Remarks
President, New York Blood Center

Unlocking the Potential of Stem Cells

We're doing a great deal of focused work on stem cells – the rare "master cells" that originate all the cells in blood.

In fact, three of our laboratories are dedicated to research that could ultimately strengthen and expand the arsenal of successful therapies used to treat cancer, AIDS, blood-related diseases and auto-immune disorders. They are the newly formed Laboratory of Stem Cell Biology, the Laboratory of Hematopoietic Growth Factors and the Laboratory of Immunogenetics.

This concentration of resources and talent is defining a worldwide leadership position for the Blood Center in stem cell research.

By working individually as well as collaboratively, these labs are exploring ways to isolate stem cells more easily, stimulate their natural production, learn how their unique properties can be used to treat some of the most intractable diseases of our time, and even uncover ways to prevent these diseases.

Drawing a blueprint

By finding out how stem cells function at the cellular and molecular levels, our scientists at the Laboratory of Stem Cell Biology plan to describe a stem cell "blueprint." With this tool, scientists could help correct flawed genetic coding in vitro, then return genetically modified stem cells into the body.

In patients with genetic disorders like sickle cell anemia, for example, doctors could transplant stem cells from a disease-free child, or genetically engineer the sick child's own stem cells in vitro and return them into his or her body to begin producing normal, healthy blood cells.

Stem cells may also be used to replace a compromised immune system with new, non-infected white blood cells that will continue to reproduce healthy cells throughout a person's life.

Although still highly experimental, animal studies have shown that removing HIV-infected white blood cells through chemotherapy or radiation and replacing them with healthy stem cells could theoretically control AIDS. The same technique might also be used to control other diseases of the immune system.

Meanwhile, stem cells could be genetically altered in vitro to resist the devastating effects of high-dose cancer drugs and then be transplanted into a cancer patient. The physician then could treat the tumor with stronger, more effective doses of chemotherapy, knowing that the newly formed drug-resistant blood cells could eliminate the drug's toxins.

The role of growth factors

As part of this basic research, scientists in the Laboratory of Hematopoietic Growth Factors are examining the basic mechanisms that cause stem cells to develop and proliferate.

They're focusing on the hormones and proteins that stimulate and control the production of stem cells to identify factors that determine whether the cells self-renew or commit to becoming progenitor cells that produce either red blood cells, platelets or various kinds of white blood cells.

We believe the commitment process is most likely activated by a Master Regulatory Gene (MRG) for each cell type. We're trying to clone the MRG found within those stem cells that can only respond to one or a limited number of growth factors. Disorders at

this level of blood cell regulation can cause diseases like leukemia and lymphoma.

We believe that by understanding how stem cells divide and differentiate, it might be possible to make cells divide and grow in predetermined ways.

The lab is investigating how genes for growth factor receptors are activated – the process that ultimately triggers terminal cell differentiation and maturation of red cells, white cells or platelets. Receptors are situated in the cell's membrane, functioning like doors through which signals are sent into the cell to direct its growth.

At the same time, the lab is investigating ways to reproduce stem cells in vitro, which would make them more available for use in bone marrow transplantation, allow individuals to donate stem cells for their own use, and provide a potentially unlimited source of stem cells for those who cannot produce their own.

The work of these laboratories is a good example of how basic research supports and strengthens the understanding and practice

of clinical disciplines such as hematology, transfusion medicine, gene therapy and immunology.

Replacing bone marrow transplantation

Armed with this growing body of knowledge about stem cells, scientists at the New York Blood Center are beginning to see practical, clinical applications unfold.

A dramatic case in point is the systematic collection, analysis and use of stem cells from placental/cord blood to reconstitute bone marrow that has been destroyed by chemotherapy or blood-related disease.

As clinical trials continue, our Stem Cell Pilot Program, under the direction of our Laboratory of Epidemiology, is demonstrating for the first time that gravely ill patients can be transplanted successfully with stem cells from placental blood of unrelated donors.

The new procedure eliminated what would have been a long, frustrating and too-often futile wait for a perfectly tissue-type-matched bone marrow donor.

Our goal is to establish a national stem cell bank by gathering thousands of units from genetically diverse donors and freezing them fully tested, typed, and ready for transplantation to any patient in need.

Collaborations among our scientists is key.

With basic research in stem cell biology continuing…

with the Laboratory of Hematopoietic Growth Factors determining the number of stem cells present in each blood sample collected…

with the Laboratory of Epidemiology collecting and testing samples for infectious diseases…

with the Laboratory of Immunogenetics typing, processing, freezing and properly storing the samples…

and finally, with the clinical laboratories of the Blood Center collaborating in this effort by conducting rigorous testing to screen the units of placental blood for infection…

the New York Blood Center is conducting an all-out effort to realize the full benefit of placental stem cell harvesting.

We believe that all of these investigations into the potential of stem cells will have a profound impact on the health and well-being of millions of people around the world, providing them with a chance for health and life they would not otherwise have.

Thank you.