October 16, 2018

Office of Science Policy
National Institutes of Health
6705 Rockledge Drive, Suite 750
Bethesda, Maryland 20892

Re: National Institutes of Health (NIH) Office of Science Policy (OSP) Recombinant or Synthetic Nucleic Acid Research: Proposed Changes to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules (NIH Guidelines)

Dear Sir/Madam:

The Biotechnology Innovation Organization (BIO) thanks the National Institutes of Health (NIH) for the opportunity to submit comments regarding the Proposed Changes to the NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial, and environmental biotechnology products.

BIO applauds the NIH's efforts to support and advance drug development for gene therapies. In particular through the streamlining of protocol registration and reporting requirements, and modification of the roles and responsibilities of the Recombinant DNA Advisory committee. As the NIH notes in the announcement, the goal is to eliminate duplication and excess as it related to gene therapy products regulation, redundancies that do not exist in most other areas of clinical research. BIO supports the comment made by the NIH indicating that, "oversight mechanisms for ensuring HGT [human gene transfer research] proceeds safely have sufficiently evolved to keep pace with new discoveries in this field." BIO believes that there is currently sufficient and robust regulatory framework in place for safe and effective development of gene therapy products.

We would like to note that in the Federal Notice, there is a requirement for an Institutional Biosafety Committee (IBC) approval from the study site before initiation of the study. However, many study sites do not have an established IBC. Therefore, this requirement could impede study initiation and enrolment as it takes a considerable amount of time to establish an IBC at the study site. Potential study sites could be dropped due to the inability to constitute an IBC. Hence, we respectfully propose that initiation of the study be allowed with Institutional Review Board (IRB) approval only at study sites where there are no IBCs. In addition, IBC may not have the same depth of experience when reviewing gene therapy protocols. We encourage the Agency to define more clearly the transfer of responsibilities, as well as the IBC review process

BIO appreciates this opportunity to submit comments regarding NIH's Recombinant or Synthetic Nucleic Acid Research: Proposed Changes to the NIH Guidelines for Research

Involving Recombinant or Synthetic Nucleic Acid Molecules. We would be pleased to provide further input or clarification of our comments, as needed.

Sincerely,

/S/

Sesquile Ramon, Ph.D.
Director, Science & Regulatory Affairs
Biotechnology Innovation Organization