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	Local Dystrophin Restoration with Antisense Oligonucleotide PRO051. New England Journal of Medicine, 2007, 357, 2677-2686.	13.9	735
2	Systemic Administration of PRO051 in Duchenne's Muscular Dystrophy. New England Journal of Medicine, 2011, 364, 1513-1522.	13.9	642
3	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
4	Entries in the Leiden Duchenne muscular dystrophy mutation database: An overview of mutation types and paradoxical cases that confirm the reading-frame rule. Muscle and Nerve, 2006, 34, 135-144.	1.0	569
5	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
6	Theoretic applicability of antisense-mediated exon skipping for Duchenne muscular dystrophy mutations. Human Mutation, 2009, 30, 293-299.	1.1	485
7	Duchenne muscular dystrophy. Nature Reviews Disease Primers, 2021, 7, 13.	18.1	448
8	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
9	FDA Approves Eteplirsen for Duchenne Muscular Dystrophy: The Next Chapter in the Eteplirsen Saga. Nucleic Acid Therapeutics, 2017, 27, 1-3.	2.0	284
10	Therapeutic developments for Duchenne muscular dystrophy. Nature Reviews Neurology, 2019, 15, 373-386.	4.9	265
11	The importance of genetic diagnosis for Duchenne muscular dystrophy. Journal of Medical Genetics, 2016, 53, 145-151.	1.5	242
12	The Pathogenesis and Therapy of Muscular Dystrophies. Annual Review of Genomics and Human Genetics, 2015, 16, 281-308.	2.5	240
13	Therapeutic antisense-induced exon skipping in cultured muscle cells from six different DMD patients. Human Molecular Genetics, 2003, 12, 907-914.	1.4	226
14	Antisense-mediated exon skipping: A versatile tool with therapeutic and research applications. Rna, 2007, 13, 1609-1624.	1.6	220
15	Delivery of oligonucleotideâ€based therapeutics: challenges and opportunities. EMBO Molecular Medicine, 2021, 13, e13243.	3.3	181
16	Antisense-Induced Multiexon Skipping for Duchenne Muscular Dystrophy Makes More Sense. American Journal of Human Genetics, 2004, 74, 83-92.	2.6	180
17	Current Status of Pharmaceutical and Genetic Therapeutic Approaches to Treat DMD. Molecular Therapy, 2011, 19, 830-840.	3.7	176
18	In vivo comparison of 2â€²â€Oâ€methyl phosphorothioate and morpholino antisense oligonucleotides for Duchenne muscular dystrophy exon skipping. Journal of Gene Medicine, 2009, 11, 257-266.	1.4	164

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19	Targeted exon skipping as a potential gene correction therapy for Duchenne muscular dystrophy. Neuromuscular Disorders, 2002, 12, S71-S77.	0.3	157
20	Phage display screening without repetitious selection rounds. Analytical Biochemistry, 2012, 421, 622-631.	1.1	149
21	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
22	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
23	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
24	Quantitative MRI and strength measurements in the assessment of muscle quality in Duchenne muscular dystrophy. Neuromuscular Disorders, 2014, 24, 409-416.	0.3	134
25	Preclinical PK and PD Studies on 2â€²-O-Methyl-phosphorothioate RNA Antisense Oligonucleotides in the mdx Mouse Model. Molecular Therapy, 2010, 18, 1210-1217.	3.7	132
26	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
27	Assessing Functional Performance in the Mdx Mouse Model. Journal of Visualized Experiments, 2014, , .	0.2	127
28	Guidelines for Antisense Oligonucleotide Design and Insight Into Splice-modulating Mechanisms. Molecular Therapy, 2009, 17, 548-553.	3.7	125
29	Dystrophin quantification and clinical correlations in Becker muscular dystrophy: implications for clinical trials. Brain, 2011, 134, 3547-3559.	3.7	125
30	Delivery is key: lessons learnt from developing spliceâ€switching antisense therapies. EMBO Molecular Medicine, 2017, 9, 545-557.	3.3	119
31	Splice Modulating Therapies for Human Disease. Cell, 2012, 148, 1085-1088.	13.5	112
32	Targeted Exon Skipping in Transgenic hDMD Mice: A Model for Direct Preclinical Screening of Human-Specific Antisense Oligonucleotides. Molecular Therapy, 2004, 10, 232-240.	3.7	111
33	Functional Analysis of 114 Exon-Internal AONs for Targeted DMD Exon Skipping: Indication for Steric Hindrance of SR Protein Binding Sites. Oligonucleotides, 2005, 15, 284-197.	2.7	108
34	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
35	Environmental 24-hr Cycles Are Essential for Health. Current Biology, 2016, 26, 1843-1853.	1.8	101
36	Exon skipping: a first in class strategy for Duchenne muscular dystrophy. Expert Opinion on Biological Therapy, 2017, 17, 225-236.	1.4	97

