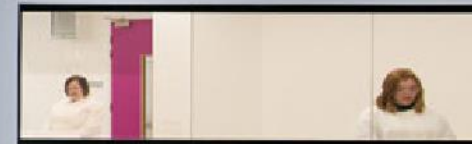


# The LentiVector® Platform Company

## A leader in gene and cell therapy

Jefferies Healthcare Conference  
New York, June 2017

John Dawson, CEO



# Forward-looking statements

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# 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 unique advantages for cell and gene therapy

3

- OXB's sought-after LentiVector<sup>®</sup> 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

<sup>1</sup> Clive Glover, GE Healthcare "Sales of cell and gene therapy will reach \$10 billion by 2021", October 2015.

# Corporate Overview



## >20 years as the leader in lentiviral vectors

- ✓ **1<sup>st</sup>** 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<sup>®</sup> 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



Discussions with several other potential partners ongoing

Products & patents licensed to



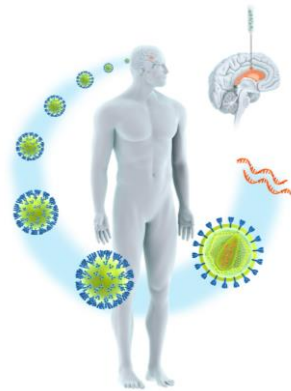
# The Gene and Cell Therapy Revolution

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. CTL019

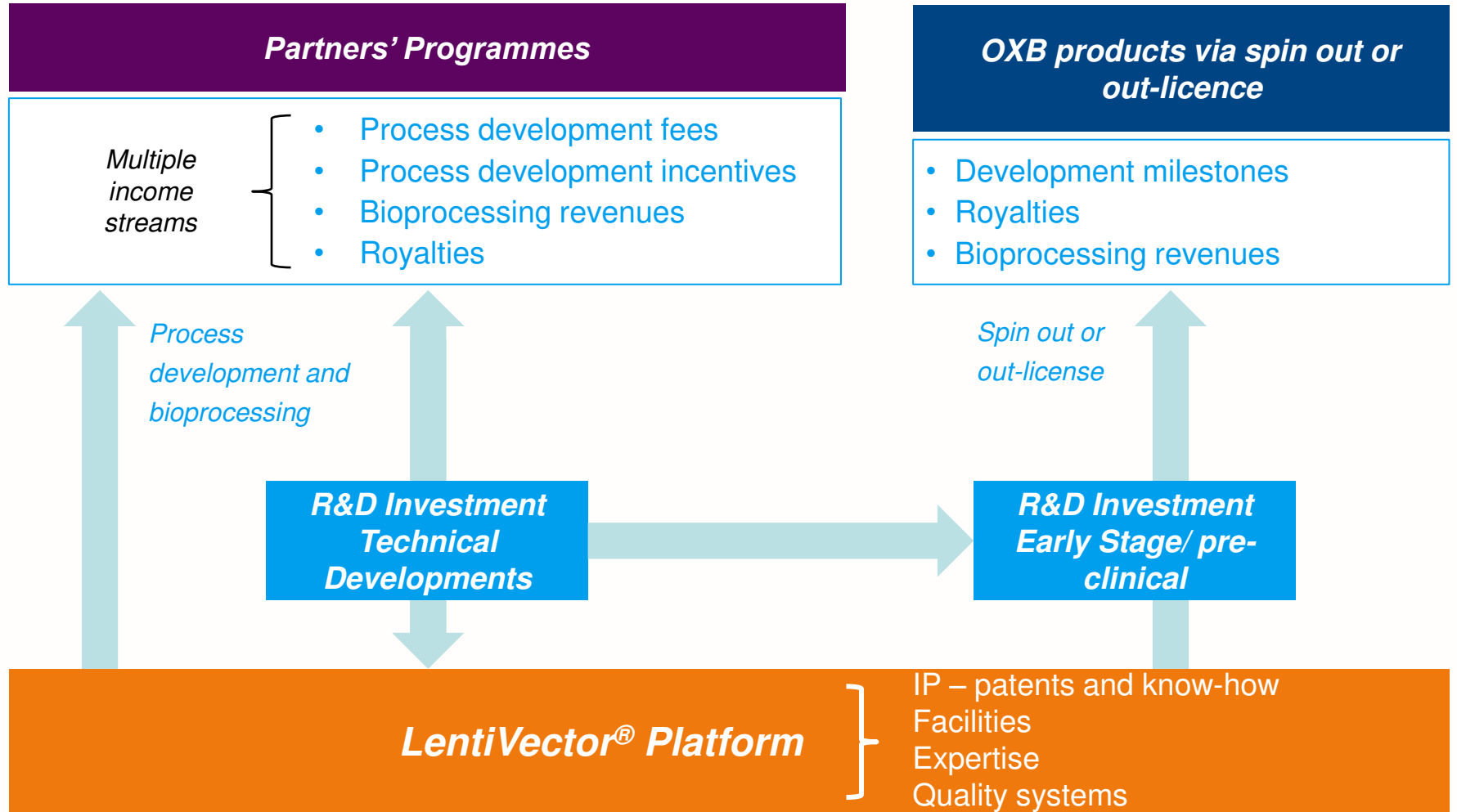
- OXB produces GMP lentiviral vector encoding CAR targeting CD19
- T-cells isolated from patient's blood and transduced with OXB vector
- Modified T-cells are infused back into the patient
- Once re-infused, the T-cells multiply, "hunt" cancer cells and destroy them



