Cutting gene therapy side effects by finding a better delivery system to the brain

Researchers have found a structure on the small viruses that deliver gene therapy that makes them better at crossing from the bloodstream into the brain.

This is a key factor for administering gene therapies at lower doses to treat brain and spinal disorders, which could reduce the number of adverse side effects, researchers say.

…

The study, which appears in Molecular Therapy, examined adeno-associated viruses (AAVs), the most commonly used virus vectors for delivering gene therapies. The natural forms of these small viruses normally infect people without causing disease. For gene therapies, scientists remove most of the AAV genome, replace it with therapeutic genetic cargo, and inject trillions of copies into the patient.

…

[T]hey isolated a closely spaced set of just eight amino acids on the viral coating that confers the ability to cross the blood-brain barrier efficiently. “Grafting that structural footprint onto another AAV strain enables it to cross into the brain much more easily,” [researcher Blake] Albright says.
The finding suggests that other AAVs used for a gene therapy targeting the brain or spinal cord might be improved by having the same or a similar set of amino acids. It would cross the blood-brain barrier more efficiently, and thus in principle would require a smaller dose to achieve therapeutic effects in the brain.

Read full, original post: Better way into brain could cut gene therapy side effects