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Editorial

Editorial Note on Gene Therapy

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EDITORIAL

Variable number tandem repeat sequences, or VNTRs, are repeat sequences that vary in length from one person to the next. Excess probe molecules are washed away after the probe molecules hybridize to DNA fragments containing the repeat sequences. After that, the blot is exposed to an X-ray film. On the film, DNA fragments that have bound to the probe molecules appear as fluorescent bands. In a trial that began in September 1990, French Anderson conducted the first therapeutic use of gene transfer as well as the first direct injection of human DNA into the nuclear genome. It is thought to be capable of curing or treating many genetic diseases over time.

Gene therapy is based on the idea of correcting a genetic problem at its root. If, for example, a mutation in a gene causes the development of a defective protein in an inherited disorder, gene therapy may be used to deliver a copy of the gene that does not contain the deleterious mutation, resulting in the production of a functional protein. Gene replacement therapy is the name for this process. The advent of CRISPR gene editing has opened up new avenues for its use and use in gene therapy, as it allows for the reversal of a genetic mutation rather than only replacing a gene. Eradication of latent human immunodeficiency virus (HIV) reservoirs and correction of the sickle cell disease mutation are examples of medical challenges. For parents, genetic engineering may be another tool to complement diet, exercise, schooling, training, cosmetics, and plastic surgery in the development of their children. Moral considerations, according to another theorist, restrict but do not preclude germline engineering.

The journal Bioethics recently published an article on the moral issues surrounding human germline genetic engineering. A full prohibition, universal provision, or professional self-regulation are all possible regulatory schemes. "Genetic treatments to improve characteristics should be deemed appropriate only in severely restricted situations" according to the American Medical Association's Council on Ethical and Judicial Affairs. For treating certain genetic disorders, gene therapy methods to substitute a defective gene with a safe gene have been suggested and are being researched. Diseases induced by autosomal recessive disorders, such as sickle cell disease, in which a person's natural phenotype or cell function may be restored in cells with the disease by a normal copy of the mutated gene, may be a good candidate for gene therapy treatment.

Three patients have died in gene therapy trials, prompting increased examination of the area. Jesse Gelsinger, who died in 1999 as a result of an immune rejection response, was the first. In 2003, one X-SCID patient died of leukaemia. A rheumatoid arthritis patient died of an infection in 2007, and an investigation determined that the death had nothing to do with gene therapy. General guidelines for human-involved scientific research include regulations on genetic modification. There are no legally binding international treaties in this region, but numerous bodies have issued guidelines for national legislation.

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