Gene Therapy

Congratulations! After several months of trying to have a baby, depending on your gender, either you or spouse are finally pregnant. All is looking well until you a standard Prenatal Health Screenings called a Karyotype. A Karyotype is a picture of every chromosome in one cell of your body (Campbell 2010 pg. 281). It appears your child has one extra chromosome, causing him/her to have Trisomy 18. More commonly known as Edwards Syndrome, this disease is a highly deadly genetic defect that almost always kills within a year (Campbell 2010 pg. 297). Now take a chance and ask yourself, what extents would you go to save your child? There currently is a controversial method of medicine being developed which would help parents in this situation. It is most commonly known as gene therapy, which is the practice of altering someone’s genetic code in order to change their physical or mental state. This could be done for reasons ranging from psychological to medical to purely personal. Currently there is no easily accessible type of gene therapy present in the United States, but it will be developed soon. If you were to choice to use this treatment your child would be born completely healthy. Their of course would be the possibility of complications, but this happens with practically any form and medicine and most likely would be less lethal then the example presented. Despite this fact Gene Therapy has meet some opposition because it is not currently well known or understood. People need to know that gene therapy will benefit society and will be safe as long as it is properly regulated.
Gene Therapy is considered “an introduction of genes into an individual for therapeutic reasons” according to a text book called Biology by Campbell (2010 pg. g-16). How this occurs is more clearly explained by an article written by Eric J. Simon called human gen therapy:Genes Without Frontiers? This Article explains that these new genes are transferred into the body through vectors creating Recombinant DNA. Recombinant DNA is a form of hybrid DNA containing some of the original genes it started off and with new preferable genes which is either added in or replaces an unwanted gene (Simon 2002 para. 6). A vector is a DNA carrier that performs this process (Simon 2002 para 11). There are two different ways to use these vectors, in vivo or ex vivo. In vivo means “in the living tissue” which means it would require inserting the vector into the individual (Simon 2002 para 12). Ex Vivo means “outside the living body” so it would involve the removal of the patients cell, altering the cells Genetic Code with a vector, and transplanting them back in(Simon 2002 para 11).  

There several different forms of these vectors. Other forms are described well by Simon in his article I mentioned earlier. The most common form is a virus that has been changed so it will not reproduce or damage the cells. This virus will transplant the desired gene to a cell instead of causing the cell to reproduce more viruses. Some different ways this can be accomplished is through Retroviruses, or Adenoviruses. Liposomes, plasmids and naked DNA can also be used but there not viral vectors. The first two are more efficient then the later but there is less of a risk that someone else will “catch that gene”. Liposomes, Plasmids, and naked DNA are less likely to cause this but are also less likely to work. (Simon 2002 para. 11) These vectors can alter the genome in two different ways. These are via “infecting” the Somatic cells or the Germline cells.
The Germline cells are embryonic cells, which means the gene that is transplanted will be passed on to the next generation. Somatic cells do not get passed on to the next generation because they are not involved in reproduction (Simon 2002 para 12). There is a lot controversy about Germline Gene Therapy and it is currently not legal to perform on humans. First off, one may wonder how changing the genetic code of the human race will affect the population. For example, Sickle cell is a genetic disorder that causes the red blood cell to be misshapen preventing them from carrying enough oxygen through the blood stream (McNeil 2011 para 1). This can be very painful and causes the person to be constantly out of breath. However people who are carriers for sickle cell (only half of their blood is misshapen) can live fairly normal lives, and are immune to Malaria ((McNeil 2011 para 3). One may ask will curing all genetic diseases could cause us to become more vulnerable to unknown threats? The answer to that question is possibly but the human race is much smarter then we give us credit for. An article written by the New York Time can be used to explain this is. Vaccines are currently being developed for malaria. They are being developed by studying Sickle Cell and showed very promising results with their patients (McNeil 2011 para 4). This shows that we have more foresight than those worried about the future of gene therapy think our society have. We are quite capable of foreseeing the future. Like James Watson, Nobel Laureate for the discovery of the structure of DNA and former Director of the Human Genome Project once said

