Jeffrey S Chamberlain

List of Publications by Year
in descending order

234 papers	19,986 citations	8172 76 h-index	11928 134 g-index
244 all docs	244 docs citations	244 times ranked	11964 citing authors

#	Article	IF	Citations
1	Deletion screening of the Duchenne muscular dystrophy locus via multiplex DNA amplification. Nucleic Acids Research, 1988, 16, 11141-11156.	6.5	1,143
2	Characterization of Dystrophin in Muscle-Biopsy Specimens from Patients with Duchenne's or Becker's Muscular Dystrophy. New England Journal of Medicine, 1988, 318, 1363-1368.	13.9	911
3	Pericytes of human skeletal muscle are myogenic precursors distinct from satellite cells. Nature Cell Biology, 2007, 9, 255-267.	4.6	899
4	Systemic delivery of genes to striated muscles using adeno-associated viral vectors. Nature Medicine, 2004, 10, 828-834.	15.2	586
5	Modular flexibility of dystrophin: Implications for gene therapy of Duchenne muscular dystrophy. Nature Medicine, 2002, 8, 253-261.	15.2	505
6	X-linked dilated cardiomyopathy. Molecular genetic evidence of linkage to the Duchenne muscular dystrophy (dystrophin) gene at the Xp21 locus.. Circulation, 1993, 87, 1854-1865.	1.6	487
7	Animal Models for Muscular Dystrophy Show Different Patterns of Sarcolemmal Disruption. Journal of Cell Biology, 1997, 139, 375-385.	2.3	441
8	Dystrophins carrying spectrin-like repeats 16 and 17 anchor nNOS to the sarcolemma and enhance exercise performance in a mouse model of muscular dystrophy. Journal of Clinical Investigation, 2009, 119, 624-635.	3.9	319
9	Muscle-specific CRISPR/Cas9 dystrophin gene editing ameliorates pathophysiology in a mouse model for Duchenne muscular dystrophy. Nature Communications, 2017, 8, 14454.	5.8	298
10	Identification and Characterization of the Dystrophin Anchoring Site on Î²-Dystroglycan. Journal of Biological Chemistry, 1995, 270, 27305-27310.	1.6	295
11	Dystrophinâ€deficient mdx mice display a reduced life span and are susceptible to spontaneous rhabdomyosarcoma. FASEB Journal, 2007, 21, 2195-2204.	0.2	283
12	Overexpression of dystrophin in transgenic mdx mice eliminates dystrophic symptoms without toxicity. Nature, 1993, 364, 725-729.	13.7	280
13	rAAV6-microdystrophin preserves muscle function and extends lifespan in severely dystrophic mice. Nature Medicine, 2006, 12, 787-789.	15.2	274
14	Expression of full-length and truncated dystrophin mini-genes in transgenic mdx mice. Human Molecular Genetics, 1995, 4, 1251-1258.	1.4	270
15	Force and power output of fast and slow skeletal muscles from mdx mice 6â€28 months old. Journal of Physiology, 2001, 535, 591-600.	1.3	268
16	High levels of AAV vector integration into CRISPR-induced DNA breaks. Nature Communications, 2019, 10, 4439.	5.8	257
17	Sarcolemma-localized nNOS is required to maintain activity after mild exercise. Nature, 2008, 456, 511-515.	13.7	251
18	Production and Characterization of Improved Adenovirus Vectors with the E1, E2b, and E3 Genes Deleted. Journal of Virology, 1998, 72, 926-933.	1.5	234

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19	Efficient transduction of skeletal muscle using vectors based on adeno-associated virus serotype 6. Molecular Therapy, 2004, 10, 671-678.	3.7	218
20	Sustained AAV-mediated Dystrophin Expression in a Canine Model of Duchenne Muscular Dystrophy with a Brief Course of Immunosuppression. Molecular Therapy, 2007, 15, 1160-1166.	3.7	207
21	Assembly of the Dystrophin-Associated Protein Complex Does Not Require the Dystrophin Cooh-Terminal Domain. Journal of Cell Biology, 2000, 150, 1399-1410.	2.3	201
22	Efficient in vivo gene expression by trans-splicing adeno-associated viral vectors. Nature Biotechnology, 2005, 23, 1435-1439.	9.4	189
23	Expression of the murine Duchenne muscular dystrophy gene in muscle and brain. Science, 1988, 239, 1416-1418.	6.0	188
24	Tibialis anterior muscles in mdx mice are highly susceptible to contraction-induced injury. Journal of Muscle Research and Cell Motility, 2001, 22, 467-475.	0.9	184
25	Design of Tissue-specific Regulatory Cassettes for High-level rAAV-mediated Expression in Skeletal and Cardiac Muscle. Molecular Therapy, 2007, 15, 320-329.	3.7	180
26	Differential expression of dystrophin isoforms in strains of mdx mice with different mutations. Human Molecular Genetics, 1996, 5, 1149-1153.	1.4	179
27	New mdx mutation disrupts expression of muscle and nonmuscle isoforms of dystrophin. Nature Genetics, 1993, 4, 87-93.	9.4	177
28	Recombinant Adeno-associated Virus Transduction and Integration. Molecular Therapy, 2008, 16, 1189-1199.	3.7	177
29	Cas9 immunity creates challenges for CRISPR gene editing therapies. Nature Communications, 2018, 9, 3497.	5.8	173
30	Encapsidated adenovirus minichromosomes allow delivery and expression of a 14 kb dystrophin cDNA to muscle cells. Human Molecular Genetics, 1996, 5, 913-921.	1.4	172
31	Forced expression of dystrophin deletion constructs reveals structure-function correlations.. Journal of Cell Biology, 1996, 134, 93-102.	2.3	170
32	Follistatin-mediated skeletal muscle hypertrophy is regulated by Smad3 and mTOR independently of myostatin. Journal of Cell Biology, 2012, 197, 997-1008.	2.3	167
33	Progress toward Gene Therapy for Duchenne Muscular Dystrophy. Molecular Therapy, 2017, 25, 1125-1131.	3.7	165
34	Adeno-Associated virus-mediated microdystrophin expression protects young mdx muscle from contraction-induced injury. Molecular Therapy, 2005, 11, 245-256.	3.7	163
35	Human and murine dystrophin mRNA transcripts are differentially expressed during skeletal muscle, heart, and brain development. Nucleic Acids Research, 1992, 20, 1725-1731.	6.5	157
36	Dp71 can restore the dystrophin-associated glycoprotein complex in muscle but fails to prevent dystrophy. Nature Genetics, 1994, 8, 333-339.	9.4	156

