Gene Patenting & Gene Therapy
House Committee on Science, Space, and Technology

Background

Gene patenting and gene therapy have been topics of debate ever since the Human Genome Project came to a conclusion in April 2003. The Human Genome Project was the international study of human DNA that culminated in the full sequence of the human genome. Ever since, the field of genetics has expanded into countless studies on the human genome and how it affects the body. The field of genetics has also been commercialized, in both the public and private sectors. Notably, through the study of the human genome, the company Myriad Genetics discovered the *BRCA1* and *BRCA2* genes and their link to breast cancer. Myriad then patented the genes so that they may be the only company to produce drugs to help prevent breast cancer and alleviate its effects. This angered many; people argued that Myriad had not actually invented anything and thus could not put a patent on the genes. Some furthered the argument by stating that Myriad could not own the genes as they are part of the human body. Others still argued that the patents prevented clinics from helping patients unless they paid a fee to Myriad Genetics. Supporters for Myriad claimed that without the monetary incentive of the patent, companies such as Myriad Genetics would not put nearly as much effort into research.

This debate was taken to the Supreme Court in June 2013 in the case of *Association for Molecular Pathology v. Myriad Genetics*. The Association for Molecular Pathology wanted Myriad's patents to be revoked. The court ruled in favor of the Association for Molecular Pathology. Justice Clarence Thomas wrote to the court that although Myriad had discovered an important gene, it was not an act of invention and any discovery, no matter how innovative, does not warrant a patent. As a result of this ruling, five of Myriad's patents were revoked. However, the court allowed for the patenting of cDNA, an artificial strand of DNA that is used in the process pioneered by Myriad. Other varieties of cDNA are used in a large variety of genetic processes. cDNA is created by taking ribosomal DNA, or rDNA, and feeding it through an enzyme known as Reverse Transcriptase. The court ruled that this process would fall under the definition required for a patent. Based off of the court's ruling, each of those strands of cDNA can be patented if it is different from any created before. Myriad Genetics still has 24 different patents on cDNA.

Gene patenting provides a massive incentive for companies to research new developments in the field of genetics. If there is only one company that can do research, there will be a large spark of competition which greatly accelerates research. This leads to many beneficial scientific advances. On the other hand, gene patenting might lead to a monopoly on the research. If Myriad had kept the patents on the *BRCA1* and *BRCA2* genes, then no other company would have been allowed to monetize their research on those genes, and as such would not have had any incentive to research them in the first place. This monopoly on research could also lead to a steep increase in price for procedures as there would not be competition to keep the prices down.

Gene therapy is another field of science that was made possible with the completion of the Human Genome Project. Gene therapy is an experimental medical technique that utilizes the manipulation of a gene in the DNA of a patient to treat diseases. There are three main types of gene therapy techniques: the replacement of a mutated gene with a healthy one, the inactivation of a mutated gene, and the introduction of a new gene to help fight the disease. Currently, gene therapy

is only being administered to combat diseases with no other known cure. Gene therapy has proven to be effective against certain diseases including: inherited disorders, some types of cancers, and certain viral infections. Gene therapy is not widely used because it is still risky, but scientists are in the process of making procedures safer and more effective. In time, scientists could eliminate certain disorders entirely. However, the idea of manipulating the DNA inside of a person remains frightening to some. There are also ethical questions to answer if gene therapy is allowed to be performed as a routine medical procedure.

In April of 2012, 7-year old Emily Whitehead was diagnosed with acute lymphoblastic leukemia. After less than a month of gene therapy, the leukemia had been cured. Emily was the first child in the U.S. to receive this treatment, whereas Chinese geneticists had been performing these operations for much longer and had much success. This is because, as some claim, Chinese gene therapy regulations are too lax. In contrast, the U.S. FDA has very strict regulations. In fact, as of today, the FDA has yet to approve any gene therapy product for mass production, only for very specific cases, such as Emily's. However, China's FDA had approved the very first gene therapy product for commercial sale, Gendicine, in 2003.

