State of the Development and Viability of Gene Therapy Ventures

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Abstract After years of being ignored due to flaws in the delivery methods, technology has advanced enough to make gene therapy an emerging biotechnology. Gene therapy is significant in that it enables treatment of many conditions for which treating the underlying cause was previously not possible. Glybera, made by Dutch company UniQure, is the only approved gene therapy treatment in the United States and Europe. UniQure and many other companies are now doing research into gene therapy with intent to commercialize the technology. This paper examines companies such as UniQure in the early stages of development (pre-IPO or recent IPO). Venture capital funding as well as clinical trial status are also studied. In addition, both the context of gene therapy in biotech and the potential pricing of treatments are examined with the ultimate goal of assessing the state of gene therapy as a new business and assessing the viability of these ventures. This report is focused on the business of gene therapy and is not intended as a detailed technical examination of the available technologies.

Keywords Gene Therapy, Commercialization, Venture Capital, Funding, Business, Biotechnology

1. Introduction

Gene therapy is one of the emerging technologies in today’s biotechnology field. To summarize what is meant by gene therapy, it is the use of engineered DNA to treat an underlying condition by delivering it directly to a patient’s cells. Most commonly, the delivery method for the therapy is genetically engineered viruses, either adenoviruses or retroviruses. Additionally, the use of a system called CRISPR/Cas9 has been developed in recent years to use as a delivery system in place of viruses. Additional details on both methods will be provided. While gene therapy can be used to treat a wide variety of conditions, its greatest use is the treatment of congenital genetic diseases. In fact, in many circumstances, gene therapy is the only way to treat the direct cause of these conditions (the genetic defect.) One of the major challenges of the field is that many of the diseases that gene therapy is used to treat are very rare, or “orphan,” diseases. Nevertheless, there is still a need for treatments and some biotech ventures have taken advantage of this need. Research into gene therapy began in the late 1980s to 1990s, and showed great promise at first, but eventually a number of incidents caused research to be greatly reduced for some time. Specifically, it was found that the therapy’s delivery methods could cause the development of cancers and trigger severe immune responses. In one case, a patient died due to the treatment. In recent years, advances in the delivery technologies have enabled gene therapy to return, and a number of companies and universities have begun research and commercialization, and the first actual treatment was approved in 2012[1]. It is notable that there do exist commercial gene therapies in China for cancer, but these largely lie outside the scope of this report due to the fact that there has been no attempt to get approval outside of China as well as due to concerns raised regarding a lax approval process and lack of data (simply put, the clinical trials performed were not up to the standard set in the United States and Europe) [2]. In addition, biotech companies have formed based on the promises of gene therapy as a new treatment, and many have attracted venture capital. Gene therapy is emerging as a new area of biotech for entrepreneurs to exploit and commercialize. The goal of this report is to survey the biotechnology field and identify the key gene therapy ventures in the industry, then identify the challenges involved in a new venture and the best ways to overcome them. Ultimately, the viability of gene therapy as a subject of biotechnology entrepreneurship will be evaluated.

At this stage it is useful to go into some detail regarding the actual mechanisms of the two methods of gene therapy discussed above. The more traditional technique utilizes a viral vector, most commonly an “adeno-associated virus” (AAV). This virus type is useful for two reasons. First, it is capable of expressing its genes in a cell’s nucleus for an extended period of time; in some cases the gene expression
has been found to last for the life of the infected organism. Second, AAV is not pathogenic, so it is far safer to use than most other virus types. In the gene therapy process, the AAV genome is modified to contain DNA that is to be delivered to cells as well as reduce any possibility of virulence. Then, the viruses are introduced to the patient through a series of treatments [3]. Other virus types are occasionally used for gene therapy but the general procedure is similar to that for AAV. The second technique mentioned above is CRISPR/Cas9. This technique utilizes a two-part system: an RNA guide that targets a specific location on the genome and an endonuclease called Cas9 that can cut out DNA and replace it with engineered DNA [4]. Right now, most gene therapy ventures are using the viral technique as the CRISPR/Cas-9 technique is still very new.

