

# The US Accounts for the Highest Number of Prevalent Cases of Spinal Muscular Atrophy (SMA), Finds New Report

*The US have highest number of SMA amongst the 7 major markets which include France, Germany, Italy, Spain, United Kingdom and Japan*

LEWES, DELAWARE, DELAWARE, UNITED STATES, May 22, 2018 /EINPresswire.com/ -- Spinal Muscular Atrophy [SMA] is a rare hereditary neuromuscular disorder caused by Bi-allelic mutation of survival motor neuron gene 1 (SMN1). In healthy people this gene produces a protein required by the muscle controlling nerves. In the deficiency of this protein these nerve cells cannot function properly which leads to fatal muscle weakness. It is one of the most common causes of infant death and mobility impairment; the severity of diseases depends on the number of backup gene SMN2 that varies from 1-4 in SMA patients. Thus, SMA can be categorized into four sub-types on the basis of severity; SMA type 1 is the most severe form with highest mortality rate whereas SMA type 4 is the mildest form with lowest impairment.

There are very scarce studies that provide sufficient epidemiology data for SMA disorder, as a large pool of population is still undiagnosed. As per the current statistics, the incidence of SMA is 1 per 5,000 to 10,000 live births and 1 in 50 people are carriers of SMA causing faulty genes. According to DelveInsight estimates, there were 38,900 diagnosed prevalent SMA cases in 2017 in the 7MM.

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As per our estimation, the 7 major market size for SMA was estimated to be 825 Million in 2017.”

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The US accounts for the highest number of prevalent cases of SMA amongst the 7 major markets (7MM). Since SMA is a low incidence neurological disorder its diagnosis was difficult in historic years but with advancement in precise diagnostic techniques (like genetic testing and carrier testing) and supportive USFDA policies, The US has a noticeable growth diagnosed cases of SMA in the last couple of years. Report forecast suggests the diagnosed prevalence of 15,000 cases of SMA in the U.S. in 2017.

SMA is the second most common fatal disease in the U.S. after cystic fibrosis, with highest mortality in Type-1 patients (Infants) i.e., <2 years of age. There had been no cure for this deadly disease, prior

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to the approval of “Spinraza”, in December 2016. Spinraza, second antisense oligonucleotide to ever receive USFDA approval is the first and only therapy to be launched for SMA that targets all 3 major types of SMA.

The drug has been considered as a ray of hope for both doctors as well as the parents of infants suffering from SMA. But, the drug comes with a hefty price tag of \$100,000 (20% discount) per injection, which leads to an annual cost of \$600,000 for the first year of treatment and then \$300,000 for the subsequent years. The developer company [Biogen, Inc.] is actively working along with the Government and SMA organizations, to come up with better reimbursement policies for Spinraza so that maximum patients could receive the benefit of drugs.



Despite of being the only therapy, Spinraza also has its own fair share of drawbacks and adverse events. For instance, the most common adverse reactions reported for Spinraza were upper respiratory infection, lower respiratory infection and constipation. Serious adverse reactions of atelectasis were more frequent in Spinraza-treated patients. Apart from these adverse effects, one of the major drawback for Spinraza is that it cannot be given to patients that are already on ventilation (as per the drug’s exclusion criteria).

The pharmaceutical companies, actively involved in the development of therapies that can help in treating SMA, are aware of the drawbacks and adverse events caused by the usage of Spinraza. They understand that there’s an urgent need for more and better treatment options for SMA. The emerging therapies that are expected to compete Spinraza include CK-2127107, RG7916, AVXS-101, Olesoxime and LMI070.

The launch of other better upcoming therapies and the drug’s patent expiry in 2023, shall negatively affect the market share of Spinraza, leading to slow & gradual decline in the revenue.

[Spinal Muscular Atrophy \(SMA\) - Market Insights, Epidemiology and Market](#) Forecast-2027 report delivers an in-depth understanding of the disease, historical & forecasted epidemiology as well as the market trends of Spinal Muscular Atrophy in the United States, EU5 (France, Germany, Italy, Spain and United Kingdom) and Japan.

The Report provides the current treatment practices, emerging drugs, market share of the individual therapies, current and forecasted market size of SMA from 2017 to 2027 segmented by seven major markets. The Report also covers current treatment practice/algorithm, market drivers, market barriers and unmet medical needs to find best of the opportunities and assess underlying potential of the market.

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Market Research Reports Inc.

+1-302-703-9904

email us here

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