PRABHA IAS IPS COACHING ACADEMY

Spinal Muscular Atrophy (SMA)

Patients have appealed for the urgent inclusion of generic Risdiplam under National Policy for Rare Diseases (NPRD).

Risdiplam: It is an oral drug that slows or halts progression of SMA by targeting the underlying genetic mechanism.

SMA is a rare, genetic, progressive, and life-limiting neuromuscular disorder that results in loss of motor neurons and progressive muscle wasting.

Caused by mutation or deletion in the Survival Motor Neuron 1 (SMN1) gene, leading to deficient SMN protein production, which is crucial for the survival of motor neurons.

5 **subtypes**: 0, I, II, III, IV. Type I (Werdnig-Hoffmann disease) is the most common and severe form.

It leads to muscle weakness, respiratory failure, and loss of mobility. No cure available.