Syncona

Syncona Update

September 2021

Syncona

Building the next generation of healthcare companies

Key Media Coverage

15 stocks that could change the world
Interactive Investor 07.09.21

Nina Kelly of Interactive Investor put a trio of investment trust holdings, including Syncona, under the microscope for review, giving an overview of Syncona's origins, being co-founded by Chief Executive Officer Martin Murphy and the world's second-largest medical charity, the Wellcome Trust.

Kelly quoted Martin from an interview he did with Interactive Investor's Lee Wild last November, when he said, "We are at a remarkable age in the field of medicine at the moment, the so-called age of personalised medicine…now we really understand how to look at patients on a one-by-one basis and design therapies that are targeted to the molecular mechanisms that are driving their diseases."

The article describes how the team at Syncona look for significant unmet need in patient populations when it comes to choosing investments, whilst remaining economically rational. There is also a summary of a selection of the portfolio companies: Gyroscope, Autolus, Purespring, Freeline and Achilles.

Achilles Therapeutics

Developing novel cancer immunotherapies targeting clonal neoantigens

Key Announcements

Achilles Therapeutics to present at upcoming investor conferences
02.09.21

Achilles announced that management would participate in the following conferences in September:

- Wells Fargo Virtual Healthcare Conference on September 10, 2021
- H.C. Wainwright 23rd Annual Global Investment Conference available beginning September 13, 2021
- Oppenheimer Fall Healthcare Life Sciences & MedTech Summit on September 22, 2021

Key Media Coverage

Raising the Stakes: Immunocore's Next Play
In Vivo 08.09.21

Joanne Shorthouse of In Vivo mentioned Achilles in her article on Immunocore. In discussing Immunocore's IPO where $297.1m was raised and the company was valued at $1.4bn in February, 2021, she mentioned Immunocore's listed peers saying, "it is in good company as fellow UK biotech companies Adaptimmune and Achilles Therapeutics raised $258m and $176m, respectively."

Autolus Therapeutics

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

Key Announcements

Autolus publishes data obe-cel (AUTO1) ALLCAR19 data in adults with r/r ALL in Journal of Clinical Anthology
01.09.21

JCO publication highlights durable response and low toxicity following treatment with obe-cel. The publication reiterates previously published data, which shows that obe-cel has an excellent safety profile, and is shown to have a durable response amongst patients with the same level of Event Free Survival (EFS) at 12 and 24 months.

Autolus announces participation in September conferences
02.09.21

Autolus announced that it would participate in the following conferences in September:

- Goldman Sachs Eleventh Annual Biotech Symposium on September 7, 2021
- 2021 Virtual Wells Fargo Healthcare Conference on September 10, 2021
- H.C. Wainwright & Co 23rd Annual Global Investment Conference September 13, 2021

Autolus announces the appointment of John H. Johnson as non-executive chairman
15.09.21

Autolus announced the appointment of John H. Johnson as non-executive chair of its Board of Directors, replacing Syncona CEO Martin Murphy, who was serving as chair in an interim capacity. John brings more than 30 years of experience in the life science industry, including in his current role of CEO of Strongbridge Biopharma plc, a NASDAQ listed commercial-stage biopharma company. Martin Murphy will continue to serve as a non-executive Board member of Autolus.

Autolus provides an update on its manufacturing facility in Stevenage, UK
16.09.21

Autolus announced that it has received planning permission to build the Company's new manufacturing facility in Stevenage, UK. The 70,000 square foot facility will ultimately provide GMP capacity for approximately 2,000 batches per year, providing strong commercial capacity for Autolus' lead obe-cel programme.

Key Media Coverage

Poseida's Solid Tumor CAR-T Shows Early Promise, But Safety Will Be Closely Watched
Scrip 01.09.21

Andrew McConaghie of Scrip writes that in the wake of its own safety scare and the halting of a competitor's trial, Poseida Therapeutics has bounced back with encouraging early safety and efficacy data from its CAR-T cell therapy candidate P-PSMA-101 for solid tumours. When positioning Poseida within a peer group of companies working on CAR-Ts against solid tumours, Autolus are mentioned as a key player.

In the clinic for Sept. 2, 2021
BioWorld 02.09.21

BioWorld summarised Autolus's results of their phase I study of obe-cel published in the Journal of Clinical Oncology. CAR T cell concentration reached very high levels at peak and persistence in peripheral blood was evident in 75% of patients at a median of 166.5 days. 20% of patients had follow-

up duration over 2 years and 85% patients achieved minimal residual disease -negative complete response at 1 month.

Peer Review: A Pfizer partner welcomes ex-ADC Therapeutics CMO Jay Feingold to the team; Amid tough sledding, Immunovant chooses Eli Lilly alum as CFO
Endpoints 17.09.21

In its appointment column Endpoints reported that Christian Itin's crew at CAR-T outfit Autolus has named longtime biotech executive John Johnson chairman of the board.

