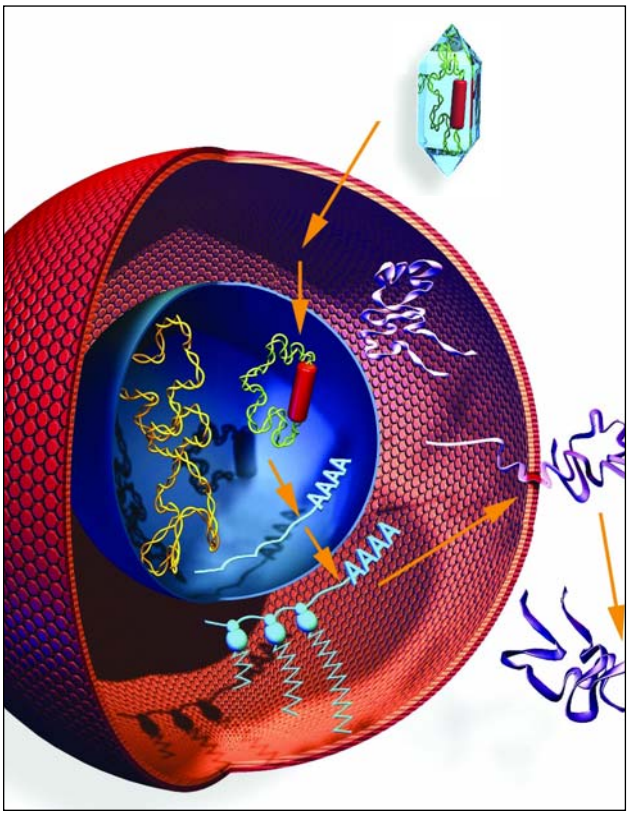
Discovery Medicine

Medicine on the Leading Edge

Volume 5, Number 25 February 2005

Content Highlights

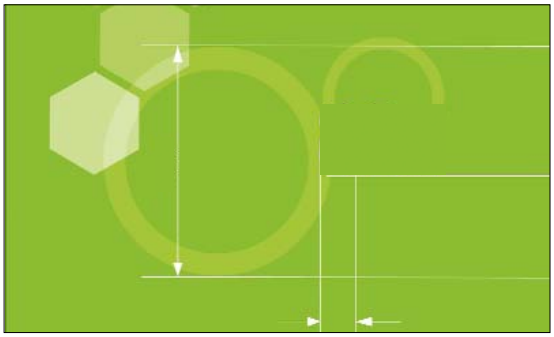


B. Acres, P. Squiban, and M. Liu; Transgene SA

Gene Therapy for Heart Disease, Cancer and AIDS

Pages 4, 18, 25, 37 and 58

Editor's Note



For many of the difficult-to-treat diseases, gene-based therapies and gene-based drug deliveries offer hope and excitement. In this issue of Discovery Medicine, articles describe the use of gene therapy to manipulate stem cells for treating heart diseases (Dr. Rosen et al. on page 18), to produce vaccines for the immunotherapy of cancer (Dr. Acres et al. on page 25 and Dr. Stevenson on page 37), and to fight HIV-1 infection (Dr. Egan on page 58).

However since their first use about two decades ago, gene therapies have remained experimental. Genes with therapeutic potential have been injected into test animals and patients in various forms -- naked or "clothed" delivered by (gene) guns, cassettes, shuttles, viruses, etc.

The efforts have been heroic. But it is time to reflect on why the gene therapy research enterprise has not been as successful in patients as scientists and physicians had hoped. To be sure, the natural barriers are formidable. Following introduction into the human body, the therapeutic genes have to battle the body's complexity, establish a hold in a hostile environment, and confront outright rejection by the body's immune system. Looking introspectively, it is clear that the vectors that have so far been used to deliver the genes are mostly inadequate to carry gene therapy onto a more successful conclusion. Their limitations have restricted us to the level of success at which we now stand.

It seems logical that the investigators in the gene therapy community first need to devote substantially more effort and resources to devising a powerful, modular, controllable, and safe gene delivery vehicle. The NIH should organize and fund "Project Gene Vector" in proportion to that applied to the "Human Genome Project." With the right tools, successful gene therapy is attainable.

Benjamin Yang, M.D., Ph.D.
Executive Editor
Noel R. Rose, M.D., Ph.D.
Editor-in-Chief

Editors

Discovery Medicine (ISSN 1539-6509) is published bi-monthly by Peter H. Rheinstein, M.D., J.D., 57 W. Timonium Rd., Ste. 207, Timonium, MD 21093-3105. Application to mail at Periodicals postage rates is pending at Timonium, MD 21093 and additional mailing offices. POSTMASTER: Send address changes to Peter H. Rheinstein, 57 W. Timonium Rd., Ste. 207, Timonium, MD 21093-3105.

Annual subscription rates: Personal print or digital edition, $49.95; institutional print plus digital edition, $349. Group bulk subscriptions are available at a reduced rate schedule which is provided upon request.

Contact/Service
57 W. Timonium Rd., Ste. 207
Timonium, MD 21093, USA
Phone: 410-252-3631
Fax: 410-252-3634
service@discoverymedicine.com
www.discoverymedicine.com