UCLB Commercialising Technology

AAV Gene Therapy for Diabetes

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Available for: Licensing

Summary

AAV gene therapy for diabetes, is a novel technology with the potential to treat diabetes type I through a stable endogenous expression of human insulin from the liver after one single administration.

Background

Diabetes, a widespread metabolic disorder, impacts approximately 3.2 million individuals in the United Kingdom and over 347 million people globally, causing 1.5 million deaths annually and exerting pressure on healthcare budgets. For instance, the National Health Service (NHS) spends approximately £10 billion annually on diabetes, accounting for 10% of its overall budget.

Technology

The research team developed a novel codon-optimized human insulin cDNA by introducing 50 nucleotide changes to the 334 bp wild type human insulin sequence. This resulted in a unique codon-optimized insulin sequence (SCAAV-HLP-codop-bINS) that was 85% similar to the wild-type sequence.

This liver-directed AAV gene therapy suggests promising potential for the treatment of type I diabetes in humans through continuous and long-term endogenous expression of human insulin in the liver after a single peripheral vein administration in patients with type I diabetes. Some benefits of this approach include:

- A single peripheral vein infusion of AAV encoding the optimized sequence leads to long-term endogenous expression of human insulin in type I diabetes patients.
- The codon-optimized expression cassette allows for more potent expression, enabling therapeutic benefits with lower doses of the AAV vector.
- Expression of insulin from the liver reduces the risk of developing neutralizing antibodies to this protein.
- It eliminates the need for lifelong insulin injections, improving quality of life and potentially reducing healthcare costs.

Pre-clinical experimental on STZ-treated diabetic mice showed that mice treated with the AAV codon-optimized cDNA significantly reduced blood glucose levels, indicating liver-mediated expression of human insulin.

Stage of development

The vector is optimised and ready for pre-clinical toxicology studies and then clinical studies.

Market

Accounting for approximately 1.6 million diagnoses cases in the US alone and with an increasing number of individuals affected by life-style-related factors contributing to the rise in diabetes incidence, the market size is poised for expansion.

In 2022, the global market size of Type 1 Diabetes was valued at USD 7.59 billion, with projections indicating an increase to around USD13.64 billion by 2030.

AAV gene therapy holds the potential to capture a significant portion of the diabetics therapeutics market, meeting the demands of a large and growing patient population worldwide.

Team

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IP

Granted patent in Switzerland, Germany, Spain, France, Italy, UK and US (EP3356395)

Further Information

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