2. Methods

In the course of the research for this paper, numerous databases were scanned for information regarding gene therapy in business. From there, companies to study were identified. As the interest of the paper is entrepreneurship large biotech companies that have existed like a long time and focus primarily on technologies other than gene therapy (i.e. Genentech) were not closely considered unless they were involved in the funding of a new venture or in a licensing agreement involving a new venture. The definition of a new venture for the subject of this paper is any company primarily backed by venture capital or partnerships with existing companies. Because of the tendency of the biotechnology industry to have very long product development cycles, many of the companies studied have actually existed for a long time but have only raised large amounts of funding or gone public recently. The paper looks at these companies’ funding sources, the types of technology being developed, and the progress on getting the technology to the market. Additionally, research was performed on the gene therapy field as a whole through a review of business news and journals.

To make the decision on the viability of gene therapy as a business venture, a variety of methods were employed. First, data from many gene therapy companies was gathered and a comparison was made to UniQure, the currently most successful of these companies as well as average IPO funding for other types of biotech. In addition, non-gene therapy treatments for specific conditions studied by the companies in this paper were examined and compared to the gene therapy treatment.

3. Literature Review

3.1. The Gene Therapy and Biotech Markets

A study of current literature shows that gene therapy is a popular subject for scientific research. Biologists and engineers are researching the applications of gene therapy to treat previously untreatable diseases. However, there is understandably very little literature related to gene therapy as a business. Such research is significant because without a market for gene therapy, treatments will remain stuck in the laboratory unable to reach all but the smallest number of patients through clinical trials. Furthermore, if there is shown to be little economic opportunity, governments and venture capitalists will hesitate to fund researchers and firms looking to work on gene therapy and research will cease as it did in the 1990s.

In addition to studying individual cases of gene therapy companies, it is important to consider the health of biotech entrepreneurship as a whole as well as the specific circumstances concerning the commercialization of gene therapy treatments. A study by Huggett showed that the past ten years have been very good for new biotech firms, but since 2012 there have been fewer rounds of funding for these new firms. Additionally, there are more and more startups forming as technology from university research is being turned into business opportunities. This decrease in funding has been attributed to fewer interested investors, risk aversion in biotech, and fewer qualified management teams. Additionally, many venture capitalists have become frustrated with the constantly changing requirements of regulatory bodies such as the FDA. The shortage is worse in Europe than in the US [5]. Nevertheless, there is still money out there, and in gene therapy’s case, there has been a surprising amount of funding in the US despite being down worldwide [6].

Examining overall funding for gene therapy in more detail, as of September 2014, venture capitalists have spent $715.8 million on gene therapy since 2010 and $653.6 million between 2000 and 2010. These numbers show a major increase in funding in recent years. Also, most funding is in the area of therapies in the brain and nervous system, eyes, and blood [7].

The fact that only one gene therapy has been commercialized so far is a concern for the field, but as most research is very recent, this may be due to the fact that most treatments are still in clinical trials. The way treatments are commercialized in terms of sales and marketing should be a concern to companies once treatments are approved. One potential problem with gene therapy is that it is often a one-time treatment, so the price for the treatment would have to be one very high single payment. Such a payment would make the treatment undesirable to insurers, especially in the case that there are existing, cheaper treatments on the market. In an article by Dr. James Wilson, he suggests an alternate payment structure that would involve annual payments of smaller amounts, on the condition that the therapy continues to work. Such a scheme would lower costs and make the treatment an easier sell to insurers and patients for companies developing the treatment [8].

