The LentiVector® Company

November 2016

biotechandmoney
INVESTIVAL SHOWCASE

OxfordBioMedica

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Gene and Cell Therapy Company With a Leading Lentiviral Vector Delivery Platform (LentiVector®)

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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]

- Upcoming product launches: Strimvelis (GSK), CD19 CAR-T (various)
- Multiple players in *ex vivo* cell therapy CAR-T, TCR, Stem Cells, NK cells, etc.
- Many *in vivo* 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 preferred choice
- Lentiviral vectors have demonstrated long-term efficacy , supporting the "one-off" treatment hypothesis

3 OXB's proprietary LentiVector® gene delivery platform

- Used for both *in vivo* and *ex vivo* lentiviral vector products
- 20 years' experience of delivering lentiviruses *in vivo*
- Combination of OXB's IP, technology, employees' expertise, bioprocessing & laboratory facilities

4 World-class bioprocessing capabilities and track-record

- Novartis CTL-019 bioprocessing contract
- Signed agreements with Immune Design and Green Cross LabCell, others in discussion
- State-of-the-art bioprocessing facilities, expertise and know-how

5 OXB's product portfolio & Royalty Streams

- OXB-102 & OXB-202 to be spun out or out-licensed, entering 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: Sanofi (SAR422459 and SAR421869); Novartis (CTL019 and an other undisclosed CAR-T programme); Immune Design (LV305) and GSK (two undisclosed rare orphan products)

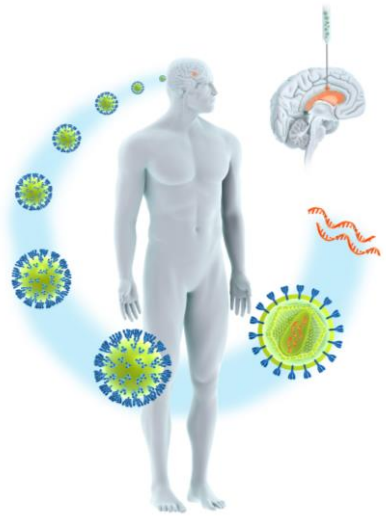
[1] Clive Glover, GE Healthcare "Sales of cell and gene therapy will reach $10 billion by 2021", October 2015.

Oxford BioMedica, the LentiVector® Company – at a glance

- 20 years' experience
  - Formed out of Oxford University in 1996 – specialising in lentiviral products
  - First to administer a lentiviral vector *in vivo* (both the brain and the eye)
  - Over 60 patients treated *in vivo* in four Phase I/II studies, with encouraging indications of efficacy lasting up to four years with 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 250 full time employees, many highly qualified and experienced
  - Quality – robust quality processes for lentiviral vector production
- Five in-house products – available for spin out or out-licensing
- Partnerships with Novartis, Green Cross LabCell and Immune Design, and ongoing discussions with several other potential partners
- Licences to Sanofi and GSK

OXB active in both *in vivo* and *ex vivo* Programs

In vivo development

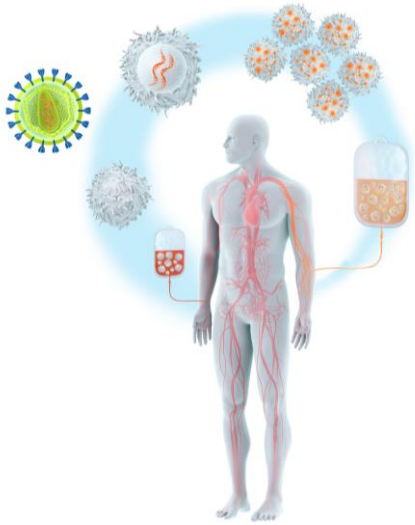


- Direct administration of lentiviral vectors to target organ *in vivo*
- Lentiviral vectors have advantages vs. AAV
  - Larger therapeutic payloads (up to 9 kb)
  - Permanent modification of dividing cells
  - No pre-existing immunity
- OXB's lentiviral vector administered to >60 patients & cumulative patient safety data >150 years

