OPEN ORPHAN PLC (LON:ORPH)

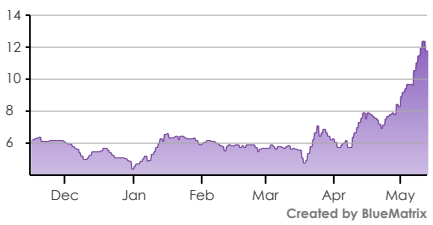
14 May 2020

proactive research

Pharmaceuticals, Biotechnology & Life Sciences

52-WEEK HIGH	**14p**
52-WEEK LOW	**5p**
PRICE	**11p**
MARKET CAP (M)	**£61**

Share Price



Major Shareholders

Cathal Friel: 8.4%	
Invesco: 9.6%	
Link Fund Solutions: 7.0%	
Shares in issue	549,038,274
Avg Three-month trading volume	7,317,040
Primary Index	AIM

Company Information

Address: 18 Fitzwilliam Place, Dublin Ireland
Website: www.openorphan.com

Analyst Details

Emma Ulker
emma.ulker@proactiveinvestors.com

Open Orphan: Scaling Up, Leveraging Valuable Assets

Open Orphan PLC (LON:ORPH) is a specialist contract research organisation (CRO) offering pharmaceutical services focused on orphan drugs. It is a world leader in the testing of vaccines and anti-viral treatments through the use of human challenge study services. With its hVIVO subsidiary now integrated, Open Orphan (ORPH) is leveraging its capabilities to the full, growing the top line and targeting near term profitability by delivering specialist pharma services across its hVIVO and Venn subsidiaries. The game-changing target to develop a coronavirus human viral challenge model would provide a unique service, potentially accelerating the development of vaccines and anti-viral treatments for COVID-19. ORPH looks to license FLU-v, a differentiated universal flu vaccine, developed by its 49% owned joint venture Imutex acquired through the hVIVO merger, offering risk-free upside prospects.

ORPH is rapidly growing its top line, building longer and higher value services contracts, and has already achieved significant cost efficiencies. Most recently it added two contracts; one for a biopharma company developing anti-viral and immunomodulatory therapies for coronavirus, another for the respiratory syncytial virus (RSV) worth £3.5mln in full-year 2020 (FY20). These new agreements are likely to be the tip of the iceberg and just by delivering a proportion of the hVIVO pipeline worth over £80mln the contracts could transform ORPH. The recent addition of COVID-19 antibody testing capability from Quotient (QTNT) has the potential to substantially increase Open Orphan's revenues given pent-up demand in the UK for antibody testing.

ORPH's game-changing near term target includes developing a coronavirus human viral challenge model (HVC) to safely and rapidly test new drugs or vaccines. The value of HVCs has been highlighted by the capacity to execute proof of concept work on novel therapeutics, such as vaccines and anti-viral drugs, including for coronavirus, and which can potentially accelerate the development of therapies and vaccines. This will be an entirely unique offering, likely to be valuable to the many companies developing COVID-19 vaccines and anti-viral treatments.

ORPH's 49% share in Imutex provides risk-free upside potential. The joint venture has a differentiated immunology pipeline including a multi-peptide, Phase III-ready universal flu vaccine programme for US/Europe. The vaccine is differentiated in ways that can overcome the challenges of current flu vaccines and lead to promising potential for licensing to pharma companies. These assets were developed prior to acquiring hVIVO, and with ongoing development to be exclusively funded via out-licensing/externally, they incur no risk or cost to ORPH. Recently management indicated that in cooperation with the SEEK Group, who own 51% of Imutex that they may look to monetise this asset through selling all of Imutex into an existing NASDAQ listed company, and via a dividend in specie; each Open Orphan shareholder would then have a share in a NASDAQ listed company as well.

We are impressed by ORPH's successful and rapid implementation of its growth strategy, and ongoing build of revenue streams via new higher value contracts, and the prospects of a potential near term launch of a coronavirus HVC can augment this. Licensing the FLU-v offers a Blue Sky scenario, and prospects look very promising given its differentiation.

Cathal Friel, chief executive officer. Founder of Open Orphan in 2016. Co-founder of Amryt Pharma PLC. Founder & chairman of Fastnet Oil & Gas. Co-founder and director of Merrion Stockbrokers before the £80mln sale in 2006.

Leo Toole, chief financial officer. Brings more than 20 years' experience in senior finance roles in Pharmaceuticals, Medical Technology and FMCG sectors.

