

The spinal muscular atrophies (SMAs), are characterized by degeneration of nerve cells (motor nuclei) within the lowest region of the brain (lower brainstem) and certain motor neurons in the spinal cord (anterior horn cells) leading to muscle weakness of the truncal, and extremity muscles initially, followed by chewing, swallowing and breathing difficulties. Motor neurons are nerve cells that transmit nerve impulses from the spinal cord or brain (central nervous system) to muscle or glandular tissue. Werdnig-Hoffmann disease is a rare disorder that affects males and females in equal numbers. The prevalence of all types of spinal muscular atrophy has been estimated to be 4-7.8 per 100,000 live births. Approximately 80% of SMA patients have the Werdnig-Hoffmann form.