4. Results
4.1. Case Studies

Presently, the most successful gene therapy venture, and the only one that has actually begun the commercialization process, is UniQure. UniQure is a Dutch company focused on gene therapy whose main product is Glybera, a therapy that is used as a treatment for lipoprotein lipase deficiency (LPLD, a condition that prevents the body from processing fat after a meal.) This AAV based treatment is only approved in Europe, but according to UniQure, Glybera is in Phase III clinical trials in the US and is expected to be approved within a few years. The company is also working on treatments for hemophilia and congestive heart failure, neither of which is currently past Phase I trials [9]. As a biotech venture, developing a new treatment is expensive, so UniQure did receive a significant amount of funding. After forming from the collapsed Amsterdam Molecular Therapeutics in 2012, UniQure received 44.5 million euros from Coller Capital, Glide Healthcare, Forbion, and Chisesi Farmaceutici. The partnership with Chisesi is important because that company is directly involved in the commercialization and marketing of the drug. In February 2014, UniQure raised $91.5 million in its IPO. Interestingly it is listed in the US on NASDAQ, which contributes to the trend of recent European biotech companies seeking out the US market [10]. Also relevant to the US market, UniQure is building a large production plant in Massachusetts that is designed for the manufacture of virus-based gene treatments. They expect to begin production in 2015 [11].

Commercialization of Glybera is an important indicator of the potential for gene therapy. UniQure has priced Glybera at approximately $1.35 million for a full course of treatment in Germany with a potential of 150 to 200 patients in Europe [12]. Although UniQure is the only company with a gene therapy product currently on the market, there are many other products in development, a large number of which are being developed by venture capital-backed enterprises. One such enterprise is Xenon Pharmaceuticals, a biotech company based out of Canada. Xenon has a two-part strategy. According to their website, Xenon is developing treatments for orphan diseases which they intend to commercialize on their own as well as treatments for more common problems with which they intend to collaborate with larger pharmaceutical companies [13]. The latter approach is the one the company is committed to most strongly right now. Xenon has licensing agreements with the major biotech companies Genentech, Teva, and Merck. The collaboration with Teva is for an osteoarthritis treatment and is the furthest along in the development process (currently in Phase II clinical trials.) Xenon also has a stake in the previously mentioned Glybera treatment by UniQure and the company is expected to receive a small royalty on that product. Xenon is one of the older biotech companies examined in this report, and to date has raised $265 in funding [14]. Xenon went public November 2014, raising $36 million in its IPO [15].

Another company that has seen success in recent years is Bluebird Bio, based out of Cambridge, Massachusetts. Bluebird studies treatments in the areas of ALD (adrenoleukodystrophy, a degenerative nervous system condition), blood diseases such as beta thalassemia (or “β-thal”), a condition in which patients do not produce properly functioning hemoglobin and must receive regular blood transfusions), sickle cell, and cancer. Their primary therapy is known as LentiGlobulin, and works by replacing defective genes with working ones in the patient’s hematopoietic (blood-producing) stem cells. This therapy is also the basis of the ALD and blood disease treatments. The ALD treatment, Lenti-D, is in a Phase II/III state, and is the furthest along in development [16]. The company received some very positive and well-publicized results in their β-thal treatment in which the treated patients gained the ability to produce functioning hemoglobin for months after treatment. Not long after this breakthrough, in June 2013, Bluebird launched their IPO [17] which raised $116 million. Aside from their IPO, since 2010, Bluebird has raised $134 million from research institutions, grants, and most prominently venture capital firms. Third Rock Ventures (a biotech venture capital firm in Boston) is the most prominent investor, currently owning 28.9 percent of the company [18, 19].

It is important to consider companies at many stages of the entrepreneurship process. The previously discussed companies are all either public or very close to becoming public. In contrast to those companies, Voyager Therapeutics is a true startup. It was started by Third Rock Ventures in February 2014 with a $45 million investment. Third Rock saw an opportunity in the lack of available treatments for central nervous system diseases such as ALS and decided to bet big on gene-based treatments. Voyager plans to take advantage of advances in Adeno-Associated Viruses (AAVs) to create new treatments [20]. Voyager is an interesting case due to its founding team. Rather than being the vision of a single entrepreneur starting from scratch, Voyager is the work of a group of highly experienced researchers with years of prior research in the field working together to solve a specific set of problems, in their case central nervous system disorders. Most of the key researchers bring with them specialized knowledge of AAVs. The company is managed by Mark Levin, one of the co-founders of Third Rock Ventures [21]. Voyager’s founders also bring with them some preclinical and clinical work, including a Stage 1B trial for Parkinson’s and preclinical work on ALS and Friedrich’s ataxia [22].

