



BULLETIN



CANADIAN GENETIC DISEASES NETWORK

CANADIAN SCIENTISTS MAKE IMPORTANT ADVANCE IN THE STUDY OF HUMAN BLOOD DISEASE

Toronto - August 31, 1998: A team of researchers, led by Canadian Genetic Diseases Network scientist Dr. John Dick at Hospital for Sick Children, has discovered a new stem cell in human blood which provides novel insights into how the blood system works. The discovery may have an impact on the therapy currently used in the treatment of some blood diseases such as leukemia, thalassemia, and sickle cell anemia.

These latest findings, published in the August 31 issue of *Nature Medicine*, build on ground-breaking research results published by Dr. Dick and his team in December 1996 when a bone marrow cell which produces blood in the human body was identified. At that time it was anticipated that in a gene therapy process, a normal gene would be inserted into the stem cell of a patient and the altered cell would then begin to produce blood containing the normal gene.

Stem cell transplants are now widely used for treating different types of cancer, anemias and auto-immune disorders. While stem cells carrying a protein marker called CD34 are commonly selected for therapy and cell transplantation in many patients with blood disease, Dr. Dick's new findings indicate that stem cells which do not express the CD34 marker and which have previously been discarded in the therapy process may, in fact, provide a target for stem cell gene therapy.

In order to study the entire human blood system and how this system develops from stem cells, Dr. Dick's team over the last 10 years has developed a biological system and successfully reproduced the entire human blood system in mice. By transplanting

human blood cells into special immune-deficient mice called NOD/SCID mice, this system provides scientists with a living organism in which to study the development of the human blood system. The significance of Dr. Dick's latest discovery which, in today's publication has been shown in animal models, will now be determined for human patients.

"The identification of this new type of stem cell is important biologically in order to understand how the blood system works. It is important clinically because we may be able to make stem cell transplants better and develop new therapies if we focus on this new stem cell," said Dr. Dick.

Dr. Michael Hayden, Scientific Director of the Canadian Genetic Diseases Network (CGDN) which is headquartered at the University of British Columbia, said "These are significant results which bring us one step closer to the better understanding and treatment of life threatening diseases, including anemias and cancer of different blood cells. The Canadian Genetic Diseases Network has been actively committed since 1990 to ongoing support and development of high calibre Canadian-led research in human genetic disease."

The Canadian Genetic Diseases Network is a nation-wide centre of research excellence which creates strategic partnerships to commercialize discoveries for the benefit of health care in Canada and worldwide. Fifty scientists and their teams, based in 18 universities, hospitals, and research centres across Canada, focus on the molecular and cellular causes of inherited disease. In 1997, CGDN was awarded a further seven years' core funding by the federal Networks of Centres of Excellence Program via the Medical Research Council of Canada, to conduct an expanded research program "From Genes to Therapies."

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