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37	Selective loss of sarcolemmal nitric oxide synthase in Becker muscular dystrophy.. Journal of Experimental Medicine, 1996, 184, 609-618.	4.2	151
38	Microdystrophin Gene Therapy of Cardiomyopathy Restores Dystrophin-Glycoprotein Complex and Improves Sarcolemma Integrity in the Mdx Mouse Heart. Circulation, 2003, 108, 1626-1632.	1.6	143
39	Long-term persistence of donor nuclei in a Duchenne muscular dystrophy patient receiving bone marrow transplantation. Journal of Clinical Investigation, 2002, 110, 807-814.	3.9	140
40	Interactions between Î²2-syntrophin and a family of microtubule-associated serine/threonine kinases. Nature Neuroscience, 1999, 2, 611-617.	7.1	139
41	Functional correction of adult mdx mouse muscle using gutted adenoviral vectors expressing full-length dystrophin. Proceedings of the National Academy of Sciences of the United States of America, 2002, 99, 12979-12984.	3.3	133
42	Systemic delivery of human microdystrophin to regenerating mouse dystrophic muscle by muscle progenitor cells. Proceedings of the National Academy of Sciences of the United States of America, 2004, 101, 3581-3586.	3.3	130
43	Immunity to Adeno-Associated Virus-Mediated Gene Transfer in a Random-Bred Canine Model of Duchenne Muscular Dystrophy. Human Gene Therapy, 2007, 18, 18-26.	1.4	129
44	Adeno-associated virus vector-mediated gene transfer into dystrophin-deficient skeletal muscles evokes enhanced immune response against the transgene product. Gene Therapy, 2002, 9, 1576-1588.	2.3	126
45	An ex vivo gene therapy approach to treat muscular dystrophy using inducible pluripotent stem cells. Nature Communications, 2013, 4, 1549.	5.8	124
46	Systemic Administration of Micro-dystrophin Restores Cardiac Geometry and Prevents Dobutamine-induced Cardiac Pump Failure. Molecular Therapy, 2007, 15, 1086-1092.	3.7	123
47	Identification of Î±-Syntrophin Binding to Syntrophin Triplet, Dystrophin, and Utrophin. Journal of Biological Chemistry, 1995, 270, 4975-4978.	1.6	121
48	Sustained Human Factor VIII Expression in Hemophilia A Mice Following Systemic Delivery of a Gutless Adenoviral Vector. Molecular Therapy, 2002, 5, 63-73.	3.7	120
49	Contraction-induced injury to single permeabilized muscle fibers frommdx, transgenicmdx, and control mice. American Journal of Physiology - Cell Physiology, 2000, 279, C1290-C1294.	2.1	117
50	Gene therapy of muscular dystrophy. Human Molecular Genetics, 2002, 11, 2355-2362.	1.4	112
51	MULTIPLEX PCR FOR THE DIAGNOSIS OF DUCHENNE MUSCULAR DYSTROPHY. , 1990, , 272-281.		111
52	Chapter 9 The Value of Mammalian Models for Duchenne Muscular Dystrophy in Developing Therapeutic Strategies. Current Topics in Developmental Biology, 2008, 84, 431-453.	1.0	109
53	Systemic Microdystrophin Gene Delivery Improves Skeletal Muscle Structure and Function in Old Dystrophic mdx Mice. Molecular Therapy, 2008, 16, 657-664.	3.7	109
54	Microutrophin Delivery Through rAAV6 Increases Lifespan and Improves Muscle Function in Dystrophic Dystrophin/Utrophin-deficient Mice. Molecular Therapy, 2008, 16, 1539-1545.	3.7	107

