



Final Results 2020

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Highlights for the year



Strong progress towards sustainable portfolio;
financial performance impacted by Autolus
share price

Significant strengthening of the capital pool with proceeds from the sales of Blue Earth and Nightstar

Capital pool of £767m

- £593m of proceeds generated by sale of Nightstar and Blue Earth - aggregate 6.6x capital invested*
- £206m of capital deployed into the portfolio; one new portfolio company

Strong operational and clinical progress across the portfolio

Nine active clinical trials and teams strengthened

- Including one pivotal study in AUTO1
- Three clinical trials commenced
- 10 senior leaders appointed across portfolio

£1.2bn NAV - 186p per share; (13.3%) total return

Performance impacted by fall in Autolus share price, which has appreciated 131% since year end (£95.5 million Syncona valuation increase)**

- Focused on long-term performance and Autolus' strong fundamentals

Portfolio companies progressing well; strength of balance sheet an increasing competitive advantage

Our differentiated platform

Founding, Building and
Funding a portfolio global
leaders

**Expert
team**

Appointments of Danny Bar Zohar as
Partner and Lorenz Mayr as Entrepreneur
in Residence strengthening the senior
team

10 senior leadership appointments to
portfolio company management teams

Sustainable,
diverse portfolio of
leading healthcare
companies

**Strategic
capital base**

£767m of capital available to support
our portfolio companies scale

Strategic value of capital
significantly increased in the current
environment

**Exceptional
science**

UK research base is globally differentiated: one
new portfolio company and in advanced stages of
foundation of a new Syncona company

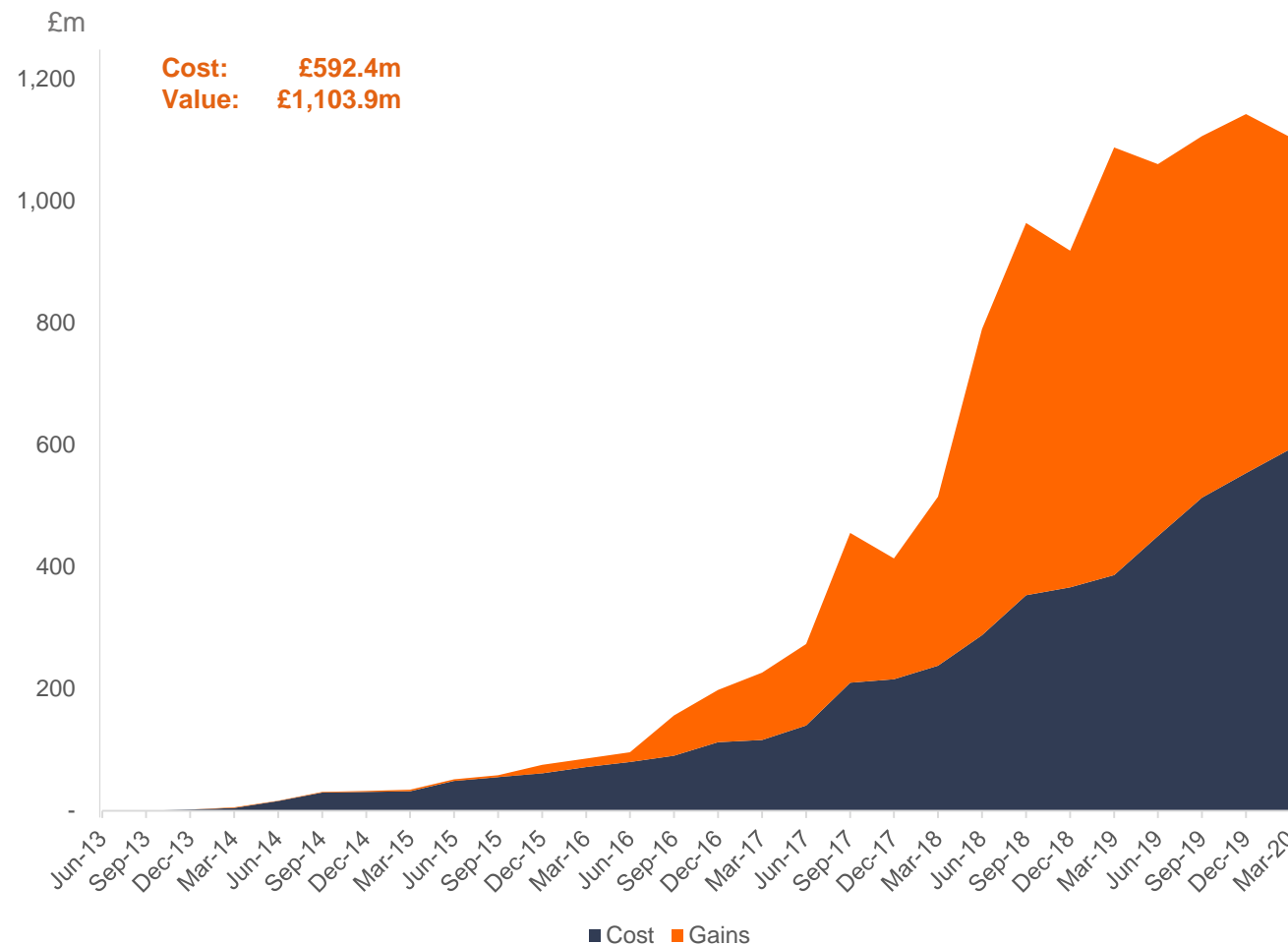
Our approach has delivered significant long term value

Strong track record; IRR of 39% - 1.9x cost generated on Syncona portfolio since 2012

Strong risk adjusted returns

- £592.4m capital deployed since 2012
- 13 Syncona portfolio companies founded
- Two companies sold:
 - Nightstar sold to Biogen for \$877m in 2019; 4.5x return (IRR 72%)
 - Blue Earth sold to Bracco Imaging for \$476m in 2019; 10x return (IRR 87%)
- Remaining life science portfolio valued at £479.5m
 - 1.0x capital invested

Figures reflect Syncona Partners original investment pre merger with BACIT



COVID-19 update

Vision to develop treatments for patients remains of profound importance

Limited impact to business continuity

- Took immediate measures to protect team and minimise disruption
- Expanded team despite remote working environment
- Continue to take a proactive approach to sourcing new opportunities
- Leveraged core expertise to provide support to The Wellcome Trust and the UK Government
- Annual donation to charities brought forward to June

Portfolio companies supported to navigate disruption

- Conducted a bottom up analysis across portfolio (cash requirements, milestone delivery)
- Varying impacts on clinical trials; working closely with companies where delays identified
- More limited impact in oncology setting, where the need for treatments is more acute
- Companies continue to generate data where patients have been treated

