Syncona

Syncona Update

March 2022

Syncona

Building the next generation of healthcare companies

Key Announcements

Freeline registered direct offering
11.03.22

Syncona entered into a definitive agreement with its portfolio company Freeline alongside other existing shareholders to purchase an aggregate of $26.1 million of its American Depositary Shares ("ADSs"), each representing one ordinary share of the company, at a price of $1.05 per ADS, in a registered direct offering.

Syncona agreed to invest $20.0 million in the offering. Following the offering, Syncona retains a stake of 55% in Freeline (amounting to 37,862,147 ordinary shares) which was valued at £30.4 million at close of business on 10 March 2022.

Freeline intends to use the net proceeds from this offering to fund activities relating to the continued development of its product candidates and gene therapy platform and for other general corporate purposes.

Corporate Presentation - March 2022
21.03.2022

Syncona uploaded its latest corporate presentation – you can access it here.

Key Media Coverage

Europe's Ophthalmology Scene Stays Hot for Biotech Funding
Labiotech 03.03.22

Jonathan Smith of Labiotech reports that ophthalmology-focused startups had a record year for venture capital funding in 2021. Impressive Series A rounds and collaborations hint at a continuing upward trend for the field in 2022. The article suggests that ophthalmological gene therapies have been the focus of numerous recent funding rounds and transactions, including the acquisition of Syncona's Gyroscope Therapeutics by Novartis.

Three stocks to provide income and growth from a diversified portfolio
MoneyWeek 18.03.22

Professional investor Gary Moglione from Momentum includes Syncona in his share tip in MoneyWeek. He says that the team has a track record of success, including the sales of Blue Earth Diagnostics, Nightstar Therapeutics and Gyroscope. The tip suggests that as Syncona-funded products move through clinical trials there will be both winners and some losers, but the management team has demonstrated its ability to develop successful businesses with asymmetric return profiles.

Can tech save healthcare?
Investors Chronicle 24.03.22

Mary McDougall of Investors Chronicle has published a feature on how converging technologies hope to revolutionise healthcare. Anthony Leatham, head of investment trust research at Peel Hunt, is quoted as anticipating "a bounce for biotechnology stocks, having been badly impacted by the macroeconomic and geopolitical backdrop". He singles out Syncona, which he says has a "very strong balance sheet position, with cash ready to take advantage of investment opportunities, alongside some forthcoming potential catalyst events including Autolus, Achilles, Freeline and Anaveon".

Achilles Therapeutics

Developing novel cancer immunotherapies targeting clonal neoantigens

Key Announcements

Achilles Reports Fourth Quarter and Year-End 2021 Financial Results and Recent Business Highlights
01.03.22

Achilles announced its financial results for the fourth quarter and year-ended December 31, 2021, and recent business highlights.

"In 2021, we generated clinical data demonstrating the unique ability of our T cell platform to detect, quantify and track our clonal neoantigen-reactive T cells, or cNeT, in vivo, giving us the analytical platform to elucidate the mechanism of action of our cNeT product. cNeT dose is now being used in our clinical trials as a release parameter, highlighting our differentiated potential to develop potency assays that we believe are essential for the successful development and regulatory approval of TIL-based therapies," said **Dr Iraj Ali, Chief Executive Officer of Achilles Therapeutics.**

Achilles announced a cash position of $266 million as at 31 December 2021 and has a cash runway to H2 2024.

Achilles to Host Key Opinion Leader Webcast to Highlight TRACERx Study Presentations from the 2022 AACR Annual Meeting
04.04.22

Achilles announced that it will host a key opinion leader (KOL) webcast to discuss the importance and unique utility of the TRACERx study in validating the identification of clonal neoantigens as targets for personalised T cell therapies and to highlight selections from the 31 posters and presentations on the TRACERx study presented at the 2022 American Association for Cancer Research (AACR) Annual Meeting.

Event details:

- Date: Thursday, April 14, 2022
- Time: 10:30am ET / 3:30pm UK
- Location: Webcast details available in the Events & Presentations section of the Achilles website

Key Media Coverage

London-based Achilles Therapeutics picks Philadelphia for its first U.S. location
Philadelphia Business Journal 01.03.22

The Philadelphia Business Journal reported that Achilles has selected Philadelphia as its first location in the United States. The company, which is developing cancer therapies by using DNA sequencing from individual patients, signed a three-year lease on a 7,243 square feet facility.

