Syncona Limited

Syncona founds Purespring with a £45m Series A Financing

- Purespring is one of the first AAV gene therapy companies focused on the kidney globally
- This represents the sixth AAV gene therapy company to be founded by Syncona, demonstrating deep expertise in this area
- Syncona will have an 84 per cent stake in the business

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Syncona Ltd, a leading healthcare company focused on founding, building and funding a portfolio of global leaders in life science, announces the foundation of a new Syncona company, Purespring Therapeutics (Purespring), with a £45.0 million Series A financing.

Purespring is one of the first AAV gene therapy companies focused on the kidney globally. It has been founded around the seminal work of Professor Moin Saleem, Professor of Paediatric Renal Medicine at the University of Bristol, where he heads a world leading group researching glomerular diseases. Purespring will seek to advance gene therapies for the treatment of chronic renal diseases that are currently poorly addressed with existing treatments.

The company will also establish an in-vivo functional screening platform, FunSel, to initially screen for protective factors that could have applications across several kidney diseases. FunSel has been developed by Professor Mauro Giacca, a leader in gene therapy of cardiovascular disorders, and provides Purespring with a target discovery platform uniquely suited to systematically move gene therapy beyond monogenic disorders.

Syncona's £45.0 million commitment to Purespring will fund the company build-out and will enable it to progress to being clinical stage. Syncona's Chief Investment Officer, Chris Hollowood, has been appointed as Chairman and Syncona Partner Dominic Schmidt will be joining the Board of Directors. The Syncona team will work closely with Purespring as it builds out its operations and management team.

Dominic Schmidt, Partner, Syncona Investment Management Limited, said: "We believe that the foundation of Purespring represents a unique opportunity to build the global leader in renal gene therapy, where we will have a strong early mover advantage combined with a bespoke platform and world-class individuals. It is one of the first companies targeting the kidney using AAV gene therapy and will seek to treat a number of debilitating chronic diseases that are currently poorly addressed with existing therapies in line with Syncona's ambitions to deliver transformational treatments to patients."

Chris Hollowood, CIO, Syncona Investment Management Limited, said: "Purespring is the sixth gene therapy company to be founded by Syncona and clearly demonstrates our proprietary company creation approach. In Moin and his team, we are collaborating with clinical and scientific leaders and working in target tissue amenable to gene therapy, whilst the collaboration with Mauro provides a path for gene therapy to fulfil its promise in highly prevalent chronic degenerative conditions. We look forward to building a world class company around this innovative science, in order to develop therapies with the potential to deliver dramatic impact for patients. Purespring is an exciting addition to our gene therapy platform, where we are strategically positioned with significant expertise in building fully integrated platform companies."

Professor Moin Saleem, Head of Bristol Renal, said: "This is an incredible opportunity to develop transformational treatments for kidney disease. Gene therapy has come of age in certain areas, but a major challenge in complex solid organs is to precisely target the genetic material to the correct cell type. Using accumulated expertise in the Bristol Renal research group we have solved this crucial hurdle, putting us in a position to deliver curative gene therapy to patients with chronic and intractable kidney diseases. Syncona have had the foresight to see this potential and partnering with their world-

leading gene therapy experience is the best possible springboard to successfully bring this technology to patients."

Professor Mauro Giacca, Professor at King's College London and the University of Trieste and former Director-General of ICGEB, said: "I am very excited to take part in this brilliant new initiative. Not only do AAV vectors hold tremendous promise for the genetic therapy of kidney hereditary disorders but they are also unique tools for the discovery of new therapeutics for common degenerative conditions.**"**

Syncona has significant expertise in gene therapy and Purespring is the sixth Syncona gene therapy company to be founded since 2012 underling the Company's leadership position in the gene therapy. Each Syncona gene therapy company has had a distinct product focus and domain expert teams that focus on high quality and rapid execution.

Alongside Purespring, Syncona's gene therapy companies have included: Nightstar (inherited retinal diseases and sold to Biogen for $977.0 million, Freeline (chronic systemic disease), Gyroscope (retinal inflammation and one of the first companies globally to move gene therapy from rare disease to highly prevalent severe diseases with no treatment options), Orbit Biomedical Device (a leading sub-retinal surgical delivery platform for delivering gene therapies to the eye, since merged with Gyroscope) and SwanBio (a CNS gene therapy company).

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Forward-looking statements - this announcement contains certain forward-looking statements with respect to the portfolio of investments of Syncona Limited. These statements and forecasts involve risk and uncertainty because they relate to events and depend upon circumstances that may or may not occur in the future. There are a number of factors that could cause actual results or developments to differ materially from those expressed or implied by these forward-looking statements. In particular, many companies in the Syncona Limited portfolio are conducting scientific research and clinical trials where the outcome is inherently uncertain and there is significant risk of negative results or adverse events arising. In addition, many companies in the Syncona Limited portfolio have yet to commercialise a product and their ability to do so may be affected by operational, commercial and other risks.

About Syncona

Syncona (LON: SYNC) is a healthcare company focused on founding, building and funding a portfolio of global leaders in life science. Our purpose is to invest to extend and enhance human life. We do this by founding and building companies to deliver transformational treatments to patients in areas of high unmet need.

Our strategy is to found, build and fund companies around exceptional science to create a dynamic portfolio of 15-20 globally leading healthcare businesses for the benefit of all our stakeholders. We focus on developing treatments for patients by working in close partnership with world-class academic founders and management teams. Our strategic balance sheet underpins our strategy enabling us to take a long-term view as we look to improve the lives of patients with no or few treatment options, build sustainable life science companies and deliver strong risk-adjusted returns to shareholders.

About ICGEB and FunSel

Established in 1983 as a special project of UNIDO, the International Centre for Genetic Engineering and Biotechnology - ICGEB is an independent intergovernmental organisation since 1994 with HQ in Trieste (Italy) and with additional laboratories in New Delhi (India) and Cape Town (South Africa). As of today, it counts 65 Member States and 20 signatory countries. The ICGEB is a not for profit IGO – any revenues generated are re-invested in research and in the funding programmes for capacity building in its Member States. The Vision of the ICGEB is to be the world's leading intergovernmental Organisation for research, training and technology transfer in the field of Life Sciences and Biotechnology. Its Mission is to combine scientific research with capacity enhancement, thereby promoting sustainable global development (www.icgeb.org).

FunSel is an in-vivo functional screening platform. It was developed at ICGEB by Professor Giacca and his team while he served as the Director-General of the organisation until 2019. He continues to head the Molecular Medicine laboratory at ICGEB Trieste, Italy.