

# Interim results 2020

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Image Freeline labs, Stevenage

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# Highlights



#### **Strong clinical progress**

- 10 active clinical trials with three new programmes initiated
- Data read-outs from Autolus and Freeline
- Three clinical trials commenced (Achilles and Gyroscope)

### Significant financial and operational milestones

- Freeline raised \$299m in a Series C and IPO
- Generation 2 and 3 companies developing key manufacturing capabilities
- Nine senior leaders appointed across portfolio, including Franz Humer (ex Chair of Roche) and Sean Bohen (ex CMO of Astrazeneca)

# New companies founded in areas of deep domain expertise

- New macrophage cell therapy company, Resolution, founded with £26.8m Series A commitment
- New \$19.0m investment in Neogene, a T-cell receptor company in Series A financing
- Purespring, one of the first AAV gene therapy companies targeting the kidney founded (post period) with £45.0m Series A commitment

# 9.6% increase in NAV to £1.4bn, 203.4p per share

- Driven by 24.8% return from life science portfolio
- Performance driven by the increase in Autolus' share price and the write up of Freeline
- Closure of Azeria; delivery of data did not support our thesis (£4.5m written off)
- Capital pool of £700.1m; £68.9m deployed in the six months

### Performance

# Clinical



# Portfolio update

#### **Private** Value: £72.4m 44% ownership

Differentiated cell therapy approach targeting solid tumours utilising Tumour Infiltrating Lymphocytes and clonal neoantigens to develop personalised treatments				
<ul> <li>Dosed first patients in phase I/II trials in melanoma and NSCLC</li> <li>Appointed Karl Peggs as CMO</li> <li>Raised £52.7m in Series C post period end</li> </ul>				
Clinical pipeline	Research   Target ID   Pre- Clinical   Clinical			
Melanoma				
Non-cell lung cancer				

Strong clinical and financial progress Clinical trials across the portfolio are broadly resuming or continuing where possible following delays due to COVID-19

Gene t	herapy	Cell th	erany
FREELINE    NASDAQ listed      Value: £227.2m      48% ownership	GYROSCOPE VISION FOR LIFE Private Value: £82.0m 80% ownership	Autolus NASDAQ listed Value: £143.7m 27% ownership	ACHILLES
Seeking to deliver constant high protein expression levels with curative potential across a broad pipeline of systemic diseases	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	Applying a broad range of technologies to build a pipeline of precisely targeted T cell therapies designed to better recognise and attack cancer	Differentiated cell thera solid tumours utilising Lymphocytes and clona develop personalised t
<ul> <li>Successfully undertook a Series C financing</li> <li>Listed on NASDAQ</li> <li>Raised \$299m in total bring in specialist, long-term capital</li> </ul>	<ul> <li>Dose escalation ongoing in phase I/II trial and first patient dosed in phase II trial</li> <li>Granted FDA fast-track designation</li> <li>Appointed Sean Bohen to the Board</li> </ul>	<ul> <li>Data in AUTO1 adult acute lymphoblastic leukaemia (adult ALL)</li> <li>Data in AUTO3 diffuse large B-cell lymphoma (DLBCL)</li> </ul>	<ul> <li>Dosed first patients in melanoma and N</li> <li>Appointed Karl Peg</li> <li>Raised £52.7m in S end</li> </ul>
Clinical pipeline Research   Target ID   Pre- Clinical   Clinical Haem. B Fabry Gaucher Haem. A Undisclosed disorders	Clinical pipeline Research   Target ID   Pre- Clinical   Clinical Dry AMD –G.A (sub-set) Dry AMD –G.A (broad) Other inflammatory	Clinical pipeline Auto 1 - pALL Auto 1 - aALL Auto 3 - DLBCL Auto 4 TCL	Clinical pipeline Research Melanoma On-cell lung cancer Other indications
Spark Pfizer uniQure	gemini Apellis	🧭 GILEAD	<b>INVANCE</b> BIOTHERAPEUTICS



