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FROM KNOWLEDGE TO ACTION APPLICATION

Title: AI-driven drug discovery and development platform for miRNA therapeutics

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Category: Science to Society

Problem (max. 50 words):
Potential multitarget medicines like microRNAs are a breakthrough in drug development, with the potential to treat multiple diseases in the near future, as shown by the most recent 2024 Nobel Prize for the discovery of miRNAs.

Unmet Need (max. 50 words):
There are several currently incurable diseases the background molecular pathology of which are very complex, therefore, single target drugs are unable to interfere with the pathology. Such diseases include myocardial infarction, post-infarction heart failure, several types of cancers, sarcopenia, etc.

Project Description (max. 200 words):
We have previously discovered cardioprotective microRNAs (oligonucleotides, we termed protectomiRs) and have 2 patent families around 4 different miRNA sequences. We have also developed miRNAtarget (mirnatarget.com), a bioinformatics tool for the analysis of the targets of differentially expressed miRNAs. The current project builds on these previous discoveries with the aim to develop a new platform technology for miRNA discovery and development with the potential for disruptive innovation in the treatment of ischemic heart diseases and cancers, leading causes of mortalities worldwide, as well as other diseasese, such as musculosceletal diseases including sarcopenia.

In the present project we aim to:
1. Develop an AI-driven microRNA drug discovery platform, built on network theoretical and machine learning approaches for the prediction of oligonucleotide sequences and combination of miRNAs as drug candidates for the treatment of multiple diseases.
2. Increase the stability and delivery efficacy of predicted oligonucleotide sequences through design and synthesis of chemically modified oligonucleotides and their lipid nanoparticle formulation.
3. Validate the AI-driven microRNA drug discovery tool by preclinical testing of therapeutic miRNA candidates up to small animal proof-of-concept stage.

Hypothesis (25 words):
Specific miRNAs or combination of them, by regulating complex gene expression patterns, may provide treatment for yet incurable complex diseases, such as cardiovascular, oncology, or musculoskeletal diseases.

Expected results
Expected results will be lead microRNA drug candidates and their combinations validated up to in vivo proof of concept stage, their intellectual property protection and dissemination in scientific journals. The innovation here is the first to develop miRNA therapies with network theory–based approach.

Call for collaboration:

We are looking for experts in miRNA-like oligonucleotide delivery and oligonucleotide chemical modification, experts in RNA drug development in any therapeutical indication.