The inside story behind the approval of the gene therapy drug Glybera

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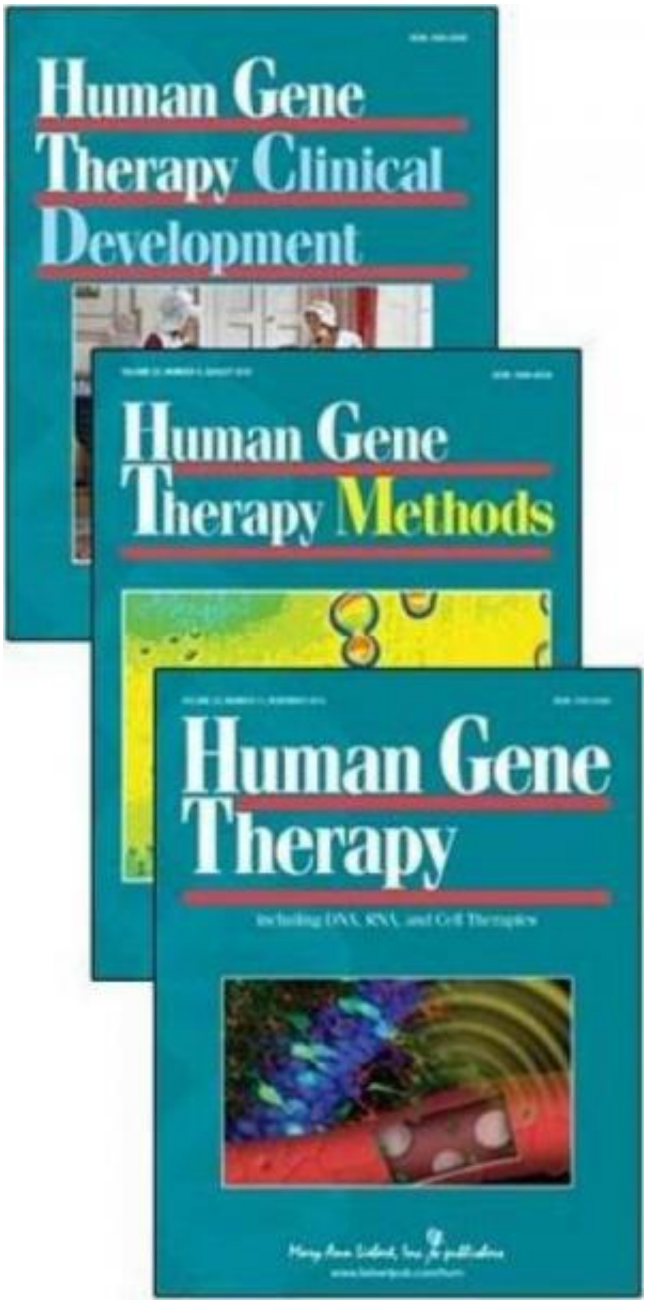

The scientists who led the team that developed Glybera, the first gene therapy drug approved for use in the Western world, provide a fascinating first-person account of their pioneering work in *Human Gene Therapy*, a peer-reviewed journal from Mary Ann Liebert, Inc., publishers. In addition, an in-depth Review reveals the inside story of the European regulatory review and approval of Glybera, chock full of twists and turn, politics, and intrigue, reviews and critiques the groundbreaking drug's path to the marketplace appears in in *Human Gene Therapy* Clinical Development. Both articles are available on the *Human Gene Therapy* website.

John Kastelein, University of Amsterdam (the Netherlands) and Colin Ross and Michael Hayden, University of British Columbia (Vancouver, BC, Canada) describe the long path to the discovery of the genetic mutation responsible for lipoprotein lipase deficiency (LPLD), a rare, inherited disease, and the subsequent work in Dr. Hayden's laboratory to develop a gene replacement therapy. In the article "From Mutation Identification to Therapy: Discovery and Origins of the First Approved Gene Therapy in the Western World," the authors recount the many failures and successes and the significant delays that finally ended on November 2, 2012, when the European Medicines Agency granted marketing approval for Glybera. The therapy will be submitted for review by the U.S. and Canadian regulatory authorities.

James M. Wilson, MD, PhD, Editor-in-Chief of *Human Gene Therapy*, and Director of the Gene Therapy Program, Department of Pathology and Laboratory Medicine, University of Pennsylvania Perelman School of Medicine, Philadelphia, and coauthors give a behind-the-scenes view

of the protracted clinical development, review, and approval process for Glybera in the Commentary "Lessons Learned from the Clinical Development and Market Authorization of Glybera." Although Glybera's safety was not an issue, its efficacy in the relatively small number of patients who received the therapy was questionable. The authors chronicle a bumpy road to market approval, fraught with rejections, reanalysis of study data, and appeals that led to commercialization of the product with the caveat of an ongoing patient registry to allow for continued review of the effectiveness of the therapy as it is used in more patients.

Provided by Mary Ann Liebert, Inc