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55	Emergent Dilated Cardiomyopathy Caused by Targeted Repair of Dystrophic Skeletal Muscle. Molecular Therapy, 2008, 16, 832-835.	3.7	106
56	Dystrophin deficiency in Drosophila reduces lifespan and causes a dilated cardiomyopathy phenotype. Aging Cell, 2008, 7, 237-249.	3.0	103
57	mdx muscle pathology is independent of nNOS perturbation. Human Molecular Genetics, 1998, 7, 823-829.	1.4	99
58	Dissecting the signaling and mechanical functions of the dystrophin-glycoprotein complex. Journal of Cell Science, 2006, 119, 1537-1546.	1.2	97
59	Prevention of Muscle Aging by Myofiber-Associated Satellite Cell Transplantation. Science Translational Medicine, 2010, 2, 57ra83.	5.8	96
60	Deletion analysis of the dystrophin-actin binding domain. FEBS Letters, 1994, 344, 255-260.	1.3	93
61	Therapeutic approaches to muscular dystrophy. Human Molecular Genetics, 2011, 20, R69-R78.	1.4	92
62	Expression of recombinant dystrophin and its localization to the cell membrane. Nature, 1991, 349, 334-336.	13.7	90
63	Viral-mediated gene therapy for the muscular dystrophies: Successes, limitations and recent advances. Biochimica Et Biophysica Acta - Molecular Basis of Disease, 2007, 1772, 243-262.	1.8	90
64	Microarchitecture Is Severely Compromised but Motor Protein Function Is Preserved in Dystrophic mdx Skeletal Muscle. Biophysical Journal, 2010, 98, 606-616.	0.2	89
65	Long-term persistence of donor nuclei in a Duchenne muscular dystrophy patient receiving bone marrow transplantation. Journal of Clinical Investigation, 2002, 110, 807-814.	3.9	89
66	Is the carboxyl-terminus of dystrophin required for membrane association? A novel, severe case of duchenne muscular dystrophy. Annals of Neurology, 1991, 30, 605-610.	2.8	88
67	Skeletal Muscle Contractions Induce Acute Changes in Cytosolic Superoxide, but Slower Responses in Mitochondrial Superoxide and Cellular Hydrogen Peroxide. PLoS ONE, 2014, 9, e96378.	1.1	88
68	Immune Evasion by Muscle-Specific Gene Expression in Dystrophic Muscle. Molecular Therapy, 2001, 4, 525-533.	3.7	87
69	Stable expression of calpain 3 from a muscle transgene in vivo: Immature muscle in transgenic mice suggests a role for calpain 3 in muscle maturation. Proceedings of the National Academy of Sciences of the United States of America, 2002, 99, 8874-8879.	3.3	87
70	Gene Therapy of mdx Mice With Large Truncated Dystrophins Generated by Recombination Using rAAV6. Molecular Therapy, 2011, 19, 36-45.	3.7	86
71	Analysis of Muscle Creatine Kinase Regulatory Elements in Recombinant Adenoviral Vectors. Molecular Therapy, 2000, 2, 16-25.	3.7	85
72	Inhibitors of sterol synthesis. Hypocholesterolemic action of dietary 5Î±-cholest-8(14)-en-3Î²-ol-15-one in rats and mice. Biochemical and Biophysical Research Communications, 1977, 78, 1227-1233.	1.0	84

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73	Prevention of dystrophic pathology in mdx mice by a truncated dystrophin isoform. Human Molecular Genetics, 1994, 3, 1725-1733.	1.4	84
74	Isolation and characterization of packaging cell lines that coexpress the adenovirus E1, DNA polymerase, and preterminal proteins: implications for gene therapy. Gene Therapy, 1997, 4, 258-263.	2.3	84
75	Adeno-associated viral vectors do not efficiently target muscle satellite cells. Molecular Therapy - Methods and Clinical Development, 2014, 1, 14038.	1.8	84
76	Stable transduction of myogenic cells with lentiviral vectors expressing a minidystrophin. Gene Therapy, 2005, 12, 1099-1108.	2.3	83
77	AAV-based gene therapies for the muscular dystrophies. Human Molecular Genetics, 2019, 28, R102-R107.	1.4	82
78	Development of Novel Micro-dystrophins with Enhanced Functionality. Molecular Therapy, 2019, 27, 623-635.	3.7	81
79	The mdx-amplification-resistant mutation system assay, a simple and rapid polymerase chain reaction-based detection of the mdx allele. , 1996, 19, 1549-1553.		80
80	Transgenic mdx mice expressing dystrophin with a deletion in the actin-binding domain display a "mild Becker" phenotype.. Journal of Cell Biology, 1996, 134, 873-884.	2.3	80
81	Sarcolemmal nNOS anchoring reveals a qualitative difference between dystrophin and utrophin. Journal of Cell Science, 2010, 123, 2008-2013.	1.2	80
82	Viral vectors for gene transfer of micro-, mini-, or full-length dystrophin. Neuromuscular Disorders, 2002, 12, S23-S29.	0.3	78
83	A Five-Repeat Micro-Dystrophin Gene Ameliorated Dystrophic Phenotype in the Severe DBA/2J-mdx Model of Duchenne Muscular Dystrophy. Molecular Therapy - Methods and Clinical Development, 2017, 6, 216-230.	1.8	78
84	Improved adenovirus packaging cell lines to support the growth of replication-defective gene-delivery vectors.. Proceedings of the National Academy of Sciences of the United States of America, 1996, 93, 3352-3356.	3.3	77
85	Successful Regional Delivery and Long-term Expression of a Dystrophin Gene in Canine Muscular Dystrophy: A Preclinical Model for Human Therapies. Molecular Therapy, 2012, 20, 1501-1507.	3.7	77
86	Immunity to Adeno-Associated Virus-Mediated Gene Transfer in a Random-Bred Canine Model of Duchenne Muscular Dystrophy. Human Gene Therapy, 2006, .	1.4	77
87	Proteomic analysis of mdx skeletal muscle: Great reduction of adenylate kinase 1 expression and enzymatic activity. Proteomics, 2003, 3, 1895-1903.	1.3	76
88	Gene Therapy Strategies for Duchenne Muscular Dystrophy Utilizing Recombinant Adeno-associated Virus Vectors. Molecular Therapy, 2006, 13, 241-249.	3.7	75
89	The Polyproline Site in Hinge 2 Influences the Functional Capacity of Truncated Dystrophins. PLoS Genetics, 2010, 6, e1000958.	1.5	73
90	Dystrophin and utrophin influence fiber type composition and post-synaptic membrane structure. Human Molecular Genetics, 2000, 9, 1357-1367.	1.4	72

