+44 20 8123 2220
info@marketpublishers.com

RNA-Targeted Small Molecules 2019: a landscape analysis of companies, technologies, targets, investors and partners from an industry perspective

https://marketpublishers.com/r/R147E2741B4EN.html
Date: September 2019
Pages: 223
Price: US$ 2,350.00 (Single User License)
ID: R147E2741B4EN

Abstracts

RNA-Targeted Small Molecules 2019: a landscape analysis of companies, technologies, targets, investors and partners from an industry perspective

This report provides you with a landscape description and analysis of discovery and development of small molecules against RNA as a drug target from an industry perspective as of September 2019.

The report brings you up-to-date with information about and analysis of

Approaches to target RNA with small molecules: splicing, translation, epitranscriptomics and direct RNA targeting;

Stakeholders in the field: technology and major pharmaceutical companies, investors and CROs;

Technologies of RNA biology and small molecule drug discovery in a repeatable and scalable way;

Preclinical and **clinical experience** with selected RNA-targeted small molecules;

Targets and **indications** selected for RNA-targeted small molecule drug discovery;

Financing situation of technology companies and key **investors** in the field

+44 20 8123 2220
info@marketpublishers.com

Partnering deals with financial terms;

Comparative assessment of technology companies.

Since 2017, **nearly US$ 1 bln** has been raised by start-up companies targeting RNA with small molecules in financing rounds and from partnering deals. This huge amount of money highlights the tremendous interest from investors and major pharmaceutical companies and the opportunities they recognize in these new approaches to target RNA with small molecules.

Nearly all small molecule-based drugs in clinical use target proteins. It is estimated that about 20,000 human proteins are expressed by the human genome and 10-15% are thought to be disease-related. However, many of them are considered as undruggable for various reasons, e.g. because they lack a distinctive motif for small molecule binding.

Originally thought to be merely a conduit for moving information encoded in the nuclear genome to the protein translational machinery in the cytoplasm via the canonical DNA-RNA-protein pathway, RNA is now increasingly known to have multiple roles, both coding and non-coding and to take myriad forms. Besides messenger RNAs (mRNAs) which encode proteins, and the ribosomal RNAs (rRNAs) involved in translating them, understanding continues to grow of the multitude of non-coding RNA (ncRNA) molecules, such as microRNA (miRNA), piwiRNA, long non-coding RNA (lncRNA), antisense RNA, short hairpin RNA and circular RNA. About 75% of the human genome is transcribed into RNA, yet only 1-2% encodes proteins.

The recent advancement in the knowledge about diversity, structural and functional information related to RNAs has put them in the lime light as a drug target. RNA has an important role in the transcription regulation, regulation of the translation, catalysis, protein function, protein transport, peptide bond formation and RNA splicing. New findings have identified RNA as a potential target in multitude of diseases including bacterial/viral infections and cancer. Just like proteins, RNAs can form well-defined tertiary structures, such as double helices, hairpins, bulges, and pseudo-knots.

With the ability to target RNA, the potential pool of drug targets would dramatically expand.

+44 20 8123 2220
info@marketpublishers.com

Strong proof-of-principle for RNA-targeted drugs has been provided by antisense oligonucleotides and synthetic RNAs that e.g. redirect the cellular RNA interference (RNAi) machinery. In addition to antisense and RNAi, which includes short interfering RNA (siRNA) and miRNA, other RNA therapeutics include mRNA, self-amplifying mRNA (samMRNA) and small activating RNA (saRNA).

However, nucleic acid-based therapeutic approaches involve large, often highly charged molecules with the associated delivery challenges, e.g. they do not pass the blood-brain barrier to to reach the brain or spinal cord, and have some toxicity issues (e.g. platelet count).

Another strategy to target RNA involves using small molecules as modulators of RNA. Small molecules are one of the most recent emerging RNA-focused therapeutics. They have several advantages over RNA molecules, including oral administration, easier entry into cells and better stability.

Several approaches are pursued to target RNA with small molecules:

mRNA Translation Regulation: Regulation of gene expression at the level of mRNA translation is a fundamental mechanism for moderating cellular events. The translation of single specific mRNAs, subsets, or even a majority of the mRNAs in a cell, is controlled almost exclusively through a multitude of interactions that occur between RNA-binding proteins and regulatory elements embedded throughout the mRNA.

RNA Splicing Modification; Post-transcriptional modification or co-transcriptional modification is a set of biological processes common to most eukaryotic cells by which an RNA primary transcript is chemically altered following transcription from a gene to produce a mature, functional RNA molecule that can then leave the nucleus and perform any of a variety of different functions in the cell. Three major steps significantly modify the chemical structure of the RNA molecule: the addition of a 5' cap, the addition of a 3' polyadenylated tail, and **RNA splicing**.

