



**Building the future**  
Preliminary results for the year ended  
31 December 2018

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March 2019

# Forward-looking statements

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# 2018 Highlights

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- **Delivery on Dual Strategy**
  - \$105 million collaboration and licence agreement with Bioverativ (now part of Sanofi)
  - \$842.5 million exclusive worldwide agreement signed with Axovant Gene Sciences for AXO-Lenti-PD (previously OXB-102) for the treatment of Parkinson's disease
  - Partnership formed with the UK Cystic Fibrosis Gene Therapy Consortium, Boehringer Ingelheim and Imperial Innovations to develop a novel inhaled gene therapy for cystic fibrosis
- **Strong Financial Growth**
  - Strong Gross Income<sup>1</sup> growth of 72% to £67.9 million (2017: £39.4 million)
  - Positive Operating EBITDA<sup>2</sup> of £13.4 million (2017: Operating EBITDA loss of £1.9 million)
  - Cash at 31 December 2018 of £32.2 million (2017: £14.3 million), reflecting significantly improved trading performance and £20.5 million placing (gross)
- **Building the Future**
  - Lease signed on 84,000 sqft facility in Oxford, fit out is on track and expected to more than double the number of GMP suites by mid 2020. Further lease signed in December 2018 for an additional 32,000 sqft premises next to Windrush Court to establish a new discovery and innovation facility
  - Staff will increase by c.40% from 432 at the end of 2018 to c.600 by the end of 2019
  - R&D collaboration announced March 2019 with Microsoft to improve cell and gene therapy delivery using artificial intelligence and machine learning
  - Expectation of new platform and proprietary pipeline partnership deals to be signed in the year

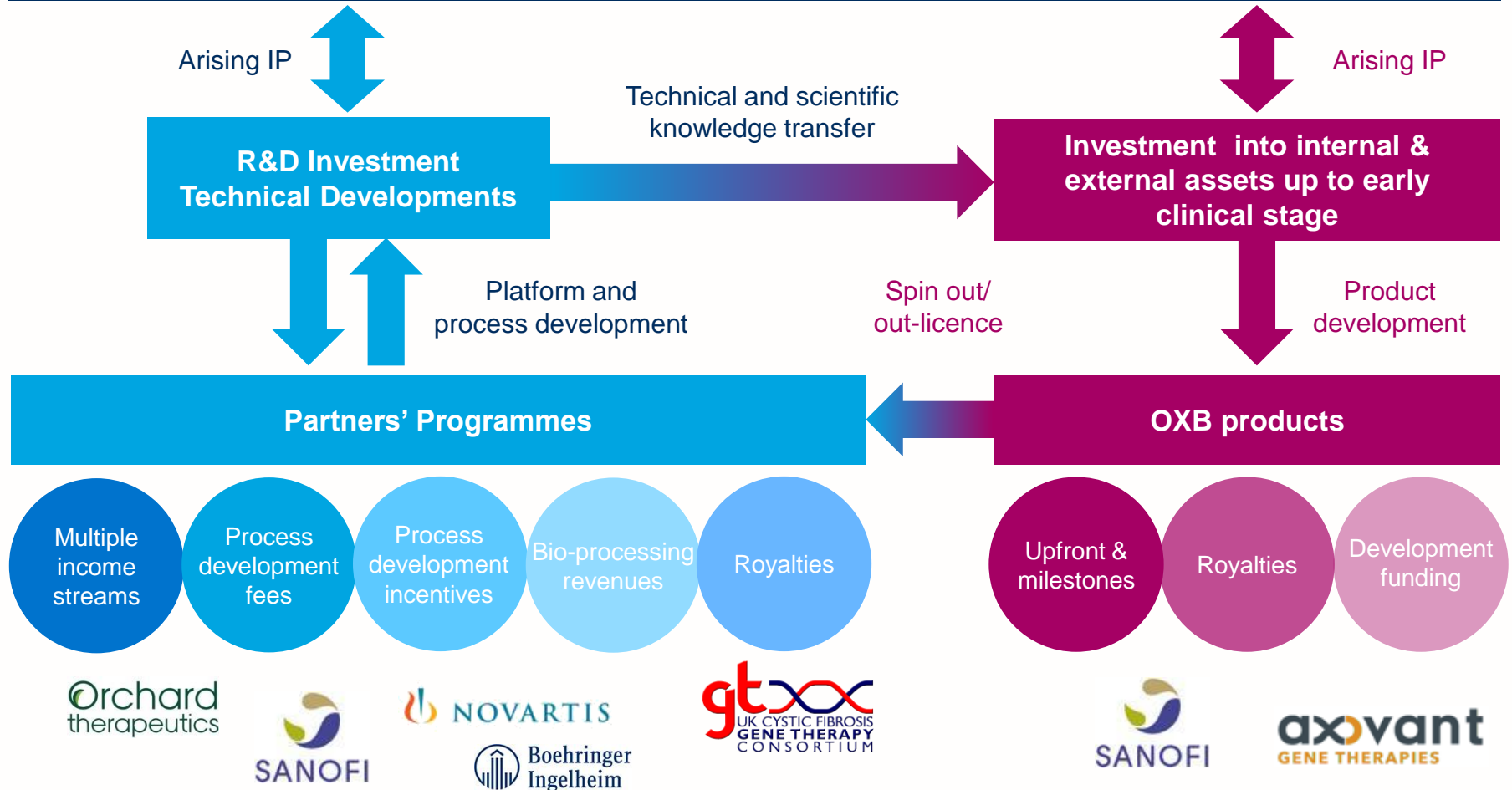
<sup>1</sup> Gross income = aggregate of revenue and other operating income