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37	The Effects of Low Levels of Dystrophin on Mouse Muscle Function and Pathology. PLoS ONE, 2012, 7, e31937.	1.1	96
38	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
39	Antisenseâ€mediated exon skipping: a therapeutic strategy for titinâ€based dilated cardiomyopathy. EMBO Molecular Medicine, 2015, 7, 562-576.	3.3	94
40	Natural disease history of the D2â€mdx mouse model for Duchenne muscular dystrophy. FASEB Journal, 2019, 33, 8110-8124.	0.2	88
41	Opportunities and challenges for antisense oligonucleotide therapies. Journal of Inherited Metabolic Disease, 2021, 44, 72-87.	1.7	86
42	Targeting Several CAG Expansion Diseases by a Single Antisense Oligonucleotide. PLoS ONE, 2011, 6, e24308.	1.1	85
43	The 10th Oligonucleotide Therapy Approved: Golodirsen for Duchenne Muscular Dystrophy. Nucleic Acid Therapeutics, 2020, 30, 67-70.	2.0	82
44	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
45	Exploring the Frontiers of Therapeutic Exon Skipping for Duchenne Muscular Dystrophy by Double Targeting within One or Multiple Exons. Molecular Therapy, 2006, 14, 401-407.	3.7	76
46	Fibronectin is a serum biomarker for <scp>D</scp>uchenne muscular dystrophy. Proteomics - Clinical Applications, 2014, 8, 269-278.	0.8	73
47	Antisense-mediated modulation of splicing: Therapeutic implications for Duchenne muscular dystrophy. RNA Biology, 2010, 7, 453-461.	1.5	71
48	Association Study of Exon Variants in the NF-ÎºB and TGFÎ² Pathways Identifies CD40 as a Modifier of Duchenne Muscular Dystrophy. American Journal of Human Genetics, 2016, 99, 1163-1171.	2.6	71
49	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
50	Comparison of skeletal muscle pathology and motor function of dystrophin and utrophin deficient mouse strains. Neuromuscular Disorders, 2012, 22, 406-417.	0.3	65
51	Overview on AON Design. Methods in Molecular Biology, 2012, 867, 117-129.	0.4	63
52	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
53	Characterization of neuromuscular synapse function abnormalities in multiple Duchenne muscular dystrophy mouse models. European Journal of Neuroscience, 2016, 43, 1623-1635.	1.2	59
54	The therapeutic potential of antisense-mediated exon skipping. Current Opinion in Molecular Therapeutics, 2008, 10, 140-9.	2.8	59

