OxfordBioMedica

The LentiVector® Platform Company

The leader in gene and cell therapy

November 2017

Forward-looking statements

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Corporate overview

>20 years as the leader in lentiviral vectors

- ✓ **1st** to administer *in vivo* (both brain and eye)
- ✓ **1st** approved advanced therapy in the US using LentiVector Enabled technology
- ✓ **>200** patients treated by Oxford BioMedica or its partners
- ✓ **Four** Phase I/II studies completed with encouraging safety and efficacy
- ✓ **Four** in-house products, available for spin out or out-licensing

Integrated LentiVector Enabled gene delivery platform

- ✓ **IP** – extensive IP comprising both patents and know-how
- ✓ **Facilities** – state-of-the-art bioprocessing and laboratory facilities
- ✓ **Employees** – over 280 full time employees, many highly qualified and experienced
- ✓ **Quality** – robust quality processes for lentiviral vector production

Partnered with

NOVARTIS

Green Cross LabCell

IMMUNE DESIGN

Orchard therapeutics

Products & patents licensed to

SANOFI

gsk GlaxoSmithKline

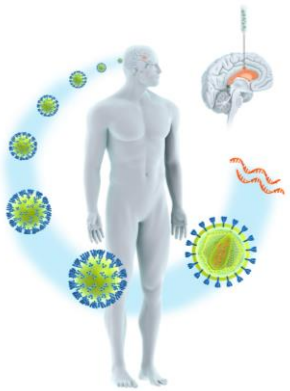
The gene and cell therapy revolution

The use of DNA to treat diseases by delivery therapeutic DNA into patients' cells

Offers potential for single treatment giving long-term or even permanent efficacy

In vivo development – e.g. OXB-102

- Lentiviral vector engineered to carry three genes encoding key enzymes for synthesis of dopamine
- The lentiviral vector is directly injected into the appropriate part of the brain, called the striatum
- The lentiviral vector genetically modifies the cells to produce dopamine



Ex vivo development – e.g. Novartis's CTL019

- OXB produces GMP lentiviral vector encoding CAR targeting CD19
- T-cells isolated from patient's blood and transduced with OXB vector
- Modified T-cells are infused back into the patient
- Once re-infused, the T-cells multiply, "hunt" cancer cells and destroy them

Lentiviral vector advantages for cell and gene therapy

- Large therapeutic payloads (up to 9 kb)
- Permanent modification of dividing cells
- No pre-existing immunity

Leading lentiviral vector delivery platform (LentiVector Enabled)

Broad range of gene and cell therapy products from multiple companies

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1

Gene and cell therapy expected to grow into a multi-billion US$ sector over the next 5-10 years[1]

- Product launches: Strimvelis® (GSK), Kymriah™ [tisagenlecleucel,CTL019; CD19 CAR-T] (Novartis), Yescarta™ [axicabtagene citoleucel] (Celgene/Kite) with others forthcoming
- 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

2

OXB's sought after LentiVector® gene delivery platform

- 1st approved advanced therapy in the US using LentiVector Enabled gene delivery technology
- 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, technology, employees' expertise, bioprocessing & laboratory facilities

3

World-class bioprocessing capabilities and track-record

- Novartis's CTL019[2] process development, bioprocessing and commercial supply
- Agreements with Immune Design, Orchard Therapeutics and Green Cross LabCell, others in discussion
- State-of-the-art bioprocessing facilities, expertise and know-how

