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**Volume 74**

# **RIBOZYME PROTOCOLS**

*Edited by*  
***Philip C. Turner***



**Humana Press**

# Ribozyme Protocols

**Mouldy Sioud**



## **Ribozyme Protocols:**

**Ribozymes and siRNA protocols** Mouldy Sioud, 2008-02-03 In this completely updated and expanded edition of a classic bench manual hands on experts take advantage of the latest advances in ribozyme DNAzyme hammerhead ribozymes and derivatives and RNA interference technologies to describe in detail the exciting and successful methods now available for gene inactivation in vitro and in vivo Their optimized techniques employ hairpin ribozymes DNAzymes hammerhead ribozymes and derivatives group I intron ribozymes RNase P ribozymes and siRNAs as well as general methods for RNA structure analysis delivery of oligonucleotides and gene therapy Also provided are novel methods for identifying accessible cellular mRNA sites group I intron and RNase P ribozyme protocols for effective design selection and therapeutic applications and the latest RNAi methods for sequence specific gene silencing in a wide variety of organisms Additional techniques cover the analysis of ribozyme structures and conformational transitions using nucleotide analog interference mapping and fluorescence resonance energy transfer the use of ribozymes in clinical and gene therapy and the use of ribozymes and DNAzymes in rodent models of human disease Each proven protocol includes a background introduction outlining the principle behind the technique step by step instructions lists of equipment and reagents and tips on troubleshooting and avoiding known pitfalls Comprehensive and up to date Ribozymes and siRNA Protocols details for experienced and novice investigators alike the many exciting advances in our understanding of nucleic acid enzymes as well as demonstrating how they may be used to analyze gene function and target validation and to productively develop novel therapeutics for human diseases Ribozyme Protocols Philip C. Turner, 2013-09-20 The purpose of Ribozyme Protocols is to provide a helpful compilation of protocols that will be of use not only to those with some experience of ribozymes but also to those wishing to use ribozymes for the first time Although it is usually impossible to cover every aspect of a scientific field I believe this book approaches that ideal and should help all readers perform meaningful experiments using ribozymes To design ribozymes one must consider whether the target site will be accessible this task can be facilitated by using computer programs that predict the folding of the target RNA Such programs are detailed in Chapters 2 and 3 If the chosen target is an RNA virus that can mutate rapidly it makes sense to consider those parts of the genome that are least likely to change during viral replication An example of how this can be done is described in Chapter 4 Although computer analysis may be a useful starting point to select target sites there seems at the moment to be no guarantee that any particular chosen site will be efficiently cleaved Some workers have deliberately bypassed this problem by using libraries of ribozyme sequences and by selecting those that actually hybridize to and or cleave the target these methods are described in Chapters 5 and 6

**Antisense and Ribozyme Methodology** Ian Gibson, 2008-09-26 Antisense and ribozymes have a relatively short yet successful history as research tools in gene expression studies and thus are considered as having high potential reagents in treating viral infections and cancer This laboratory companion provides detailed information on the potential advantages and

limitations of this methodology It critically discusses potential pitfalls presents strategies for choosing targets and delivery systems so as to allow the selection of the optimum methodology for achieving fast and reliable experimental success with any human or other biological system For researchers technicians and advanced graduates in experimental medicine molecular and cell biology

**Plant Virology Protocols** Gary D. Foster, Sally Taylor, 2008-02-03 The aim of Plant Virology Protocols is to provide a source of information to guide the reader through the wide range of methods involved in generating transgenic plants that are resistant to plant viruses To this end we have commissioned a wide ranging list of chapters that will cover the methods required for plant virus isolation RNA extraction cloning coat protein genes introduction of the coat protein gene into the plant genome and testing transgenic plants for resistance The book then moves on to treatments of the mechanisms of resistance the problems encountered with field testing and key ethical issues surrounding transgenic technology Although Plant Virology Protocols deals with the cloning and expression of the coat protein gene the techniques described can be equally applied to other viral genes and nucleotide sequences many of which have also been shown to afford protection when introduced into plants The coat protein has however been the most widely applied and as such has been selected to illustrate the techniques involved Plant Virology Protocols has been divided into six major sections containing 55 chapters in total

Vision Research Protocols P. Elizabeth Rakoczy, 2008-02-02 Elizabeth Rakoczy and a team of leading clinical and experimental scientists describe in step by step detail the key techniques essential to effective molecular biological research in ophthalmology and optometry These readily reproducible methods are adapted to the special requirements of vision research with coverage that ranges from the most basic to the most sophisticated technologies Included are methods for the down regulation of gene expression new gene therapy techniques and for the development of transgenic and knockout animal models for testing novel therapies Eminently accessible and clinically relevant Vision Research Protocols provides experimental and biomedical investigators in ophthalmology and optometry with a rich panoply of most powerful tools with which to ask and answer all the important questions emerging from the dramatically advancing work in vision research today

