

Anticipating the Nobel: Non-coding RNAs in diagnostics and therapy

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Presentation

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The speaker presented an overview of non-coding RNAs, particularly microRNAs, highlighting their evolution from considered 'junk DNA' to potential diagnostic biomarkers and therapeutic targets, with several RNA-based drugs now FDA-approved for non-cancer conditions.

KEY POINTS

- MicroRNAs are evolutionarily conserved molecules with excellent stability (located in exosomes and bound to proteins) that can be detected in tissue, blood, and feces, making them potential biomarkers for cancer and inflammatory conditions.
- The speaker described GastroClear, a microRNA-based diagnostic test approved in Singapore for gastric cancer, which analyzed a 12-microRNA panel in prospective studies; negative results had very few missed cancers, but the speaker noted 1400 false-positive patients required endoscopy.
- Several FDA-approved RNA-interference drugs now exist, including Patisiran for hereditary transthyretin amyloidosis (showing clinical improvement in phase 3 trials) and drugs targeting hemophilia, porphyria, and hypercholesterolemia, with a recent well-tolerated study targeting hsd17b13 for NAFLD published in Journal of Hepatology.
- The speaker stated that despite 20 years of research, no microRNA therapeutics are approved yet, with challenges including delivery to specific locations and cells, though RNA-interference technology shows promise.
- In the Q&A, the speaker acknowledged that microRNA-based diagnosis for pancreatic cancer faces specificity issues, as microRNA-21 is elevated in many cancers, and translation to clinical use remains difficult.

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