Professor Brendan Buckley, non-executive chairman. Chief medical officer of ICON until 2017. Doctoral graduate in biochemistry of Oxford University. Member of the EMA Committee for orphan medical products, 2000-2003.

Recent industry deal precedent includes Sanofi Pasteur, which licensed SK Chemicals' influenza vaccine manufacturing technology in 2018 for up to US $155mln. In 2018 Pfizer and Germany's BioNTech formed a new collaboration worth up to US$425mln to create a new flu vaccine based on messenger RNA technology including US$120mln upfront paid to BioNTech. We look forward to more news flow that might include new contracts, licensing FLU-v or clarification of the recommended regulatory pathway, and further progress with the coronavirus challenge study development.

Creating a differentiated and valuable niche

ORPH has established a differentiated niche as a CRO achieving the successful integration of its hVIVO and Venn Life Sciences subsidiaries, which collectively offer world-class pharma services, and have a track record of delivering to leading pharma companies and research institutions.

In summary:

hVIVO offers the world's only 24-bed quarantine clinic and on-site virology laboratory including a world-leading portfolio of 8 controlled human infection viral study models.

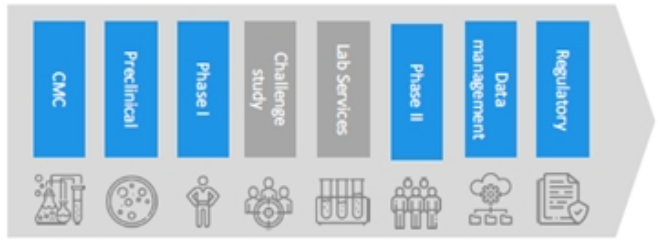
Venn Life Sciences provides CMC, preclinical, PK and medical writing, phase I & II clinical trials design and execution, post-trial data management, statistics, trial randomisation and regulatory expertise.

Company Structure

Source: Open Orphan

The combined business offers a focused and complementary range of services – from preclinical to Phase II studies, forming an attractive niche focused on anti-viral and orphan drugs and vaccines.

hVIVO and Venn Synergistic Services



Source: Open Orphan

Open Orphan aims to multiply up the average duration and value of contracts it agrees with its biopharma clients

ORPH's strategic aims are very clear; to achieve rapid top-line growth and profitability by leveraging capacity and revenue synergies while achieving operational efficiencies and cutting cost. It also aims to multiply up the average duration and value of contracts it agrees with its biopharma clients, from as low as £0.5mln per year previously prior to acquiring Venn and hVIVO, to between £10-£20mln typically across a 3-8 year contract. Achieving this would not only provide recurring revenues but also the opportunity to bring in-house a number of complementary services provided by the Venn subsidiary.

The transition is now largely complete following the transformational hVIVO merger in January, including headcount reduction leading to c £4mln in savings initially, and recently culminating in executive chairman Cathal Friel reaffirming his hands-on role to lead ORPH's vigorous strategy. The ongoing targets are to continue to leverage the excellent potential synergies of its services, notably including data management, biostatistics and medical writing, which can minimise outsourcing costs, all adding up to build a pipeline of higher value contracts over a longer duration.

The value of human viral challenge models has been highlighted recently for the capacity to execute proof-of-concept work on novel therapeutics, including vaccines, immunomodulators and antivirals, including for coronavirus, and which can potentially abbreviate the development of such therapies and vaccines.

The main principles of human viral challenge models are:

- To inoculate volunteers with a challenge virus in a controlled setting, and to monitor the impact on the disease of an experimental drug vs placebo. The target is to test the efficacy and safety of the drug and obtain proof of concept data much quicker than can be achieved in the field.
- Challenge studies enable efficacy endpoints to be monitored much earlier compared to conventional Phase I studies that can be used to minimise risk to volunteers, accelerate development and to generate broader endpoints to support ongoing regulatory studies.

ORPH's potentially game-changing near term target is to develop a coronavirus challenge study model — this can enable Phase I studies of anti-viral drugs and vaccines to be implemented — enabling healthy volunteers to be recruited, inoculated or treated, challenged with a virus and closely monitored through to recovery. The potential value of this approach was highlighted in an article published in Nature and which is attracting significant industry interest.

