

RNA Targeting Small Molecules Therapeutics Market In 2029

The Business Research Company's RNA Targeting Small Molecules Therapeutics Global Market Report 2025 – Market Size, Trends, And Forecast 2025-2034

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to Surpass \$4 billion in 2029. In comparison, the Small Molecule Active Pharmaceutical Ingredient market, which is considered as its parent market, is expected to be approximately \$279 billion by 2029, with RNA Targeting Small Molecules Therapeutics to represent around 1% of the parent market. Within the broader Pharmaceuticals industry, which is expected to be \$2,355 billion by 2029, the RNA Targeting Small Molecules Therapeutics market is estimated to account for nearly 0.2% of the total market value.

Which Will Be the Biggest Region in the RNA Targeting Small Molecules Therapeutics Market in 2029

North America will be the largest region in the RNA targeting small molecules therapeutics market in 2029, valued at \$1,763 million. The market is expected to grow from \$1,221 million in 2024 at a compound annual growth rate (CAGR) of 8%. The strong growth can be attributed to the increasing incidence of genetic disorders and strategic collaboration and partnerships.

Which Will Be The Largest Country In The Global RNA Targeting Small Molecules Therapeutics Market In 2029?

The USA will be the largest country in the RNA targeting small molecules therapeutics market in 2029, valued at \$1,573 million. The market is expected to grow from \$1,091 million in 2024 at a compound annual growth rate (CAGR) of 8%. The strong growth can be attributed to the increasing incidence of genetic disorders and rising cancer prevalence.

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What will be Largest Segment in the RNA Targeting Small Molecules Therapeutics Market in

2029?

The RNA targeting small molecules therapeutics market is segmented by offering into mRNA (messenger ribonucleic acid) translation modulators, RNA (ribonucleic acid) splicing modification, direct RNA (ribonucleic acid) targeting and other offerings. The RNA (ribonucleic acid) splicing modification market will be the largest segment of the RNA targeting small molecules therapeutics market segmented by offering, accounting for 65% or \$2,606 million of the total in 2029. The RNA (ribonucleic acid) splicing modification market will be supported by increasing understanding of splicing errors as drivers of genetic disorders, rising prevalence of cancers and neuromuscular diseases linked to aberrant splicing, demand for therapies correcting splicing pathways, growing development of small molecules capable of modulating spliceosome activity, investment in precision medicine targeting RNA isoforms and collaborations between academia and industry to develop splicing-specific therapeutic candidates.

The RNA targeting small molecules therapeutics market is segmented by therapeutic indication into lung fibrosis, cancer, neurodegenerative diseases, autoimmune, inflammatory and other therapeutic indications. The neurodegenerative diseases market will be the largest segment of the RNA targeting small molecules therapeutics market segmented by therapeutic indication, accounting for 65% or \$2,588 million of the total in 2029. The neurodegenerative diseases market will be supported by the rising prevalence of conditions such as ALS, Alzheimer's and Parkinson's, increasing evidence linking RNA splicing errors to neurodegeneration, demand for disease-modifying therapies beyond symptomatic treatments, growth of small molecule candidates targeting toxic RNA repeats, high investment in neuroscience drug discovery and collaborations focusing on correcting RNA dysfunctions in neurons.

The RNA targeting small molecules therapeutics market is segmented by application into drug discovery, oncology research and disease identification. The drug discovery market will be the largest segment of the RNA targeting small molecules therapeutics market segmented by application, accounting for 56% or \$2,247 million of the total in 2029. The drug discovery market will be supported by the growing use of RNA structures as new druggable targets, increasing adoption of high-throughput RNA screening technologies, expansion of RNA-focused chemical libraries, demand for small molecules to complement RNA-based biologics, rising use of computational tools for RNA-ligand prediction and growing pharmaceutical investment in early-stage RNA-targeted drug pipelines.

The RNA targeting small molecules therapeutics market is segmented by end user into hospitals, research laboratories, pharmaceutical and biotechnology companies and other end-users. The pharmaceutical and biotechnology companies' market will be the largest segment of the RNA targeting small molecules therapeutics market segmented by end user, accounting for 58% or \$2,301 million of the total in 2029. The pharmaceutical and biotechnology companies' market will be supported by the growing investment in RNA drug pipelines, rising number of strategic partnerships for RNA therapeutic development, demand for differentiated small molecule portfolios alongside biologics, expansion of licensing deals for RNA-targeting technologies, increasing focus on first-in-class RNA drugs and strong M&A activity to acquire RNA-specialized

startups.

What is the expected CAGR for the RNA Targeting Small Molecules Therapeutics Market leading up to 2029?

The expected CAGR for the RNA targeting small molecules therapeutics market leading up to 2029 is 8%.

