

Editorial

## Genetic therapy: on the brink of a new future

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The field of genetic therapeutics, the focus of our new journal, *Genetic Vaccines and Therapy*, is poised to enter a potentially vast new arena of medical progress within this decade. Worldwide interest exists among biopharmaceutical companies, governments, the military and medical practitioners in the rapid development and deployment of novel DNA-based agents. The possibilities are virtually unlimited: new treatments for emerging and re-emerging diseases; effective and specific immunization against the viral scourges of HIV, smallpox and others; the genetic correction of hereditary defects, such as cystic fibrosis; more effective and specific antitumour therapies genetically tailored to the individual; and gene-specific preventive measures for degenerative diseases such as Alzheimer's, arthritis, and arteriosclerosis [1].

Much good and useful work has been done in the field of genetic medicine, but much remains to be perfected. Gene-based medicine has played a role and will continue to be of critical importance in creating vaccines and anti-viral therapies for HIV, hepatitis, herpes and other viruses, in new strategies for the prevention and treatment of emerging diseases, and for a secure and certain biodefense plan against microbial attack. For example, cancer patients may look forward to a swift and specific treatment regimen based on detailed genetic analysis of their tumour type and individual genetic makeup that can be used to design DNA-based antitumour agents with minimal side effects [2].

The burgeoning wealth of new information generated by genome sequencing and gene chip analysis, combined with the enhanced proteomics databases available today opens up huge possibilities for designing new therapeutic

agents. The use of DNA to synthesize proteins with functional or antagonistic properties is unlimited. DNA delivery methods that can potentially target DNA to a specific type of cell or organ, or to a patient's specific tumour type, are under intense investigation. Side effects arising from system-wide application of a drug, as in chemotherapy would be avoided by targeted DNA delivery. Maximum efficacy will be obtained by getting the agent directly to the area in need. The relatively long-lasting capacity of DNAs to generate their products *in situ* alleviates the need for continued dosing with potentially toxic and expensive drugs.

As witnessed by the death of one gene therapy patient and the development of leukemia in two others [3], the type of delivery method and carrier for the DNA is of utmost importance. While the research possibilities for designing therapeutics are unlimited, they are only as good as the safety and efficacy of the method of administration. We must focus on the new information from genomics and proteomics, but we cannot afford to overlook the need for extremely rigorous research on gene delivery methods. Research continues in designing viral vectors including adenovirus, adeno-associated virus and lentivirus that are devoid of adverse consequences [4]. In nonviral approaches, nanoparticles of cationic lipids or other biodegradable polymers such as chitosan are also under investigation [5,6].

The launch of our new open access online journal, *Genetic Vaccines and Therapy*, will serve as a sounding board for new research at the forefront of genetic engineering and gene-delivery technology for the prevention and treatment of disease. Open access means that all articles pub-

lished in GVT will be freely available to all to read online or download. The elimination of the hard-copy print format will decrease the cost of publication considerably, and the absence of subscription fees will increase readership and dissemination of new research findings all over the world. The democratization of science is finally achievable through open access.

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