miRNAs FOR DIAGNOSIS, PROPHYLAXIS AND TREATMENT OF AUTOPHAGIC DISEASES

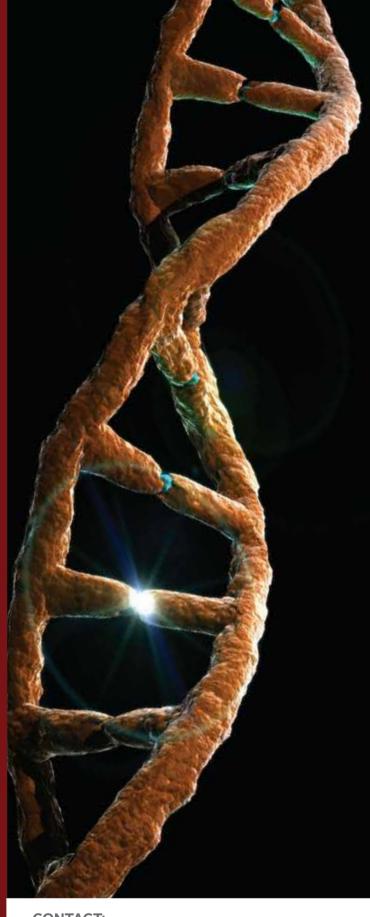
This novel technology introduces an efficient way to identify the cellular degradation in cellular functions by utilizing specic miRNA families to diagnose existing or potential pathologies enabling fast and efficient diagnosis, prevention and therapy of a wide range of autophagy-related diseases such as cancer, alzheimers, early dementia and hereditary diseases.

Potential Applications

Medical

This class of miRNAs have potential applications in diagnosis, prophylaxis and treatment of diseases involving autophagic abnormalities such as:

- Neurodegenerative and nervous system diseases
- Cancer
- · Heart and liver diseases
- Ageing
- Myopathies
- Auto-immune and inflammatory diseases and immune deficiencies
- Infectious diseases
- Ischemic diseases
- Diabetes
- Axonal injury
- · Lysosomal storage diseases



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Customer Benefits

- miRNAs are generally more stable than proteins and other forms of RNA, hence are suitable candidates for incorporation into point-of-care diagnostic kits
- miRNAs are organic molecules and natural antisense interactors
- miRNA expression profiles can be used to diagnose disease states as deregulated miRNAs contribute to the initiation and development of diseases

Technology Features & Specifications

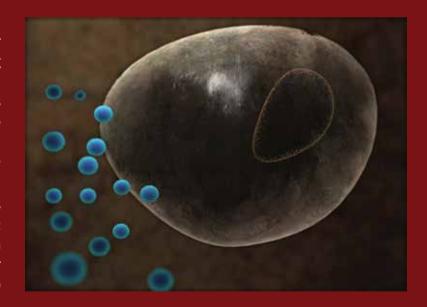
This technology involves regulation of the autophagic pathway at multiple levels via a specific miRNA family or its inhibitors. Mechanistically, miR-376 families of microRNAs were shown by us to decrease cellular levels of key autophagy proteins Beclin 1 and ATG4. Many miRNAs are master regulators that modulate many genetic, biological and pathological processes. Mouse models mimicking human disease show that miRNAs play important roles in various disease progression, hence are good targets for therapeutic development. Their small size (22-24 nucleotides in length) and organic nature make them very attractive for drug development.

Therapeutics and diagnostics can be designed around these miRNAs to enable efficient and fast ways to diagnose, prevent or treat diseases arising from excess autophagy by blocking autophagic pathways in cells. Conversely, the diseases occurring because of deficiencies in autophagy can be controlled by suppressing these miRNAs with inhibitors, thereby reinstating autophagic pathways.

Market Trends and Opportunities

MicroRNA-based for research diagnostics and treatment is a promising emerging field in drug research and several large pharmaceutical companies have already invested substantially into miRNA-related research and therapeutics. (see Melnikova I, Nature Reviews in Drug Discovery 2007).

The total global microRNA market was valued at nearly US\$338.3 million in 2012 and expected to reach US\$763 million in 2017 after increasing at a five-year compound annual growth rate (CAGR) of 17.7%.



Our identification of the "hsa-miR-376" family of miRNAs as potential candidates for drug and diagnostics development is timely to meet the growing demands of this new market segment. RNA therapeutics offer the advantage of low cost synthesis and stability, and as organic molecules that can be targeted specially are anticipated to have less side effects on patients.



