Record Nr. UNINA9910141646903321 Minicircle and miniplasmid DNA vectors [[electronic resource]]: the **Titolo** future of nonviral and viral gene transfer / / edited by Martin Schleef Pubbl/distr/stampa Weinheim, Germany, : Wiley-Blackwell, 2013 **ISBN** 3-527-67042-4 1-299-24148-4 3-527-67043-2 Descrizione fisica 1 online resource (259 p.) Altri autori (Persone) SchleefM (Martin) Disciplina 615.895 Soggetti Gene therapy Genetic vectors **Plasmids** Lingua di pubblicazione Inglese **Formato** Materiale a stampa Livello bibliografico Monografia Description based upon print version of record. Note generali Nota di bibliografia Includes bibliographical references and index. Nota di contenuto Minicircle and Miniplasmid DNA Vectors: The Future of Non-Viral and Viral Gene Transfer; Contents; List of Contributors; Preface; 1 Minicircle Patents: A Short IP Overview of Optimizing Nonviral DNA Vectors; 2 Operator-Repressor Titration: Stable Plasmid Maintenance without Selectable Marker Genes; 2.1 Introduction; 2.2 Antibiotics and Metabolic Burden: 2.3 The Mechanism of ORT: 2.4 ORT Strain Development; 2.5 ORT Miniplasmids; 2.6 DNA Vaccine and Gene Therapy Vectors; 2.7 ORT-VAC: Plasmid-Based Vaccine Delivery Using Salmonella enterica; 2.8 Recombinant Protein Expression 2.9 Conclusions and Future DevelopmentsReferences; 3 Selection by RNA-RNA Interaction: Maximally Minimized Antibiotic Resistance-Free Plasmids: 3.1 Gene Therapy and DNA Vaccines: Emerging Technologies; 3.1.1 Therapeutic Plasmids: General Design Principles; 3.2 Therapeutic Plasmids: Novel Design and the Problem of Selection; 3.2.1 Replication Control of ColE1-Type Plasmids as an Alternative Selection Marker: 3.2.2 The MINIback Concept: Selection by RNA-RNA Interaction; 3.2.3 Improved Production Processes by MINIback Plasmids; 3.2.4 Improving

Sequence Composition: 3.2.5 Efficient Gene Transfer

3.3 Conclusions Acknowledgments; References; 4 Plasmid-Based

Medicinal Products - Focus on pFAR: A Miniplasmid Free of Antibiotic Resistance Markers: 4.1 Introduction: Rationale for the Development of Biosafe DNA Plasmid Vectors: 4.2 Specific Requirements for the Use of DNA Product as Medicines; 4.2.1 Requirements for Plasmid Quality and Purity; 4.2.2 Requirements for the Removal of Antibiotic Resistance Markers from Plasmid DNA; 4.2.2.1 Requirements for Biosafe Plasmids; 4.2.2.2 Positive Impact on the Removal of Antibiotic Resistance Markers 4.2.2.3 Effect of Plasmid Size on Gene Transfer Efficiency In Vitro and In Vivo 4.3 Nonviral Gene Vectors Devoid of Antibiotic Resistance Markers: 4.3.1 Generalities; 4.3.2 Selection Systems Devoid of Antibiotic Resistance Markers; 4.3.2.1 Complementation of Host Auxotrophy by a Function-Encoded Plasmid: 4.3.2.2 The Operator-Repressor Titration (ORT) System; 4.3.2.3 Protein-Based Antidote/Poison Selection Systems; 4.3.2.4 RNA-Based Selection Marker; 4.3.2.5 Suppression of a Nonsense Mutation: 4.4 The pFAR Plasmid Family: 4.4.1 Description of the Antibiotic-Free Selection System 4.4.2 pFAR Vectors Promote Efficient Expression in Several Types of Mammalian Cells 4.4.2.1 In Vitro Transfection Study; 4.4.2.2 In Vivo Transfection Studies; 4.4.3 Concluding Remarks on the pFAR4 Biosafe Miniplasmid; 4.5 Concluding Remarks and Perspectives: Acknowledgments: References: 5 Plasmid DNA Concatemers: Influence of Plasmid Structure on Transfection Efficiency; 5.1 Introduction; 5.2 Plasmid DNA Topology and Size; 5.3 Plasmid DNA Concatemers; 5.4 Conclusions; Acknowledgments; References; 6 Analytical Tools in Minicircle Production; 6.1 Introduction

## Sommario/riassunto

This first title on the topic provides complete coverage, including the molecular basis, production and possible biomedical applications. Written by the most prominent academic researchers in the field as well as by researchers at one of the world's leading companies in industrial production of minicircle DNA, this practical book is aimed at everyone who is directly or indirectly involved in the development of gene therapies.

6.1.1 Gene Transfer for Therapy, Vaccination, and Stem Cells