The LentiVector® Company
A leader in gene and cell therapy

BIO CEO & Investor Conference
New York, 13-14 February 2017

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Corporate Overview

>20 years as the leader in lentiviral vectors

- ✓ **1st** to administer *in vivo* (both brain and eye)
- ✓ **>60** patients treated *in vivo*
- ✓ **Four** Phase I/II studies completed with encouraging safety and efficacy
- ✓ **Five** in-house products, available for spin out or out-licensing

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

Partnered with

NOVARTIS

IMMUNE DESIGN

Green Cross LabCell

Orchard therapeutics

Discussions with several other potential partners ongoing

Products & patents licensed to

SANOFI

gsk GlaxoSmithKline

Leading Lentiviral Vector Delivery Platform (LentiVector®)
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]

- 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* 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 preferred choice
- Lentiviral vectors have demonstrated long-term efficacy , supporting 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, technology, employees' expertise, bioprocessing & laboratory facilities

4 World-class bioprocessing capabilities and track-record

- Novartis CTL-019 process development and bioprocessing
- Agreements with Immune Design, Orchard Therapeutics and Green Cross LabCell, others in discussion
- State-of-the-art bioprocessing facilities, expertise and know-how

5 OXB's product portfolio & Royalty Streams

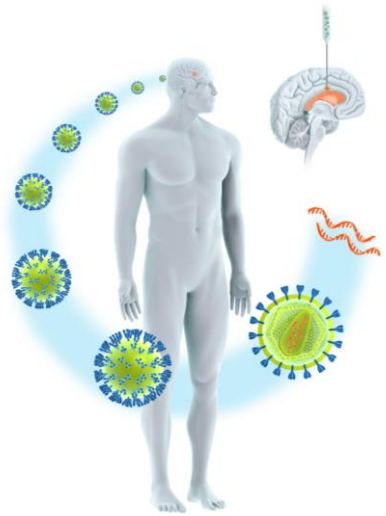
- OXB-102 & OXB-202 to be spun out or out-licensed, entering Phase I/II clinical studies in next 12 months and OXB-302 is a CAR-T pre-clinical programme targeting solid tumours
- Milestones & Royalties on partners' products: Sanofi (SAR422459 and SAR421869); Novartis (CTL019 and an other undisclosed CAR-T programme); Immune Design (LV305) Orchard Therapeutics (ADA-SCID and MPS IIIA) 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.

The Gene and Cell Therapy Revolution

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

In vivo development

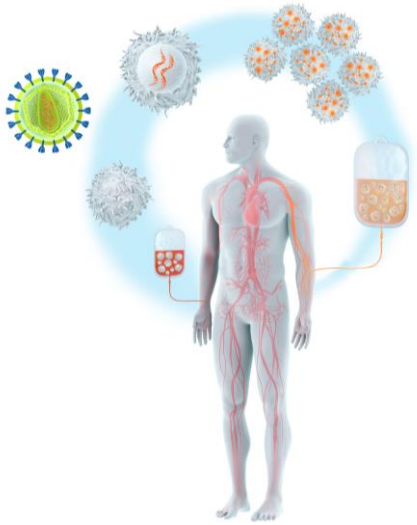


- Direct administration of lentiviral vectors to target organ *in vivo*
- Lentiviral vectors have advantages vs. AAV
  - Larger therapeutic payloads (up to 9 kb)
  - Permanent modification of dividing cells
  - No pre-existing immunity
- OXB's lentiviral vector administered to >60 patients & cumulative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