Anaveon

Developing biologics to modulate the function of cytokines and provide substantial benefit to cancer patients

Key Announcements

Anaveon To Present First Clinical Data From The Phase I/II Study Of ANV419 At The AACR Annual Meeting 2022
09.03.22

Anaveon announced that it will present first clinical data from the ongoing Phase I/II study of ANV419, a powerful and selective interleukin-2 (IL-2) agonist in patients with solid tumours, in a poster presentation at the American Association for Cancer Research (AACR) Annual Meeting in New Orleans, Louisiana, April 8 to April 13, 2022.

Key Media Coverage

New pinhead-sized beads kill ovarian, colorectal cancers by producing interleukin-2: mouse study
FierceBiotech 08.03.22

Anaveon was mentioned in an article by FierceBiotech which referenced the wave of biotech companies trying to create the next generation of IL-2 therapies. On the back of research news from Rice University, author Kyle LaHucik mentioned that Pfizer joined Novartis in backing Anaveon to bankroll its IL-2 agonist in solid tumours in December 2021.

The IL-2 cancer pipeline remains deep, diverse
BioCentury 18.03.22

Danielle Golovin of BioCentury reports on the IL-2 cancer pipeline across the industry, stating that Anaveon has ANV419, an IL-2 fused to an antibody that binds with high affinity to the IL-2RA binding domain of IL-2.

Autolus Therapeutics

Developing next generation programmed T cell therapies for the treatment of cancer

Key Announcements

Autolus announces publication describing novel cell programming technology
03.03.22

Autolus announced the publication of an article in BioTechniques describing a novel technology that provides for very low levels of expression of one gene module, while maintaining high levels of expression of other gene modules expressed from the same promotor.

“Our approach to solid tumours combines multiple gene modules in CAR T cells to drive the desired set of properties we believe are essential to maximize anti-tumour activity without increasing toxicity. Selectively adjusting expression levels became an important technology to establish therapeutic windows,” added **Martin Pule, chief scientific officer and founder of Autolus**. "In this paper we successfully applied this technology for highly restricted IL-12 release which increases CAR T anti-tumour activity in an immunocompetent mouse model without inducing systemic toxicity.”

Autolus Reports Fourth Quarter and Full Year 2021 Financial Results and Operational Progress
10.03.22

Autolus announced its operational and financial results for the fourth quarter and full year ended December 31, 2021.

“We rounded off the 2021 financial year announcing a collaboration with Blackstone Life Sciences, adding $150M in capital with an additional $100M in potential milestone payments triggered by future development progress of obe-cel, as well as positive clinical data from our pipeline, notably data from the Phase Ib portion of the FELIX study of obe-cel in adult ALL patients. Recruitment is ongoing in the Phase II portion of this pivotal study and we look forward to announcing initial Phase II data this year, as well as starting preparations for submitting a BLA in 2023,” said **Dr. Christian Itin, Chief Executive Officer of Autolus.**

Autolus announced a cash position of $310 million as at 31 December 2021 and a cash runway which extends to 2024.

Autolus Receives EMA Orphan Medical Product Designation for obe-cel for Treatment of Acute Lymphoblastic Leukaemia
31.03.22

Autolus announced that the European Medicines Agency (EMA) had granted obe-cel, Autolus’ leading CAR T clinical candidate, Orphan Medical Product Designation for treatment of acute lymphoblastic leukaemia (ALL) patients.

Autolus Corporate Presentation - March 2022
31.03.2022

Autolus uploaded its latest corporate presentation – you can access it here

Key Media Coverage

Other news to note for March 3, 2022
BioWorld 03.03.22

BioWorld’s daily newsletter reports that new data from Autolus demonstrated a method for expressing stable low levels of a toxic gene for treating solid tumours.

[Sanofi lures Blackstone backing in unusual deal for multiple myeloma drug]
BioPharma Dive 15.03.22

Kristin Jensen of BioPharma Dive reports on Sanofi and Blackstone's new partnership, as Blackstone expands its commitment to help develop important medicines in critical therapeutic areas. Jensen references Blackstone's investment into Autolus in November, to support them to develop a cell-based cancer treatment as part of this strategy.

Clade Therapeutics

Discovering and delivering scalable next generation induced pluripotent stem cell (iPSC)-derived medicines

Key Announcements

Clade Therapeutics Announces the Appointment of Derek Hei, Ph.D., as Chief Technology Officer
10.03.22

Clade announced the appointment of Derek Hei, Ph.D., as its Chief Technology Officer. Dr Hei joins Clade with deep expertise in the cell therapy space and over two decades of experience leading manufacturing teams at biotechnology companies. He most recently served as Senior Vice President of Preclinical and Clinical Manufacturing, Cell and Gene Therapies at Vertex Pharmaceuticals, where he was responsible for leading chemistry, manufacturing, and controls activities for multiple cell and gene therapy programmes.

