

Multicomponent nanoparticles and use thereof

Market sector: gene therapy, nanomedicine

Type of opportunity: licensing and/ or co-development

Scope of the problem

Gene therapy has arisen as a pioneering technique to treat or improve the health condition of the patient by modifying the patient's cells genetically (direct employment of nucleic acids as medicine).

The major historical problem is to develop efficient and safe systems for the delivery of therapeutic genes (modified DNA) into the target cells. The delivery of modified DNA into cells (transfection) can be accomplished by multiple methods (vectors). The present vectors used for gene therapy are broadly classified as Viral vectors (recombinant, biological nanoparticles), Non-viral vectors and engineered vectors (Naked DNA, inorganic nanoparticles, dendrimers, oligonucleotides). The efficiency of transfecting host cells is relatively high with viral vectors compared to non-viral methods, however the major advantage of using non-viral vectors is its bio-safety. Non-viral vectors have drawn significant attention due to its less immunotoxicity. Use of non-viral vectors in clinical trials has increased in the last years while that of viral vector saw significant decrease. Advances in efficiency, specificity, gene expression duration and safety led to an increased number of non-viral vector products entering clinical trials.

Unfortunately, none of the currently available non-viral vectors fulfills ideal vector properties. This has led to research focus on suitable ideal vector delivery system.

Patient need addressed: diseases occurring due to defect in either a single gene or set of genes due to mutation (cancer, rare diseases, cardiovascular diseases, ophthalmology)

Our innovation:

- Innovative non-viral gene delivery nanoparticles for cell transfection and selection
- The new multicomponent particles enhance the transfection efficiency on different cell types
- The specific components of the nanoparticle are: a superparamagnetic metallic oxide, certain poly(beta-amino ester)s and a polynucleotide
- These nanoparticles allows the rapid and massive separation of particle-containing cells by application of a strong magnetic field
- It can also be used as a magnetic resonance contrast for magnetic resonance imaging and tracking of labelled cells, when said cells are used for clinical or live animal research

Competitive advantages: enhances cell transfection efficiency, promotes particle endocytosis for delivery of the target polynucleotide and facilitate rapid and massive separation of particle-containing cells by application of a strong magnetic field.

Market size/ opportunity: The global gene therapy market was valued at \$584 million in 2016, and is estimated to reach \$4,402 million by 2023. Non-viral vector is anticipated as one of the most lucrative segments (Chandra and Kunselallied, Allied Market Research, February 2018). Cancer and rare diseases will be the drivers for gene therapy drug market growth during the period 2018-2028 (Visiongain Report, Jun 12, 2018).

Global nanomedicine market expected to reach \$261,063 Million by 2023 (Allied Market Research, Nov. 2, 2017).

Intellectual property

Spanish patent application P201830507 (May 25, 2018)

International patent PCT to be applied