

RNA Targeting Small Molecule Drug Discovery Market Size, Share and Growth Outlook 2026: By Offering, By Indication, By Application, By End User, Companies to 2034

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Abstracts

The global RNA Targeting Small Molecule Drug Discovery Market size is forecast to increase from \$2.61 Billion in 2026 to \$11.73 Billion in 2034 at a CAGR of 20.67% between 2026 and 2034.

The RNA Targeting Small Molecule Drug Discovery market report provides detailed analysis and outlook of RNA Targeting Small Molecule Drug Discovery segments including By Offering (mRNA Translation Modulators, RNA Splicing Modification, Direct RNA Targeting, Other Offerings), By Indication (Cancer, Infectious Diseases, Metabolic Diseases, Neurological Diseases, Other Indications), By Application (Drug Discovery, Oncology Research, Disease Identification), By End User (Pharmaceutical and Biopharmaceutical Companies, Academic and Research Institutes, Hospitals, Research Laboratories) across global and regional markets. Further, analysis and outlook across 21 countries in North America, Europe, Asia Pacific, Middle East, Africa, and South America are provided in the study.

RNA Targeting Small Molecule Drug Discovery Industry Overview

Small Molecule RNA Modulation Is Emerging as a New Frontier in Precision Medicine

The RNA targeting small molecule drug discovery industry is rapidly evolving as researchers increasingly recognize RNA as a highly attractive therapeutic target beyond conventional protein-based drug discovery approaches. Advances in RNA structural biology, transcriptomics, medicinal chemistry, and computational drug design have

enabled the identification of druggable RNA structures involved in cancer, rare genetic diseases, and neurological disorders. Unlike traditional therapies that indirectly influence RNA expression, RNA-targeted small molecules are designed to bind directly to disease-associated RNA transcripts or modify RNA processing pathways, creating opportunities for highly selective and disease-modifying interventions. This emerging therapeutic field is attracting significant investment as companies seek to expand the scope of precision medicine beyond conventional target classes.

RNA Degradation and RNA Modification Pathways Are Creating Novel Oncology Opportunities

One of the most active areas of development within RNA-targeted drug discovery involves therapies designed to selectively degrade oncogenic RNA transcripts or alter RNA regulatory mechanisms that drive cancer progression. Supporting this trend, Remix Therapeutics reported positive clinical findings from its Phase 1/2 ARIA study evaluating REM-422, a first-in-class small molecule designed to selectively degrade MYB messenger RNA in patients with adenoid cystic carcinoma. The program demonstrated substantial clinical activity, including durable responses and high disease control rates, highlighting the therapeutic potential of targeted RNA degradation strategies. Similarly, STORM Therapeutics advanced the clinical development of STC-15, a first-in-class METTL3 inhibitor that modifies messenger RNA methylation pathways to reprogram malignant cellular behavior. The company's recent financing activities and progression into Phase 2 clinical evaluation underscore growing industry confidence in RNA epigenetic modulation as a viable therapeutic approach.

Rational RNA Targeting Platforms Are Expanding Applications Beyond Oncology

The RNA targeting small molecule field is also expanding into rare genetic and neuromuscular diseases through advances in rational molecular design and direct RNA binding technologies. Companies are increasingly developing therapeutics capable of selectively interacting with pathogenic RNA structures that have historically been considered undruggable. Reflecting this trend, Arrakis Therapeutics presented preclinical evidence supporting its rationally designed RNA-targeted small molecule platform, demonstrating direct interaction with toxic DMPK expansion transcripts responsible for Myotonic Dystrophy Type 1. By targeting the underlying molecular drivers of disease rather than downstream symptoms, these approaches represent a significant advancement in precision medicine. Collectively, these developments highlight the industry's transition toward highly selective, mechanistically driven RNA-targeting platforms capable of addressing a broad range of oncology, rare disease, and

genetic disorders.

