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# The impact of gene therapy

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*...hort stint of post-doctoral work at NCL, Dr Ganesh Sambasivam joined Syngene (Biocon Group ...t its inception. There he rose to be VP and CSO in a span of 12 years and built a good cohesive ...ts with diverse chemistry skills. In August 2006, he moved-on and founded Anthem Biosciences, ...lleagues from Syngene.*

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**Mr Ganesh Sambasivam,** Founder & CSO, Anthem Biosciences

Worldwide experts are of the opinion that gene therapy's current status is similar to that of monoclonal antibodies 15 years ago and that the net market worth is estimated to be over $20 billion. The world's first commercialized gene therapy drug is Gendicine made by China's Shenzhen SiBiono Gene Tech. Gendicine is now being reviewed by the Drugs Controller General of India (DCGI) to decide on allowing clinical trials in India. Known as Recombinant Ad-p53 Anti-cancer injection this drug is used to treat head and neck squamous cell carcinoma. Data from Indian clinical trials could help significantly in obtaining regulatory approvals in other developing and developed nations. The only other commercialized gene therapy product is Rexin G from Epeius Biotechnologies in California, currently approved for use only in the Philippines for treatment of metastatic breast cancer, pancreatic cancer, osteosarcoma and soft tissue sarcoma. HGF DNA plasmid, a drug from Sosei and Daiichi Sankyo investigated for treatment of peripheral vascular disease was filed for approval in Japan. Success in discrete medical trials has also been witnessed in treating conditions like myelination disorder X-linked ALD (adenoleukodystrophy), ADA-SCID (adenosine deaminase deficiency-related Severe Combined Immunodeficiency) and Leber congenital amaurosis).

Although gene therapy failed to live up to its promise in early 1990s, it was generally accepted that the discouraging outcome was not due to an adequate insights into the genomics involved rather due to the lack of an optimal gene delivery system.

Success in gene therapy has now been acknowledged as an outcome determined by optimal gene delivery systems. Retroviral vectors have long been used in gene therapy clinical trials. However, this system suffers from serious setbacks such as weak titers and inefficient encapsidation of the therapeutic gene, thus decreasing their therapeutic value. Unlike retroviruses, lentivectors do not necessarily require cell division for proviral integration. Lentivectors couple the advantage of facilitating a highly efficient and stable transgene expression to its ability to transduce a variety of cell types that are not amenable to transduction by other vectors. Anthem Biosciences, Bangalore, a prominent CRO, has widely used the Lentivector system to develop stable engineered cells lines for protein expression as well as for gene knockdown. Possibly, the first Indian company to use this system for engineering mammalian cells, Anthem has validated several engineered cell lines that serves as the launching pad for the various cell-based platforms intended to facilitate high-throughput screening of chemical entities targeting specific disease conditions. This has provided a shot in the arm for Anthem's gene therapy program currently in the pipeline.

Although several clinical trials are currently on in several parts of the world, gene therapy still appears to be in the infancy stage in India. Studies are however being conducted for oral cancer at the Tata Memorial Hospital, Mumbai. Narayana Nethralaya, a speciality eye hospital in Bangalore is in the process of using gene therapy to treat Inherited retinal disorders in children. Although a recent Financial Express report indicates that Intas Pharma will launch Gendicine in India, there appear to be no reports of any company in India that has taken the initiative to invest in and explore the potential of this remarkable technology in improving the quality of health care in India. In this regard, Anthem Biosciences aims to exploit its significant expertise in genetic engineering to design and implement gene therapy strategies that could potentially prove to be a boon to the millions that suffer from genetic disorders.