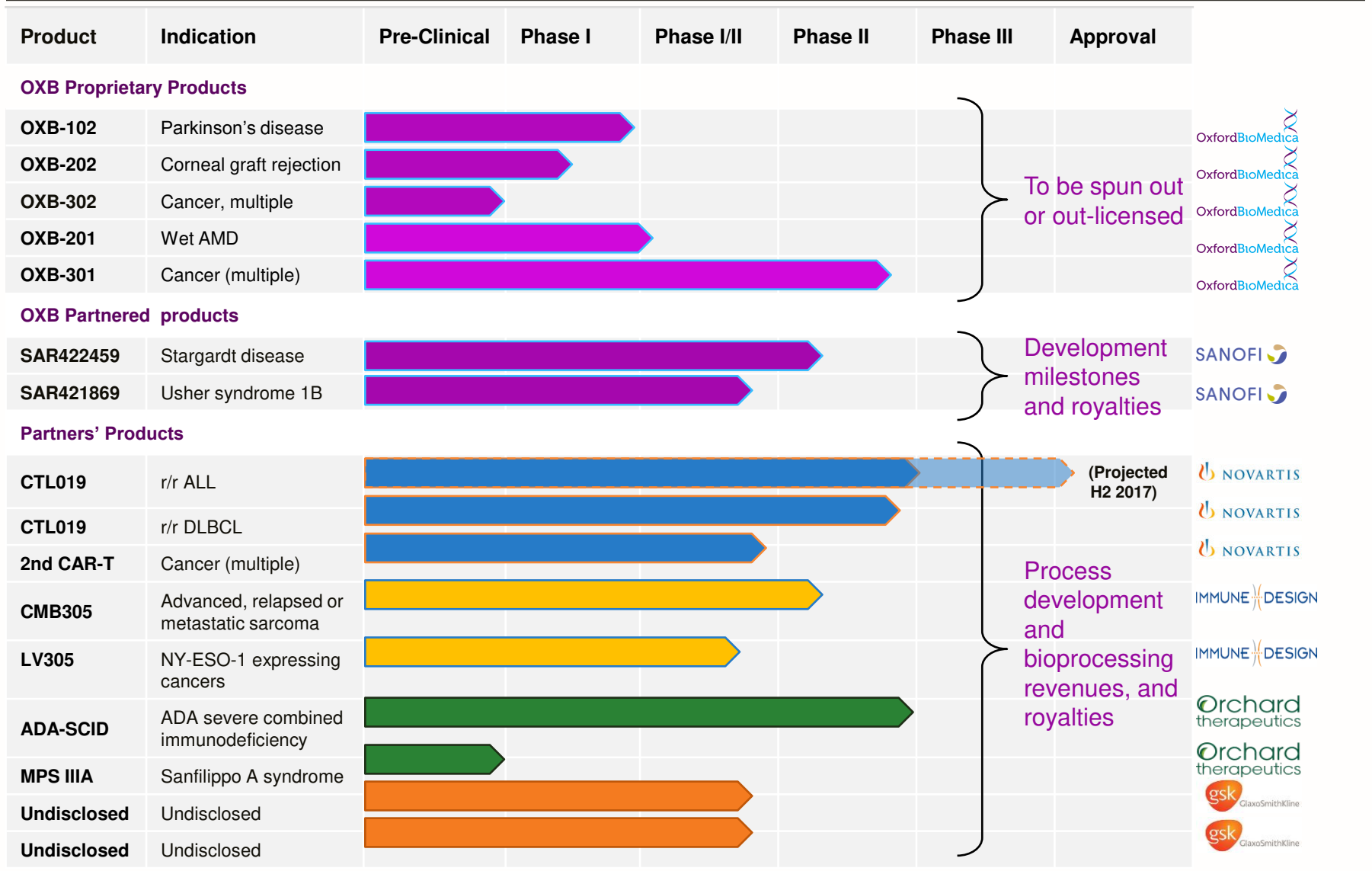
## Lentiviral vector advantages for cell and gene therapy

