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The Alliance for Regenerative Medicine Releases Statement of Principles on Genome Editing

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WASHINGTON, D.C. – August 27, 2019 – The **Alliance for Regenerative Medicine** (ARM), the international advocacy organization representing the cell and gene therapy and broader regenerative medicine sector, today released a Therapeutic Developers' Statement of Principles, setting forth a bioethical framework for the use of gene editing in therapeutic applications.

The statement, developed by ARM's Gene Editing Task Force and signed by 13 therapeutic developers using gene editing technologies, specifies five key principles for the ethical use of gene editing and genetic modification. This technology, in which DNA is inserted, replaced, removed, or modified at particular locations in the human genome, has the potential to provide a durable or curative effect for patients suffering from a wide variety of serious or potentially fatal genetic disorders. The Statement of Principles has been developed to provide a public perspective from industry on the use these technologies.

The principles stated in the document are:

1. **We endorse investigation of therapeutic applications of somatic cell gene editing:**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.

2. **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.

3. **We call for the continued evolution of national and regional regulatory frameworks governing the development of somatic cell gene editing techniques:** 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.

4. **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.

5. **Common commitment:** 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.

Janet Lambert, CEO of ARM, commented, “Gene editing is a rapidly developing technology that represents one of the most exciting developments in medicine. These techniques will be integral to the next generation of advanced therapeutics and we welcome their potential to provide important, and potentially life-saving, treatments for patients.

“As with all breakthrough biotechnologies, we need to exercise caution and good stewardship in our research and development practices and ensure that work involving the genetic modification of cells takes place within the bioethical framework outlined in these principles.”

Sandy Macrae, CEO of Sangamo Therapeutics and co-chair of ARM’s Gene Editing Task Force, commented, “We are delighted to have worked with our sector peers and alongside ARM to agree to these common principles and standards as we look to establish best practices and uphold the highest standards whilst developing these exciting technologies. As therapeutic developers utilizing gene editing technologies, we must ensure that our efforts, above all else, are carried out in a safe and ethical

matter. With our current base of knowledge this means focussing solely on approaches that do not alter the germline unless and until ethical and potential safety questions with respect to germline gene editing are adequately addressed."

The full document is available here link.

A full list of signatories includes:

- 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

About The Alliance for Regenerative Medicine

The Alliance for Regenerative Medicine (ARM) is an international multi-stakeholder advocacy organization that promotes legislative, regulatory and reimbursement initiatives necessary to facilitate access to life-giving advances in regenerative medicine worldwide. ARM also works to increase public understanding of the field and its potential to transform human healthcare, providing business development and investor outreach services to support the growth of its member companies and research organizations. Prior to the formation of ARM in 2009, there was no advocacy organization operating in Washington, D.C. to specifically represent the interests of the companies, research institutions, investors and patient groups that comprise the entire regenerative medicine community. Today, ARM has more than 350 members and is the leading global advocacy organization in this field. To learn more about ARM or to become a member,
visit http://www.alliancerm.org.

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