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Gene editing to prevent hereditary diseases

Alcimed, an innovation and new business consulting firm, highlights recent scientific developments in gene editing and discusses its future outlook.

June 19, 2017. Gene editing accelerates due to CRISPR/Cas9.

After several decades of slow development because of high cost, low efficiency, and poor accuracy of past techniques, the field of gene editing has undergone a dramatic expansion since the first application of CRISPR/Cas9 in 2012. Earlier this year, two major events occurred that were largely unnoticed by the general public, but have major bearing on the future of gene editing for somatic cells (all cells apart from the reproductive cells).

The ruling on intellectual property for CRISPR/Cas9 allows further industry investments to edit the genome of somatic cells.

Judges gave the first ruling in the patent battle for the rights to the CRISPR/Cas9 technology, favoring the Broad Institute over UC Berkeley. Although the dust has not settled on this legal matter, biotech companies, such as Editas, which have already licensed from the Broad Institute, can move forward to clinical applications of gene editing in somatic cells. Modifying the genome of somatic cells has enormous potential to treat rare genetic diseases, as well as more common diseases, such as heart failure and Alzheimer's.

For the first time, experts have openly considered the possibility of clinical trials for germline editing to prevent serious hereditary diseases, despite complex regulatory and ethical hurdles.

The second recent event is the publication of a report by experts from the National Academies of Sciences, Engineering, and Medicine, recommending that clinical trials be considered for therapies that modify the germline.

Editing the genome of germline cells (eggs, sperm, or embryos) is ethically fraught, as it creates inheritable modifications. Nevertheless, this new report acknowledges the powerful drivers of developing treatments for inheritable diseases for which there is no alternative. For example, there has been a rapid adoption of non-invasive prenatal testing, which analyzes the fetus' circulating DNA in the mother's blood to detect genetic anomalies.[1] Another trend that may highlight potential interest in germline editing is the surge of prophylactic surgery to reduce cancer risk.[2]

Alcimed expects increasing interest for germline editing, as Pharma and biotech leaders engage with the public and collaborate with lawmakers to advance gene therapy.

The blindingly fast-paced research shows how quickly germline editing could become feasible. Apart from clinical therapies, this technology has numerous potential applications, such as hornless cows and pest control for agriculture, or the eradication of mosquito-borne diseases. Nevertheless, the American public is at least 68% worried about the safety and responsible use of germline editing technology.[3] Alcimed anticipates that Pharma and biotech will look to leverage these developments and respond to whether or not the same types of applications can be extended to human lives.

About Alcimed

ALCIMED (www.alcimed.com) is an innovation & new business consulting firm, specialized in life sciences (healthcare, biotech, agri-food), chemicals, materials, energy; as well as aeronautics, space & defense. ALCIMED relies on a team of 200 highly-skilled individuals to help its clients with exploring and developing their uncharted territories, covering four key areas: New Technologies, Market Innovation, High-Growth Geographies, and Strategic Foresight. ALCIMED is headquartered in Paris and has offices in Lyon & Toulouse in France, in Germany, Belgium, Switzerland, the UK, the USA and in Singapore.

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1 State of the NIPT market

2 Annals of Surgery

3 Pew Research