<sup>2</sup> Operating EBITDA = Earnings Before Interest, Tax, Depreciation, Amortisation and Share options

# Strategy: Leveraging our LentiVector<sup>Enabled</sup> delivery platform

## LentiVector<sup>®</sup> Platform

IP – patents and know-how | Facilities | Expertise | Quality systems



# Deal 1: Sanofi (Bioverativ) haemophilia partnership

## Sanofi (Bioverativ) partnership agreement signed in Feb-18

Product development agreement

Factor VIII and Factor IX preclinical programmes for haemophilia A and B

Non-exclusive IP licence

\$5m upfront and potential to receive in excess of \$100m for product development and regulatory & sales related milestones

OXB to receive royalties on sales

### Bioverativ

*John G. Cox, CEO Bioverativ said:*

*“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”*

## Current status and expectations

- In January 2018, Sanofi announced the proposed acquisition of Bioverativ for \$11.6bn. In February 2019 Bioverativ was fully integrated into the Sanofi Group
- Currently in process development stage for haemophilia A & B to allow successful production of material for clinical development
- Sales of products to treat haemophilia in the 7 major markets reached **\$6.7bn** in 2016 and is forecast to reach **\$8.0bn** by 2026<sup>1</sup>

<sup>1</sup> PharmaPoint Haemophilia A and B Global Drug Forecast & Market Analysis to 2026, Published Global Data July 2017

# Deal 2: Axovant licence agreement

## Axovant Gene Therapies deal signed in Jun-18

World wide exclusive licensing agreement for AXO-Lenti-PD (OXB-102)

Unmet need in Parkinson's disease (includes all indications)

Headline value \$842.5m

\$30m upfront and the potential to receive \$55m for specified development milestones, in excess of \$757.5m for specified regulatory & sales related milestones

Receive 7-10% tiered royalties on sales

axovant  
GENE THERAPIES

**Pavan Cheruvu, CEO Axovant said:**

*"Axovant, together with our parent company Roivant, remains committed to developing innovative treatments for serious degenerative conditions such as Parkinson's disease, and we are excited to partner with Oxford BioMedica, a recognised global leader in cell and gene therapy. OXB-102 is a potentially best-in-class gene therapy with the potential to transform Parkinson's disease treatment. This is an area of significant unmet medical need and a major market opportunity. Advancing this high-quality candidate is a key priority for the team at Axovant and we very much look forward to working with Oxford BioMedica."*