Direct RNA Targeting: RNA can form complex three-dimensional structures through canonical Watson-Crick base pairing and complex tertiary interactions that are mediated by non-canonical bonds. Such structures can be as intricate and stable as those formed by proteins and can recognize small-molecule ligands, other nucleic acids, or proteins with high affinity and specificity. Modern molecular techniques provide in-depth insides to the RNA structure and function. X-ray crystallography, nuclear magnetic resonance, and cryo-electron microscopy yielded a solid foundation for understanding the chemical

+44 20 8123 2220
info@marketpublishers.com

and structural basis of RNA functions at atomic resolution. The development of RNA-centric deep-sequencing probing techniques opened up the possibility for the global assessment of RNA structures at a single nucleotide resolution, and in various biological contexts.

Indirect RNA Targeting – Epitranscriptomics: The epitranscriptome includes all the biochemical modifications of the RNA (= the transcriptome) within a cell. Epitranscriptomics involves all functionally relevant changes to the transcriptome that do not involve a change in the ribonucleotide sequence. Thus, RNAs are indirectly targeted via the proteins they interact with.

What will you find in the report?

Profiles of technology companies active in the field;

Description of Big Pharma’s role in the field (in-house R&D, partnering and investing);

Comprehensive description and analysis of emerging technologies with a directed, intentional approach to drug-RNA structure;

Preclinical and clinical profiles of RNA-targeted small molecules in all phases of development;

Technology selection and preferences of major pharma;

Key characteristics of technologies;

Target selection and competition of drug candidates;

Description and analysis of financing rounds (capital raised, investors);

Economic terms of collaboration and licensing deals;

Sources of financing.

Who will benefit from the report?

+44 20 8123 2220
info@marketpublishers.com

Venture capital, private equity and investment managers;

Managers of Big Pharma venture capital firms;

Financial analysts;

Business development and licensing (BDL) specialists;

CEO, COO and managing directors;

Corporate strategy analysts and managers;

Chief Technology Officer;

R&D Portfolio, Technology and Strategy Management;

Clinical and preclinical development specialists.

+44 20 8123 2220
info@marketpublishers.com

Contents

+44 20 8123 2220
info@marketpublishers.com

5 DIRECT RNA TARGETING BY SMALL MOLECULES

6 INDIRECT RNA-TARGETED (EPITRANSCRIPTOMIC) SMALL MOLECULES

+44 20 8123 2220
info@marketpublishers.com

+44 20 8123 2220
info@marketpublishers.com

List Of Tables

LIST OF TABLES

+44 20 8123 2220
info@marketpublishers.com

COMPANIES MENTIONED

AC Immune
Accent Therapeutics
Anima Biotech
Arrakis Pharmaceuticals
Biogen
Boehringer Ingelheim
Bristol-Myers Squibb
Celgene
eFFECTOR Therapeutics
Eli Lilly
Eloxx Pharmaceuticals
Epics Therapeutics
Expansion Therapeutics
Gotham Therapeutics
H3 Biomedicine
ImStar Therapeutics
Merck
Novartis
Novation Pharmaceuticals
Nymirum
Panorama Medicine
Pfizer
PTC Therapeutics
Ribometrix
Roche (Genentech)
Saverna Therapeutics
Skyhawk Therapeutics
STORM Therapeutics
Takeda Pharmaceutical Co
TargetRNA
Twentyeight-Seven Therapeutics
UCB (The RNA Medicines Company)

+44 20 8123 2220
info@marketpublishers.com

I would like to order

Product name: RNA-Targeted Small Molecules 2019: a landscape analysis of companies, technologies, targets, investors and partners from an industry perspective

Product link: https://marketpublishers.com/r/R147E2741B4EN.html

Price: US$ 2,350.00 (Single User License / Electronic Delivery)

If you want to order Corporate License or Hard Copy, please, contact our Customer Service:
info@marketpublishers.com

Payment

To pay by Credit Card (Visa, MasterCard, American Express, PayPal), please, click button on product page https://marketpublishers.com/r/R147E2741B4EN.html

To pay by Wire Transfer, please, fill in your contact details in the form below:

First name:
Last name:
Email:
Company:
Address:
City:
Zip code:
Country:
Tel:
Fax:
Your message:

**All fields are required

Custumer signature _____

Please, note that by ordering from marketpublishers.com you are agreeing to our Terms & Conditions at https://marketpublishers.com/docs/terms.html

To place an order via fax simply print this form, fill in the information below and fax the completed form to +44 20 7900 3970

+44 20 8123 2220
info@marketpublishers.com