Strong capital pool; companies well positioned to manage through disruption

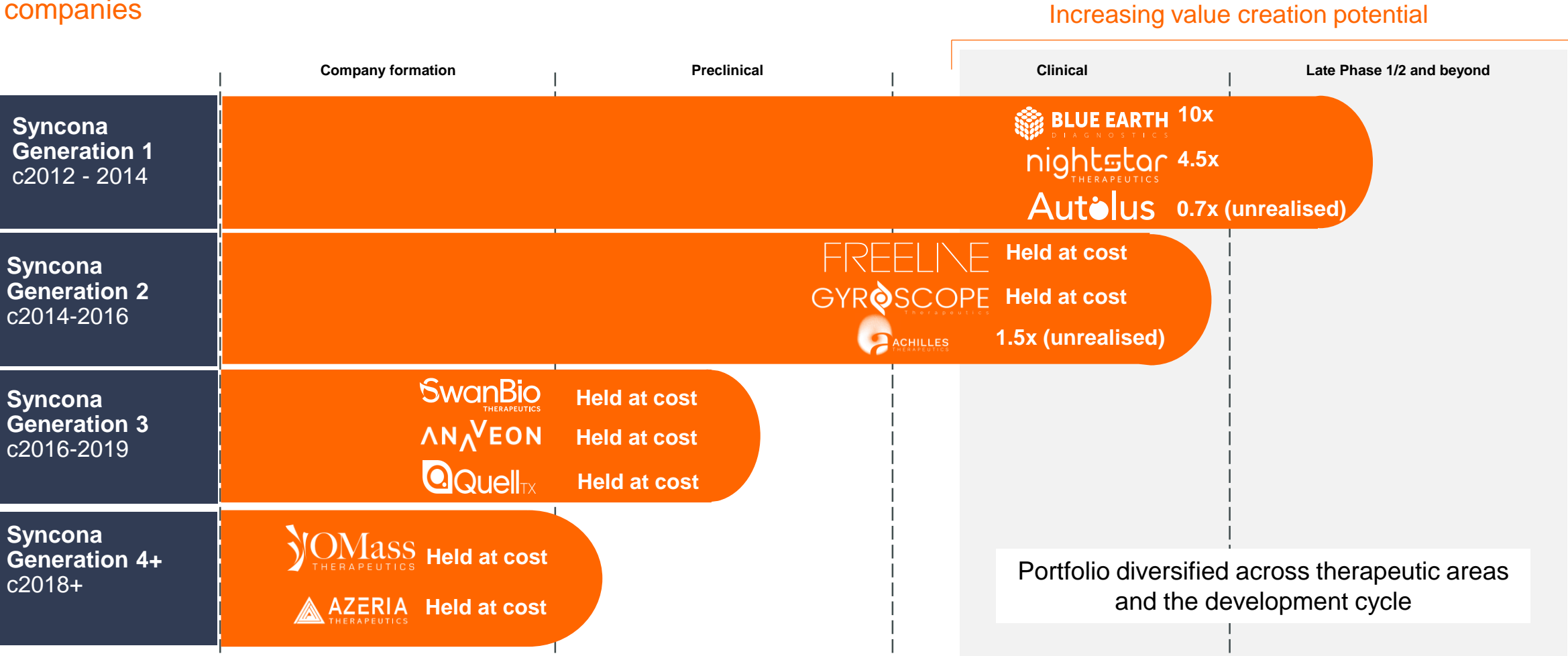
Portfolio update

Chris Hollowood, CIO

Significant value creation opportunity



Strong clinical progress during the year at Autolus and Generation 2 companies



Lead programme moving to pivotal and positive data in AUTO3

High level of clinical activity in end-stage patients

Value: £77.0m

Cell therapy, 27% ownership

Clinical progress:

- AUTO1 data shows high level of clinical activity in end stage cancer patients, good safety profile and potential for durable responses
- AUTO1 programme has progressed to a pivotal study – IND and CTA approval
- Released positive data in AUTO3 DLBCL programme – favourable safety profile potentially enable for use in outpatient setting; out patient cohort initiated in Q2 CY2020
- AUTO4 potentially delayed by COVID-19 disruption by one quarter, however pre-clinical data expected for T cell lymphoma and solid tumour programs at AACR covering AUTO5, AUTO6NG and AUTO7

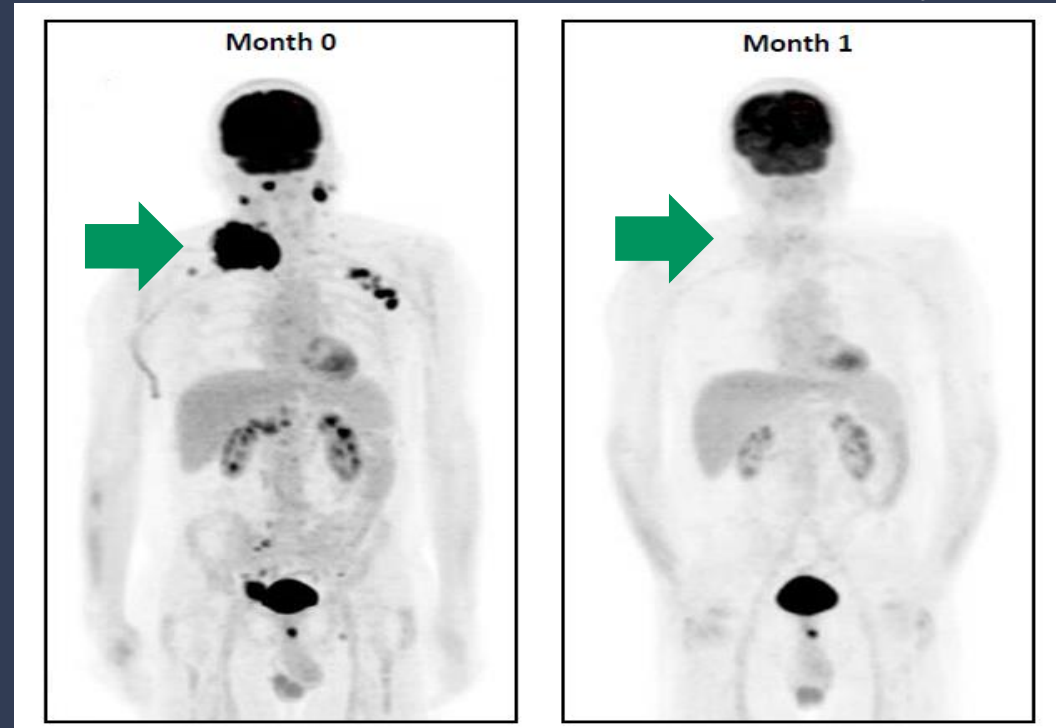
COVID-19 update: based on current expectations we anticipate the impact on most operations will be minimal

Autolus

Complete Responses Seen in bulky tumors with good safety profile

Pre AUTO3

Post AUTO3 Day 28



Encouraging data in lead programme

Differentiated opportunity to target broad pipeline of systemic disorders

Value: £150.7m

Gene therapy, 79% ownership

Operational progress






- CEO and CMO appointed
- Continued to build out manufacturing; commercial scale

Clinical progress:

- Lead programme in haemophilia B seeking to deliver FIX activity in the normal range (50-150%)
- Six patients* have completed follow-up for at least 6 months
 - amongst them, three have FIX activity levels over 50%
- Highly encouraging data; potential for best-in-class product for patients
- Business seeking to identify optimal dose to move to a pivotal study
- Reported data in its second clinical programme in Fabry's disease, showed that gene therapy can deliver sustained levels of the required enzyme

FREELINE

Clinical pipeline leveraging the same proprietary platform


Programme	Research	IND enabling studies	Phase 1/2	Next Milestone	Patient No (US & EU5)**
Haemophilia B FLT180a				Dose Selection	9,000
Fabry FLT190 and FLT191				Results from dose escalation	9,000
Gaucher FLT200 and FLT201				CTA/IND	6,000
Haemophilia A FLT210				CTA/IND	38,000
Undisclosed inflammatory disorders				Candidate Selection	50,000 – 200,000

COVID-19 update: experienced delays across clinical programmes, expects to be able to publish further data from Haemophilia B lead trial this year and dose the next patient in its Fabry trial this financial year

Gyroscope: ongoing operational and clinical progress



Targeting the treatment of dry AMD by using gene therapy to restore balance to the complement system



Value: £73.0m
Gene therapy
Ownership: 80%

Operational progress

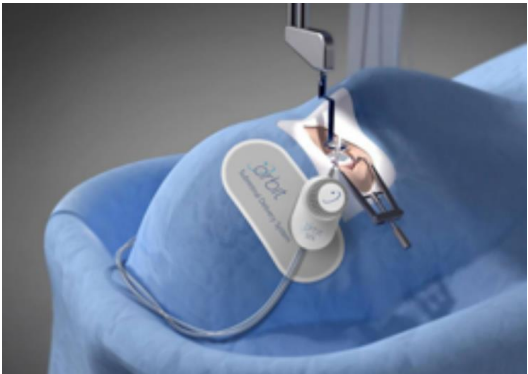
- Appointment of Nadia Waheed as CMO and Jane Hughes as CSO
- Continued to build out manufacturing; commercial scale

Clinical progress

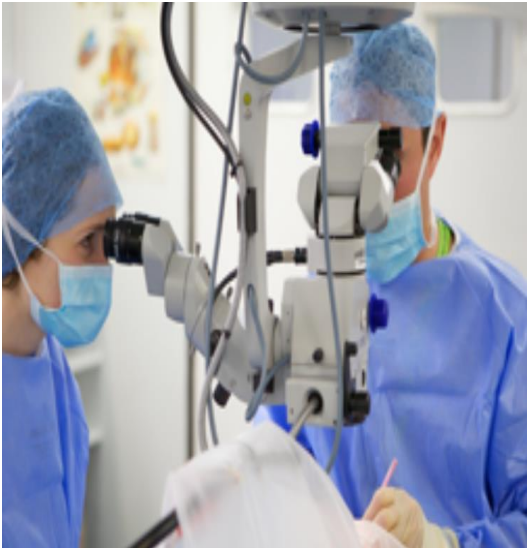
- Ongoing dose escalation in phase I/II trial for treatment of dry AMD
- No safety issues seen to date

COVID-19 update:

Delays to lead programme, closely monitoring with lead programme targeting elderly population; however expect to report initial data from phase I/II trial and commence phase II trial this financial year



The device shown is not approved for human use



Candidate	Indication	Status			
		Research	Target ID	Pre-clinical	Clinical
GT005	Geographic Atrophy (defined sub-set)				
GT005/7	Geographic Atrophy (broad population)				
GT005/7	Other inflammatory retinal disease				

Achilles: strong progress with first patient dosing



Developing tumour infiltrating lymphocyte therapies designed to target clonal neoantigens (present on all tumour cells)



Value: £72.4m
Cell therapy
Ownership: 44%

Operational progress:

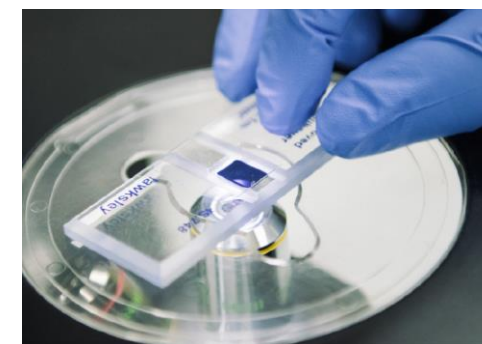
- Appointment of CSO, Sergio Quezada
- Appointment of exceptional Scientific Advisory Board
- Carsten Boess, 30 years financial experience, appointed to Board
- Continued to build out manufacturing; commercial scale

Clinical progress:

- Enrolling patients in its phase I/II trials in NSCLC and melanoma
- Post period end, dosed first patient in melanoma study
- Expects to report first patient dosed in NSCLC in the near future
- Expect to report initial data from both trials in H1 CY2021

COVID-19 update:






Navigating impact well and currently still able to dose patients



Disease	Status		
	Pre-clinical	Phase 1/2	Pivotal
Metastatic/recurrent melanoma			
Advanced non-small cell lung cancer			
Other indications			

Strong progress across preclinical companies

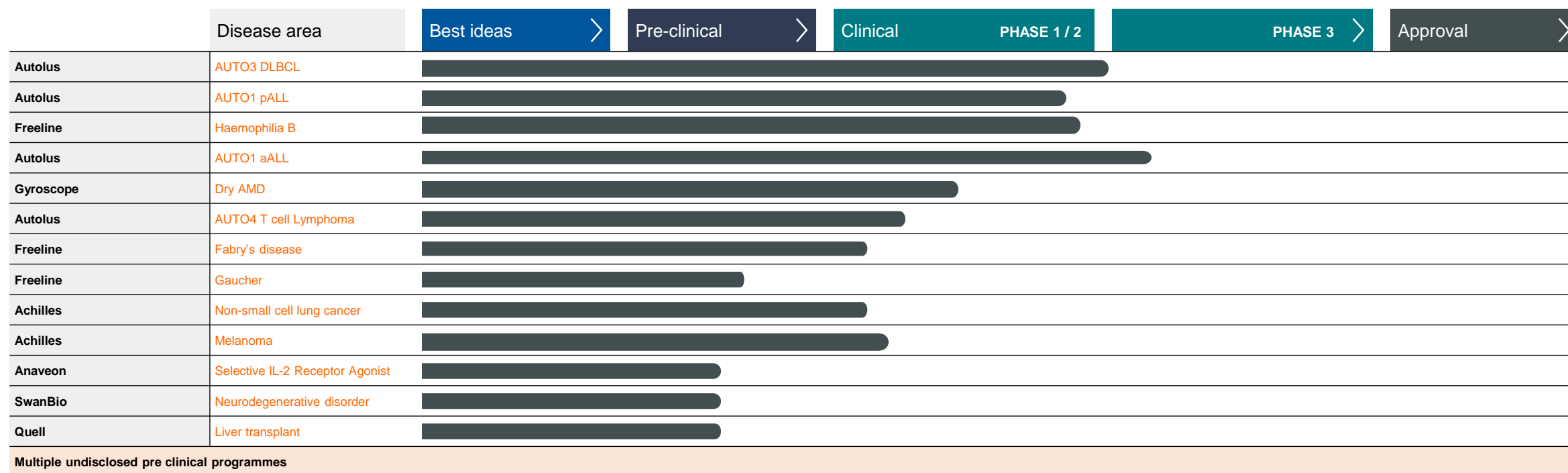
Building out management teams and manufacturing capabilities; making strides towards the clinic

Company	Focus	Value	Progress	Clinical progress
 SwanBio THERAPEUTICS	Gene therapy	£18.5m	<ul style="list-style-type: none"> – Team build out – Continuing to develop a scalable manufacturing process for commercial supply 	<ul style="list-style-type: none"> – Pre-clinical development continues with lead programme – Developing pipeline indications
 OMass THERAPEUTICS	Small molecule	£14.6m	<ul style="list-style-type: none"> – Continue to recruit senior leadership team 	<ul style="list-style-type: none"> – Progressing a pipeline of small molecule therapeutics, including its lead programme into pre-clinical development
 ANVEON	Biologics	£12.3m	<ul style="list-style-type: none"> – Leadership team build out – Expanding operations 	<ul style="list-style-type: none"> – Clinical candidate nomination
 QuellTX	Cell therapy	£8.3m	<ul style="list-style-type: none"> – Appointment of CEO – Team and manufacturing build out 	<ul style="list-style-type: none"> – Clinical candidate nomination in lead programme in liver transplant (post period end)
 AZERIA THERAPEUTICS	Small molecule	£6.5m	<ul style="list-style-type: none"> – Focused on pre-clinical development of lead programme 	<ul style="list-style-type: none"> – Generating pre-clinical data to test the core technical premise behind our investment

Strong clinical progress



Significant clinical progress with next generation companies benefiting from increased capital and Syncona platform



Data key driver of value; number of near term catalysts in the portfolio

Founding new companies

Martin Murphy, CEO

What do we look for in a scientific asset?

Technology

Globally leading academics

Intellectual Property



Transformational efficacy for patients in areas of high unmet need



Defined, commercial lead programme with pipeline potential



Opportunity to develop differentiated platform or no incumbent



Therapeutic areas where Syncona has deep domain expertise



Defined patient segments / targeted markets

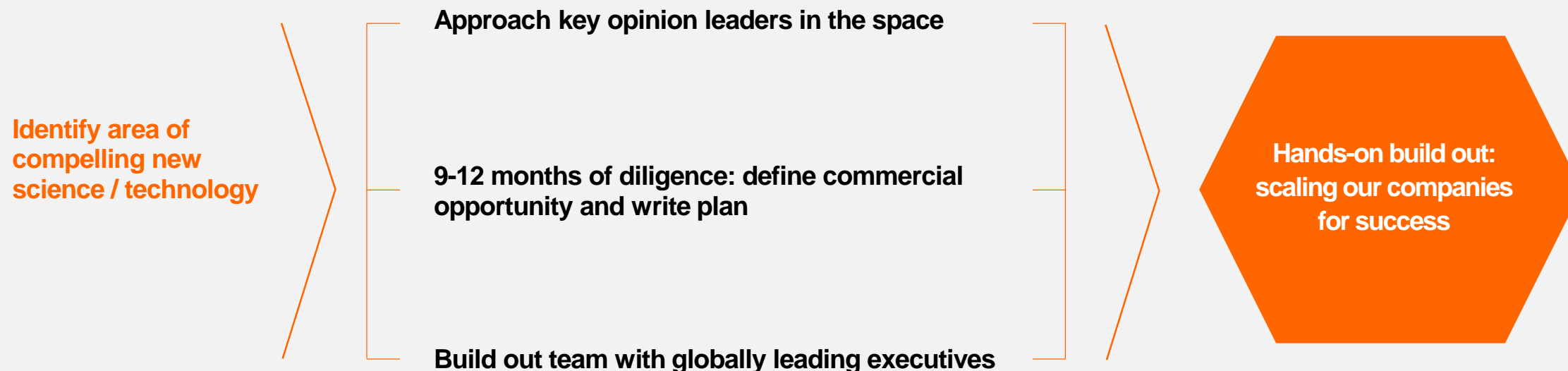


Accelerated development and regulatory pathways

Our approach to company creation and development

Translating technology to products to reach full value potential

Our partnership approach provides a strategic premium



Building a macrophage cell therapy company



Potential new Syncona company in area of deep domain expertise

Source	2018	Sourced through Wellcome Trust network		Engaged world leading KOL, Prof Stuart Forbes, at the University of Edinburgh		Developed research plan to de-risk technology
Syncona Collaborations 2018-2020	£1.4m	Research Collaboration with the University of Edinburgh in 2018		Syncona team identified key technical milestones required to underpin commercial viability of macrophage cell therapy		Key research milestones delivered; 9-12 months of diligence to write plan and structure investment