Challenge study model development requires data to be developed in order to recreate the conditions of the infection, including knowledge of symptoms and pathogenesis of the infection, and in particular the duration and nature of immune response, so it is likely to take up to six months to develop and test such a model. Clearly this will be an entirely unique offering likely to be valuable to the many companies developing vaccines and anti-viral treatments, including for other forms of coronavirus or in case of new strains entering circulation — helping to shorten development timelines and conduct studies more safely.

The recent addition of antibody testing capability through ORPH's contract with NASDAQ-listed Quotient is a significant enhancement. The CE-marked MosaiQ by Quotient system and MosaiQ COVID-19 Antibody Microarray provides a high degree of accuracy; 99.8% specificity and 100% sensitivity. Antibody testing to detect SARS COV-2 antibodies — which can show if a person has previously been infected with COVID-19 can be incorporated into the workflow or offered as a standalone service — within a safe and hygienic environment of the hVIVO quarantine facility.

The latter is the latest of a stream of new contracts announced since ORPH's acquisition of Venn, demonstrating that it is acting on its aim of building visibility of earnings and validating its expertise

New Contracts and a Growing Pipeline

In April hVIVO initiated testing on an anti-viral/anti-inflammatory treatment for COVID-19 through its new contract with Nearmedic International Ltd, a Moscow-based biopharma company. The contract highlights ORPH's virology expertise. hVIVO will be testing the drug against 'a panel of viruses to include influenza virus, "normal", circulating beta coronavirus and ultimately against COVID-19.' The deal terms were not disclosed although we estimate that delivering a coronavirus challenge model can help achieve incremental or higher revenues to realise the longer term potential of this and other contracts in the future.

A separate contract with a US biotechnology company for respiratory syncytial virus (RSV) human challenge study signed in May is projected to deliver £3.5mln in revenue in 2020, and illustrates the value of ORPH's complementary in#house CRO services of hVIVO and Venn Life Sciences following the completion of the merger with hVIVO.

The latter is the latest of a stream of new contracts announced since ORPH's acquisition of Venn, demonstrating that it is acting on its aim of building visibility of earnings and validating its expertise. We anticipate that this is the tip of the iceberg since the challenge study clinic can run up to around 13 studies per year at peak capacity; the company has stated that the hVIVO pipeline of identified and pitched for contracts building towards over £80mln, so even delivering a proportion of this can be transformational for ORPH.

All studies conducted to date have met primary endpoints including appropriate induction of immune response

Imutex provides risk-free upside; ORPH's 49% ownership of Imutex a joint venture formed in 2016 with SEEK Group provides upside to its underlying CRO business — through three differentiated and potentially attractive immunology assets;

- FLU-v - a differentiated multi-peptide, Phase III-ready universal flu vaccine programme in US/Europe.
- AGS v — a Phase I vaccine for mosquito saliva borne diseases including Zika virus/malaria - Phase Ib study completed by National Institutes of Health (NIH) — external funding for follow on studies via NIH/Innovate UK.
- A proprietary peptide modelling platform with potential applications in vaccine development including against COVID 19.

Notably, these assets are being funded externally, so they present opportunity for off-balance-sheet monetisation, including via licensing, so they incur no risk or cost to ORPH.

The vaccine is differentiated in ways that can overcome the challenges of current flu vaccines and lead to interesting potential for licensing to pharma companies. These include vaccination frequency (estimated once every five years) and strain coverage (pandemic and A & B in both humans and animals).

All studies conducted to date have met primary endpoints including appropriate induction of immune response. Notable benefits also include avoiding the limits of scale-up posed by the traditional vaccine development in hens' eggs. Phase IIb results were recently published in a peer-reviewed journal the **Annals of Internal Medicine**.

Positive outlook; the company is delivering on its buy and build growth strategy and can multiply up its commercial prospects, by achieving maximum capacity by continuing to successfully grow its service contract base, and in particular by launching its coronavirus challenge study model. A deal for the Universal flu vaccine is a blue kky scenario for the company although prospects are very promising. Recent deal precedent includes Sanofi Pasteur which licensed SK Chemicals influenza vaccine manufacturing technology in 2018 for up to US $155mln.

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London
+44 207 989 0813
The Business Centre
6 Wool House
74 Back Church Lane
London E1 1AF

New York
+1 347 449 0879
767 Third Avenue
Floor 17
New York
NY 10017

Vancouver
+1 604-688-8158
Suite 965
1055 West Georgia Street
Vancouver, B.C. Canada
V6E 3P3

Sydney
+61 (0) 2 9280 0700
Suite 102
55 Mountain Street
Ultimo, NSW 2007