Gene therapy recent clinical applications

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GENE THERAPY / GENETICS AND GENOMICS / SPECIAL REPORTS

As we said in a previous article gene therapy is facing a new promising stage (see WHY). On 9 January this year, a review was published in **JAMA** on the latest advances in **gene therapy**, making reference to specific advances in humans.

- The first refers to gene therapy treatment of a 7-year-old boy with a serious life-threatening skin disease, **epidermolysis bullosa**, which affected 80% of his body. In children who suffer this disease, the skin is severely and extensively eroded. In this case, the experts regenerated small areas of skin with transgenically produced skin using a retroviral vector that contained the normal *LAMB3* gene, which is the one that, when altered, causes the disease. The skin grafts were applied to most of his body in three successive surgical procedures. After 21 months of follow-up, the skin developed normally and was not rejected. The study authors think that the transplanted skin will remain stable, allowing the child to be cured. They are now conducting clinical trials for other forms of epidermolysis bullosa, a disease that affects around 500,000 people worldwide.

- Another different team recently conducted the first **human gene editing** experiments, specifically in a man with a hereditary metabolic disorder called mucopolysaccharidosis type II or Hunter's syndrome that affescts 150.000 male newborn (see HERE). The experiments are being carried out at the San Francisco Benioff Children´s Hospital in Oakland California. On this occasion, an experimental gene, *SB-913*, is being evaluated in 9 adults with this disease. If the disease is not properly treated, patients may die before 20 years of age due to airway obstruction, respiratory infections or heart failure.
- Another application of gene therapy is occurring in the **field of cystic fibrosis** (see BMJ article) .
- New advances have also been made using gene therapy in the field of hemophilia. In fact, a recent small clinical trial has shown that the concentration of factor VIII can reach practically normal levels in 52 weeks after a single injection of factor VIII in **hemophilia A patients** (see HERE). These *early trials* have been extended with two recent studies published in the New England Journal of Medicine (**see HERE** and **HERE**https://www.nejm.org/doi/full/10.1056/NEJMoa1708538), which describe two successful clinical trials for two types of hemophilia, A and B. While we cannot go into the technical details of either trial here, both are well founded from a **basic point of view** https://www.nature.com/articles/nm.4492. These results are not only encouraging for this specific disease, hemophilia, but could also mean a positive step forward in the field of gene therapy and more specific treatments, such as those that are taking place in eye pathology and **central nervous system diseases (see HERE).**

See our article and video about a gene therapy for "Bubble boy" disease approved in Europe last year.

From the Observatory of Bioethics of the Catholic University of Valencia, we congratulate ourselves on these medical advances that open an avenue to treat important diseases until now without a specific treatment and especially because we do not find bioethical difficulties in these gene therapies practices (see HERE).

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