



### Volume 2, Number 10

#### **An experimental model of gene therapy for hemophilia B**

Mark A. Kay's team (Stanford University School of Medicine, Stanford, CA, USA) devised a gene therapy model for hemophilia B that relies upon the use of a viral vector designed for liver-specific expression of the gene encoding human factor IX in the mouse. Yant et al. demonstrate significant and sustained (up to 24 weeks post-treatment with recombinant virus) expression of the therapeutic gene.

Yant, S.R., et al., **Transposition from a Gutless Adeno-Transposon Vector Stabilizes Transgene Expression *in vivo***, *Nat Biotechnol* 20 (10) : 999-1005 (2002) (doi : 10.1038/nbt738).

#### **An experimental gene therapy trial for $\beta$ -thalassemia**

A consortium of gene therapists headed by Philippe Leboulch (Massachusetts Institute of Technology, Cambridge, MA, USA) and R. Keith Humphries (University of British Columbia, Vancouver, British Columbia, Canada) published the results of an experimental gene therapy trial aimed at treating  $\beta$ -thalassemic mice. The proposed treatment led to the normalization of disease symptoms, to the extent that  $\beta$ -thalassemic mice were indistinguishable from normal mice.

Imren, S., et al., **Permanent and Panerythroid Correction of Murine  $\beta$ -Thalassemia by Multiple Lentiviral Integration in Hematopoietic Cells**, *Proc Natl Acad Sci USA* 99 (22) : 14380-14385 (2002) (doi : 10.1073/pnas.212507099).

#### **A pregnancy hormone involved in red blood cell production ?**

Janis L. Abkowitz's team at the University of Washington (Seattle, WA, USA) report the case of a woman diagnosed with aplastic anemia (deficiency in red blood cell production), whose symptoms had been markedly reduced during the third trimester of her last two pregnancies as well as throughout lactation. It appears that prolactin, a pituitary hormone whose primary function is the induction of milk synthesis by the mammary gland, would also stimulate red blood cell production.

Abkowitz, J. L., et al., **Response of Diamond-Blackfan Anemia to Metoclopramide : Evidence for a Role for Prolactin in Erythropoiesis**, *Blood* 100 (8) : 2687-2691 (2002).

#### **An undescribed transfusion reaction**

In a short article published in *The Lancet*, Wallis (Freeman Hospital, Newcastle upon Tyne, United Kingdom) et al. report the case of a transfused newborn subsequently diagnosed with severe neutropenia (marked decrease in neutrophil blood level). Anti-neutrophil antibodies present in the transfused plasma were probably responsible for this novel type of transfusion reaction.

Wallis, J. P., et al., **Transfusion-Related Alloimmune Neutropenia : An Undescribed Complication of Blood Transfusion**, *Lancet* 360 (9339) : 1073-1074 (2002).

#### **A clinical trial evaluating a blood substitute**

The results of a clinical trial designed for assessing the security and preliminary efficacy of a synthetic blood substitute made up of a perfluorocarbon emulsion suggest a low level of efficacy, yet sufficient to reduce modestly blood transfusion support during coronary artery bypass graft surgery.

Hill, S. E. (Duke North Hospital, Durham, NC, USA), et al., **Perflubron Emulsion (AF0144) Augments Harvesting of Autologous Blood : A Phase II Study in Cardiac Surgery**, *J Cardiothorac Vasc Anes* 16 (5) : 555-560 (2002) (doi : 10.1053/jcan.2002.126947).

#### **Relative sensitivities of different hepatitis B virus (HBV) detection tests**

Holger Hennig (University of Lübeck, Lübeck, Germany) et al.'s study reveals that the detection of antibodies directed against the core of HBV particles among blood donors contributes to a reduction in HBV transfusion transmission risk.

Hennig, H., et al., **Frequency and Load of Hepatitis B Virus DNA in First-Time Blood Donors with Antibodies to Hepatitis B Core Antigen**, *Blood* 100 (7) : 2637-2641 (2002) (doi : 10.1182/blood-2002-03-0798).