Even if gene therapy regulations are made more relaxed in the U.S., there are ethical questions that need to be answered. For example, what constitutes a trait as a disorder? Gene therapy has thus far been only used to treat diseases. However, some have theorized that gene therapy could actually be used to alter any genetic trait, such as one's skin color or personality. However, many do not agree with altering DNA to this degree. In addition, there would be many social implications to this kind of gene therapy. Furthermore, if gene therapy would be commercialized, the prices would be very high. This would prevent many in poor economic conditions from receiving treatment. Finally, gene therapy can be used to enhance athletic and intellectual skill. This introduces a host of new issues, primarily for athletic and intellectual competitions. Should people who have been genetically altered to be better in these fields be allowed to compete with those who have not?

Gene therapy can potentially prevent many inherited disorders, including many forms of cancer. It can also be used to alter one's DNA in many other ways. This could open an entire branch of cosmetic genetic therapy. If the regulations were made less strict, there would be both medical and economic benefits. However, many are frightened by the concept of altering natural DNA.

Republican Point of View

Republicans are against both gene patenting and gene therapy. Their argument is that gene patenting encourages 'research monopolies' where only one company is allowed to research a gene. This causes prices for drugs created by that company to be unfairly high. John Watson, one of the co-discoverers of the double-helix structure of DNA, is against gene patents because of these research monopolies. "Scientists should be permitted to experiment on human genes free from any threat of patent infringement," he said. "Life's instructions ought not be controlled by legal monopolies created at the whim of Congress or the courts." In addition, republicans hold that a patent can only be given for something invented not something discovered, like a human gene. The party is split about the patenting of cDNA. Some state that it was created by the company, and as such it can be patented. However, others argue that because cDNA is created from a strand of DNA, companies are not truly inventing something new. Republicans are also against releasing any

FDA restrictions on gene therapy. However, most of the party agrees that regulations do not need to be made any more stringent than they already are. They argue that the current restrictions will prevent companies from creating any harmful products while also not restricting research. Most republicans are opposed to any sort of gene therapy that is not purely medical. However, there is some argument as to what defines a medical need for gene therapy.

Democratic Point of View

Democrats are for most gene patenting and gene therapy. They believe gene patenting and therapy promote healthy competition in research as many companies will be incentivized to make money with the patent. In addition, that competition will accelerate genetic research. Lori Andrews, a director of the Institute for Science, Law and Technology at the IIT Chicago-Kent College of Law, wrote in her post-ruling analysis, "the Myriad decision was good news for the pharmaceutical and biotech industries, enabling drug companies to tailor drugs to different patient populations and diagnostics developers to design new tests without paying royalty fees." This proved to be true as after the ruling, three competing companies of Myriad, Ambry Genetics, GeneDx, and DNATraits, all announced the availability of new *BRCA* tests. Democrats further argue that while the discovery of a gene's link to a disorder is not enough to warrant a patent, what a company does with that information is and that company should be the only one to work with that gene. Democrats are also pro gene therapy because it will prevent disorders. It will be able to do this on a larger scale if the products are able to be commercialized, and the only way to do that would be to release some of the restrictions of the FDA. In addition, democrats argue that there are methods to lower the price of gene therapy so that everyone can get the treatment that they need. One of these methods is the Affordable Care Act which would provide affordable health care to those in need. Most democrats are also pro cosmetic gene therapy as it would give a large economic incentive for companies to research gene therapy, again accelerating research.

Conclusion

Gene patenting and gene therapy both have the potential to help many people in need of treatment that they otherwise could not receive. Gene patenting will incentivize companies to research these treatments. However, the companies will then sell their products at potentially unfair prices to their consumers. With gene patenting illegal, there would be less incentive for companies to research genetics. Patenting laws also require one to invent something, not discover, as is evident in the Myriad Genetics case. Gene therapy can help many people on a larger scale if the FDA's regulations were made more relaxed, but then the products produced may be of a subpar quality. Furthermore, cosmetic gene therapy can be performed, but some believe that it is against nature to do so.

