

WT RNA/ASO duplexes (5'-3')/(3'-5')	-ΔG° ₃₇	Tm ^a	Mut RNA/ASO duplexes (5'-3')/(3'-5')	-ΔG° ₃₇	Tm ^a	Inhibitor ^{b,c} influence ΔΔG° ₃₇	WT-Mut RNA ΔΔG° ₃₇	Percentage hydrolysis of RNA <i>in vitro</i> ^d			Expression of RNA in <i>HeLa</i> cells		
<i>Transversion C/G (C692G, APP gene)</i>								WT RNA	Mut RNA	Difference	WT RNA	Mut RNA	Selectivity
UUUGCAGAAGAUG AAACGUCU Ib1	9.29±0.10	53.1	UUUGGAGAAGAUG AAACGUCU	4.60±0.18	22.8	0 ^b	4.69	87.4 ± 2.4 ^e (10.5 ± 4.5) ^f	98.6 ± 1.8 ^e (-4.60 ± 6.0) ^f	11.2 ± 3.0	82%	36%	46% (25/50) ^j
UUUGCAGAAGAUG AAACGUCUUC Ib2	12.60±0.07	66.9	UUUGGAGAAGAUG AAACGUCUUC	8.64±0.03	46.4	(0) ^c	3.96	11.7 ± 2.5 ^e (86.2 ± 4.5) ^h	98.8 ± 4.9 ^e (-4.80 ± 7.5) ^h	87.1 ± 5.50	67%	28%	39% (25/50)
UUUGCAGAAGAUG AAACCTCTTCUAC bKM	9.81±0.08	48.6	UUUGGAGAAGAUG AAACCTCTTCUAC	17.92±0.59	67.2	8.63 ^b (5.32) ^c	-8.11	97.9 ± 3.8 ⁱ (0.0)	94.0 ± 5.7 ⁱ (0.0)	-3.9 ± 6.85	49%	52%	3% (50) ^k
<i>Transition G/A (G717A, APP gene)</i>													
GUGAUCGUCAUCA UAGCAGU Id1	9.31±0.03	56.1	GUGAUCUCAUCA UAGCAGU	7.71±0.04	44.4	0	1.60	30.3 ± 7.7 (27.4 ± 17.6)	67.3 ± 2.9 (16.3 ± 13.0)	37.0 ± 8.2	-	-	-
GUGAUCGUCAUCA CUAGCAGUAG Id2	15.01±0.15	72.9	GUGAUCUCAUCA CUAGCAGUAG	10.36±0.10	53.5	(0)	4.65	10.7 ± 3.2 (47.7 ± 16.8/)	72.4 ± 3.8 (11.2 ± 13.1)	61.7 ± 5.0	-	-	-
GUGAUCGUCAUCA CACTAGTAGTAGU dKM	10.48±0.05	57.3	GUGAUCUCAUCA CACTAGTAGTAGU	10.98±0.09	61.3	1.67 (-4.03)	-0.50	57.7 ± 15.8 (0.0)	83.6 ± 12.6 (0.0)	25.9 ± 20.8	123%	31%	92% (50)
<i>Transition A/G (A693G, APP)</i>													
UUUGCAGAAGAUG GUCUUCU Ie1	9.24±0.04	51.6	UUUGCAGGAGAUG GUCUUCU	7.52±0.01	43.0	0	1.72	78.2 ± 1.9 (18.7 ± 2.5)	99.6 ± 0.7 (-1.9 ± 2.4)	21.4 ± 2.0	-	-	-
UUUGCAGGAGAUG GUCUUCUAC Ie2	11.88±0.09	58.7	UUUGCAGGAGAUG GUCUUCUAC	10.44±0.06	52.0	(0)	1.44	14.3 ± 2.1 (82.6 ± 2.7)	56.6 ± 2.6 (41.1 ± 3.5)	42.3 ± 3.3	50%	5%	45% (25/100)
UUUGCAGGAGAUG AAACGTCTTCUAC eKM	11.69±0.15	57.3	UUUGCAGGAGAUG AAACGTCTTCUAC	17.00±0.48	73.2	8.76 (5.12)	-5.31	96.9 ± 1.7 (0.0)	97.7 ± 2.3 (0.0)	0.8 ± 3.3	80%	37%	43% (50)
<i>Transition G/A (G46A, SNCA)</i>													
ACCAAGGAGGGAG UUCUCC Ik1	11.83±0.10	67.9	ACCAAGAAGGGAG UUCUCC	7.76±0.02	63.4	0	4.07	15.9 ± 1.2 (70.4 ± 4.9)	64.8 ± 3.4 (13.0 ± 15.0)	48.9 ± 3.9	100%	35%	65% (25/50)
ACCAAGGAGGGAG GGUCCUCC Ik2	20.98±0.58	82.8	ACCAAGAAGGGAG GGUCCUCC	13.45±0.17	63.5	(0)	7.53	0	18.2 ± 4.4 (59.6 ± 15.4)	18.2	70%	23%	47% (25/75)
ACCAAGGAGGGAG UGGTCTTCCUC kKM	20.59±0.50	78.8	ACCAAGAAGGGAG UGGTCTTCCUC	23.95±0.43	81.3	12.12 (2.97)	-3.36	86.3 ± 4.8 (0.0)	77.8 ± 14.6 (0.0)	-8.5 ± 15.4	28%	17%	10% (50)
<i>Transition G/A (G53A, SNCA)</i>													
GGUGUGGCAACAG CACCGUU Ih1	10.33±0.07	60.4	GGUGUGCAACAG CACCGUU	6.22±0.05	34.2	0	4.11	15.9 ± 2.0 (81.0 ± 2.9)	99.3 ± 0.8 (-0.2 ± 0.9)	83.4 ± 2.1	60%	15%	45% (25/100)
