



**Figure S1.** USH2A protein levels of untreated and TRIDS-treated patient-derived cells relative to healthy donor cells.

We calculated the relative USH2A expression levels in patient-derived cells versus healthy control fibroblasts. The band intensity for USH2A (Figure 3A) of control fibroblasts was set to 100%. A significant decrease of USH2A protein levels were detected in patient-derived fibroblasts treated with DMSO, 10  $\mu\text{g}/\mu\text{L}$  Ataluren-treated or Gentamicin (Genta) compared to healthy control cells. No significant differences in protein levels were observed between healthy control cells and patient-derived cells treated with 5  $\mu\text{g}/\text{ml}$  Ataluren.  $p < 0.001$ (\*\*\*),  $p < 0.01$  (\*\*).

**Table S1.** Summary of the number of ciliated cells in patient-derived cells and healthy donor cells following TRID-treatment.

Cell Line	Treatment	Total Number	Ciliated Cells	Unciliated Cells
<b>Healthy donor</b> ( $n = 4$ )	Untr.	198	149	49
	DMSO	221	153	68
	Gentamicin	205	133	72
	Ataluren	180	133	47
<b>Patient-derived</b> ( $n = 4$ )	Untr.	97	51	46
	DMSO	100	50	50
	Gentamicin	93	35	58
	Ataluren	119	82	37