Annemieke Aartsma-Rus

List of Publications by Year
in descending order

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200	13,149	29994 54	26548 107
papers	citations	h-index	g-index

217	217	217	10915
all docs	docs citations	times ranked	citing authors

#	Article	IF	Citations
1	Preparing n-of-1 Antisense Oligonucleotide Treatments for Rare Neurological Diseases in Europe: Genetic, Regulatory, and Ethical Perspectives. Nucleic Acid Therapeutics, 2022, 32, 83-94.	2.0	35
2	The Role of Patient Involvement When Developing Therapies. Nucleic Acid Therapeutics, 2022, 32, 118-122.	2.0	2
3	In Vitro Delivery of PMOs in Myoblasts by Electroporation. Methods in Molecular Biology, 2022, 2434, 191-205.	0.4	3
4	The therapeutic potential of soluble activin type IIB receptor treatment in a limb girdle muscular dystrophy type 2D mouse model. Neuromuscular Disorders, 2022, 32, 419-435.	0.3	1
5	Letter by Duan et al Regarding Article, â€œTherapeutic Exon Skipping Through a CRISPR-Guided Cytidine Deaminase Rescues Dystrophic Cardiomyopathy In Vivoâ€• Circulation, 2022, 145, e872-e873.	1.6	0
6	Opportunities and challenges for antisense oligonucleotide therapies. Journal of Inherited Metabolic Disease, 2021, 44, 72-87.	1.7	86
7	Developments in reading frame restoring therapy approaches for Duchenne muscular dystrophy. Expert Opinion on Biological Therapy, 2021, 21, 343-359.	1.4	20
8	Eighteen-Year Disease Progression and Survival in CADASIL. Journal of Stroke, 2021, 23, 132-134.	1.4	8
9	Duchenne muscular dystrophy. Nature Reviews Disease Primers, 2021, 7, 13.	18.1	448
10	Peripheral blood transcriptome profiling enables monitoring disease progression in dystrophic mice and patients. EMBO Molecular Medicine, 2021, 13, e13328.	3.3	16
11	Delivery of oligonucleotideâ€based therapeutics: challenges and opportunities. EMBO Molecular Medicine, 2021, 13, e13243.	3.3	181
12	â€˜N of 1â€™ therapies need a better model. Nature Medicine, 2021, 27, 939-939.	15.2	11
13	Low human dystrophin levels prevent cardiac electrophysiological and structural remodelling in a Duchenne mouse model. Scientific Reports, 2021, 11, 9779.	1.6	6
14	Plasma lipidomic analysis shows a disease progression signature in mdx mice. Scientific Reports, 2021, 11, 12993.	1.6	7
15	Authorâ€™s Response to: Rebuttal to: Simvastatin Treatment Does Not Ameliorate Muscle Pathophysiology in a Mouse Model for Duchenne Muscular Dystrophy, Verhaart et al. 2020. Journal of Neuromuscular Diseases, 2021, 8, 867-868.	1.1	1
16	Case Report: The Genetic Diagnosis of Duchenne Muscular Dystrophy in the Middle East. Frontiers in Pediatrics, 2021, 9, 716424.	0.9	7
17	Sharing â€œNegativeâ€•Results in Neuromuscular Research: A Positive Experience. Journal of Neuromuscular Diseases, 2021, 8, 765-767.	1.1	2
18	The RNA-binding profile of the splicing factor SRSF6 in immortalized human pancreatic Î²-cells. Life Science Alliance, 2021, 4, e202000825.	1.3	14

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19	7â€²,5â€²-alpha-bicyclo-DNA: new chemistry for oligonucleotide exon splicing modulation therapy. Nucleic Acids Research, 2021, 49, 12089-12105.	6.5	6
20	Orphan Medicine Incentives: How to Address the Unmet Needs of Rare Disease Patients by Optimizing the European Orphan Medicinal Product Landscape Guiding Principles and Policy Proposals by the European Expert Group for Orphan Drug Incentives (OD Expert Group). Frontiers in Pharmacology, 2021, 12, 744532.	1.6	18
