A LentiVector® Company, and a leader in gene and cell therapy

Jefferies Healthcare Conference
New York, June 2016

Tim Watts, Chief Financial Officer
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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 a leading lentiviral vector delivery platform (LentiVector®)

1. Gene and cell therapy is predicted to grow into a multi-billion US$ sector over the next 5-10 years
   - 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.
   - Several in vivo clinical studies, particularly in ophthalmology and CNS

2. 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

3. OXB’s sought-after LentiVector® 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

4. 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 programme 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)

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1 Clive Glover, GE Healthcare “Sales of cell and gene therapy will reach $10 billion by 2021”, October 2015.
Oxford BioMedica, a 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

1. Product research and development
   - Clinical and regulatory expertise
   - Four product candidates approved for the clinic
   - One further product-approved for the clinic before end of 2016

2. IP Ownership
   - Corporate know-how
   - Trade secrets, materials
   - Proprietary analytics
   - Lentiviral vector patent estate

3. Expertise
   - 20 years experience in lentiviral vector sector
   - Expert staff
   - Three GMP production suites
   - Process R&D
   - Laboratories for GMP analytical testing
Oxford BioMedica, a LentiVector® Company - at a glance

- 20 years’ experience
  - Formed out of Oxford University in 1996 – specialising in lentiviral products
  - 1st 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, Immune Design and Green Cross LabCell, 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

- Listed on LSE (OXB.L); current market capitalisation £149m ($218M)

1 As of 02 June 2016
## 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

<table>
<thead>
<tr>
<th>Product</th>
<th>Indication</th>
<th>Research/Pre-Clinical</th>
<th>Phase I</th>
<th>Phase I/II</th>
<th>Phase II</th>
<th>Phase III</th>
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<td><strong>Priority programmes</strong></td>
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<tr>
<td>OXB-102</td>
<td>Parkinson’s disease (Central Nervous System)</td>
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<td>OXB-202</td>
<td>Corneal graft rejection (Ophthalmology)</td>
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<td><strong>Other candidates</strong></td>
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<tr>
<td>OXB-201</td>
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<td><strong>Partnered /IP enabled &amp; royalty bearing products</strong></td>
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<tr>
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<tr>
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### 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

#### Programme Status

- Phase I/II regulatory approval submission underway
  - Study protocol approved by MHRA (UK authority) and submission Q2 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
- Expect preliminary readout from first cohort towards the end of 2017

#### Market size

**Parkinson’s disease affects millions of people worldwide**

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

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1 PharmaPoint Parkinson’s Disease Global Forecast & Market Analysis to 2022, Global Data June 2015
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

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

Pre-clinical Data

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

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

1 Human organ and tissue transplantation. Report by the Secretariat. Executive Board EB112/5. 112th session, Provisional agenda item 4.3. World Health Organisation. May 2003
4 Scripps et al. European Society for Gene and Cell Therapy (ESGCT) Abstract# P283, October 2013
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

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

Illustrative Results

Expression of 5T4 on primary and metastatic human tumours:

- Targeting 5T4 expression on solid tumours with OXB-302 (CAR-T 5T4) leads to tumour killing in in vivo models

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
- 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

Innovation and optimisation to build long-term value
Intellectual Property
## LentiVector® Platform IP & Key Intellectual Property

<table>
<thead>
<tr>
<th>Expiration</th>
<th>2017/2018</th>
<th>2021/2023</th>
<th>2029</th>
<th>2034</th>
<th>Beyond</th>
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<td><strong>Patents</strong></td>
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<tr>
<td>3rd generation minimal vectors</td>
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<tr>
<td>Safety features for clinical use</td>
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<tr>
<td>Downstream processing of bioprocessed vector</td>
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<td>TRiP system for improved bioprocessing titres</td>
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<tr>
<td><strong>Know-how</strong></td>
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<tr>
<td>Lentiviral vector know-how relating to: Bioprocessing Processes, Cell and Vector Engineering and Proprietary Analytics</td>
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**Licence payments and royalty interest in third party products**

- Sanofi - SAR422459 & SAR421869
- Novartis - CTL-019 & 2nd CAR-T
- Immune Design - LV305
- GSK - Two rare orphan indications

**Expiration**

- 2017/2018
- 2021/2023
- 2029
- 2034
- Beyond
Oxford BioMedica Facilities in the UK

Harrow House & Chancery Gate
19, 375 sq.ft (1,800 sq.m)
- cGMP production facility
- GMP QC microbiology laboratories
- Raw material testing
- GMP cold chain warehouse & office space

Windrush Court (opening)
- Corporate HQ & Laboratories
  71,955 sq. ft (6,684 sq. m)
- GMP Warehouse Hub
  2,691 sq. ft (250 sq. m).

Yarnton
18,300 sq. ft (1,700 sq. m)
- cGMP production facility

Facilities less than 1 hour from London Heathrow Airport

Source: https://resources.oncourse.iu.edu/access/content/user/leema/profilepage/oxford.html
State-of-the-art Bioprocessing Facilities (all located in Oxford, UK)

Two separate bioprocessing sites (total clean rooms 1,200m²/12,917ft²)

Harrow House
- Two independent GMP clean room suites (GMP1 and GMP2) totalling 640m²/6,889ft²
- Potential for further expansion

Yarnton
- GMP2 facility designed for up to two 200L single use bioreactors
- Potential for use with 200L single use bioreactors

Windrush Court Laboratories
- Nine Tissue Culture Laboratories with 24 Microsafety Cabinets
- Three Bio Safety Laboratory Category 3 (BSL-C3) Laboratories
- Two Process Research and Development (PR&D) Laboratories
- One PCR suite
- Separate QC Chemistry and Microbiology Laboratories
- Clinical Analysis Laboratory
- Separate HPLC and FACS Suites

Laboratories (2,136m²/22,992ft²)
Future Vision and Summary
<table>
<thead>
<tr>
<th>Cohort</th>
<th>Dose Administration</th>
<th>3 months (UPDRS)</th>
<th>6 months (UPDRS)</th>
<th>1 year (UPDRS)</th>
<th>2 years (UPDRS)</th>
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<tbody>
<tr>
<td>1, n=3</td>
<td>1x Original Mean</td>
<td>27%</td>
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<td>Max. up to 30%</td>
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<tr>
<td></td>
<td>Mean 30%</td>
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<td>Max. up to 50%</td>
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<td>2, n=3</td>
<td>2x Original Mean</td>
<td>28%</td>
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<td>Max. up to 53%</td>
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<tr>
<td></td>
<td>Mean 34%</td>
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<td>Max. up to 53%</td>
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<tr>
<td>3, n=3</td>
<td>2x Enhanced Mean</td>
<td>26%</td>
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</table>

**Near-term Catalysts**

- **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, a 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*

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

**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 leading gene and cell therapy company

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

2. Lentiviral vectors have advantages over other vector types

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

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