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

# Strategy: Leveraging Our LentiVector® Delivery Platform



# Products Pipeline - Proprietary and Partnered



# Clinical Lentiviral Vector Experience

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- OXB's lentiviral vector administered to >100 patients (by OXB or its partners) and cumulative patient safety data >300 years

## ***In Vivo***

- OXB-101 - 15 patients treated via stereotactic delivery<sup>1</sup>
  - 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<sup>2</sup>
  - Safe and well tolerated with SAR421869 cohort 1 out to 2 years<sup>3</sup>

## ***Ex Vivo***

- 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.*)

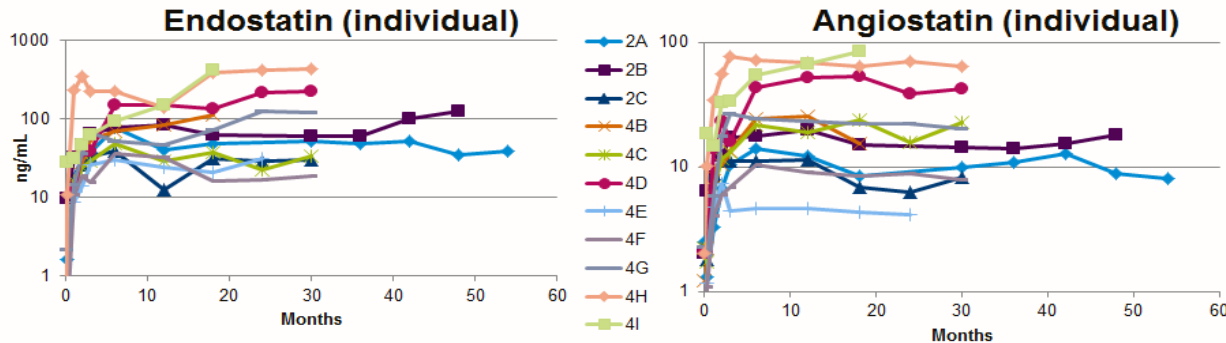
2 Binley *et al.* Transduction of Photoreceptors With Equine Infectious Anemia Virus Lentiviral Vectors: Safety and Biodistribution of StarGen for Stargardt Disease. *IOVS* 54 (6): 4061-4071, 2013

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.



