

## Gene therapy pipeline in oncology is rich but at early stage'

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**Singapore:** Despite few products having so far reached the market and captured strong revenues, the gene therapy pipeline remains large, with 906 products in active development across all stages, highlights analysis firm GBI Research.

However, the majority of these remain in early steps of development, with 76 percent at either the discovery or preclinical stage.

According to the analysis firm, 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 percent of gene therapy clinical trials between 1989 and 2012.

Mr Dominic Trewartha, managing analyst, GBI Research, said, "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."

GBI Research's report also states that, 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 explained, "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.