

Laurent J Servais

List of Publications by Year in descending order

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Version: 2024-02-01

134
papers

7,471
citations

57758

44
h-index

60623

81
g-index

139
all docs

139
docs citations

139
times ranked

6950
citing authors

#	ARTICLE	IF	CITATIONS
1	Nusinersen versus Sham Control in Infantile-Onset Spinal Muscular Atrophy. <i>New England Journal of Medicine</i> , 2017, 377, 1723-1732.	27.0	1,533
2	Safety and efficacy of drisapersen for the treatment of Duchenne muscular dystrophy (DEMAND II): an exploratory, randomised, placebo-controlled phase 2 study. <i>Lancet Neurology</i> , The, 2014, 13, 987-996.	10.2	279
3	Anti-HMGR Autoantibodies in European Patients With Autoimmune Necrotizing Myopathies. <i>Medicine (United States)</i> , 2014, 93, 150-157.	1.0	235
4	Risdiplam in Type 1 Spinal Muscular Atrophy. <i>New England Journal of Medicine</i> , 2021, 384, 915-923.	27.0	229
5	Increased dystrophin production with golodirsen in patients with Duchenne muscular dystrophy. <i>Neurology</i> , 2020, 94, e2270-e2282.	1.1	207
6	Long-term microdystrophin gene therapy is effective in a canine model of Duchenne muscular dystrophy. <i>Nature Communications</i> , 2017, 8, 16105.	12.8	175
7	Development of the <scp>P</scp>erformance of the <scp>U</scp>pper <scp>L</scp>imb module for <scp>D</scp>uchenne muscular dystrophy. <i>Developmental Medicine and Child Neurology</i> , 2013, 55, 1038-1045.	2.1	173
8	Aminopyridines Correct Early Dysfunction and Delay Neurodegeneration in a Mouse Model of Spinocerebellar Ataxia Type 1. <i>Journal of Neuroscience</i> , 2011, 31, 11795-11807.	3.6	137
9	Risdiplam-Treated Infants with Type 1 Spinal Muscular Atrophy versus Historical Controls. <i>New England Journal of Medicine</i> , 2021, 385, 427-435.	27.0	137
10	Sexual health care in persons with intellectual disabilities. <i>Mental Retardation and Developmental Disabilities Research Reviews</i> , 2006, 12, 48-56.	3.6	136
11	<p>Clinical Evidence Supporting Early Treatment Of Patients With Spinal Muscular Atrophy: Current Perspectives<p>. <i>Therapeutics and Clinical Risk Management</i> , 2019, Volume 15, 1153-1161.	2.0	135
12	Nusinersen treatment of spinal muscular atrophy: current knowledge and existing gaps. <i>Developmental Medicine and Child Neurology</i> , 2019, 61, 19-24.	2.1	115
13	Onasemnogene abeparvovec gene therapy for symptomatic infantile-onset spinal muscular atrophy type 1 (STRIVE-EU): an open-label, single-arm, multicentre, phase 3 trial. <i>Lancet Neurology</i> , The, 2021, 20, 832-841.	10.2	112
14	Forelimb Treatment in a Large Cohort of Dystrophic Dogs Supports Delivery of a Recombinant AAV for Exon Skipping in Duchenne Patients. <i>Molecular Therapy</i> , 2014, 22, 1923-1935.	8.2	108
15	Prospective and longitudinal natural history study of patients with Type 2 and 3 spinal muscular atrophy: Baseline data NatHis-SMA study. <i>PLoS ONE</i> , 2018, 13, e0201004.	2.5	107
16	Inactivation of Calcium-Binding Protein Genes Induces 160 Hz Oscillations in the Cerebellar Cortex of Alert Mice. <i>Journal of Neuroscience</i> , 2004, 24, 434-441.	3.6	106
17	Downregulation of myostatin pathway in neuromuscular diseases may explain challenges of anti-myostatin therapeutic approaches. <i>Nature Communications</i> , 2017, 8, 1859.	12.8	102
18	Onasemnogene abeparvovec for presymptomatic infants with two copies of SMN2 at risk for spinal muscular atrophy type 1: the Phase III SPR1NT trial. <i>Nature Medicine</i> , 2022, 28, 1381-1389.	30.7	99

