

Long-term study shows less frequent and severe pancreatitis following gene therapy for LPLD

September 29 2016

Up to 6 years after receiving a single treatment with the gene therapy product lipoprotein lipase (LPL), patients with the debilitating genetic disease LPL deficiency (LPLD) had about 50% fewer episodes of pancreatitis than before receiving the treatment. None of the study participants suffered severe pancreatitis following gene therapy and only one required admission to the intensive care unit for treatment of LPLD, which can be fatal, according to a study published in *Human Gene Therapy*.

The article "Long-Term Retrospective Analysis of Gene Therapy with Alipogene Tiparvovec and Its Effect on Lipoprotein Lipase Deficiency-Induced Pancreatitis ([online.liebertpub.com/doi/full ... 10.1089/hum.2015.158](http://online.liebertpub.com/doi/full/10.1089/hum.2015.158))," is coauthored by an international team of researchers led by Daniel Gaudet, Université de Montreal (Canada), Academic Medical Center and uniQure BV, Amsterdam, and Erasmus Medical Centre, Rotterdam (The Netherlands), Chiesi Farmaceutici, Parma (Italy), Denis Diderot University, Paris (France), and University Hospital of North Midlands (U.K.).

The researchers compared the frequency and severity of definite and probable pancreatitis or acute abdominal pain in 19 [patients](#) for equal time periods before and after gene [therapy](#). They report that a single dose of alipogene tiparvovec, designed to restore LPL activity in patients with LPLD, was associated with both a lower frequency and severity of

pancreatitis events and, consequently, with reduced healthcare costs over the 6-year follow-up period.

"As the first EMA-licensed clinical gene therapy in history, Glybera occupies an important place in the history of human 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 Medical School, Worcester, MA. "The 6-year follow up data presented here demonstrate that the long-term persistence of rAAV that has long been observed in preclinical settings will be true in patients as well."

More information: Daniel Gaudet et al. Long-Term Retrospective Analysis of Gene Therapy with Alipogene Tiparvovec and Its Effect on Lipoprotein Lipase Deficiency-Induced Pancreatitis, *Human Gene Therapy* (2016). [DOI: 10.1089/hum.2015.158](https://doi.org/10.1089/hum.2015.158)

Provided by Mary Ann Liebert, Inc

Citation: Long-term study shows less frequent and severe pancreatitis following gene therapy for LPLD (2016, September 29) retrieved 26 April 2024 from <https://medicalxpress.com/news/2016-09-long-term-frequent-severe-pancreatitis-gene.html>

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