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bluebird bio and Novo Nordisk enter into research agreement

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The research collaboration will utilise bluebird bioâ??s proprietary mRNA-based megaTALâ?¢ technology



bluebird bio and 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.

Marcus Schindler, senior vice president for Global Drug Discovery in Novo Nordisk said, "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. 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."

Philip Gregory, D. Phil, chief scientific officer, bluebird bio said, "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. 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."

MegaTALs are a single-chain fusion enzyme that combines the natural DNA cleaving processes of Homing Endonucleases (HEs) with the DNA binding region of transcription activator-like (TAL) effectors. TALs are easily engineered proteins that recognize specific DNA sequences. This protein fusion architecture allows the generation of extremely active and highly specific and compact nucleases that are compatible with all current viral and non-viral cell delivery methods.

For people with haemophilia, bleeds often occur in the joints, particularly knees and ankles. Bleeds can also occur in the muscles, soft tissues, gastrointestinal tract or even the brain. Trauma, major surgery, tooth extractions or other minor surgical interventions require medical supervision to manage the associated bleeding. Without treatment, bleeds are painful and can cause lasting damages and lead to impaired mobility.