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19	Purkinje cell dysfunction and alteration of long-term synaptic plasticity in fetal alcohol syndrome. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2007, 104, 9858-9863.	7.1	97
20	Distinctive Serum miRNA Profile in Mouse Models of Striated Muscular Pathologies. <i>PLoS ONE</i> , 2013, 8, e55281.	2.5	97
21	Newborn screening programs for spinal muscular atrophy worldwide: Where we stand and where to go. <i>Neuromuscular Disorders</i> , 2021, 31, 574-582.	0.6	94
22	Onasemnogene abeparvovec for presymptomatic infants with three copies of SMN2 at risk for spinal muscular atrophy: the Phase III SPR1NT trial. <i>Nature Medicine</i> , 2022, 28, 1390-1397.	30.7	93
23	Nusinersen in patients older than 7 months with spinal muscular atrophy type 1. <i>Neurology</i> , 2018, 91, e1312-e1318.	1.1	91
24	New treatments in spinal muscular atrophy: an overview of currently available data. <i>Expert Opinion on Pharmacotherapy</i> , 2020, 21, 307-315.	1.8	91
25	Safety and efficacy of once-daily risdiplam in type 2 and non-ambulant type 3 spinal muscular atrophy (SUNFISH part 2): a phase 3, double-blind, randomised, placebo-controlled trial. <i>Lancet Neurology</i> , The, 2022, 21, 42-52.	10.2	89
26	Serum Profiling Identifies Novel Muscle miRNA and Cardiomyopathy-Related miRNA Biomarkers in Golden Retriever Muscular Dystrophy Dogs and Duchenne Muscular Dystrophy Patients. <i>American Journal of Pathology</i> , 2014, 184, 2885-2898.	3.8	85
27	Proteomics profiling of urine reveals specific titin fragments as biomarkers of Duchenne muscular dystrophy. <i>Neuromuscular Disorders</i> , 2014, 24, 563-573.	0.6	85
28	Dermatomyositis With or Without Anti-Melanoma Differentiation-Associated Gene 5 Antibodies. <i>American Journal of Pathology</i> , 2016, 186, 691-700.	3.8	78
29	European ad-hoc consensus statement on gene replacement therapy for spinal muscular atrophy. <i>European Journal of Paediatric Neurology</i> , 2020, 28, 38-43.	1.6	74
30	Triheptanoin dramatically reduces paroxysmal motor disorder in patients with GLUT1 deficiency. <i>Journal of Neurology, Neurosurgery and Psychiatry</i> , 2016, 87, 550-553.	1.9	73
31	Impaired Presynaptic High-Affinity Choline Transporter Causes a Congenital Myasthenic Syndrome with Episodic Apnea. <i>American Journal of Human Genetics</i> , 2016, 99, 753-761.	6.2	68
32	X-linked myotubular myopathy. <i>Neurology</i> , 2019, 92, e1852-e1867.	1.1	66
33	Newborn screening for SMA in Southern Belgium. <i>Neuromuscular Disorders</i> , 2019, 29, 343-349.	0.6	65
34	Longitudinal functional and NMR assessment of upper limbs in Duchenne muscular dystrophy. <i>Neurology</i> , 2016, 86, 1022-1030.	1.1	63
35	Pharmacokinetics and safety of single doses of drisapersen in non-ambulant subjects with Duchenne muscular dystrophy: Results of a double-blind randomized clinical trial. <i>Neuromuscular Disorders</i> , 2014, 24, 16-24.	0.6	62
36	Quantitative NMRI and NMRS identify augmented disease progression after loss of ambulation in forearms of boys with Duchenne muscular dystrophy. <i>NMR in Biomedicine</i> , 2015, 28, 1150-1162.	2.8	62