Key Media Coverage

BlueRock, Vertex vet Hei becomes CTO at Clade
BioCentury 12.03.22

BioCentury's appointment's newsletter reports that Clade hired Derek Hei as Chief Technology Officer. Hei was SVP of preclinical and clinical manufacturing, cell and gene therapies at Vertex Pharmaceuticals and previously was chief of manufacturing and technical operations at BlueRock Therapeutics.

Freeline Therapeutics

Focused on developing curative gene therapies for chronic systemic diseases

Key Announcements

Freeline Announces First Patient Dosed in the Phase I/II B-LIEVE Dose-Confirmation Trial in Haemophilia B
09.03.22

Freeline announced that the first patient was dosed in its Phase I/II B-LIEVE dose-confirmation clinical trial of FLT180a for the treatment of haemophilia B, a debilitating genetic bleeding disorder caused by a deficiency in the clotting factor IX protein. FLT180a uses a potent and proprietary adeno-associated virus vector capsid (AAVS3) to deliver the functioning gene of a variant of human factor IX and restore normal blood clot formation.

Freeline Announces $26.1 Million Registered Direct Offering of American Depositary Shares
11.03.22

Freeline announced that it had entered into a definitive agreement with Freeline's majority shareholder, Syncona Portfolio Limited, a subsidiary of Syncona Limited, and certain other existing shareholders to purchase an aggregate of $26.1 million of its American Depositary Shares ("ADSs"), each representing one ordinary share of the company, at a price of $1.05 per ADS, in a registered direct offering. Syncona agreed to invest $20.0 million in the offering.

Freeline Announces Updated Development Plan and Timelines for FLT190 for People with Fabry Disease
24.03.22

Freeline announced an updated clinical development plan and timelines for FLT190 for people with Fabry disease, a rare, inherited lysosomal storage disorder resulting in cell abnormalities and organ dysfunction that affect small blood vessels, tissues and organs including skin, kidneys, heart, and the nervous system. Freeline will proceed immediately to the second dose cohort (1.5e12 vg/kg) in the MARVEL-1 dose escalation study, with the first patient expected to be dosed in mid-2022.

Freeline Reports Full Year 2021 Financial Results and Business Highlights
31.03.22

Freeline reported financial results for the full year 2021 and provided a business update.

"2022 is shaping up to be a watershed year for Freeline, building on the strong foundation we put in place in the second half of last year," **said Michael Parini, Chief Executive Officer of Freeline**. "Under new leadership, our streamlined organisation has executed with urgency and increased financial discipline on a refocused set of clinical programs and corporate priorities. We also have recently strengthened our balance sheet to enable us to deliver meaningful clinical data readouts through 2022 and beyond to demonstrate the value of our promising gene therapy candidates as we advance on the path towards pivotal Phase III studies. Additionally, we are working on a new R&D strategy to explore the application of our science and platform technologies to new disease areas, including extending these strengths to efforts beyond rare monogenic disorders."

Freeline had a cash position of $118 million as at 31 December 2021, and has a cash runway up to H2 2023.

Freeline Corporate Presentation - March 2022
31.03.2022

Freeline uploaded its latest corporate presentation – you can access it here

Key Media Coverage

Freeline Therapeutics doses first subject in haemophilia B therapy trial
Clinical Trials Arena 10.03.22

Clinical Trials Arena reports that Freeline has dosed the first subject in the Phase I/II B-LIEVE clinical trial of its therapy FLT180a to treat haemophilia B. The article quotes Freeline's Chief Medical Officer Pamela Foulds: "We are optimistic that the combination of a low dose (7.7e11vg/kg) of our high-expression AAVS3 capsid with a short prophylactic immune management regimen can get and keep haemophilia B patients in the normal range of Factor IX expression."

Emerging Startups Take Aim at Lysosomal Storage Disorders
Labiotech 10.03.22

Jonathan Smith of Labiotech reports that lysosomal storage disorders have proved difficult to tackle in the biotech industry. Smith suggests that gene therapy is one way of tackling lysosomal disorders, referring to Freeline as a European gene therapy hopeful in the space.

Financings for March 11, 2022
BioWorld 11.03.22

BioWorld's daily newsletter reports that Freeline announced that it entered a definitive agreement with its majority shareholder, Syncona, and certain other existing shareholders, to purchase an aggregate of $26.1 million of its American depositary shares (ADSs), each representing one ordinary share of the company.

Homology gets clarity on how to resolve pheNIX gene therapy hold
FierceBiotech 24.03.22

Annalee Armstrong of FierceBiotech reports on the immunosuppressive strategy Homology is implementing, comparing it with Freeline Therapeutics, who have approached things similarly in their gene therapy studies.

