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List of Publications by Year in descending order

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Version: 2024-02-01

38
papers

3,505
citations

471477

17
h-index

361001

35
g-index

38
all docs

38
docs citations

38
times ranked

4548
citing authors

#	ARTICLE	IF	CITATIONS
1	Single-Dose Gene-Replacement Therapy for Spinal Muscular Atrophy. <i>New England Journal of Medicine</i> , 2017, 377, 1713-1722.	27.0	1,642
2	Longitudinal effect of eteplirsen versus historical control on ambulation in <scp>D</scp>uchenne muscular dystrophy. <i>Annals of Neurology</i> , 2016, 79, 257-271.	5.3	428
3	Ataluren in patients with nonsense mutation Duchenne muscular dystrophy (ACT DMD): a multicentre, randomised, double-blind, placebo-controlled, phase 3 trial. <i>Lancet, The</i> , 2017, 390, 1489-1498.	13.7	365
4	Assessment of Systemic Delivery of rAAVrh74.MHCK7.micro-dystrophin in Children With Duchenne Muscular Dystrophy. <i>JAMA Neurology</i> , 2020, 77, 1122.	9.0	226
5	Five-Year Extension Results of the Phase 1 START Trial of Onasemnogene Apeparvovec in Spinal Muscular Atrophy. <i>JAMA Neurology</i> , 2021, 78, 834.	9.0	135
6	AVXS-101 (Onasemnogene Apeparvovec) for SMA1: Comparative Study with a Prospective Natural History Cohort. <i>Journal of Neuromuscular Diseases</i> , 2019, 6, 307-317.	2.6	124
7	Follistatin Gene Therapy for Sporadic Inclusion Body Myositis Improves Functional Outcomes. <i>Molecular Therapy</i> , 2017, 25, 870-879.	8.2	84
8	Genetics and Emerging Treatments for Duchenne and Becker Muscular Dystrophy. <i>Pediatric Clinics of North America</i> , 2015, 62, 723-742.	1.8	71
9	Long-term treatment with eteplirsen in nonambulatory patients with Duchenne muscular dystrophy. <i>Medicine (United States)</i> , 2019, 98, e15858.	1.0	61
10	Gene Delivery for Limb-Girdle Muscular Dystrophy Type 2D by Isolated Limb Infusion. <i>Human Gene Therapy</i> , 2019, 30, 794-801.	2.7	34
11	Twice-€weekly glucocorticosteroids in infants and young boys with Duchenne muscular dystrophy. <i>Muscle and Nerve</i> , 2019, 59, 650-657.	2.2	32
12	Clinical trial readiness in non-ambulatory boys and men with duchenne muscular dystrophy: MDA-DMD network follow-up. <i>Muscle and Nerve</i> , 2016, 54, 681-689.	2.2	29
13	Comparison of Long-term Ambulatory Function in Patients with Duchenne Muscular Dystrophy Treated with Eteplirsen and Matched Natural History Controls. <i>Journal of Neuromuscular Diseases</i> , 2021, 8, 469-479.	2.6	22
14	Progress in treatment and newborn screening for Duchenne muscular dystrophy and spinal muscular atrophy. <i>World Journal of Pediatrics</i> , 2019, 15, 219-225.	1.8	21
15	Assessment of disease progression in dysferlinopathy. <i>Neurology</i> , 2019, 92, .	1.1	20
16	Development of a standard of care for patients with valosin-containing protein associated multisystem proteinopathy. <i>Orphanet Journal of Rare Diseases</i> , 2022, 17, 23.	2.7	19
17	Development of the sporadic inclusion body myositis physical functioning assessment. <i>Muscle and Nerve</i> , 2016, 54, 653-657.	2.2	17
18	Assessing Dysferlinopathy Patients Over Three Years With a New Motor Scale. <i>Annals of Neurology</i> , 2021, 89, 967-978.	5.3	17