21	Progression and Classification of Granular Osmiophilic Material (GOM) Deposits in Functionally Characterized Human NOTCH3 Transgenic Mice. Translational Stroke Research, 2020, 11, 517-527.	2.3	16
22	Antisense-Mediated Skipping of Dysferlin Exons in Control and Dysferlinopathy Patient-Derived Cells. Nucleic Acid Therapeutics, 2020, 30, 71-79.	2.0	4
23	Longitudinal serum biomarker screening identifies malate dehydrogenase 2 as candidate prognostic biomarker for Duchenne muscular dystrophy. Journal of Cachexia, Sarcopenia and Muscle, 2020, 11, 505-517.	2.9	27
24	Uniform sarcolemmal dystrophin expression is required to prevent extracellular microRNA release and improve dystrophic pathology. Journal of Cachexia, Sarcopenia and Muscle, 2020, 11, 578-593.	2.9	24
25	TCTEX1D1 is a genetic modifier of disease progression in Duchenne muscular dystrophy. European Journal of Human Genetics, 2020, 28, 815-825.	1.4	36
26	Sensitive and reliable evaluation of single-cut sgRNAs to restore dystrophin by a GFP-reporter assay. PLoS ONE, 2020, 15, e0239468.	1.1	8
27	Simvastatin Treatment Does Not Ameliorate Muscle Pathophysiology in a Mouse Model for Duchenne Muscular Dystrophy. Journal of Neuromuscular Diseases, 2020, 8, 1-19.	1.1	9
28	Improving translatability of preclinical studies for neuromuscular disorders: lessons from the TREAT-NMD Advisory Committee for Therapeutics (TACT). DMM Disease Models and Mechanisms, 2020, 13, .	1.2	18
29	The mRNA Binding Proteome of Proliferating and Differentiated Muscle Cells. Genomics, Proteomics and Bioinformatics, 2020, 18, 384-396.	3.0	5
30	Blood-derived biomarkers correlate with clinical progression in Duchenne muscular dystrophy. Journal of Neuromuscular Diseases, 2020, 7, 231-246.	1.1	20
31	Implications of increased S100Î² and Tau5 proteins in dystrophic nerves of two mdx mouse models for Duchenne muscular dystrophy. Molecular and Cellular Neurosciences, 2020, 105, 103484.	1.0	5
32	Premature termination codons in the DMD gene cause reduced local mRNA synthesis. Proceedings of the National Academy of Sciences of the United States of America, 2020, 117, 16456-16464.	3.3	30
33	Tumor Necrosis Factor Receptor SF10A (TNFRSF10A) SNPs Correlate With Corticosteroid Response in Duchenne Muscular Dystrophy. Frontiers in Genetics, 2020, 11, 605.	1.1	9
34	Longitudinal metabolomic analysis of plasma enables modeling disease progression in Duchenne muscular dystrophy mouse models. Human Molecular Genetics, 2020, 29, 745-755.	1.4	19
35	The 10th Oligonucleotide Therapy Approved: Golodirsen for Duchenne Muscular Dystrophy. Nucleic Acid Therapeutics, 2020, 30, 67-70.	2.0	82
36	Naturally occurring NOTCH3 exon skipping attenuates NOTCH3 protein aggregation and disease severity in CADASIL patients. Human Molecular Genetics, 2020, 29, 1853-1863.	1.4	12

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37	The use of genetically humanized animal models for personalized medicine approaches. DMM Disease Models and Mechanisms, 2020, 13, dmm041673.	1.2	22
38	Moving neuromuscular disorders research forward: from novel models to clinical studies. DMM Disease Models and Mechanisms, 2020, 13, .	1.2	6
