# Advances and challenges in gene therapy for rare diseases

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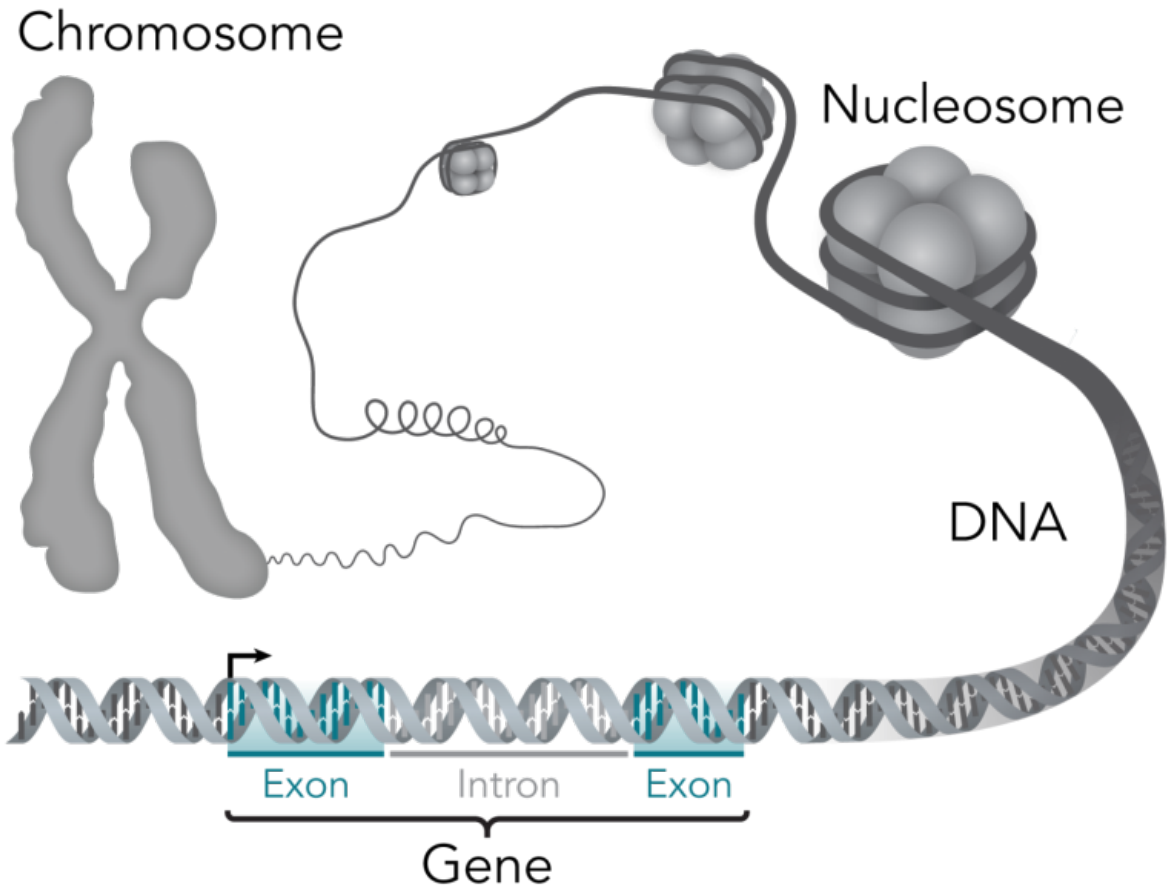


This stylistic diagram shows a gene in relation to the double helix structure of DNA and to a chromosome (right). The chromosome is X-shaped because it is dividing. Introns are regions often found in eukaryote genes that are removed in the splicing process (after the DNA is transcribed into RNA): Only the exons encode the protein. The diagram labels a region of only 55 or so bases as a gene. In reality, most genes are hundreds of times longer. Credit: Thomas

A new review article in *Human Gene Therapy* summarizes the significant milestones in the development of gene therapy medicinal products that have facilitated the treatment of a significant number of rare diseases. The article also describes the challenges in the progress of gene therapy for rare diseases.

Juan Bueren, from Centro de Investigaciones Energéticas Medioambientalies y Tecnológicas (CIEMAT), and President of the European Society for Gene and Cell Therapy (ESGCT); and Alberto Auricchio, from Telethon Institute of Genetics and Medicine (TIGEM), and Vice-President of the European Society for Gene and Cell Therapy, co-authored the article titled "Advances and Challenges in the Development of Gene Therapy Medicinal Products for Rare Diseases."

"Advances in the generation of integration competent vectors have markedly improved the efficacy and also the safety associated to ex vivo hematopoietic stem cell gene therapies during the last decade," state the authors. "In addition to ex vivo gene therapy, in vivo gene therapy has shown to be safe and effective therapy in humans. However, also some challenges remain to make this transformative therapeutic approach widely available."

"Professors Bueren and Auricchio, as the leaders of the ESGCT, provide a uniquely comprehensive perspective on the development of molecular therapies for rare diseases in Europe," says Editor in Chief Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education and Dean, Provost, and Executive Deputy Chancellor, University of Massachusetts Medical School.

**More information:** Juan A. Bueren et al, Advances and Challenges in the Development of Gene Therapy Medicinal Products for Rare Diseases, *Human Gene Therapy* (2023). DOI: 10.1089/hum.2023.152