GGUGUGGCAACAG ACACCGUUGU Ih2	16.12±0.13	75.1	GGUGUGCAACAG ACACCGUUGU	10.58±0.06	54.1	(0)	5.54	5.9 ± 2.3 (91.0 ± 3.1)	50.8 ± 3.4 (48.3 ± 3.5)	44.9 ± 4.1	45%	11%	34% (25/100)
GGUGUGGCAACAG CCACACTGTTGUC hKM	16.29±0.09	72.9	GGUGUGCAACAG CCACACTGTTGUC	18.99±0.34	73.8	8.61 (2.87)	-2.90	96.9 ± 2.1 (0.0)	99.1 ± 0.3 (0.0)	2.2 ± 2.3	46%	30%	16% (100)
<i>Transition C/U (C4U, SOD1)</i>													
CGAAGGCCGUGUG UCCGGCA Ia1	12.41±0.12	71.8	CGAAGGUCGUGUG UCCGGCA	10.12±0.19	56.1	0	2.29	50.5 ± 3.1 (46.0 ± 4.5)	99.6 ± 1.3 (-5.7 ± 7.9)	49.1 ± 3.4	50%	32%	18% (75/50)
CGAAGGCCGUGUG CUCCGGCAC Ia2	17.04±0.65	80.1	CGAAGGUCGUGUG CUCCGGCAC	16.56±0.09	69.5	(0)	0.48	21.3 ± 1.6 (75.0 ± 3.7)	81.9 ± 2.0 (12.0 ± 8.0)	60.6 ± 2.6	46%	19%	27% (25/50)
CGAAGGCCGUGUG GCUTTCAGCAC aKM	17.62±0.34	71.2	CGAAGGUCGUGUG GCUTTCAGCAC	20.24±0.15	78.7	7.83 (3.20)	-2.62	96.3 ± 3.3 (0.0)	93.9 ± 7.4 (0.0)	-2.4 ± 8.1	110%	55%	55% (50)
<i>Transversion G/C (G291C, SCA3)</i>													
GCAGGGGACCUG GUCCCC If1	14.41 ± 1.13	68.8	GCAGCGGACCUG GUCCCC	6.47 ± 0.05	36.5	0	7.94	11.7 ± 2.3 (58.4 ± 3.4)	31.1 ± 1.9 (2.6 ± 2.1)	19.4 ± 3.0	-	-	-
GCAGGGGACCUG CGUCCCCUG If2	20.91 ± 0.87	82.7	GCAGCGGACCUG CGUCCCCUG	11.35 ± 0.07	59.2	(0)	9.56	0	2.7 ± 1.5 (31.0 ± 1.9)	2.7	-	-	-
GCAGGGGACCUG CGUCGCCTGGAU fKM	17.94 ± 0.75	66.5	GCAGCGGACCUG CGUCGCCTGGAU	27.7 ± 2.9	80.3	13.29 (6.79)	-9.76	70.1 ± 2.5 (0.0)	33.7 ± 1.1 (0.0)	-36.4 ± 2.7	79%	13%	66% (50)

a – melting temperature of the duplex at concentration 10⁻⁴M, b – differences in thermodynamic stability of the WT RNA/short inhibitor (Ix1) and Mut RNA/gapmer (xKM) duplexes, c – differences in thermodynamic stability of WT RNA/longer inhibitor (Ix2) and Mut RNA/gapmer (xKM) duplexes, d – errors calculated with standard deviation, e – for cleavage of WT or Mut RNA shorter inhibitors and gapmer were used, f – differences in hydrolysis of WT or Mut RNA in presence of gapmer only and the appropriate RNA in the presence of both the gapmer and shorter inhibitor, g – for cleavage of WT or Mut RNA the longer inhibitor and gapmer were used, h – differences in hydrolysis of WT or Mut RNA in the presence of gapmer only and the appropriate RNA in the presence of both the gapmer and longer inhibitor, i – for cleavage of WT or Mut RNA only the gapmer was used, j – the abbreviation 25/50 refers to transfection of the *HeLa* cells with 25 nM and 50 nM concentrations of inhibitor and gapmer, respectively, k – the abbreviation 50 refers to transfection of the *HeLa* cells with 50 nM of gapmer only, empty cells (-) – statistically insignificant results were obtained. Underlined letters indicated the position of the SNP, red letters corresponded to 2'-O-methylated RNA, blue letters indicated the position of the LNA nucleotides, the bolded region corresponded to the DNA fragment within the gapmer, and the remaining letters correspond to the RNA.