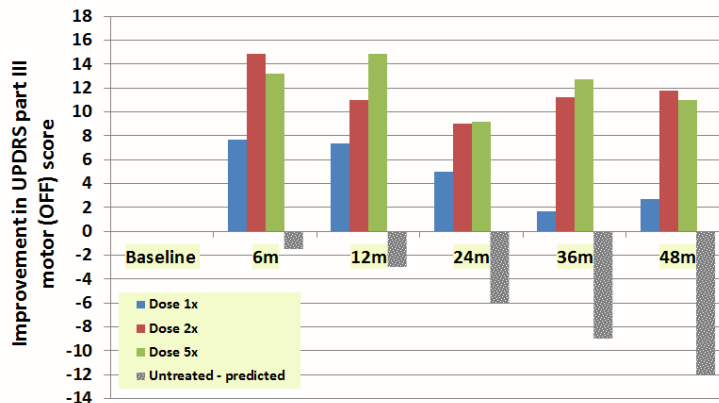
# LentiVector® Platform Evidence of Long-term Duration

- Long-term four year follow up data for OXB-201<sup>1</sup>
  - 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<sup>2</sup>



- At 12m, all patients had equal or better UPDRS Part III OFF scores than at baseline
- At 24m 12 out of 14 patients<sup>2</sup>, at 36m 10 out of 11 of patients<sup>2</sup>, 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 1-2pt/year

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

<sup>2</sup> 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

## 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 produce CTL019 for clinical studies; extendable
- Process Improvements:
  - Collaboration in process development
  - Performance incentives paid on achievement of targets
- \$90m over 2014-2017

## Achievements to date

- Multiple CTL019 clinical study batches supplied to Novartis since October 2014
- Multiple confirmed purchase orders through 2017
- ELIANA clinical study data announced December 2016. On 29 March 2017, the FDA accepted the BLA filing and granted a priority review for CTL019
- Novartis have indicated potential blockbuster status
- r/r DLBCL granted FDA Breakthrough Therapy designation
- Successful development of 200 litre process with significant productivity improvements

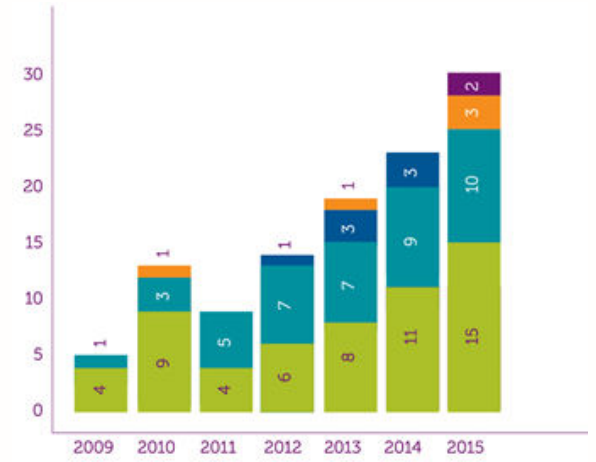
## Forward Looking

- JULIET study (DLBCL) data expected 12 June 2017
- CTL019 approval expected Q3 2017
- OXB will be sole manufacturer for product launch
- Royalty flow expected to start in H2 2017.
- 2<sup>nd</sup> CAR-T programme (undisclosed indication) to expand

