Joint Statement of Intellia Therapeutics & CRISPR Therapeutics: Position Regarding Human Germline Gene Editing

December 1, 2015

Therapeutic applications of gene editing, including CRISPR/Cas9 and other CRISPR-based technologies, provide an unparalleled opportunity for treating, or even curing, patients with serious genetic disorders. These exciting, recently discovered technologies have the potential to bring new treatments with remarkable speed and relative simplicity to patients.

Most genetic disorders typically manifest in somatic (non-germline) cells. Treating somatic cells with older gene therapy methods is widely accepted, with a well-established and tested regulatory framework that already exists in both the United States and Europe. The vast majority of genetic and other diseases can be addressed by gene editing of these somatic cells and do not require modification of germline cells, the source of genetic information passed on to subsequent generations.

Regulatory agencies have already approved somatic cell gene editing technologies for testing in patients and the existing regulatory framework is both sound and appropriate for gene editing research. As such, any delays in research or product development caused by unnecessarily modifying existing regulations would adversely impact patients whose lives are dependent on the availability of new treatments for severe and sometimes fatal genetic diseases.

We acknowledge that some applications of gene editing raise important ethical issues, specifically when used for non-therapeutic purposes or for modifying the genomes of germline cells with the intention of affecting future generations. Before embarking on the potential clinical applications of germline gene editing, our society must address the challenging safety and ethical questions raised by changing the genetic makeup inherited by future generations. This can only be achieved through discussion among a broad range of stakeholders.

We are supportive of the structured global dialogue on germline gene editing hosted by the United States National Academies of Medicine and Sciences, the Chinese Academy of Sciences, and the Royal Society of the United Kingdom. We note that germline gene editing is outside of the scope of our companies' research and development. We are dedicated to discovering and developing gene editing-based treatments for serious diseases using only non-germline somatic cells. This is the greatest area of patient need, where the benefits and risks are best understood, and where the ethical support is unambiguous.

To that end, we are committed to:

- Using gene editing technologies, including CRISPR/Cas9, only for patient benefit.
- Focusing on treating serious diseases of high unmet need.
- Refraining from directly modifying germline cells, including sperm, egg or embryonic tissue, or developing any clinical applications of germline gene editing.

We believe it is of the utmost importance – and ethical relevance – to advance, without delay, the powerful potential of gene editing and CRISPR-Cas9 in particular, to treat the millions of patients who suffer from very serious diseases. Their needs must be at the forefront of our minds as we advance gene editing both in the laboratory and in the public forum. These are exciting times for the medical community and patients alike as we develop breakthrough treatments for some of the toughest diseases facing individuals and society today.

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