CRISPR/Cas9 in Human Research—A Call for Unity in Medical Ethics

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The CRISPR-associated RNA-guided endonuclease Cas9 system has revolutionized the field of genetic editing, having almost unlimited potential in bioengineering and medicine. The ability to remove a pathophysiological abnormality through therapeutic intervention using the CRISPR/Cas9 system represents a true possibility of treating rare and complex genetic disorders. Since first being described in 2012, more than 1,500 publications have reported the use of this technology in various scientific areas. At least 10 companies are currently exploring the utility of this type of gene editing in human diseases.

Recently, an investigator at the Southern University of Science and Technology in Shenzhen, China, Dr. Jiankui He, reported the first use of gene editing in humans, whereby he described the birth of twin girls lacking the CCR5 gene.1 As first reported at the Human Genome Editing Conference held in Hong Kong in late November of 2018, Dr. He described the as-yet-unsubstantiated results of using the CRISPR/Cas9 technology to genetically remove the CCR5 gene from human embryos, resulting in live birth. Since then, the scientific and medical communities have largely denounced this work, notably including the Committee of Genome Editing of the Genetics Society of China and of the Chinese Society for Stem Cell Research.2

Although Dr. He’s work has not been confirmed either informally or through peer-reviewed publication, it has raised serious concerns and important discussions on the ethics of human research with regard to gene editing and the urgent need for international agreement on the ethical responsibilities that will follow in this field. The medical ethics of human embryo research have been debated for many years, but the report of a scientist pursuing genetic manipulation through human birth has escalated the need for establishing a firm commitment to strong ethical standards in medical research.

There have been great advances in the understanding of CRISPR/Cas9 over a relatively short period of time; however, some studies, including one in 2016 describing genome damage, large deletions and genetic crossover events as a result of the gene editing process, have served to underscore the reality that we do not yet fully understand the changes occurring and the potential pathogenic consequences of such events.3 Without complete knowledge of the process itself nor of the potential serious consequences of the end result, it is imperative that we, as a scientific community, pause the further use of gene editing of human embryos, at least until the knowledge base has progressed. The rogue use of CRISPR/Cas9 and failure of peer-reviewed oversight threatens the fundamental basis of scientific research as a whole.

Research that pushes established boundaries in an ethical manner, that questions previously held dogma, and that constantly strives to improve and refine scientific knowledge represents the core aim of Exploratory Research and Hypotheses in Medicine. Yet, these types of explorations must be conducted in a methodical manner and not applied to human research until the consequences are fully understood and a consensus is established on acceptable standards. We are not there yet with the CRISPR/Cas9 technology, and until we are rogue human research must not happen: primum non nocere.

References