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37	Natural history of Type 2 and 3 spinal muscular atrophy: 2-year NatHis-SMA study. <i>Annals of Clinical and Translational Neurology</i> , 2021, 8, 359-373.	3.7	58
38	Upper Limb Strength and Function Changes during a One-Year Follow-Up in Non-Ambulant Patients with Duchenne Muscular Dystrophy: An Observational Multicenter Trial. <i>PLoS ONE</i> , 2015, 10, e0113999.	2.5	58
39	Long-Term Safety and Efficacy Data of Golodirsen in Ambulatory Patients with Duchenne Muscular Dystrophy Amenable to Exon 53 Skipping: A First-in-human, Multicenter, Two-Part, Open-Label, Phase 1/2 Trial. <i>Nucleic Acid Therapeutics</i> , 2022, 32, 29-39.	3.6	58
40	244th ENMC international workshop: Newborn screening in spinal muscular atrophy May 10-12, 2019, Hoofddorp, The Netherlands. <i>Neuromuscular Disorders</i> , 2020, 30, 93-103.	0.6	55
41	Systematic literature review of the economic burden of spinal muscular atrophy and economic evaluations of treatments. <i>Orphanet Journal of Rare Diseases</i> , 2021, 16, 47.	2.7	54
42	Targeted calretinin expression in granule cells of calretininnull mice restores normal cerebellar functions. <i>FASEB Journal</i> , 2006, 20, 380-382.	0.5	51
43	Upper Limb Evaluation and One-Year Follow Up of Non-Ambulant Patients with Spinal Muscular Atrophy: An Observational Multicenter Trial. <i>PLoS ONE</i> , 2015, 10, e0121799.	2.5	49
44	Serum proteomic profiling reveals fragments of MYOM3 as potential biomarkers for monitoring the outcome of therapeutic interventions in muscular dystrophies. <i>Human Molecular Genetics</i> , 2015, 24, 4916-4932.	2.9	49
45	Severe dystonia, cerebellar atrophy, and cardiomyopathy likely caused by a missense mutation in TOR1AIP1. <i>Orphanet Journal of Rare Diseases</i> , 2014, 9, 174.	2.7	43
46	STAC3 variants cause a congenital myopathy with distinctive dysmorphic features and malignant hyperthermia susceptibility. <i>Human Mutation</i> , 2018, 39, 1980-1994.	2.5	42
47	Four-year longitudinal study of clinical and functional endpoints in sporadic inclusion body myositis: Implications for therapeutic trials. <i>Neuromuscular Disorders</i> , 2014, 24, 604-610.	0.6	41
48	BK Channels Control Cerebellar Purkinje and Golgi Cell Rhythmicity In Vivo. <i>PLoS ONE</i> , 2009, 4, e7991.	2.5	39
49	A Movement Monitor Based on Magneto-Inertial Sensors for Non-Ambulant Patients with Duchenne Muscular Dystrophy: A Pilot Study in Controlled Environment. <i>PLoS ONE</i> , 2016, 11, e0156696.	2.5	38
50	Sitting in patients with spinal muscular atrophy type 1 treated with nusinersen. <i>Developmental Medicine and Child Neurology</i> , 2020, 62, 310-314.	2.1	36
51	Emerging therapies for Duchenne muscular dystrophy. <i>Lancet Neurology</i> , The, 2022, 21, 814-829.	10.2	35
52	Downregulation of the Glial GLT1 Glutamate Transporter and Purkinje Cell Dysfunction in a Mouse Model of Myotonic Dystrophy. <i>Cell Reports</i> , 2017, 19, 2718-2729.	6.4	33
53	Long-term data with idebenone on respiratory function outcomes in patients with Duchenne muscular dystrophy. <i>Neuromuscular Disorders</i> , 2020, 30, 5-16.	0.6	33
54	Fast oscillation in the cerebellar cortex of calcium binding protein-deficient mice: a new sensorimotor arrest rhythm. <i>Progress in Brain Research</i> , 2005, 148, 165-180.	1.4	32

