Phenotype correction of hemophilia A mice by spliceosome-mediated RNA trans-splicing. [1]

Enviado por Mariano Garcia-Blanco [2] el 9 octubre 2012 - 5:49pm

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Título	Phenotype correction of hemophilia A mice by spliceosome-mediated RNA trans-splicing.
Publication Type	Journal Article
Year of Publication	2003
Autores	Chao, H [3], Mansfield, GS [4], Bartel, RC [5], Hiriyanna, S [6], Mitchell, LG [7], García-Blanco, MA [8], Walsh, CE [9]
Journal	Nat Med
Volume	9
Issue	8
Pagination	1015-9
Date Published	2003 Aug
ISSN	1078-8956
Palabras clave	Animals [10], Blood Coagulation [11], Cell Line [12], Disease Models, Animal [13], Factor VIII [14], Gene Therapy [15], Hemophilia A [16], Humans [17], Mice [18], Mice, Knockout [19], Phenotype [20], RNA [21], RNA Splicing [22], Spliceosomes [23]

Abstract	Conventional gene therapy of hemophilia A relies on the transfer of factor VIII (FVIII; encoded by the F8 gene) cDNA. We carried out spliceosome-mediated RNA trans-splicing (SMaRT) to repair mutant FVIII mRNA. A pre-trans-splicing molecule (PTM) corrected endogenous FVIII mRNA in F8 knockout mice with the hemophilia A phenotype, producing sufficient functional FVIII to correct the hemophilia A phenotype. This is the first description of phenotypic correction of a genetic defect by RNA repair in a knockout animal model. Our results indicate the feasibility of using SMaRT to repair RNA for the treatment of genetic diseases.
DOI	10.1038/nm900 [24]
Alternate Journal	Nat. Med.
PubMed ID	12847523 [25]

Links
[1] https://www.cienciapr.org/es/phenotype-correction-hemophilia-mice-spliceosome-mediated-rna-trans-splicing?language=en [2] https://www.cienciapr.org/es/user/garci001?language=en [3] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=707 [4] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=605 [5] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=708 [6] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=709 [7] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=706 [8] https://www.cienciapr.org/es/user/13/biblio?language=en [9] https://www.cienciapr.org/es/biblio?language=en&f%5Bauthor%5D=710 [10] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=1 [11] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=922 [12] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=335 [13] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=378 [14] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=923 [15] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=829 [16] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=924 [17] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=9 [18] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=357 [19] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=925 [20] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=540 [21] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=790 [22] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=808 [23] https://www.cienciapr.org/es/biblio?language=en&f%5Bkeyword%5D=802 [24] http://dx.doi.org/10.1038/nm900 [25] https://www.ncbi.nlm.nih.gov/pubmed/12847523?dopt=Abstract