



October 15, 2018

Office of Science Policy  
National Institutes of Health  
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The following are comments from the Alliance for Regenerative Medicine (ARM) in response to the policy ("the policy") published in the Federal Register on August 17, 2018 entitled "Recombinant or Synthetic Nucleic Acid Research: Proposed Changes to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules (NIH Guidelines).

ARM is the leading international organization advocating for policies to support research and commercialization of regenerative medicine, cell and gene therapy, and other advanced therapies. Its more than 300 members include life sciences companies, research institutions, clinical centers, patient advocacy groups, and investors.

Since its inception, ARM has worked with the NIH to promote research and eventual commercialization of gene therapy. ARM has previously supported the Recombinant DNA Advisory Committee (RAC) in its mission to publicly discuss novel technologies. ARM has also expressed concerns with the duplicative nature of RAC review and reporting requirements.

Consequently, ARM strongly supports for the policy detailed by the NIH in the policy. Specifically, ARM welcomes the changes to NIH Guidelines that no longer require submissions to the RAC for gene therapy clinical trials. Gene therapy research and clinical development have become more prevalent in the last 40 years and the understanding of the complex scientific, ethical, and legal issues related to recombinant DNA technology has grown as well. The RAC was initially created because of the novelty of gene transfer and concerns about public acceptance and understanding of the technology.

This is no longer true. Several gene therapy products have now been approved by the US Food and Drug Administration. ARM has previously noted that gene therapy has significantly advanced since the establishment of the RAC, and Institutional Biosafety Committees (IBCs) have sufficient expertise and experience to ensure patient safety.

In addition, with hundreds of trials underway – many in later stages – the FDA knowledge of gene transfer and related technologies provides appropriate oversight. FDA has issued over a dozen guidance documents on the development of cell and gene-based therapies, including several in the last year. Additional review from the RAC is no longer needed. The review and



data reporting requirements as previously required by NIH Guidelines were duplicative and ARM is pleased that NIH has removed them from its guidelines.


We were also pleased to see that the policy maintains an ongoing role for the RAC. ARM supports the role of the RAC as a public forum to advise on issues related to new biotechnologies. For example, ARM would endorse RAC efforts to organize scientific workshops on gene therapy, particularly for novel approaches.

In addition, ARM recommends that the RAC clarify the role of IBCs going forward. The RAC should consider providing general guidance for IBCs (including potentially templates such as shortened/simplified Appendix M.). We anticipate this will be particularly helpful for IBCs that do not have a lot of experience with gene therapy.

ARM has previously expressed concern about the confidentiality of data provided to the RAC. Since commercial sponsors will no longer be required to report to the RAC nor have individual protocols reviewed, we anticipate this concern no longer applies. ARM encourages the NIH/RAC to clarify it will no longer require such information. However, due to the previous policy, the RAC remains in possession of trade secret and confidential commercial information. RAC should clarify that it will notify sponsors if any of their data may become publicly available.

ARM looks forward to continuing to work with the NIH/RAC to foster research in gene therapy and related technologies.

Respectfully,

A handwritten signature in black ink, reading "Robert J. Fall". The signature is written in a cursive, flowing style.

Director, U.S. Policy and Advocacy