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91	Activation of JNK1 contributes to dystrophic muscle pathogenesis. Current Biology, 2001, 11, 1278-1282.	1.8	71
92	Functional Deficits in nNOSÎ¼-Deficient Skeletal Muscle: Myopathy in nNOS Knockout Mice. PLoS ONE, 2008, 3, e3387.	1.1	71
93	Gene and cellâ€mediated therapies for muscular dystrophy. Muscle and Nerve, 2013, 47, 649-663.	1.0	71
94	Mdx mice inducibly expressing dystrophin provide insights into the potential of gene therapy for Duchenne muscular dystrophy. Human Molecular Genetics, 2000, 9, 2507-2515.	1.4	70
95	Expression of Dp260 in muscle tethers the actin cytoskeleton to the dystrophin-glycoprotein complex and partially prevents dystrophy. Human Molecular Genetics, 2002, 11, 1095-1105.	1.4	68
96	Developments in gene therapy for muscular dystrophy. Microscopy Research and Technique, 2000, 48, 223-238.	1.2	67
97	Muscle engraftment of myogenic progenitor cells following intraarterial transplantation. Muscle and Nerve, 2006, 34, 44-52.	1.0	66
98	Phenotypic Improvement of Dystrophic Muscles by rAAV/Microdystrophin Vectors Is Augmented by Igf1 Codelivery. Molecular Therapy, 2005, 12, 441-450.	3.7	64
99	Improved Production of Gutted Adenovirus in Cells Expressing Adenovirus Preterminal Protein and DNA Polymerase. Journal of Virology, 1999, 73, 7835-7841.	1.5	64
100	rAAV6â€Microdystrophin Rescues Aberrant Golgi Complex Organization in mdx Skeletal Muscles. Traffic, 2007, 8, 1424-1439.	1.3	63
101	Emerging strategies for cell and gene therapy of the muscular dystrophies. Expert Reviews in Molecular Medicine, 2009, 11, e18.	1.6	63
102	Truncated dystrophins can influence neuromuscular synapse structure. Molecular and Cellular Neurosciences, 2009, 40, 433-441.	1.0	62
103	Progress and prospects of gene therapy clinical trials for the muscular dystrophies. Human Molecular Genetics, 2016, 25, R9-R17.	1.4	62
104	Cell-lineage regulated myogenesis for dystrophin replacement: a novel therapeutic approach for treatment of muscular dystrophy. Human Molecular Genetics, 2008, 17, 2507-2517.	1.4	60
105	Dystrophin Delivery to Muscles of mdx Mice Using Lentiviral Vectors Leads to Myogenic Progenitor Targeting and Stable Gene Expression. Molecular Therapy, 2010, 18, 206-213.	3.7	60
106	Dystrophin mutations predict cellular susceptibility to oxidative stress. , 2000, 23, 784-792.		59
107	Multiply deleted [E1, polymerase-, and pTP-] adenovirus vector persists despite deletion of the preterminal protein. Journal of Gene Medicine, 2000, 2, 250-259.	1.4	57
108	A highly functional mini-dystrophin / GFP fusion gene for cell and gene therapy studies of Duchenne muscular dystrophy. Human Molecular Genetics, 2006, 15, 1610-1622.	1.4	52

