1. Record Nr. UNINA9910552988503321

Autore	Arechavala-Gomeza Virginia
Titolo	Antisense RNA Design, Delivery, and Analysis / / edited by Virginia Arechavala-Gomeza, Alejandro Garanto
Pubbl/distr/stampa	New York, : Springer Nature, 2022 New York, NY : , : Springer US : , : Imprint : Humana, , 2022
ISBN	9781071620106 107162010X
Edizione	[1st ed. 2022.]
Descrizione fisica	1 online resource (XVII, 422 p. 69 illus., 59 illus. in color.)
Collana	Methods in Molecular Biology, , 1940-6029 ; ; 2434
Disciplina	615.5
Soggetti	Therapeutics Biomaterials Nucleic acids Nucleic Acid Àcids nucleics RNA Teràpia genètica Llibres electrònics
Lingua di pubblicazione	Inglese
Formato	Materiale a stampa
Livello bibliografico	Monografia
Nota di contenuto	Introduction and History of the Chemistry of Nucleic Acids Therapeutics -- Antisense RNA Therapeutics: A Brief Overview -- Design of Bifunctional Antisense Oligonucleotides for Exon Inclusion -- Design and Delivery of SINEUP: A New Modular Tool to Increase Protein Translation -- How to Design U1 snRNA Molecules for Splicing Rescue -- Conjugation of Nucleic Acids and Drugs to Gold Nanoparticles -- Determination of Optimum Ratio of Cationic Polymers and Small Interfering RNA with Agarose Gel Retardation Assay -- Generation of Protein-Phosphorodiamidate Morpholino Oligomer Conjugates for Efficient Cellular Delivery via Anthrax Protective Antigen -- Development and Use of Cellular Systems to Assess and Correct Splicing Defects -- Modeling Splicing Variants Amenable to Antisense Therapy by Use of CRISPR-Cas9-Based Gene Editing in HepG2 Cells --

In Vitro Models for the Evaluation of Antisense Oligonucleotides in Skin -- In Vitro Delivery of PMOs in Myoblasts by Electroporation -- Rapid Determination of MBNL1 Protein Levels by Quantitative Dot Blot for Evaluation of Antisense Oligonucleotides in Myotonic Dystrophy Myoblasts -- Evaluation of Exon Skipping and Dystrophin Restoration in In Vitro Models of Duchenne Muscular Dystrophy -- Generation of Human iPSC-Derived Myotubes to Investigate RNA-Based Therapies In Vitro -- Eye on a Dish Models to Evaluate Splicing Modulation -- Establishment of In Vitro Brain Models for AON Delivery -- Considerations for Generating Humanized Mouse Models to Test Efficacy of Antisense Oligonucleotides -- Generation of Humanized Zebrafish Models for the In Vivo Assessment of Antisense Oligonucleotide-Based Splice Modulation Therapies -- Use of Small Animal Models for Duchenne and Parameters to Assess Efficiency upon Antisense Treatment -- In Vivo Models for the Evaluation of Antisense Oligonucleotides in Skin -- Delivery of Antisense Oligonucleotides to the Mouse Retina -- Delivery of Antisense Oligonucleotides to the Mouse Brain by Intracerebroventricular Injections -- Intrathecal Delivery of Therapeutic Oligonucleotides for Potent Modulation of Gene Expression in the Central Nervous System -- Preclinical Safety Assessment of Therapeutic Oligonucleotides -- Preclinical Evaluation of the Renal Toxicity of Oligonucleotide Therapeutics in Mice -- Protocol for Isolation and Culture of Mouse Hepatocytes (HCs), Kupffer Cells (KCs), and Liver Sinusoidal Endothelial Cells (LSECs) in Analyses of Hepatic Drug Distribution -- Patent Considerations When Embarking on New Antisense Drug Programs.

Sommario/riassunto

This open access volume gathers a variety of models, delivery systems, and approaches that can be used to assess RNA technology for exploiting antisense as a therapeutic intervention. Beginning with a section on the design of antisense technology and their delivery, the book continues by covering model systems developed to evaluate efficacy, both in vivo and in vitro, as well as methods to evaluate preclinically the toxicity associated with these new potential drugs, and intellectual property considerations. Written for the highly successful Methods in Molecular Biology series, chapters include introductions to their respective topics, lists of the necessary materials and reagents, step-by-step, readily reproducible laboratory protocols, and tips on troubleshooting and avoiding known pitfalls. Authoritative and practical, Antisense RNA Design, Delivery, and Analysis provides basic knowledge and a large collection of methods to facilitate the workof newcomers to this vibrant and expanding field. This book was conceived thanks to the network DARTER (Delivery of Antisense RNA Therapeutics). DARTER is funded by the EU Cooperation of Science and Technology (COST), which aims to enhance interaction and collaborations between researchers in Europe and other countries.