gritstone

# Momentum building in preclinical companies



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

Company	Focus	Value	Operational progress	Clinical progress	Competitors
	Cell therapy	£19.9m	<ul><li>Team build out</li><li>Manufacturing build out</li></ul>	<ul><li>Clinical candidate nomination</li><li>Pipeline development (neuroinflammation)</li></ul>	
пеооделе	Cell therapy	£11.8m	<ul> <li>Series A financing of \$110 million</li> <li>Appointment of Franz Humer as Chair</li> </ul>	<ul> <li>Pre-clinical development</li> </ul>	PACT
() RTx	Cell therapy	£1.8m	<ul> <li>Series A financing of £26.8m commitment</li> </ul>	<ul> <li>Pre-clinical development</li> </ul>	Resolution with first mover advantage
SwanBio THERAPEUTICS	Gene therapy	£33.0m	<ul> <li>Team build out</li> <li>Continuing to develop a scalable manufacturing process for commercial supply</li> <li>Completed engineering run of its product</li> </ul>	<ul> <li>Pre-clinical development continues with lead programme</li> <li>Made strong progress in its clinical trial design</li> <li>Developing pipeline indications</li> </ul>	Passage Bio
ΛΝ <sub>Δ</sub> νεον	Biologics	£12.4m	<ul><li>Continued team build out</li><li>Expanding operations</li></ul>	<ul> <li>Clinical candidate nomination</li> </ul>	NEKTAR synth@rx
<b>VOMASS</b> THERAPEUTICS	Small molecule	£14.6m	<ul> <li>Continue to recruit senior leadership team</li> </ul>	<ul> <li>Progressing a pipeline of small molecule targets</li> </ul>	N/A

### Azeria



Rigorous risk management driven by data and disciplined capital allocation

# Syncona makes initial investment in Azeria

#### High quality scientific insights

- Encouraging target validation and drug discovery work
- New target and mechanism of action in an area of high unmet need

#### **Financing structure**

- Investment of £6.5m as first tranche of £29.5m commitment
- Milestones for next tranche designed to generate data that tests the thesis

#### Key risk

 Pre-clinical data outcomes to validate academic discovery in industrial setting

#### **Further pre-clinical work**

#### **Rigorous process**

 Azeria undertakes further pre-clinical studies to deliver high quality, robust and reproducible data

# High quality and collaborative team

- Azeria management team were data driven and ran best-in-class process

#### Data does not support further investment

#### Review of the data

- Syncona team review the data in partnership with Azeria team
- Data does not support further investment

#### Decision taken to close the business

- Syncona team acted quickly to recover as much value as possible from the investment
- Limit further costs and reallocate our time and investment capacity

#### Worked closely with the company

- Supporting the Azeria team where needed

Sourcing and company foundation

# Syncona platform: a growing competitive advantage



Platform enables rapid translation of basic scientific research into companies with the potential to be global leaders



Sourcing technology in growing areas has led to multiple Syncona companies and investments





The strength of our platform and the depth of our diligence allows us to identify new areas where there is the potential to found multiple companies

### Resolution: harnessing the healing properties of macrophages

Macrophage cells are a key immune cell type

Based on the research of Prof. Stuart Forbes and Prof. John Campbell from the University of Edinburgh

Built over a 3-year partnership between Syncona and the University:

- Research Collaboration launched in Jan 2018 to develop the technology further
- Series A commitment of £26.8m from Syncona
- Vision to develop an autologous macrophage cell therapy for treatment of liver cirrhosis
- Syncona partners Ed Hodgkin and Gonzalo Garcia to become CEO and Chief of Staff respectively



# **Neogene Therapeutics**

Investment in exciting T cell receptor company in area of deep domain expertise



Significant potential to build leading T cell receptor company

# Purespring: one of the first kidney AAV gene therapy companies

New Syncona company in area of deep domain expertise



#### Foundation of one of the first AAV gene therapy company's globally to target the kidney



# Financing strategy

# Funding a marketed product company

Life science companies require significant capital to deliver marketed product strategy

- Significant capital is required to take a drug from discovery through to market approval
- Capital requirements increase as company progresses through the development cycle and clinical trials
- In the fields of cell and gene therapy, significant capital required to develop robust manufacturing platforms
- To build winners in life science, important to build out pipelines and manufacturing to enable companies to complete globally

Syncona

### 8-10

Years regulatory timeframe for Third Wave therapies

>\$18bn1

Amount of private and public capital raised by cell and gene therapy companies in last three years

