

# Physician Views: Are neurologists ready to embrace gene therapy?

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## Abstracts

As recently noted by Forbes, the biotech bull market of 2013 has helped to revive an approach to drug development that had become largely abandoned and pursued by only a few devoted believers. Between the beginning of last year and early April 2014, however, 11 different companies specialising in gene therapy raised \$618 million from venture capitalists and the public markets.

Momentum for this approach has been driven not only by a more palatable financing environment, but the relatively recent approval of the world's first gene therapy product (uniQure's Glybera for the treatment of lipoprotein lipase deficiency) and the steady accumulation of clinical data that have helped to shape more robust sentiment around the commercial opportunity of these products.

Parkinson's disease – an area bereft of significant clinical breakthroughs in recent years – has emerged as a focal point for a handful of gene therapy developers such as Oxford BioMedica and Voyager Therapeutics. The latter is hoping to replace the use of current therapies, which raise dopamine levels in Parkinson's disease patients, by developing a gene therapy that will trigger the patient's brain to produce more dopamine.

With this in mind, FirstWord is polling US and EU-5 based neurologists to ascertain how practising physicians currently view and evaluate the gene therapy development space. Specifically we are asking them...

How familiar they are with gene therapy

What they perceive to be the key benefits of gene therapy?

What are their specific concerns with gene therapy?

What is the highest priority issue that needs resolving over the next 12 months?

Which stakeholder group they expect to play the most important role in driving usage of gene therapy?

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