Solid Biosciences, LLC Logo

Synpromics Announces Gene Therapy Research Partnership with Solid Biosciences

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Synpromics Ltd, the leader in gene control, today announces a new research partnership with Solid Biosciences, LLC. Under the terms of the agreement, Synpromics will provide Solid Biosciences access to a set of key muscle-selective promoter candidates to be used in the development of new treatment options for Duchenne muscular dystrophy (DMD).

The use of Synpromics' synthetic promoters will enable the enhancement of Solid Biosciences' investigational gene therapy candidates for the treatment of DMD. These muscle-selective promoters have been designed using Synpromics' PromPTÒ technology and bioinformatics expertise, and will be evaluated by Solid Biosciences for optimal product specific gene control.

> "We are excited to leverage our PromPTÒ technology platform to generate promoter candidates closely matching the precise criteria and specific product requirements for Solid Biosciences' muscle gene medicine programme in Duchenne," David Venables, CEO of Synpromics, commented. "The collaboration between the scientific teams will enable the effective and rapid in vivo evaluation of the candidate promoters as compared to industry standards."

"Synpromics' cutting-edge technology has the potential to enhance our gene therapy development efforts, helping us to fulfil our promise to bring meaningful therapies to all patients with this devastating disease," said Joel Schneider, PhD, Chief Technology Officer, Head of Exploratory R&D at Solid Biosciences. "This type of collaboration is key for building our next-generation gene therapy portfolio as we focus on enhancing fundamental aspects of our gene delivery and protein expression capabilities."

Duchenne muscular dystrophy (DMD) is a genetic muscle-wasting disease that is progressive, irreversible and ultimately fatal. DMD affects approximately one in every 3,500 to 5,000 live male births. Symptoms of DMD usually manifest between three to five years of age. As the disease progresses, patients are typically wheelchair-bound by their early teens and succumb to respiratory or heart failure in early adulthood. There is no cure for DMD and, for the vast majority of patients, there are no satisfactory treatments. This research collaboration will help advance Solid's programme to develop optimal product candidates for the company's AAV muscle-directed gene therapy research programme.

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Notes to Editors

About Synpromics

Synpromics is the leader in gene control, improving human health by enabling safer, more effective cell and gene medicines through proprietary genomics, bioinformatics and intelligent data-driven design. The company has developed PromPTÒ, its multi- dimensional bioinformatics database that enables productspecific promoter design and selection empowering the next generation of cell and gene based medicines and bioprocessing applications. The company operates in a diverse range of fields, including broad applications in cell and gene based medicine, biologics manufacturing and viral vector bioprocessing. Current partners include Adverum, uniQure, AGTC, GE Healthcare, Homology Medicines, Inc and Sartorius-Stedim Cellca as well as numerous undisclosed partners in the pharmaceutical sector.

About Synthetic Promoters

Naturally occurring promoters have evolved for biological functions but have limitations when utilised in industrial or therapeutic applications. Synthetic promoters with DNA sequences not found in nature are designed to better regulate gene activity and precisely control protein production. Synpromics creates highly specific promoters designed to drive gene expression at the desired level and specificity in any cell type, tissue or environmental condition. Each synthetic promoter represents a novel invention and thus can be patented.

For more information visit www.synpromics.com