Genes at stake

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pISSN 2093-2278
eISSN 2093-2286

JPIS Journal of Periodontal & Implant Science

Editorial

J Periodontal Implant Sci 2015;45:37-37
http://dx.doi.org/10.5051/jpis.2015.45.2.37

Received: Apr. 20, 2015
Accepted: Apr. 20, 2015

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We are living in the era of genetic engineering and looking forward to its eventual clinical application to life-threatening human diseases. In this context, we appreciate the UK government's decision in February to allow the clinical application of mitochondrial DNA transfer techniques to women who carry defective mitochondrial genes. Such genes can produce unhealthy offspring.

Now we face another report from daring researchers in China (doi: 10.1007/s13238-015-0153-5). This month, Dr. Junjiu Huang and colleagues at Sun Yat-Sen University in Guangzhou performed human embryo genome editing to correct β-thalassemia by adopting the clustered regularly interspaced short palindromic repeat-associated system (CRISPR/Cas9). Although they found that CRISPR/Cas9 could effectively cleave the endogenous β-globin gene (HBB), the efficiency of homologous recombination-directed repair of HBB was low and the edited embryos were mosaic. They concluded that gene editing by this method is not practical for clinics, highlighting the pressing need to improve the accuracy of gene editing technology.

Previous human genetic modification efforts have been focused on non-reproductive somatic cells using zinc-finger nucleases, DNA-binding proteins that can be engineered to induce a double-strand break in a section of DNA incorporating a new stretch of DNA into a selected location, to fix a mutation that could cause disease. CRISPR/Cas9 is the newest and simplest method to use RNA molecules that recognize specific human DNA sequences. Human embryonic research using CRISPR/Cas9 raises anxiety because it shows that we are still not ready to precisely control our reproductive cells.

In light of the outcomes reported by Dr. Huang and colleagues, we should recognize the urgent need for guidelines on genomic editing studies on human germ cells. Our knowledge about genes remains limited, and the outcome of embryonic gene editing can be confirmed after birth only. We must first and foremost clarify whether and when genomic editing research in reproductive cells should take place. As responsible scientists, we can expect vigorous debate over the benefit to patients.

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