39	Pathological mechanism and antisense oligonucleotide-mediated rescue of a non-coding variant suppressing factor 9 RNA biogenesis leading to hemophilia B. PLoS Genetics, 2020, 16, e1008690.	1.5	4
40	Assessment of Behavioral Characteristics With Procedures of Minimal Human Interference in the mdx Mouse Model for Duchenne Muscular Dystrophy. Frontiers in Behavioral Neuroscience, 2020, 14, 629043.	1.0	3
41	Detailed genetic and functional analysis of the hDMDdel52/mdx mouse model. PLoS ONE, 2020, 15, e0244215.	1.1	15
42	Title is missing!. , 2020, 16, e1008690.		0
43	Title is missing!. , 2020, 16, e1008690.		0
44	Title is missing!. , 2020, 16, e1008690.		0
45	Title is missing!. , 2020, 16, e1008690.		0
46	Title is missing!. , 2020, 16, e1008690.		0
47	Title is missing!. , 2020, 16, e1008690.		0
48	Dystrophin deficiency leads to dysfunctional glutamate clearance in iPSC derived astrocytes. Translational Psychiatry, 2019, 9, 200.	2.4	18
49	Phenotype predictions for exon deletions/duplications: A user guide for professionals and clinicians using Becker and Duchenne muscular dystrophy as examples. Human Mutation, 2019, 40, 1630-1633.	1.1	8
50	Nonclinical Exon Skipping Studies with 2â€²-O-Methyl Phosphorothioate Antisense Oligonucleotides in mdx and mdx-utrnâ^'/â^' Mice Inspired by Clinical Trial Results. Nucleic Acid Therapeutics, 2019, 29, 92-103.	2.0	9
51	What We Have Learned from 10 Years of DMD Exon-Skipping Trials. , 2019, , 745-758.		0
52	Therapeutic developments for Duchenne muscular dystrophy. Nature Reviews Neurology, 2019, 15, 373-386.	4.9	265
53	Advancing Nucleic Acid Therapeutics by Setting Uniform Standards for Experimental Controls. Nucleic Acid Therapeutics, 2019, 29, 115-115.	2.0	0
54	A modified diet does not ameliorate muscle pathology in a mouse model for Duchenne muscular dystrophy. PLoS ONE, 2019, 14, e0215335.	1.1	2

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55	Natural disease history of the D2â€mdx mouse model for Duchenne muscular dystrophy. FASEB Journal, 2019, 33, 8110-8124.	0.2	88
56	Evidence-Based Consensus and Systematic Review on Reducing the Time to Diagnosis of Duchenne Muscular Dystrophy. Journal of Pediatrics, 2019, 204, 305-313.e14.	0.9	24
57	A Sequel to the Eteplirsen Saga: Eteplirsen Is Approved in the United States but Was Not Approved in Europe. Nucleic Acid Therapeutics, 2019, 29, 13-15.	2.0	42
58	Serum Neurofilament light correlates with CADASIL disease severity and survival. Annals of Clinical and Translational Neurology, 2019, 6, 46-56.	1.7	24
59	RD-Connect, NeurOmics and EURenOmics: collaborative European initiative for rare diseases. European Journal of Human Genetics, 2018, 26, 778-785.	1.4	55
60	Tracking disease progression nonâ€invasively in Duchenne and Becker muscular dystrophies. Journal of Cachexia, Sarcopenia and Muscle, 2018, 9, 715-726.	2.9	47
61	Low dystrophin levels are insufficient to normalize the neuromuscular synaptic abnormalities of mdx mice. Neuromuscular Disorders, 2018, 28, 427-442.	0.3	15
62	Crossâ€sectional serum metabolomic study of multiple forms of muscular dystrophy. Journal of Cellular and Molecular Medicine, 2018, 22, 2442-2448.	1.6	25
63	Measuring DNA hybridization using fluorescent DNA-stabilized silver clusters to investigate mismatch effects on therapeutic oligonucleotides. Journal of Nanobiotechnology, 2018, 16, 37.	4.2	5
