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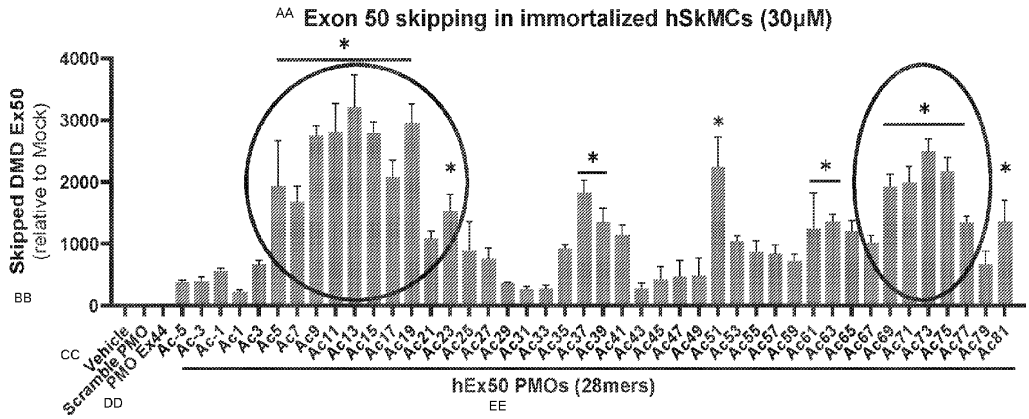
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(54) Title: ANTIBODY-OLIGONUCLEOTIDE CONJUGATE COMPOSITIONS AND METHODS OF INDUCING DMD EXON 50 SKIPPING



AA Saut d'exon 50 dans hSkMC immortalisées
BB Ex50 de DMD sauté (par rapport à simulation)
CC Véhicule
DD PMO mêlés
EE PMO hEx52 (28mères)

FIG. 1

(57) Abstract: Disclosed herein are antibody-oligonucleotide conjugates and pharmaceutical compositions comprising the antibody-oligonucleotide conjugates that induce an alteration in an incorrectly spliced dystrophin mRNA transcript to induce DMD exon 50 skipping. Also described herein include methods for treating muscle dystrophy including Duchenne muscular dystrophy by administering antibody-oligonucleotide conjugates or a pharmaceutical composition comprising the antibody-oligonucleotide conjugates that induce an alteration in an incorrectly spliced dystrophin mRNA transcript to induce DMD exon 50 skipping.

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