The four companies listed above are good examples of newer companies focused on gene therapy. However, it is important to consider as many companies as possible in this analysis. Spark Therapeutics is studying therapies for rare eye diseases among others and raised $72.8 in May 2014. Dimension Therapeutics raised $30 million in June 2014 and is studying hemophilia. Audentes Therapeutics raised $30 million and is researching a rare muscle condition called X-linked myotubular myopathy [23]. One more company, Sangamo Biosciences, is focusing their research on HIV, and
In addition to the companies working on more traditional techniques, in the last two years companies developing CRISPR/Cas9 based gene therapy have emerged. There are three such companies: Editas Medicine, Intellia, and CRISPR Therapeutics. Editas Medicine is the oldest and most established of the three companies. Co-founded in 2013 by Harvard scientist George Church, Editas is developing the CRISPR/Cas9 technologies as well as the related TALENs technology. At the present time, Editas is not focused on a specific disease but is instead trying to get the technology into a viable state. Editas has raised $43 million in venture capital. Like the other Massachusetts startups discussed above, Third Rock Ventures has put a significant amount of money into the company and plays a role in its direction [25]. Intellia Therapeutics is also based in Massachusetts and is using a suite of tools developed by Berkeley startup Caribou Biosciences. It was started by Atlas Venture in 2014 with with a $15 million round of capital. Intellia has not settled on a set of diseases to treat but has stated they would like to focus on blood diseases and blood cancers [26]. CRISPR Therapeutics is based in Switzerland and has raised $25 million in funding. Like the other companies in the field, no disease focus has yet been determined [27].

4.2. Cost of Treatments

Many gene therapies are treatments for conditions with no known cure and very limited treatment options. In the case of these therapies there is no real way to gauge the potential market aside from studies of the number of affected people. Other therapies are for conditions for which there are treatments available (but no permanent cure). It could be said that these temporary treatments represent the cost to the health care system of these conditions. For example, in the case of hemophilia, there is no permanent treatment. However, there are prophylactic clotting factor treatments that must be administered on a per week basis. The cost of these treatments is estimated to be $300,000 per year [28]. A gene therapy for hemophilia would cost more up front, but would eliminate the need for ongoing treatment. For osteoarthritis, a 2007 study found that osteoarthritis accounted for an average increase of about $6,000 per year per person with the condition in health care costs (including those paid by insurers) in the United States [29]. One more condition to study for which gene therapy is in development is sickle cell. A study found that the average lifetime cost of treatment is $460,151 [30]. ALS is an example of a condition that is less manageable than the others listed above. A study by MDA found that the average annual cost of illness per patient is $63,692 [31].

5. Discussion

Based on the presented data and the success of the companies listed gene therapy is a viable venture under certain circumstances. Of course, based on the case studies, the most important part of a gene therapy venture (or any venture) is having a potentially viable product. While UniQure is the only truly proven company, most of the other companies examined have treatments in Phase I or later. Interestingly, the amount of funding a company receives is not necessarily related to the state of clinical trial. Xenon Pharmaceuticals is the most funded company of those studied, and the best explanation for this fact is that it has been around a long time and has multiple corporate partnerships. In other cases, a strong clinical trial program is good for funding, as is the case with Spark and Bluebird Bio, both having reached Stage III. Voyager Therapeutics presents a sort of ideal starting point for a gene therapy company, with an ample amount of funding and an experienced management team built in. The fact that their researchers brought with them clinical trial data from university research is also very important. Using UniQure as a comparison, UniQure formed from an existing company that held trial data, so that data definitely gives a company an advantage.

The next part of assessing a gene therapy company is a look at the actual treatments. The following table shows a list of the conditions under research by the eight gene therapy companies studied:
Most of the treatments in this table were discussed in the case studies above. Many of them are in preclinical or Stage I trials. The most interesting thing to take away from this data is that blood diseases (sickle cell, β-thal, hemophilia) are a very popular area of research for early-stage companies. These diseases are all genetic, as are the majority of the conditions being studied. After all, gene therapy is the only way to treat genetic conditions on a permanent basis. More importantly, all the conditions being treated (with the exception of cancer) are not truly curable. The lack of a permanent treatment for these conditions represents the pain in the market for gene therapy. Therefore, it makes sense that a gene therapy venture should study such a condition.

