A New Class of piRNA-based Cancer Therapeutics

OCR Number: OCR 6901

Description:

PIWI-interacting RNAs (piRNAs) are a novel class of small noncoding RNAs (24-32 nt), that guide PIWI proteins to transposons and stabilize the genome. piRNA class includes >30,000 unique types with <100 gene targets, functions both in cytoplasm and in nucleus and mediates both transcriptional and post-transcriptional regulation.

Advantages of piRNA-based therapeutic approach compared to siRNA and miRNA-based approaches:

- **higher target specificity**, due to longer seed sequences of piRNAs.
- **higher tissue specificity**, as many PIWI proteins are expressed only in tumor cells.
- **gene-specific DNA methylation and gene silencing at transcriptional level**.
- **higher efficacy and lower toxicity**.

- Demonstrated cancer-specific in-vitro and in-vivo efficacy in several cancer models, including liver cancer and glioblastoma. Potential diagnostic and research tool applications.
- **Lead Innovator**: Yong Zhu, Ph.D.
- **IP status**: PCT/US17/19741 filed.
- **References**: Oncotarget (in press); CEBP (2016); Carcinogenesis (2015).

![U87 xenograft tumor growth](chart.png)

Table: piR-8041 reduces cell growth by ~50%. 
Top: Bioluminescence measurements of luciferase-expressing intracranial tumors at multiple time points. Bottom: Images of representative mice from each treatment group on day 10 after tumor implantation.

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