

Therapeutic (r)evolution in SMA: where are we now and what about the future?

Abstract

From the arrival of groundbreaking disease modifying treatments to the continuous refinement of existing symptomatic approaches, the journey of Spinal Muscular Atrophy (SMA) therapeutics has witnessed a profound (r)evolution over the past decade. Through an exploration of current achievements, including SMN2 splicing modifiers and gene replacement therapies, the implementation of newborn screening, and upcoming innovative pharmaceutical interventions, the transformative impact on patient outcomes will be illuminated. By navigating the trajectory of SMA treatment modalities, we aim to provide insights into the present state and future prospects of therapeutic(r)evolution in SMA management.