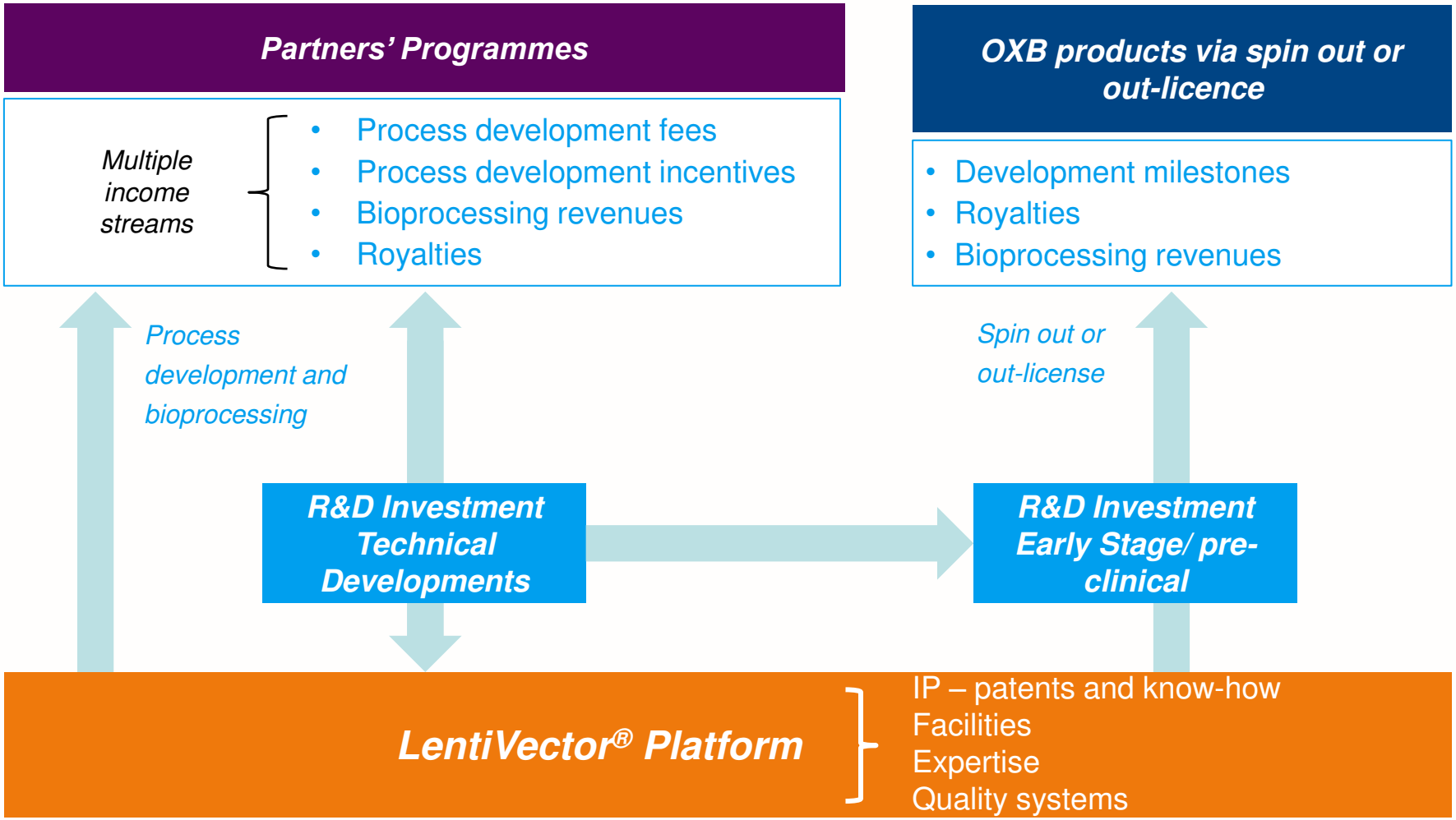
4

OXB's product portfolio & Royalty Streams

- OXB-102 & OXB-202 to be spun out or out-licensed, continue to be prepared for Phase I/II clinical studies in next 12 months and OXB-302 is a CAR-T pre-clinical programme targeting solid tumours
- Milestones & Royalties on partners' products:

[1] Clive Glover, GE Healthcare "Sales of cell and gene therapy will reach $10 billion by 2021", October 2015.

