

# RNA-BASED THERAPIES

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- 1 INTRODUCTION: RNA, THE SUPERLATIVE BIOMOLECULE  
From the Isolation of Nuclein to the Chemical and Structural  
Characterization of Nuclei Acids  
Natural Functions of RNA in Cells  
Potentially Therapeutic Features of RNA  
Advantages of RNA-Based Therapies  
Natural and Modified RNA
- 2 DRUGS BASED ON THE CAPACITY OF RNA TO FORM  
DEFINED THREE-DIMENSIONAL STRUCTURES: APTAMERS  
Definition of Aptamers  
RNA Mimetics Suitable for Aptamer Function  
Preclinical and Clinical Utilization of Aptamers  
Lead Company: Eyetech Pharmaceutical, Inc.
- 3 SYNTHETIC RNA FOR GENE REGULATION: ANTISENSE, RIBOZYMES,  
AND SIRNA  
Antisense and Ribozymes  
Small Inhibitory RNA (siRNA)  
Delivery of RNA Molecules for Suppression of Gene Expression
- 4 RNA FOR PROTEIN PRODUCTION: mRNA  
Definition of mRNA  
RNA Mimetics Suitable for mRNA Function  
Preclinical and Clinical Utilization of mRNA  
Lead Company: MERIX Biosciences
- 5 RNA FOR IMMUNOSTIMULATION: DOUBLE-STRANDED  
AND STABILIZED RNA  
Definition of Immunostimulating RNAs  
Preclinical and Clinical Utilization of Immunostimulating RNA  
Lead Company: Hemispherx Biopharma, Inc.