

[PTC-AADC gene therapy shows holistic improvements in children with AADC deficiency](#)

PTC Therapeutics shows five-year results for its novel gene therapy, PTC-AADC, which leads to profound improvements in children with aromatic L-Amino acid decarboxylase (AADC) deficiency, a previously intractable, fatal and devastating rare disorder of the central nervous system. Children treated with PTC-AADC developed motor function and cognitive skills not previously seen, such as holding up their head, sitting or standing with support and communicating, and these persisted for up to 10 years. PTC-AADC is currently under review by the European Medicines Agency's Committee for Medicinal Products for Human Use. A Biologics License Application is expected to be submitted to the U.S. Food and Drug Administration by the end of 2021.

[Voyager Therapeutics and Pfizer enter License Option Agreement for AAV capsids](#)

Voyager Therapeutics entered an agreement through which Pfizer may exercise options to license novel capsids generated from Voyager's RNA-driven TRACER™ (Tropism Redirection of AAV by Cell-type-specific Expression of RNA) screening technology as part of Pfizer's efforts to develop, manufacture, and commercialize gene therapies, utilizing two undisclosed transgenes to treat certain neurologic and cardiovascular diseases.

[Solid Biosciences reports improved lung function for DMD clinical trial](#)

Solid Biosciences' gene therapy trial for SGT-001 for Duchenne Muscular Dystrophy (DMD) improves lung function in six patients as part of the IGNITE DMD Phase I/II clinical trial. The improvements were seen a year after a single infusion of the SGT-001 gene therapy into the vein. SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy that systemically delivers a synthetic dystrophin gene, called microdystrophin, to the body.

[Forge Biologics and Solid Biosciences collaborate on viral vector contract development and cGMP manufacturing](#)

Forge Biologics, a cell and gene therapy-focused contract development and manufacturing organization (CDMO) and Solid Biosciences, working in Duchenne Muscular Dystrophy gene therapy have entered a partnership to advance the development and manufacturing of SGT-003, Solid's next generation gene therapy program for Duchenne. SGT-003 is a preclinical candidate that combines a next-generation and rationally designed capsid with Solid's proprietary nNOS-containing microdystrophin and has demonstrated enhanced muscle tropism and microdystrophin expression compared to AAV9 in vivo. Forge will provide an adeno-associated viral (AAV) vector process, scale-up engineering and cGMP manufacturing services for SGT-003.

[Sarepta Therapeutics initiates EMBARK, a global study for SRP-9001 gene therapy](#)

Sarepta Therapeutics, in partnership with Roche, is initiating EMBARK, a pivotal study of SRP-9001 for the treatment of Duchenne muscular dystrophy. SRP-9001 is an investigational gene transfer therapy intended to deliver the micro-dystrophin-encoding gene to muscle tissue for the targeted production of the micro-dystrophin protein. Sarepta is responsible for global development and manufacturing for SRP-9001 and plans to commercialize SRP-9001 in the United States upon receiving FDA approval.