## Current status and expectations

- In March 2019, Axovant reported three month data from first cohort in the open-label, dose-escalation portion of the ongoing study. Axovant plans to proceed to the second dose of AXO-Lenti-PD, with the first subject in this second cohort to be dosed in Q2 2019
- Sales of products to treat Parkinson's disease in the 7 major markets reached **\$3.1bn** in 2016 and is forecast to reach **\$8.8bn** by 2026<sup>1</sup>

<sup>1</sup> Parkinson's Disease: Global Forecast and Market . Analysis to 2026, Published Global Data May 2018

# Deal 3: BI/UK CFGTC/Imperial Innovations

## BI/UK CFGTC/Imperial Innovations partnership agreement signed in Aug-18

Process development agreement

Responsible for process & analytical development, scale up of manufacture and generation of material for toxicology studies

Exclusive option & licence agreement

With BI for lentiviral vector technology to manufacture, register and commercialise the lentiviral gene therapy for the treatment of CF

Undisclosed terms



**Dr Clive Wood, Senior Corporate Vice President Discovery Research said:**

*“Through this collaboration, we are joining forces with some of the top talents in this disease space to propel treatment advances forward. Bringing together our existing expertise as a leader for nearly a century in the discovery and development of therapies that have advanced patient care in respiratory diseases with the gene therapy knowledge of our partners, we aim to unlock unprecedented opportunities for patients with this devastating disease, who are desperately waiting for better treatment options”*

## Current status and expectations

- Currently the CF gene therapy product is in pre-clinical development with plans to manufacture material for toxicology studies
- Sales of products to treat Cystic Fibrosis in the 7 major markets reached **\$2.2bn** in 2015 and is forecast to reach **\$8.6bn** by 2025<sup>1</sup>