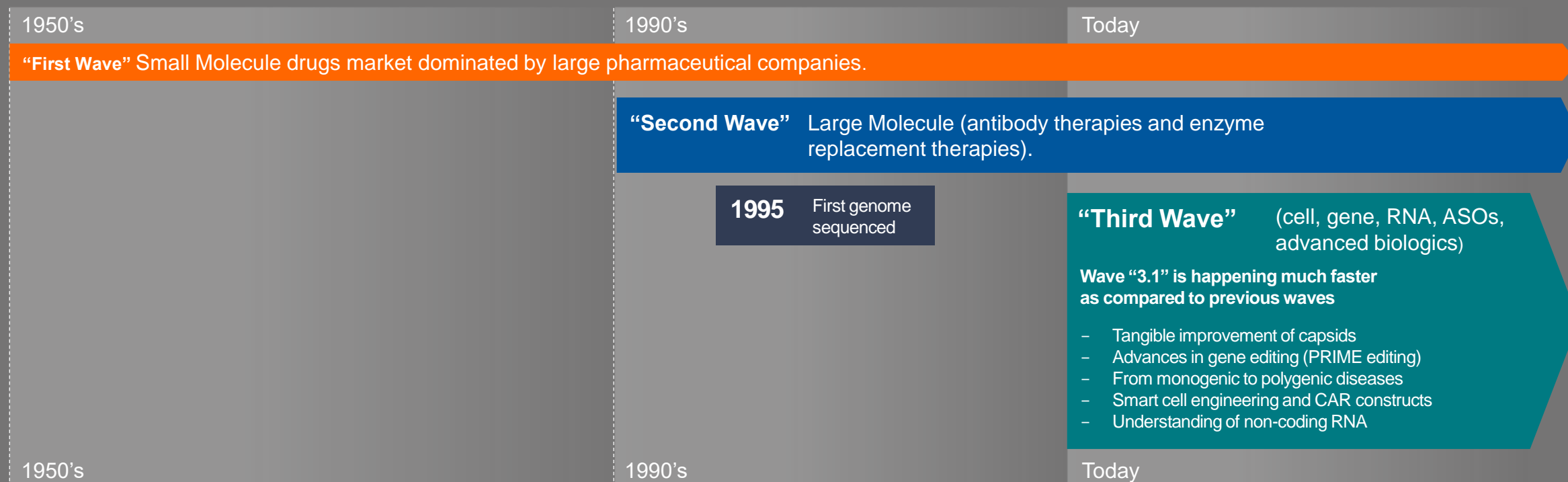
Opportunity to found first engineered macrophage cell therapy company

Market environment

Danny Bar-Zohar, Partner

The next frontier of innovation

A new era: small biotechs capable of developing and commercialising breakthrough therapies



Forces we should take into account:

- Pricing and access
- R&D attrition rates
- Cost to Go/No-Go decision points

Our response:

- Leverage biomedical innovation to secure *transformative patient outcomes*
- Use *high quality data and advanced analytics* in select cases of discovery, diagnosis and development
- Go deeper into science; enhance *segmentation* of patient populations and use carefully selected *surrogate markers* to *shorten timelines and bring medicines that matter, faster*

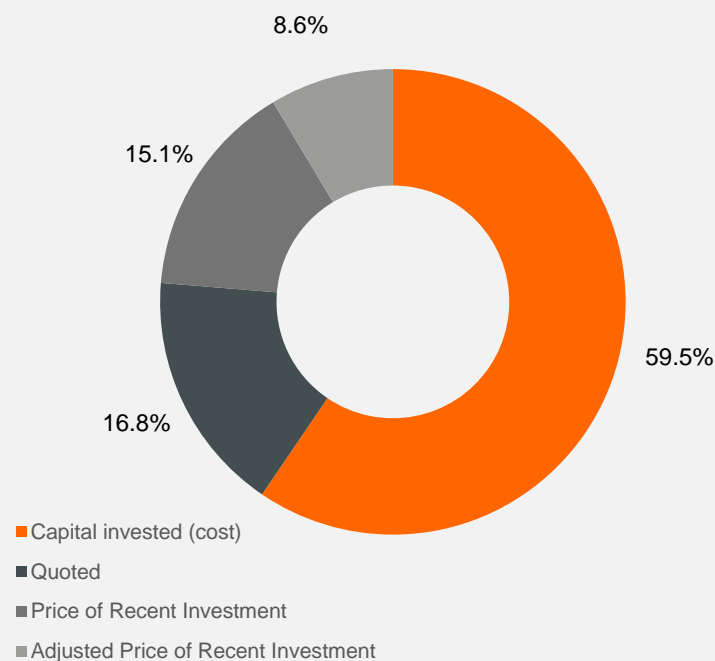
Financial review

John Bradshaw, CFO

Financial review

NAV £1,246.5m (185.6p per share) – decline in the year driven by fall in Autolus share price

Life science portfolio valuation



Life sciences portfolio of £479.5m; a return of (25.0%)

- Aggregate £91.2m* uplift from the sale of Blue Earth and financing in Achilles outweighed by £280.9m decline in Autolus share price

Significantly strengthened capital pool of £767.0m

- Sales of Blue Earth and Nightstar generated £592.6m of proceeds
- Managed with a focus on liquidity and capital preservation
- In March 2020, moved quickly to liquidate the fixed income products, preserving liquidity and protecting the capital pool from volatile market conditions
- 90% held in cash and short-term UK Treasury bills at year end

Rigorous approach to recognising increases in value: for 59.5 per cent of the life science portfolio, primary input to fair value is capital invested (cost)

- Clinical trial delays across the portfolio resulting from the COVID-19 pandemic are not currently expected to have any impact on valuations of privately held companies

Significant capital commitment in the year: scaling our portfolio

Portfolio is well funded and strongly positioned for long-term success



– £206.4 million of investment in the year into existing portfolio companies and to new company Azeria Therapeutics

– Autolus and Generation 2 companies scaling through the clinic, delivering on milestones and requiring significant capital to progress

Financing strategy

Deep pool of capital
underpins our strategy



Core to delivery of strategy

Provides flexibility and control to
take a long-term view

Ability to maintain large Syncona
ownership stakes

Certainty of funding key to delivering
strategy; seek to maintain 2-3 years
funding runway



Our approach

Long-term approach providing capital at scale

Disciplined approach; dependent on specifics
of company, scale of the opportunity, risk,
capital requirement and the size of Syncona's
balance sheet

Option to bring in like-minded partners to
diversify risk and enable companies to
capitalise on their ambitions



Capital deployment

Conducted a bottom up analysis across
portfolio, looking at the implications for
cash requirements and milestone delivery
as a result of COVID-19

Well positioned to fund our portfolio
as it delivers key milestones

Anticipate capital deployment to be
£150-250m, depending on whether our
portfolio can access third party capital
(where appropriate)

Outlook and summary

Martin Murphy, CEO

Portfolio company outlook



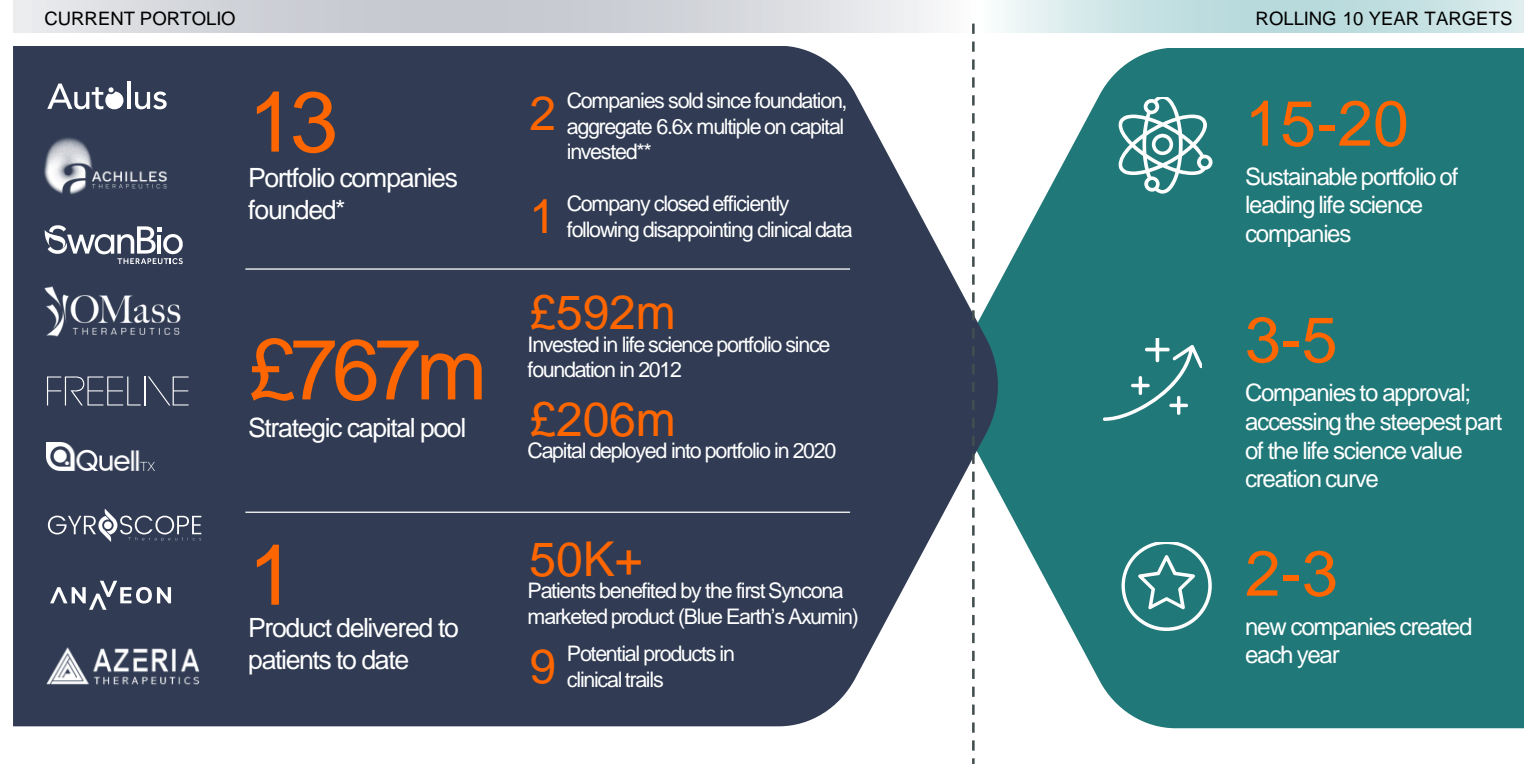
Portfolio well positioned with catalysts ahead