64	Cyclic Peptides to Improve Delivery and Exon Skipping of Antisense Oligonucleotides in a Mouse Model for Duchenne Muscular Dystrophy. Molecular Therapy, 2018, 26, 132-147.	3.7	19
65	Genetic therapies for spinal muscular atrophy type 1. Lancet Neurology, The, 2018, 17, 111-112.	4.9	7
66	227 th ENMC International Workshop:. Neuromuscular Disorders, 2018, 28, 185-192.	0.3	5
67	A multicenter comparison of quantification methods for antisense oligonucleotide-induced DMD exon 51 skipping in Duchenne muscular dystrophy cell cultures. PLoS ONE, 2018, 13, e0204485.	1.1	14
68	Voluntary exercise improves muscle function and does not exacerbate muscle and heart pathology in aged Duchenne muscular dystrophy mice. Journal of Molecular and Cellular Cardiology, 2018, 125, 29-38.	0.9	15
69	Exon 51 Skipping Quantification by Digital Droplet PCR in del52hDMD/mdx Mice. Methods in Molecular Biology, 2018, 1828, 249-262.	0.4	4
70	Why dystrophin quantification is key in the eteplirsen saga. Nature Reviews Neurology, 2018, 14, 454-456.	4.9	20
71	Influence of full-length dystrophin on brain volumes in mouse models of Duchenne muscular dystrophy. PLoS ONE, 2018, 13, e0194636.	1.1	10
72	A dystrophic Duchenne mouse model for testing human antisense oligonucleotides. PLoS ONE, 2018, 13, e0193289.	1.1	44

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73	Delivery is key: lessons learnt from developing spliceâ€switching antisense therapies. EMBO Molecular Medicine, 2017, 9, 545-557.	3.3	119
74	FDA Approval of Nusinersen for Spinal Muscular Atrophy Makes 2016 the Year of Splice Modulating Oligonucleotides. Nucleic Acid Therapeutics, 2017, 27, 67-69.	2.0	144
75	Stakeholder collaboration for spinal muscular atrophy therapy development. Lancet Neurology, The, 2017, 16, 264.	4.9	10
76	FDA Approves Eteplirsen for Duchenne Muscular Dystrophy: The Next Chapter in the Eteplirsen Saga. Nucleic Acid Therapeutics, 2017, 27, 1-3.	2.0	284
77	Exon skipping: a first in class strategy for Duchenne muscular dystrophy. Expert Opinion on Biological Therapy, 2017, 17, 225-236.	1.4	97
78	Translational Research in Europe for the Assessment and Treatment for Neuromuscular Disorders (TREAT-NMD). Neuropediatrics, 2017, 48, 211-220.	0.3	9
79	Development of Exon Skipping Therapies for Duchenne Muscular Dystrophy: A Critical Review and a Perspective on the Outstanding Issues. Nucleic Acid Therapeutics, 2017, 27, 251-259.	2.0	144
80	Cytokine Profiling of Serum Allows Monitoring of Disease Progression in Inclusion Body Myositis. Journal of Neuromuscular Diseases, 2017, 4, 327-335.	1.1	8
81	Mimicking Cardiac Fibrosis in a Dish: Fibroblast Density Rather than Collagen Density Weakens Cardiomyocyte Function. Journal of Cardiovascular Translational Research, 2017, 10, 116-127.	1.1	38
82	New function of the myostatin/activin type I receptor (ALK4) as a mediator of muscle atrophy and muscle regeneration. FASEB Journal, 2017, 31, 238-255.	0.2	24
83	<scp>RNA</scp>â€based therapies for genodermatoses. Experimental Dermatology, 2017, 26, 3-10.	1.4	28
84	Prevalence, incidence and carrier frequency of 5qâ€“linked spinal muscular atrophy â€“ a literature review. Orphanet Journal of Rare Diseases, 2017, 12, 124.	1.2	391
85	Comparative mass spectrometric and immunoassayâ€based proteome analysis in serum of Duchenne muscular dystrophy patients. Proteomics - Clinical Applications, 2016, 10, 290-299.	0.8	27
