Medium

May 15, 2019

# The FDA in The Era of Gene Therapy

**By Veronica Pamoukaghlian Viera**

Last year, the FDA vowed to boost its efforts to advance the development of new gene therapies. To support innovation, the agency said, it had already approved three gene therapy products while also issuing valuable guidance for players in the field.

Gene therapy is a technique used to treat certain disorders by inserting a gene into a patient's cells rather than resorting to riskier and often less effective drugs or surgery. Current regulations establish that research involving genetic modifications in human embryos is forbidden.

A recent study, however, 'edited' babies DNA in order to make them immune to the HIV virus. As with any regulatory framework developed around innovation, FDA regulations risk hindering advance in the new field. While it is important to make sure scientists are not "designing" babies tailored to their parents' taste; in many cases, gene therapy as applied to embryos can be crucial for research.

> The high cost of some of these genetic treatments can make them be perceived by pharma as bad investments. The challenge in the near future will be to find a way to incentivize research and development, even when a specific gene therapy is tailored to treat a small group of individuals with certain genetic characteristics.
>
> — *Greg Glover*
> Pharmaceutical law expert and non-practicing physician

Genetic modification in humans falls into different categories. Gene therapy and genetic editing of babies are fundamentally and ethically different, and this is something regulators need to understand. Gene therapy is used to treat a disease, while genetic enhancement means making changes in healthy organisms. Moreover, if DNA alterations do not affect reproductive cells, the future children of the subjects undergoing the processes will not suffer any genetic modifications.

The type of gene therapy being developed today is focused on altering somatic rather than reproductive cells, in order to treat diseases where faulty genes play a significant role.

When scientists first started cloning living beings, there



Continued on next page

was a similar discussion. Researchers cannot 'play god,' many people claimed. But, then like now, if we ban scientific advancement for ethical reasons, we need to ensure that there is truly an ethical problem, and one that poses more risks than the benefits the questioned innovations will bring.

A United Nations panel has recommended that genetic editing of reproductive human cells be banned. The UN's Bioethics Committee said in a 2015 statement that, "interventions on the human genome should be admitted only for preventive, diagnostic or therapeutic reasons and without enacting modifications for descendants."

But the UN cannot simply implement these policies globally. The inherent risk is that some countries will have more lax — or even non-existent — regulations, and that 'unethical' scientists will migrate there, much like massive fortunes and dirty money flock to tax havens from countries with stringent anti-money laundering regulations and high taxes on wealth.

Efforts to unify currency and create a uniform system that would make tax evasion impossible have failed time and again. And it doesn't look like it is going to be any easier to globally regulate biomedicine. Some jurisdictions, however, have made some progress in that respect. Under the European Convention on Human Rights, "the genetic constitution of the individual is to be protected against unlawful interventions seeking to modify the germline."

For Greg Glover, a pharmaceutical law expert and non-practicing physician, personalized medicine is the future, but it will pose many challenges. "The high cost of some of these genetic treatments can make them be perceived by pharma as bad investments. The challenge in the near future will be to find a way to incentivize research and development, even when a specific gene therapy is tailored to treat a small group of individuals with certain genetic characteristics. A recent analysis by Goldman Sachs found that curing hepatitis-C was not a sustainable business model for GILEAD Sciences, and I believe the key to using innovative biotech and gene therapy to cure disease is to start thinking of collective benefits, rather than benefits for separate entities. The ethical aspects of genetic modification are important, but if we don't make it worthwhile for the industry to develop new therapies, we are going to have even bigger problems."

In spite of ethical discussions, the advance of gene therapy seems unstoppable today. A recent survey showed there were over 800 related applications with the FDA. The only approved products so far are Kymriah and Yescarta, designed to treat cancers of the blood, and Luxturna, for an inherited disorder that affects the retina.

To keep up with the field, the FDA has had to grow its base of gene therapy experts. Meanwhile the National Institute of Health is revamping its DNA research committee to become the Novel and Exceptional Technology and Research Advisory Committee, amping up its efforts to evaluate the ethical and safety challenges of the new applications of emerging biotechnologies.

The FDA's guidance documents on gene therapy, released last year, received abundant public commentary, and will soon be finalized. The agency is actively trying to establish itself as an organization dedicated to foster the development of this disruptive technology, rather than hinder its growth. ■