Addressing unmet needs for inherited neuromuscular diseases

Novartis has acquired Kate Therapeutics to further enhance and strengthen our ongoing efforts to advance gene therapies for patients.

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- *Acquisition of Kate Therapeutics enhances and strengthens Novartis strategic priorities in gene therapy and neuroscience innovation*
- *Transaction includes enabling technology platforms and therapeutic candidates for DMD, FSHD and DM1, currently in preclinical development*
- *Transaction value of up to USD 1.1bn in total, comprising an upfront payment and potential additional milestone payments*

Neuromuscular diseases are a group of disorders that impair the functioning of muscles and nerves, disrupting nerve signal transmission and muscle contraction and movement. Many of these disorders are genetic in nature and present significant challenges to patients and their families.

At Novartis, we are committed to addressing unmet needs for patients living with inherited neuromuscular diseases. This includes innovating novel gene therapies, which aim to address these diseases by either replacing faulty genes or blocking the effects of toxic RNA or proteins. To further enhance and strengthen our ongoing efforts to advance gene therapies for patients, Novartis has acquired Kate Therapeutics, a San Diego-based, preclinical stage biotechnology company, focused on developing adeno-associated virus (AAV)-based gene therapies to treat genetically defined neuromuscular diseases.

“We are delighted to bring Kate Therapeutics’ platform technologies and programs together with Novartis scientific expertise and leadership in gene therapy and neuroscience innovation,” said Fiona Marshall, President of Biomedical Research at Novartis. “We have been highly impressed with the rigor and potential of Kate’s science, and we are confident this acquisition will further enhance our ability to bring forward new therapeutic options for patients living with neuromuscular diseases.”

Kate Therapeutics’ primary programs include preclinical candidates for Duchenne muscular dystrophy (DMD), facioscapulohumeral dystrophy (FSHD), and myotonic dystrophy type 1 (DM1). Kate’s Directed Evolution of AAV Capsid Leveraging *In Vivo* Expression of Transgene RNA (DELIVER) platform combines diverse capsid library generation with transcript-based *in vivo* selection and machine learning to evolve functional capsid variants. DELIVER has been used to evolve a novel class of liver de-targeted muscle-tropic capsids, which transduce both skeletal muscle and cardiac tissue with potency and selectivity *in vivo*, while avoiding the liver.

“This acquisition builds on our expertise and leadership in neuroscience drug discovery and brings to Novartis talent, expertise and capabilities that are highly complementary to our ongoing internal efforts,” said Robert Baloh, Global Head of Neuroscience Research at Novartis. “It reflects our commitment to addressing unmet medical needs in neuroscience and tackling the limitations of existing gene therapies for patients with inherited neuromuscular conditions.”

Kate Therapeutics’ technology platforms integrate capsid and cargo technologies to deliver payloads to

desired tissues, while potentially mitigating off-target effects to tissues such as the liver. This approach aims to improve both the efficacy and safety of gene therapies, opening potential possibilities for treating complex diseases previously difficult to address with current technologies, including inherited neuromuscular diseases.

Under the terms of the agreement, Kate Therapeutics' shareholders are entitled to receive up to USD 1.1bn, comprising a cash payment that was paid at closing of the transaction and additional amounts payable on achievement of specified milestones.

Learn more about Neuroscience research at Novartis.

Learn more about Novartis Gene Therapies.

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