*Cancer Gene Therapy* Nagy Habib, 2006-04-11 With the coming of the new millennium we are witnessing a revolution in our understanding of cancer genetics These are very exciting times Today we have at our disposal the technology to diagnose abnormalities in our cancer genes and the means to correct the deficit and very soon we will have the complete sequence of the human genome With the use of gene chip technology the way doctors will be able to assess patients will change completely Today we can diagnose abnormalities in ten thousand genes and within a short period of time we will be able to screen through our genome and discover potential abnormalities in our proto oncogenes tumour suppressor genes differentiating genes apoptotic genes and pro inflammatory genes In this book various authors have highlighted specific genes that could be expressed overexpressed neutralised or hnessed to achieve cancer control The problem of transferring the therapeutic gene into the cancer cell has been partly addressed with major developments in the

field of naked plasmid DNA adenovirus retrovirus and adeno associated viruses However further improvements are yet to be made to achieve significant gene transfer Gene expression in particular specificity of gene transfer is obviously an important issue and one which is highlighted in this book by the use of specific promoter

**Target Discovery and Validation Reviews and Protocols** Mouldy Sioud, 2008-02-04 Target discovery is a field that has existed for several years but is so vibrant today because of the recent progress in our understanding of the molecular mechanisms of many human diseases and the technical advances in target identification and validation More sophisticated gene profiling technologies such as DNA microarrays and serial analysis of gene expression permit rapid identification of lead targets Moreover analysis of gene networks in living organisms allows the identification of target genes that operate in defined physiological pathways With the sequencing of several genomes completed and the rapidly growing gene expression databases there is now greater impetus than ever before for in silico discovery of therapeutic targets Also recent advances in genetic technologies have increased our ability to generate mouse models for human diseases The implications of these genetically modified animals in drug development are several including identification of new drug targets predicting efficacy and uncovering possible side effects Together these recent technical advances should allow researchers to make the most informed choice early and advance the chosen targets toward clinical studies Regarding cancers any difference between a cancer and a normal cell could potentially be exploited as a therapeutic target The hope is that drugs targeting specific constituents or pathways in cancer cells will provide more effective therapy either alone or in combination with other currently used anticancer drugs In addition to drug targets identifying new target antigens remains as much of a challenge as improving tumor vaccines already in the clinic

**Combinatorial Chemistry in Biology** Michael Famulok, Ernst-L. Winnacker, Chi-Huey Wong, 2013-06-29 The essence of combinatorial chemistry or techniques involving molecular diversity is to generate enormous populations of molecules and to exploit appropriate screening techniques to isolate active components contained in these libraries This idea has been the focus of research both in academia and in the pharmaceutical or biotechnology industry Its developments go hand in hand with an exploding number of potential drug targets emerging from genomics and proteomics research When the editors of Current Topics in Microbiology and Immunology encouraged us to assemble the present volume on Combinatorial Chemistry in Biology we immediately felt that this might prove quite beneficial for the audience of this series The field of combinatorial chemistry extends over a broad range of disciplines from synthetic organic chemistry to biochemistry from material sciences to cell biology Each of these fields may have its own view on this topic something which is reflected in a growing number of monographs and special editions of journals devoted to this issue or aspects thereof The title of the present volume of Springer Verlag's series suggests that it also has its own special focus And generally speaking this is not wrong we would even claim the special focus of this volume is on the immunologically relevant aspects of combinatorial chemistry

**Confocal Microscopy** Stephen W. Paddock, 2008-02-03 **Advances in Hematopoietic Stem Cell Transplantation**

**and Molecular Therapy** Rainer Haas,Ralf Kronenwett,Georg Sczakiel,2012-12-06 Blood stem cell transplantation has become an established therapeutic option in the treatment of malignant diseases At the same time molecular therapeutic approaches i e gene therapy are promising alternatives to the classical treatment of cancer The proceedings give an overview of the biology of hematopoietic progenitor cells peripheral stem cell transplantation in patients with hematological malignancies recent developments in molecular diagnosis and gene therapeutic concepts and show that successful future developments require cooperative research connecting basic science with clinical application      **Gene Therapy Methods**

M. Ian Phillips,2002-02-22 This volume in the prestigious Methods in Enzymology series discusses methods currently used in preclinical and clinical gene therapy Subjects covered in this book such as the use of adeno associated virus delivery for treatment of Parkinson s disease are topical and are presented in the methods oriented style popularized by this series Discusses methods currently used in preclinical and clinical gene therapy Covers the use of adeno associated virus delivery for treatment of Parkinson s disease

