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).

![Figure](U87 xenograft tumor growth)

The above graph shows the effect of piR-8041 on U87 xenograft tumor growth. The treatment significantly reduces tumor growth by approximately 50% compared to the control group.

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