# Business development

## - increasing lentiviral vector clinical/pre-clinical trial activity

Examples of companies working in clinical development



Initiated lentivirus clinical trials by year and phase

Phase

- Phase I
- Phase I/II
- Phase II
- Phase II/III
- Phase III

Source: Journal of Gene Medicine, August 2016

Examples of companies working in pre-clinical development




# Proprietary R&D Activity

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

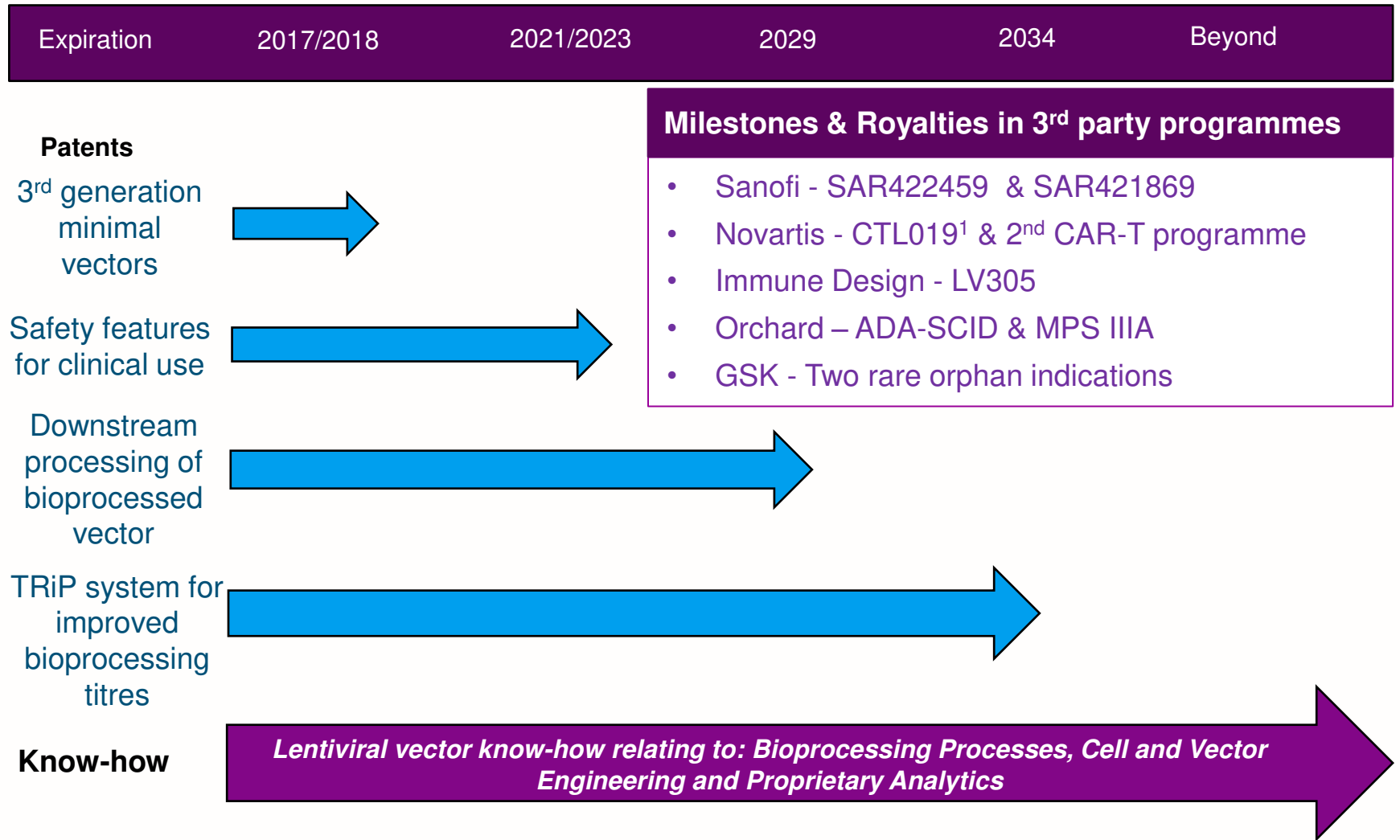
- Several ocular orphan diseases programmes
- CNS orphan disease programme
- Respiratory orphan disease programme
- Gene-modified NK cell therapeutics with Green Cross LabCell for cancer

## 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



<sup>1</sup> 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



# Potential catalysts over next 12 months

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- Novartis progress
  - Data from JULIET (adult r/r DLBCL study) expected June 2017
  - Confirmation of OXB commercial supply agreement for CTL019 vector
  - FDA approval of CTL019 for r/r ALL and product launch
  - Submission of DLBCL for approval
- LentiVector<sup>®</sup> delivery platform
  - Approval to supply lentiviral vector for commercial use
  - 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

# 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 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





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