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55	Antisense-induced exon skipping for duplications in Duchenne muscular dystrophy. BMC Medical Genetics, 2007, 8, 43.	2.1	58
56	Cellâ€type specific regulation of myostatin signaling. FASEB Journal, 2012, 26, 1462-1472.	0.2	57
57	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
58	RD-Connect, NeurOmics and EURenOmics: collaborative European initiative for rare diseases. European Journal of Human Genetics, 2018, 26, 778-785.	1.4	55
59	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
60	Non-sequential and multi-step splicing of the dystrophin transcript. RNA Biology, 2016, 13, 290-305.	1.5	52
61	Assessment of the feasibility of exon 45â€“55 multiexon skipping for duchenne muscular dystrophy. BMC Medical Genetics, 2008, 9, 105.	2.1	49
62	Therapeutic exon skipping for dysferlinopathies?. European Journal of Human Genetics, 2010, 18, 889-894.	1.4	47
63	Preventing Formation of Toxic N-Terminal Huntingtin Fragments Through Antisense Oligonucleotide-Mediated Protein Modification. Nucleic Acid Therapeutics, 2014, 24, 4-12.	2.0	47
64	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
65	Tracking disease progression nonâ€invasively in Duchenne and Becker muscular dystrophies. Journal of Cachexia, Sarcopenia and Muscle, 2018, 9, 715-726.	2.9	47
66	Comparative analysis of antisense oligonucleotide sequences targeting exon 53 of the human DMD gene: Implications for future clinical trials. Neuromuscular Disorders, 2010, 20, 102-110.	0.3	44
67	A dystrophic Duchenne mouse model for testing human antisense oligonucleotides. PLoS ONE, 2018, 13, e0193289.	1.1	44
68	Therapeutic NOTCH3 cysteine correction in CADASIL using exon skipping:in vitroproof of concept. Brain, 2016, 139, 1123-1135.	3.7	43
69	Overview on DMD Exon Skipping. Methods in Molecular Biology, 2012, 867, 97-116.	0.4	42
70	Translational and Regulatory Challenges for Exon Skipping Therapies. Human Gene Therapy, 2014, 25, 885-892.	1.4	42
71	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
72	Dual exon skipping in myostatin and dystrophin for Duchenne muscular dystrophy. BMC Medical Genomics, 2011, 4, 36.	0.7	40

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73	Measuring clinical effectiveness of medicinal products for the treatment of Duchenne muscular dystrophy. Neuromuscular Disorders, 2015, 25, 96-105.	0.3	39
74	A 3 months mild functional test regime does not affect disease parameters in young mdx mice. Neuromuscular Disorders, 2010, 20, 273-280.	0.3	38
75	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
76	Accurate quantification of dystrophin mRNA and exon skipping levels in Duchenne Muscular Dystrophy. Laboratory Investigation, 2010, 90, 1396-1402.	1.7	37
77	Exonic Sequences Provide Better Targets for Antisense Oligonucleotides Than Splice Site Sequences in the Modulation of Duchenne Muscular Dystrophy Splicing. Oligonucleotides, 2010, 20, 69-77.	2.7	37
78	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
79	TCTEX1D1 is a genetic modifier of disease progression in Duchenne muscular dystrophy. European Journal of Human Genetics, 2020, 28, 815-825.	1.4	36
80	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
81	Gene therapy for Duchenne muscular dystrophy. Current Opinion in Neurology, 2012, 25, 588-596.	1.8	34
82	Autophagy is Impaired in the Tibialis Anterior of Dystrophin Null Mice. PLOS Currents, 2013, 5, .	1.4	34
83	Progress in therapeutic antisense applications for neuromuscular disorders. European Journal of Human Genetics, 2010, 18, 146-153.	1.4	33
84	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
85	Splicing modulation therapy in the treatment of genetic diseases. The Application of Clinical Genetics, 2014, 7, 245.	1.4	33
86	Development of Antisenseâ€Mediated Exon Skipping as a Treatment for Duchenne Muscular Dystrophy. Annals of the New York Academy of Sciences, 2009, 1175, 71-79.	1.8	32
87	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
88	New Momentum for the Field of Oligonucleotide Therapeutics. Molecular Therapy, 2016, 24, 193-194.	3.7	32
89	Overview on Applications of Antisense-Mediated Exon Skipping. Methods in Molecular Biology, 2012, 867, 79-96.	0.4	30
90	DMD transcript imbalance determines dystrophin levels. FASEB Journal, 2013, 27, 4909-4916.	0.2	30