Strategy: leveraging our LentiVector® delivery platform



Product pipeline

LentiVector Enabled

Product	Indication	Pre-Clinical	Phase I	Phase I/II	Phase II	Phase III	Approval
OXB Proprietary Products							
OXB-102	Parkinson's disease						
OXB-202	Corneal graft rejection						
OXB-302	Cancer, multiple						
OXB-201	Wet AMD						
OXB Partnered products							
SAR422459	Stargardt disease						
SAR421869	Usher syndrome 1B						
Partners' Products							
CTL019[1,2]	r/r ALL						
CTL019[1]	r/r DLBCL						
2nd CAR-T	Cancer (multiple)						
CMB305	Advanced, relapsed or metastatic sarcoma						
LV305	NY-ESO-1 expressing cancers						
ADA-SCID	ADA severe combined immunodeficiency						
MPS IIIA	Sanfilippo A syndrome						
Undisclosed	Undisclosed						
Undisclosed	Undisclosed						

To be spun out or out-licensed

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Development milestones and royalties

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Process development and bioprocessing revenues, and royalties[3]

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IMMUNE DESIGN

IMMUNE DESIGN

Orchard therapeutics

Orchard therapeutics

gsk GlaxoSmithKline

gsk GlaxoSmithKline

[1] USAN name is tisagenlecleucel
[2] Approved in the US only
[3] GSK partnership is a option fee and royalty agreement

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Clinical lentiviral vector experience

OXB's lentiviral vector administered to >200 patients (by OXB or its partners) and cumulative patient safety data >400 years

In Vivo

- OXB-101 - 15 patients treated via stereotactic delivery[1]
  - Safe and well tolerated with cohort 1 out to 8 years
- OXB-201 - 21 patients treated via subretinal delivery
  - Safe and well tolerated with cohort 1 out to 5 years
  - Protein expression from transgenes observed at latest time point (4yr)
- SAR422459/SAR421869 – Over 20 patients treated via subretinal delivery
  - Safe and well tolerated with SAR422459 cohort 1 out to 5 years[2]
  - Safe and well tolerated with SAR421869 cohort 1 out to 4 years[3]

Ex Vivo

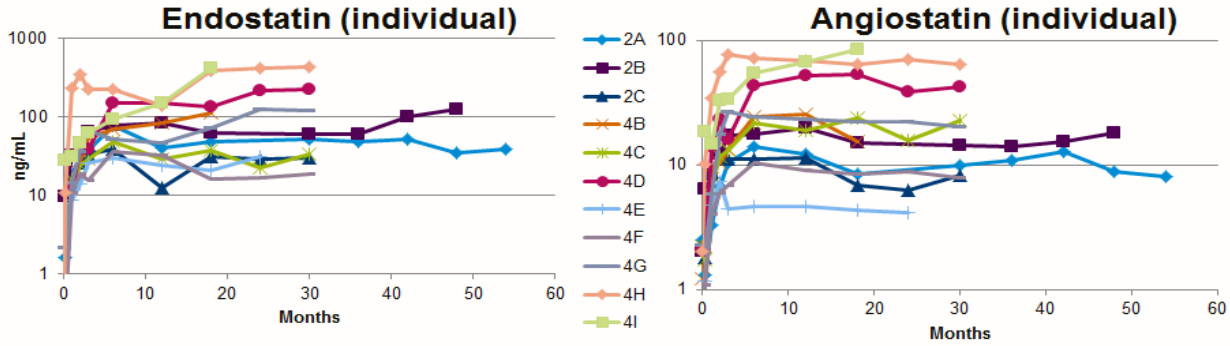
- Tisagenlecleucel (CTL019) – ELIANA and JULIET clinical studies

- Ongoing safety profile is very well tolerated
- No transgene related immune responses observed

[1] Published in *The Lancet* January 2014 (Palfi *et al.*)
[2] Binley et al. Transduction of Photoreceptors With Equine Infectious Anemia Virus Lentiviral Vectors: Safety and Biodistribution of StarGen for Stargardt Disease. IOVS 54 (6): 4061-4071, 2013
[3] Weleber et al. Early findings in a Phase I/IIa clinical programme for Usher syndrome 1B (USH1B; MIM #276900). ARVO Meet Abstr. 2286 (B0191), 2015