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

Example: OXB-102

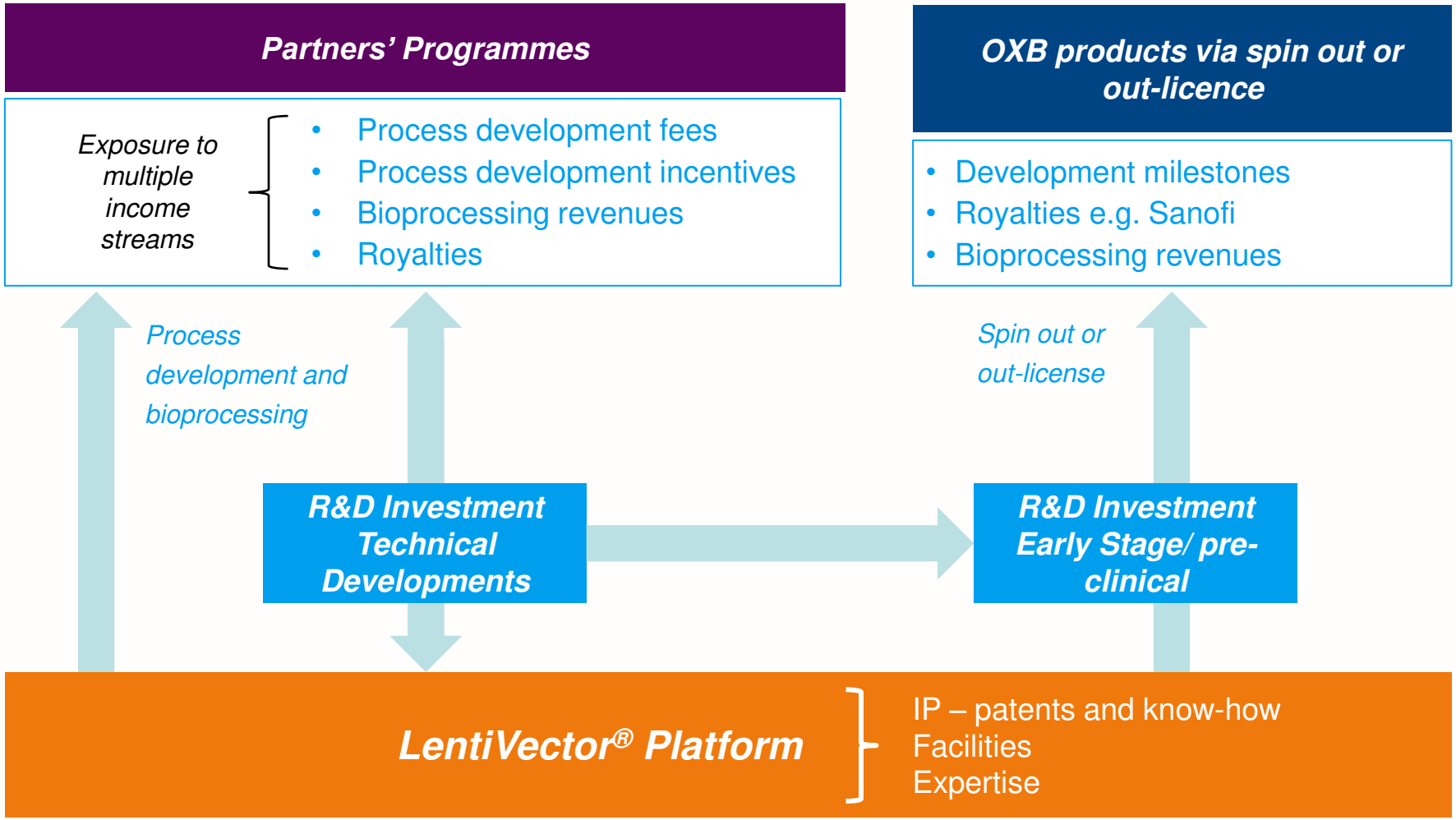
Ex vivo development



- 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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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
ADA-SCID	ADA severe combined immunodeficiency							Orchard therapeutics
MPS IIIA	Sanfilippo A syndrome							Orchard therapeutics
Undisclosed x2	Undisclosed x2							gsk GlaxoSmithKline

