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Extracellular vesicles as modulators of immune responses to Adeno-Associated Virus (AAV) gene therapy vectors

Stanford researchers have developed a novel technology using extracellular vesicles (EVs) to selectively suppress immune responses to AAV vectors, enabling safer and more effective gene therapy.

Adeno-associated virus (AAV) gene therapy faces a critical safety barrier: severe immune reactions that have caused patient deaths and derailed major clinical programs. Current solutions depend on prolonged use of broad immunosuppressants, which blunt the entire immune system and expose patients to life-threatening complications. These risks jeopardize both patient safety and the commercial viability of AAV therapies.

To address this issue, Stanford researchers have developed a technology that uses extracellular vesicles (EVs) to specifically modulate innate immune responses to AAV vectors in human immune cells. EVs can be co-administered with AAV therapy, targeting immune activation precisely when it occurs, without the need for prolonged systemic immunosuppression. By acting directly at the source of immune recognition, EVs prevent dangerous inflammatory cascades, reduce or eliminate the need for broad immunosuppressants, and enable the safe delivery of therapeutic AAV doses. This targeted approach not only improves patient safety but also de-risks clinical programs, offering a clear pathway to effective, commercially viable gene therapies.

Stage of Development:
Research – in vitro

Applications

- Gene therapy developers
- Clinicians
- Patients

Advantages

- Targeted modulation
- Safe as it reduces risk of infections and sepsis
- Co-administered with therapy
- De-risks development

Innovators

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