OxfordBioMedica

Discover. Realise.

Gene therapy is now

Annual report and accounts 2017

Oxford BioMedica in brief

Oxford BioMedica is a pioneer of gene and cell therapy with a leading position in lentiviral vector and cell therapy research, development and bioprocessing. Gene and cell therapy is the treatment of disease by the delivery of therapeutic DNA into a patient's cells. This can be achieved either *in vivo* (referred to as gene therapy) or *ex vivo* (referred to as cell therapy), the latter being where the patient's cells are genetically modi ed outside the body before being re-infused.

Oxford BioMedica is focused on developing life changing treatments for serious diseases. Oxford BioMedica and its subsidiaries (the "Group") have built a sector leading lentiviral vector delivery platform (LentiVector ®), which the Group leverages to develop *in vivo* and *ex vivo* products both in-house and with partners. The Group has created a valuable proprietary portfolio of gene and cell therapy product candidates in the areas of oncology, ophthalmology and CNS disorders.

The Group has also entered into a number of partnerships, including with Novartis, Sano , GlaxoSmithKline, Bioverativ, Orchard Therapeutics, GC LabCell and Immune Design, through which it has long-term economic interests in other potential gene and cell therapy products. Oxford BioMedica is based across several locations in Oxfordshire, UK and employs more than 300 people.

OxfordBioMedica | LentiVector Enabled

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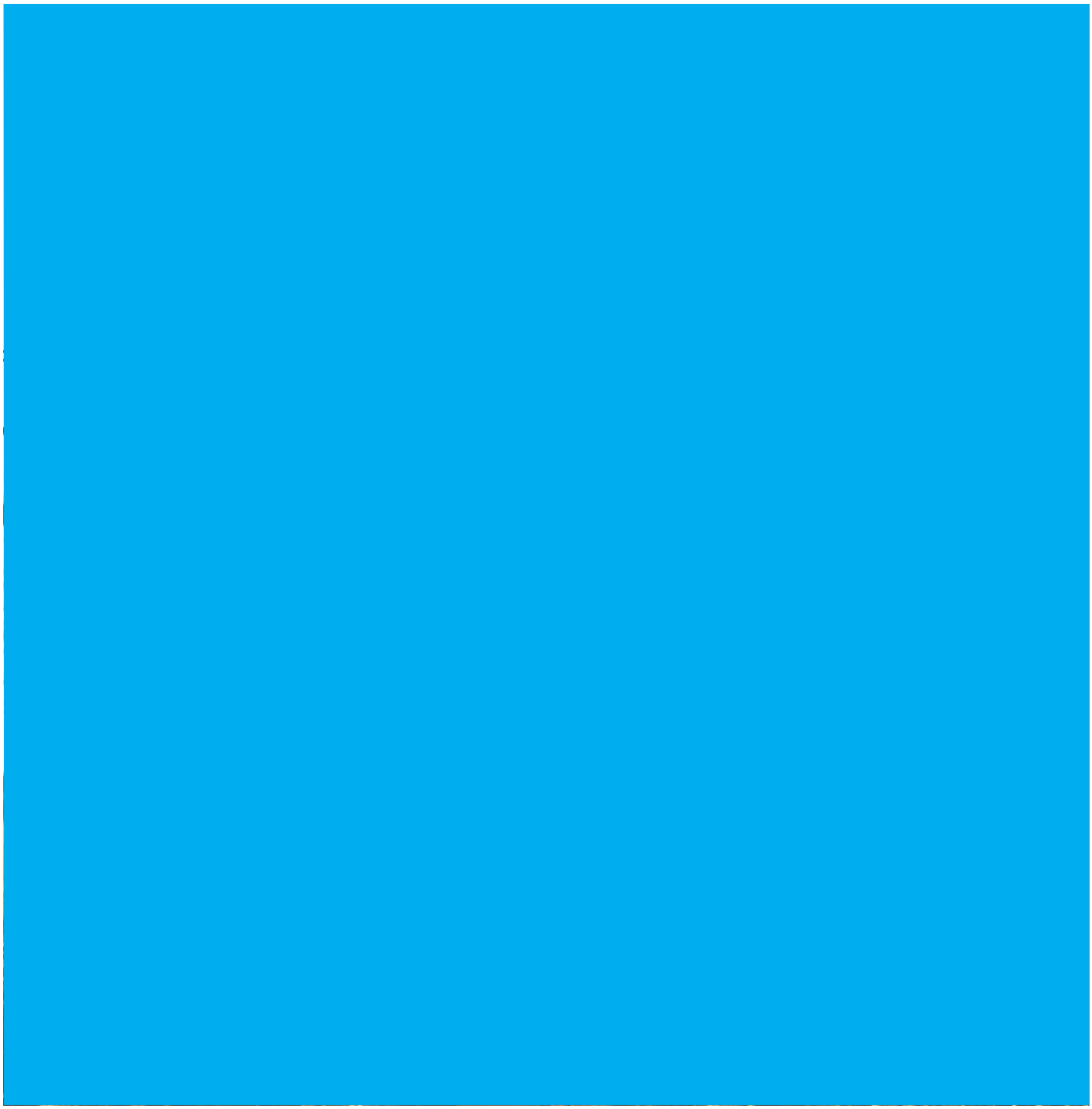
At the front and centre

Oxford BioMedica is a leading gene and cell therapy company and our work is now beginning to deliver life changing treatments.

