Subject : [redacted] testimonial for inclusion of Risdiplam (oral drug for the treatment for Spinal Muscular Atrophy) in the WHO list of essential medicines -written by mother [redacted].

Dear Expert committee members,

Our 5-year-old daughter [redacted] is suffering from genetic neuromuscular disease Spinal Muscular Atrophy, type 1 and is receiving the drug Risdiplam through Compassionate Use Program (CUP) by Roche Pharmaceuticals from the year 2020. We, [redacted] - her parents, have witnessed the benefits of this drug and would request you to include Risdiplam in the WHO model list of essential medicines (EML) and WHO model list of essential medicines for children (EMLc).

Our daughter was unable to sit without support and would constantly fall ill before drug. As parents its very fulfilling to watch her achieving a new milestone – rolling, which enables her to move from one place to another which was never possible before. We are very happy to note that her immunity has also improved, probably due to better strength of respiratory muscles with Risdiplam. She had combatted a Covid Infected too, in January 2022, which was mild on her and no hospital admission was required.

Although we are receiving this miracle drug free of cost now, we know that this CUP is for a limited period after which we will have to buy this drug. The cost of Risdiplam in India is INR 72 lakhs per year (as per her weight). This high cost is unaffordable for us like any other average Indian family. Its frightening to think what would happen to our daughter without this drug as this disease is progressive in nature.

We believe that inclusion of Risdiplam in the WHO list of essential medicines would make the access of this medicine possible for all SMA patients like my daughter. We earnestly hope our plea would be considered urgently and this drug will be added in list as soon as possible.

Heartfelt thanks for reading this letter.

Sincerely,

[redacted]

Parents of [redacted]