To look at the health of gene therapy startups relative to other biotech, it is worth considering IPO funding, as that is a very available metric that shows the market for a company. The results of the case studies show that four of the eight companies studied made their IPOs in the past two years. The table below summarizes this data along with the average IPO for a biotech companies in 2013.

### Table 1. List of treatments in development by gene therapy ventures.

| Condition          | Companies Researching | Condition Type |
|--------------------|-----------------------|----------------|
| LPLD               | 1                     | Genetic        |
| Congestive Heart Failure | 1                  | Non-genetic    |
| Osteoarthritis     | 1                     | Non-genetic    |
| Postherpetic Neuralgia | 1                  | Virus-caused   |
| Erythromelagia     | 1                     | Genetic        |
| ALS                | 1                     | Varies         |
| Friedrich’s Ataxia | 1                     | Genetic        |
| ALD                | 1                     | Genetic        |
| Cancer             | 1                     | Varies         |
| HIV                | 1                     | Virus-caused   |
| Alzheimer’s        | 1                     | Non-genetic    |
| Parkinson’s        | 2                     | Non-genetic    |
| Huntington’s       | 2                     | Genetic        |
| Sickle Cell        | 2                     | Genetic        |
| Beta-thalassemia   | 2                     | Genetic        |
| Hemophilia         | 3                     | Genetic        |

The gene therapy companies are performing about average, though it is notable that both Bluebird bio and UniQure have both raised above the average and have treatments in Phase III clinical trials or later, indicating that the market favors companies with products near commercialization.

For studying commercialization viability, UniQure remains the only example. The previously stated $1.3 million price tag is so high primarily because of the rarity of the condition Glybera treats. The fact that the company is willing to price it so highly indicates a willingness of insurers to pay that price, so the price of future therapies might be dependent on the availability of patients. The data on non-gene therapy treatments listed in the results is given to provide an idea regarding the cost of current treatments. Additionally, since one of the minor goals of this research is to provide a picture of the current state of gene therapy to insurance providers who would be paying for the treatments in most cases, a discussion of the cost of treatments and non-treatment is part of that picture. The costs of these treatments are in a sense are very similar to cost of non-treatment since they are the cost of the temporary treatments. True, permanent treatments are not available, a problem gene therapy aims to fix. Looking at the numbers, just assuming for a moment that any given gene therapy treatment would be a one-time $1.3 million payment, paying $1.3 million once is preferable in the case of hemophilia but not sickle cell or osteoarthritis. However, those two conditions are far more common than LPLD, so the treatments should be less. There should not be an issue with business viability from a cost perspective, but more cases are necessary to come to a real conclusion.

Lastly, at this point in time it is far too early to make any significant conclusions regarding the novel CRISPR/Cas9 gene therapies or the companies researching them. The technology is promising and the startups developing it have attracted venture capital but they are years away from any sort of commercialization. Opportunity is available for startups looking to work with this technology, as the IP deal which grants Editas the only current patent on CRISPR/Cas9 in the US, allows licensing of technology to third parties with ideas regarding diseases Editas has chosen not to pursue. This potential for innovation stems from the fact that the number of conditions that can be potentially treated with this technology is too large for one company to handle by itself [33].

### Table 2. Gene therapy IPOs. Data for average from FierceBiotech [32].

| Company             | IPO (millions $) |
|---------------------|------------------|
| Bluebird bio        | 116              |
| UniQure             | 91.5             |
| Xenon Pharmaceuticals| 36               |
| Biotechnology Average | 73.6            |

6. Conclusions

This report makes three conclusions. First, gene therapy as a part of the biotech industry is healthy. Beyond UniQure, there look to be multiple companies commercializing products in the coming years. Second, gene therapy looks to be a viable form of business for those with experience in the field. A successful gene therapy startup will be working on a treatment for a condition for which there is no permanent option, and will bring in some sort of prior research through its founding team. Lastly, there needs to be further research into pricing. The single up-front payment might not be ideal, and for treatments for conditions more common than LPLD, there may be a formula for an ideal price.
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