# Gene Editing Task Force – Therapeutic Developers' *Statement of Principles*

As developers of therapeutics that utilize gene editing technologies, we support a bioethical framework for genetic modification consistent with these principles:

## I. We Endorse Investigation of Therapeutic Applications of Somatic Cell Gene Editing

As researchers and developers of gene editing technologies, we support the use of gene editing techniques for therapeutic modifications to somatic cells under the oversight of relevant national or regional regulatory bodies.

Human gene editing, a technique by which DNA is inserted, replaced, removed or corrected at specific locations within the human genome, is a rapidly developing technology sector with the potential to provide important, perhaps life-saving, treatments for patients.

Gene-edited somatic cells do not pass genetic modifications to subsequent generations, in contrast to gene editing of the germline which results in heritable changes. We do not support gene editing of the germline (sperm, eggs, fertilized embryos) for the purposes of human implantation. Further, we do not support implantation of a human embryo carrying gene modified cells. Most genetic diseases can be treated directly in affected somatic cells without modifying the germline, and we support such therapeutic use.

Somatic cells account for the majority of cells in the human body, including those in our internal organs, skin, bones, blood, and connective tissue. In addition, we consider pluripotent stem cells as somatic cells for the purposes of this document. Gene editing therapies targeting somatic cells for therapeutic application are under clinical evaluation in a number of countries.

Regulated, clinical validation of somatic cell-based gene editing technologies for non-inherited genetic modification is, and should remain, the primary objective of the therapeutic development community and, in contrast to germline gene editing, offers the most acceptable near-term path to potentially transformative therapeutic benefits for patients.

## II. We Support the Use of Gene Editing Standards to Facilitate the Development of Safe and Efficacious Gene Editing Therapies

We recognize and support the ongoing work of the NIST Genome Editing Consortium, US Pharmacopeia, International Organization for Standardization (ISO), and other recognized standards development organizations to formulate gene editing standards that address key

concepts such as off-target effects and their impact on tumor suppressors and oncogenes as well as the measurement and monitoring of genetic mosaicism.

## III. We Call for the Continued Evolution of National and Regional Regulatory Frameworks Governing the Development of Somatic Cell Gene Editing Techniques

Existing national and regional regulatory frameworks, such those of FDA and EMA, provide sound and effective oversight for the development of gene editing techniques for therapeutic modification of somatic cells. We believe that evolving national and regional regulatory frameworks are important to support appropriate development of these technologies and should act as the primary regulatory and enforcement mechanism. It is our belief that arbitrary and ancillary oversight bodies or processes may carry the risk of delaying research and development efforts, which in turn would adversely impact afflicted patient populations.

## IV. We Assert that Germline Gene Editing Is Currently Inappropriate in Human Clinical Settings

We, as therapeutic developers utilizing gene editing technologies, are not modifying human germline cells for use in human clinical studies. Gene editing technologies have not matured to the point where human trials of edited germline cells are appropriate. Many important safety, ethical, legal, and societal issues involved with this type of gene editing remain unresolved.

## V. Common Commitment

We, as therapeutic developers utilizing gene editing technologies, are solely focused on somatic cell approaches to therapeutic treatments and cures for disease. Unless and until ethical and potential safety questions with respect to germline gene editing are adequately addressed, we do not support or condone germline gene editing in human clinical trials or for human implantation. We believe that these are international concerns and would be supportive of an effort to discuss therapeutic gene editing issues on a global stage.

We the undersigned are committed to this Statement of Principles:

**Audentes Therapeutics**
**bluebird bio**
**BlueRock Therapeutics**
**Caribou Biosciences**
**Casebia Therapeutics**
**CRISPR Therapeutics**
**Editas Medicine**
**Homology Medicines**
**Intellia Therapeutics**
**LogicBio Therapeutics**
**Precision Biosciences**
**Sangamo Therapeutics**
**Tmunity Therapeutics**