AMERICAN SOCIETY OF HEMATOLOGY
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April 3, 2012

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NIH Center for Regenerative Medicine
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Submitted electronically to: Scott.lipnick@nih.gov; nihcrm@mail.nih.gov

Dear Dr. Lipnick:

The American Society of Hematology (ASH) appreciates the opportunity to respond to the National Institutes of Health (NIH) Office of Strategic Coordination Request for Information: Defining Unmet Needs for Clinical Translation of Cell-Based Therapies (NOT-RM-12-008) issued on March 2, 2012.

ASH represents approximately 14,000 clinicians and scientists committed to the study and treatment of blood and blood-related diseases. These diseases encompass malignant hematologic disorders such as leukemia, lymphoma, and myeloma; non-malignant conditions including anemia and hemophilia; and congenital disorders such as sickle cell anemia and thalassemia. In addition, hematologists have been pioneers in the fields of stem cell biology, regenerative medicine, bone marrow transplantation, transfusion medicine, gene therapy, and development of many drugs for the prevention and treatment of heart attacks and strokes.

ASH applauds NIH's recent efforts in expanding opportunities for translational application of stem cell research in the institutes' intramural program, especially the establishment of the intramural Center for Regenerative Medicine. ASH believes that the Center will be an important resource to the scientific community by coordinating efforts that facilitate translation of technical and scientific advances in stem- and progenitor cell-based technologies to the clinic.

The NIH must ensure that resources adequately meet the needs of basic discovery, translational and clinical applications of this evolving technology. ASH urges the NIH to coordinate efforts that focus on studying both the potential efficacy and the safety of cell-based therapies, including patient-specific database registries for effectively tracking outcomes and adverse events of subjects receiving cellular reagents. In addition, efforts should be directed towards developing a consensus around the utility of animals and animal models to test efficacy and safety of cellular products.

Specifically, the Society has identified the following unmet needs that currently impede successful clinical translation of cell-based therapies, and believes the Center for Regenerative Medicine could play a role in coordinating efforts to address these needs:

1. The need for development, characterization (by flow cytometry, cytogenetics, cell doublings, transcriptome, proteome, secretome analyses etc.) and banking of specific cell lines to serve as standards and controls, as has been done for embryonic stem cell lines. These cell lines would include, but not be limited to, iPS cell lines, mesenchymal stromal cell lines, CD34+ cells and other hematopoietic cells.
2. The need of easy access to a repository of banked standards and controls for use as reference materials by the investigators as a service provided by the Center.
3. The need to develop more appropriate animal models to test the efficacy and safety of cell-based therapies.
4. The need for a re-examination of the clinical relevance of genetic instability of cultured cell products.
5. The need to devise the most appropriate design of early phase clinical trials for cell-based therapies and regenerative medicine. ASH also recommends the Center organize a workshop on this topic. Efforts should be directed towards building a consensus for the design of clinical trials across multiple disease disciplines that optimizes the opportunity for data collection and dissemination; the following topics should be addressed in this effort:

    a. Assurance of adequate characterization of the cellular product to be used in human trials.
    b. A defined long-term follow-up plan for all trials utilizing stem cell derivative products.
    c. A defined plan to bank a portion of all cell products infused into human patients, or at a minimum, DNA from donor samples for future interrogation.
    d. Solicitation of input from multiple disciplines to maximize opportunities for data collection.

The American Society of Hematology looks forward to working with the NIH and the Center for Regenerative Medicine on these important issues. We would be happy to provide further information and be a resource for the Center. Please contact ASH Senior Manager for Scientific Affairs, Ulyana V. Desiderio, Ph.D., at (202) 776-0544 or udesiderio@hematology.org for any additional information.

Sincerely yours,

Armand Keating, M.D.
President