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

Example: OXB-102

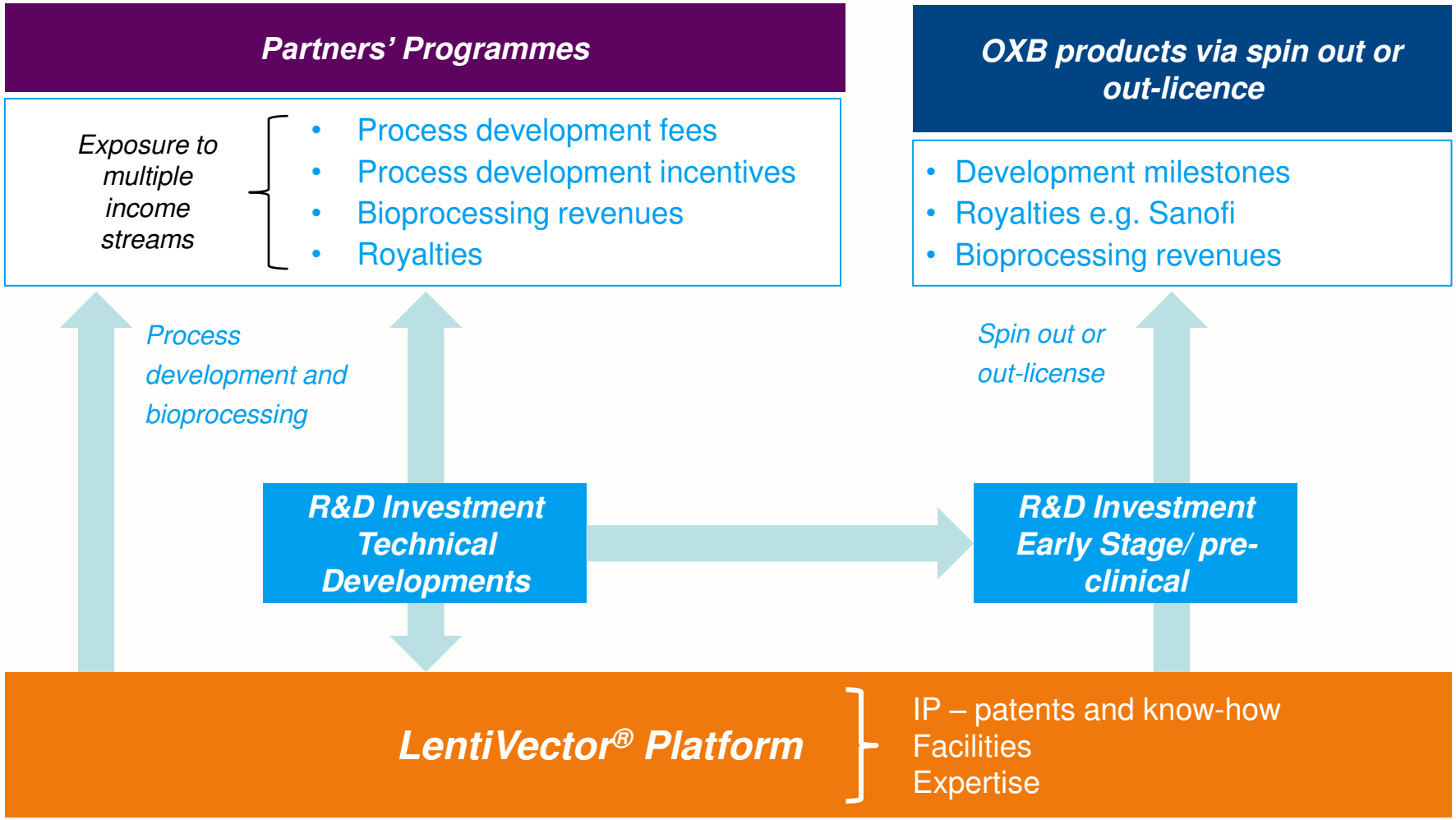
Ex vivo development



- OXB produces GMP lentiviral vector encoding CAR targeting CD19
- White blood cells (T-cells) isolated from patients
- Vector used to transduce expanded T-cells
- The modified T-cells are infused back into the patient
- Once inside the patient, the T-cells multiply, 'hunt' cancer cells and destroy them
- OXB's own CAR-T program targets 5T4 tumour associated antigen

Example: Novartis' CTL019 & OXB-302

Leveraging Our LentiVector® Delivery Platform



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Oxford BioMedica CAR-T partnership

NOVARTIS

Overview

- Non-exclusive licence to OXB's IP:
  - Up fronts (2014) and future royalties
- Lentiviral Vector bioprocessing:
  - Initial three year contract to manufacture CTL019 for clinical studies; extendable
- Process Improvements:
  - Contract to develop next generation vector processing, switch from adherent cell factories (Process "A") to single-use, serum-free, suspension process (Process "B")
  - Milestones paid on achievement of targets

