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January 6, 2012

Francis S. Collins, MD, PhD  
NIH Director  
National Institutes of Health  
Building 1 - Shannon Building, 126  
1 Center Dr  
Bethesda, MD 20892-2152

Dear Dr. Collins, *Francis*

I am excited to write this letter on behalf of the American Society of Gene & Cell Therapy (ASGCT) and all the Society's past Presidents. In September, ASGCT had the opportunity to host a Symposium on the NIH campus in partnership with the Trans-NIH Gene Therapy Group.

The primary goal of the Symposium was to update the NIH community on current scientific issues in gene and cell therapy and to assess areas that are most promising for translational initiatives. The Symposium was also an occasion to review the challenges faced by investigators moving into the clinic and to discuss examples of technical and regulatory hurdles that have been successfully addressed.

The Symposium was an outstanding success, with over 400 registrants primarily from the NIH and FDA in attendance. Throughout the course of the Symposium, a number of themes presented themselves, including an overwhelming realization that our members continue to see clinical validation in a number of disease areas (ocular, neuro, blood, and many others listed below). We realize that the field has come of age and these seminal signs of gene transfer and cell therapy clinical success are a direct result of the vision and support from NIH leadership.

Based on the presentations at the NIH symposium, in the coming years we expect to see continued and accelerated pace of additional advances as platform delivery systems, such as AAV, Lenti, and others become validated in additional disease indications (e.g. Leber congenital Amaurosis and additional 22 single gene ocular disorders that can benefit from this platform). More importantly, the ongoing success in the Hemophilia B trial (6 patients treated now have circulating levels of FIX out as long as 2 years--Dec 2011 NEJM) has demonstrated in over \$400,000.00 in saving/patient to the British Health Care system, clearly demonstrating the potential of this field to impact the cost of health care in the US.

With this backdrop, the society would like to provide you and the NIH with a roadmap of disease indications that we feel will be viable gene therapy product(s) in the next 5-7 years with selected support from NIH. Specifically our research community has identified a "Target 10" group of diseases and disorders listed below based on early clinical success:

- Leber congenital amaurosis (LCA)
- Adenosine deaminase severe combined immunodeficiency (ADA-SCID)
- Hemophilia
- X-linked severe combined immunodeficiency
- Parkinson disease
- Age-Related Macular Degeneration (AMD)
- Adrenoleukodystrophy (ALD)
- Thalassemia
- Epstein Barr Virus (EBV) Lymphoma
- Melanoma

As stated above the clinical success of gene and cell therapy for these diseases will reach well beyond the effective treatment of one indication. We are collaborating with the Office of Rare Diseases Research to classify rare diseases by common clinical phenotype. With rare diseases grouped together, our hope is that we will no longer encounter diseases that are “too rare to treat”.

In addition, the Society has long recognized the need for more effective and timely review of gene therapy products to ensure vital therapeutics are able to move efficiently from bench to bedside. ASGCT has, therefore, established a panel of experts to evaluate the evolving role of the NIH Recombinant DNA Advisory Committee (RAC) in fulfilling its mandate to advise you on the conduct and oversight of research involving recombinant DNA. At the conclusion of the evaluation, we will respectfully deliver a set of recommendations as to how the Society believes the role of the RAC could better meet the needs of the overall gene and cell therapy research community, NIH oversight and public awareness.

Next Spring, the incoming ASGCT President, Dr. Xandra Breakefield, will be in touch to schedule follow up meetings with the NIH Institute Directors regarding the progress of the “Target 10” therapeutics, the ORDR classification of rare diseases, and our recommendations on the evolving role of the RAC.

Thank you again for the opportunity to host a Symposium on the NIH campus and for consideration of the Society’s observations and suggestions. I am happy to discuss these issues with you further to determine the most productive and expeditious path forward in this exciting area of translational science. I can be reached by phone or email at your convenience [(919) 962 – 3285; rjs@med.unc.edu].

Sincerely,

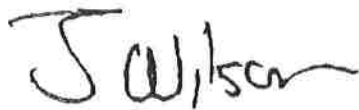
A handwritten signature in black ink, appearing to read "R. Jude Samulski". The signature is fluid and cursive, with the first name "R. Jude" and last name "Samulski" clearly distinguishable.

R. Jude Samulski, PhD  
ASGCT President


Co-Signed by the Past Presidents of ASGCT:



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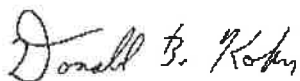
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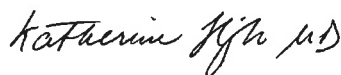
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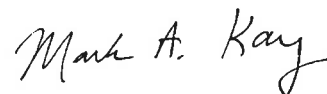
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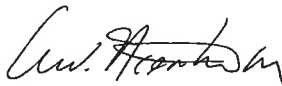
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cc: ASGCT Board of Directors