To be spun out or out-licensed

Development milestones and royalties

Process development and bioprocessing revenues, and royalties

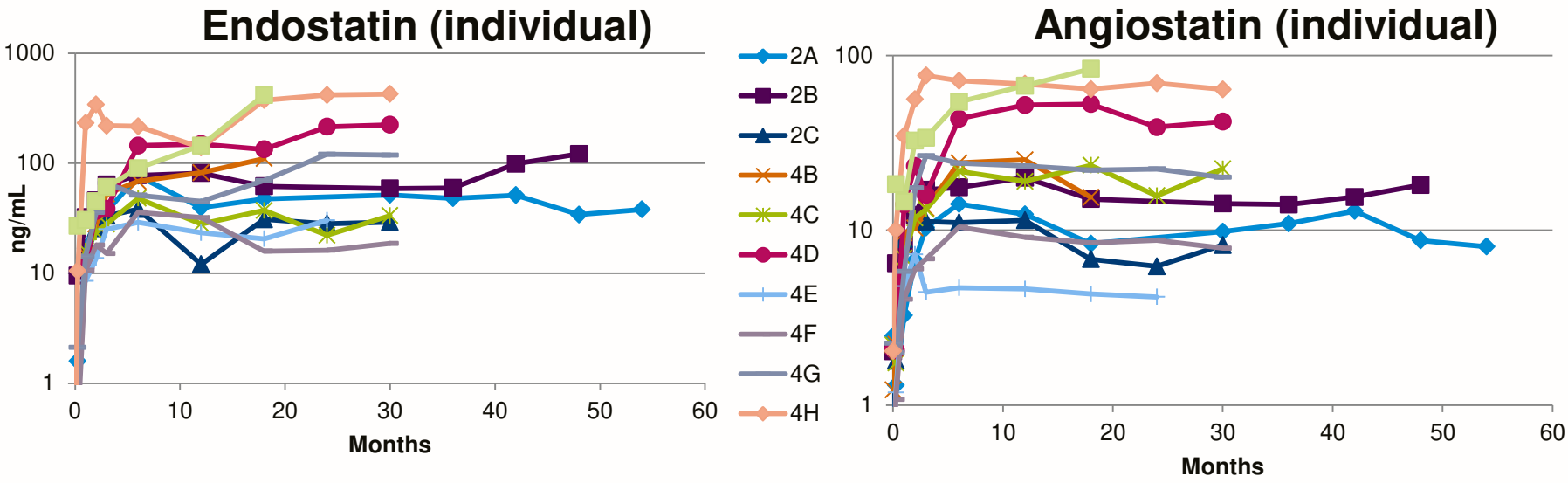
Clinical Lentiviral Vector Experience

- OXB-101 - 15 patients treated via stereotactic delivery[1]
  - Safe and well tolerated with cohort 1 out to 7 years
- OXB-201 - 21 patients treated via subretinal delivery
  - Safe and well tolerated with cohort 1 out to 4 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 3 years[2]
  - Safe and well tolerated with SAR421869 cohort 1 out to 2 years[3]
- 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)**

[1] Binley, K et al. Oral Presentation at ASGCT Conference, Washington DC, May 2016

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Partnered/IP Enabled & Royalty Bearing Products

Partnerships/royalty bearing products

- Sanofi (SAR422459, SAR421869)
  - Clinical analysis services
  - Bioprocessing support services
- Novartis (CTL019, unnamed CAR-T)
  - Process development collaboration
  - Bioprocessing
- Immune Design (LV305)
  - Process development collaboration
  - Bioprocessing
- Orchard Therapeutics (ADA-SCID, MPS IIIA)
  - Process development collaboration
  - Bioprocessing
- GlaxoSmithKline (2 products for undisclosed rare indications)
  - License to operate under OXB patents

Novartis CAR-T Partnership

Overview of 2014 Contract

- 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:
  - Collaboration in process development
  - Performance incentives paid on achievement of targets

Achievements to date

- Multiple CTL019 batches supplied to Novartis since October 2014 for use in clinical studies – and multiple confirmed purchase orders through 2017
- Successful development of 200 litre process. Pilot studies suggest significant productivity improvement
- ELIANA clinical study data announced December 2016. Novartis plan to file CTL019 BLA "early 2017". Approval expected in 2017 due to FDA Breakthrough Therapy designation

