Gene transfer to solid tissues for the sustained production of therapeutic proteins after implantation in patients

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Two major approaches are used in clinical applications of gene therapy, the direct introduction of the expression vector in vivo, or by the gene transduction of cells in culture, followed by transplantation of the engineered cells to the patient. We have developed an alternative approach through gene transduction to three dimensional native skin micro-organs, maintained ex vivo as organ cultures, and followed by autologous implantation of the engineered skin micro-organ tissue to patients. This methodology was first applied for the sustain production of erythropoietin (EPO) to patients with severe anemia, due to kidney failure. Following extensive preclinical studies, a phase I-II clinical study was initiated with thirteen patients. Within a week of the engineered micro-organ autologous implantation, recombinant EPO levels were detected in the blood circulation, followed by extensive erythropoiesis and the hematocrit was elevated to the clinically required level.

Following the description of my present research, I will reminiscent the development of a method for synthetic DNA amplification in the Khorana lab back in 1971, a method that was later in 1985 was named PCR.