“I just can’t indicate how silly I think it [The Sanctity of the human gene pool] is. I mean, sure, we have great respect for the human species. We like each other. We’d like to be better, and we take pleasure in great achievements by other people. But evolution can be just damn cruel, and to say that we’ve got a perfect genome and there’s some sanctity to, I’d just like to know where that idea comes from. It’s utter silliness. And the other thing,
because no one really has the guts to say it, I mean if we could make better human beings by knowing how to add genes, why shouldn’t we do it? If scientist don’t play god, who will?” (Sadler and Zeilder, 2004, pg 231)

One also may think this “playing God” Watson was talking about will lead to no good. An article called The Slippery Slope Argument of Gene Therapy written by Veikko Launis, clearly demonstrates these fears. It claims if humans were to “play God”, it would lead to Eugenics. It claims that it is not far of a stretch for humans to want to alter themselves to go to wanting to alter each other. This article gave a quote from Bishop which said “If voluntary euthanasia were legalized, there is good reason to believe that at a later date another bill for compulsory euthanasia would be legalized. . .” Launis’s “Slippery Slope Argument is very similar to Bishops argument against voluntary euthanasia (Launis 2010, para 6). But then he explains that this Slippery Slope article is an logical fallacy. He also explains that as long as our society is strongly against Eugenics, we will not practice it. There would have to be some form of social upheaval that changed the views of our populations (Launis 2011, para 22). So as long as we are able to make regulatory mechanisms, they will follow what our societies moral values, and the fact we are so worried about Germline Gene Therapy leading to Eugenics indicates how strongly we are against it.

There is just as much controversy over in utero Gene Therapy because it is a form of Germline Gene Therapy and other fears which will be mentioned soon. Even though it may be the only way to cure some serious genetic diseases, like trisomy 18 which kills the child before they could get the treatment on their own accords, many people are unsure about in utero Gene Therapy. This form of therapy literally means Gene Therapy before birth. Therefore the unborn
child would be exposed to gene therapy before birth (Gededs, and Spink 2004 para 16). This raises controversy for many two main reasons. One is the parents are choosing the fate of their children, and the other is a general lack of understanding about Gene Therapy and social beliefs. For example an article called Identify and Disabilities, helps explains the first fear I mentioned. It asks how do we really know if a child alter before birth will really be the same child? Would an altered child this be a different child, or just a “new and improved version” of the child (Belshaw 2000 para. 6)? This is an ethical decision that is hard to be proven either way. However what can be proven is that it might be the only way to save a child that would die from rare genetic disease. I can also easily be proven the more you knew about gene therapy, and your world view is a large determining factor in what you believe about gene therapy. In 2002 M.D.R. Evans, Jonathan Kelley, and Esmail D. Zanjani did a study on the public opion of gene therarpy, and wrote an article in 2005 about their results called The Ethics of Gene Therapy and Abortion: Public Opinion. This study was done on a sample of 1403 people in Australia ( Evans, Kelley, and Zanjani 2005 pg 223). They ask questions about different situations one might face as upcoming parents, and whether Germline Gene Therapy or Abortion would be acceptable for these scenarios. It gave four possible scenarios and asked whether or not Germline Gene Therapy or abortion would be acceptable. The Scenarios where the child has a serious defect which would within a few years, if the child has a minor defect which could be lived with but would make life difficult, the child will be aggressive, and for cosmetic reasons. Germline Therapy had a 40 percent approval, 23 percent undecided and 36 percent were undecided when asked about a fatal disease. On the other hand abortion had a 72 percent approval rate when asked about a fatal disease. This demonstrates that although people may not want their child to live with a fatal disease, to the point of aborting a pregnancy, there is something making gene therapy seem
unacceptable. Also the lack of a general consensus, there is a lack of knowledge on the topic. For a livable minor defect which would make life more difficult, the child will be aggressive, and for cosmetic reasons abortion the public opinion leaned towards no, especially for the later. This means complete termination of a pregnancy is not optimal to the general population to solve minor genetic diseases and cosmetic reasons. However, the public was split about fifty-fifty with a slight leaning towards no when asked about aggression and a minor defect. Still, just like abortion, the public strongly disapproved of cosmetic reasons (Evans et al. 2005 pg. 226). Therefore, the general public is unsure about Gene Therapy when asked about different scenarios except that doing it for cosmetics reasons are undesirable to the majority of people. This is not surprising when you consider that there are people who believe that something as simple as performing Somatic Gene Therapy should be banned because it will lead to Eugenics. More interestingly this survey helped show that this polarity was caused by a lack of understanding of Gene Therapy and people’s views. For example those who were interesting in genetic (thus a greater understanding) were more likely to approve gene therapy. Those who were non-religious and had a Scientific world view (believed in the big bang, or evolution) were more likely to approve of Gene Therapy according to the survey (Evans et al 2005 pg. 231). This indicates that people are basing their views of gene therapy on a lack of understanding and personal beliefs, instead of how it benefit society and how safe or unsafe it may be.

