

Researchers describe incentivized programs to aid gene therapy developers

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Providing an overview of the submissions process and examples of U.S. Food and Drug Administration (FDA) applications for Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPDD), a new article can help developers of gene therapies for rare genetic diseases. The article is published in the journal *Human Gene Therapy*.

Anne Pariser and Elizabeth Ottinger, from the National Center for Advances in Translational Sciences (NCATS), National Institutes of Health, and co-authors describe the ODD and RPDD programs, which provide [financial incentives](#) for the development of diagnostic drugs, [preventive measures](#), and treatments of diseases affecting small patient populations.

To facilitate the standardization of gene therapy development of rare genetic diseases, the NCATS developed the Platform Vector Gene Therapy (PaVe-GT) program. The first adeno-associated virus gene therapy product for the treatment of PCCA-related propionic academia received ODD in 2021 and RPDD in 2022. In this article, members of the PaVe-GT program emphasize the significance of these incentive programs in stimulating [drug development](#) and illustrate how developers of gene therapies can utilize FDA guidance to prepare ODD or RPDD applications.

"The PaVe-GT program is pioneering regulatory and clinical trial approaches to broaden the impact of gene therapy," 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 Chan Medical School.

More information: Richa Madan Lomash et al, Successfully Navigating Food and Drug Administration Orphan Drug and Rare Pediatric Disease Designations for AAV9-hPCCA Gene Therapy: The National Institutes of Health Platform Vector Gene Therapy Experience, *Human Gene Therapy* (2023). [DOI: 10.1089/hum.2022.232](https://doi.org/10.1089/hum.2022.232)

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