### Competing on a global scale requires significant capital

- £1.4bn\* raised by Syncona companies
  - £723.1m committed by Syncona
- Strong balance sheet enables us to invest in our companies over the long-term
- As companies scale and enter the clinic significant capital is required
- Balance sheet expansion has increased our ability to invest at scale with conviction
- Our balance sheet is a strategic and competitive advantage; gives us flexibility to bring in specialist institutional investors at the right time and price



### How we fund our companies at a global competitive scale

NASDAQ is a core funding mechanism for companies with significant capital requirements

- NASDAQ is the deepest, most liquid expert pool of life science capital
- Syncona has a strong track record in building globally competitive UK businesses that have successfully listed on NASDAQ
- The ability to access this pool of capital sets our companies up with the right shareholders to meet their funding needs
- We seek to maintain significant ownership positions in these companies and continue to fund them through financing rounds over the long-term
- Holding a number of listed companies may bring volatility to our NAV over the short-term; we believe this will be outweighed by long-term value creation





# Financial review

# **Financial review**

NAV of £1,366.7m, 203.4p; capital pool of £700.1m

#### NAV increase of 9.6% in the six months to **30 September 2020**

- Life science portfolio valued at £666.6m, a return of 24.8% in six months:
  - Performance driven by the increase in the Autolus share price and the write-up of Freeline in its recent Series C financing and IPO
  - Azeria closed resulting in a write off of £4.5m
- Capital base of £700.1m; £68.9m of capital deployed in the six months
- Post period end:
  - £42.9m capital deployed
  - £10.7m write up of Achilles following its £52.7m Series C financing; Syncona retains a 34% holding

- Clinical stage
- Pre-clinical stage
- Drug discovery





<sup>\*</sup>Percentage holdings reflect Syncona's ownership stake at the point full current commitments are invested 19 \*\*Cost indicates that the fair value has been determined to be equal to the total funding invested by Syncona

### Balance sheet strength is strategic and a key differentiator

Life science companies requires significant capital as they scale

#### Syncona capital base



to fund growing life science portfolio and found new companies



based on further investment in our existing portfolio and the opportunities we see in our investment pipeline



#### Strong capital base is central to delivery of strategy

- Founding investors have the best ability to set strategy
- Life science companies require significant capital as they scale; ability to maintain influence through financing rounds essential
- Balance sheet strength provides best negotiating position for external financing rounds or M&A
- Capital to execute ambitious vision optimises ability to attract the best academics, founders, managers and partners

#### **Disciplined approach**

- Each financing dependent on company specifics (scale of opportunity, risk, capital requirement) and size of Syncona's balance sheet
- Funding commitments tranched and based on milestone delivery

# Outlook and summary

# Portfolio company outlook Strong momentum in the portfolio with near term catalysts



Company	Status of pipelines	Next steps
Autelus	Four programmes in clinical trials	<ul> <li>Progress on AUTO1 pivotal trial</li> <li>Decision regarding move to Phase II in AUTO3 DLBCL</li> <li>Initial data in Phase I AUTO4 programme CY2021</li> </ul>
FREELINE	Two lead programmes in Phase I/II clinical trials, pipeline of preclinical programmes	<ul> <li>Publish further data in its lead programme in haemophilia B FY2021 and initiate pivotal study in CY 2021</li> <li>Dose its next patient in its second programme in Fabry's when its safe to do so</li> </ul>
GYROSCOPE VISION FOR LIFE	Lead programme completed dose escalation in Phase I/II trial and initiation of Phase II trial	<ul> <li>Initial data from its lead phase I/II trial targeting dry AMD FY2021</li> </ul>
ACHILLES	Two lead programmes in Phase I/II trials	<ul> <li>Report initial data in H1 CY2021 from its melanoma and NSCLC studies</li> </ul>
	Lead programme in pre clinical development	<ul> <li>Complete first clinical manufacturing batch in this financial year.</li> <li>Expand leadership team</li> </ul>
OMass	Seeking to build pipeline of therapeutics	- Initiation of pre-clinical development of lead programme
<b>AN VEON</b>	Nominated clinical candidate in lead programme	<ul> <li>Initiation of phase I/II clinical trial FY2022</li> </ul>
	Nominated clinical candidate in lead programme	<ul> <li>Initiation of phase I/II clinical trial FY2022</li> </ul>
RTx	Pre-clinical development of lead programme	<ul> <li>Company and leadership team build out</li> </ul>
neogene	Pre-clinical development of lead programme	<ul> <li>Company and leadership team build out</li> </ul>
Purespring	Pre-clinical development of lead programme	<ul> <li>Company and leadership team build out</li> </ul>

