Nanoparticle-delivered CRISPR tools could treat hemophilia, cystic fibrosis and muscular dystrophy

More and more scientists are using the powerful new gene-editing tool known as CRISPR/Cas9, a technology isolated from bacteria, that holds promise for new treatment of such genetic diseases as cystic fibrosis, muscular dystrophy and hemophilia. But to work well, the new gene-clipping tool must be delivered safely across the cell membrane and into its nucleus, a difficult process that can trigger the cell's defenses and "trap" CRISPR/Cas9, greatly reducing its treatment potential.

Now, researchers in nanochemistry expert Vincent Rotello's laboratory at the University of Massachusetts Amherst have designed a delivery system using nanoparticles to assist CRISPR/Cas9 across the membrane and into the cell nucleus while avoiding entrapment by cellular machinery.

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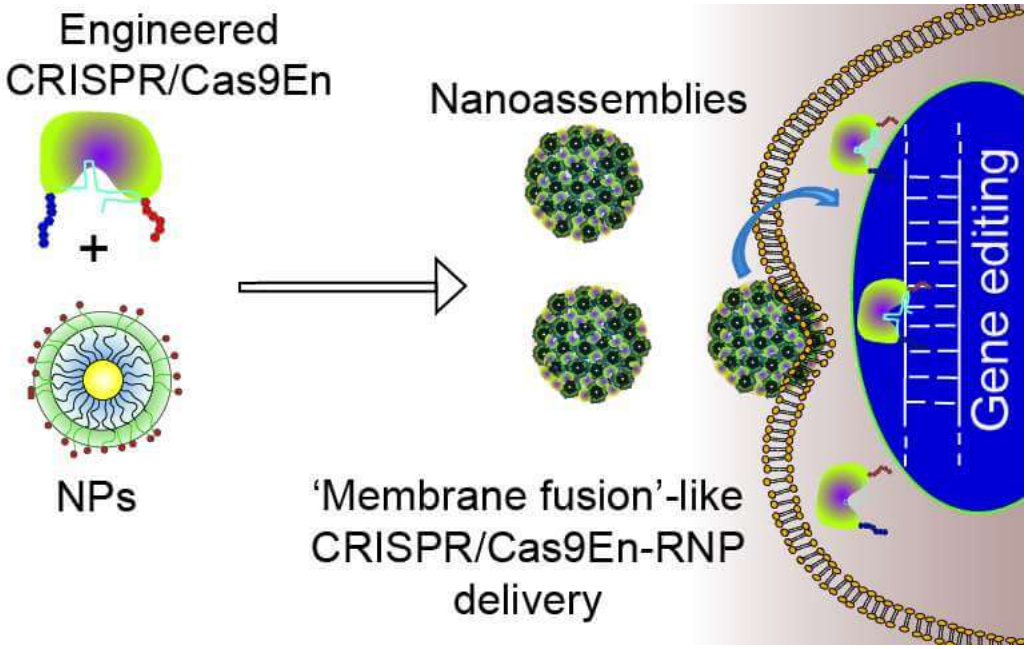
He points out that since CRISPR's potential was first discovered in 2012, gene editing or genome engineering has quickly become an intense research topic in biology and medicine. The goal is to treat otherwise incurable genetic diseases by manipulating diseased genes. "However, to achieve this, biotech and pharmaceutical companies are constantly searching for more efficient CRISPR delivery methods," he adds.



A cartoon shows gene editing through engineered CRISPR/Cas9En delivery in the Rotello lab at UMass Amherst. The researchers have overcome an obstacle in the technology by designing a delivery

system using nanoparticles to assist CRISPR/Cas9 across the cell membrane and into the nucleus while avoiding entrapment by cellular machinery. Credit: UMass Amherst.

[The study can be found here.]

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post: Overcoming hurdles in CRISPR gene editing to improve treatment