86	Accurate Dystrophin Quantification in Mouse Tissue; Identification of New and Evaluation of Existing Methods. Journal of Neuromuscular Diseases, 2016, 3, 77-90.	1.1	13
87	Environmental 24-hr Cycles Are Essential for Health. Current Biology, 2016, 26, 1843-1853.	1.8	101
88	Association Study of Exon Variants in the NF-Î²B and TGFÎ² Pathways Identifies CD40 as a Modifier of Duchenne Muscular Dystrophy. American Journal of Human Genetics, 2016, 99, 1163-1171.	2.6	71
89	Antisense Oligonucleotide-mediated Exon Skipping as a Systemic Therapeutic Approach for Recessive Dystrophic Epidermolysis Bullosa. Molecular Therapy - Nucleic Acids, 2016, 5, e379.	2.3	59
90	Characterization of neuromuscular synapse function abnormalities in multiple Duchenne muscular dystrophy mouse models. European Journal of Neuroscience, 2016, 43, 1623-1635.	1.2	59

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91	Stakeholder cooperation to overcome challenges in orphan medicine development: the example of Duchenne muscular dystrophy. Lancet Neurology, The, 2016, 15, 882-890.	4.9	77
92	The importance of genetic diagnosis for Duchenne muscular dystrophy. Journal of Medical Genetics, 2016, 53, 145-151.	1.5	242
93	Non-sequential and multi-step splicing of the dystrophin transcript. RNA Biology, 2016, 13, 290-305.	1.5	52
94	New Momentum for the Field of Oligonucleotide Therapeutics. Molecular Therapy, 2016, 24, 193-194.	3.7	32
95	Therapeutic NOTCH3 cysteine correction in CADASIL using exon skipping:in vitroproof of concept. Brain, 2016, 139, 1123-1135.	3.7	43
96	In-Depth Characterization of Protein Disulfide Bonds by Online Liquid Chromatography-Electrochemistry-Mass Spectrometry. Journal of the American Society for Mass Spectrometry, 2016, 27, 50-58.	1.2	18
97	Evaluation of 2â€™-Deoxy-2â€™-fluoro Antisense Oligonucleotides for Exon Skipping in Duchenne Muscular Dystrophy. Molecular Therapy - Nucleic Acids, 2015, 4, e265.	2.3	20
98	Antisenseâ€mediated exon skipping: a therapeutic strategy for titinâ€based dilated cardiomyopathy. EMBO Molecular Medicine, 2015, 7, 562-576.	3.3	94
99	The NOTCH3 score: a pre-clinical CADASIL biomarker in a novel human genomic NOTCH3 transgenic mouse model with early progressive vascular NOTCH3 accumulation. Acta Neuropathologica Communications, 2015, 3, 89.	2.4	20
100	An update on RNA-targeting therapies for neuromuscular disorders. Current Opinion in Neurology, 2015, 28, 515-521.	1.8	18
101	Circulating Biomarkers for Duchenne Muscular Dystrophy. Journal of Neuromuscular Diseases, 2015, 2, S49-S58.	1.1	20
102	Response to: Studying the role of dystrophin-associated proteins in influencing Becker muscular dystrophy disease severity. Neuromuscular Disorders, 2015, 25, 530-531.	0.3	1
103	The Pathogenesis and Therapy of Muscular Dystrophies. Annual Review of Genomics and Human Genetics, 2015, 16, 281-308.	2.5	240
104	The dystrophin gene and cognitive function in the general population. European Journal of Human Genetics, 2015, 23, 837-843.	1.4	6
105	The TREAT-NMD DMD Global Database: Analysis of More than 7,000 Duchenne Muscular Dystrophy Mutations. Human Mutation, 2015, 36, 395-402.	1.1	507
106	Response to: Evaluation of the serum matrix metalloproteinase-9 as a biomarker for monitoring disease progression in Duchenne muscular dystrophy. Neuromuscular Disorders, 2015, 25, 446-447.	0.3	3
