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Oncology leads development in promising gene therapy pipeline

07 October 2015 | Features | By BioSpectrum Bureau

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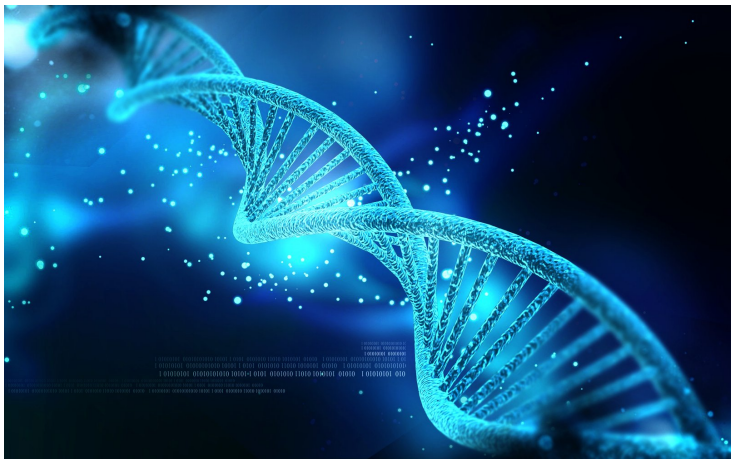

However, the majority of these remain in early steps of development, with 76% at either the discovery or preclinical stage, according to business intelligence provider GBI Research.

The company's latest report states that oncology, infectious diseases, genetic disorders, cardiovascular diseases and ophthalmological indications are the most active pipeline sectors, as well as the most widely studied in terms of the number of clinical trials.

There are 266 pipeline gene therapies in active development for oncology, more than double that of central nervous system disorders, which is the second-largest therapy area.

Furthermore, oncology also accounted for 64% of gene therapy clinical trials between 1989 and 2012.

Mr Dominic Trewartha, managing analyst, GBI Research, says: "Oncology is the predominant area for gene therapy developments due to its high prevalence and genetically driven pathophysiology.

"One reason for the large overall pipeline is the potential for these therapies to develop strong drugs by targeting diseases on a genetic level. Although no products have yet fulfilled this promise, developers expect this in the future."

Based on pipeline activity, Isis Pharmaceuticals is a major player in the gene therapy research and development space, with 31 gene silencing-based programs in development across the key therapy areas.

Sarepta and Alnylam Pharmaceuticals are also key players, with 25 and 24 pipeline products in development, respectively.

Mr Trewartha explains: "In the next decade, these late-stage pipeline developments may translate into clinically and commercially successful gene therapies entering the market. However, high pipeline failure rates due to challenges in

developing safe and efficient delivery vectors will remain a barrier.

"Other potential obstacles to gene therapy development include difficulties in manufacturing and purifying viral vectors, as well as increased regulatory oversight by the US Food and Drug Administration and general caution from worldwide regulatory bodies following previous trial deaths," concludes the analyst.