

The LentiVector® Platform Company A leader in gene and cell therapy

Oxford BioMedica and Bioverativ Collaboration and Licence Agreement





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World's leading gene and cell therapy company



LentiVector Enabled

Pioneers of unique LentiVector-Enabled technology – partnered in first ever approved gene therapy launched in the US market¹ and further validated through the deal with Bioverativ

01



Partnerships with Novartis,
Bioverativ, Immune
Design, GC LabCell and
Orchard Therapeutics.
Products and patents
licensed to Sanofi and
GlaxoSmithKline and own
pipeline of assets

02

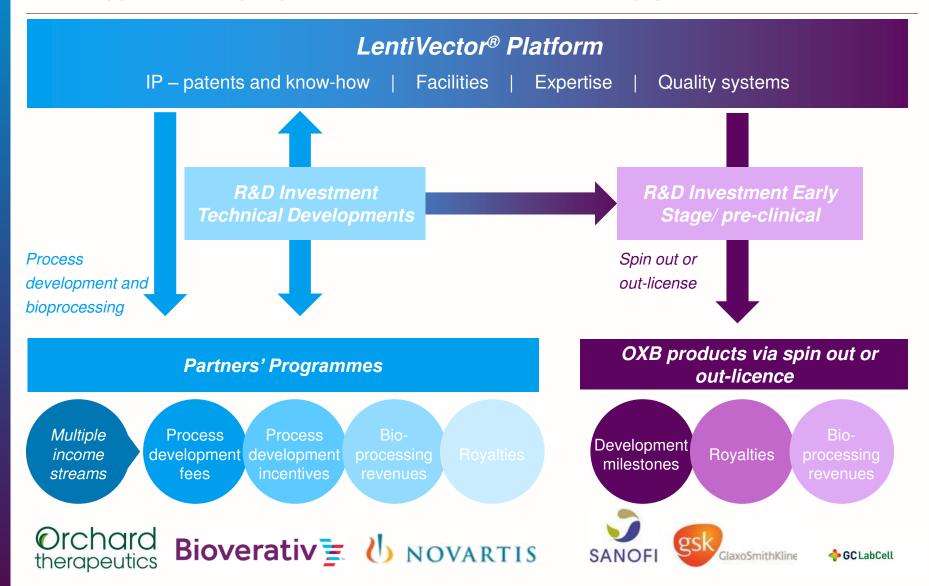


World class FDA and MHRA approved state-of-the-art bioprocessing and laboratory facilities for clinical and commercial supply

03



Strategy: Leveraging our LentiVector Enabled delivery platform





Collaboration & Licence Agreement with Bioverativ

Collaboration overview

- Development of *in vivo* gene therapy for:
 - haemophilia A (Factor VIII deficiency)
 - haemophilia B (Factor IX deficiency)
- Licence to Oxford BioMedica's LentiVector Enabled technology
- Access to Oxford BioMedica's industrial-scale manufacturing technology
- Potential for clinical supply agreement

Key Terms

- \$5 million on closing
- Up to \$100 million for:
 - product development
 - regulatory and sales-related milestones
- Undisclosed royalty payable on net sales

Bioverativ

- Bioverativ was an independent biopharmaceutical company spun out of Biogen's haemophilia business in 2017 but is now subject to an acquisition by Sanofi
- Bioverativ is dedicated to transforming the lives of people with haemophilia, cold agglutinin disease and other rare blood disorders
- Bioverativ's haemophilia therapies were the first major advancements in the treatment of haemophilia A and B in two decades
- Bioverativ is currently still listed on the US NASDAQ market and has a market capitalisation of around \$6bn
- Revenues of around \$890m in FY 2016¹

"Beginning with hemophilia, we will lead by doing what's right for patients and those who care for them, and by actively working with the blood disorders community to accelerate innovation and develop life-changing treatments"

Bioverativ

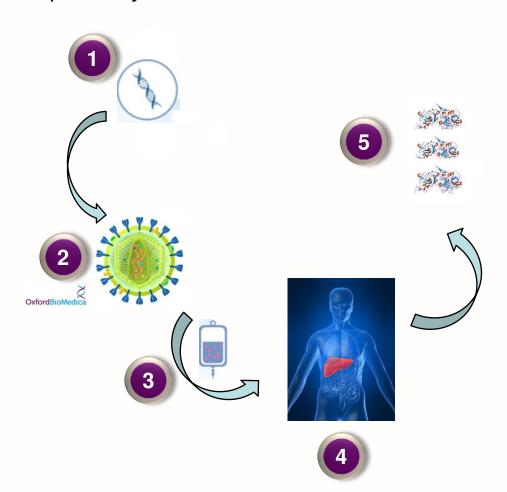


Haemophilia Market

- Haemophilia is an x-linked hereditary bleeding disorder characterised by impaired blood coagulation as result of deficiencies in the production or function of coagulation Factor VIII (FVIII) in haemophilia A or Factor IX (FIX)
- In the 7 major markets, diagnosed prevalent cases of haemophilia A and haemophilia B will rise from 51,500 in 2016 to around 52,600 in 2026¹
- US has the highest number of prevalent cases at around 34%²
- Sales of products to treat haemophilia in 7 major markets reached \$6.7bn in 2016 and is forecast to reach \$8.0bn by 2026³
- Unmet need for one-off long term treatments
- AAV vectors have been employed for development of gene therapies for haemophilia, but duration of treatment effect and potential for treating young patients is not yet determined

