Biotechnology Innovation Organization

Position Statement on Human Genome Editing

Adopted September 2017

Genome editing is an emerging technology that allows for the targeted modification of DNA for a multitude of applications. In human health, researchers are exploring ways to use genome editing of somatic cells to treat or prevent genetically defined human diseases.

Clinical use of genome editing in somatic cells, or non-heritable cells, is currently subject to oversight similar to that of other advanced biotherapeutic technologies such as gene therapy. Specifically, the combined activities and responsibilities of the US Food and Drug Administration (FDA), through its statutory role as the regulator of drug development, and the NIH/Recombinant DNA Advisory Committee (RAC), as the forum for public discussion, serve in this oversight capacity to protect patients while ensuring that important research moves ahead.

In February 2017, the National Academy of Sciences and the National Academy of Medicine Committee on Human Gene Editing, after careful review of scientific, ethical and public views on human genome editing, released a report supporting the current regulatory and oversight mechanism for somatic cell gene editing for the potential treatment of disease. The report also concluded that genome editing in humans for purposes other than treating or preventing a disease or disability should not proceed at this time. BIO fully agrees with these two conclusions.

Regarding the clinical use of genome editing in germline cells (where the DNA edit is passed on to future generations), the 2017 authors noted that “there is a need for caution in any move toward germline editing, but that caution does not mean prohibition.” The Committee concluded that under a very strict set of 10 criteria, including when 1) no other reasonable alternatives exist, 2) government restrictions have expired, and 3) research on risk/benefit standards has advanced in the field, certain germline editing could be permissible for the treatment of disease.

BIO views the science of germline genome editing as having not advanced sufficiently for clinical applications to be appropriate at this time. As scientific developments progress, BIO urges continued discussion and engagement on this topic with important stakeholders, including members of the patient, caregiver, regulatory, legal, academic, ethical, and faith communities, to determine if and under which conditions this status quo should be changed.