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19	Correlation of knee strength to functional outcomes in becker muscular dystrophy. <i>Muscle and Nerve</i> , 2013, 47, 550-554.	2.2	16
20	The 100-meter timed test: Normative data in healthy males and comparative pilot outcome data for use in Duchenne muscular dystrophy clinical trials. <i>Neuromuscular Disorders</i> , 2017, 27, 452-457.	0.6	16
21	Reliability and validity of active seated: An outcome in dystrophinopathy. <i>Muscle and Nerve</i> , 2015, 52, 356-362.	2.2	15
22	Natural History of Steroid-Treated Young Boys With Duchenne Muscular Dystrophy Using the NSAA, 100m, and Timed Functional Tests. <i>Pediatric Neurology</i> , 2020, 113, 15-20.	2.1	14
23	Modeling functional decline over time in sporadic inclusion body myositis. <i>Muscle and Nerve</i> , 2017, 55, 526-531.	2.2	12
24	Motor Function Test Reliability During the NeuroNEXT Spinal Muscular Atrophy Infant Biomarker Study. <i>Journal of Neuromuscular Diseases</i> , 2018, 5, 509-521.	2.6	12
25	Psychometric validation of a patient-reported measure of physical functioning in sporadic inclusion body myositis. <i>Muscle and Nerve</i> , 2016, 54, 658-665.	2.2	11
26	ACTIVE (Ability Captured Through Interactive Video Evaluation) workspace volume video game to quantify meaningful change in spinal muscular atrophy. <i>Developmental Medicine and Child Neurology</i> , 2020, 62, 303-309.	2.1	10
27	Emerging therapeutic options for sporadic inclusion body myositis. <i>Therapeutics and Clinical Risk Management</i> , 2015, 11, 1459.	2.0	9
28	Use of the Children's Hospital of Philadelphia Infant Test of Neuromuscular Disorders (CHOP INTEND) in X-Linked Myotubular Myopathy: Content Validity and Psychometric Performance. <i>Journal of Neuromuscular Diseases</i> , 2021, 8, 63-77.	2.6	9
29	Remote Delivery of Motor Function Assessment and Training for Clinical Trials in Neuromuscular Disease: A Response to the COVID-19 Global Pandemic. <i>Frontiers in Genetics</i> , 2021, 12, 735538.	2.3	9
30	Measuring change in inclusion body myositis: clinical assessments versus imaging. <i>Clinical and Experimental Rheumatology</i> , 2022, 40, 404-413.	0.8	6
31	Validity and Reliability of the Neuromuscular Gross Motor Outcome. <i>Pediatric Neurology</i> , 2021, 122, 21-26.	2.1	5
32	Assessing the Relationship of Patient Reported Outcome Measures With Functional Status in Dysferlinopathy: A Rasch Analysis Approach. <i>Frontiers in Neurology</i> , 2022, 13, 828525.	2.4	4
33	Development of Duchenne Video Assessment scorecards to evaluate ease of movement among those with Duchenne muscular dystrophy. <i>PLoS ONE</i> , 2022, 17, e0266845.	2.5	4
34	Comparison of strength testing modalities in dysferlinopathy. <i>Muscle and Nerve</i> , 2022, 66, 159-166.	2.2	3
35	Functional outcome measures in young, steroid-naïve boys with Duchenne muscular dystrophy. <i>Neuromuscular Disorders</i> , 2022, 32, 460-467.	0.6	2
36	Prediction of Clinical Outcomes of Spinal Muscular Atrophy Using Motion Tracking Data and Elastic Net Regression. , 2018, , .		1

#	ARTICLE	IF	CITATIONS
37	Random forest: random results or meaningful insights for patients with facioscapulohumeral muscular dystrophy?. Brain, 2021, , .	7.6	0
38	Measuring change in inclusion body myositis: clinical assessments versus imaging.. Clinical and Experimental Rheumatology, 2022, 40, 404-413.	0.8	0