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Adeno-associated virus vectors for human gene therapy

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Abstract

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Adeno-associated virus (AAV) is a small, non-enveloped virus that contains a single-stranded DNA genome. It was the first gene therapy vector approved in the Western world in November 2012 for clinical use to treat patients with lipoprotein lipase deficiency (LPLD). AAV made history and put human gene therapy in the forefront again. More than four decades of research on AAV vector biology and human gene therapy has generated a huge amount of valuable information. Over 100 AAV serotypes and variants have been isolated and at least partially characterized. A number of them have been used for preclinical studies in a variety of animal models. Several AAV vector production platforms, especially the baculovirus-based system have been established for commercial-scale AAV vector production. AAV purification technologies such as density gradient centrifugation, column chromatography, or a combination, have been well developed. More than 117 clinical trials have been

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