Company	Status of pipeline	Next catalysts
Autolus	Four programmes in clinical trials	<ul style="list-style-type: none"> – Decision regarding move to Phase II in AUTO3 DLBCL Q3 CY2020 – Initial data in Phase I AUTO4 programme
FREELINE	Two lead programmes in Phase I/II clinical trials, pipeline of preclinical programmes	<ul style="list-style-type: none"> – Publish further data in its lead programme in haemophilia B FY2021 – Dose its next patient in its second programme in Fabry's FY2021
GYROSCOPE	Lead programme in Phase I/II clinical trial	<ul style="list-style-type: none"> – Initial data from its lead phase I/II trial targeting dry AMD FY2021 – Commence phase II trial in dry AMD FY2021
ACHILLES THERAPEUTICS	Enrolling patients in Phase I/II clinical trial	<ul style="list-style-type: none"> – Dose the first patient in its Phase I/II study in NSCLC in the near future – Report initial data in H1 CY2021 from its melanoma and NSCLC studies
SwanBio THERAPEUTICS	Lead programme in pre clinical development	<ul style="list-style-type: none"> – Complete first clinical manufacturing batch in this financial year – Expand leadership team
OMass THERAPEUTICS	Seeking to build pipeline of therapeutics	<ul style="list-style-type: none"> – Initiation of pre-clinical development of lead programme
ANVEON	Nominated clinical candidate in lead programme	<ul style="list-style-type: none"> – Initiation of phase I/II clinical trial FY2022
QuellTX	Nominated clinical candidate in lead programme	<ul style="list-style-type: none"> – Initiation of phase I/II clinical trial FY2022
AZERIA THERAPEUTICS	Pre-clinical development of lead programme	<ul style="list-style-type: none"> – Further pre-clinical data generated to test technical thesis

Summary

Syncona platform creates value from the commercialisation of life science innovation

- Clinical stage companies in a strong position to deliver key milestones in the year ahead
- Excellent progress towards our goal of building a sustainable portfolio of 15-20 companies
- Significant opportunity ahead for Syncona to continue to capitalise on globally differentiated research base in UK/EU
- Strong capital pool provides a strategic advantage; well positioned to navigate current environment
- Strong ongoing support for Syncona Foundation; increased annual donation to 0.35% of NAV



• *Includes sales of Blue Earth and Nightstar, closure of 14MG and merger of Orbit and Gyroscopic
• **Sales of Nightstar and Blue Earth, original Syncona Partners capital invested

09:30: Live Q&A session

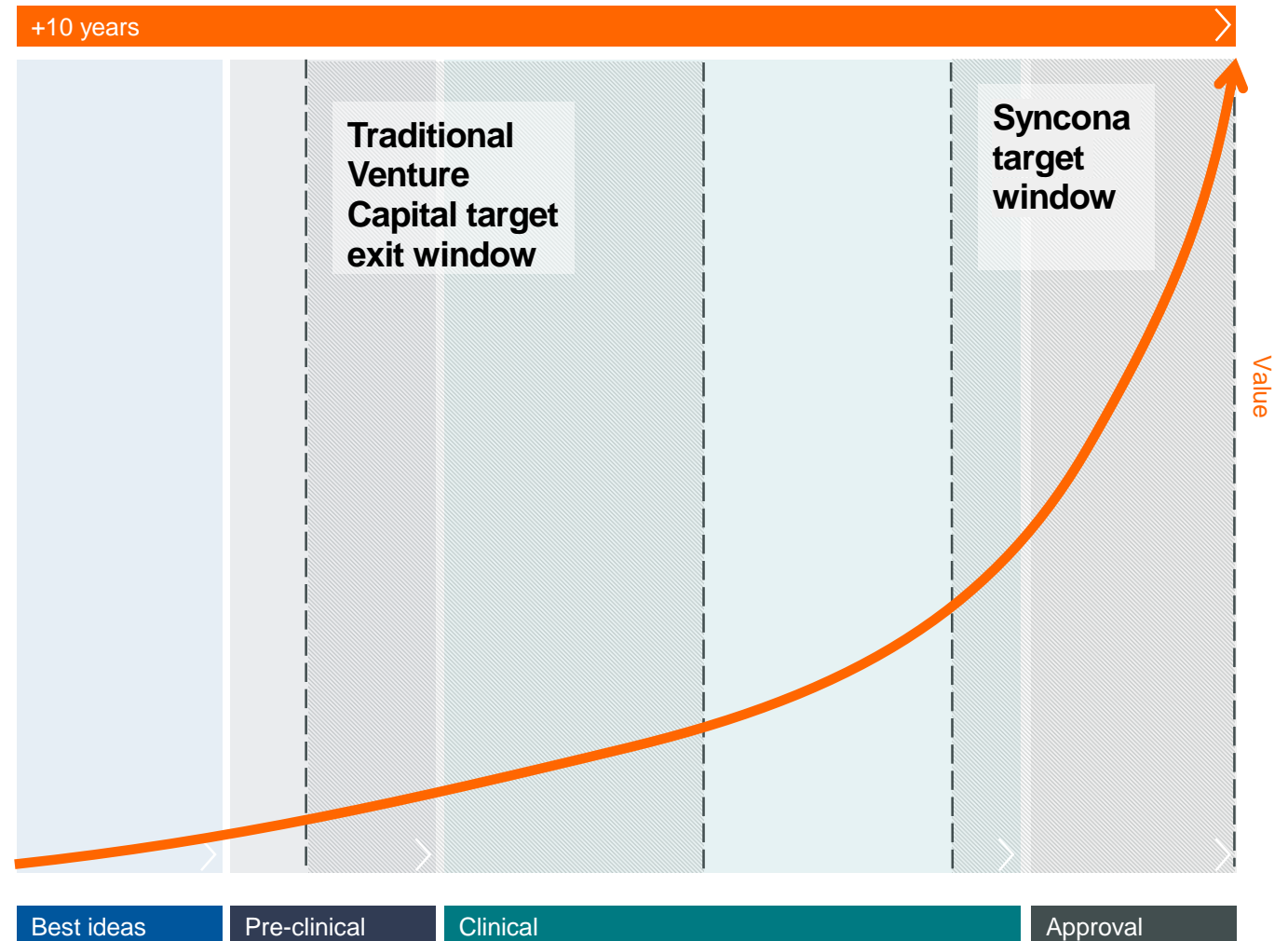
Please register to listen using the link below

Capturing the out return in life science

Strategy designed to deliver strong risk adjusted returns for shareholders

Out return in life science weighted towards late development and product approval:

- Set companies up with the ambition of taking products to market
- Target the steepest part of the valuation curve



Graph is illustrative and assumes successful clinical development and approval, Syncona team view

Managing risk and reward while executing the strategy

Optimising risk-adjusted returns

Not all companies will remain solely owned

We will syndicate financing rounds; dependent on specifics of company, scale of the opportunity, risk, capital requirement and the size of Syncona's balance sheet

