Figure S1: Diagram of the tested ASOs mapped to their position relative to exon 23 (gray bar) and its flanking intronic sequences (black lines).
Figure S2: The effect of ASOs targeted to skip over exon 23 in 16HBEge W1282X cells. 16HBEge W1282X cells were transfected with 10nM of each ASO. RNA was extracted and the levels of CFTR transcripts with and without exon 23 were measured 24 hrs after transfection. A-G. Representative PCR gels of the 79.
Figure S3: The effect of ASOs targeted to skip over exon 23 on the level of CFTR transcripts in 16HBEge W1282X cells. 16HBEge W1282X cells were transfected with each ASO. The values shown are the average fold change (mean±SEM) from 3 independent experiments relative to cells treated with a control ASO.
Figure S4: ASOs for exon 23 skipping result in generation of mature CFTR protein in 16HBEge W1282X cells. The additive effect of the ASOs and correctors was analyzed, 16HBE14o-WT cells were used as a control. A-F. Representative gels.
Figure S5: The effect of ASOs targeted to skip over exon 23 on the RNA level in WT and F508del cells. 16HBE14o- WT (blue), 16HBEge F508del (green) and 16HBEge W1282X (purple) cells were transfected with each ASO A. Examples of PCR gels of the ASOs that showed specificity to the W1282X allele. B. Quantification of the PCR results using Image Studio software. The values shown are the average fold change (mean±SEM) from 4 independent experiments relative to cells treated with a control ASO. dF- F508del mutation
Figure S6: The effect of ASOs targeted to skip over exon 23 in WT and F508del cells on the protein level. A. 16HBE14o- WT cells B. 16HBEge F508del cells
Figure S7: The effect of free uptake of SPL23-2 and SPL23-3 on exon 23 skipping in 16HBE14o- WT cells. A. 16HBEge W1282X and B. 16HBE14o- WT cells were treated with each ASO. Examples of PCR gels showing specificity to the W1282X allele. C. The effect of ASOs targeted to skip over exon 23 in 16HBEWT cells on the protein level.