# Summary

Syncona platform creates value from the commercialisation of life science innovation

- Continued strong long term performance; 40% IRR and 2.0x money multiple on portfolio
- Companies continue to scale fast and significant capital requirements ahead
- Strong capital pool provides strategic advantage in current environment
- Three new companies added to the portfolio in the last six months
- Significant opportunity ahead for Syncona to continue to capitalise on globally differentiated research base in UK/EU



### **10-year targets**

15-20

sustainable portfolio

of leading life science companies

2-3 new companies created each year



3-5

companies to approval; accessing the steepest part of the life science value creation curve

# Q&A Dial in: 0203 107 0289 Conference ID: 1643547

# Appendix

### Our approach has delivered significant long term value

Strong track record; IRR of 40% - 2.0x cost generated on Syncona portfolio since 2012

#### Strong risk adjusted returns

- £661.5m capital deployed since 2012
- 14 Syncona portfolio companies founded
- Two companies sold<sup>:</sup>
  - 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 £666.6m
  - 1.2x capital invested





# 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 C population p.a	Deportunity in and differentiation of lead programme	Key comparators <sup>2</sup>	Key risks <sup>1</sup>
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 <sup>3*</sup>	No CAR T there are a second to care.	<ul> <li>CAR-T active programmes in clinical development for ALL include Gilead<sup>7</sup></li> </ul>	<ul> <li>Differentiated product required</li> <li>Complex manufacturing</li> </ul>
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 9.5k <sup>8**</sup>	<ul> <li>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</li> <li>Opportunity to deliver a single dose cure for patients by achieving FIX levels in the 'normal' range in the blood of 50-150%</li> <li>Utilising a novel, proprietary capsid and industrialised proprietary manufacturing platform</li> </ul>	<ul> <li>Active clinical programmes in gene therapy for Haem B include: Spark/Pfizer<sup>9</sup>, UniQure<sup>10</sup></li> </ul>	<ul> <li>Highly competitive environment</li> <li>Differentiated product required</li> <li>Manufacturing</li> </ul>
<b>Gyroscope</b> 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 <sup>11**</sup>	<ul> <li>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 treatments<sup>12</sup>.</li> <li>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</li> <li>Gene therapy may stimulate a patient's cells to produce the proteins needed to restore balance to the complement system</li> <li>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.</li> </ul>	<ul> <li>No directly competitive gene therapy approach targeting complement system</li> <li>Apellis<sup>13</sup> (clinical); Gemini (pre-clinical)<sup>14</sup>, Hemera<sup>15</sup> (non-gene therapy)</li> </ul>	<ul> <li>Highly innovative concept which is currently unsupported by a significant existing data set</li> </ul>
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 <sup>16*</sup>	<ul> <li>Unmet medical need: lung cancer, of which NSCLC accounts for approximately 85%<sup>17</sup>, with limited treatment options and is the leading cause of cancer deaths<sup>18</sup>.</li> <li>TILs have shown convincing efficacy in solid tumours<sup>19</sup></li> <li>Achilles' world leading bioinformatics platform, PELEUS<sup>™</sup> is built on exclusive access to world largest study of tumour evolution in lung cancer (TRACERx)</li> <li>Achilles process uses the patient's own genomic information to create a truly personalised medicine targeting the clonal neoantigens</li> </ul>	<ul> <li>Key competitors in the neoantigen/ personalised immunotherapy space include: lovance<sup>20</sup>, Neon Therapeutics<sup>21</sup>, Gritstone Oncology<sup>22</sup></li> </ul>	<ul> <li>Highly innovative concept in an emerging space</li> <li>Significant manufacturing challenge</li> <li>Increasing competition</li> </ul>



# Significant opportunity in earlier stage portfolio

Potential to deliver multiple approved products delivering transformational treatment for patients.

