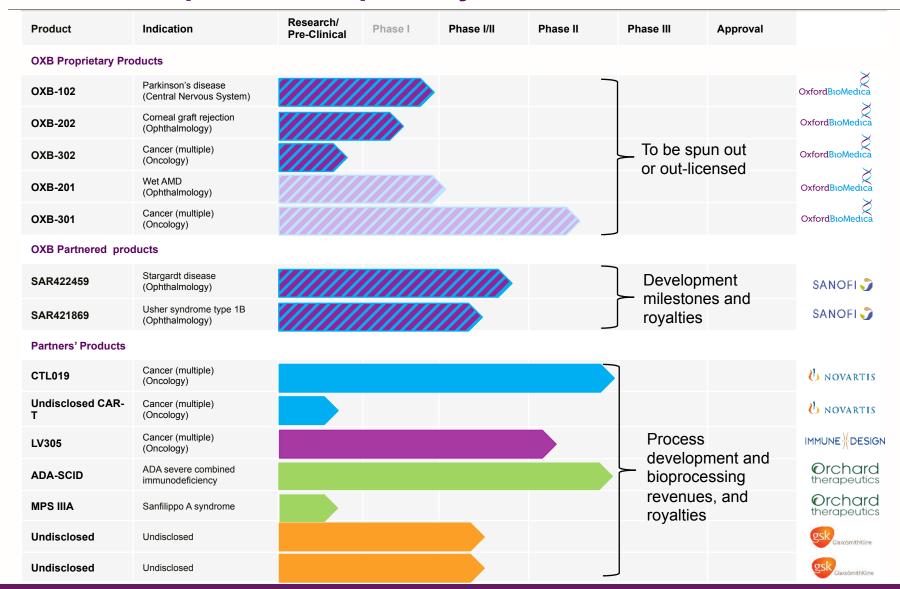








# **Products Pipeline - Proprietary and Partnered**

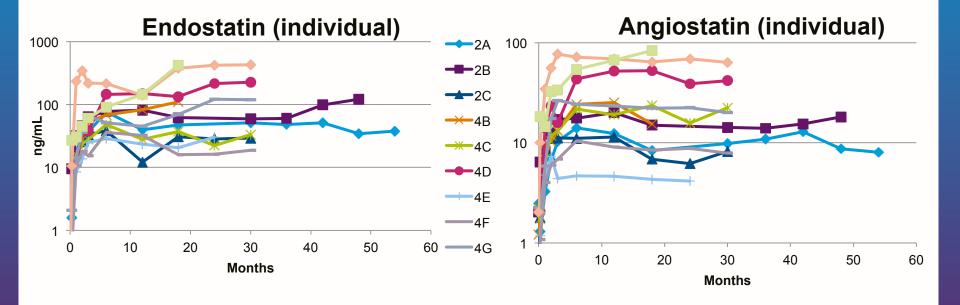


## **Delivery of In-House Programmes**

- 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
  - Clinical study material manufactured
  - SPV progress Ongoing discussions with VCs and Big Pharma
- OXB-202 (prevention of corneal graft rejection)
  - Clinical study material manufactured
  - 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

## LentiVector® Platform Evidence of Long-term Duration

- Long-term four year follow up data for OXB-201<sup>1</sup>
  - Dose responsive expression of proteins
  - Long term follow up continues



Persistent expression out to >4 years so far (ongoing)