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109	Increased sphingosine-1-phosphate improves muscle regeneration in acutely injured mdx mice. Skeletal Muscle, 2013, 3, 20.	1.9	52
110	Functional capacity of dystrophins carrying deletions in the N-terminal actin-binding domain. Human Molecular Genetics, 2007, 16, 2105-2113.	1.4	49
111	Characterization of aquaporinâ€4 in muscle and muscular dystrophy. FASEB Journal, 2002, 16, 943-949.	0.2	48
112	Gene Therapy in Large Animal Models of Muscular Dystrophy. ILAR Journal, 2009, 50, 187-198.	1.8	48
113	Cachexia in Cancer â€" Zeroing in on Myosin. New England Journal of Medicine, 2004, 351, 2124-2125.	13.9	46
114	Immunity and AAV-Mediated Gene Therapy for Muscular Dystrophies in Large Animal Models and Human Trials. Frontiers in Microbiology, 2011, 2, 201.	1.5	46
115	Differential expression of the skeletal muscle proteome inmdx mice at different ages. Electrophoresis, 2004, 25, 2576-2585.	1.3	45
116	Regional localization of the murine Duchenne muscular dystrophy gene on the mouse X chromosome. Somatic Cell and Molecular Genetics, 1987, 13, 671-678.	0.7	44
117	Variable rescue of microtubule and physiological phenotypes in mdx muscle expressing different miniaturized dystrophins. Human Molecular Genetics, 2018, 27, 2090-2100.	1.4	44
118	Myofiber branching rather than myofiber hyperplasia contributes to muscle hypertrophy in mdx mice. Skeletal Muscle, 2014, 4, 10.	1.9	43
119	Loss of niche-satellite cell interactions in syndecan-3 null mice alters muscle progenitor cell homeostasis improving muscle regeneration. Skeletal Muscle, 2016, 6, 34.	1.9	43
120	Immune Responses to AAV in Canine Muscle Monitored by Cellular Assays and Noninvasive Imaging. Molecular Therapy, 2010, 18, 617-624.	3.7	40
121	Gene therapy for Duchenne muscular dystrophy. Expert Opinion on Orphan Drugs, 2015, 3, 1255-1266.	0.5	40
122	Inhibitory Control Over Ca2+ Sparks via Mechanosensitive Channels Is Disrupted in Dystrophin Deficient Muscle but Restored by Mini-Dystrophin Expression. PLoS ONE, 2008, 3, e3644.	1.1	40
123	Evaluation of Vascular Delivery Methodologies to Enhance rAAV6-mediated Gene Transfer to Canine Striated Musculature. Molecular Therapy, 2009, 17, 1427-1433.	3.7	38
124	Onset of Experimental Severe Cardiac Fibrosis Is Mediated by Overexpression of Angiotensin-Converting Enzyme 2. Hypertension, 2009, 53, 694-700.	1.3	38
125	Integrated expression analysis of muscle hypertrophy identifies Asb2 as a negative regulator of muscle mass. JCI Insight, 2016, 1, .	2.3	38
126	Spectrin-like repeats from dystrophin and alpha-actinin-2 are not functionally interchangeable. Human Molecular Genetics, 2002, 11, 1807-1815.	1.4	37

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127	Animal Models of Muscular Dystrophy. Progress in Molecular Biology and Translational Science, 2012, 105, 83-111.	0.9	37
128	L-Type Ca2+ Channel Function Is Linked to Dystrophin Expression in Mammalian Muscle. PLoS ONE, 2008, 3, e1762.	1.1	37
129	Molecular and cellular adaptations to chronic myotendinous strain injury in mdx mice expressing a truncated dystrophin. Human Molecular Genetics, 2008, 17, 3975-3986.	1.4	36
130	Fluorophoreâ€labeled myosinâ€specific antibodies simplify muscleâ€fiber phenotyping. Muscle and Nerve, 2008, 37, 104-106.	1.0	34
131	Cardiac Consequences to Skeletal Muscle-Centric Therapeutics for Duchenne Muscular Dystrophy. Trends in Cardiovascular Medicine, 2009, 19, 49-54.	2.3	34
132	Muscular dystrophy: The worm turns to genetic disease. Current Biology, 2000, 10, R795-R797.	1.8	32
133	Syntrophin binds directly to multiple spectrin-like repeats in dystrophin and mediates binding of nNOS to repeats 16â€“17. Human Molecular Genetics, 2018, 27, 2978-2985.	1.4	31
134	Optimization of Multiplex PCRs. , 1994, , 38-46.		31
135	Characterization of Dystrophin and Utrophin Diversity in the Mouse. Human Molecular Genetics, 1999, 8, 593-599.	1.4	30
136	[13] Generation and growth of gutted adenoviral vectors. Methods in Enzymology, 2002, 346, 224-246.	0.4	30
137	Lentiviral transduction of microglial cells. Glia, 2005, 50, 48-55.	2.5	30
138	Gene Replacement Therapies for Duchenne Muscular Dystrophy Using Adeno-Associated Viral Vectors. Current Gene Therapy, 2012, 12, 139-151.	0.9	30
139	Phosphoenolpyruvate Carboxykinase (GTP): Characterization of the Human PCK1 Gene and Localization Distal to MODY on Chromosome 20. Genomics, 1993, 16, 698-706.	1.3	29
140	Gutted adenoviral vector growth using E1/E2b/E3-deleted helper viruses. Journal of Gene Medicine, 2002, 4, 480-489.	1.4	29
141	Viral vector-mediated gene therapies. Current Opinion in Neurology, 2015, 28, 522-527.	1.8	29
142	Characterization of patients with glycerol kinase deficiency utilizing cDNA probes for the Duchenne muscular dystrophy locus. Human Genetics, 1989, 83, 122-126.	1.8	28
143	Expression of the dystrophin isoform Dp116 preserves functional muscle mass and extends lifespan without preventing dystrophy in severely dystrophic mice. Human Molecular Genetics, 2011, 20, 4978-4990.	1.4	28
144	Therapy of Genetic Disorders: Novel Therapies for Duchenne Muscular Dystrophy. Current Pediatrics Reports, 2014, 2, 102-112.	1.7	28