Freeline Therapeutics

Focused on developing curative gene therapies for chronic systemic diseases

Key Announcements

Freeline announces participation in September conferences
07.09.21

Freeline announced its participation in the following conferences through September:

- H.C. Wainwright 23rd Annual Global Investment Conference on September 12, 2021
- Morgan Stanley 19th Annual Global Healthcare Conference on September 15, 2021

Freeline announces Orphan Drug Designation for FLT201 for the treatment of Gaucher Disease
09.09.21

Freeline announced that both the U.S. Food and Drugs Administration and European Commission had granted Orphan Drug Designation for FLT201 for the treatment of Gaucher Disease. Clinical trial site initiation for this treatment is on track for the year-end.

Key Media Coverage

Regulatory actions for Sept. 9, 2021
BioWorld 09.09.21

BioWorld reported that Freeline's AAV gene therapy, FLT201, received Orphan Drug Designation from the Food and Drug Administration and European Commission for the treatment of Gaucher disease.

Gaucher disease pipeline: Data Byte
BioCentury 30.09.21

In BioCentury's Gaucher disease pipeline update, Gunjan Ohri of BioCentury reported that behind the five marketed therapies for Gaucher disease, all of which treat Type I Gaucher and were approved before 2015, is a pipeline of at least three clinical and 11 preclinical candidates, according to BioCentury's BCIQ database.

Among the in vivo gene therapy developers, Freeline Therapeutics, Eli Lilly subsidiary Prevail Therapeutics and Coave Therapeutics are each taking an AAV-based approach, whereas Generation Bio is developing a non-viral gene therapy using closed ended DNA and cell-targeted lipid nanoparticle.

Gyroscope Therapeutics

Developing gene therapies and surgical delivery systems for retinal diseases

Key Announcements

Gyroscope announces presentation of further positive interim data in Phase I/II FOCUS study
30.09.21

Gyroscope announced positive interim data from the ongoing FOCUS Phase I/II clinical trial for its one-time gene therapy, GT005. Safety data from 28 patients showed GT005 continues to be well tolerated with no treatment-related serious adverse events. There was no evidence of clinically significant GT005-induced inflammation. Biomarker data from 13 patients, ranging from approximately seven months post treatment to nearly two years, continued to demonstrate sustained increases in vitreous complement factor I (CFI) levels compared to baseline in the majority of patients, as well as sustained decreases in downstream proteins associated with complement system activation.

Neogene Therapeutics

Pioneering the development of next-generation, fully personalized engineered T cells therapies for a broad spectrum of cancers

Key Announcements

Neogene appoints Han Lee as Chief Financial Officer
14.09.21

Dr Lee previously served as CFO of Arcellx, Inc, and previous to this was a Senior Director in the Corporate Development and Ventures group at AstraZeneca, where he focused on mergers and acquisitions, corporate strategy and co-managed their equity portfolio.

Key Media Coverage

Chutes & Ladders—AltruBio snags new CMO with resume at Sublimity, Ardea, Amgen and Abbott
FierceBiotech 17.09.21

In its appointments column Fierce Biotech reports that Neogene Therapeutics named Han Lee, Ph.D., its CFO. Lee joins the preclinical cancer biotech from Arcellx, where he was CFO and led $200 million in financings.

Purespring Therapeutics

One of the first AAV gene therapy companies focused on the kidney globally

Key Announcements

Purespring strengthens senior leadership with appointment of Chief Medical Officer and Chief Development Officer
01.09.21

Purespring announced the appointment of Dr Ronny Renfurm as Chief Medical Officer, and Julian Hanak as Chief Development Officer. Dr Renfurm joins from Astellas, with Julian Hanack joining from Biogen, where he was SVP and Head of CMC at NightstaRx Therapeutics. Also joining Purespring are Alan Griffith, who joins as Vice President of Research, and Amanda Weiss, who has joined as Vice President of CMC.

Key Media Coverage

Chutes & Ladders—FDA's top 2 vaccine leaders to depart in the fall as questions loom over booster shots
FierceBiotech 03.09.21

In its appointments column FierceBiotech reports Gene therapy biotech Purespring Therapeutics named Ronny Renfurm, M.D., its CMO after stints as a consultant and previously group head of Astellas Pharma's nephrology unit. Julian Hanak was selected as chief development officer after being a senior vice president at NightstaRx Therapeutics, acquired by Biogen.

Quell Therapeutics

Developing engineered T regulatory (T-reg) cell therapies

Key Announcements

Quell significantly expands clinical manufacturing capacity for its engineered Treg cell therapies through collaboration with Cell and Gene Therapy Catapult
23.09.21

Collaboration will facilitate an expanded manufacturing footprint to enable clinical supply of Quell's pipeline, including QEL-001 in liver transplantation and pipeline programs in neuroinflammation and autoimmunity. This will add a second GMP facility to Quell's manufacturing operations and allow the Company to efficiently develop and scale the production of its novel multi-modular engineered Treg cell therapies for clinical trials.

Key Media Coverage

Other news to note for Sept. 23, 2021
BioWorld 23.09.21

BioWorld reports that Quell Therapeutics, is expanding its manufacturing capacity to produce its engineered Treg cell therapies through a deal with Cell and Gene Therapy Catapult.