107	Studying the role of dystrophin-associated proteins in influencing Becker muscular dystrophy disease severity. Neuromuscular Disorders, 2015, 25, 231-237.	0.3	11
108	SlicePie: a novel analytical approach for the detection of alternative, non-sequential and recursive splicing. Nucleic Acids Research, 2015, 43, e80-e80.	6.5	17

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109	Measuring clinical effectiveness of medicinal products for the treatment of Duchenne muscular dystrophy. Neuromuscular Disorders, 2015, 25, 96-105.	0.3	39
110	Imperatives for DUCHENNE MD: a Simplified Guide to Comprehensive Care for Duchenne Muscular Dystrophy. PLOS Currents, 2015, 7, .	1.4	16
111	Splicing modulation therapy in the treatment of genetic diseases. The Application of Clinical Genetics, 2014, 7, 245.	1.4	33
112	The Dynamics of Compound, Transcript, and Protein Effects After Treatment With 2OMePS Antisense Oligonucleotides in mdx Mice. Molecular Therapy - Nucleic Acids, 2014, 3, e148.	2.3	28
113	Preclinical Studies on Intestinal Administration of Antisense Oligonucleotides as a Model for Oral Delivery for Treatment of Duchenne Muscular Dystrophy. Molecular Therapy - Nucleic Acids, 2014, 3, e211.	2.3	13
114	Novel Ex Vivo Culture Method for the Study of Dupuytren's Disease: Effects of TGFÎ² Type 1 Receptor Modulation by Antisense Oligonucleotides. Molecular Therapy - Nucleic Acids, 2014, 3, e142.	2.3	24
115	Targeting TGF-Î² Signaling by Antisense Oligonucleotide-mediated Knockdown of TGF-Î² Type I Receptor. Molecular Therapy - Nucleic Acids, 2014, 3, e156.	2.3	26
116	Development of a Web Course on Gene Therapy by the International Consortium of Gene Therapy. Molecular Therapy, 2014, 22, 482.	3.7	0
117	Affinity proteomics within rare diseases: a <scp>BIO</scp>â€<scp>NMD</scp> study for blood biomarkers of muscular dystrophies. EMBO Molecular Medicine, 2014, 6, 918-936.	3.3	105
118	Dystrophin Analysis in Clinical Trials. Journal of Neuromuscular Diseases, 2014, 1, 41-53.	1.1	20
119	Antisense-Mediated Exon Skipping: Networking to Meet Opportunities and to Overcome Challenges. Nucleic Acid Therapeutics, 2014, 24, 1-3.	2.0	12
120	A Novel Feed-Forward Loop between ARIH2 E3-Ligase and PABPN1 Regulates Aging-Associated Muscle Degeneration. American Journal of Pathology, 2014, 184, 1119-1131.	1.9	27
121	Fibronectin is a serum biomarker for <scp>D</scp>uchenne muscular dystrophy. Proteomics - Clinical Applications, 2014, 8, 269-278.	0.8	73
122	Antisense-mediated exon skipping: Taking advantage of a trick from Mother Nature to treat rare genetic diseases. Experimental Cell Research, 2014, 325, 50-55.	1.2	32
123	Preventing Formation of Toxic N-Terminal Huntingtin Fragments Through Antisense Oligonucleotide-Mediated Protein Modification. Nucleic Acid Therapeutics, 2014, 24, 4-12.	2.0	47
124	Translational and Regulatory Challenges for Exon Skipping Therapies. Human Gene Therapy, 2014, 25, 885-892.	1.4	42
125	Peptide Conjugation of 2â€²-O-methyl Phosphorothioate Antisense Oligonucleotides Enhances Cardiac Uptake and Exon Skipping in mdx Mice. Nucleic Acid Therapeutics, 2014, 24, 25-36.	2.0	52
126	Quantitative MRI and strength measurements in the assessment of muscle quality in Duchenne muscular dystrophy. Neuromuscular Disorders, 2014, 24, 409-416.	0.3	134

