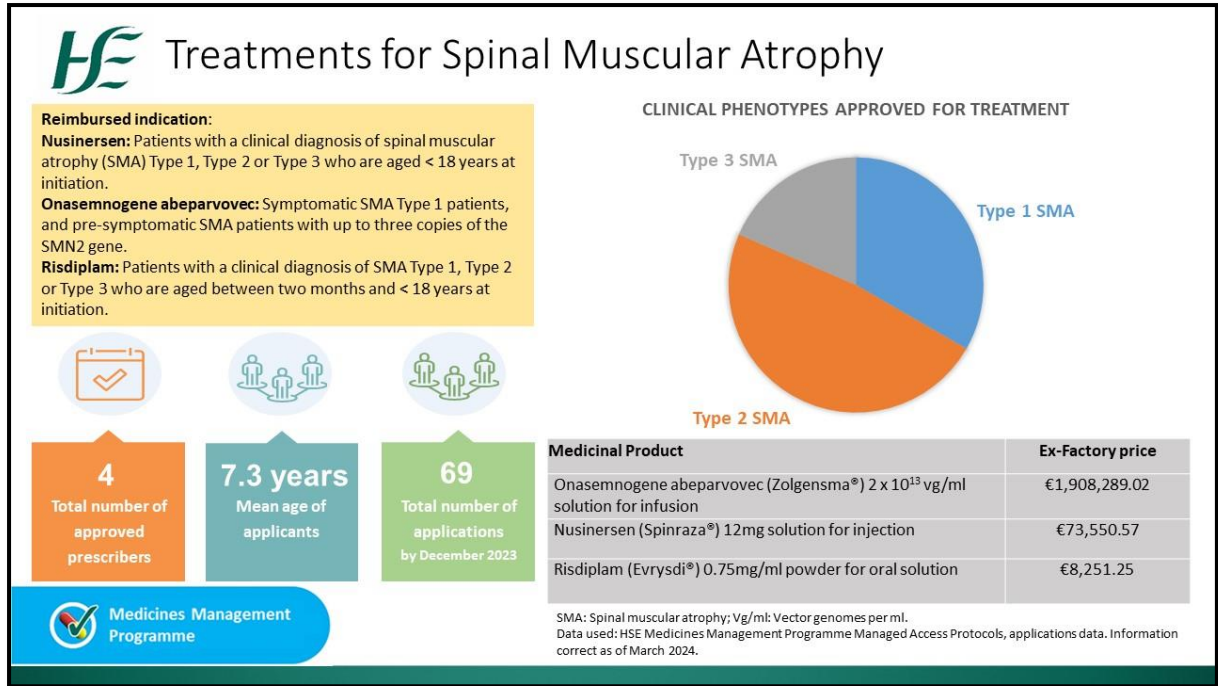


UPDATE ON TREATMENTS FOR SPINAL MUSCULAR ATROPHY



The HSE reimburses treatments for spinal muscular atrophy (SMA) subject to managed access protocols (MAP). The first MAP for a treatment for SMA was for nusinersen, an antisense oligonucleotide, introduced in July 2019. This was followed by a MAP for onasemnogene abeparvovec, a gene therapy product, in October 2021, and risdiplam, a messenger ribonucleic acid splicing modifier, in September 2023. The aim of these MAPs is to provide patients with certain subtypes of SMA with access to these high-cost treatments.

To date, the HSE have approved four prescribers under these protocols. By December 2023, 69 applications for these treatments were submitted to the HSE Medicines Management Programme. The mean age of applicants was 7.3 years at date of application.

April 2024

HSE Medicines Management Programme.