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The History of 40 Years of AAV Gene Therapy

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Work on the development of AAV vectors for human gene therapy began in the late ‘70s and continued over the next 40 years to where we are today. We will review the development of the unique AAV genetic system, the development of the first vectors, the early clinical trials, and the remarkable progress in vector technology, production technology and process development that has made AAV the most versatile and powerful vector system currently in use today. We will also focus on problems that need to be solved today to move the field forward to the next phase of developing products for the pharmacy shelf. And we will review several recent clinical trials to illustrate some of the issues involved in treating human genetic and idiopathic diseases.