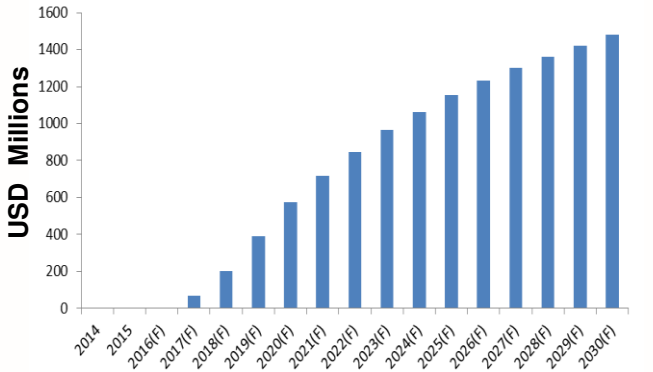
Forward Looking

- OXB will be sole manufacturer for commercial launch expected in H2 2017
- Royalty flow expected to start in H2 2017. Novartis have indicated potential blockbuster status
- Work on second CAR-T programme (undisclosed indication) set to expand

Novartis lists CTL019 as one of its late stage potential blockbuster products

- Novartis global Phase II clinical trial (ELIANA) evaluating efficacy and safety of CTL019 in r/r ALL in paediatric and young adults was presented at ASH, 03 December 2016 (Abstract #221)
  - 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
- Novartis R&D update on 25 January 2017 included CTL019 in its list of late stage potential blockbuster products
- Analysts forecast[1,2] at least $1 billion worldwide peak sales for CTL019

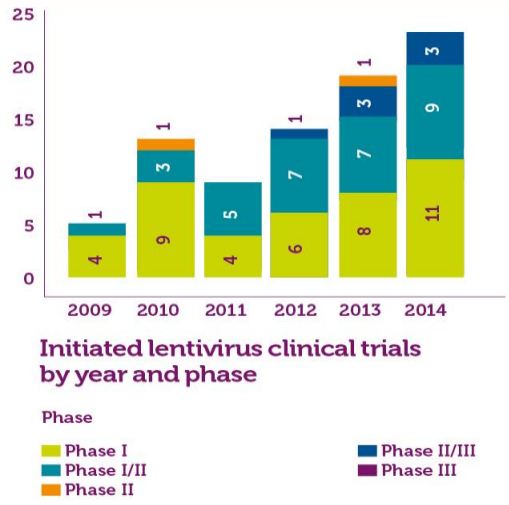
CTL019 consensus sales forecasts [1]



Results set the stage for filing of CTL019 with the FDA in early 2017 for paediatric and young adult r/r ALL and with EMA (PRIME designation) late in 2017. Launch planned for H2 2017 with blockbuster status and important sustainable revenue stream to Oxford BioMedica

[1] Global Data Pharma eTrack Product Sales/Analyst Consensus, March 2016. Forecasts are derived from Leerink Partners, Cowen & Co., Auerbach Grayson & Co.
[2] Jefferies note published 25 January 2017.

