AMERICAN SOCIETY of GENE & CELL THERAPY

March 1, 2012

Amy Patterson, MD
Director
NIH Office of Biotechnology Activities
Office of Science Policy/OD/NIH
6705 Rockledge Dr, Suite 700
Bethesda, MD 20892

Dear Dr. Patterson,

We are writing to you as President and President-Elect of the American Society of Gene & Cell Therapy (ASGCT) on behalf of the ASGCT membership and Board of Directors. In September 2011, ASGCT hosted an NIH Gene Therapy Symposium on the NIH campus. An overwhelming theme that emerged from the Symposium was the need for more effective and timely regulatory review of gene therapy protocols to ensure vital therapeutics are able to move efficiently from bench to bedside.

To address the need for more effective review of gene therapy products, ASGCT established a panel of experts to evaluate the evolving role of the NIH Recombinant DNA Advisory Committee (RAC) in fulfilling its mandate to advise the NIH Director on the conduct and oversight of research involving recombinant DNA.

As you know, the RAC is a component of the Office of Biotechnology Activities (OBA), which includes several functions comprising NIH guidelines for Research Involving Recombinant DNA, oversight on Institutional Biosafety Committees (IBCs), the electronic resource for reporting on adverse events (GeMCRIS), organization of workshops, safety symposia and policy conferences, and management of three or four meetings per year of the RAC Advisory Committee in a public forum for detailed review of selected protocols.

Our research community acknowledges that the RAC has been an important component in monitoring gene and cell therapies as they transition from basic research to early clinical trials. In particular, they have excelled in identifying specific areas that need to be discussed in depth and have been instrumental in assembling the right groups of investigators to present and discuss these topics in open forum. Over the past 15 to 20 years, the research community, FDA, and local IRBs and IBCs have all benefited from these valuable interactions. With this backdrop, it has also become evident that the need to review individual protocols has diminished as the research community has acquired extensive safety data and the general public has become satisfied that these protocols have not led to modification of the human genome or to creation of novel transmissible pathogenic agents. In parallel, over a thousand clinical trials have been carried out in this field, with an increasing knowledge and awareness of critical issues within the regulatory agencies responsible for their oversight, i.e. the Food and Drug Administration (FDA), Office of Human Subjects Research Protection (OHRP), the Cell, Tissue and Gene Therapy Advisory Committee of the FDA, the Department of Health and Human Service (HHS) and various institutional review groups, such as Institutional Human Studies Review Boards (IRBs) and Institutional Biosafety Committees (IBCs). These learning opportunities have been extended and continue to be facilitated by ASGCT offering clinical trial training courses, which have increased the knowledge base of the individual investigators in this field.

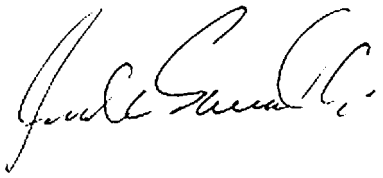
Based on the extensive safety data of this field with many protocols using similar agents and delivery methods, the ASGCT Board of Directors believes that the RAC would be more effective in its purpose by focusing on broader issues being encountered in the field rather than review of individual protocols.

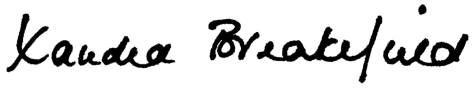
The ASGCT Board of Directors suggests that the NIH guidelines be amended such that the RAC would terminate review of individual clinical protocols and would instead identify new areas of research that require a public forum for discussion and review.

The recommendation would be in-line with the mandate of the RAC to advise the NIH Director on issues of concern to the public, which historically have been modification of the human genome at the germ cell level, and creation and dissemination of novel transmissible pathogenic vectors, neither of which remain issues for the commonly used gene therapy vectors.

We look forward to an ongoing discussion with the OBA on the evolving role of the RAC and how we can all work toward bringing vital new therapeutics to the clinic. Please feel free to contact us through the ASGCT Executive Director, Mary Dean (mdean@asgct.org).

Sincerely,





R. Jude Samulski, PhD
ASGCT President

Xandra O. Breakefield, PhD
ASGCT President-Elect

cc: Jacqueline Corrigan-Curay, MD, JD