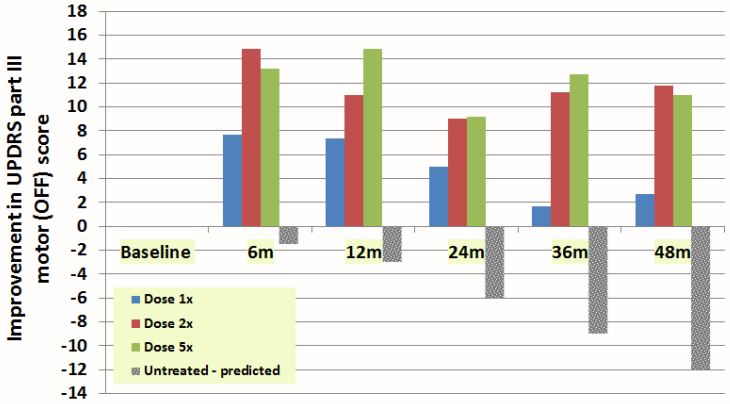
LentiVector® platform evidence of long-term duration

- Long-term four year follow up data for OXB-201[1]
  - Dose responsive expression of proteins
  - Long term follow up continues



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

- OXB-101 efficacy analysis by cohort out to four years[2]



- At 12m, all patients had equal or better UPDRS Part III OFF scores than at baseline
- At 24m 12 out of 14 patients[2], at 36m 10 out of 11 of patients[2], at 48m 8 out of 10 patients had equal or better UPDRS Part III OFF scores than at baseline
  - UPDRS Part III OFF score expected to increase 2-3 pt/year

[1] Campochiaro PA, et al. "Lentiviral vector gene transfer of endostatin/angiostatin for macular degeneration (GEM) study". Hum Gene Ther. 28 (1) 99-111, 2017

[2] Summary of 12 month and three year follow up data of the Phase I/II study with ProSavin® (OXB-101); Source: Palfi et al. Oral presentation AANS Conference, May 2015

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Novartis CAR-T partnership

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October 2014 & July 2017 contracts

- Non-exclusive licence to OXB's IP:
  - $14m upfronts (Oct 2014) and future royalties
- Lentiviral vector bioprocessing:
  - Initial 3 year contract (Oct 2014) to produce CTL019[1] for clinical studies. Now superseded by
  - July 2017 contract for commercial & clinical supply of vector for CTL019[1] and other CAR-T products (potentially in excess of $100m over 3 years)
- Process development collaboration:
  - Multiple performance incentives paid on achievement of targets

Current status and expectations

- FDA ODAC CTL019[1] meeting held 12 July 2017; unanimous vote (10 to 0) for approval
- **CTL019[1] approved 30 August, underpinning bioprocessing revenues and start of royalty flow**
- Novartis have suggested CTL019[1] has blockbuster potential
- CTL019[1] 2nd indication r/r DLBCL granted FDA Breakthrough Therapy designation; sBLA submitted to the FDA 31 October 2017. MAA submission to EMA 06 November 2017 for both paediatric r/r ALL and adult r/r DLBCL indications
- Successful development of 200 litre process with significant productivity improvements
- 2nd CAR-T programme (undisclosed indication) to expand

[1] USAN name is tisagenlecleucel

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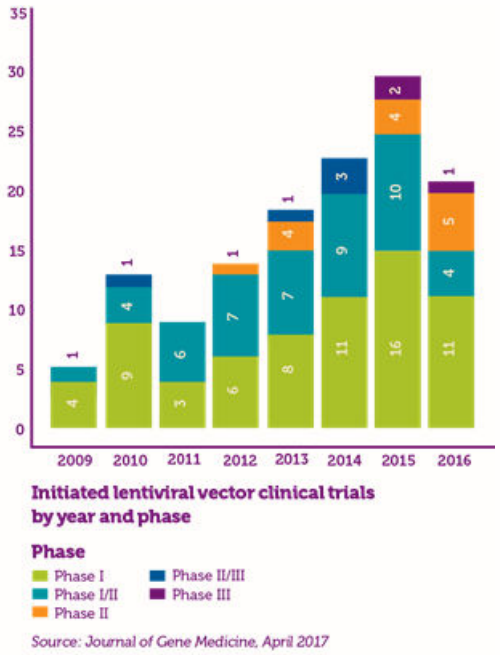
Business development – extensive lentiviral vector clinical/pre-clinical trial activity