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127	Low dystrophin levels in heart can delay heart failure in mdx mice. Journal of Molecular and Cellular Cardiology, 2014, 69, 17-23.	0.9	47
128	Assessing Functional Performance in the Mdx Mouse Model. Journal of Visualized Experiments, 2014, , .	0.2	127
129	Dystrophin Analysis in Clinical Trials. Journal of Neuromuscular Diseases, 2014, 1, 41-53.	1.1	10
130	Advances in therapeutic RNA-targeting. New Biotechnology, 2013, 30, 299-301.	2.4	11
131	Innovating therapies for muscle diseases. Handbook of Clinical Neurology / Edited By P J Vinken and G W Bruyn, 2013, 113, 1497-1501.	1.0	8
132	Dose-Dependent Pharmacokinetic Profiles of 2â€²-O-Methyl Phosphorothioate Antisense OligonucleotidesinmdxMice. Nucleic Acid Therapeutics, 2013, 23, 228-237.	2.0	23
133	Antisense-mediated isoform switching of steroid receptor coactivator-1 in the central nucleus of the amygdala of the mouse brain. BMC Neuroscience, 2013, 14, 5.	0.8	12
134	Ataxin-3 protein modification as a treatment strategy for spinocerebellar ataxia type 3: Removal of the CAG containing exon. Neurobiology of Disease, 2013, 58, 49-56.	2.1	66
135	Exon skipping and gene transfer restore dystrophin expression in hiPSC-cardiomyocytes harbouring DMD mutations. Stem Cells and Development, 2013, , 150127064140000.	1.1	5
136	Low dystrophin levels increase survival and improve muscle pathology and function in dystrophin/utrophin doubleâ€knockout mice. FASEB Journal, 2013, 27, 2484-2495.	0.2	94
137	Dystrophin-deficient pigs provide new insights into the hierarchy of physiological derangements of dystrophic muscle. Human Molecular Genetics, 2013, 22, 4368-4382.	1.4	134
138	DMD transcript imbalance determines dystrophin levels. FASEB Journal, 2013, 27, 4909-4916.	0.2	30
139	Exon Skipping and Gene Transfer Restore Dystrophin Expression in Human Induced Pluripotent Stem Cells-Cardiomyocytes Harboring DMD Mutations. Stem Cells and Development, 2013, 22, 2714-2724.	1.1	56
140	Inhibition of IL-1 Signaling by Antisense Oligonucleotide-mediated Exon Skipping of IL-1 Receptor Accessory Protein (IL-1RAcP). Molecular Therapy - Nucleic Acids, 2013, 2, e66.	2.3	18
141	Antisense-Oligonucleotide Mediated Exon Skipping in Activin-Receptor-Like Kinase 2: Inhibiting the Receptor That Is Overactive in Fibrodysplasia Ossificans Progressiva. PLoS ONE, 2013, 8, e69096.	1.1	30
142	Generation of Embryonic Stem Cells and Mice for Duchenne Research. PLOS Currents, 2013, 5, .	1.4	8
143	Autophagy is Impaired in the Tibialis Anterior of Dystrophin Null Mice. PLOS Currents, 2013, 5, .	1.4	34
144	Guidance in Social and Ethical Issues Related to Clinical, Diagnostic Care and Novel Therapies for Hereditary Neuromuscular Rare Diseases: â€œTranslatingâ€œ the Translational. PLOS Currents, 2013, 5, .	1.4	15

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145	Gene therapy for Duchenne muscular dystrophy. Current Opinion in Neurology, 2012, 25, 588-596.	1.8	34
146	Long-term Exon Skipping Studies With 2â€²-O-Methyl Phosphorothioate Antisense Oligonucleotides in Dystrophic Mouse Models. Molecular Therapy - Nucleic Acids, 2012, 1, e44.	2.3	36
147	Prednisolone Treatment Does Not Interfere with 2â€²-O-Methyl Phosphorothioate Antisense-Mediated Exon Skipping in Duchenne Muscular Dystrophy. Human Gene Therapy, 2012, 23, 262-273.	1.4	14