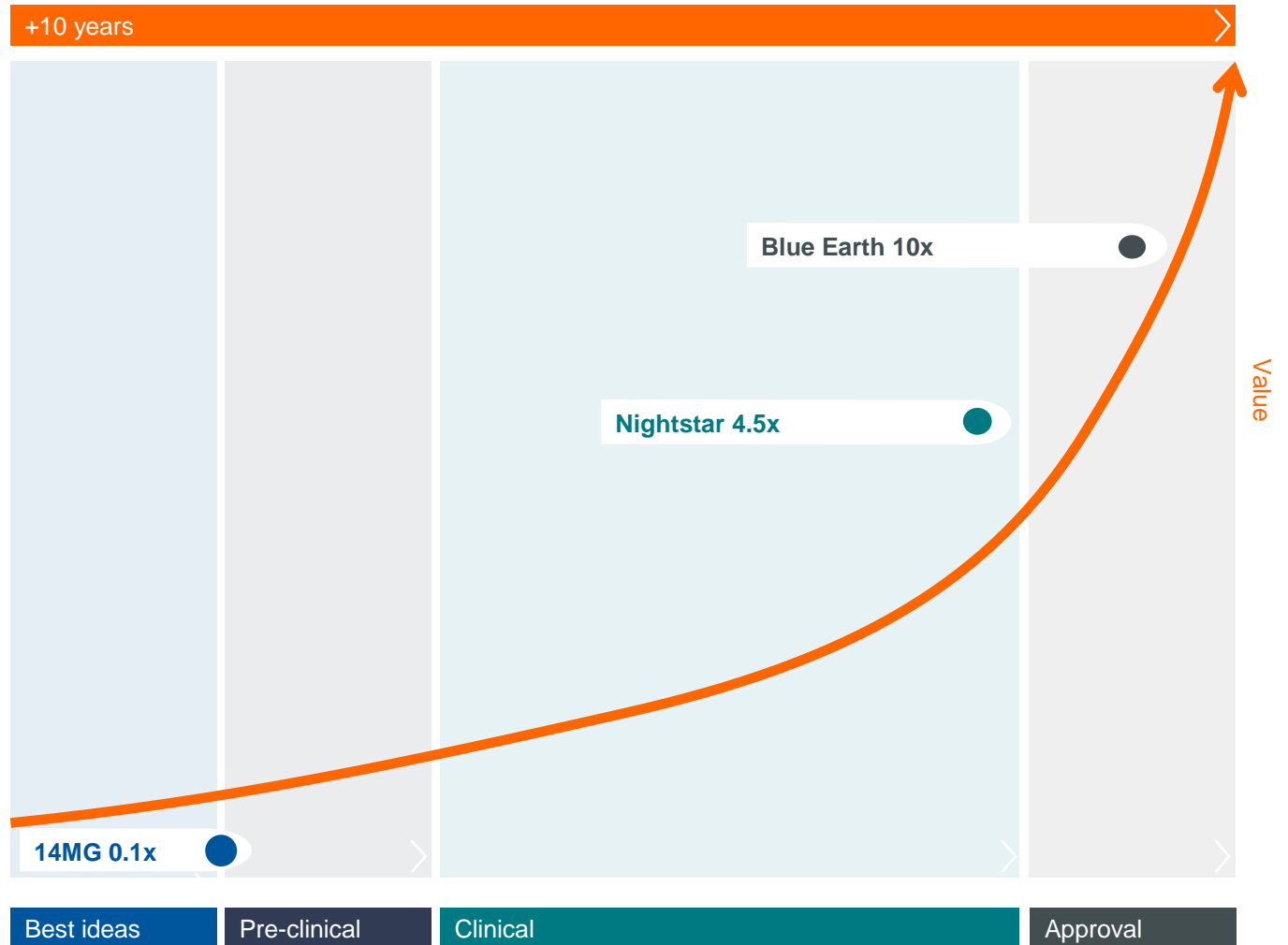
We will sell companies when it makes sense

Driven by the balance of risk and reward – clear view on risk adjusted value of a company at any point in time, permits effective evaluation of opportunities

Some companies won't succeed

When issues arise we aim to take action as quickly as possible.

Portfolio of 15-20 companies supports the delivery of 10 year targets



Graph is illustrative, Syncona team view

Financial review

NAV of £1,246.5m
(185.6p); capital pool of £767.0m

- Realised
- Clinical stage
- Pre-clinical stage
- Drug discovery



Portfolio company	Ownership* %	31 March 2019 value £m	Net invested/ returned the period £m	Valuation change in period £m	FX movement £m	31 March 2020 value £m (Fair value)	Valuation basis (Fair value)**	% of NAV
BLUE EARTH Realised	-	267.5	(336.8)	69.3		-	Sale Price	-
nightstar Realised	-	255.8	(255.8)	-		-	Sale price	-
Autolus	27	328.2	29.7	(284.7)	3.8	77.0	Quoted	6.2
FREELINE	79	93.5	55.6	-	1.6	150.7	Cost	12.1
GYROSCOPE	80	28.9	44.1	-	-	73.0	Cost	5.9
ACHILLES	44	16.2	32.8	23.4	-	72.4	Recent financing (within 0-6 months)	5.8
SwanBio	79	5.3	12.9		0.3	18.5	Cost	1.5
ANAVEON	51	3.7	8.0		0.6	12.3	Cost	1.0
QuellTX	69	8.3	-	-	-	8.3	Cost	0.7
AZERIA	60	-	6.5	-	-	6.5	Cost	0.5
OMass	49	3.5	11.1	-	-	14.6	Cost	1.2
Syncona Investments		44.5	5.7	(4.2)	0.2	46.2		3.6
Total		1,055.4	(386.2)	(196.2)	6.5	479.5		38.5

*Percentage holdings reflect Syncona's ownership stake at the point full current commitments are invested

**Cost indicates that the fair value has been determined to be equal to the total funding invested by Syncona

Significant opportunity across lead programmes

Potential to deliver multiple approved products which will cornerstone the creation of leading life science companies

Company & investment thesis	Lead programme / disease population p.a	Opportunity in and differentiation of lead programme	Key comparators ²	Key risks ¹
Autolus Applying a broad range of technologies to build a pipeline of precisely targeted T cell therapies designed to better recognise and attack cancer cells	AUTO1 ALLCAR19 Phase 1/2 in Adult Acute Lymphoblastic Leukaemia 3k ^{3*}	<ul style="list-style-type: none"> Unmet medical need: only 30-40% of patients with Adult ALL achieve long term remission with combination chemotherapy, the current standard of care⁴ No CAR-T therapy approved for adult ALL for patients AUTO1 targets a differentiated safety profile (reduce high grade CRS⁵) and improved persistence to address limitations of current T cell therapies 	<ul style="list-style-type: none"> CAR-T active programmes in clinical development for ALL include Gilead⁷ 	<ul style="list-style-type: none"> Differentiated product required Complex manufacturing
Freeline Seeking to deliver constant high protein expression levels with curative potential across a broad pipeline of systemic diseases; opportunity to deliver curative gene therapies	B-AMAZE Phase 1/2 in Haemophilia B 9k ^{8**}	<ul style="list-style-type: none"> Unmet medical need: current standard of care, Enzyme Replacement Therapy (infusions of FIX into the blood), requires regular administration and FIX activity does not remain stable Opportunity to deliver a single dose cure for patients by achieving FIX levels in the 'normal' range in the blood of 50-150% Utilising a novel, proprietary capsid and industrialised proprietary manufacturing platform 	<ul style="list-style-type: none"> Active clinical programmes in gene therapy for Haem B include: Spark/Pfizer⁹, UniQure¹⁰ 	<ul style="list-style-type: none"> Highly competitive environment Differentiated product required Manufacturing
Gyroscope A novel company developing gene therapy beyond rare disease by understanding the immune system and the role genetics play in a patient's risk of developing late stage AMD.	FOCUS Phase 1/2 in Dry Age-Related Macular Degeneration 2m ^{11**}	<ul style="list-style-type: none"> Unmet medical need: age related macular degeneration is one of the leading causes of permanent vision impairment for people aged 65 and older with no approved treatments¹². Research suggests that when a part of the immune system, the complement system, is overactive it leads to inflammation that can damage healthy eye tissues Gene therapy may stimulate a patient's cells to produce the proteins needed to restore balance to the complement system Developing a subretinal delivery system to safely, precisely and consistently deliver therapies into the eye and help scale the surgical procedure for larger patient populations. 	<ul style="list-style-type: none"> No directly competitive gene therapy approach targeting complement system Apellis¹³, Gemini¹⁴, Hemera¹⁵ 	<ul style="list-style-type: none"> Highly innovative concept which is currently unsupported by a significant existing data set
Achilles Differentiated cell therapy approach targeting solid tumours utilising Tumour Infiltrating Lymphocytes & clonal neoantigens to develop personalised treatments	Phase 1/2 Non small cell lung cancer 234k ^{16*}	<ul style="list-style-type: none"> Unmet medical need: lung cancer, of which NSCLC accounts for approximately 85%¹⁷, with limited treatment options and is the leading cause of cancer deaths¹⁸. TILs have shown convincing efficacy in solid tumours¹⁹ Achilles' world leading bioinformatics platform, PELEUS™ is built on exclusive access to world largest study of tumour evolution in lung cancer (TRACERx) Achilles process uses the patient's own genomic information to create a truly personalised medicine targeting the clonal neoantigens 	<ul style="list-style-type: none"> Key competitors in the neoantigen/ personalised immunotherapy space include: Iovance²⁰, Neon Therapeutics²¹, Gritstone Oncology²² 	<ul style="list-style-type: none"> Highly innovative concept in an emerging space Significant manufacturing challenge Increasing competition

Significant opportunity in earlier stage portfolio

Potential to deliver multiple approved products delivering transformational treatment for patients