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Proprietary R&D activity

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

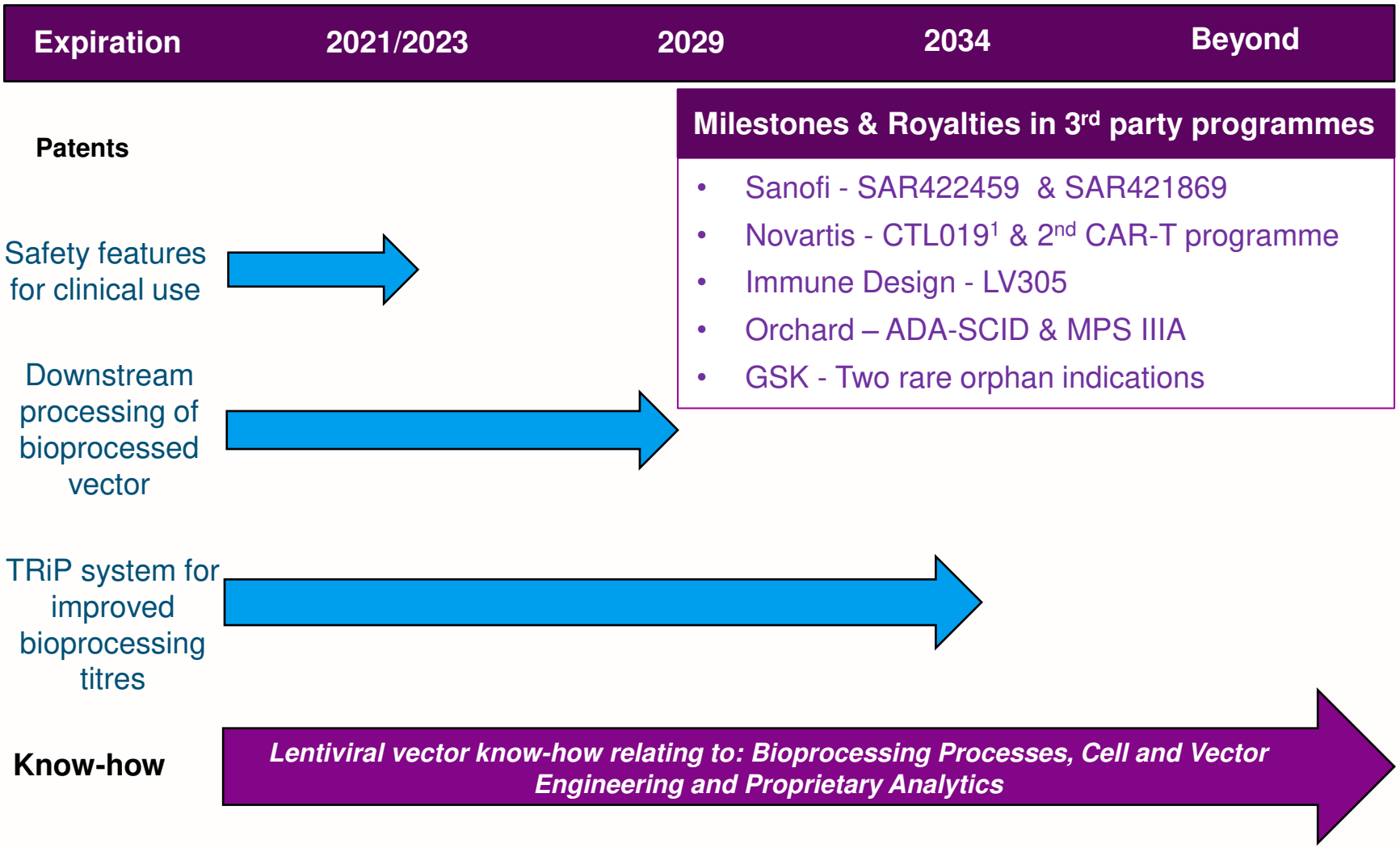
- Several ocular orphan diseases programmes
- CNS orphan disease programme
- Respiratory orphan disease programme
- Gene-modified NK cell therapeutics with Green Cross LabCell for cancer