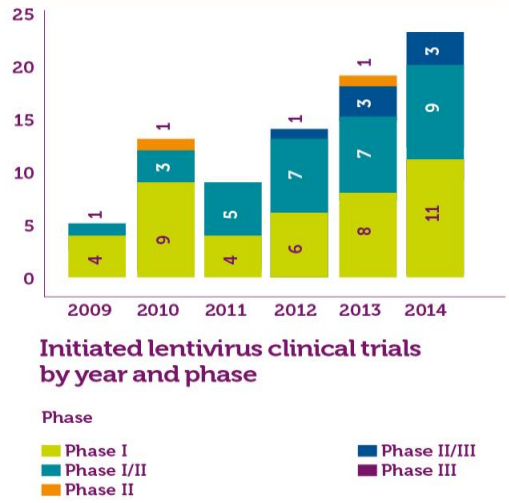
Achievements to date

- Multiple Process A CTL019 batches supplied to Novartis since October 2014 for use in clinical studies – and multiple confirmed purchase orders through to Q2 2017
- Successful development of Process B - 200 litre validation batches underway in H2 2016 – pilot studies suggest significant productivity improvement
- Novartis on course to file CTL019 BLA in early 2017, with approval expected in 2017 due to Breakthrough Therapy designation

Forward Looking

- BLA CMC section based on OXB's Process A, so OXB will be sole manufacturer for commercial launch expected in H2 2017
- Royalty flow expected to start in H2 2017
- Work on second CAR-T programme (undisclosed indication) set to expand

Examples of companies conducting clinical trials with lentiviral vectors



Source: Journal of Gene Medicine, July 2015

Example of Companies working in pre-clinical development with lentiviral based vectors

Products, IP and Facilities

Products Pipeline

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

Product	Indication	Research/ Pre-Clinical	Phase I	Phase I/II	Phase II	Phase III	Approval	
OXB-102	Parkinson's disease (Central Nervous System)							OxfordBioMedica
OXB-202	Corneal graft rejection (Ophthalmology)							OxfordBioMedica
OXB-302	Cancer (multiple) (Oncology)							OxfordBioMedica
OXB-201	Wet AMD (Ophthalmology)							OxfordBioMedica
OXB-301	Cancer (multiple) (Oncology)							OxfordBioMedica
OXB Partnered products								
SAR422459	Stargardt disease (Ophthalmology)							SANOFI
SAR421869	Usher syndrome type 1B (Ophthalmology)							SANOFI
IP enabled & royalty bearing products								
CTL019	Cancer (multiple) (Oncology)							NOVARTIS
Undisclosed CAR-T	Cancer (multiple) (Oncology)							NOVARTIS
LV305	Cancer (multiple) (Oncology)							IMMUNE DESIGN
Undisclosed	Undisclosed							gsk GlaxoSmithKline
Undisclosed	Undisclosed							gsk GlaxoSmithKline