# Building the Future – Capacity Expansion to 226,000 sqft

Current

Future



## WINDRUSH COURT

State of the art laboratories



## HARROW HOUSE & CHANCERY GATE

FDA and MHRA approved facilities



## YARNTON

FDA & MHRA approved GMP manufacturing facility



## DISCOVERY & INNOVATION FACILITY (2019)

Non GMP research laboratories



## THE FUTURE (2020)

4x cGMP production & 2x filling facilities under construction



Current 110,000 sqft

+

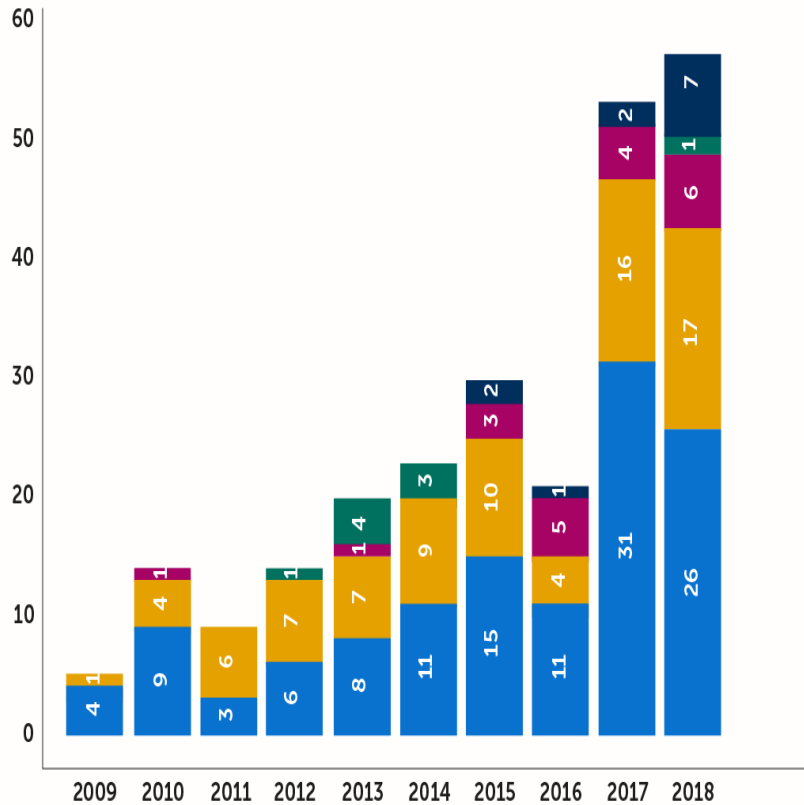
32,000 sqft

84,000 sqft\*

\* Initial phase 45,000 sqft



# Extensive lentiviral vector clinical/pre-clinical trial activity



Initiated lentiviral vector clinical trials by year and phase

Phase

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

Source: Journal of Gene Medicine, December 2018

FDA Commissioner – Scott Gottlieb  
(15 January 2019)



"We anticipate that by 2020 we will be receiving more than 200 INDs [in gene and cell therapy products] per year, building upon our total of more than 800 active cell-based or directly administered gene therapy INDs currently on file with the FDA. **And by 2025, we predict that the FDA will be approving 10 to 20 cell and gene therapy products a year.**"

Selected companies working in the lentiviral vector area

Product	Indication	Pre-Clinical	Phase I	Phase I/II	Phase II	Phase III	Approval
<b>LentiVector<sup>®</sup> platform</b>							
Kymriah <sup>®1</sup>	r/r ALL	[Progress bar: Pre-Clinical to Phase III]					
Kymriah <sup>®1</sup>	r/r DLBCL	[Progress bar: Pre-Clinical to Phase III]					
2nd CAR-T	Cancer (multiple)	[Progress bar: Pre-Clinical to Phase I]					
AXO-Lenti-PD	Parkinson's disease	[Progress bar: Pre-Clinical to Phase I]					
Factor VIII	Haemophilia A	[Progress bar: Pre-Clinical to Phase I]					
Factor IX	Haemophilia B	[Progress bar: Pre-Clinical to Phase I]					
OTL-101	ADA severe combined immunodeficiency	[Progress bar: Pre-Clinical to Phase III]					
OTL-201	MPS-III A	[Progress bar: Pre-Clinical to Phase I]					
Other	undisclosed	[Progress bar: Pre-Clinical to Phase I]					
CFTR gene	Cystic Fibrosis	[Progress bar: Pre-Clinical to Phase I]					

Process development and bioprocessing revenues, and royalties

*In vivo programmes*     *Ex vivo programmes*

<sup>1</sup> USAN name is tisagenlecleucel

Product	Indication	Pre-Clinical	Phase I	Phase I/II	Phase II	Phase III	Approval
<b>OXB Partnered Products</b>							
AXO-Lenti-PD	Parkinson's disease						
SAR422459 <sup>1</sup>	Stargardt disease						
SAR421869 <sup>1</sup>	Usher syndrome 1B						
<div style="display: flex; align-items: center; justify-content: center;"> <p style="color: red; font-weight: bold;">Development milestones and royalties</p> </div>							
<b>OXB Proprietary Unencumbered Products</b>							
OXB-202	Corneal graft rejection						
OXB-302	Cancer, multiple						
OXB-201	Wet AMD						
OXB-204	LCA10						
OXB-208	RP1						
OXB-103	ALS						



