



APP ABSTRACT - APP 2026 - 092

REWRITING AUTISM AT THE GENETIC LEVEL : EMERGING GENE THERAPY STRATEGIES TRANSFORMING AUTISM SPECTRUM DISORDER TREATMENT – A COLLECTIVE REVIEW

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Abstract:

Autism Spectrum Disorder (ASD) is a complex neurodevelopmental condition marked by repetitive behaviors and social communication challenges. While traditional treatments like behavioral therapy and antipsychotics manage symptoms such as irritability and hyperactivity, they do not address the underlying biological causes. Recent genomic research has identified mutations in genes responsible for synaptic development and neuronal signaling—such as SHANK3, MECP2, FMR1, and CNTNAP2—as primary drivers of the disorder. The review examines the application of three innovative molecular pillars designed to target these genetic roots: Viral Vector-Mediated Gene Delivery: Utilizing engineered adeno-associated viruses (AAVs), specifically serotypes like AAV9, to deliver functional "replacement" copies of genes across the blood-brain barrier. CRISPR-Cas9 Genome Editing: Acting as "molecular scissors," this complex uses a guide RNA to physically cut and permanently repair DNA mutations or reactivate silenced genes. Antisense Oligonucleotides (ASOs): Functioning as a "genetic dimmer switch," these synthetic strands target mRNA to silence faulty genes or modify protein splicing without permanently changing the host genome. Improved synaptic communication between neurons.

Restoration of neuronal network function. A measurable reduction in autism-like behavioral abnormalities. The potential for long-lasting, disease-modifying outcomes rather than lifelong symptom management.

Gene therapy represents a paradigm shift toward precision medicine for ASD. While the potential for reversing core deficits is high, significant hurdles remain, including ensuring the safety of brain-wide delivery and minimizing off-target genetic effects. Future progress depends on advancing early genetic diagnosis and conducting rigorous clinical trials to transition these "molecular tools" into standard clinical practice.

Keywords: Autism Spectrum Disorder, Genome Editing, Antisense Oligonucleotide Therapies, Viral Vector-Mediated Delivery