What Will Be The Growth Driving Factors In The Global RNA Targeting Small Molecules Therapeutics Market In The Forecast Period?

The rapid growth of the global RNA targeting small molecules therapeutics market leading up to 2029 will be driven by the following key factors that are expected to reshape drug discovery, personalized medicine, and advanced therapeutic development worldwide.

Government Initiatives For Research And Development In Healthcare - The government initiatives for research and development in healthcare will become a key driver of growth in the RNA targeting small molecules therapeutics market by 2029. Government initiatives in healthcare research and development are expected to drive the growth of the RNA-targeting small molecule therapeutics market by offering financial support, tax incentives and research grants to biotechnology and pharmaceutical companies. These measures help ease the high costs of drug discovery and clinical trials, enabling faster innovation. Public-private partnerships further support the development of advanced therapies and broader access to new technologies. In addition, favorable regulatory policies streamline approval processes, encouraging greater investment in novel RNA-targeting treatments. As a result, the government initiatives for research and development in healthcare is anticipated to contributing to a 1.0% annual growth in the market.

Venture Capital And Pharma Investment In RNA - The venture capital and pharma investment in RNA will emerge as a major factor driving the expansion of the RNA targeting small molecules therapeutics market by 2029. These funding sources provide the necessary capital for research, preclinical studies and clinical trials, which are otherwise highly resource intensive. Strategic investments from pharmaceutical companies also bring technical expertise, regulatory support and global market access, accelerating product development. Venture capital fosters innovation by enabling startups to explore advanced technologies and high-risk projects. Such financial backing reduces barriers to entry, strengthens the competitive landscape and expedites commercialization. Consequently, venture capital and pharma investment in RNA capabilities is projected to contributing to a 0.8% annual growth in the market.

Increasing Incidence Of Genetic Disorders - The increasing incidence of genetic disorders will serve as a key growth catalyst for the RNA targeting small molecules therapeutics market by 2029. RNA-targeting therapies offer an innovative approach to addressing the underlying causes of genetic disorders by modulating gene expression or correcting defective RNA mechanisms. With the rising prevalence of genetic conditions, demand for advanced and targeted treatments is increasing. This trend presents significant opportunities for pharmaceutical and biotechnology

companies to accelerate research, expand clinical trial pipelines and introduce innovative RNA-based therapies to the market. Therefore, this increasing incidence of genetic disorders operations is projected to supporting to a 0.5% annual growth in the market.

Rising Prevalence Of Cancer - The rising prevalence of cancer will become a significant driver contributing to the growth of the RNA targeting small molecules therapeutics market by 2029. The growing prevalence of cancer is driving demand for innovative and effective treatment solutions, boosting interest in RNA-targeting small molecule therapeutics. By addressing cancer at the genetic and molecular level, these therapies are attracting increased research investments and accelerating clinical adoption, thereby supporting market expansion. Many types of cancer are linked to errors in Ribonucleic Acid (RNA) processes such as splicing, translation, or the activity of non-coding RNAs. With the rising number of cancer cases, there is an increasing need for small molecule therapeutics that specifically target these RNA-related abnormalities. Consequently, the rising prevalence of cancer strategies is projected to contributing to a 0.2% annual growth in the market.

Access the detailed RNA Targeting Small Molecules Therapeutics Market report here: <https://www.thebusinessresearchcompany.com/report/rna-targeting-small-molecules-therapeutics-global-market-report>

What Are The Key Growth Opportunities In The RNA Targeting Small Molecules Therapeutics Market in 2029?

The most significant growth opportunities are anticipated in the RNA-targeting small molecule therapeutics in pharmaceutical and biotechnology market, the RNA targeting and splicing modification therapeutics market, the RNA-targeting small molecule therapeutics for neurodegenerative diseases market, and the RNA-targeting small molecule drug discovery market. Collectively, these segments are projected to contribute over \$3 billion in market value by 2029, driven by breakthroughs in RNA biology, rising demand for precision therapeutics, and the expanding pipeline of small molecules designed to modulate RNA function. This surge reflects the growing shift toward innovative therapeutic modalities that enable targeted modulation of gene expression, offering new treatment pathways for complex and previously undruggable diseases. As advancements in structural biology, high-throughput screening, and AI-enabled drug discovery accelerate development timelines, these segments are poised to fuel transformative growth within the broader RNA-targeting therapeutics industry.

The RNA-targeting small molecule therapeutics in pharmaceutical and biotechnology market is projected to grow by \$764 million, the RNA targeting and splicing modification therapeutics market by \$753 million, the RNA-targeting small molecule therapeutics for neurodegenerative diseases market by \$735 million, and the RNA-targeting small molecule drug discovery market by \$682 million over the next five years from 2024 to 2029.

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