*In vivo programmes*    *Ex vivo programmes*

<sup>1</sup> Sanofi are seeking a partner to out-license these assets



## Financial review

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# 2018 Financial Highlights

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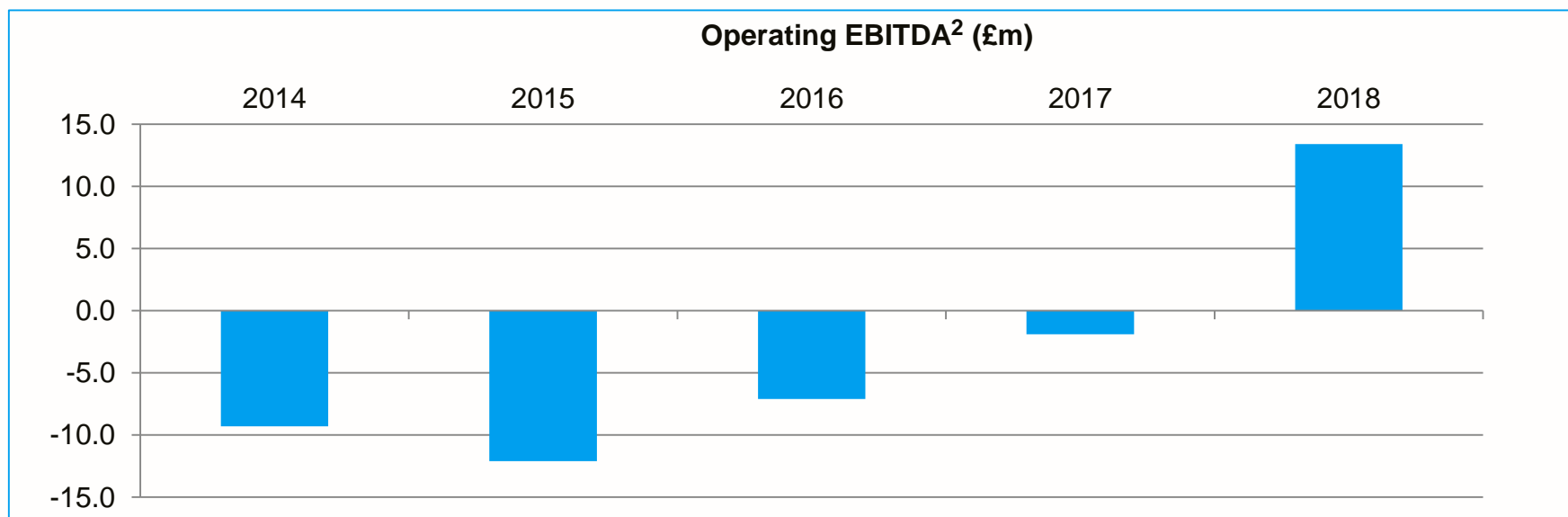
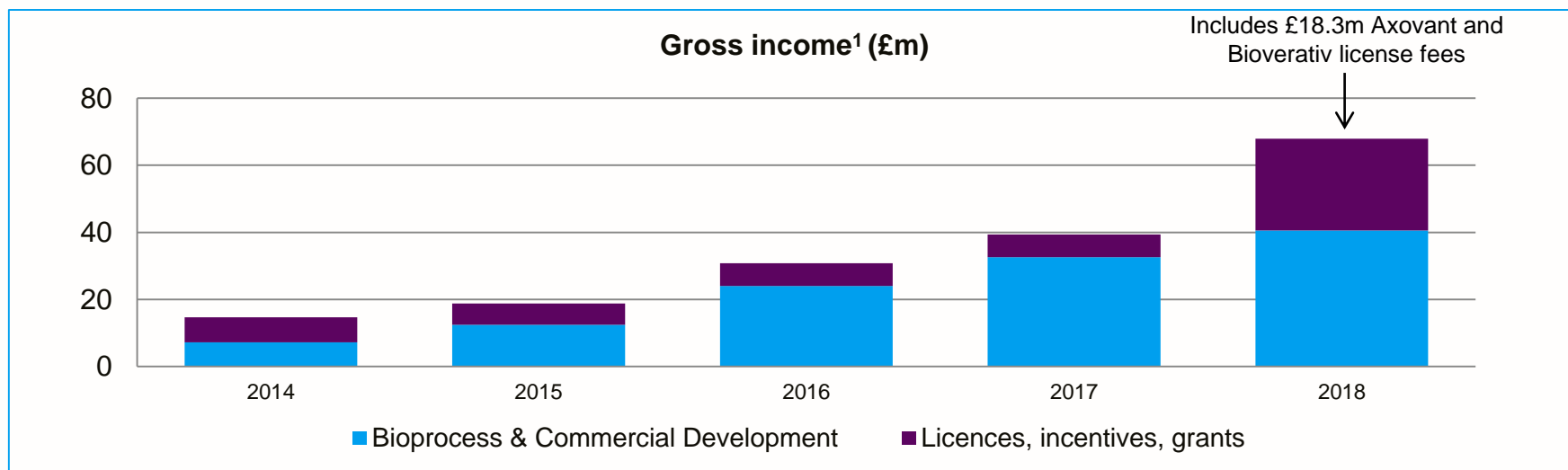
- 72% increase in gross income<sup>1</sup> to £67.9 million (2017: £39.4 million)
- Operating expenses<sup>2</sup> increased by 38% to £31.7 million (2017: £22.9 million)
- Operating EBITDA<sup>3</sup> of £13.4 million (2017: EBITDA loss of £1.9 million)
- Operating profit for the period of £13.9 million (2017: loss of £5.7 million)
- Licence income of £18.3 million (due to Axovant and Bioverativ (Sanofi) deals), segmented by Product (£10.2 million) and Platform (£8.1 million)
- Cash inflow, before financing activities, of £2.8 million (2017: £1.0 million)
- Gross proceeds of £20.5 million raised through a placing to fund the proposed expansion and fit-out of the additional bioprocessing facilities at a new facility in Oxford
- Cash at 31 December 2018 of £32.2 million (31 December 2017: £14.3 million), reflecting significantly improved trading performance and placing proceeds
- £5 million capital expenditure grants received from Innovate UK to support the UK's efforts to produce viral vectors and ensure adequate supply to service expected demand