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

LentiVector® platform IP



[1] USAN name is tisagenlecleucel

Oxford BioMedica facilities in the UK

Facilities less than 1 hour from London Heathrow Airport:

Windrush Court

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


Harrow House & Chancery Gate
19,375 sq.ft (1,800 sq.m)

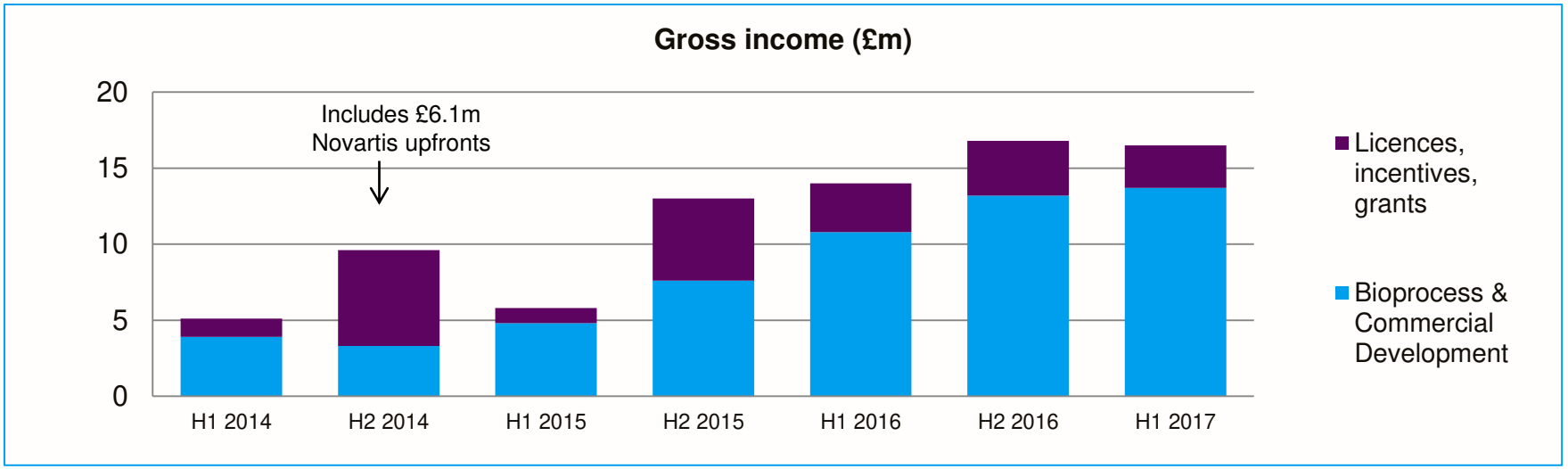
- cGMP production facility
- Two clean room suites
- GMP QC microbiology laboratories
- Raw material testing
- GMP cold chain warehouse & office space

