

Molecular Therapy

From “Gene Therapy” to “Gene and Cell Therapy”: Why?

A proposal to change the name of the American Society of Gene Therapy (ASGT) to the American Society of Gene and Cell Therapy (ASGCT) has recently been adopted by the 2008 Membership Committee and forwarded to the Board of Directors of ASGT. The Board has unanimously approved the proposal, which is now being forwarded to the general membership of the Society for a final vote in April of 2009. The Membership Committee’s rationale in making this forward-looking proposal is that the concept of gene therapy includes gene-modified cell therapy, and an inclusive name will empower the Society to expand its membership base and help foster further collaborative research to advance the use of genes and cells as medicine to treat disease.

When the concept of gene therapy was conceived in the 1980s, bone marrow transplantation had already been in clinical practice for decades, and there was a spirited debate among investigators as to whether targeted genetic disorders would first be corrected by gene therapy or cell therapy. Looking back, it is apparent that the debate was based more on individual preferences than on science, because from the very beginning there were two main strategic means to achieve therapeutic gene transfer in animals and humans: *in vivo* or *ex vivo*. “*In vivo* gene therapy” was typically used in reference to the direct delivery of genes into diseased organisms so as to achieve a therapeutic outcome. In contrast, “*ex vivo* gene therapy” usually referred to the delivery of therapeutic genes into cultured cells *in vitro*, followed by transplantation of the gene-corrected cells into the diseased hosts. Although the Society was named the ASGT when it was established in the mid-1990s, the concept of correcting diseases using gene-modified cells was already fairly well established and practiced in animals. Indeed, one of the first scientific committees created by the ASGT was the Hematopoietic Cell Gene Therapy Committee, which organizes symposia for the Society’s annual meetings that have been among the best-attended sessions year after year.

Since the birth of Dolly the sheep more than a decade ago, the striking advances in the area of

embryonic stem (ES) cell biology and the enormous potential of using cells or tissues derived from ES cells to treat a host of diseases have caught the imagination of the scientific community as well as the lay public, because it can eventually eliminate the need for donated organs for transplantation and will dramatically enhance the ability to treat a multitude of diseases with regenerative medicine. A major limitation of the application of ES cell-derived tissues and cells for transplantation purposes is the need to prevent graft rejection in recipients via lifelong immunosuppression, as is currently the case for organ transplantation. One way to address this limitation is by introducing immune-evasive genes into the ES cells before their differentiation into mature cells and tissues *in vitro* and transplantation *in vivo*, so that the need for immunosuppressive medications can be minimized or eliminated. Even more exciting is the recent development of vector-mediated genetic reprogramming of terminally differentiated adult cells into induced pluripotent stem (iPS) cells, which exhibit many characteristics that imply they may be an excellent alternative to ES cells for cell-based therapies and regenerative medicine. Engineered tissues derived from iPS cells obtained from patients might be autologously transplanted back into the same patients without the need for lifelong immunosuppression; this would have distinct advantages over the allogeneic transplantation procedures currently in use. Recognizing this exceptional opportunity, the ASGT has recently constituted a new scientific committee, the Embryonic/Somatic Stem Cell and Tissue Engineering Committee.

Thus, the proposal to change the name of the Society from ASGT to ASGCT is appropriate and timely, because it recognizes the indisputable scientific facts and serves to bring together investigators with diverse backgrounds for productive interactions and cross-fertilization. Ultimately, it will expand the horizon of gene and cell therapy and facilitate the development of novel biotherapeutics to treat a wide variety of diseases—a goal that is enthusiastically and energetically shared by all investigators in the field.

Should the general membership approve the proposed name change, the Membership

Committee has recommended that the following concrete steps, among others, be implemented to maximize its positive impact. First, the term “cell therapy” must be included in the Society’s mission statement. Second, the Society’s leadership should contact the leadership of the International Society for Cellular Therapy (ISCT) to assure them that our name change represents an opportunity for both societies to collaborate and advance our common interests and to initiate a dialogue with them on the needs and interests of cell therapists so as to better recruit these researchers into our society. Third, the Society leadership should reach out to leading investigators and the research communities in adult and ES cell biology as well as iPS cell biology. Fourth, the Program Committee should ensure that cell therapy is richly

represented in the symposium programs of the Society’s annual meetings. Fifth, the Nominating Committee will need to make a concerted effort to identify and nominate prominent cell therapists into the Society’s leadership positions. Finally, the editorial team of *Molecular Therapy*—the Society’s official journal—is encouraged to increase even further the important efforts they have made over the past few years to recruit Editorial Board members with expertise in cell therapy and stem cell engineering, in addition to continuing their successful solicitation of outstanding articles in these areas.

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