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91	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
92	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
93	Less is more: therapeutic exon skipping for Duchenne muscular dystrophy. Lancet Neurology, The, 2009, 8, 873-875.	4.9	28
94	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
95	<scp>RNA</scp>â€based therapies for genodermatoses. Experimental Dermatology, 2017, 26, 3-10.	1.4	28
96	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
97	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
98	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
99	Gene expression profiling to monitor therapeutic and adverse effects of antisense therapies for Duchenne muscular dystrophy. Pharmacogenomics, 2006, 7, 281-297.	0.6	26
100	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
101	Crossâ€sectional serum metabolomic study of multiple forms of muscular dystrophy. Journal of Cellular and Molecular Medicine, 2018, 22, 2442-2448.	1.6	25
102	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
103	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
104	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
105	Serum Neurofilament light correlates with CADASIL disease severity and survival. Annals of Clinical and Translational Neurology, 2019, 6, 46-56.	1.7	24
106	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
107	Dose-Dependent Pharmacokinetic Profiles of 2â€²-O-Methyl Phosphorothioate Antisense OligonucleotidesinmdxMice. Nucleic Acid Therapeutics, 2013, 23, 228-237.	2.0	23
108	The use of genetically humanized animal models for personalized medicine approaches. DMM Disease Models and Mechanisms, 2020, 13, dmm041673.	1.2	22

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109	New insights in geneâ€derived therapy: the example of Duchenne muscular dystrophy. Annals of the New York Academy of Sciences, 2010, 1214, 199-212.	1.8	21
110	Dystrophin Analysis in Clinical Trials. Journal of Neuromuscular Diseases, 2014, 1, 41-53.	1.1	20
111	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
112	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
113	Circulating Biomarkers for Duchenne Muscular Dystrophy. Journal of Neuromuscular Diseases, 2015, 2, S49-S58.	1.1	20
114	Why dystrophin quantification is key in the eteplirsen saga. Nature Reviews Neurology, 2018, 14, 454-456.	4.9	20
115	Blood-derived biomarkers correlate with clinical progression in Duchenne muscular dystrophy. Journal of Neuromuscular Diseases, 2020, 7, 231-246.	1.1	20
116	Developments in reading frame restoring therapy approaches for Duchenne muscular dystrophy. Expert Opinion on Biological Therapy, 2021, 21, 343-359.	1.4	20
117	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
118	Assessment of cardiac function in three mouse dystrophinopathies by magnetic resonance imaging. Neuromuscular Disorders, 2012, 22, 418-426.	0.3	19
119	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
120	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
121	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
122	An update on RNA-targeting therapies for neuromuscular disorders. Current Opinion in Neurology, 2015, 28, 515-521.	1.8	18
123	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
124	Dystrophin deficiency leads to dysfunctional glutamate clearance in iPSC derived astrocytes. Translational Psychiatry, 2019, 9, 200.	2.4	18
125	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
126	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