RNA Targeting Small Molecule Drug Discovery Market Trends, Growth Drivers, Competitive Landscape, and Future Opportunities

The global RNA Targeting Small Molecule Drug Discovery market is witnessing increasing investments in innovation, product development, digital transformation, artificial intelligence integration, healthcare infrastructure expansion, and strategic partnerships across developed and emerging economies. Key Companies in the industry include- F. Hoffmann-La Roche Ltd, Novartis AG, AstraZeneca PLC, Bristol-Myers Squibb Company, PTC Therapeutics, Inc., Ionis Pharmaceuticals, Inc., Arrakis Therapeutics, Anima Biotech Inc., Ribometrix, Skyhawk Therapeutics. The RNA Targeting Small Molecule Drug Discovery market is expected to remain one of the most closely watched segments in the global healthcare industry, with companies focusing on niche market segments. As healthcare systems across the US, Europe, Asia-Pacific, Latin America, and Middle East & Africa continue to prioritize efficiency, access, and innovation, the RNA Targeting Small Molecule Drug Discovery industry outlook remains shaped by rising healthcare expenditure, demographic change, digital transformation, and product innovation.

The report provides detailed market analysis including-

- Growth RNA Targeting Small Molecule Drug Discovery Market size outlook across 3 scenarios- High growth, reference, and Low growth cases

- Market Trends, Drivers, Potential Opportunities, and Challenges faced by RNA Targeting Small Molecule Drug Discovery companies

- Porter's Five forces analysis- Bargaining power of buyers and sellers, Threat of Substitutes and new entrants, and Intensity of competitive rivalry

- Detailed SWOT Analysis of global and regional RNA Targeting Small Molecule Drug Discovery markets

- Competitive analysis including business description, product analysis, and financial profiles

- Key country specific analysis detailing key factors shaping the short-term and long-term outlook

Recent industry developments and news including mergers, acquisitions, product launches, expansions, and company announcements

RNA Targeting Small Molecule Drug Discovery Market Competitive Benchmarking and Company Analysis

Leading companies in RNA Targeting Small Molecule Drug Discovery industry include- F. Hoffmann-La Roche Ltd, Novartis AG, AstraZeneca PLC, Bristol-Myers Squibb Company, PTC Therapeutics, Inc., Ionis Pharmaceuticals, Inc., Arrakis Therapeutics, Anima Biotech Inc., Ribometrix, Skyhawk Therapeutics. The RNA Targeting Small Molecule Drug Discovery market remains moderately to highly fragmented, with competition expected to intensify as companies accelerate investments in innovation, geographic expansion, strategic partnerships, and portfolio diversification through 2034. In developed markets such as the United States, Germany, France, the United Kingdom, and Canada, competition is increasingly centered on innovation, reimbursement positioning, and value-based healthcare solutions. Meanwhile, emerging markets including China, India, Brazil, and countries across the Middle East and Africa continue to present significant opportunities for expansion due to rising healthcare expenditure, growing patient populations, and increasing access to healthcare services.

What to expect in US RNA Targeting Small Molecule Drug Discovery Markets in 2026 and beyond- Market Size, Share, Growth Rate, and Forecast to 2034

The US healthcare expenditure is forecast to reach \$8.2 Trillion in 2034 from \$5.5 Trillion in 2026 based on the National Health Expenditure Accounts (NHEA) data. With an aging population, rising chronic disease burden, and increasing migration toward minimally invasive and outpatient care, the RNA Targeting Small Molecule Drug Discovery market remains one of the strongest-performing segments in the country.

The US RNA Targeting Small Molecule Drug Discovery Companies are opting new business models, optimized pricing models, industry partnerships, and AI-enabled back end transformations to enhance efficiency and cost management. The US RNA Targeting Small Molecule Drug Discovery market faces successive waves of challenging trends, with strong opportunities across select segments. The CMS plan to implement Medicaid from 2027 is driving states to build eligibility verification systems throughout 2026. Looking ahead to 2034, we anticipate stronger results underpinned by opportunities exist across RNA Targeting Small Molecule Drug Discovery industry. On

the medical device front, over 7,000 device manufacturers continue to gain from increasing demand from demand for implantable devices, surgical instruments, monitoring equipment, and diagnostic systems.

