HÉMA-QUÉBEC

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A Monthly Newsletter Summarizing Important Advances in Transfusion

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A non-viral approach to gene therapy for hemophilia B

Working on a mouse experimental model, Michele B. Calos (Stanford University School of Medicine, Stanford, CA, USA) and coworkers designed and tested a gene therapy for hemophilia B that involves the insertion of the therapeutic gene into the host's genome without relying upon a viral vector. Mid- to long-term effectiveness and safety make this strategy promising.

Olivares, E. C., et al. (2002). **Site-specific genomic integration produces therapeutic Factor IX levels in mice**. Nat Biotechnol 20 (11) : 1124-1128 (doi : 10.1038/nbt753).

Why platelets cannot be stored in the cold?

It has been known for decades that platelets stored at 4°C are cleared much more rapidly after transfusion than platelets stored at 20-25°C. Hoffmeister (Brigham and Women's Hospital, Boston, MA, USA) et al.'s results indicate that cold temperatures trigger changes in the localization of certain proteins on the surface of platelets, thereby making them prone to clearance.

Hoffmeister, K. M., et al. (2003). **The clearance mechanism of chilled blood platelets**. Cell 112 (1) : 87-97.

Efficacy of a human hemoglobin-based blood substitute

The results of a multicenter trial, led by Steven A. Gould (Northfield Laboratories Inc., Evanston, and University of Illinois at Chicago College of Medicine, Chicago, IL, USA), suggest that PolyHeme (Northfield Laboratories Inc.), a substitute made up of human hemoglobin polymers, enhances survival of patients after a massive blood loss consequent to trauma or surgery.

Gould, S. A., et al. (2002). **The life-sustaining capacity of human polymerized hemoglobin when red cells might be unavailable**. J Am Coll Surg 195 (4) : 445-452; discussion 452-455.

Specific detection of prions causing Transmissible Spongiform Encephalopathy (TSE)

TSEs, such as Creutzfeld-Jakob disease and its variant form, are associated with the accumulation of aberrant forms of the prion protein in the brain. Jiri G. Safar (University of California, San Francisco, CA, USA) and coworkers designed a sensitive immunoassay capable of distinguishing the aberrant and the normal forms of the prion. Future will tell whether this assay will be capable of diagnosing TSEs during the asymptomatic stage.

Safar, J. G., et al. (2002). **Measuring prions causing bovine spongiform encephalopathy or chronic wasting disease by immunoassays and transgenic mice**. Nat Biotechnol 20 (11) : 1147-1150 (doi : 10.1038/nbt748).

Detection of viral pathogens using high-density microarrays

Joseph L. DeRisi's team (University of California, San Francisco, CA, USA) tested a high-density microarray specially designed for the detection and identification, with a good level of confidence, of a broad array of viral pathogens. This technology is easily amenable to high-throughput screening at a competitive cost.

Wang, D., et al. (2002). **Microarray-based detection and genotyping of viral pathogens**. Proc Natl Acad Sci U S A 99 (24) : 15687-15692 (doi : 10.1073/pnas.242579699).

A method for purifying immunoglobulins with enhanced yield

Current plasma fractionation methods give on average a 50% recovery in immunoglobulins. The preparative electrophoresis method of Philip Roeth and coworkers (Gradiport Ltd., Sydney, Australia) yielded recoveries exceeding 90%, at a purity comparable to that of commercial immunoglobulin preparations.

Li, G., et al. (2002). **Purification of human immunoglobulin G: a new approach to plasma fractionation**. Vox Sang 83 (4) : 332-338 (doi : 10.1046/j.1423-0410.2002.00241.x).