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127	SplicePie: a novel analytical approach for the detection of alternative, non-sequential and recursive splicing. Nucleic Acids Research, 2015, 43, e80-e80.	6.5	17
128	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
129	Peripheral blood transcriptome profiling enables monitoring disease progression in dystrophic mice and patients. EMBO Molecular Medicine, 2021, 13, e13328.	3.3	16
130	Imperatives for DUCHENNE MD: a Simplified Guide to Comprehensive Care for Duchenne Muscular Dystrophy. PLOS Currents, 2015, 7, .	1.4	16
131	Low dystrophin levels are insufficient to normalize the neuromuscular synaptic abnormalities of mdx mice. Neuromuscular Disorders, 2018, 28, 427-442.	0.3	15
132	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
133	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
134	Detailed genetic and functional analysis of the hDMDdel52/mdx mouse model. PLoS ONE, 2020, 15, e0244215.	1.1	15
135	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
136	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
137	The RNA-binding profile of the splicing factor SRSF6 in immortalized human pancreatic Î²-cells. Life Science Alliance, 2021, 4, e202000825.	1.3	14
138	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
139	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
140	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
141	Antisense-Mediated Exon Skipping: Networking to Meet Opportunities and to Overcome Challenges. Nucleic Acid Therapeutics, 2014, 24, 1-3.	2.0	12
142	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
143	Opportunities and challenges for the development of antisense treatment in neuromuscular disorders. Expert Opinion on Biological Therapy, 2011, 11, 1025-1037.	1.4	11
144	Advances in therapeutic RNA-targeting. New Biotechnology, 2013, 30, 299-301.	2.4	11

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145	Studying the role of dystrophin-associated proteins in influencing Becker muscular dystrophy disease severity. Neuromuscular Disorders, 2015, 25, 231-237.	0.3	11
146	â€˜N of 1â€™ therapies need a better model. Nature Medicine, 2021, 27, 939-939.	15.2	11
147	Antisense-mediated exon skipping to correct IL-12Rî²1 deficiency in T cells. Blood, 2009, 113, 4548-4555.	0.6	10
148	Stakeholder collaboration for spinal muscular atrophy therapy development. Lancet Neurology, The, 2017, 16, 264.	4.9	10
149	Influence of full-length dystrophin on brain volumes in mouse models of Duchenne muscular dystrophy. PLoS ONE, 2018, 13, e0194636.	1.1	10
150	Dystrophin Analysis in Clinical Trials. Journal of Neuromuscular Diseases, 2014, 1, 41-53.	1.1	10
151	Translational Research in Europe for the Assessment and Treatment for Neuromuscular Disorders (TREAT-NMD). Neuropediatrics, 2017, 48, 211-220.	0.3	9
152	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
153	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
154	Tumor Necrosis Factor Receptor SF10A (TNFRSF10A) SNPs Correlate With Corticosteroid Response in Duchenne Muscular Dystrophy. Frontiers in Genetics, 2020, 11, 605.	1.1	9
155	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
156	Cytokine Profiling of Serum Allows Monitoring of Disease Progression in Inclusion Body Myositis. Journal of Neuromuscular Diseases, 2017, 4, 327-335.	1.1	8
157	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
158	Sensitive and reliable evaluation of single-cut sgRNAs to restore dystrophin by a GFP-reporter assay. PLoS ONE, 2020, 15, e0239468.	1.1	8
159	Eighteen-Year Disease Progression and Survival in CADASIL. Journal of Stroke, 2021, 23, 132-134.	1.4	8
160	Generation of Embryonic Stem Cells and Mice for Duchenne Research. PLOS Currents, 2013, 5, .	1.4	8
161	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
162	Genetic therapies for spinal muscular atrophy type 1. Lancet Neurology, The, 2018, 17, 111-112.	4.9	7

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163	Plasma lipidomic analysis shows a disease progression signature in mdx mice. Scientific Reports, 2021, 11, 12993.	1.6	7
164	Case Report: The Genetic Diagnosis of Duchenne Muscular Dystrophy in the Middle East. Frontiers in Pediatrics, 2021, 9, 716424.	0.9	7
165	The dystrophin gene and cognitive function in the general population. European Journal of Human Genetics, 2015, 23, 837-843.	1.4	6
166	Moving neuromuscular disorders research forward: from novel models to clinical studies. DMM Disease Models and Mechanisms, 2020, 13, .	1.2	6
167	Low human dystrophin levels prevent cardiac electrophysiological and structural remodelling in a Duchenne mouse model. Scientific Reports, 2021, 11, 9779.	1.6	6
168	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
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