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

Proprietary R&D Activity

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

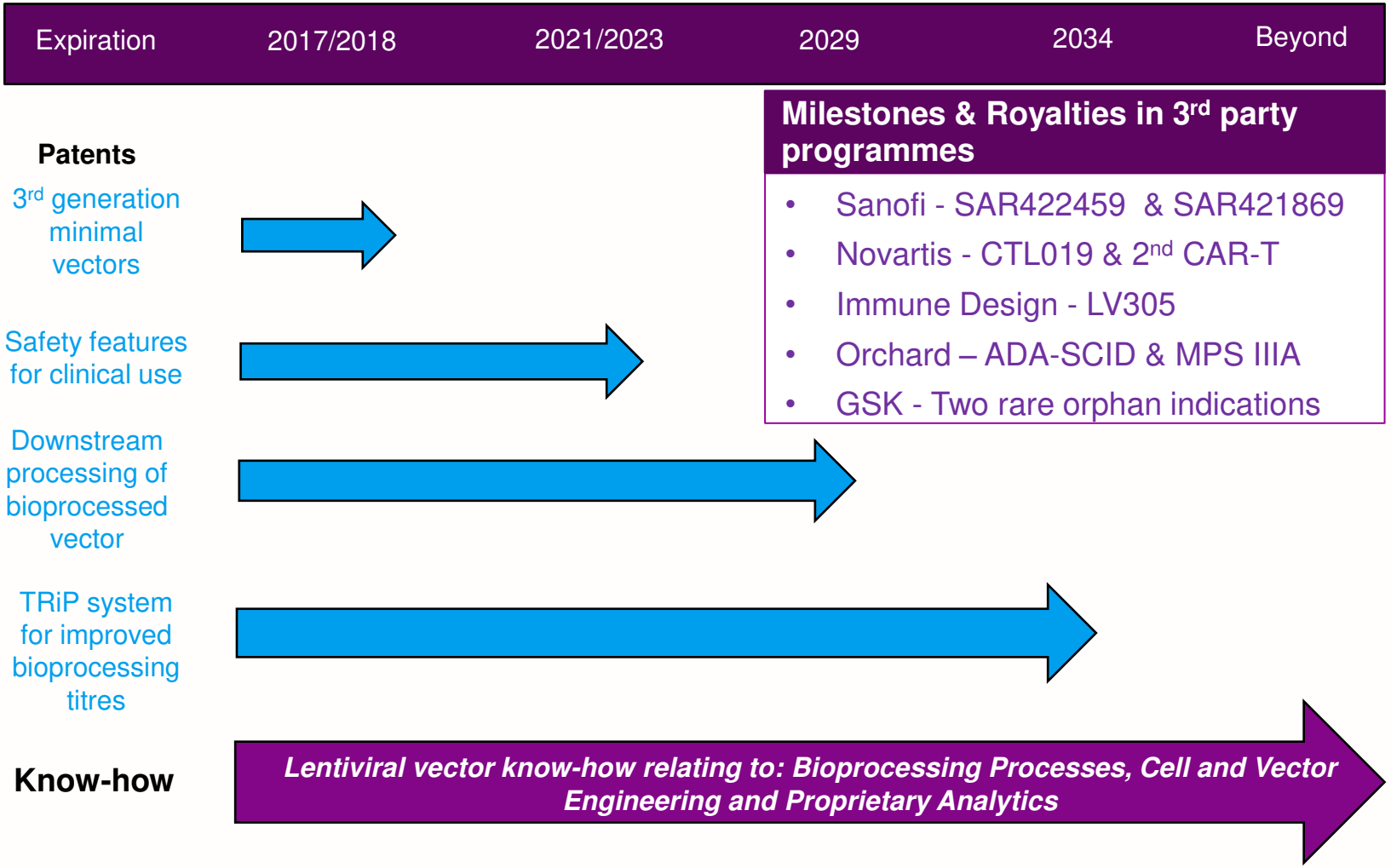
- Several ocular orphan diseases programmes
- CNS orphan disease programme
- Respiratory orphan disease programme
- Gene-modified NK cell therapeutics with Green Cross LabCell for cancer

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

Potential Near-term Catalysts (Next 12 Months)

- Novartis catalysts
  - CTL-019 ELIANA global registration Phase II study results in paediatric r/r ALL presented (Abstract # 221) at ASH 3 Dec 2016
  - CTL-019 BLA submission expected early 2017
  - Commercial supply agreement
  - FDA approval/product launch – royalties start
- LentiVector® delivery platform
  - Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates
  - 200L bioreactor serum-free suspension process confirmed and operational
    - 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

Orchard Therapeutics

- ADA-SCID pivotal trial close to completion
- MPS IIIA 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

OxfordBioMedica

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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