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Titolo	Antisense-Based Drugs and Therapeutics: Preclinical and Clinical Considerations for Development
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Altri autori (Persone)	FerrariNicolay SeguinRosanne
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Nota di contenuto	Mechanisms of oligonucleotide actions -- The medicinal chemistry of antisense oligonucleotides -- Cellular pharmacology of antisense oligonucleotides -- Pharmacokinetics and pharmacodynamics of antisense oligonucleotides -- Tissue distribution, metabolism and clearance -- Hybridization-independent effects : principles and specific considerations for oligonucleotide drugs -- Hybridization-dependent effects : the prediction, evaluation and consequences of unintended target hybridization -- Class-related proinflammatory effects -- Exaggerated pharmacology -- Genotoxicity tests for novel oligonucleotide-based therapeutics -- Reproductive and developmental toxicity testing strategies for oligonucleotide-based therapeutics -- Specific considerations for preclinical development of inhaled oligonucleotides -- Lessons learned in oncology programs -- Inhaled antisense for treatment of respiratory disease -- Antisense oligonucleotides for treatment of neurology diseases -- Nucleic acids as adjuvants -- Splice-switching oligonucleotides -- CMC aspects for the clinical development of spiegelmers.
Sommario/riassunto	A comprehensive review of contemporary antisense oligonucleotides

drugs and therapeutic principles, methods, applications, and research Oligonucleotide-based drugs, in particular antisense oligonucleotides, are part of a growing number of pharmaceutical and biotech programs progressing to treat a wide range of indications including cancer, cardiovascular, neurodegenerative, neuromuscular, and respiratory diseases, as well as other severe and rare diseases. Reviewing fundamentals and offering guidelines for drug discovery and development, this book is a practical guide covering all key aspects of this increasingly popular area of pharmacology and biotech and pharma research, from the basic science behind antisense oligonucleotides chemistry, toxicology, manufacturing, to safety assessments, the design of therapeutic protocols, to clinical experience. Antisense oligonucleotides are single strands of DNA or RNA that are complementary to a chosen sequence. While the idea of antisense oligonucleotides to target single genes dates back to the 1970's, most advances have taken place in recent years. The increasing number of antisense oligonucleotide programs in clinical development is a testament to the progress and understanding of pharmacologic, pharmacokinetic, and toxicologic properties as well as improvement in the delivery of oligonucleotides. This valuable book reviews the fundamentals of oligonucleotides, with a focus on antisense oligonucleotide drugs, and reports on the latest research underway worldwide. Helps readers understand antisense molecules and their targets, biochemistry, and toxicity mechanisms, roles in disease, and applications for safety and therapeutics Examines the principles, practices, and tools for scientists in both pre-clinical and clinical settings and how to apply them to antisense oligonucleotides Provides guidelines for scientists in drug design and discovery to help improve efficiency, assessment, and the success of drug candidates Includes interdisciplinary perspectives, from academia, industry, regulatory and from the fields of pharmacology, toxicology, biology, and medicinal chemistry Oligonucleotide-Based Drugs and Therapeutics belongs on the reference shelves of chemists, pharmaceutical scientists, chemical biologists, toxicologists and other scientists working in the pharmaceutical and biotechnology industries. It will also be a valuable resource for regulatory specialists and safety assessment professionals and an important reference for academic researchers and post-graduates interested in therapeutics, antisense therapy, and oligonucleotides.