Antisense-induced exon skipping for duplications in Duchenne muscular dystrophy

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#	Paper	IF	Citations
53	Antisense-mediated exon skipping: a versatile tool with therapeutic and research applications. *Rna*, **2007**, 13, 1609-24	5.8	181
52	Assessment of the feasibility of exon 45-55 multiexon skipping for Duchenne muscular dystrophy. *BMC Medical Genetics*, **2008**, 9, 105	2.1	43
51	Nanopolymers improve delivery of exon skipping oligonucleotides and concomitant dystrophin expression in skeletal muscle of mdx mice. *BMC Biotechnology*, **2008**, 8, 35	3.5	27
50	Microarray-based mutation detection in the dystrophin gene. *Human Mutation*, **2008**, 29, 1091-9	4.7	93
49	Molecular-targeted therapy for Duchenne muscular dystrophy: progress and potential. *Molecular Diagnosis and Therapy*, **2008**, 12, 99-108	4.5	7
48	Efficacy of systemic morpholino exon-skipping in Duchenne dystrophy dogs. *Annals of Neurology*, **2009**, 65, 667-76	9.4	324
47	Theoretic applicability of antisense-mediated exon skipping for Duchenne muscular dystrophy mutations. *Human Mutation*, **2009**, 30, 293-9	4.7	393
46	RNA-targeting approaches for neuromuscular diseases. *Trends in Molecular Medicine*, **2009**, 15, 580-91	11.5	22
45	Interventions for muscular dystrophy: molecular medicines entering the clinic. *Lancet, The*, **2009**, 374, 1849-56	40	36
44	Exon-skipped dystrophins for treatment of Duchenne muscular dystrophy: mass spectrometry mapping of most exons and cooperative domain designs based on single molecule mechanics. *Cytoskeleton*, **2010**, 67, 796-807	2.4	15
43	Progress in therapeutic antisense applications for neuromuscular disorders. *European Journal of Human Genetics*, **2010**, 18, 146-53	5.3	31
42	Therapeutic exon skipping for dysferlinopathies?. *European Journal of Human Genetics*, **2010**, 18, 889-94	5.3	43
41	Reply to L□y et al. *European Journal of Human Genetics*, **2010**, 18, 971-971	5.3	2
40	New insights in gene-derived therapy: the example of Duchenne muscular dystrophy. *Annals of the New York Academy of Sciences*, **2010**, 1214, 199-212	6.5	18
39	Dystrophin isoform induction in vivo by antisense-mediated alternative splicing. *Molecular Therapy*, **2010**, 18, 1218-23	11.7	17
38	Comparative analysis of antisense oligonucleotide sequences targeting exon 53 of the human DMD gene: Implications for future clinical trials. *Neuromuscular Disorders*, **2010**, 20, 102-10	2.9	37
37	Personalized exon skipping strategies to address clustered non-deletion dystrophin mutations. *Neuromuscular Disorders*, **2010**, 20, 810-6	2.9	13

36	Bioinformatic and functional optimization of antisense phosphorodiamidate morpholino oligomers (PMOs) for therapeutic modulation of RNA splicing in muscle. *Methods in Molecular Biology*, **2011**, 709, 153-78	1.4	9
35	Dystrophinopathies. *Handbook of Clinical Neurology / Edited By P J Vinken and G W Bruyn*, **2011**, 101, 11-39		14
34	Three gene-targeted mouse models of RNA splicing factor RP show late-onset RPE and retinal degeneration. **2011**, 52, 190-8		49
33	Impending therapies for Duchenne muscular dystrophy. *Current Opinion in Neurology*, **2011**, 24, 415-22	7.1	42
32	Bodywide skipping of exons 45-55 in dystrophic mdx52 mice by systemic antisense delivery. *Proceedings of the National Academy of Sciences of the United States of America*, **2012**, 109, 13763-8	11.5	118
31	Exon skipping for nonsense mutations in Duchenne muscular dystrophy: too many mutations, too few patients?. *Expert Opinion on Biological Therapy*, **2012**, 12, 1141-52	5.4	32
30	Overview on DMD exon skipping. *Methods in Molecular Biology*, **2012**, 867, 97-116	1.4	39
29	Engineering multiple U7snRNA constructs to induce single and multiexon-skipping for Duchenne muscular dystrophy. *Molecular Therapy*, **2012**, 20, 1212-21	11.7	40
28	Optimizing antisense oligonucleotides using phosphorodiamidate morpholino oligomers. *Methods in Molecular Biology*, **2012**, 867, 143-67	1.4	7
27	Splicing therapy for neuromuscular disease. *Molecular and Cellular Neurosciences*, **2013**, 56, 169-85	4.8	64
26	Rescue of cardiomyopathy through U7snRNA-mediated exon skipping in Mybpc3-targeted knock-in mice. *EMBO Molecular Medicine*, **2013**, 5, 1128-45	12	66
25	Targeted exon skipping to correct exon duplications in the dystrophin gene. *Molecular Therapy - Nucleic Acids*, **2014**, 3, e155	10.7	25
24	New developments in the use of gene therapy to treat Duchenne muscular dystrophy. *Expert Opinion on Biological Therapy*, **2014**, 14, 209-30	5.4	50
23	Skipping multiple exons of dystrophin transcripts using cocktail antisense oligonucleotides. *Nucleic Acid Therapeutics*, **2014**, 24, 57-68	4.8	46
22	Molecular diagnosis of dystrophinopathies using a multi-technique analysis algorithm. *Muscle and Nerve*, **2014**, 49, 249-56	3.4	5
21	The first exon duplication mouse model of Duchenne muscular dystrophy: A tool for therapeutic development. *Neuromuscular Disorders*, **2015**, 25, 827-34	2.9	15
20	Joint mouse-human phenome-wide association to test gene function and disease risk. *Nature Communications*, **2016**, 7, 10464	17.4	91
19	Correction of the Exon 2 Duplication in DMD Myoblasts by a Single CRISPR/Cas9 System. *Molecular Therapy - Nucleic Acids*, **2017**, 7, 11-19	10.7	27

