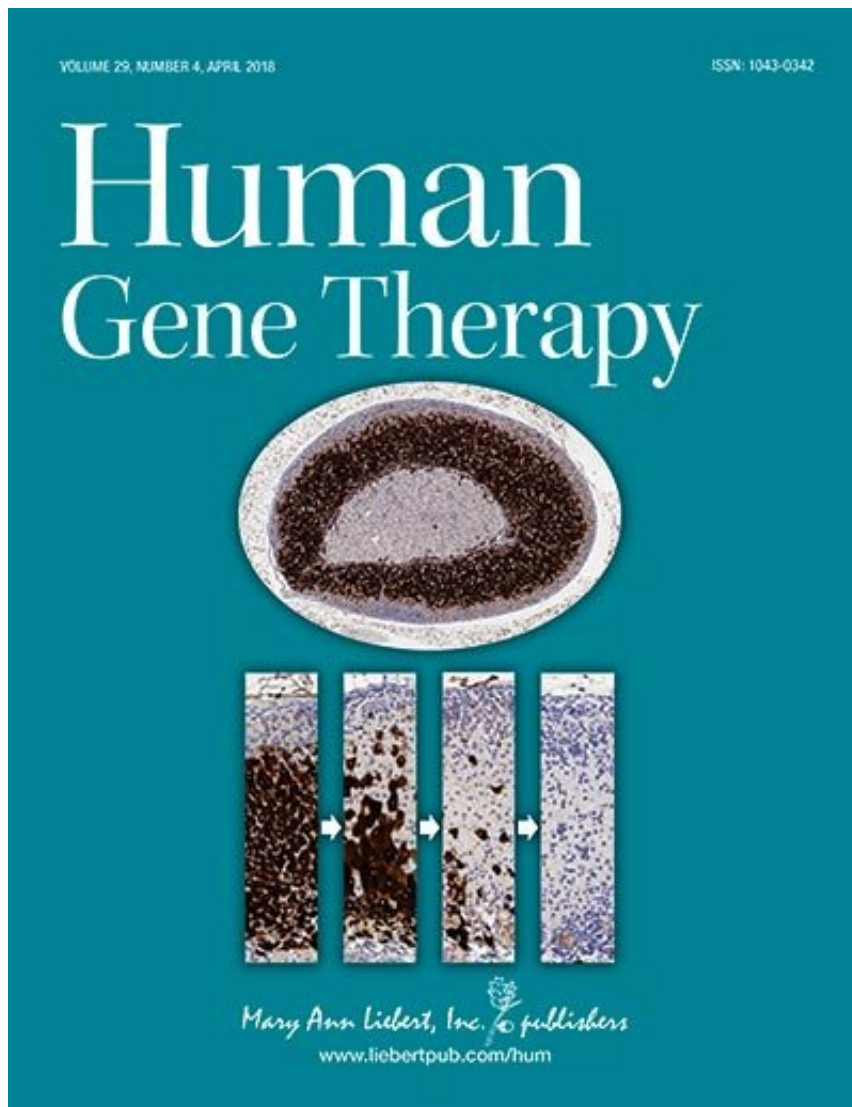


Gene therapy for lipoprotein lipase deficiency yields promising results

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Credit: Mary Ann Liebert, Inc., publishers

During the first 18 months after treatment with ali-pogene tiparvovec, a gene therapy recently approved in Europe to treat lipoprotein lipase deficiency (LPLD), the first patient to receive the treatment had no abdominal pain or episodes of pancreatitis, following a history of 37 pancreatitis attacks. The patient was able to discontinue plasmaphere and described improved quality of life, as reported in *Human Gene Therapy*.

The article "Gene Therapy in Lipoprotein Lipase Deficiency (LPLD): Case Report on the First Patient Treated with Alipogene Tiparvovec under Daily Practice Conditions" was coauthored by Ursula Kassner, Tim Hollstein, Thomas Grenkowitz, Marion Wühle-Demuth, Bastian Salewsky, Ilja Demuth, Elisabeth Steinhagen-Thiessen, from Charité - Universitätsmedizin Berlin, and Michaela Dippel, MD Medscript, Bad Dürheim, Germany.

LPLD is a rare inherited disorder associated with an increased incidence of inflammation of the pancreas due to elevated triglyceride levels. Gene therapy with alipogene tipar-vovec offers a potential cure for LPLD, which is currently treated with plasmapheresis when maintenance of an ultra-low fat diet, use of fibrates, and other triglyceride-lowering therapies prove inadequate. Alipogene tiparvovec is an adeno-associated virus 1 (AAV1) [gene therapy](#) administered via intramuscular injections together with immunosuppression.

Uniqure, the manufacturer of alipogene tiparvovec (marketed as Glybera), has ceased post-marketing studies and withdrawn the product from the market in the European Union due to very limited demand for its use. The withdrawal of Glybera was not related to any risk-benefit concerns.

"While Glybera has not continued in commercial sales, it is very gratifying to gene therapy translational researchers to see a case report of a safe and effective gene [therapy](#) product in clinical use," 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.

More information: Ursula Kassner et al, Gene Therapy in Lipoprotein Lipase Deficiency: Case Report on the First Patient Treated with Alipogene Tiparvovec Under Daily Practice Conditions, *Human Gene Therapy* (2018). [DOI: 10.1089/hum.2018.007](https://doi.org/10.1089/hum.2018.007)

Provided by Mary Ann Liebert, Inc

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