RNA Therapeutics: The Evolving Landscape of RNA Therapeutics

RNA Therapeutics: The Evolving Landscape of RNA Therapeutics provides a comprehensive overview of RNA therapeutic modalities, from bench to bedside, with an emphasis on the increasingly impactful areas of gene therapy, oligonucleotide therapeutics, gene editing, and delivery. International leaders in the field have examined RNA-based therapeutic tools that have been developed to date to modulate cellular processes such as transcription, translation, and protein function. Approved RNA-based therapies and lessons learned from failed therapies are discussed in depth, as are evolving advances in RNA biochemical analysis and similar advances that are enabling the clinical application of RNA-based therapies.

Later sections discuss delivery technologies, remaining hurdles in research and translation, the therapy development process from the lab to the clinic, and novel RNA-based therapies currently in development.

Key Features

- Features leading experts in the field of RNA therapeutics, spanning all classes of RNA therapies
- Provides a detailed examination of approved RNA therapies and lessons learned from failed therapeutics
- Covers all aspects of therapeutic discovery and preclinical development as well as clinical translation, manufacturing, and regulatory aspects

About the Editors

Dr. Paloma Giangrande is the VP of Biology at Wave Life Sciences, Cambridge, MA and Adjunct Associate Professor of Internal Medicine at the University of Iowa, Iowa City, IA. Her career has been dedicated to the investigation and development of RNA-based therapeutic approaches (including RNA aptamers, siRNAs, and mRNA) for cancer, cardiovascular disease, rare diseases, and critical illness. As a consequence, she has developed a deep understanding of these technologies, diseases, and associated disease mechanisms. She is also an internationally recognized expert in oligonucleotide therapeutics and delivery. Towards this end, she was the first to demonstrate that RNA ligands (aptamers) can be used to deliver therapeutic siRNAs to target cells. Her 60+ publications and 9+ patents are a testament to this body of work and her commitment to the development of novel RNA-based therapies for many diseases.

Dr. Vittorio de Franciscis is Senior Collaborator at the CNR Institute of Genetic and Biomedical Research (IRGB) Milan, Italy. For more than 10 years, his research interest has focused on translating the understanding of the molecular basis of neoplastic transformation in the design of selective therapies based on the use of therapeutic RNAs (aptamers, siRNAs, miRNAs). In his laboratory, they demonstrated the possibility of generating aptamers that bind to and inhibit high-affinity transmembrane receptors involved in cancer progression (including EGFR, AXL, PDGFR, IR, and Eph). For the selection of aptamers, they developed a variant of the SELEX technology that makes use of living cells as a complex target. They have recently shown that such RNA aptamers against RTK can be used as targeting moieties for the selective delivery of therapeutic miRNAs and anti-miRs against tumor cells and tissues.

Dr. John J. Rossi is Lidow Family Research Endowed Chair and Professor in the Department of Molecular and Cellular Biology, Beckman Research Institute of the City of Hope. He currently serves as the Morgan and Helen Chu Dean's Chair and as the Dean of Irell & Manella Graduate School of Biological Sciences. He served as Associate Director of Laboratory Research—City of Hope Comprehensive Cancer Center for City of Hope. He joined City of Hope, Inc. (COH) in 1980 as Assistant Research Scientist in the Department of Molecular Genetics. He was Chairman of the Division of Biology in 1992. In 1993, COH bestowed its highest honor upon him by naming him to its Gallery of Medical and Scientific Achievement for his pioneering work at the molecular level in the battle against AIDS and other major diseases.

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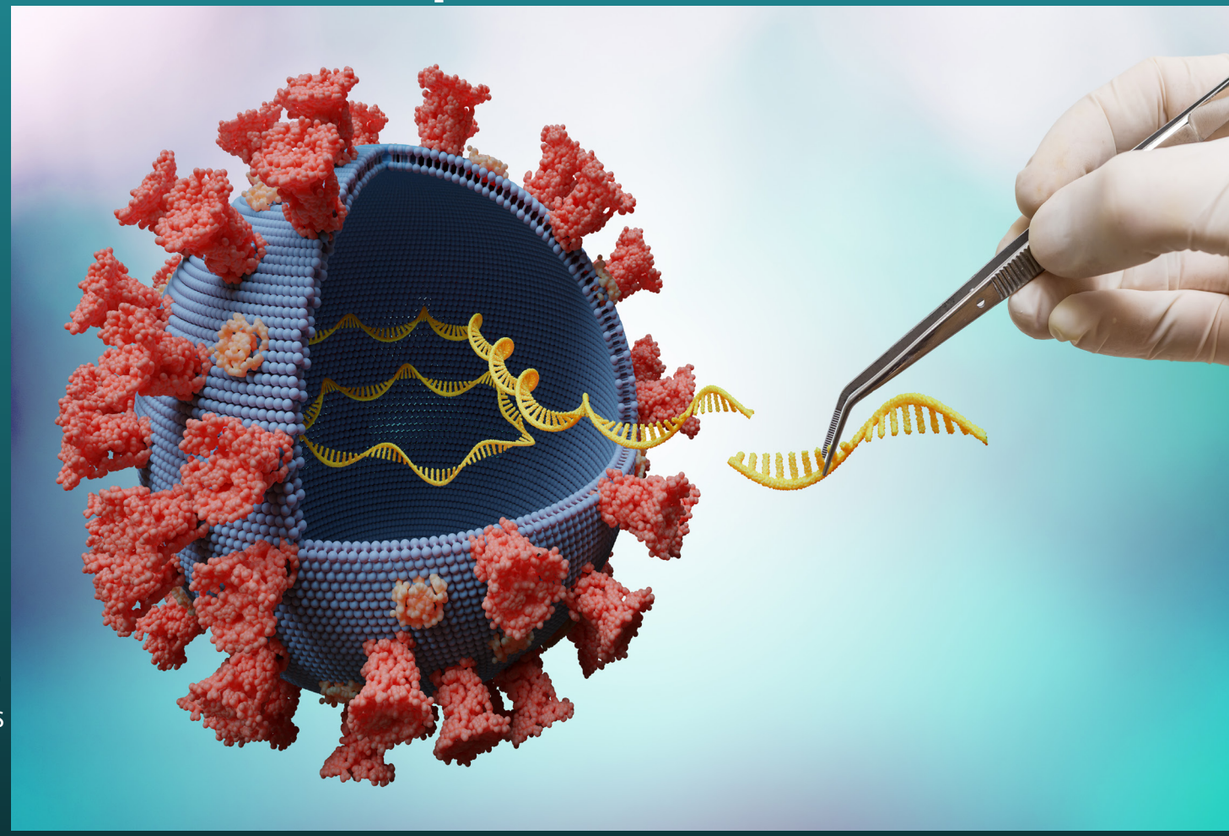

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