Company	Investment thesis	Key comparators <sup>2</sup>	Key risks <sup>1</sup>
SwanBio Gene therapy focused on neurological disorders where there is existing proof of concept	<ul> <li>Unmet medical need: one of the most common monogenic neurological disorders, with no available therapies for severely debilitating progressive movement disorder</li> <li>Gene therapy has the potential to be transformational in neurology<sup>23</sup></li> <li>one-off delivery mechanism and hundreds of single gene disorders</li> <li>First programme in preclinical development for an inherited neurodegenerative disease in which the causative gene is definitively known and well characterized</li> </ul>	Several clinical trials for gene therapy within CNS field, including programmes within Voyager <sup>24</sup> , Uniqure <sup>25</sup> , Amicus <sup>26</sup> , Prevail Therapeutics <sup>27</sup> and PTC Therapeutics <sup>28</sup>	<ul> <li>Manufacturing and delivery challenges in the CNS (substantial dose required)</li> <li>Clinical endpoints can be challenging to define</li> </ul>
Quell Engineered cell therapy company addressing immune dysregulation	<ul> <li>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 survival<sup>29</sup></li> <li>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</li> <li>Potential pipeline to treat serious, chronic conditions mediated by the immune system; in the autoimmune setting alone, there are &gt;70 chronic disorders estimated to affect over 4% of the population<sup>30</sup></li> <li>Pre-clinical stage: first programme to address solid organ transplant</li> </ul>	T Reg field is nascent; TX Cell/Sangamo <sup>31</sup>	<ul> <li>Highly innovative concept, limited clinical data supporting application of CAR-T technology in Treg cells</li> </ul>
Anaveon Immuno-oncology company developing a selective IL-2 Receptor Agonist	<ul> <li>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 toxicity<sup>32</sup></li> <li>Preclinical stage, developing a selective Interleukin 2 ("IL-2) Receptor Agonist with improved administration and tox burden</li> <li>Wide potential utility across multiple oncology indications in large markets<sup>33</sup></li> </ul>	Companies developing products in the IL-2 field include: Nektar <sup>34</sup> , Roche <sup>35</sup> , Alkermes <sup>36</sup> , Synthorx <sup>37</sup> .	<ul><li>Highly competitive</li><li>Technical risk around product</li></ul>
OMASS Drug Discovery platform with differentiated technology	<ul> <li>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</li> </ul>	N/A	<ul> <li>Pre clinical and clinical attrition of potential drugs</li> </ul>



### 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.

#### "Second Wave"

1990's Large Molecule (antibody therapies enzyme replacement therapies).

### The "Third Wave"

#### Today

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



Small molecules	8	2	?
Second wave	2	8	?
Third wave	0	0	?

Number of monogenetic disorders, less than 100

with treatments today

80%

of rare diseases are of genetic origins

'Third Wave' therapies approved in the US



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
- Pre-clinical stage
- Drug discovery





# Rich and broad pipeline of products

# Rapidly progressing pipeline in areas of high unmet need

Multiple undisclosed pre clinical programmes



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- 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-1a3b0bfbd56a. 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-1a3b0bfbd56a
- 5. Cytokine Release Syndrome
- 6. Source: Autolus see Autolus corporate presentation November 2019 https://autolus.gcs-web.com/static-files/cd8dc1d9-6a7b-496d-933f-1a3b0bfbd56a
- 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: Gyroscope estimate. Age related macular degeneration, of which one type is dry AMD, is estimated to affect 195.6 million people globally (<u>https://www.who.int/publications-detail/world-report-on-vision</u>). Gyroscope'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 <a href="https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/lung-cancer/incidence">https://www.cancerresearchuk.org/health-professional/cancer-statistics/statistics-by-cancer-type/lung-cancer/incidence</a>.
- 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://gritstoneoncology.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. http://ir.amicusrx.com/news-releases/news-release-details/amicus-therapeutics-acquires-gene-therapy-portfolio-ten-clinical
- 27. https://www.prevailtherapeutics.com/
- 28. http://ir.ptcbio.com/news-releases/news-release-details/ptc-therapeutics-announces-strategic-gene-therapy-licensing
- 29. Source: https://www.ema.europa.eu/en/documents/scientific-guideline/guideline/clinical-investigation-immunosuppressants-solid-organ-transplantation\_en.pdf
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- 32. Source: https://www.cancernetwork.com/renal-cell-carcinoma/managing-toxicities-high-dose-interleukin-2
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