To be spun out or out-licensed

Development milestones and royalties

Process development and bioprocessing revenues, and royalties

Proprietary R&D Activity

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

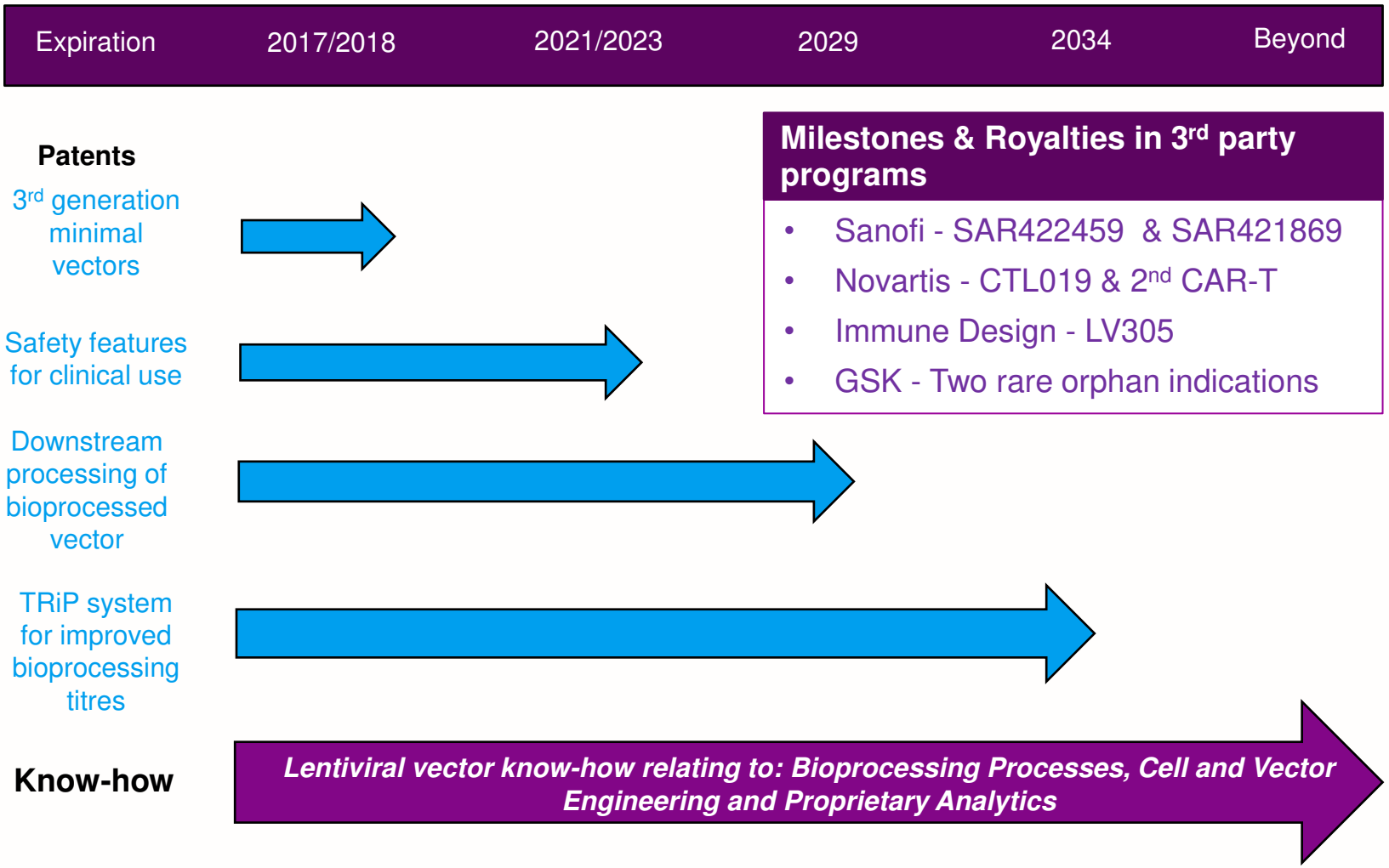
- Several ocular orphan diseases programmes
- CNS orphan disease programme
- Respiratory orphan disease programme
- Gene-modified NK cell therapeutics with Green Cross LabCell for cancer

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 manufacturing
  - 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



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
- 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: more than 250 employees

Source: https://resources.oncourse.iu.edu/access/content/user/leema/profilepage/oxford.html

State-of-the-art Bioprocessing Facilities (Oxford, UK)

Two separate bioprocessing sites (total clean rooms 1,200m^2/12,917ft^2)

Laboratories (2,136m^2/22,992ft^2)

Harrow House

- Two independent GMP clean room suites (GMP1 and GMP2) totalling 640m^2/6,889ft^2
- GMP2 facility designed for up to two 200L single use bioreactors
- Potential for further expansion

Yarnton

- One independent GMP clean room suite (GMP4) of 560m^2/6,028ft^2
- Potential for use with 200L single use bioreactors

Windrush Court Laboratories

- Nine Tissue Culture Laboratories with 24 Microsafety Cabinets
- Two Analytical Services Group (ASG) Laboratories
- Cell Engineering Laboratory
- Three Bio Safety 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

Future Vision and Summary

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Potential Near-term Catalysts (Next 12 Months)

- Novartis catalysts
  - Novartis CTL-019 study results
  - Novartis CTL-019 BLA submission
  - Milestones and royalties
- 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 in OXB-102 and OXB-202 Phase I/ II clinical studies with appropriate partner

Vision of Oxford BioMedica – by end 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 assumed

Sanofi

- SAR422459 to be in a pivotal trial (Phase IIb/Phase III)

Immune Design

- LV305 progressing well in clinical development

OXB Products with Partners

- 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

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

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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 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 has world-class bioprocessing facilities and collaboration track-record in the field
5. 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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