

MOLECULAR THERAPY

*The Journal of the American
Society of Gene Therapy*

Editor-in-Chief
Inder M. Verma

Editor
Fintan R. Steele

Associate Editors
Malcolm Brenner
Leaf Huang
Elizabeth G. Nabel
Irving L. Weissman
James M. Wilson

Editorial Board
Eduardo Aguilar-Cordova
Jean-Paul Behr
R. Michael Blaese
Helen M. Blau
David Bodine
Xandra O. Breakefield
Hermann Bujard
Parris R. Burd
Jeffrey Chamberlain
Seng H. Cheng
Charles Coutelle
Ronald G. Crystal
David T. Curiel
Olivier Danos
Beverly L. Davidson
John E. Dick
Cynthia E. Dunbar
Victor J. Dzau
Philip L. Felgner
Theodore Friedmann
Fred H. Gage
Joseph C. Glorioso
Markus Grompe
Stephen D. Hauschka
Katherine High
Jeffrey M. Isner
Yasufumi Kaneda
George Karpati
Mark A. Kay
Susan Kingsman
Donald B. Kohn
Jeffrey M. Leiden
Kam Leong
Margaret A. Liu
Dusty Miller
Richard C. Mulligan
Nicholas Muzyczka
Gary J. Nabel
Luigi Naldini
Arthur W. Nienhuis
Garry P. Nolan
Drew Pardoll
Michel Perricaudet
John J. Rossi
David Russell
Stephen J. Russell
Michel Sadelain
Cyrus R. Safinya
Izumu Saito
R. Jude Samulski
Alan E. Smith
George Stamatoyannopoulos
Frank Szoka, Jr.
Didier Trono
Dinko Valerio
Matthew D. Weitzman
David A. Williams
Jon A. Wolff
Savio L. C. Woo

Specific, Substantial, and Credible

In the January 5, 2001 issue of the *Federal Register*, the U.S. Patent and Trademark Office (PTO) published its final (for now) guidelines on patenting genes. Despite high-profile resistance from several patient groups and self-styled "consumer-protection" organizations to *any* patenting, the PTO managed to strike a reasonably even balance in the new guidelines, demanding that anyone who seeks a patent on a gene can only receive one if he/she demonstrates "specific, substantial, and credible" utility. In short, no ESTs or SNPs need apply (although one or two exceptions may sneak through). And no more "making up" specious uses just to stake a temporary claim to a snippet of sequence.

Most of the public comments the PTO received following the December 1999 publication of the interim guidelines, both propatenting and antipatenting, outlined the dire consequences that would follow one or the other decision. Patenting genes will slow down research, result in further distance between the health care "haves" and the "have-nots," and unfairly take away a person's "natural" assets, according to the antipatenters. Propatenting forces also stated that gene therapy research, or at least drug development, would screech to a halt without patent protection and that fundamental constitutional rights to intellectual property would be critically wounded. While welcoming the suggested new guidelines, they expressed concern that the utility bar not be set too high, suggesting this would have the same effect as disallowing any gene patents.

Divining the future is not an exact science, despite the assertive plethora of 1-800-GO-TAROT advertisements on late-night television. Nevertheless, we have enough modern drug-development history behind us to know that new drugs, including gene therapies, will not be available in any reasonable time without some financial incentive, i.e., intellectual property rights, to the developers of such therapies. The PTO has acted reasonably in resetting the bar at an attainable height: You can have a patent if you can demonstrate the utility of your discovery.

The new guidelines put the burden of demonstrating utility directly on the applicant, but also place an even greater burden on the patent examiner. Thus, the PTO needs to ensure that its examiners who consider gene patent applications are fully conversant with the field and the technology at stake. Indeed, some of the public comments made in response to the interim guidelines took issue with the examiner training materials rather than the definitions of utility. This should be a high priority of the PTO in reining in some of the excesses of the current patenting frenzy. Another should be revisiting some patents already issued that do not meet the new guidelines.

Although a good start, this is only one early battle in the gene-patenting wars. Significant opponents to any gene patenting are just getting started, and they make arguments that, while not strong, certainly resonate with the general public. When Jeremy Rifkin asks, "What might it mean for subsequent generations to grow up thinking of all life as mere invention?," he is doing more than setting up a reductionist straw horse: He is appealing to a general fear about genes and gene therapy and a general distrust of scientists and pharmaceutical companies.

How do we combat such demagoguery? For our part, we need to exert ourselves in better public education about gene therapy, from its basic research through its clinical development, including the important role of intellectual property. That said, we also need to be reasonable in sharing resources and research findings with others in the field. We also need to encourage greater openness by the biopharmaceutical industry about their research programs and perhaps even eventual pricing decisions about newly developed gene therapies. This is certainly in keeping with the spirit, if not the letter, of the moderate and measured gene-patenting guidelines promulgated by the PTO.

Fintan R. Steele
Editor, Molecular Therapy

This Editorial is identified by doi:10.1006/mthe.2001.0261.