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55	Very Low Residual Dystrophin Quantity Is Associated with Milder Dystrophinopathy. <i>Annals of Neurology</i> , 2021, 89, 280-292.	5.3	32
56	First Regulatory Qualification of a Novel Digital Endpoint in Duchenne Muscular Dystrophy: A Multi-Stakeholder Perspective on the Impact for Patients and for Drug Development in Neuromuscular Diseases. <i>Digital Biomarkers</i> , 2021, 5, 183-190.	4.4	32
57	Three years pilot of spinal muscular atrophy newborn screening turned into official program in Southern Belgium. <i>Scientific Reports</i> , 2021, 11, 19922.	3.3	32
58	High frequency of COH1 intragenic deletions and duplications detected by MLPA in patients with Cohen syndrome. <i>European Journal of Human Genetics</i> , 2010, 18, 1133-1140.	2.8	31
59	Spinal muscular atrophy care in the COVID-19 pandemic era. <i>Muscle and Nerve</i> , 2020, 62, 46-49.	2.2	31
60	Effect of chronic ethanol ingestion on Purkinje and Golgi cell firing in vivo and on motor coordination in mice. <i>Brain Research</i> , 2005, 1055, 171-179.	2.2	27
61	Cholesterol metabolism is a potential therapeutic target in Duchenne muscular dystrophy. <i>Journal of Cachexia, Sarcopenia and Muscle</i> , 2021, 12, 677-693.	7.3	25
62	Stride Velocity 95th Centile: Insights into Gaining Regulatory Qualification of the First Wearable-Derived Digital Endpoint for use in Duchenne Muscular Dystrophy Trials. <i>Journal of Neuromuscular Diseases</i> , 2022, 9, 335-346.	2.6	25
63	From Electrophysiology to Chromatin: A Bottom-Up Approach to Angelman Syndrome. <i>Annals of the New York Academy of Sciences</i> , 2004, 1030, 599-611.	3.8	24
64	The administration of antisense oligonucleotide golodirsen reduces pathological regeneration in patients with Duchenne muscular dystrophy. <i>Acta Neuropathologica Communications</i> , 2021, 9, 7.	5.2	24
65	Age dependence of strain determinant on mice motor coordination. <i>Brain Research</i> , 2005, 1039, 37-42.	2.2	23
66	Methylome and transcriptome profiling in Myasthenia Gravis monozygotic twins. <i>Journal of Autoimmunity</i> , 2017, 82, 62-73.	6.5	23
67	Downregulation of miRNA-29, -23 and -21 in urine of Duchenne muscular dystrophy patients. <i>Epigenomics</i> , 2018, 10, 875-889.	2.1	23
68	Risdiplam in types 2 and 3 spinal muscular atrophy: A randomised, placebo-controlled, dose-finding trial followed by 24 months of treatment. <i>European Journal of Neurology</i> , 2023, 30, 1945-1956.	3.3	23
69	Effect of simple spike firing mode on complex spike firing rate and waveform in cerebellar Purkinje cells in non-anesthetized mice. <i>Neuroscience Letters</i> , 2004, 367, 171-176.	2.1	22
70	Relationship between muscle impairments, postural stability, and gait parameters assessed with lower-trunk accelerometry in myotonic dystrophy type 1. <i>Neuromuscular Disorders</i> , 2016, 26, 428-435.	0.6	22
71	Therapies in preclinical and clinical development for Angelman syndrome. <i>Expert Opinion on Investigational Drugs</i> , 2021, 30, 709-720.	4.1	22
72	Chiari type I malformation causing central apnoeas in a 4-month-old boy. <i>European Journal of Paediatric Neurology</i> , 2009, 13, 463-465.	1.6	21