It's an exciting time for science, our partners, shareholders, and most of all the patients who are now bene ting from marketed products – giving people su ering from some of the world's most serious diseases far more than hope.

We are at the front and centre of gene and cell therapy and experiencing strong demand for our world-leading proprietary LentiVector-Enabled ™ technology.



Enabling 'one shot' treatments

LentiVector ® studies suggest gene expression may be maintained inde nitely (for more than six years), o ering patients the prospect of long-term clinical bene t following a single administration.

Valuable research tool

The LentiVector ® platform is also used as a valuable research tool.

Multi-billion $ market sector

The use of DNA to treat diseases is expected to grow into a multi-billion $ sector over the next few years with several products already approved in the US and Europe. The large growth in lentiviral vector based clinical trials shows *ex vivo* treatments are leading the way.

Read more about the gene and cell therapy sector on page 10.

Targeting unmet needs

Oxford BioMedica's pipeline focuses on a diverse range of cancers, Parkinson's, central nervous system disorders, and ocular conditions. However, our LentiVector ® delivery system could be used for many more diseases for which there are currently unmet needs.

Our LentiVector ® delivery platform is used in all of our own gene and cell therapy products in development.

See our LentiVector® based product pipeline on page 13 for more information.

>20 years

Gene and cell therapy expertise
The LentiVector ® delivery system, built on more than 20 years of experience, has the potential to be used for many unmet conditions and disease areas.

140 sites

Clinical trials
There are currently over 140 sites worldwide carrying out clinical trials with lentiviral vectors.

Enabling new treatments

LentiVector ® is a key component of Novartis' Kymriah ™ cell therapy treatment for blood cancer and the first lentiviral enabled product to be approved in the USA.

We are the sole lentiviral vector manufacturer for this product which was launched in the USA in September 2017.

Find out more about our commercial supply agreement with Novartis and our new collaboration and licence agreement with Bioverativ in the Chief Executive's statement on page 24 and the 2017 performance review on page 26.

Sharing in success

In addition, we have partnerships and collaborations with Bioverativ, Orchard Therapeutics, GC LabCell and Immune Design where we retain a potentially royalty generating financial interest in their products in return for expertise and bioprocessing work.