Questions to Consider

i. Does gene patenting promote competition or research monopolies?
ii. Should cDNA be able to be patented if individual genes cannot be?

iii. Can the process pioneered by a company through the use of a gene be patented?
iv. If not through gene patenting, how can companies be rewarded for their research?
v. What should be done with the FDA's restrictions on gene therapy?
vi. How does China's gene therapy restrictions compare with the U.S. restrictions?
vii. Is cosmetic gene therapy ethical?
viii. Should individuals be allowed to genetically alter their intelligence or athleticism?
ix. In what ways could the price of gene therapy be reduced?
x. What is the difference between medical gene therapy and cosmetic gene therapy?

Sources for Additional Research

- Nature (Academic Journal)
- http://www.nature.com/index.html
- Genetics Home Reference
- http://ghr.nlm.nih.gov/
- Genomics Law Report
- http://www.genomicslawreport.com/
- National Human Genome Research Institute
- https://www.genome.gov/
- Gene Therapy Net
- http://www.genetherapynet.com
- Science Daily
- http://www.sciencedaily.com/
- The Scientist Magazine
- http://www.the-scientist.com
- The Guardian on Science
- http://www.theguardian.com/science
- Scientific American
- http://www.scientificamerican.com/
- U.S. Food and Drug Administration Website
- http://www.fda.gov/

Bibliography

"All About the Human Genome Project." *National Human Genome Research Institute*. Last modified March 8, 2014. Accessed July 31, 2015. http://www.genome.gov/10001772.

Bergeson, Emilie R. "The Ethics of Gene Therapy." *NDSU*. Accessed July 31, 2015. https://www.ndsu.edu/pubweb/~mcclean/plsc431/students/bergeson.htm.

Cook-Deegan, Robert. "Australian Appeals Court Upholds Patents on Isolated BRCA1 DNA." *Genomics Law Report*. Last modified September 30, 2014. Accessed July 31, 2015. http://www.genomicslawreport.com/index.php/category/badges/myriad-gene-patent-litigation/.

"Gene Therapy Legislation in the US." *Gene Therapy Net*. Accessed July 31, 2015. http://www.genetherapynet.com/united-states-of-america.html.

Gray, Leila. "U.S. Supreme Court Decision to Bar Gene Patents Opens Genetic Test Options." *UW Today*. Last modified June 20, 2013. Accessed July 31, 2015. http://www.washington.edu/news/2013/06/20/u-s-supreme-court-decision-opens-genetic-test-options/.

Hunt, Sonia Y. "Controversies in Treatment Approaches: Gene Therapy, IVF, Stem Cells, and Pharmacogenomics." *Nature*. Accessed July 31, 2015. http://www.nature.com/scitable/topicpage/controversies-in-treatment-approaches-gene-therapy-ivf-792.

Klein, Roger D. "Gene Patents and Genetic Testing in the United States."*Nature Biotechnology*. Accessed July 31, 2015. http://www.nature.com/nbt/journal/v25/n9/full/nbt0907-989.html#close.

Lewis, Ricki. "A Brief History of DNA Patents." *DNA Science Blog*. Last modified June 20, 2013. Accessed July 31, 2015. http://blogs.plos.org/dnascience/2013/06/20/a-brief-history-of-dna-patents/.

Marshall, Eliot, and Michael Price. "U.S. Supreme Court Strikes Down Human Gene Patents." *Science Insider*. Last modified June 13, 2013. Accessed July 31, 2015. http://news.sciencemag.org/people-events/2013/06/u.s.-supreme-court-strikes-down-human-gene-patents.

Pearson, Sue, Hepeng Jia, and Keiko Hendachi. "China Approves First Gene Therapy." *Nature Biotechnology*. Accessed July 31, 2015. http://www.nature.com/nbt/journal/v22/n1/full/nbt0104-3.html.

Thompson, Nicholas. "Gene Blues." *Washington Monthly*. Accessed July 31, 2015. http://www.washingtonmonthly.com/features/2001/0104.thompson.html.

"What Is Gene Therapy?" *Genetics Home Reference*. Last modified July 27, 2015. Accessed July 31, 2015. http://ghr.nlm.nih.gov/handbook/therapy/genetherapy.