

Non-Viral Delivery Method for Gene Therapy (Yissum)

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Genetic material encapsulated in polymeric nanoparticles

Categories	Drug delivery, nanotechnology, nanoencapsulation
Development Stage	Proof of concept in vitro and in vivo
Patent Status	US and PCT applications filed
Market Size	Nanotechnology-enabled drug delivery systems will generate over \$1.7 billion in 2009 and over \$4.8 billion in 2012

Highlights

- Safe, efficient gene therapy delivery with improved cellular permeability
- Efficient transfection, controlled-release and therapeutic delivery of genetic material such as genes, pDNA, antisense, siRNA, oligonucleotides and peptides/proteins.
- Encapsulation of the genetic material in biodegradable and biocompatible polymeric nanoparticles improves cellular permeability, stability, targeting, and sustained release properties

Our Innovation

Biologically and medically relevant genetic material encapsulated in biocompatible and biodegradable polymeric nanoparticles form an efficient, safe, targeted, non-viral delivery system that achieves high intracellular expression levels of the gene product.

Key Features

- System enables control of release rate kinetics and timing of DNA/antisense dosing for therapy involving long-term localized gene suppression
- Improved DNA or antisense oligonucleotide material for treatment of pathological disorders and proliferative disorders such as restenosis and cancer

Development Milestones

- In vitro and in vivo delivery of bioactive pDNA and antisenses
- Sustained delivery and expression of encapsulated gene material
- Formulation of nanoparticles of novel antisenses (AS, PDGFR?) in PLGA
- Synthesis evaluation of a targetor peptide with high affinity to sub-endothelial tissue
- Seeking industry collaboration for further development

The Opportunity

- Frost & Sullivan predict that revenues in the worldwide gene therapy markets which were expected to total \$150 million in 2005 could reach \$5.7 billion in 2011
- US drug delivery market will reach nearly \$91 billion by 2009

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