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