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145	Therapy for neuromuscular disorders. Current Opinion in Genetics and Development, 2009, 19, 290-297.	1.5	27
146	Heparin-binding correlates with increased efficiency of AAV1- and AAV6-mediated transduction of striated muscle, but negatively impacts CNS transduction. Gene Therapy, 2013, 20, 497-503.	2.3	27
147	Muscle Structure Influences Utrophin Expression in mdx Mice. PLoS Genetics, 2014, 10, e1004431.	1.5	27
148	A B2 Repeat Insertion Generates Alternate Structures of the Mouse Muscle Î³-Phosphorylase Kinase Gene. Genomics, 1993, 16, 139-149.	1.3	26
149	Gene therapy for muscular dystrophy â€“ a review ofpromising progress. Expert Opinion on Biological Therapy, 2003, 3, 803-814.	1.4	26
150	PCR analysis of dystrophin gene mutation and expression. Journal of Cellular Biochemistry, 1991, 46, 255-259.	1.2	24
151	DIAPHRAGM MUSCLE STRIP PREPARATION FOR EVALUATION OF GENE THERAPIES IN mdx MICE. Clinical and Experimental Pharmacology and Physiology, 2008, 35, 725-729.	0.9	24
152	Gene therapy for muscular dystrophy ? a review of promising progress. Expert Opinion on Biological Therapy, 2003, 3, 803-814.	1.4	22
153	ACE inhibitor bulks up muscle. Nature Medicine, 2007, 13, 125-126.	15.2	21
154	Recombinant adenoâ€associated viral (<scp>rAAV</scp>) vectors mediate efficient gene transduction in cultured neonatal and adult microglia. Journal of Neurochemistry, 2016, 136, 49-62.	2.1	21
155	Micro-utrophin Improves Cardiac and Skeletal Muscle Function of Severely Affected D2/mdx Mice. Molecular Therapy - Methods and Clinical Development, 2018, 11, 92-105.	1.8	21
156	Efficient Rescue of Gutted Adenovirus Genomes Allows Rapid Production of Concentrated Stocks Without Negative Selection. Human Gene Therapy, 2002, 13, 519-531.	1.4	20
157	Prosurvival Factors Improve Functional Engraftment of Myogenically Converted Dermal Cells into Dystrophic Skeletal Muscle. Stem Cells and Development, 2016, 25, 1559-1569.	1.1	20
158	A move in the right direction. Nature, 2006, 444, 552-553.	13.7	19
159	Biodistribution and Safety Profile of Recombinant Adeno-Associated Virus Serotype 6 Vectors following Intravenous Delivery. Journal of Virology, 2008, 82, 7711-7715.	1.5	19
160	Sequencing protocols to genotype mdx, mdx^{4cv}, and mdx^{5cv} mice. Muscle and Nerve, 2010, 42, 268-270.	1.0	19
161	Localization of Dlg at the mammalian neuromuscular junction. NeuroReport, 1998, 9, 2121-2125.	0.6	18
162	Expression of the 71 kDa dystrophin isoform (Dp71) evaluated by gene targeting. Brain Research, 1999, 830, 174-178.	1.1	18

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163	AAV6 Vector Production and Purification for Muscle Gene Therapy. Methods in Molecular Biology, 2018, 1687, 257-266.	0.4	18
164	Stabilization of the cardiac sarcolemma by sarcospan rescues DMD-associated cardiomyopathy. JCI Insight, 2019, 4, .	2.3	18
165	Characterization of 10 New Polymorphic Dinucleotide Repeats and Generation of a High-Density Microsatellite-Based Physical Map of the BRCA1 Region of Chromosome 17q21. Genomics, 1994, 24, 419-424.	1.3	17
166	Donor origin of multipotent adult progenitor cells in radiation chimeras. Blood, 2005, 106, 3646-3649.	0.6	17
167	Duchenne Muscular Dystrophy Models Show Their Age. Cell, 2010, 143, 1040-1042.	13.5	17
168	Engineered DNA plasmid reduces immunity to dystrophin while improving muscle force in a model of gene therapy of Duchenne dystrophy. Proceedings of the National Academy of Sciences of the United States of America, 2018, 115, E9182-E9191.	3.3	17
169	Micro-dystrophin gene therapy prevents heart failure in an improved Duchenne muscular dystrophy cardiomyopathy mouse model. JCI Insight, 2021, 6, .	2.3	17
170	Multi-Parametric MRI at 14T for Muscular Dystrophy Mice Treated with AAV Vector-Mediated Gene Therapy. PLoS ONE, 2015, 10, e0124914.	1.1	17
171	Viral vectors for gene transfer to striated muscle. Current Opinion in Molecular Therapeutics, 2004, 6, 491-8.	2.8	16
172	Electromyographic studies inmdx and wild-type C57 mice. Muscle and Nerve, 2006, 33, 208-214.	1.0	15
173	Engraftment potential of dermal fibroblasts following in vivo myogenic conversion in immunocompetent dystrophic skeletal muscle. Molecular Therapy - Methods and Clinical Development, 2014, 1, 14025.	1.8	15
174	Gene Therapy Rescues Cardiac Dysfunction in Duchenne Muscular DystrophyÂMice by Elevating Cardiomyocyte Deoxy-Adenosine Triphosphate. JACC Basic To Translational Science, 2019, 4, 778-791.	1.9	15
175	Progress Toward Gene Therapy of Duchenne Muscular Dystrophy. Seminars in Neurology, 1999, 19, 323-332.	0.5	14
176	Immune Responses to rAAV6: The Influence of Canine Parvovirus Vaccination and Neonatal Administration of Viral Vector. Frontiers in Microbiology, 2011, 2, 220.	1.5	14
177	Phosphorylation within the cysteine-rich region of dystrophin enhances its association with Î²-dystroglycan and identifies a potential novel therapeutic target for skeletal muscle wasting. Human Molecular Genetics, 2014, 23, 6697-6711.	1.4	14
178	Perspectives on Best Practices for Gene Therapy Programs. Human Gene Therapy, 2015, 26, 127-133.	1.4	14
179	DXS28 (C7) maps centromeric to DXS68 (L1-4) and DXS67 (B24) by deletion analysis. Genomics, 1990, 7, 442-444.	1.3	13
180	Age-dependent Dystrophin Loss and Genetic Reconstitution Establish a Molecular Link Between Dystrophin and Heart Performance During Aging. Molecular Therapy, 2011, 19, 1821-1825.	3.7	13