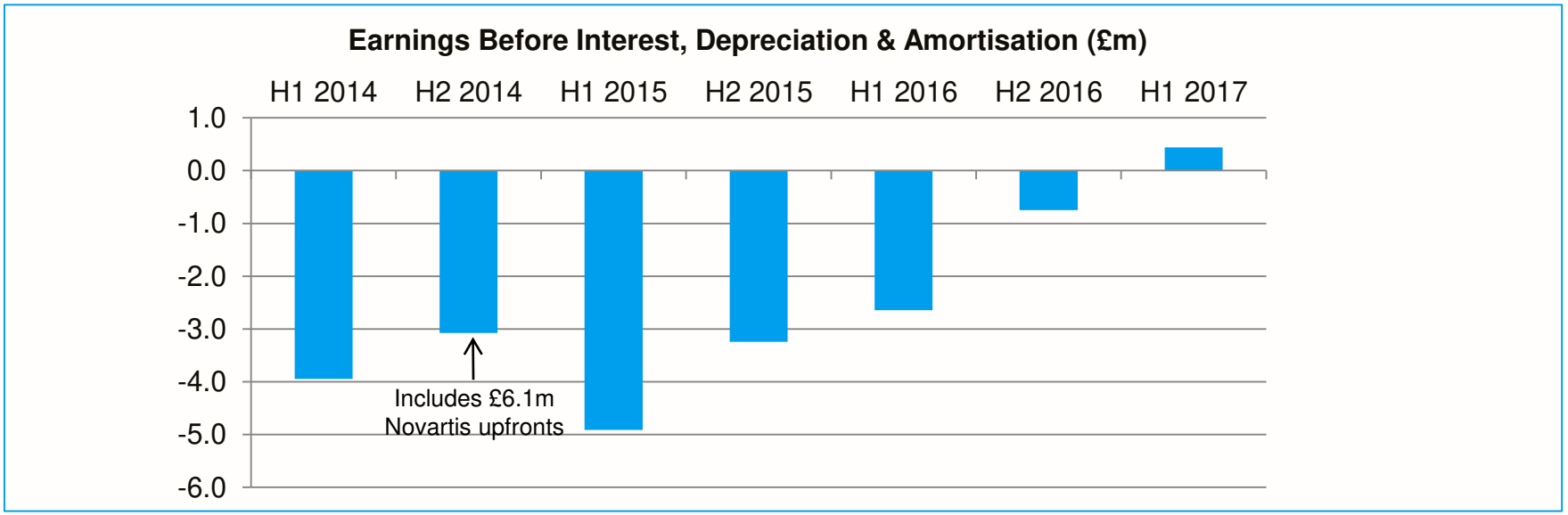

Yarnton
18,300 sq.ft (1,700 sq.m)

- cGMP production facility
- One clean room suite


See “Introduction to Oxford BioMedica” video - http://www.oxfordbiomedica.co.uk

Gross income[1] and EBIDA[2]





[1] Gross income = aggregate of revenue and other operating income
[2] EBIDA = Earnings Before Interest, Depreciation and Amortisation

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Upcoming news flow

- Novartis progress
  - 2nd CAR-T programme to enter clinic
  - Royalties commence from Novartis/CTL019[1]

- LentiVector® delivery platform
  - 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

[1] USAN name is tisagenlecleucel

Vision of Oxford BioMedica – by end of 2018

Core LentiVector® platform 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 product partnership opportunities

Product pipeline

OXB priority products
- Successful spin outs and/or out-licensing
- Products progressing in Phase I/II studies

Novartis
- Oxford BioMedica supplying commercial material
- Royalties from CTL019[1]
- Second CAR-T product into clinical development
- Further CAR-T programmes

Sanofi
- SAR422459 progressing towards 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

Several further partnerships
- Economic interests in a range of gene and cell therapy products

Bioprocessing

Facilities operating at high capacity

[1] USAN name is tisagenlecleucel

Summary: the leading gene and cell therapy company

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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 unique advantages for cell and gene therapy
   - 1st approved advanced therapy in the US using LentiVector Enabled technology

3. 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 track-record in the field

4. OXB's product interests include own clinical and preclinical pipeline either spun out or out-licensed and an economic interest in partners' products

[1] Clive Glover, GE Healthcare "Sales of cell and gene therapy will reach $10 billion by 2021", October 2015.

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