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PATENT

Nanovesicles and its use for nucleic acid delivery

Market sector: nanomedicine, oncology

Type of opportunity: licensing and/ or co-development

Scope of the problem

RNA therapeutics is an emerging field with a promising number of targets around all the transcriptome, which includes small RNAs like small interfering RNA (siRNA), microRNA (miRNA), among others. Although, the RNA based-therapies may be an alternative to chemoresistant tumors, the in vivo administration is still a challenge in the field, due to the rapid clearance and degradation of small RNAs in the bloodstream.

As of today, nanomedicine promises the precise delivery of drugs to disease sites (i.e. tumours) with reduced off-target toxicities. Liposomal nanovesicles are one of the most promising candidates for drug delivery in nanomedicine. However, these liposomal-based medicinal products still present unsolved drawbacks such as poor colloidal stability, short half-life when administered in the blood circulation, nonspecific bio-distribution and modification of endogenous RNA machinery.

Novel engineered non-liposomal nanovesicles, named **Quatsomes** (**QS**), have been developed to overcome some of the previously stated problems related to application of RNA based therapeutics in the treatment of human diseases, such as cancer.

Patient need addressed: neuroblastoma

Our innovation:

- Development of the new QS, an innovative tool for nucleic acid delivery, allowing the transported nucleic acid to perform its activity inside the cell (cytosol).
- The engineered QS are composed by preservatives and sterol derivates, all elements FDA and EMA approved .
- These QS have shown great in vitro transfection efficiencies and good biocompatibility, comparable to other commercial transfection reagents (e.g. Lipofectamine® 2000) and also potent anti-tumoural activity in neuroblastoma cell lines.
- Development of a pharmaceutical composition comprising a therapeutically effective amount of the nanovesicle and a pharmaceutically acceptable excipient or vehicle
- The nanovesicle or the pharmaceutical composition can be used as a medicament for the treatment of diseases that use nucleic acids as therapeutic agent (e.g. cancer)
- These QS can be also used as a bioimaging and theranostic tool.

Competitive advantages: Engineered QS are prepared by a single-step and robust CO2-based methodology that can be scaled up while performed under GMP conditions. CO2-based methodology guarantees superior structural homogeneity, both regarding size and lamellarity, and reproducibility. The nanovesicles are chemically and physically stable over time (several months). QS shown great RNA protection from ribonuclease-mediated degradation. QS are versatile and tuneable drug carrier which can be functionalized either with targeting units or imaging agents obtaining multifunctional nanovesicles.

Market size/ opportunity: Global nanomedicine market expected to reach $261,063 Million by 2023 (Allied Market Research, Nov. 2, 2017). Neuroblastoma is the third most common cancer in children and accounts for about 15 percent of pediatric cancer-related deaths (Marketline 2018).

Intellectual property

International patent application, PCT (May 12, 2020)
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