Canada- Proximity to the US and healthcare similarities to EU5 countries fuel sales of Canadian RNA Targeting Small Molecule Drug Discovery markets

Canada's strong RNA Targeting Small Molecule Drug Discovery sales performance is underpinned by an aging population and a well-developed healthcare infrastructure. Steady growth in new brand spending in rural and urban locations fuel the long-term prospects of small and medium-sized enterprises across medical, diagnostic, and therapeutic devices. The Canadian RNA Targeting Small Molecule Drug Discovery market presents significant opportunities for U.S. exporters of medical devices, with the U.S. being Canada's largest trading partner for this sector. Potential advantages including specialized materials, advanced manufacturing techniques, and digital technologies support the launch of new products in the country.

Germany RNA Targeting Small Molecule Drug Discovery Trends and Perspectives to 2034- Financial sustainability, hospital restructuring, demographic pressures, and digitization of care delivery continue to shape the German healthcare industry.

Germany continues to remain the largest RNA Targeting Small Molecule Drug Discovery market in Europe, driven by over €600 Billion healthcare expenditure, €12 Billion medical device R&D expenditure, statutory health insurance system covering 90% German population, nationwide rollout of the electronic patient record (ePA), and large-volume of RNA Targeting Small Molecule Drug Discovery population. In particular, Research and development in Germany fuels the commercialization of cutting-edge technologies. Companies across the Germany RNA Targeting Small Molecule Drug Discovery industry value chain are focusing on both domestic markets and exports. The country is also driving digital adoption with the Hospital Future Act driving hospitals to upgrade their information systems by 2027. Over the forecast period, aging population, rising healthcare costs, and increasing procedural volumes drive the RNA Targeting Small Molecule Drug Discovery market outlook.

France Market Size, Growth Rate, and Forecast Analysis to 2034- Universal healthcare system, high public healthcare expenditure, and strong government support RNA Targeting Small Molecule Drug Discovery sales through 2034

France RNA Targeting Small Molecule Drug Discovery companies are emphasizing on

opportunities for rapid, at-scale innovation to boost profitability over the long-term. The country's National Health Insurance spending target (ONDAM) estimates 3.7% growth in the country's healthcare expenditure. Over the forecast period, expenditure control measures, chronic disease management initiatives, workforce reforms, and efforts to improve system efficiency drive the long-term prospects.

The biggest 2026 policy frame is the PLFSS 2026. The law sets the Maladie branch spending target at €271.4 billion for 2026 and fixes the ONDAM at €117.5 billion for city care, €112.8 billion for health establishments, and €18.3 billion for elderly-care establishments and services. France's market is also being pulled by demographics. INSEE estimates that on 1 January 2026 France had 69.1 million inhabitants, with 22% aged 65 or over. INSEE also reported that 2025 births were 645,000 and deaths were 651,000, producing a negative natural balance of about 6,000 for the first time since the end of the Second World War.

UK RNA Targeting Small Molecule Drug Discovery Market Size, Share, and Growth Projections to 2034- Rapid growth driven by new and existing brands across the industry value chain

Small high-need consumer segments remain key priority of RNA Targeting Small Molecule Drug Discovery distributors in the UK industry. Continuous launch of new products coupled with high expenditures support the market outlook. The UK Government financing remains the dominant funding source at 81.3% of total healthcare expenditure, or ?280 billion in 2025. According to the ONS, total healthcare spending grew 7.7% nominally and 3.9% in real terms from 2024 to 2025. Similarly, out-of-pocket spending was ?49 billion (14.1%) and voluntary health insurance was ?9.5 billion (2.8%). The market is driven by rapid digital adoption with NHS England's plan to give more than 500,000 staff access to new AI tools.

China RNA Targeting Small Molecule Drug Discovery Market Growth Drivers, Revenue Trends, and Forecast- Medical insurance coverage is rapidly expanding over the past few years

China RNA Targeting Small Molecule Drug Discovery market is undergoing a structural shift from hospital-centric care toward a more integrated system emphasizing primary care, outpatient services, and long-term care. Chinese local players are emerging as a strong pillar of RNA Targeting Small Molecule Drug Discovery industry, offering opportunities for both competition and partnership. Over the forecast period, new and innovative product launches remain key elements driving market outlook. China's

healthcare industry is increasingly centered on expanding healthcare capacity, improving access to advanced treatments, and reducing dependence on imported technologies.

