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Supplemental Information

Efficient SMN Rescue following Subcutaneous Tricyclo-DNA Antisense Oligonucleotide Treatment

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Supplementary figures

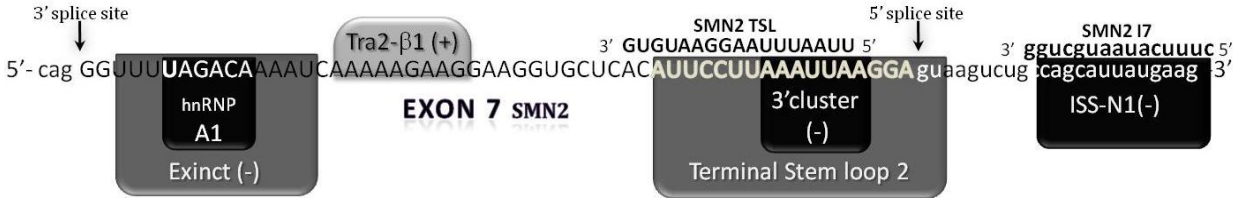

Figure S1: **Schematic representation of regulatory elements located within exon 7 and a part of intron 7 of *SMN2*.**

The sequence of *SMN2* exon 7 and adjacent intron 7 are given. Positive elements that promote exon 7 inclusion and negative elements promoting exon 7 skipping are indicated by (+) and (−). The two sequences of AONS used in this paper are indicated : SMN2 TSL and SMN2 I7.

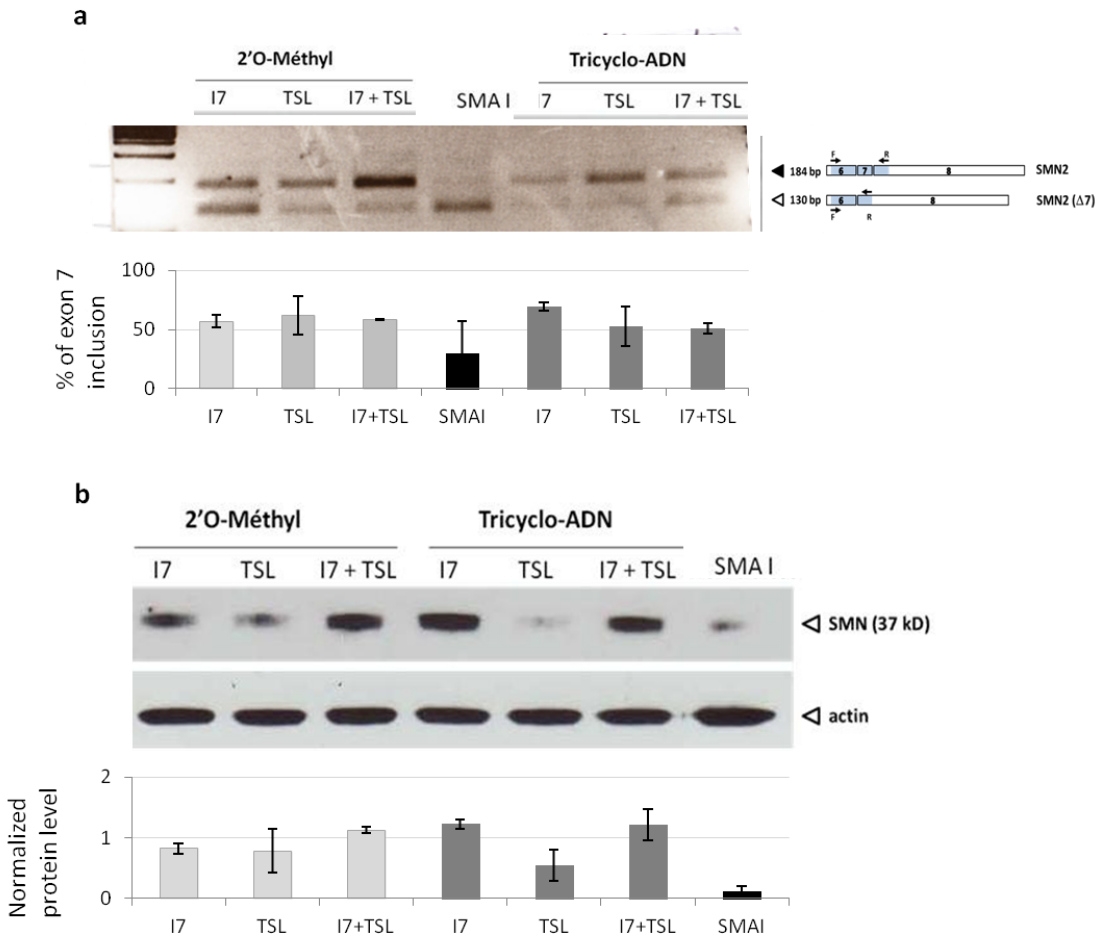

Figure S2: **Comparative evaluation of *in vitro* efficiency of antisens oligonucleotides.**

a) Detection of the inclusion of exon 7 of SMN2 mRNA by RT-PCR in patients SMA I fibroblasts. The primers used for amplification are located on exon 6 and 8, and the PCR product is visualized on 3% agarose gel. (Lane 1) Cells treated with 2'O-Me I7, (Lane 2) cells treated with 2'O-Me TSL, (Lane 3) cells treated with both 2'O-Me, (Lane 4) cells of untreated patients**,** (Lane 5) SMA I fibroblasts treated with TcI7 (Lane 6) SMA I fibroblasts treated with Tc-TSL, (Lane 7) cells treated with both tcDNA. The first band at 184 bp corresponds to SMN1 and SMN2 with mutated exon 7, the second band at 130 bp is SMN2 amplification product without exon 7. **b)** Detection of the SMN protein by Western blot in fibroblasts and normalization with actin. (Lane 1) SMA fibroblasts treated with 2'O-Me I7, (Lane 2) SMA fibroblasts treated with 2'O-Me TSL, (Lane 3) SMA fibroblasts treated with 2'O-Me I7 and TSL, (Lane 4) SMA fibroblasts treated with Tc I7, (Lane 5) SMA fibroblasts treated with Tc

TSL, (Lane 6) SMA fibroblasts treated with Tc I7 and TSL, (Lane 7) untreated SMA fibroblasts.