Company	Investment thesis	Key comparators ²	Key risks ¹
SwanBio Gene therapy focused on neurological disorders where there is existing proof of concept	<ul style="list-style-type: none"> Unmet medical need: one of the most common monogenic neurological disorders, with no available therapies for severely debilitating progressive movement disorder Gene therapy has the potential to be transformational in neurology²³ One-off delivery mechanism and hundreds of single gene disorders First programme in preclinical development for an inherited neurodegenerative disease in which the causative gene is definitively known and well characterized 	Several clinical trials for gene therapy within CNS field, including programmes within Voyager ²⁴ , Uniqure ²⁵ , Prevail Therapeutics ²⁶ and PassageBio ²⁷	<ul style="list-style-type: none"> Manufacturing and delivery challenges in the CNS (substantial dose required) Clinical endpoints can be challenging to define
Quell Engineered cell therapy company addressing immune dysregulation	<ul style="list-style-type: none"> Unmet medical need: current standard of care for prevention of solid organ transplant rejection is life-long immunosuppression which results in an array of serious long-term side effects (e.g. renal function, malignancy, infection, cardiovascular disease) materially impacting patient quality of life and long-term survival²⁸ Novel cell therapy approach using T-regulatory cells with a suppressive action to downregulate the immune system to treat conditions including solid organ transplant rejection, autoimmune and inflammatory diseases Potential pipeline to treat serious, chronic conditions mediated by the immune system; in the autoimmune setting alone, there are >70 chronic disorders estimated to affect over 4% of the population²⁹ Pre-clinical stage: first programme to address solid organ transplant 	T Reg field is nascent; TX Cell/Sangamo ³⁰	<ul style="list-style-type: none"> Highly innovative concept, limited clinical data supporting application of CAR-T technology in Treg cells
Anaveon Immuno-oncology company developing a selective IL-2 Receptor Agonist	<ul style="list-style-type: none"> Unmet medical need: Human Interleukin 2 "IL-2" approved as a medicine for the treatment of metastatic melanoma and renal cancer, but with a frequent administration schedule and significant toxicity³¹ Preclinical stage, developing a selective Interleukin 2 ("IL-2) Receptor Agonist with improved administration and tox burden Wide potential utility across multiple oncology indications in large markets³² 	Companies developing products in the IL-2 field include: Nektar ³³ , Roche ³⁴ , Alkermes ³⁵ , Synthorx ³⁶ .	<ul style="list-style-type: none"> Highly competitive Technical risk around product
OMASS Drug Discovery platform with differentiated technology	<ul style="list-style-type: none"> Opportunity to build a drug discovery platform employing a differentiated Modified Mass Spectrometry technology with the potential to yield high quality chemical hits to discover novel small molecule drug therapeutics for a variety of complex targets, including membrane receptors 	N/A	<ul style="list-style-type: none"> Pre clinical and clinical attrition of potential drugs
Azeria Pioneer factor drug discovery company developing treatments for hormone resistant breast cancer	<ul style="list-style-type: none"> Significant unmet patient need in oestrogen receptor positive breast cancer where c.30% of patients progress to late stage endocrine resistant disease Scientific insights by Azeria's academic founder have led to a new approach to target an essential pioneer factor pivotal in tumour growth, progression and maintenance of oestrogen receptor positive luminal breast cancer 	Companies developing therapies for oestrogen receptor positive luminal breast cancer include Eisai and AstraZeneca	<ul style="list-style-type: none"> Highly innovative concept in emerging space

An expert multi-disciplinary team

Our unique skill set



Scientific



Commercial



Company creation



Investment



A life sciences team with a track record of creating value in the life science sector

Martin Murphy
CEO



Chris Hollowood
CIO



John Bradshaw
CFO



Danny Bar Zohar
Partner



Lorenz Mayr
Entrepreneur
in Residence



Elisa Petris
Partner



Edward Hodgkin
Partner



Dominic Schmidt
Partner



Magda Jonikas
Partner



Alex Hamilton
Partner



Freddie Dear
Partner



Michael Kyriakides
Partner



Alice Renard
Partner



Gonzalo Garcia
Partner



Hitesh Thakrar
Partner



An inflection point for Third Wave therapies



Syncona has established a leadership position in a new wave of technologies

“First Wave”

1950's

Small Molecule drugs, market dominated by large pharmaceutical companies.

01

“Second Wave”

1990's

Large Molecule (antibody therapies enzyme replacement therapies).

02

The “Third Wave”

Today

Advanced Biologics and genetic medicines in areas such as gene therapy, cell therapy and DNA sequencing.

03

Top Ten Drugs*

	2006	2016	2026
Small molecules	8	2	?
Second wave	2	8	?
Third wave	0	0	?

10,000**

Number of monogenetic disorders, less than 100 with treatments today

80%

of rare diseases are of genetic origins

9

'Third Wave' therapies approved in the US

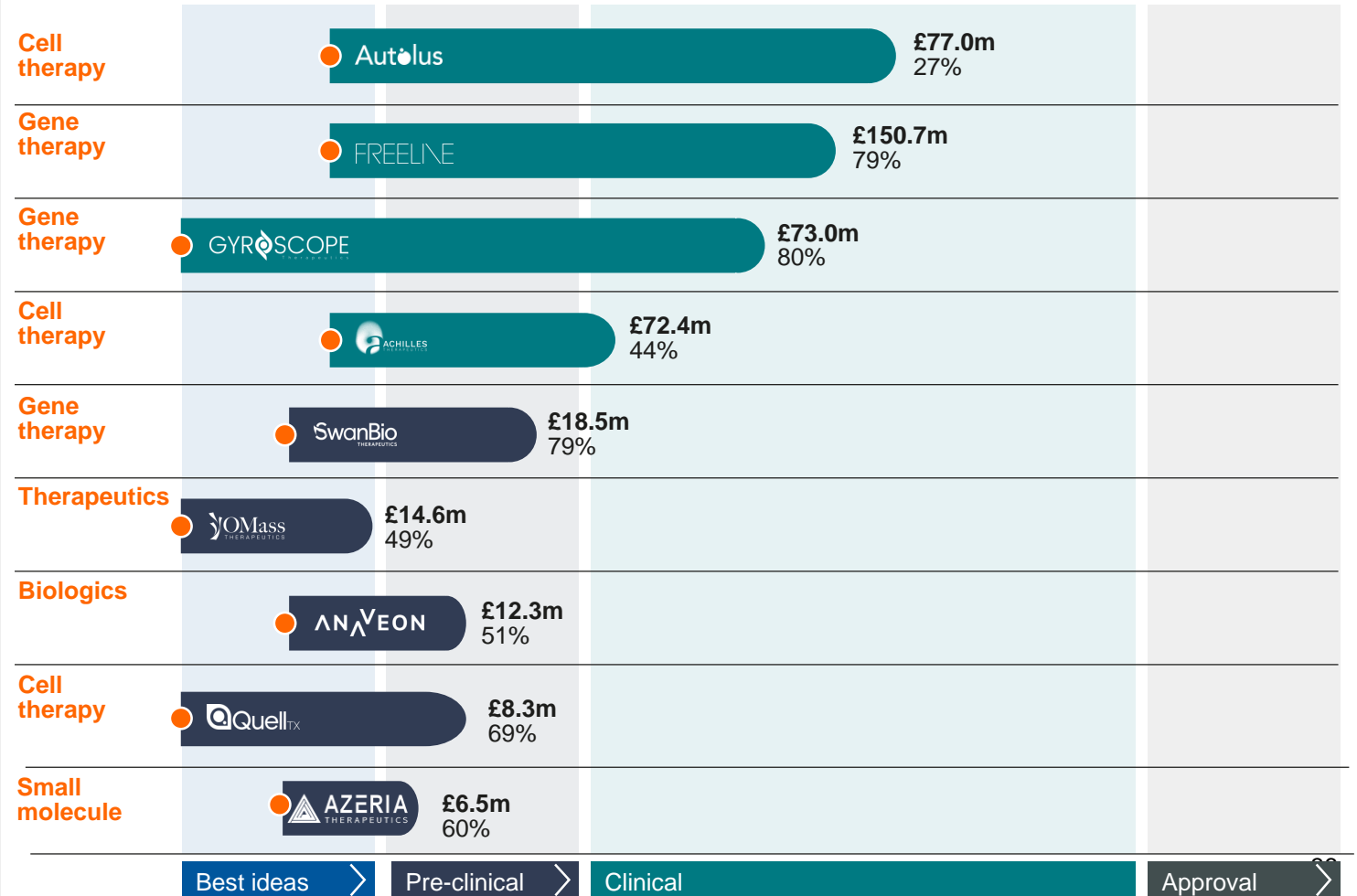
27%***

Predicted growth for Third Wave companies average CAGR sales per annum between 2018 and 2021

A differentiated and focused portfolio

Companies in specialist and innovative areas of healthcare across the development cycle

- Syncona investment point
- Clinical stage company
- Preclinical stage company
- Drug discovery company



Founding, Building and Funding NightStar

Origination, commercial vision, and operation



Nov 2012
First meeting with Robert MacLaren



Jan 2014
Syncona founds the company with Series A financing of \$12m; Syncona CIO, Chris Hollowood is appointed Chairman

