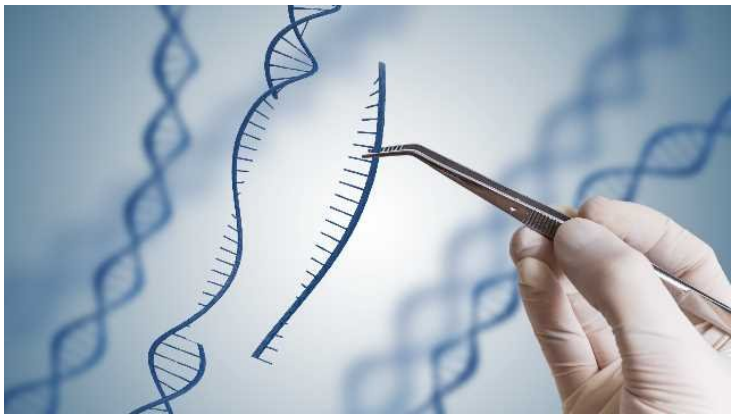
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Novo Nordisk, bluebird bio plan next-gen in vivo genome editing

October 11, 2019 | News

For haemophilia and other severe genetic diseases



US headquartered bluebird bio, Inc. and Denmark based Novo Nordisk have announced that they have entered into a three-year research collaboration to jointly develop next-generation in vivo genome editing treatments for genetic diseases, including haemophilia.

During the three-year research collaboration, bluebird and Novo Nordisk will focus on identifying a development gene therapy candidate with the ambition of offering people with haemophilia A a lifetime free of factor replacement therapy.

The research collaboration will utilise bluebird bio's proprietary mRNA-based megaTAL™ technology that has the potential to provide a highly specific and efficient way to silence, edit or insert genetic components.

Aligned with Novo Nordisk's haemophilia portfolio, the research collaboration will initially focus on correcting FVIII-clotting factor deficiency, with the potential to explore additional therapeutic targets.

"We are pleased to announce our collaboration with bluebird whose demonstrated capabilities in gene therapy will enable the next-generation of innovative products to make a significant impact on patients' lives," said Marcus Schindler, senior vice president for Global Drug Discovery in Novo Nordisk. "This important research collaboration aimed at addressing genetic diseases at the DNA level reflects Novo Nordisk's enduring commitment and dedication to inventing disease-modifying medicines that can truly change the lives of people living with haemophilia and other genetic diseases."

"bluebird has made tremendous progress on enabling an in vivo gene editing platform based on our megaTAL technology, including important advances in high-quality mRNA production and purification," said Philip Gregory, D. Phil, chief scientific officer, bluebird bio. "We believe this technology has the potential to create a highly differentiated approach to the treatment of many severe genetic diseases. Moreover, we are thrilled to be able to combine this new platform technology with Novo Nordisk's deep expertise in haemophilia research and therapeutics. We believe this collaboration will move us toward our shared goal of recoding the treatment paradigm and substantially reduce the burden of disease for patients with factor VIII deficiency."