<sup>1</sup> Gross income = aggregate of revenue and other operating income

<sup>2</sup> Operating expenses = R&D and bioprocessing costs plus admin expenses excluding depreciation, amortisation & share options

<sup>3</sup> Operating EBITDA = Earnings Before Interest, Tax, Depreciation, Amortisation and Share options

# Gross income<sup>1</sup> and EBITDA<sup>2</sup>

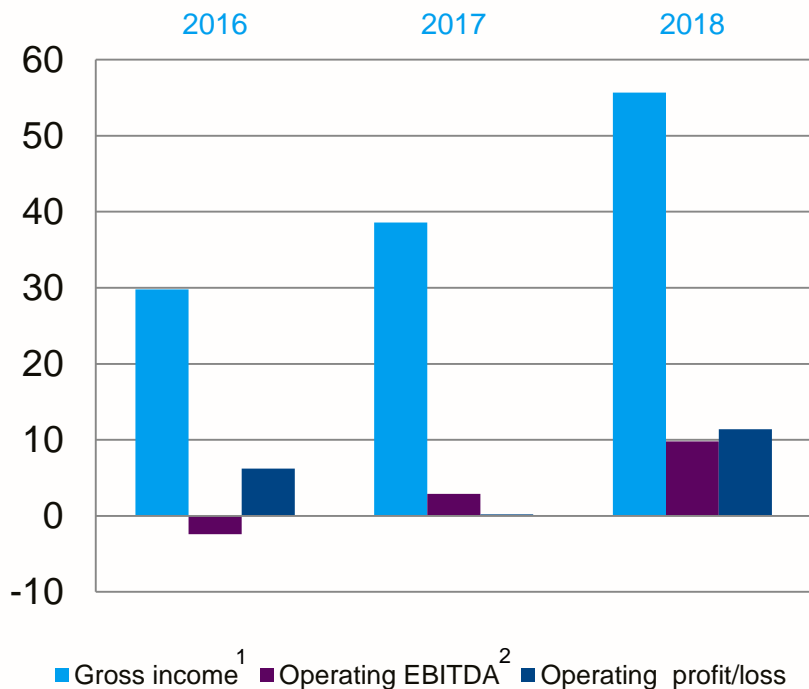


<sup>1</sup> Gross income = Aggregate of revenue and other operating income

<sup>2</sup> Operating EBITDA = Earnings Before Interest, Tax, Depreciation, Amortisation, Revaluation of investments and Share based payments