In the clinic for March 25, 2022
BioWorld 25.03.22

BioWorld's daily newsletter reports that Freelines' FLT-190 in Fabry disease has advanced to second dose cohort (1.5e12 vg/kg) in the MARVEL-1 dose-escalation study; the first patient expected to be dosed in mid-2022.

OMass Therapeutics

Using novel biochemistry techniques, native mass spectrometry and custom chemistry to deliver novel medicines against highly validated but inadequately drugged targets.

Key Announcements

OMass Establishes Scientific Advisory Board of Leading Experts in the Use of Biophysical Approaches to Drug Discovery
07.03.22

OMass announced the establishment of its scientific advisory board (SAB), comprising of world-renowned experts across biophysical technologies.

The company's newly formed SAB will guide OMass' mission of improving the lives of patients with orphan or immunology diseases through the development of novel, differentiated small molecules against hard-to-drug targets. The members of the SAB will help ensure OMass continues to utilise and develop its drug discovery platform to its full potential.

Key Media Coverage

OMass establishes scientific advisory board
MedNous 11.03.22

MedNous covered the establishment of OMass' five-member scientific advisory board. Chairing the board is Carol Robinson, founder of OMass, and professor of chemistry and founding director of the Kavli Institute for Nanoscience Discovery at Oxford University.

Quell Therapeutics

Developing engineered T regulatory (T-reg) cell therapies

Key Media Coverage

5 Dealmaking Trends To Watch In 2022
In Vivo 21.03.22

In Vivo provided an overview of dealmaking in the biopharma industry in 2021 and assessed how 2022 might compare. At the most recent Genesis Conference in December 2021, Luke Henry, Chief Business Officer of Quell Therapeutics, said: “There’s capital out there in the VCs and in the crossover funds that is there to be deployed. There’s a large amount of dry powder that's gone into those funds over the last two or three years that has to work through the system...So that means as a young company, financing a business is not easy, but it's certainly easier than other periods of time.”

Resolution Therapeutics

Developing macrophage cell therapies to repair organ damage, including treatment of end-stage chronic liver disease

Key Announcements

Resolution and panCELLa Inc announce a Research Evaluation & Option Agreement to develop iPSC-derived macrophage cell therapy for inflammatory organ disease
05.04.22

Resolution and panCELLa announced a research collaboration to develop iPSC-derived macrophage cell therapies. Under the agreement, panCELLa is providing its hypo-immunogenic engineered iPSC technology to Resolution. Resolution is using its proprietary macrophage engineering and manufacturing technology to develop "off-the-shelf" macrophage cell therapies. As part of this agreement Resolution has an option to obtain commercial rights to use the panCELLa technology in the field of macrophage biology.

Resolution announced a £10m extended Series A financing from Syncona Ltd
05.04.22

Resolution announced the completion of a £10 million extension to the Series A financing from Syncona. This follows the £26.6 million Series A investment by Syncona announced in December 2020, after a successful collaboration with the University of Edinburgh Centre for Regenerative Medicine, which has provided the foundation of Resolution's technology platform.

"There is strong momentum behind Resolution and we are expanding the expertise within the organisation, whilst progressing our platform and programs," added **Edward Hodgkin, Partner of Syncona Investment Management Limited, who serves as Chairman and CEO of Resolution.** "Our proprietary understanding of the properties of macrophages required for organ repair puts us in a unique position to develop both autologous and allogeneic macrophage cell therapy, where the balance between macrophage function and prevention of immune rejection will be key to success."

SwanBio Therapeutics

Developing leading-edge medicines to deliver dramatic clinical efficacy for the treatment of neurological diseases

Key Announcements

SwanBio to Present New Preclinical Data Showcasing AAV-Based Gene Therapy Candidate for Adrenomyeloneuropathy at American Academy of Neurology Annual Meeting
04.03.22

SwanBio announced that it will present data supporting the clinical advancement of its lead candidate, SBT101, at the American Academy of Neurology (AAN) 2022 Annual Meeting. The meeting is being held in Seattle April 2-7, 2022, and virtually April 24-26, 2022.

FDA Grants Orphan Drug Designation to SBT101, the First Investigational AAV-Based Gene Therapy for Adrenomyeloneuropathy (AMN)
15.03.22

SwanBio announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation to the company's lead candidate, SBT101. SBT101 is being investigated as a potential treatment for patients with adrenomyeloneuropathy (AMN). SwanBio plans to initiate a randomised, controlled Phase I/II clinical trial to assess the safety and efficacy of SBT101 in the second half of 2022.