# Egypt fights Spinal Muscular Atrophy

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Abstract

SMA (spinal muscular atrophy) is a neuromuscular illness caused by a genetic (inherited) mutation that causes muscles to weaken and waste away. Motor neurons, a type of nerve cell in the spinal cord that controls muscle movement, are lost in people with SMA. Many pharmaceutical companies continue to invest heavily in research into SMA therapies. Many additional prospective medications, such as valproic acid, phenylbutyrate, hydroxyurea, albuterol, gabapentin, riluzole, olesoxime, and rapamycin, have failed to generate adequate outcomes regarding disease progression.

Keywords: spinal muscular atrophy, medications, hydroxyurea, neuromuscular illness

**Introduction**

SMA (spinal muscular atrophy) is a neuromuscular illness caused by a genetic (inherited) mutation that causes muscles to weaken and waste away. Motor neurons, a type of nerve cell in the spinal cord that controls muscle movement, are lost in people with SMA. Muscles do not receive nerve signals that cause them to move unless these motor neurons are present. Atrophy is a medical term that means "to shrink." Due to a lack of use, specific muscles in SMA become smaller and weaker [1].

**Categories of SMA**

SMA is divided into four categories: Type 1 (abnormally severe): Type 1 SMA, commonly known as Werdnig-Hoffman disease, affects about 60% of patients with SMA. Symptoms arise shortly after birth or within the first six months of an infant's life. Type 1 SMA babies have trouble eating and sucking. They don't reach regular developmental milestones such as holding their heads up or sitting. As muscles weaken, children are more susceptible to respiratory infections and lung collapse (pneumothorax). The majority of children with type 1 SMA die before they reach the age of two [2]. Type 2 (intermediate): Symptoms of type 2 SMA (also known as Dubowitz disease) occur between the ages of six and eighteen months. This kind usually affects the lower extremities. Type 2 SMA children may be able to sit up but not walk. The majority of children with type 2 SMA grow up to be adults [3]. Type 3 (mild): After a child's first 18 months of life, symptoms of type 3 SMA (also known as Kugelbert-Welander or juvenile-onset SMA) arise. Some patients with type 3 diabetes do not show symptoms until they are well into adulthood. Mild muscle weakness, trouble walking, and frequent respiratory infections are all Type 3 symptoms. Symptoms can impair one's ability to walk or stand over time. Type 3 SMA does not cause a significant reduction in life expectancy [4]. Type 4 (adult): The adult version of SMA usually doesn't show up until the mid-30s. Because the signs of muscle weakness develop slowly, most persons with type 4 remain mobile and live full lives [5].

**Neuromuscular illness**

CAIRO, July 2, 2021: According to President Abdel Fattah El-directions, Sisi's Egypt's Health Minister Hala Zayed said the country will begin treating 10 out of 32 instances of Spinal Muscular Atrophy (SMA). According to Zayed, the therapy will be carried out using the world's most costly medicine, which costs $2-3 million per dose [6]. According to Zayed, the therapy endeavour would begin with ten cases under the age of two years. She stated that the political leadership has mandated the treatment of all SMA patients and that a long-term procedure has been established to address instances older than two years. She went on to say that this segment, on the other hand, will necessitate lifelong treatment. Spinal muscular atrophy (SMA) is an uncommon neuromuscular illness that is usually identified in infancy or early childhood, and if left untreated, can lead to death [7].

Last week, as he evaluated vehicles and equipment utilized in the development of communities covered in the Haya Karima initiative, Sisi spoke about the country's efforts to heal children suffering from muscle atrophy. According to him, the treatment for muscle atrophy costs up to $3 million per child, and Egypt is eager to secure it even though many other countries cannot afford it [8]. "In the early months after a child's birth, there are methods for early identification of this condition. If we knew, we could provide treatment, which may cost up to $3 million per child," El-Sisi said. Zayed, who was present at the ceremony, stated that 204 cases of muscular atrophy had been discovered in Egypt, with more than 32 of them being treatable. Egypt will

begin treating muscle atrophy cases next week, according to Zayed. The president encouraged civil society groups to participate in the effort [9].

Egypt's Ministry of Health has confirmed that the first ten cases will be treated at Nasser Institute and Ain Shams Hospital. According to the ministry, the medicine used to cure muscle atrophy is expensive and will be offered for treatment in Egypt. The Egyptian Medicine Authority has previously announced the registration of the first drug for the treatment of muscular atrophy on the Egyptian market [10]. According to the EDA, the medicine will help to alleviate patient suffering while also eradicating the problem of drug smuggling. Patients with muscle atrophy have filed concerns with the government's complaints system, prompting the authorities to work as quickly as possible to address their needs [11].

## Conclusion

Many pharmaceutical companies continue to invest heavily in research into SMA therapies. Many additional prospective medications, such as valproic acid, phenylbutyrate, hydroxyurea, albuterol, gabapentin, riluzole, olesoxime, and rapamycin, have failed to generate adequate outcomes regarding disease progression [12]. Clinical study data for a variety of additional potential therapies are still forthcoming. 18 Pharma is still looking for a cure for SMA that is both effective and affordable. Clinical trials on the three approved medications are still underway, and pharmaceutical companies are looking into how combination therapy could help patients [13].

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