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Physician Views: How Comfortable are Doctors with the Commercial Use of Gene Therapy?

https://marketpublishers.com/r/P6A735721DFEN.html
Date: March 2015
Pages: 0
Price: US$ 695.00 (Single User License)
ID: P6A735721DFEN

Abstracts

Gene therapy has been around for decades, yet in a sense the concept has recently been made to feel new again thanks to some notable breakthroughs and milestones, not least of which was the European marketing approval for uniQure and Chiesi's Glybera (alipogene tiparvovec) issued in late 2012 – the first-ever for a gene therapy in the Western world.

In the early days, gene therapy engendered high hopes as the thought of replacing non-functional genes with working versions sounded like an elegant, straight forward means for treating all manner of disease. In practice, however, the scientific complexities of designing and delivering the agents turned out to be far more difficult than initially envisioned, while serious safety questions slowed progress following events like the death of Jesse Gelsinger, who died in 1999 just weeks after receiving an experimental gene therapy in a clinical trial being conducted by the University of Pennsylvania.

The field has come a long way in the intervening years thanks to advances in the basic understanding of the downstream impact of replacing or modifying a gene sequence, along with significant improvements in the vectors used to safely and consistently deliver gene therapies to the target tissues. What's more, clinicians working on product candidates have improved the design and conduct of studies in an effort to avoid incidents like the Gelsinger tragedy that cast a pall over the entire industry.

As for notable milestones, the first commercially approved gene therapy was developed by Shenzhen SiBiono GeneTech, which received the go-ahead from China's health authority in 2003 to market Gendicine for use in treating head and neck cancer. A different product from Intrexon called Advexin that was engineered to express the same p53 gene was subsequently rejected by the FDA.

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The biggest watershed moment for the field is undoubtedly the approval of Glybera, which is now commercially available in Europe for a rare inherited disorder called lipoprotein lipase deficiency (LPLD), while more recently companies like bluebird bio and Spark Therapeutics generated impressive clinical data with programmes of their own. These and other events have generated useful momentum for the entire field as additional gene therapy products continue to make their way into and through the clinic. The success has in turn generated renewed interest from both private and public equity investors as well as pharmaceutical companies, such as Bayer and Sanofi, which have willingly provided much-needed resources to companies working in the field in the form of financing and partnerships, helping to drive what could turn out to be a virtuous circle of success.

But what impact has the success of various gene therapies in clinical studies had on the sentiment among doctors, and how widespread has its acceptance as a therapeutic modality become in the decade and half since critical safety issues first emerged? To gain better understanding about the views of doctors on the potential of gene therapy, FirstWord PLUS is polling US- and EU5-based endocrinologists, haematologists, neurologists and ophthalmologists, asking them…

> Gene therapy has not revolutionised the field of medicine the way some predicted it might decades ago but the science has, after many fits and starts, gained some long-anticipated momentum recently thanks to several exciting clinical readouts and the EU commercial launch of Glybera from uniQure and Chiesi – the first-ever for a gene therapy in the US or Europe. In general, how would you describe your sentiment towards gene therapy?
>
> What are your thoughts about the long-term safety and how might these affect your prescribing habits once a gene therapy?
>
> What is the biggest challenge facing widespread adoption of a gene therapy?
>
> Glybera, a gene therapy for lipoprotein lipase deficiency, was recently launched in Germany at a price of more than 1 million euros ($1.1 million). How concerned are you that gene therapies like this may prove cost prohibitive?
>
> Now that a gene therapy is approved in Europe and several heading towards marketing submissions in the next couple years, do you believe the field has reached a tipping point where clinical and regulatory success stories will begin

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