#	Article	IF	Citations
181	Dystrophin Gene-Editing Stability Is Dependent on Dystrophin Levels in Skeletal but Not Cardiac Muscles. Molecular Therapy, 2021, 29, 1070-1085.	3.7	13
182	Muscle Fiber Type-Predominant Promoter Activity in Lentiviral-Mediated Transgenic Mouse. PLoS ONE, 2011, 6, e16908.	1.1	13
183	Translating the Genomics Revolution: The Need for an International Gene Therapy Consortium for Monogenic Diseases. Molecular Therapy, 2013, 21, 266-268.	3.7	12
184	Muscling in: Gene therapies for muscular dystrophy target RNA. Nature Medicine, 2010, 16, 170-171.	15.2	11
185	Preclinical Studies for Gene Therapy of Duchenne Muscular Dystrophy. Journal of Child Neurology, 2010, 25, 1149-1157.	0.7	11
186	Mechanical and non-mechanical functions of Dystrophin can prevent cardiac abnormalities in Drosophila. Experimental Gerontology, 2014, 49, 26-34.	1.2	11
187	Tetranucleotide repeat polymorphism at D17S846 maps within 40 kb of GAS at 17q12â€“q22. Human Molecular Genetics, 1993, 2, 1080-1080.	1.4	10
188	Enhanced effect of microdystrophin gene transfection by HSV-VP22 mediated intercellular protein transport. BMC Neuroscience, 2007, 8, 50.	0.8	10
189	Micro-dystrophin AAV Vectors Made by Transient Transfection and Herpesvirus System Are Equally Potent in Treating mdx Mouse Muscle Disease. Molecular Therapy - Methods and Clinical Development, 2020, 18, 664-678.	1.8	10
190	The dynamics of dystroglycan. Nature Genetics, 1999, 23, 256-258.	9.4	9
191	An intracellular delivery vehicle for protein transduction of micro-dystrophin. Journal of Drug Targeting, 2005, 13, 81-87.	2.1	9
192	Herpes Simplex Virus VP22 Enhances Adenovirus-Mediated Microdystrophin Gene Transfer to Skeletal Muscles in Dystrophin-Deficient (mdx) Mice. Human Gene Therapy, 2007, 18, 490-501.	1.4	9
193	Surrogate gene therapy for muscular dystrophy. Nature Medicine, 2019, 25, 1473-1474.	15.2	9
194	PCR analysis of muscular dystrophy in mdx mice. , 1993, 3, 167-189.		8
195	Duchenne muscular dystrophy. Current Opinion in Genetics and Development, 1991, 1, 11-14.	1.5	7
196	Genetics and pathogenic mechanisms of cardiomyopathies in the Drosophila model. Drug Discovery Today: Disease Models, 2008, 5, 125-134.	1.2	7
197	Translation of Cardiac Myosin Activation With 2-Deoxy-ATP to Treat Heart Failure Via an Experimental Ribonucleotide Reductase-Based Gene Therapy. JACC Basic To Translational Science, 2016, 1, 666-679.	1.9	7
198	Non-invasive tracking of disease progression in young dystrophic muscles using multi-parametric MRI at 14T. PLoS ONE, 2018, 13, e0206323.	1.1	7