How gene therapy addresses haemophilia

In vivo gene therapy to produce FVIII and FIX to treat haemophilia A and B respectively

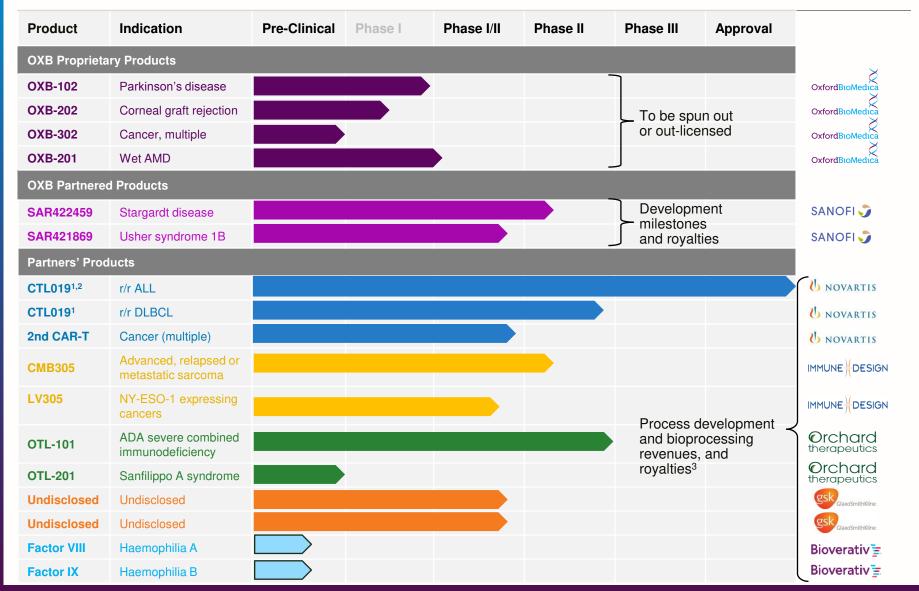


- DNA encoding either FVIII or FIX is produced
- 2. Lentiviral vector carrying either FVIII or FIX genes
- 3. Systemic or intra-hepatic injection of the lentiviral vector manufactured by OXB
- 4. Once inside the patient, the lentiviral vector reaches the liver and inserts into the hepatocyte genome in order to produce the appropriate proteins
- 5. New FVIII or FIX proteins released into the bloodstream for a potential one-time treatment



Product pipeline





¹ USAN name is tisagenlecleucel

² Approved in the US only

³ GSK partnership is a option fee and royalty agreement

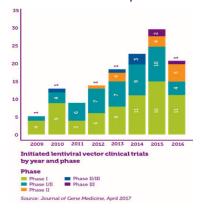
Extensive lentiviral vector clinical/pre-clinical trial activity



The New York Times

New York Times, Gina Kolata (27 November 2017)

"Few gene-therapy companies have the factories or expertise to make the viruses for use in clinical trials, where standards are exacting and comprehensive. The firms that can do it are swamped with orders and requests."





















Summary: World's leading gene and cell therapy company





Collaboration and licence agreement signed with Bioverativ. \$5m upfront and up to \$100m in product related milestones, regulatory and sales related milestones. Royalties on net sales on both products

01



The haemophilia market is large, more than \$6bn in sales in 2016, with an unmet need for one-off long term therapy

02

LentiVector Enabled

Gene therapy approaches to treat haemophilia are transformative and this partnership may offer patients an important new treatment option

03



Expected upcoming news flow

Partners' programmes progress

2nd CAR-T programme to enter clinic

Royalties commence from Novartis/CTL019¹

Expected approval for additional adult r/r DLBCL indication in US in Q1 2018.

Expected EMA approval for paediatric r/r ALL and adult r/r DLBCL in EU in Q2 2018

Bioverativ gene therapy product for haemophilia A & B progressing towards the clinic

LentiVector® delivery platform

Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates by end of 2018

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® enabled platform

Lead gene-modified NK cell therapeutic candidate emerging from the GC Lab Cell research collaboration

Technical developments – continuous improvement of the LentiVector® enabled platform

Feeds further product partnership opportunities

Product pipeline

OXB priority products

- Successful spin-outs and/or out-licensing
- Products progressing into Phase I/II studies

Novartis

- Oxford BioMedica supplying commercial material
- Royalties from CTL019¹
- 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

- OTL-101 pivotal trial close to completion
- OTL-201 in clinical development

Bioverativ

 Haemophilia A & B gene therapy progressing towards the clinic

Several further partnerships

 Economic interests in a range of gene and cell therapy products

Bioprocessing

Facilities operating at high capacity

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