Subject: Request for including Risdiplam for treatment of Spinal Muscular Atrophy in the WHO Model List of Essential Drugs

Dear Secretary and Expert Committee,

11-03-2023

I am writing to request the inclusion of Risdiplam on the World Health Organization (WHO) Model List of Essential Medicines (EML) for the treatment of Spinal Muscular Atrophy (SMA).

Greetings from Chennai, India. Please allow me to introduce myself as one of the 100000 people afflicted with Spinal Muscular Atrophy (SMA) in India. I am [redacted], 35 years old, and I have been confined to a wheelchair most of my life.

I have the lost my ability to stand, walk, or even keep my body upright in my wheelchair. I have very limited use of my limbs (less than 10%). Additionally, the severe scoliosis of my back causes unbearable pain in my muscles and keeps me up the entire night. The SMA has also started affecting my breathing abilities.

Despite my disabilities, I have pursued my passion for science, excelled in academics at school and college, and went on to earn my Ph.D in Bioinformatics. I have published over 14 peer reviewed articles in reputed journals, authored 2 textbooks, and continue to research. I currently teach over 300 students every year at the [redacted]. I am in terrible pain most of the day and night, but I am up and ready the next morning to meet and engage with my students to get them excited about learning.

I am the sole breadwinner of my family. We don't hold any real estate property or other assets (we live in a rental apartment). My father passed away 2 years back, and my mother is my caretaker. I also have to bear the added expense of a full-time caretaker to carry and put me in my wheelchair and assist me with everything—including my feeding, personal hygiene, and help with holding the phone and navigating through my computer while I teach.

Risdiplam is a promising new treatment for SMA that has been approved by regulatory agencies such as the US Food and Drug Administration (FDA) and the European Medicines Agency (EMA). The drug works by increasing the production of survival motor neuron (SMN) protein, which is essential for the survival of motor neurons. Risdiplam has shown to be effective in clinical trials and has been well-tolerated by patients with SMA.

Last year through a crowdfunding started by my well-wishers, I was able to raise around Rs.60 lakhs and was able to afford Roche's Evrysdi (Risdiplam) for one year. After taking Evrysdi (Risdiplam) for the last one year, I have noticed considerable improvement in my neck control and very importantly my breathing has been much easier. Evrysdi (Risdiplam) also helped me regain my health after a severe attack of COVID in July 2022. I have not been able to raise further funds through crowdfunding to continue the treatment this year. So far, I have only 2 lakhs for the treatment. On my limited income (4.75 lakhs per year), I would never be able to afford to pay for this expensive wonder-drug.

In order to sustain my health status and in a desperation to keep myself alive, I have been taking some loans from extended family and friends to buy Risdiplam for a few more months.

I believe that the inclusion of Risdiplam on the WHO Model List of Essential Medicines would have a significant impact on the treatment of SMA globally. It would increase access to this life-changing treatment for patients in low- and middle-income countries and help to reduce the burden of SMA on individuals, families, and healthcare systems worldwide.

I beg the WHO to consider including Risdiplam on the EML for the treatment of SMA and to work towards ensuring its availability and affordability for all those who need it.

Thank you for your attention to this important matter.

With Regards,

[redacted]

Proud SMA Warrior - [redacted]