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#### (54) COMPOSITIONS AND METHODS FOR MODULATION OF SMN2 SPLICING IN A SUBJECT

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(57) ABSTRACT

Disclosed herein are compounds, compositions and methods for modulating splicing of SMN2 mRNA in a subject. Also provided are uses of disclosed compounds and compositions in the manufacture of a medicament for treatment of diseases and disorders, including spinal muscular atrophy.



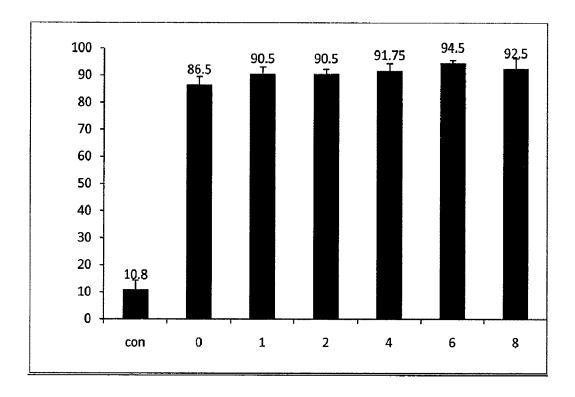


Figure 1

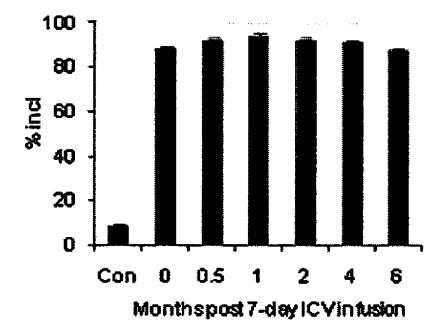
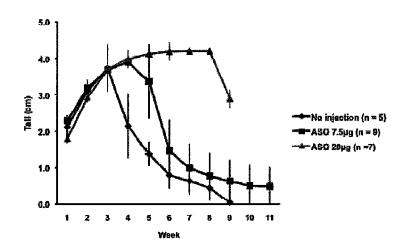


Figure 2

A



В

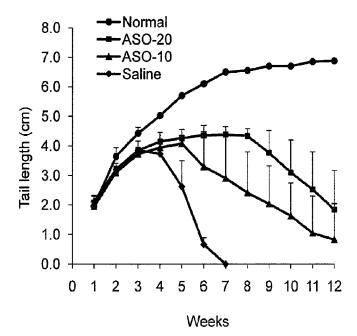
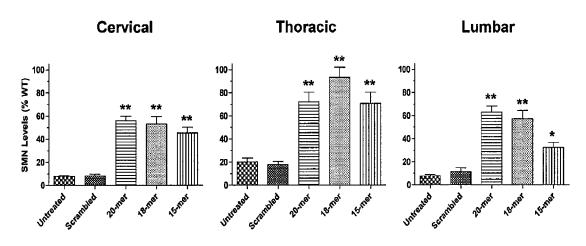


Figure 3



### **SMN Western Blots of SMA**



4 μg dose, 16 days post-

Figure 4

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