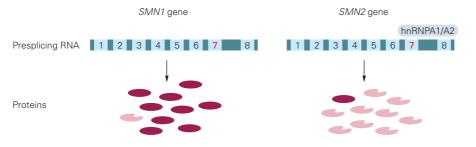
A Production of normal levels of the SMN protein



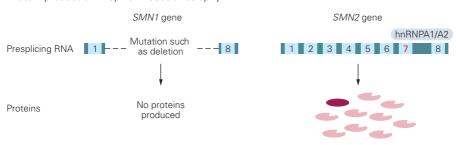
Normal protein from full-length

mRNA (exons 1-8) Abnormal protein

from short mRNA

(missing exon 7)

B Protein production in spinal muscular atrophy



C Restoration of therapeutic levels of the SMN protein

