OMTN, Volume 21

Supplemental Information

Antisense Oligonucleotide Therapeutic

Approach for Suppression of Ataxin-1

Expression: A Safety Assessment

Brennon O'Callaghan, Bente Hofstra, Hillary P. Handler, Holly B. Kordasiewicz, Tracy Cole, Lisa Duvick, Jillian Friedrich, Orion Rainwater, Praseuth Yang, Michael Benneyworth, Tessa Nichols-Meade, Wesley Heal, Rachel Ter Haar, Christine Henzler, and Harry T. Orr

Supplemental information

Table S1: RT-qPCR Primers

Figure S1

Figure S2

Figure S3

Table S1: RT-qPCR Primers

<u>Primer</u>	Sequence	Probe	Roche Catalog #
mDcx1 1171F	AGCTGACTCAGGTAACGACCA	11	UPL 04685105001
mDcx1 1241R	GCTTTGACTTAGGTGTTGAGAGC		
mBace1 809F	TGCATCGCTACTACCAGAGG	104	UPL 04682225001
mBace1 934R	CATGAGGGATGCTCACCAG		
mCcnd1 918F	CAACGCACTTTCTTTCCAGAG	16	UPL 04686896001
mCcnd1 993R	AGGGCTTCAATCTGTTCCTG		
mGfra1 1675R	TGCCCGATACATTGGATTTC	98	UPL 04692152001
mGfra1 1607F	TTCCCACACACGTTTACCA		
mSpred1 664F	GAGACGGCGACTTCTGACA	9	UPL 04685075001
mSpred1 749R	AGCCATCCACCACTTGAGTC		
mEtv5 407R	AGCTCCCGTTTGATCTTGG	10	UPL 04685091001
mEtv5 330F	TGAGCAGTTTGTCCCAGATTT		
mAtxn1 F	CACGGTCATTCAGACCACACA	FAM-CAGCCACGG/ZEM	V/CCTTCTACGCTGG-IABKFQ
mAtxn1 R	GGTAGCCGATGACAGGAGGTT		
mGapd	Roche		5046211001



Fig. S1 Three ICV injections of *Atxn1* targeting ASO stabilized motor performance and improved life span but did not improve failure to gain weight. (A) Motor performance on an accelerating rotarod at ages 5, 12 and 25 weeks. Data are presented as the mean ± SEM. **P < 0.01, ***P < 0.001, 1-way ANOVA. (B) Kaplan-Meyer curves depicting mouse survival. (C) Failure-to-gain weight phenotype. Weights measured weekly.



Fig. S2 RT qPCR of 2Q/2Q mice; untreated, non-targeting ASO and Atxn1 targeting ASO was performed for four representative CIC influenced genes associated with gliomas. 2-way ANOVA showed no significant difference between groups



Fig. S3 Protein dilution Western blot of ATXN1, BACE1, and TUBULIN.