The National Healthcare Security Administration reported that by end-2024, China's basic medical insurance covered 1.32662 billion people and the coverage rate was 95%. Regional disparities in consumer spending trends continue to become more pronounced in the Chinese RNA Targeting Small Molecule Drug Discovery industry. Over the forecast period, demand will keep shifting toward geriatrics, chronic disease management, rehabilitation, long-term care, and outpatient care, while pricing pressure will remain intense in drugs and consumables because reimbursement.

India RNA Targeting Small Molecule Drug Discovery Market Landscape: Current Size and Long-Term Growth Outlook - Increased pricing pressures in US market is encouraging domestic vendors to expand across India

Indian RNA Targeting Small Molecule Drug Discovery market is witnessing the rapid emergence of an ecosystem that brings together diverse companies across the industry value chain. Further, large-scale healthcare public and private investments and a steady growth in chronic conditions is driving sales of pharmaceuticals and medical devices. Further, non-retail channel is experiencing volume decrease and patients are migrating to the retail. Indian medical device firms are also combining precision engineering with lower labor costs to make world-class diagnostics, robotics, and critical care devices.

Brazil RNA Targeting Small Molecule Drug Discovery market remains price-driven, with products domestically manufactured and accessibility offering potential opportunities

Healthcare expenditure in Brazil exceeds 10% of GDP, with the country among the highest healthcare spenders in Latin America. ANS reported 53.2 million medical-plan beneficiaries in December 2025, while IBGE projects a steady rise in older-age cohorts, with people aged 60+ already representing about 23% of the population. The price sensitive market access is broad through the public system, private coverage adds a sizeable premium layer, and reimbursement, procurement, and hospital efficiency remain key buying drivers.

Middle East and Africa RNA Targeting Small Molecule Drug Discovery Industry Trends and Perspectives to 2034

According to the World Bank, the Middle East and North Africa population exceeds 500

million, while Sub-Saharan Africa's population exceeds 1.2 billion, making the broader MEA region one of the fastest-growing healthcare demand centers globally. The GCC countries including Saudi Arabia, United Arab Emirates, Qatar, and Kuwait continue to account for a disproportionately large share of regional healthcare spending. Government-led programs such as Saudi Arabia's Vision 2030 are accelerating investments in hospital infrastructure, private-sector participation, medical technology adoption, and healthcare digitalization. On the other hand, South Africa, Egypt, Nigeria, and Kenya remain key healthcare markets due to their large populations, expanding private healthcare sectors, and growing investments in healthcare delivery systems.

RNA Targeting Small Molecule Drug Discovery Market Segmentation

By Offering

mRNA Translation Modulators

RNA Splicing Modification

Direct RNA Targeting

Other Offerings

By Indication

Cancer

Infectious Diseases

Metabolic Diseases

Neurological Diseases

Other Indications

By Application

Drug Discovery

Oncology Research

Disease Identification

By End User

Pharmaceutical and Biopharmaceutical Companies

Academic and Research Institutes

Hospitals

Research Laboratories

Leading Companies in RNA Targeting Small Molecule Drug Discovery Industry (2026)

F. Hoffmann-La Roche Ltd

Novartis AG

AstraZeneca PLC

Bristol-Myers Squibb Company

PTC Therapeutics, Inc.

Ionis Pharmaceuticals, Inc.

Arrakis Therapeutics

Anima Biotech Inc.

Ribometrix

Skyhawk Therapeutics

Countries Included

North America- US, Canada, Mexico

Europe- Germany, France, UK, Spain, Italy, Nordics, Others

Asia Pacific- China, India, Japan, South Korea, Australia, Southeast Asia, Others

Latin America- Brazil, Argentina, Others

Middle East and Africa- Saudi Arabia, UAE, Other Middle East, South Africa, Other Africa

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 - Infectious Diseases
 - Metabolic Diseases
 - Neurological Diseases
 - Other Indications
- By Application
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AstraZeneca PLC

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PTC Therapeutics, Inc.

Ionis Pharmaceuticals, Inc.

Arrakis Therapeutics

Anima Biotech Inc.

Ribometrix

Skyhawk Therapeutics

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