If Gene therapy is properly regulated, Eugenics, as well as other forms of malpractice will not be a major problem. This is talked about in a article from a scholarly journal called *Gene Therapy Progress and Prospects? Bringing gene therapy in medical practice: the evolution of international ethics and the regulatory environment* written by Genetics Science Policymaker D. Geddes and doctor for the Department of Respiratory Medicine In Royal Brompton Hospital,
London, UK. They explain that we should view Gene therapy just like it was a drug. Therefore if it is properly regulated there is no need to worry about Eugenics or other forms of malpractice. Although gene therapy is bound to have some side effects just like all drugs do. This can be seen in a trial done on X-SCIDS patients. More commonly known as the “bubble boy syndrome”, X-SCIDS causes someone to have a much weakened immune system if any at all. In October and December of 2002, Necker Hospital in Paris, announced two of its patients developed leukemia when trying to use Gene Therapy to cure this disease. And although it was promising, 9 out of 10 of their patients showed long term improvement, Gene Therapy definitely needs to be regulated to avoid harmful side effects such as cancer (Gededs, and Spink 2004 para 5). Also in September of 1999, a volunteer, named Jesse Gelsinger, died from an Adenovirus that was reported to have over 600 serious side effects, including death (Gededs, and Spink 2004 para 6). Although Scary this should be expected because there are around 2.2 Million severe adverse drug reactions in the USA every year (Gededs, and Spink 2004 para 5). Yet these drugs continue to be used because of the many benefits they provide, and they are regulated enough that we do not have to worry too much about their side effects.

Gene Therapy is no exception and has had several jumps in medicine over the years. For example, X-SCIDS, has been researched a lot over the years and has had many breakthrough since the two boys developed Leukemia. One of the most successful one happened in May of 2000 when a French team of scientist were able to cause such a great improvement their two patients could safely leave their home. Also there has been a study done on Familial hypercholesterolema. This Disease causes the affected person’s liver to not properly metabolize lipoprotein cholesterol, leading to abnormal high levels of cholesterol in the blood. This often causes the person to die very young, often by twenty, and sometime even before they reach age
of five (Simon 2002 para 17). Studies have been done model off of this X-SCIDS study, and there has great success. There has been 40 studies done in the United States between the years 1999 to 2002 and only 2 of them had deaths, which is rather low in medicine (Simon 2002 para 22). Since these forms of gene therapy had a lot of successes, limited side effects show that Gene Therapy have great potential in curing diseases and if properly regulated is fairly safe by medical standards.

Therefore, Gene Therapy will come and benefit our society, and will be safe because of our abilities to regulate the medical field. Fears about the future of mankind, Eugenics, or from other personal issues will not come true because we have these fears. Gene therapy will happen because we live in a free society and have a responsible medical field that sets up regulation to avoid malpractice. The fear of malpractice will allow gene therapy to be regulated in a way it will be safe by medical standards. So remember that child your about to have. If your twenty year in the future it could be born normal. Now isn’t that a comforting thought.
References


Evans, M. D. R., Jonathan Kelley and Esmail D. Zanjani, The Ethics Of Gene Therapy and Abortion: Public Opinion. Fetal Diagnosis and Therapy. 20(3)


