





70 Supplemental Figure S5. Chimeric antisense U1 snRNA-induced exon 9a skipping in 66W1 cell lines transfected 71 with RPGR mutant minigene. (a) RT-PCR analysis of RPGR mRNA levels on RNA from 661W cells transfected with 72 wild-type minigene (MINI wt, lane 1) and mutant minigene (MINI mut, lane 2). M: marker (sizes in base pairs indicated 73 by numbers on the side). NT: Non-transfected: RNA extracted from 661W cells not transfected. NC: Negative Control 74 (No-template control: no cDNA was included in the reaction). The analysis has been performed in triplicate, and one 75 representative gel is shown. The histogram represents the densitometric analysis of the bands relative to the two different 76 isoforms (E9a+ and E9a-) normalized to GAPDH (p<0.001). Data are shown as mean \pm S.D (n=3). (b) Semiquantitative 77 RT-PCR of RNA from 661W cells transfected with RPGR mutant minigene (MINI mut) alone or in combination with 78 chimeric U1 snRNAs. E9a reduction using U1 3' or U1 3'5' is significant (p<0.01) compared to U1 Scramble. One 79 representative gel of three and densitometric analysis of E9a+ and E9a- amplicons, from three independent experiments, 80 are shown. M: marker (sizes in base pairs indicated by numbers on the side). NC: Negative Control (No-template control: 81 no cDNA was included in the reaction). GAPDH is used as an internal control. Data are shown as mean \pm S.D (n=3). 82 Statistical analysis was performed with a global statistical test and is reported in Supplemental Table S1 (p-value: *P < 83 0.05; ***P < 0.001; ****P < 0.0001). 84

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