Gene therapy is a 'disruptive science' ready for commercial development

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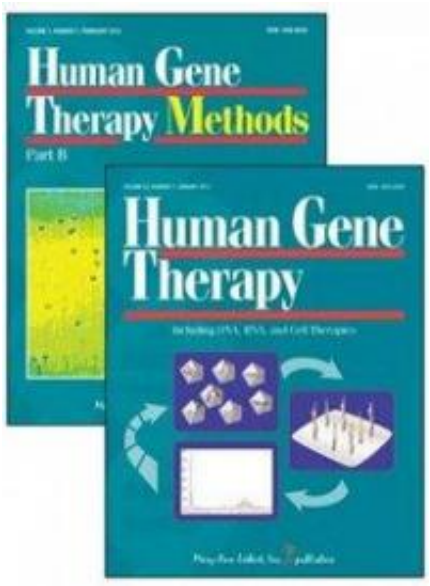

Human Gene Therapy, the official journal of nine international societies, is an authoritative peer-reviewed journal published monthly in print and online that presents reports on the transfer and expression of genes in mammals, including humans. The journal is expanding to 18 issues, to include part B *HGT Methods*. Credit: ©2012, Mary Ann Liebert Inc., publishers

The time for commercial development of gene therapy has come. Patients with diseases treatable and curable with gene therapy deserve access to the technology, which has demonstrated both its effectiveness and feasibility, says James Wilson, MD, PhD, Editor-in-Chief of *Human Gene Therapy* in a provocative commentary and accompanying videocast

. *Human Gene Therapy* and *Human Gene Therapy (HGT) Methods* are peer-reviewed journals published by Mary Ann Liebert, Inc..

Until recently, gene therapy has been reserved for severe diseases with few treatment options. But the recent report of its successful use to treat hemophilia B, which would offer patients a therapeutic alternative that could replace the need for regular, lifelong protein replacement infusions, has brought gene therapy to the forefront as a technology capable of competing with and disrupting traditional forms of treatment. Although gene therapy for hemophilia B is still in early-stage clinical testing, a similar approach is in development to treat hemophilia A, and together these life-threatening diseases represent a $6.5 billion market for current protein replacement therapies.

The technical feasibility of gene therapy "has been established in multiple diseases and with different technology platforms," says Dr. Wilson, in the Commentary "It's Time for Gene Therapy to Get Disruptive!" He predicts that "2012 will usher in an era of commercial development of gene therapy that, although likely to begin slowly, will quickly gather momentum."

"The scientific community has been promising for years that disruptive change would follow from investments in biomedical research, such as the doubling of NIH and the sequencing of the human genome," says Terence R. Flotte, MD, Celia and Isaac Haidak Professor of Medical Education Dean, Provost, and Executive Deputy Chancellor, Gene Therapy Center, and Departments of Pediatrics and Microbiology & Physiologic Systems, University of Massachusetts Medical School. "Dr. Wilson's comments strike at the heart of fulfilling that promise. Now that the technology is working, the next phase is to develop commercially viable models for gene therapy in the health care marketplace, so that these therapies can be delivered to the patients who need them."

Provided by Mary Ann Liebert, Inc.