Jan 2015
David Fellows appointed as Chief Executive

Syncona approach Oxford to licence further programs from Robert's group

Mar 2017
Syncona identify Stargardt's as an attractive program

Jul 2017
Series C financing of \$45m; Syncona invests \$12.5m

Mar 2017
Receives RMAT designation in Choroideremia

Sep 2017
Announces positive proof-of-concept data in XLRP

Follow-on financing of \$83m with Syncona investing in \$18m

Sep 2012
Identification of retinal gene therapy as a core area of interest where a Company can get built

Mar 2013
Initial discussions on terms with Oxford

Mar 2014
David Fellows appointed non-executive director

Nov 2015
Series B financing of \$35m; Syncona invests \$10m

Nov 2017
NITE licence Stargardt program from Oxford

Sep 2017
\$76m listing on NASDAQ; Syncona invests \$14m

Mar 2018
Initiates Pivotal trial in Choroideremia

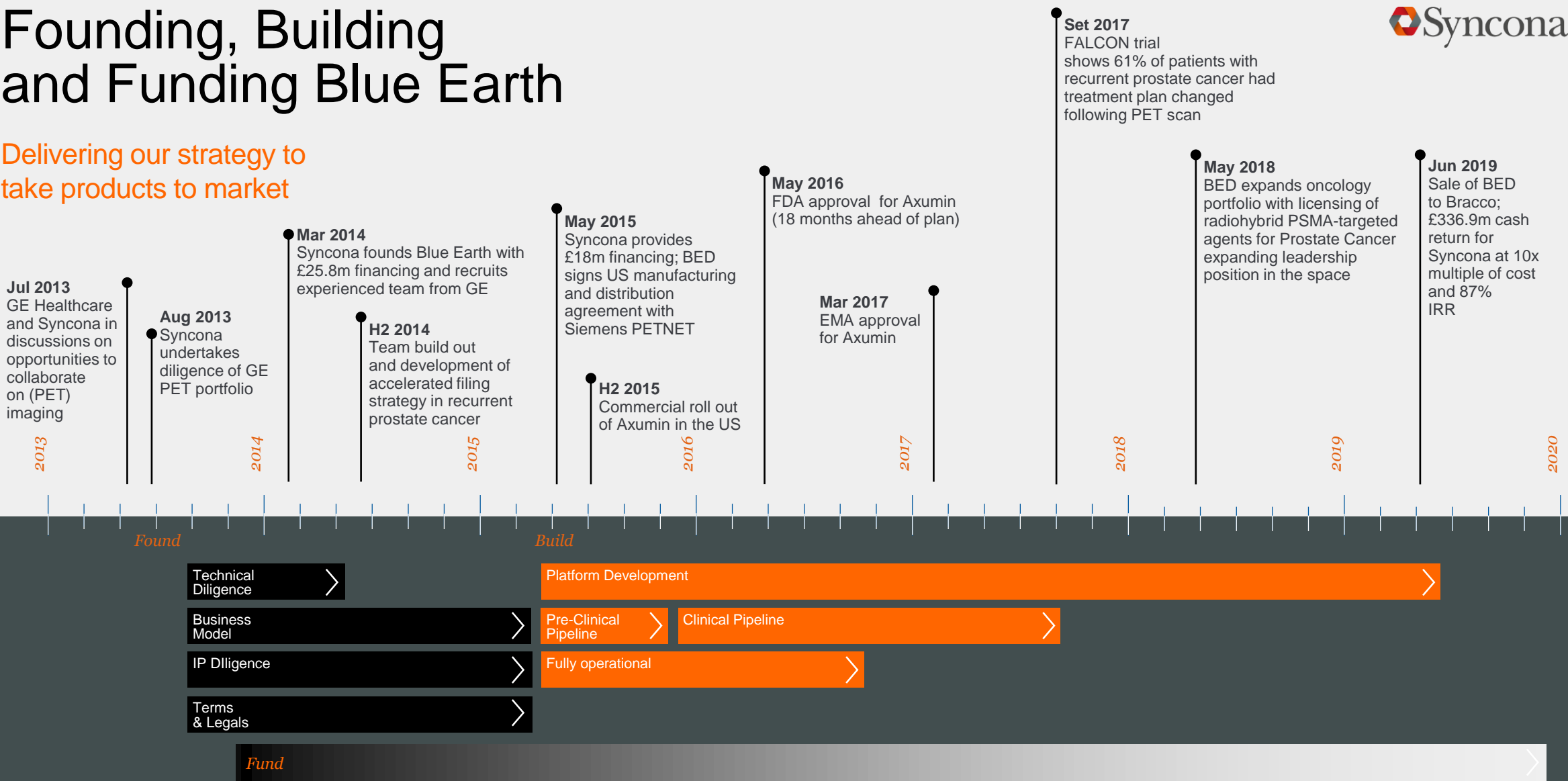
Mar 2019
Agreement to be acquired by Biogen for \$877m

Nov 2018
Planned initiation of Phase II/III study in XLRP

Founding, Building and Funding Blue Earth



Delivering our strategy to take products to market



1. Syncona investment team analysis of key risks facing the companies; the companies are subject to other known and unknown risks, uncertainties and other factors
2. Syncona investment team analysis of lead programmes in this area, indicative only
3. Source: Autolus – see Autolus corporate presentation November 2019 <https://autolus.gcs-web.com/static-files/cd8dc1d9-6a7b-496d-933f-1a3b0bfb56a>. Autolus project the addressable population at 3,000 patients US & EU5
4. Source: Autolus – see Autolus corporate presentation November 2019 <https://autolus.gcs-web.com/static-files/cd8dc1d9-6a7b-496d-933f-1a3b0bfb56a>
5. Cytokine Release Syndrome
6. Source: Autolus – see Autolus corporate presentation November 2019 <https://autolus.gcs-web.com/static-files/cd8dc1d9-6a7b-496d-933f-1a3b0bfb56a>
7. <https://www.gilead.com/science-and-medicine/pipeline>
8. Source: Freeline analysis of prevalence in US and EU5. Analysis is based on World Federation of Haemophilia Global Annual Survey 2017 <http://www1.wfh.org/publications/files/pdf-1714.pdf> and National Haemophilia Foundation; CDC.
9. <https://sparktx.com/scientific-platform-programs/>
10. <http://www.uniqure.com/gene-therapy/hemophilia.php>
11. Source: Gyroscopic estimate. Age related macular degeneration, of which one type is dry AMD, is estimated to affect 195.6 million people globally (<https://www.who.int/publications-detail/world-report-on-vision>). Gyroscopic's estimate is that there is a population of 2 million people in the US & EU5 with geographic atrophy, which is late stage dry AMD.
12. Source: WHO <https://www.who.int/blindness/causes/priority/en/index7.html>
13. <https://www.apellis.com/focus-pipeline.html>
14. <https://www.geminitherapeutics.com/approach-progress/>
15. <https://www.hemerabiosciences.com/clinical-trials/>
16. Source: Achilles calculation of US and UK prevalence. There are 275,000 new cases of lung cancer in US and UK each year, of which 85% are estimated to be NSCLC. US: 228,150 <https://seer.cancer.gov/statfacts/html/lungb.html>; UK: 47,235 <https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/lung-cancer/incidence>.
17. Source: American Cancer Society <https://www.cancer.org/cancer/small-cell-lung-cancer/about/key-statistics.html>
18. Source: American Cancer Society <https://www.cancer.org/cancer/lung-cancer/about/key-statistics.html>
19. Source: Rosenberg et al 2011 <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3131487/pdf/nihms286994.pdf>
20. <https://www.iovance.com/clinical/pipeline/>
21. <https://neontherapeutics.com/product-pipeline/>
22. <https://griststoneoncology.com/our-pipeline/>
23. See for example existing approved product Zolgensma for spinal muscular atrophy – <https://www.zolgensma.com/>
24. <https://www.voyagertherapeutics.com/our-approach-programs/gene-therapy/>
25. <http://uniqure.com/gene-therapy/huntingtons-disease.php>
26. <https://www.prevailtherapeutics.com/>
27. Source: <https://www.passagebio.com/company/about-passage-bio/default.aspx>
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30. <https://investor.sangamo.com/news-releases/news-release-details/sangamo-and-txcell-announce-completion-acquisition-sangamo>
31. Source: <https://www.cancernetwork.com/renal-cell-carcinoma/managing-toxicities-high-dose-interleukin-2>
32. Source: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC4938354/>
33. <https://www.nektar.com/pipeline/rd-pipeline/nktr-214>
34. https://www.roche.com/research_and_development/who_we_are_how_we_work/pipeline.htm: RG7835
35. <https://investor.alkermes.com/news-releases/news-release-details/alkermes-announces-clinical-collaboration-fred-hutchinson-cancer>
36. <https://synthorx.com/therapeutics/>