

New Adaptable Nanoparticle Platform Enables Enhanced Delivery of Gene Therapies

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Scientists have developed polypeptide-based materials that act as effective vectors for delivering gene therapies. The first-of-its-kind platform enables the vectors to be adapted to suit the specific gene therapy cargo.

The work, led by researchers from [RCSI University](#) of Medicine and Health Sciences and funded by Science Foundation [Ireland](#), is published in [Biomaterials Science](#).

A major challenge for gene therapies is preparing them in a way that can deliver the genetic information into the host cells. For the Covid-19 vaccines that use mRNA technology, the genetic information is delivered in a lipid nanoparticle to maintain its stability and deliver it into cells. The success of the COVID vaccines has established nanoparticles as key to the development of many advanced therapies.

The researchers developed a platform that produces bespoke star-shaped polypeptide nanoparticles, which effectively deliver a range of therapies, including gene therapies. Crucially, these polypeptides are more flexible and easier to handle than lipids. To demonstrate the potential of this material, the researchers used it to deliver a gene therapy that regenerated bone.

In preclinical work, the researchers loaded the material with DNA molecules that promote bones and blood vessels to regrow. They placed these nanomedicines in a scaffold that could be implanted into a defect site and deliver the genetic cargo into infiltrating host cells. The gene-loaded scaffold accelerated bone tissue regeneration, with a six-fold increase in new bone formation compared to a scaffold alone.

“With the success of the COVID-19 vaccines, the potential of gene therapies is becoming apparent, and advanced nanoparticle delivery systems are key to enabling their use clinically. We have shown that these nanoparticles have real potential to be a game changer in the delivery of gene therapies,” said Professor Sally-Ann Cryan, the study’s senior author and Professor of Drug Delivery, RCSI.

“While more testing is needed before these therapies can be used clinically, our platform allows us to design our polypeptides to meet a variety of delivery scenarios and provide tailored solutions to gene delivery challenges,” added Professor Andreas Heise, project collaborator and Professor of Polymer Chemistry, RCSI.

“We are developing this patent-protected technology towards commercialisation, with support from an Enterprise [Ireland](#) Commercialisation Fund Award, and are seeking expressions of interest from industry partners and investors.”

Read the [original article](#) on Royal College of Surgeons in [Ireland](#).