**Annual Reports in Medicinal Chemistry** ,1995-10-10 Praise for the Series This series is one of the very few annual publications which justify the title of an absolute must for the pharmacologist chemist or physician who is interested in the chemistry of drug development EnzymologiaThis book is strongly recommended for researchers teachers students administrators in short anyone whose interests impinge on medicinal chemistry In view of the work s documented reference value it is a must for inclusion in the scientist s personal library Journal of Medicinal ChemistryAll topics are covered in sufficient depth with extensive references to allow either the specialist or the novice to be informed of the latest developments in a particular area of medicinal chemistry Norman Gilman Hoffmann La Roche Inc in the JOURNAL OF PHARMACEUTICAL SCIENCESAdvances in Medicinal Chemistry continues to provide timely and critical reviews of important topics in medicinal chemistry together with an emphasis on emerging topics in the biological sciences which are expected to provide the basis for entirely new future therapies      *RNA Processing* Steve J. Higgins,B. D. Hames,1994 This two volume set provides detailed practical guidance on all major aspects of RNA processing Each procedure is clearly explained so that the reader can follow all of the key stages of a successful experimental investigation

**RNA Modification** ,2007-09-13 The presence of modified nucleotides in cellular RNAs has been known for decades and over 100 distinct RNA modifications have been characterized to date While the exact role of many of these modifications is still unclear many are highly conserved across evolution and most contribute to the overall fitness of the organism In recent years new methods and bioinformatics approaches have been developed for the dissection of modification pathways and functions These methods intersect a number of related fields ranging from RNA processing to comparative genomics and systems biology In addition many of the techniques described in this volume have broad applicability particularly in regards to the isolation characterization and reconstitution of ribonucleoprotein complexes expanding the experimental repertoire available to all RNA researchers      *Gene Therapy* ,1997-08-12 Each volume of Advances in Pharmacology provides a rich

collection of reviews on timely topics Emphasis is placed on the molecular bases of drug action both applied and experimental Volume 40 GeneTherapy features important new research on gene transfers and therapy in the herpes simplex virus anti tumor immunity steroid receptors cystic fibroses and more Key Features It provides an excellent overview on a series of topics on gene therapy for the serious investigator

**Handbook of RNA Biochemistry** Roland K. Hartmann,Albrecht Bindereif,Astrid Schøn,Eric Westhof,2015-10-06 The second edition of a highly acclaimed handbook and ready reference Unmatched in its breadth and quality around 100 specialists from all over the world share their up to date expertise and experiences including hundreds of protocols complete with explanations and hitherto unpublished troubleshooting hints They cover all modern techniques for the handling analysis and modification of RNAs and their complexes with proteins Throughout they bear the practising bench scientist in mind providing quick and reliable access to a plethora of solutions for practical questions of RNA research ranging from simple to highly complex This broad scope allows the treatment of specialized methods side by side with basic biochemical techniques making the book a real treasure trove for every researcher experimenting with RNA

**Hepatitis Delta Virus** John L. Casey,2006-09-02 Hepatitis delta virus which causes severe acute and chronic liver disease was discovered following the detection of a novel antigen antibody system in hepatitis B virus carriers Currently HDV is classified as a subviral satellite of hepatitis B virus However unlike other satellite viruses the dependence of HDV on HBV is limited solely to the provision of an envelope of hepatitis B surface antigen for virus assembly Research into the molecular virology of the HDV life cycle has revealed a fascinating collection of biology These insights are now beginning to be translated into new potential treatment strategies There are currently still an estimated 15 million HDV carriers worldwide

**Cell Engineering** Mohamed Al-Rubeai,2007-11-23 Integrating advances in molecular biology into bioprocesses presents a continuous challenge to scientists and bioengineers This series is conceived to help meet this challenge It examines and assesses the feasibility of new approaches for the modification of cellular function such as gene expression protein processing secretion glycosylation immortalisation proliferation and apoptosis as well as the systematic study of the metabolic genotype phenotype relationship The series provides detailed coverage of the methodology for improving cellular properties of cells used in the production of biopharmaceuticals gene and cell therapies and tissue engineering It also seeks to explain the cellular mechanisms underlying in vitro physiological activity and productivity This volume which is based on presentations at the European Workshop on Animal Cell Engineering held in Costa Brava Spain contains a collection of chapters relating to cellular function and modification by leading authorities in several different areas of basic research and the biopharmaceutical industry

**Index Medicus** ,2004 Vols for 1963 include as pt 2 of the Jan issue Medical subject headings

*Biomedical Polymers and Polymer Therapeutics* Emo Chiellini,Junzo Sunamoto,Claudio Migliaresi,Raphael M. Ottenbrite,Daniel Cohn,2007-05-08 Proceedings of the Third International Symposium on Frontiers in Biomedical Polymers including Polymer Therapeutics From Laboratory to Clinical Practice held May 23 27 1999 in Shiga

Japan This book focuses on the progress and unique discoveries in the interdisciplinary scientific and technological area of biomedical application of polymers The topics include polymeric materials for biomedical and pharmaceutical applications as well as polymeric materials in therapeutics



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