Nicolas Wein

List of Publications by Year in descending order

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NICOLAS WEIN

#	Article	IF	CITATIONS
1	Translation from a DMD exon 5 IRES results in a functional dystrophin isoform that attenuates dystrophinopathy in humans and mice. Nature Medicine, 2014, 20, 992-1000.	30.7	113
2	Efficient bypass of mutations in dysferlin deficient patient cells by antisense-induced exon skipping. Human Mutation, 2010, 31, 136-142.	2.5	80
3	Genetics and Emerging Treatments for Duchenne and Becker Muscular Dystrophy. Pediatric Clinics of North America, 2015, 62, 723-742.	1.8	71
4	A Naturally Occurring Human Minidysferlin Protein Repairs Sarcolemmal Lesions in a Mouse Model of Dysferlinopathy. Science Translational Medicine, 2010, 2, 50ra69.	12.4	69
5	Personalized gene and cell therapy for Duchenne Muscular Dystrophy. Neuromuscular Disorders, 2018, 28, 803-824.	0.6	45
6	Clinical phenotypes as predictors of the outcome of skipping around <scp><i>DMD</i></scp> exon 45. Annals of Neurology, 2015, 77, 668-674.	5.3	38
7	Translational Research and Therapeutic Perspectives in Dysferlinopathies. Molecular Medicine, 2011, 17, 875-882.	4.4	36
8	UMD-DYSF, a novel locus specific database for the compilation and interactive analysis of mutations in the dysferlin gene. Human Mutation, 2012, 33, E2317-E2331.	2.5	35
9	Exon 32 Skipping of Dysferlin Rescues Membrane Repair in Patients' Cells. Journal of Neuromuscular Diseases, 2015, 2, 281-290.	2.6	29
10	Lack of Toxicity in Nonhuman Primates Receiving Clinically Relevant Doses of an AAV9.U7snRNA Vector Designed to Induce <i>DMD</i> Exon 2 Skipping. Human Gene Therapy, 2021, 32, 882-894.	2.7	29
11	Dp412e: a novel human embryonic dystrophin isoform induced by BMP4 in early differentiated cells. Skeletal Muscle, 2015, 5, 40.	4.2	28
12	The first exon duplication mouse model of Duchenne muscular dystrophy: A tool for therapeutic development. Neuromuscular Disorders, 2015, 25, 827-834.	0.6	27
13	Efficient Skipping of Single Exon Duplications in DMD Patient-Derived Cell Lines Using an Antisense Oligonucleotide Approach. Journal of Neuromuscular Diseases, 2017, 4, 199-207.	2.6	27
14	Pre-clinical dose-escalation studies establish a therapeutic range for U7snRNA-mediated DMD exon 2 skipping. Molecular Therapy - Methods and Clinical Development, 2021, 21, 325-340.	4.1	21
15	Identification of Different Genomic Deletions and One Duplication in the Dysferlin Gene Using Multiplex Ligation-Dependent Probe Amplification and Genomic Quantitative PCR. Genetic Testing and Molecular Biomarkers, 2009, 13, 439-442.	0.7	18
16	Designed U7 snRNAs inhibitÂDUX4Âexpression and improve FSHD-associated outcomes inÂDUX4Âoverexpressing cells and FSHD patient myotubes. Molecular Therapy - Nucleic Acids, 2021, 23, 476-486.	5.1	17
17	Intron mutations and early transcription termination in Duchenne and Becker muscular dystrophy. Human Mutation, 2022, 43, 511-528.	2.5	16
18	Fullâ€length dysferlin expression driven by engineered human dystrophic blood derived <scp>CD</scp> 133+ stem cells. FEBS Journal, 2013, 280, 6045-6060.	4.7	12

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19	X-linked muscular dystrophy in a Labrador Retriever strain: phenotypic and molecular characterisation. Skeletal Muscle, 2020, 10, 23.	4.2	12
20	Absence of Significant Off-Target Splicing Variation with a U7snRNA Vector Targeting <i>DMD</i> Exon 2 Duplications. Human Gene Therapy, 2021, 32, 1346-1359.	2.7	8
21	Therapeutic exon â€~switching' for dysferlinopathies?. European Journal of Human Genetics, 2010, 18, 969-970.	2.8	7
22	U7 snRNA, a Small RNA with a Big Impact in Gene Therapy. Human Gene Therapy, 2021, 32, 1317-1329.	2.7	5
23	Becker muscular dystrophy with widespread muscle hypertrophy and a non-sense mutation of exon 2. Neuromuscular Disorders, 2013, 23, 192.	0.6	1
24	Direct Reprogramming of Human Fibroblasts into Myoblasts to Investigate Therapies for Neuromuscular Disorders. Journal of Visualized Experiments, 2021, , .	0.3	0
25	Alternate Translational Initiation of Dystrophin: A Novel Therapeutic Approach. , 2019, , 371-382.		0