Docket #: S23-214

AAV-Titer: Cell Lines for Improved Quality Control & Dosing of Clinical Gene Therapies

Scientists in the Carette Lab at Stanford have developed AAV-Titer cell lines that enable (1) improved and standardized in vitro potency assays (2) determination of a functional titer of AAV vectors of different serotypes and containing different promotors.

Quality control of gene therapy products is key to effective and safe clinical deployment. One hurdle to appropriate quality control is that most clinical AAV vectors do not transduce efficiently in tissue culture systems, making it difficult to measure titer and potency, which are important for quality assurance and dosing. Currently, the quality control of gene therapy products based on AAV vectors consists of determining the physical characteristics of the particles and a crude measure of "genome titer," which does not directly measure the ability of the viral particle to infect cells. This can lead to viral preparation varying widely in their quality and potency.

To allow for direct in vitro measurement of AAV potency, Stanford inventors have created a cell line with greatly enhanced susceptibility to AAV transduction. By tuning AAV receptor (AAVR) expression and inhibiting one key gene, the inventors have engineered AAV-Titer, cell lines that are highly suitable for functional titration and potency assay development for clinical gene therapy.

Stage of Development
Prototype cell line currently available

Applications

- Quality control and R&D of clinical gene therapies

Advantages

- More accurate and consistent titer measurement for AAVs used in clinical gene therapies
- Better quality AAVs in gene therapies leading to better and more predictable clinical response for clinical trials and treatment

Patents

- Published Application: WO2025024460

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