

Induction of cryptic pre-mRNA splice-switching by antisense oligonucleotides

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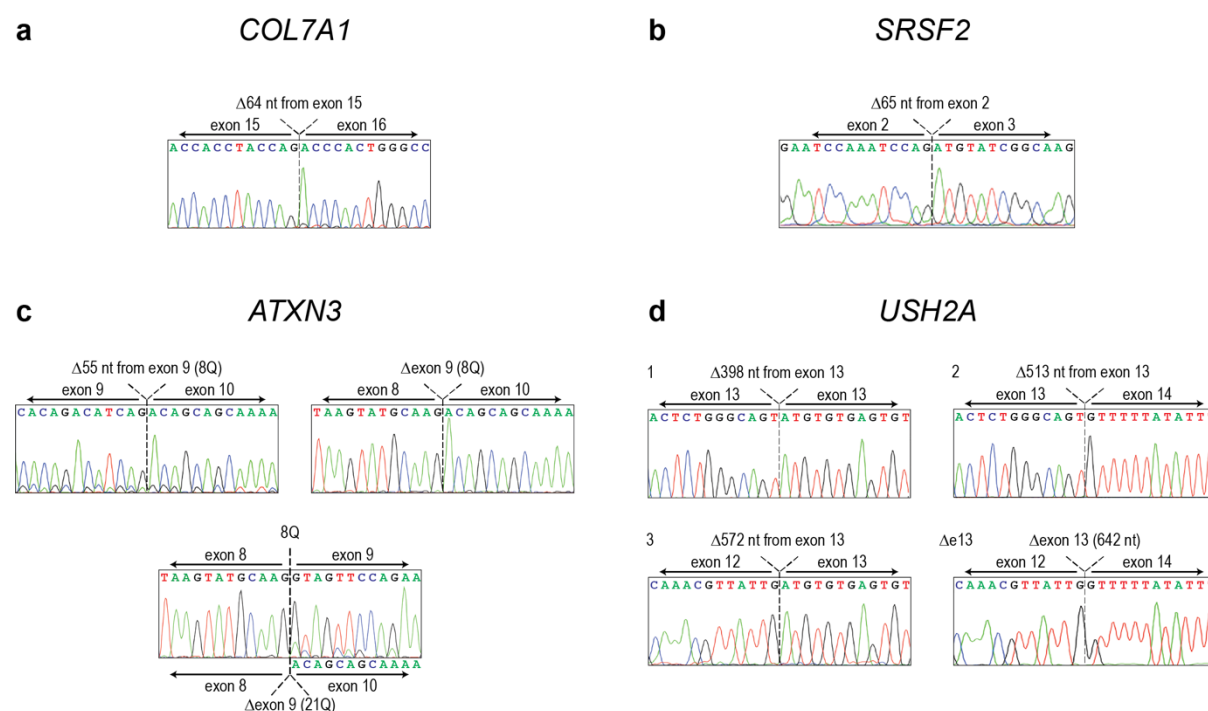
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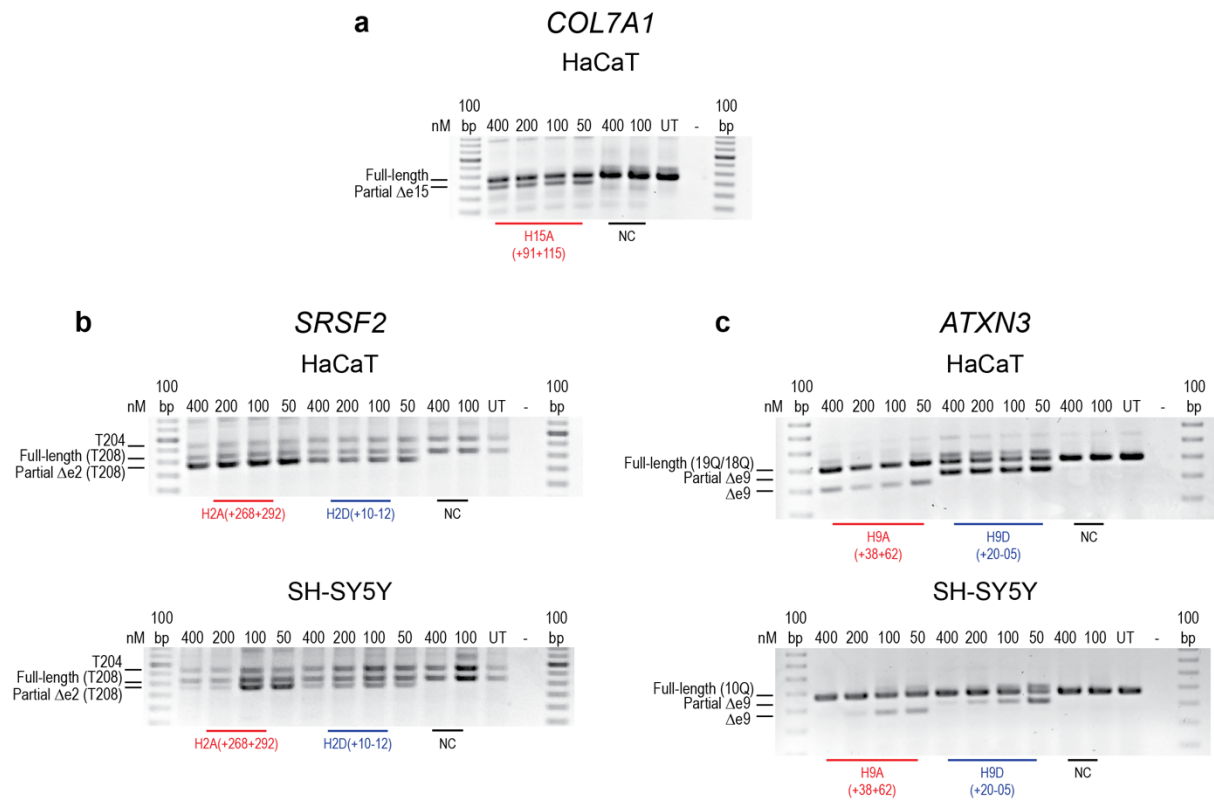
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¶ these authors contributed equally to this work