# Segmental analysis

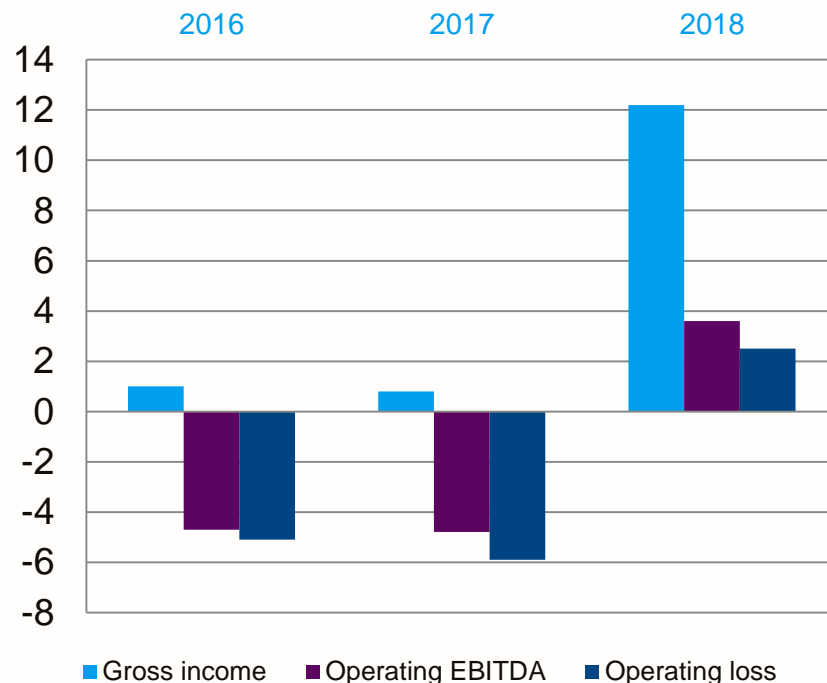
## Platform segment (£m)



### Platform segment

- Gross income received from the bioprocessing and process development activities for third parties. Also includes investing in LentiVector® platform technology
- Generating cash (2018 EBITDA £9.8m & Op Profit £11.4m vs 2017 EBITDA £2.9m & Op profit £0.2m)
- Infrastructure plans in place to support further growth

## Product segment (£m)



### Product segment

- Covers costs of investing in the development of in vivo and ex vivo gene and cell therapy products (discovery, pre-clinical and clinical studies) which are owned by the Group
- Costs include employees and directly related internal costs, external project expenditure, and allocation of Group overheads

<sup>1</sup> Gross income = aggregate of revenue and other operating income

<sup>2</sup> Operating EBITDA = Earnings Before Interest, Tax, Depreciation, Amortisation, Revaluation of investments and Share based payments

# Outlook for 2019

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- Expect gross income to continue to grow driven by:
  - Increasing Novartis bioprocessing and royalty income
  - Multiple revenue generating partners beyond Novartis
  - Expectation of additional new platform partnerships to be added during the year
  - Partnering discussions ongoing to out-license further proprietary pipeline programmes
- Existing infrastructure is near capacity so current investment is essential to maintain leadership, increase productivity and expand facilities to meet customers' long term needs
  - Fit out of 84,000 sqft facility is proceeding on track for occupation by mid 2020 adding in the first phase four additional GMP suites and two fill /finish suites. In addition, the discovery and innovation facility (32,000 sqft) is on track for occupation in Q1 2019
  - Cost growth will be required to support the anticipated revenue growth and investment into the LentiVector® Platform
  - Continued modest investment in pipeline programmes to maintain momentum and enhance value, with board approval to take one pipeline candidate though to Phase I/II.



# Expected newsflow

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## Partners' programmes

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### Novartis

2nd CAR-T programme in development

Royalty stream from Novartis/CTL019<sup>1</sup> increasing in 2019

### Orchard Therapeutics

Intends to file a BLA for ADA-SCID during 2020

### Sanofi (Bioverativ)

Sanofi gene therapy product for haemophilia A & B progressing towards clinical development material in the next 12 months

## LentiVector<sup>®</sup> delivery platform

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Further contracts with new and existing partners giving us long-term economic interest in partners' product candidates expected in the next 12 months

Invest in further development of platform to improve the volume and yield from bioprocessing and efficacy of vector transduction of target cells during 2019