148	Antisense oligonucleotide mediated exon skipping as a potential strategy for the treatment of a variety of inflammatory diseases such as rheumatoid arthritis. Annals of the Rheumatic Diseases, 2012, 71, i75-i77.	0.5	7
149	Overview on DMD Exon Skipping. Methods in Molecular Biology, 2012, 867, 97-116.	0.4	42
150	Cellâ€type specific regulation of myostatin signaling. FASEB Journal, 2012, 26, 1462-1472.	0.2	57
151	Exon skipping for DMD. Orphanet Journal of Rare Diseases, 2012, 7, A20.	1.2	1
152	Digenic inheritance of an SMCHD1 mutation and an FSHD-permissive D4Z4 allele causes facioscapulohumeral muscular dystrophy type 2. Nature Genetics, 2012, 44, 1370-1374.	9.4	582
153	Comparison of skeletal muscle pathology and motor function of dystrophin and utrophin deficient mouse strains. Neuromuscular Disorders, 2012, 22, 406-417.	0.3	65
154	Assessment of cardiac function in three mouse dystrophinopathies by magnetic resonance imaging. Neuromuscular Disorders, 2012, 22, 418-426.	0.3	19
155	Splice Modulating Therapies for Human Disease. Cell, 2012, 148, 1085-1088.	13.5	112
156	Overview on Applications of Antisense-Mediated Exon Skipping. Methods in Molecular Biology, 2012, 867, 79-96.	0.4	30
157	Overview on AON Design. Methods in Molecular Biology, 2012, 867, 117-129.	0.4	63
158	The Effects of Low Levels of Dystrophin on Mouse Muscle Function and Pathology. PLoS ONE, 2012, 7, e31937.	1.1	96
159	Phage display screening without repetitious selection rounds. Analytical Biochemistry, 2012, 421, 622-631.	1.1	149
160	The Effect of 6-Thioguanine on Alternative Splicing and Antisense-Mediated Exon Skipping Treatment for Duchenne Muscular Dystrophy. PLOS Currents, 2012, 4, .	1.4	4
161	Systemic Administration of PRO051 in Duchenne's Muscular Dystrophy. New England Journal of Medicine, 2011, 364, 1513-1522.	13.9	642
162	Current Status of Pharmaceutical and Genetic Therapeutic Approaches to Treat DMD. Molecular Therapy, 2011, 19, 830-840.	3.7	176

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163	The risks of therapeutic misconception and individual patient (n=1) â€œtrialsâ€•in rare diseases such as Duchenne dystrophy. Neuromuscular Disorders, 2011, 21, 13-15.	0.3	19
164	Serum matrix metalloproteinase-9 (MMP-9) as a biomarker for monitoring disease progression in Duchenne muscular dystrophy (DMD). Neuromuscular Disorders, 2011, 21, 569-578.	0.3	132
165	BMP antagonists enhance myogenic differentiation and ameliorate the dystrophic phenotype in a DMD mouse model. Neurobiology of Disease, 2011, 41, 353-360.	2.1	33
166	Dual exon skipping in myostatin and dystrophin for Duchenne muscular dystrophy. BMC Medical Genomics, 2011, 4, 36.	0.7	40
167	Opportunities and challenges for the development of antisense treatment in neuromuscular disorders. Expert Opinion on Biological Therapy, 2011, 11, 1025-1037.	1.4	11
168	Dystrophin quantification and clinical correlations in Becker muscular dystrophy: implications for clinical trials. Brain, 2011, 134, 3547-3559.	3.7	125
169	Targeting Several CAG Expansion Diseases by a Single Antisense Oligonucleotide. PLoS ONE, 2011, 6, e24308.	1.1	85
170	Progress in therapeutic antisense applications for neuromuscular disorders. European Journal of Human Genetics, 2010, 18, 146-153.	1.4	33
171	Therapeutic exon skipping for dysferlinopathies?. European Journal of Human Genetics, 2010, 18, 889-894.	1.4	47
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