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Supplemental Information

Combining Engineered U1 snRNA and Antisense Oligonucleotides to Improve the Treatment of a *BBS1* Splice Site Mutation

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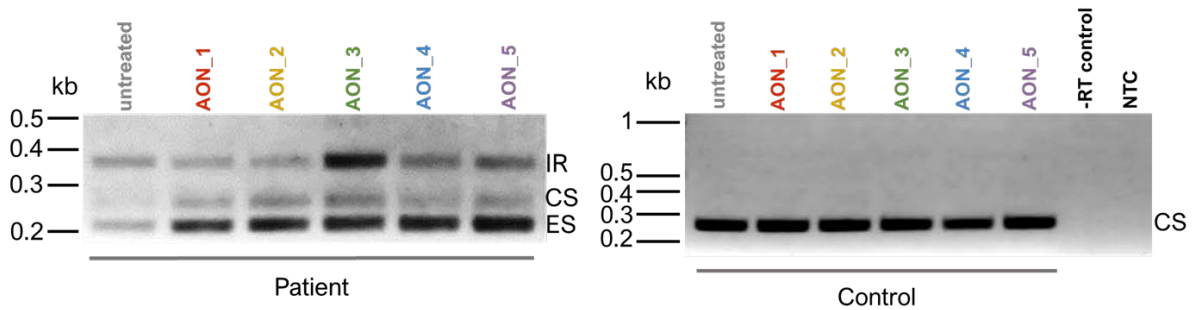
1 Supplemental Material

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3 Combining designed U1 snRNA and antisense oligonucleotides to improve the treatment of a *BBS1*
4 splice site mutation.

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Figure S1:



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7 **Fig. S1: Verification of AON effects during the apoptosis assay.** Cells were lysed and RT-PCR reactions
8 were performed after the apoptosis assays. AON concentrations were 36 nM. The efficacy of the tested
9 AONs to reduce intron 5 retention in the patient samples resembles the results shown in Fig. 3. The AON
10 treatment did not induce splice defects in control cells. NTC: none template control; -RT control: PCR
11 control reaction using a template with RNA but no reverse transcriptase was added during cDNA-
12 synthesis. CS: correctly spliced transcript, ES: exon skipping, IR: intron retention, kb: kilo bases.

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