## In-house products

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Advancement of AXO-Lenti-PD (formerly OXB-102) for Parkinson's disease into second cohort expected in Q2 2019

Spin out / out-license of at least one in-house product candidate during 2019

<sup>1</sup> USAN name is tisagenlecleucel



# Appendix

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# Consolidated statement of comprehensive income

	Group	
	2018 Total £'000	2017 Total £'000
<b>Continuing operations</b>		
Revenue	66,778	37,590
Cost of sales	(22,763)	(18,442)
<b>Gross profit</b>	<b>44,015</b>	<b>19,148</b>
Research, development and bioprocessing costs	(29,714)	(21,611)
Administrative expenses	(7,433)	(7,276)
Other operating income	1,064	1,774
Revaluation of investments	5,983	2,297
<b>Operating profit/(loss)</b>	<b>13,915</b>	<b>(5,668)</b>
Finance income	71	38
Finance costs	(8,972)	(6,131)
<b>Profit/(loss) before tax</b>	<b>5,014</b>	<b>(11,761)</b>
Taxation	2,527	2,744
<b>Profit/(loss) and total comprehensive income/(expense) for the year</b>	<b>7,541</b>	<b>(9,017)</b>
<b>Basic earnings/(loss) per ordinary share</b>	<b>11.57p</b>	<b>(14.5p)</b>
<b>Diluted earnings/(loss) per ordinary share</b>	<b>10.94p</b>	<b>(14.5p)</b>

# Balance sheet

	Group	
	2018	2017
	£'000	£'000
<b>Assets</b>		
<b>Non-current assets</b>		
Intangible assets	117	97
Property, plant and equipment	31,791	25,370
Investments	10,966	2,954
	<b>42,874</b>	<b>28,421</b>
<b>Current assets</b>		
Inventories	4,251	3,332
Trade and other receivables	30,585	17,088
Current tax assets	2,446	2,232
Cash and cash equivalents	32,244	14,329
	<b>69,526</b>	<b>36,981</b>
<b>Current liabilities</b>		
Trade and other payables	11,422	8,690
Contract liabilities	9,242	13,072
	<b>20,664</b>	<b>21,762</b>
<b>Net current assets</b>	<b>48,862</b>	<b>15,219</b>
<b>Non-current liabilities</b>		
Loans	41,153	36,864
Provisions	1,287	630
Contract liabilities	14,276	-
Deferred tax liability	279	-
	<b>56,995</b>	<b>37,494</b>
<b>Net assets</b>	<b>34,741</b>	<b>6,146</b>
<b>Equity attributable to owners of the parent</b>		
Ordinary share capital	33,034	31,076
Share premium account	172,074	154,224
Other reserves	3,509	3,509
Accumulated losses	(173,876)	(182,663)
<b>Total equity</b>	<b>34,741</b>	<b>6,146</b>

# Statement of cash flows

	Group	
	2018 £'000	2017 £'000
<b>Cash flows from operating activities</b>		
Cash generated from / (used in) operations	(9, 214)	(1,533)
Tax credit received	3,654	4,530
Overseas tax paid	-	(18)
Net cash generated from / (used in) operating activities	<b>12,868</b>	2,979
<b>Cash flows from investing activities</b>		
Purchases of property, plant and equipment	(10,103)	(1,969)
Purchases of intangible assets	(45)	-
Interest received	52	38
Net cash used in investing activities	<b>(10, 096)</b>	(1,931)
<b>Cash flows from financing activities</b>		
Proceeds from issue of ordinary share capital	21,184	385
Costs of share issues	(1,376)	-
Interest paid	(4,665)	(10,800)
Loans received	-	38,897
Loans repaid	-	(30,536)
Net cash generated from / (used in) financing activities	<b>15,143</b>	(2,054)
<b>Net increase / (decrease) in cash and cash equivalents</b>	<b>17,915</b>	(1,006)
Cash and cash equivalents at 1 January	<b>14,329</b>	15,335
<b>Cash and cash equivalents at 31 December</b>	<b>32,244</b>	14,329



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