[**The Resurgence of Gene Therapy**](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/)

Posted on [January 15, 2013](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/) by [admin](http://www.liftstream.com/blog/author/admin/)

[Share on linkedin](http://www.addthis.com/bookmark.php?v=300&winname=addthis&pub=LiftStream&source=tbx-300,wpp-2.5.1&lng=en&s=linkedin&url=http%3A%2F%2Fwww.liftstream.com%2Fblog%2Fthe-resurgence-of-gene-therapy%2F&title=The%20Resurgence%20of%20Gene%20Therapy&ate=AT-LiftStream/-/-/50f8f6465c730e8e/2/50f8f647bd1db163&frommenu=1&uid=50f8f647bd1db163&ufbl=1&ct=1&pre=http%3A%2F%2Fwww.linkedin.com%2FgroupAnswers%3FviewQuestionAndAnswers%3D%26trk%3Deml-anet_dig-b_nd-pst_ttle-cn%26ut%3D0rb-WfT56huRA1%26gid%3D2141807%26discussionID%3D205406601&tt=0&captcha_provider=nucaptcha)[Share on facebookShare on twitter](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/?goback=%2Egde_2141807_member_205406601)[Share on email](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/?goback=%2Egde_2141807_member_205406601)[More Sharing Services](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/?goback=%2Egde_2141807_member_205406601)

Authored by [James Sheppard](http://www.liftstream.com/blog/the-resurgence-of-gene-therapy/uk.linkedin.com/pub/james-sheppard/25/844/604/)

Gene therapy has a chequered past and is still capable of drawing strong opinions both for and against its use. The recently European approval of Glybera (UniQure) for the treatment of lipoprotein lipase deficiency (LPLD) has been seen by many as a turning point for the technology. [Liftstream](http://www.liftstream.com/) takes a look here at the recent past of gene therapy and what the future has in store.

Gene therapy is the insertion of genes into an individual’s cells and tissues to treat a disease, such as a hereditary disease in which a deleterious mutant gene is replaced with a functional one. Gene therapy was first conceptualised in 1972 but the first clinical trial in humans wasn’t conducted until 1990. The very first trial using gene therapy was conducted in the USA when Ashanti DeSilva was treated for ADA-SCID. Since then a number of gene therapy trials have been conducted for an increasing variety of diseases. Gene therapy has often found a home in rare disease research. This is partly due to the fact that many rare diseases are the result of genetic mutations, cystic fibrosis is one such example. It is also very difficult to scale up production of gene therapy into a large scale commercially viable operation because at present there is a lack of manufacturing capability and capacity.

The success of Ashanti De Silva was the catalyst for more clinical and pre-clinical activities. The field of gene therapy was progressing, but not at the speed the public demanded. Clinical trials were regularly failing and in many cases not even getting started. In 1999, a single event was to leave an everlasting mark on the field of gene therapy. A man by the name of [Jesse Gelsinger](http://www.nytimes.com/1999/11/28/magazine/the-biotech-death-of-jesse-gelsinger.html?pagewanted=all&src=pm)died whilst enrolled in a gene therapy trial. Jesse was the first person to be publically identified as having died in a clinical trial for gene therapy. Jesse was being treated for ornithine transcarbamylase deficiency in a clinical trial at the University of Pennsylvania. On September 17th 1999, Jesse died from a massive immune response and multiple organ failure. Jesse’s death radically altered the gene therapy field. It would later come to light that Jesse should not have even been participating in the trial due to high ammonia levels that should have excluded him from the trial.

Following his death, many companies, investors, researchers and also the public began to turn their backs on the field of gene therapy.  Regulators became wary about backing clinical trials and many investors were not prepared to take the risk. This led to many projects being scrapped before they even reached clinical testing. However, there were research groups who decided to persevere with the technology. The [University of California](http://www.newscientist.com/article/dn3520-undercover-genes-slip-into-the-brain.html) were experimenting with gene therapy to treat Parkinson’s disease and conducted a phase I trial in 2008 with 5 volunteers and The Moorfield Eye Hospital, London (partnered with University College London) were pioneering gene therapy to treat Lebers Congential Amaurosis (LCA) and in 2007 successfully treated their first trial patient.

Coverage of the LCA trial once again sparked the public’s interest in gene therapy. The success of the Moorfield trial was just one in a series of events which began the resurgence of gene therapy. Gene therapies’ comeback was arguably completed in 2012 with the regulatory approval of UniQure’s Glybera. Glybera is the first gene therapy approved for sale in the western world. This caps a remarkable turnaround from years of false starts and high profile problems.

The resurgence in gene therapy has been greatly aided by a recent boom in investment. This is partially due to a number of positive clinical trial results being published, which lead to a renewed hope in the field. Glybera’s marketing authorisation approval of course does not ensure commercial success, especially in such a challenging pricing environment. Yet, the promise of commercial opportunity will stimulate investors to watching closely the commercial launch of Glybera and how well payers will tolerate a drug potentially priced at $1m. UniQure are not the only company to benefit from this renewed interest. Bluebird Bio, the US based biotech, is currently conducting clinical studies for gene therapy in childhood cerebral ALD which has recently moved towards phase III clinical studies. Gene therapy is no longer only found in small biotechs and university research groups. Global giant GlaxoSmithKline (GSK) in 2010 signed a deal to commercialise gene therapy for ADA-SCID, the same disease for which Ashanti DeSilva was treated. Although this is a great step forward, it also signals how little the field has developed commercially in the past 20 years. This is not to say that advances in academia have not been achieved. Researchers at the Cedars-Sinai Heart Institute in Los Angeles have created biological pacemaker cells in guinea pigs by using gene therapy. This success prompted hope for patients who are not currently eligible for electronic pacemakers.

Other big pharma companies are also interested in gene therapy with Novartis having licensed in treatments from GenVec and shortly following this, Pfizer announced they would licence Tacere Therapeutics’ hepatitis C treatment. Genzyme (now  Sanofi) have also invested well over $200m in gene therapy research over the past 10 years. This is a considerable improvement from where the market was only 10 years ago. However gene therapy is unlikely to produce large scale revenues for big pharma for many years to come.

The gene therapy market has definitely seen a revival of late, led by some truly innovative and persistent companies.  Although the market still has some way to go, advances are being made. Glybera represents a talismanic product for the industry and moves someway to repairing gene therapy’s damaged reputation. The public will still be wary of the technology and in the coming year’s education of healthcare providers and patients will be crucial. The technology will also have to over-come other hurdles including long term safety and pricing & reimbursement challenges highlighted by touted $1m a year cost for Glybera. Ultimately the goal for drugs makers is to move gene therapy beyond advances against rare diseases to mainstream illnesses such as Alzheimer’s and Cancer which affect millions of people globally. This is a goal which sits on a very distant horizon. However, with UniQure’s Glybera signally potential, the future for gene therapy once again looks very promising.