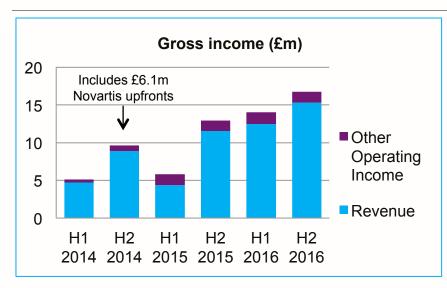


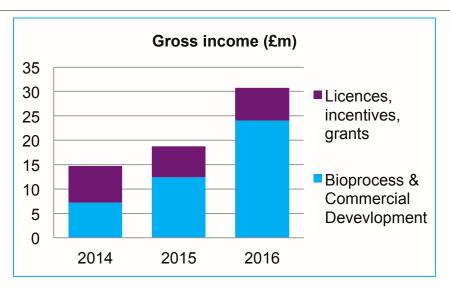
## **2016 Financial Highlights**

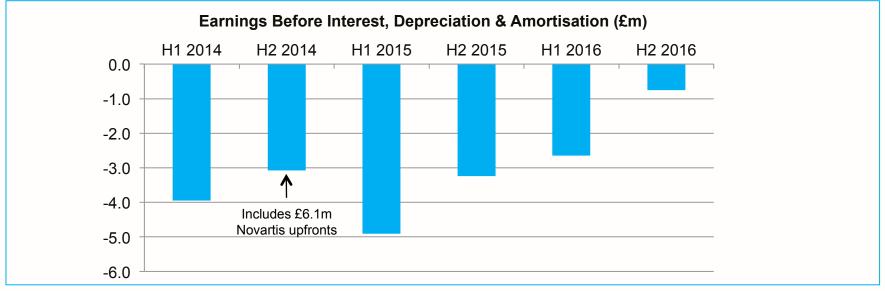
- 64% increase in gross income<sup>1</sup> to £30.8m (2015: £18.8m)
- 4% increase in operating expenses<sup>2</sup> to £26.1m (2015: £25.1m)
- EBITDA<sup>3</sup> loss reduced to £7.1m (2015: £12.1m)
  - H2 2016 EBITDA loss only £1.9m
- Operating loss £11.3m (2015: £14.1m)
- Net cash used in operating activities reduced to £5.1m (2015: £13.1m)
- Capital expenditure £6.5m (2015: £16.7m)
  - H2 2016 capex only £0.5m
- Cash of £15.3m (31 Dec 2015: £9.4m) including £8.1m ring-fenced under Oberland agreement
- 2016 fundraising net proceeds of £17.5m



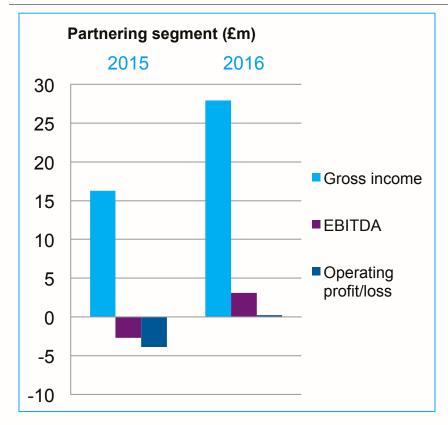
## Gross income<sup>1</sup> and EBIDA<sup>2</sup>





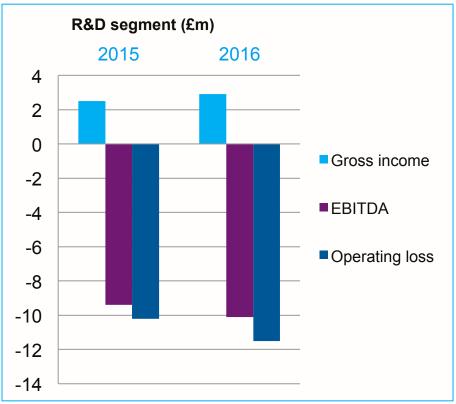


## Segmental analysis





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



#### R&D segment

- Covers costs of investing in LentiVector® technology and product development (discovery, pre-clinical and preparation for clinical studies)
- Costs include employees and directly related internal costs, external project expenditure, and allocation of Group overheads

## Financial outlook

- Expect gross income to continue to grow strongly
  - 3 GMP suites and laboratories fully operational
  - 3 revenue generating partners with potential for new partners in 2017
- Infrastructure in place
  - Only modest cost growth needed to support additional batch manufacture and process development
  - Spend on product candidates ready for clinical studies will be low
  - Will continue to spend on pre-clinical product ideas and LentiVector® technology
- Capital expenditure in 2017 expected to be relatively modest



## **Potential 2017 Catalysts**

## Novartis progress

- Confirmation of Novartis CTL019 BLA submission
- Data from DLBCL study (expected Q2 2017)
- Confirmation of OXB commercial supply agreement for CTL019 vector
- FDA approval of CTL019 and product launch

## LentiVector® delivery platform

- Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates
- Successful development of 200L bioreactor serum-free suspension process to produce lentiviral vectors at significantly lower cost per dose

## In-house products

- Successful spin out / out-license of in-house product candidates, delivering potential up-fronts, bioprocessing revenues, development milestones and royalties
- First patients dosed with one or more clinical products in Phase I/ II clinical studies with appropriate partner

## Vision of Oxford BioMedica – By End of 2018

#### Core LentiVector® R&D

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

Lead gene-modified NK cell therapeutic candidate emerging from the GCLC research collaboration

Technical developments – continuous improvement of the LentiVector® platform

Feeds further partnership / monetisation opportunities

### **Partnerships and Licences**

#### **Novartis**

- CTL019 launched
- Oxford BioMedica supplying commercial material
- Royalties from CTL019
- Second CAR-T product into clinical development
- Further CAR-T programmes

#### Sanofi

SAR422459 to be in a pivotal trial

#### **Immune Design**

LV305 progressing well in clinical development

#### **Orchard Therapeutics**

- ADA-SCID pivotal trial close to completion
- MPS IIIA in clinical development

#### **OXB Products with Partners**

Progressing well through Phase I/II studies

#### **Multiple further partnerships**

Which give Oxford BioMedica economic interests in a range of gene and cell therapy products and process development revenue / income opportunities

## **Bioprocessing**

Facilities operating at, or very, near capacity



# **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 advantages over other vector types





 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 own clinical and preclinical pipeline either spun out or out-licensed and an economic interest in partners' products

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