I am speaking to you both in the capacity of the President-Elect of the American Society of Gene and Cell Therapy and as a research scientist who has dedicated my career to the development of this emerging medical field.

By way of background from our perspective, the NIH Recombinant DNA Advisory Committee (RAC) was formed in 1974 to advise the NIH Director on the conduct and oversight of research involving recombinant DNA, as well as to serve as a public forum for discussion of these issues. Over time the RAC came to serve as an arbitrator of all clinical protocols in gene therapy - with authority to approve or disapprove these trials. In 1995 Dr. Harold Varmus, then Director of NIH requested an ad hoc expert review committee to assess "the changing role of the RAC, the ways it may need to modify its operations, and how it should function to coordinate and facilitate productive gene therapy research". At the time these experts recommended that the RAC only review in public those protocols thought by a subset of its members to have potential issues of concern, and to no longer have a regulatory role in the decision as to whether these protocols would move forward. This regulatory function was deemed to be amply provided by the Food and Drug Administration, as well as Institutional Review Boards and Biosafety Committees. In the intervening 16 years investigators in the field of gene therapy and administrators in these regulatory agencies have become highly knowledgeable and experienced in review of these protocols, with thousands of trials having been conducted and many showing promising benefit. We feel, therefore, that it is time to reevaluate the mission and modus operandi of the RAC to better serve the need of the research community and public at whole.

The scientists and physicians in our society fully acknowledge the important role the RAC has played in the development of these new medical therapies during their transition from basic research to clinical trials. In particular the RAC has excelled in identifying specific areas of experimental research that benefited from further in depth discussions, and they have been instrumental in assembling knowledgeable groups of investigators to present and discuss these topics in open forum.

Based on the currently extensive safety data in the field—now approaching 20 years, with many protocols using well established agents frequently reviewed by FDA and delivery methods no longer considered novel, the ASGCT Board of Directors believes the RAC would serve a more effective function by focusing on broader issues encountered by the field, rather than on the review of individual protocols. Our 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 potential modification of the human genome at the germ line level, and the risk of creation and dissemination of novel transmissible pathogenic agents. With respect to the gene transfer community neither of these issues have been a problem with the commonly used gene therapy vectors employed today. In fact, to the contrary, after 20 years of testing and over 1,000 trials, no evidence exist at all to support the current regulatory burden the RAC request from gene transfer protocols.
Based on these considerations and in response to deliberations by an ASGCT Panel of Experts, the Board of Directors unanimously voted at a recent meeting to approve the following recommendation.

“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.”