

## Spinal Muscular Atrophy Treatment Market Size, Share & Trends Analysis Report By Type (Type1, Type 2), By Treatment (Gene Therapy, Drug), By Drug (Spinraza, Zolgensma), By Route Of Administration, By Region, And Segment Forecasts, 2022 - 2030

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## Abstracts

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Spinal Muscular Atrophy Treatment Market Growth & Trends

The global spinal muscular atrophy treatment market size is expected to reach USD 18.0 billion by 2030, according to a new study by Grand View Research Inc. This is expected to expand at a CAGR of 18.6% from 2022 to 2030. This growth can be attributed to the increasing product approval and launches. For instance, in August 2020, the Food and Drug Administration approved Evrysdi (risdiplam) developed by Genentech, Inc., for the treatment of pediatric patients aged two months and older with spinal muscular atrophy (SMA).

In addition, in May 2020, Novartis AG received conditional approval to its SMA candidate Zolgensma (onasemnogene abeparvovec) from the European Commission (EC) to treat patients with SMA. It is only approved gene therapy in Europe. In March 2021, PTC Therapeutics, Inc., received marketing approval for Evrysdi (risdiplam) from the EMA for the treatment of patient with type1, 2, and 3 spinal muscular atrophy. Thus, increasing number of product approvals is expected to drive the spinal muscular atrophy treatment market.

Novartis AG is supporting SMA patients by offering medicine at free of cost through global Managed Access Program (MAP). This program provides 100 doses of



Zolgensma at free of charge to eligible SMA patients across continents including North America, South America, Asia, Australia, Europe, and Africa. Thus, presence of such supportive programs for Zolgensma strengthens promotional activities and also aid in generating revenue.

Global SMA newborn screening and easy access to diagnostic tools should be included in routine approach. This would ensure early diagnosis and timely access to treatment. For instance, in July 2021, a charlotte baby received a breakthrough gene therapy treatment for Spinal Muscular Atrophy (SMA) at UNC Medical Center in Chapel Hill, N.C., after diagnosis with spinal muscular atrophy through Early Check newborn screening program. Such screening programs are expected to boost early diagnosis and treatment of disease.

Moreover, there are several extensive research collaborations undertaken by pharmaceutical companies with research institutes for developing novel therapies for the treatment of spinal muscular atrophy patients. For instance, in March 2021, PTC Therapeutics, Inc., and The Spinal Muscular Atrophy Foundation entered into research collaboration to advance scientific research and developing new treatment to treat patients with SMA.

However, high costs associated with the treatment and low availability of participant for clinical trial purposes may restrain market growth over the forecast period. The high cost related to research and development of an orphan drug is a major factor of rising prices of product. For instance, the most commonly used drug Spinraza costs around USD 125,000 per injection and USD 750,000 annually. Furthermore, Zolgensma (AVXS-101) costs USD 2.1 million for one time treatment.

Spinal Muscular Atrophy Treatment Market Report Highlights

By type, the type-1 segment held the largest market share in 2021 due to higher prevalence and wide availability of products for the treatment of patients with type-1 spinal muscular atrophy

By treatment, the gene therapy segment is expected to be the fastest growing segment over the forecast period due to increase market penetration. Zolgensma is the only approved gene therapy in 37 countries to treat patient with SMA

By drug, the Spinraza segment dominated the market in 2021 due to presence



of supportive reimbursement policies and increasing awareness about products. Currently, Spinraza is applicable for full reimbursement in three European countries Italy, Norway, Netherlands

By route of administration, the injection segment dominated the market in 2021 due to high safety, efficacy, and tolerability associated with infusion drugs to SMA patients through this route of administration

Asia Pacific is expected to be the fastest growing region during the forecast period. The growth of region is attributable to the entry of new products into the region. For instance, in July 2021, F. Hoffmann-La Roche Ltd launched Evrysdi (risdiplam) for the treatment of patient with spinal muscular atrophy in India



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