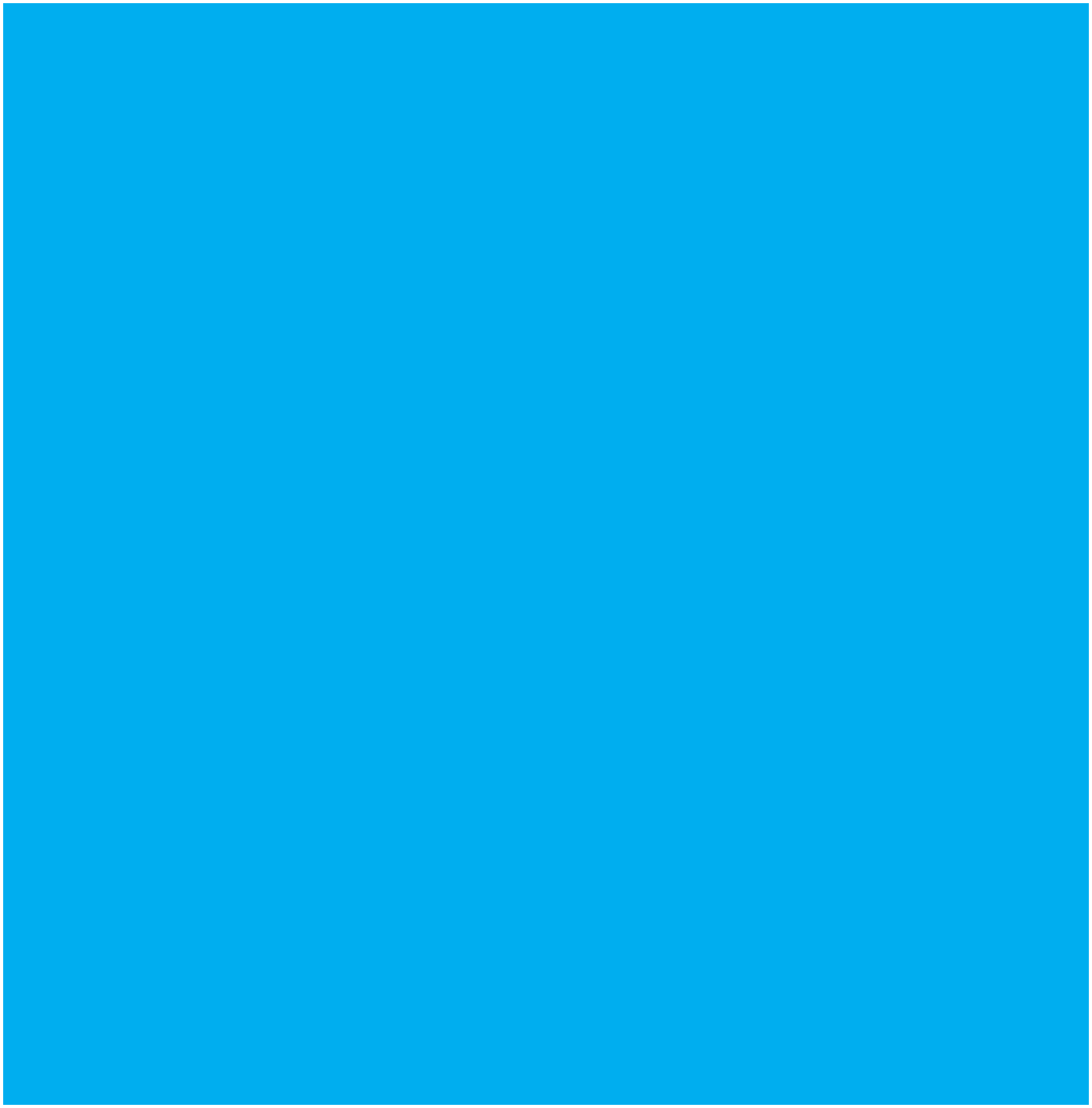
Oxford BioMedica also has products and IP licensed to Sanofi and GSK.

1st approved

FDA approved advanced therapy
The Novartis Kymriah ™ treatment for leukaemia that uses Oxford BioMedica's LentiVector ® technology is the first FDA approved advanced therapy.

First-in-class therapy

Novartis' Kymriah ™ (CTL019) is a a novel treatment approach for paediatric and young adult patients with B-cell acute lymphoblastic leukaemia. Trials showed an 83% overall remission rate in this patient population which has limited treatment options and historically poor outcomes.



Progressing our own pipeline

We are progressing towards approval to start Phase I/II clinical trials of OXB-102 for Parkinson's disease.

OXB-102 genetically modi es cells to produce dopamine, replacing that which is lost during the course of the disease. Unlike current drug treatment options, which loses e cacy with long-term use, OXB-102 is designed to provide patient bene t for a number of years following a single administration.

Read more about OXB-102 and our other product candidates in the 2017 performance review on page 28.

7 products

Oxford BioMedica pipeline
Our own product development programmes have had seven regulatory approvals for clinical studies in the USA and Europe in ocular indications and Parkinson's disease.

Developing our partners' products

We help partners by providing unrivalled lentiviral IP and technical know-how such as our state-of-the-art laboratories and GMP clean room suites.

The US FDA completed a pre-licence inspection of our facilities as part of the Biologics License Application (BLA) review process for Novartis' Kymriah™. The UK MHRA granted Oxford BioMedica a manufacturer/ importer licence for commercial production and supply of lentiviral vectors following a successful inspection of our facilities.

Download our latest 'Discover the facts' brochure on lentiviral vector development, scale-up, analytics and GMP bioprocessing from the Oxford BioMedica website: www.oxfordbiomedica.co.uk

200

Patients treated
With our own and partners products using our LentiVector-Enabled ™ delivery based therapy.

Journey to profitability

Oxford BioMedica is now a business with rapidly growing revenues from process development, bioprocessing and royalty generating partnerships. Continued positive revenue growth is driving us closer to pro tability.

Understand more about our nancial position and how we are getting closer to our goal of becoming a sustainable business in the nancial review on page 32.

Ideally placed

Our nancial interest in a diverse range of products is growing and we will continue to invest in technology and proprietary gene and cell therapy concepts.

Gene therapy is now coming of age and we are ideally placed to bene t from the growth in this valuable sector over the coming years.

+$100m

Licence to supply LentiVector® to Novartis
In July 2017 we signed an agreement to supply lentiviral vectors for CTL019 and other undisclosed CAR-T products from Novartis, and could potentially receive in excess of $100 million.

-2%

Debt restructuring
The new $55 million loan facility with Oaktree is on much better terms than our previous Oberland agreement. The cost of the loan including all related fees will be around 13%, a signi cant reduction from the 15% cost of the Oberland facility.

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