18	Exon skipping: a first in class strategy for Duchenne muscular dystrophy. *Expert Opinion on Biological Therapy*, **2017**, 17, 225-236	5.4	75
17	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	5	23
16	Quantitative Antisense Screening and Optimization for Exon 51 Skipping in Duchenne Muscular Dystrophy. *Molecular Therapy*, **2017**, 25, 2561-2572	11.7	51
15	Immortalized Muscle Cell Model to Test the Exon Skipping Efficacy for Duchenne Muscular Dystrophy. *Journal of Personalized Medicine*, **2017**, 7,	3.6	25
14	Invention and Early History of Exon Skipping and Splice Modulation. *Methods in Molecular Biology*, **2018**, 1828, 3-30	1.4	13
13	Antisense oligonucleotides in neurological disorders. *Therapeutic Advances in Neurological Disorders* , **2018**, 11, 1756286418776932	6.6	67
12	Splice-Switching Oligonucleotides. **2018**, 445-489		
11	Nonclinical Exon Skipping Studies with 2′-O-Methyl Phosphorothioate Antisense Oligonucleotides in mdx and mdx-utrn-/- Mice Inspired by Clinical Trial Results. *Nucleic Acid Therapeutics*, **2019**, 29, 92-103	4.8	6
10	Mutational spectrum of dystrophinopathies in Singapore: Insights for genetic diagnosis and precision therapy. *American Journal of Medical Genetics, Part C: Seminars in Medical Genetics*, **2019**, 181, 230-244	3.1	3
9	Single Exon Skipping Can Address a Multi-Exon Duplication in the Dystrophin Gene. *International Journal of Molecular Sciences*, **2020**, 21,	6.3	1
8	Developments in reading frame restoring therapy approaches for Duchenne muscular dystrophy. *Expert Opinion on Biological Therapy*, **2021**, 21, 343-359	5.4	8
7	Iranian Registry of Duchenne and Becker Muscular Dystrophies: Characterization and Preliminary Data. *Journal of Neuromuscular Diseases*, **2021**, 8, 251-259	5	0
6	Gene-Targeting Therapeutics for Neurological Disease: Lessons Learned from Spinal Muscular Atrophy. *Annual Review of Medicine*, **2021**, 72, 1-14	17.4	5
5	Targeted genome editing in vivo corrects a Dmd duplication restoring wild-type dystrophin expression. *EMBO Molecular Medicine*, **2021**, 13, e13228	12	3
4	CRISPR-Cas Gene Perturbation and Editing in Human Induced Pluripotent Stem Cells. *CRISPR Journal*, **2021**, 4, 634-655	2.5	0
3	Antisense-Mediated Exon Skipping for Duchenne Muscular Dystrophy. **2010**, 69-84		1
2	Optimizing exon skipping therapies for DMD. *Acta Myologica*, **2007**, 26, 179-84	1.6	48
1	Transiently expressed CRISPR/Cas9 induces wild-type dystrophin in vitro in DMD patient myoblasts carrying duplications.. *Scientific Reports*, **2022**, 12, 3756	4.9	0