Supplementary materials



Supplementary Figure S1. Sanger sequencing confirms transcripts resulting from AO treatment in four different gene transcript targets. (a) COL7A1 exon 15 new donor site activated. (b) SRSF2 exon 2 new donor site activated. (c) ATXN3 exon 9 new donor site activated in the 8Q transcript and complete exon 9 removal from the 8Q transcript. Sanger sequencing reveals a secondary transcript, complete removal of exon 9 from the 21Q transcript, as well as the full-length 8Q transcript. (d) USH2A exon 13 new donor (2), new acceptor (3), combination of a new donor and new acceptor (1), and complete exon 13 (Δe13) removal from the full-length transcript.



Supplementary Figure S2. Activation of cryptic splice sites by AO-mediated splice switching in multiple cell lines. Reverse transcription-PCR analysis after transfection with antisense oligonucleotides (AOs), at various nM concentrations indicated above the gel image for **(a)** *COL7A1* H15A(+91+115) tested in HaCaT, **(b)** *SRSF2* H2A(+268+292) and H2D(+10-12) tested in both HaCaT and SH-SY5Y, and **(c)** *ATXN3* H9A(+38+62) and H9D(+20-05) testing in both HaCaT and SH-SY5Y. Multiple transcript isoforms (noted as T204 and T208) according to Ensembl. NC, negative control sequence synthesized as 2'-OMe PS; UT, untreated; 100 bp, 100 base pair DNA ladder; nM, nanomolar. The gels were cropped for presentation. Full-length gel images are presented in Supplementary Figure S3.

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[illegible]

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2ry, no AO

H12A(+269+297) mask

2ry, H12A(+269+297)

~~~ LMNA exon 11 ~~~

2ry, no AO

H11A(+211+245) mask



[illegible]

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H13A(+161+183) mask

2ry, H13A(+161+183)

[illegible]

**Supplementary Figure S4. RNA secondary structures.**

# ATXN Exon 9 HSF output (JSON)

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