Enhancing Purity and Safety of AAV Vector Preparations for Gene Therapy

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A novel method has been developed to resolve the mass landscape of whole viral particles, capsid intermediates, and viral genomes present in adeno-associated virus (AAV) vector preparations based on charge detection mass spectrometry (CDMS). CDMS not only provides an accurate analysis of diversity within AAV vector preparations, but it can dramatically shorten the analysis time. CDMS can also enhance current quality control protocols for AAV vector analysis, help identify potential contaminants, and also shed light on AAV capsid dynamics during the infectious pathway. This method will have a significant impact on clinical AAV vector preparations which often contain variable levels of full or partial-genome containing particles and empty vectors. Consequently, the composition of these preparations can influence vector dose-related immunotoxicity in patients. This novel method has the potential to greatly increase the efficiency, purity, and safety of AAV therapy.
Features and Advantages

- Novel method that analyzes the entire landscape of AAV vector preparations
- Can establish the mass and stoichiometry of AAV capsids complexed with exogenous proteins, nucleic acids, or carbohydrates
- Determines the purity of recombinant AAV preparations for clinical applications
- Shortens the analysis time from hours to minutes, and can be seamlessly integrated into current quality control protocols

Related Publications:

- Resolving Adeno-Associated Viral Particle Diversity With Charge Detection Mass Spectrometry

Inventors

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