#	Article	IF	Citations
199	Expressing a Z-disk nebulin fragment in nebulin-deficient mouse muscle: effects on muscle structure and function. Skeletal Muscle, 2020, 10, 2.	1.9	7
200	Muscular Dystrophy Meets the Gene Chip. Journal of Cell Biology, 2000, 151, F43-F46.	2.3	6
201	499. Intravenous Delivery of a Novel Micro-Dystrophin Vector Prevented Muscle Deterioration in Young Adult Canine Duchenne Muscular Dystrophy Dogs. Molecular Therapy, 2016, 24, S198-S199.	3.7	6
202	Comparison of dystrophin expression following gene editing and gene replacement in an aged preclinical DMD animal model. Molecular Therapy, 2022, 30, 2176-2185.	3.7	6
203	Region-specific cosmids and STRPs identified by chromosome microdissection and fish. Genomics, 1995, 25, 413-420.	1.3	5
204	Monitoring disease activity noninvasively in the mdx model of Duchenne muscular dystrophy. Proceedings of the National Academy of Sciences of the United States of America, 2018, 115, 7741-7746.	3.3	5
205	Packaging Cell Lines for Generating Replication-Defective and Gutted Adenoviral Vectors. , 2003, 76, 153-166.		4
206	Cross-species conservation of a polymorphic dinucleotide repeat in the dystrophin gene. Mammalian Genome, 1992, 3, 290-292.	1.0	3
207	Functional Enhancement of Skeletal Muscle by Gene Transfer. Physical Medicine and Rehabilitation Clinics of North America, 2005, 16, 875-887.	0.7	3
208	Microutrophin expression in dystrophic mice displays myofiber type differences in therapeutic effects. PLoS Genetics, 2020, 16, e1009179.	1.5	3
209	A Genetic Intervention Stands a Skip Away from Clinical Tests. Science, 2012, 338, 1431-1432.	6.0	2
210	Extracorporeal Delivery of rAAV with Metabolic Exchange and Oxygenation. Scientific Reports, 2013, 3, 1538.	1.6	2
211	Multiply deleted [E1, polymerase-, and pTP-] adenovirus vector persists despite deletion of the preterminal protein. , 2000, 2, 250.		2
212	Isolation of Tetranucleotide Repeat Polymorphisms Flanking theBRCA1Gene. Genomics, 1996, 32, 163-167.	1.3	1
213	Gene transfer to skeletal muscle. , 2005, , .		1
214	Relevance of motoneuron specification and programmed cell death in embryos to therapy of ALS. Birth Defects Research Part C: Embryo Today Reviews, 2005, 75, 294-304.	3.6	1
215	Broad Transgenic, and Cardiac-Specific Viral Mediated, Over-Expression of Ribonucleotide Reductase Increases In Vivo Cardiac Contractility. Biophysical Journal, 2012, 102, 615a.	0.2	1
216	Gene Therapy of Skeletal Muscle Disorders Using Viral Vectors. , 2012, , 1045-1051.		1

#	Article	IF	Citations
217	Removing the Immune Response From Muscular Dystrophy Research. Molecular Therapy, 2013, 21, 1821-1822.	3.7	1
218	AAV6-Mediated Overexpression of Ribonucleotide Reductase (R1R2) Enhances 2-Deoxy-ATP Concentration in Vivo and Improves Cardiac Function. Biophysical Journal, 2014, 106, 345a.	0.2	1
219	Developments in gene therapy for muscular dystrophy. Microscopy Research and Technique, 2000, 48, 223.	1.2	1
220	Systemic Gene Delivery for Muscle Gene Therapy. , 2010, , 163-179.		1
221	A Boost for Muscle with Gene Therapy. New England Journal of Medicine, 2022, 386, 1184-1186.	13.9	1
222	Erratum to â€œEfficient Transduction of Skeletal Muscle Using Vectors Based on Adeno-associated Virus Serotype 6â€• Molecular Therapy, 2009, 17, 1482.	3.7	0
223	On fixing broken muscleâ€¦fall seven times, stand up eightâ€”Japanese proverb. Muscle and Nerve, 2010, 41, 737-739.	1.0	0
224	11. Gene Therapy Mediated Increase in dATP Improves Cardiac Performance in Models of Systolic Heart Failure. Molecular Therapy, 2015, 23, S5.	3.7	0
225	398. Sustained Expression of a Canine Micro-Dystrophin Lead To Improvement of Limb Muscle Function in Dystrophic Dogs Following Large Scale AAV-Mediated Treatment. Molecular Therapy, 2015, 23, S158.	3.7	0
226	611. Alpha-Dystrobrevin-3 Prevents Myopathy and Restores Diaphragm Function in the Alpha-Dystrobrevin Null Mouse. Molecular Therapy, 2015, 23, S242-S243.	3.7	0
227	613. Characterization of IPSC-Derived Myogenic Progenitors Isolated from Mouse Models of Duchenne Muscular Dystrophy. Molecular Therapy, 2015, 23, S243-S244.	3.7	0
228	617. Histologic Assessment of Geriatric Esophagus Revels Therapeutic Targets in Wild-Type and Dystrophic Mouse Models. Molecular Therapy, 2015, 23, S245.	3.7	0
229	380. Therapeutic Capacity of AAV-Delivered Muscle-Specific-Expressed Micro-Utrophin (Î”R4-R21/Î”CT) in mdX4cv Skeletal Muscles. Molecular Therapy, 2016, 24, S151-S152.	3.7	0
230	501. In Vivo Gene Editing for Duchenne Muscular Dystrophy. Molecular Therapy, 2016, 24, S199.	3.7	0
231	AAV-Mediated Delivery of Ribonucleotide Reductase and Microdystrophin Rescues Function in Dystrophic Mice. Biophysical Journal, 2018, 114, 541a.	0.2	0
232	Gene Replacement Therapy for Duchenne Muscular Dystrophy. , 2019, , 327-337.		0
233	Donor Origin of Multipotent Adult Progenitor Cells in Radiation Chimeras.. Blood, 2005, 106, 1395-1395.	0.6	0
234	Therapeutic Gene Transfer to Skeletal Muscle. , 0, , 123-128.		0