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73	Non-Ambulant Duchenne Patients Theoretically Treatable by Exon 53 Skipping have Severe Phenotype. <i>Journal of Neuromuscular Diseases</i> , 2015, 2, 269-279.	2.6	21
74	Hierarchical Bayesian modelling of disease progression to inform clinical trial design in centronuclear myopathy. <i>Orphanet Journal of Rare Diseases</i> , 2021, 16, 3.	2.7	21
75	Financial cost and quality of life of patients with spinal muscular atrophy identified by symptoms or newborn screening. <i>Developmental Medicine and Child Neurology</i> , 2023, 65, 67-77.	2.1	20
76	Anterior spinal artery syndrome after aortic surgery in a child. <i>Pediatric Neurology</i> , 2001, 24, 310-312.	2.1	19
77	Global versus individual muscle segmentation to assess quantitative MRI-based fat fraction changes in neuromuscular diseases. <i>European Radiology</i> , 2021, 31, 4264-4276.	4.5	19
78	Immediate Relief of Mycoplasma pneumoniae Encephalitis Symptoms After Intravenous Immunoglobulin. <i>Pediatric Neurology</i> , 2009, 41, 375-377.	2.1	18
79	Oligo-astrocytoma in LZTR1-related Noonan syndrome. <i>European Journal of Medical Genetics</i> , 2020, 63, 103617.	1.3	17
80	RESTORE: A Prospective Multinational Registry of Patients with Genetically Confirmed Spinal Muscular Atrophy - Rationale and Study Design. <i>Journal of Neuromuscular Diseases</i> , 2020, 7, 145-152.	2.6	17
81	Risdiplam: an investigational survival motor neuron 2 (SMN2) splicing modifier for spinal muscular atrophy (SMA). <i>Expert Opinion on Investigational Drugs</i> , 2022, 31, 451-461.	4.1	17
82	Effects of maternal alcohol consumption during breastfeeding on motor and cerebellar Purkinje cells behavior in mice. <i>Neuroscience Letters</i> , 2009, 455, 4-7.	2.1	16
83	Mild clinical presentation in KLHL40-related nemaline myopathy (NEM 8). <i>Neuromuscular Disorders</i> , 2016, 26, 712-716.	0.6	16
84	Home-Based Monitor for Gait and Activity Analysis. <i>Journal of Visualized Experiments</i> , 2019, , .	0.3	15
85	Quantitative nuclear magnetic resonance imaging detects subclinical changes over 1 year in skeletal muscle of GNE myopathy. <i>Journal of Neurology</i> , 2020, 267, 228-238.	3.6	14
86	Remote Digital Monitoring for Medical Product Development. <i>Clinical and Translational Science</i> , 2021, 14, 94-101.	3.1	14
87	Newborn screening of duchenne muscular dystrophy specifically targeting deletions amenable to exon-skipping therapy. <i>Scientific Reports</i> , 2021, 11, 3011.	3.3	14
88	Therapeutic interventions for spinal muscular atrophy: preclinical and early clinical development opportunities. <i>Expert Opinion on Investigational Drugs</i> , 2021, 30, 519-527.	4.1	14
89	High urinary ferritin reflects myoglobin iron evacuation in DMD patients. <i>Neuromuscular Disorders</i> , 2018, 28, 564-571.	0.6	13
90	Normalized grip strength is a sensitive outcome measure through all stages of Duchenne muscular dystrophy. <i>Journal of Neurology</i> , 2020, 267, 2022-2028.	3.6	13

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91	Lessons Learned from Discontinued Clinical Developments in Duchenne Muscular Dystrophy. <i>Frontiers in Pharmacology</i> , 2021, 12, 735912.	3.5	13
92	Real-world and natural history data for drug evaluation in Duchenne muscular dystrophy: suitability of the North Star Ambulatory Assessment for comparisons with external controls. <i>Neuromuscular Disorders</i> , 2022, 32, 271-283.	0.6	13
93	Onasemnogene abeparvovec for the treatment of spinal muscular atrophy. <i>Expert Opinion on Biological Therapy</i> , 2022, 22, 1075-1090.	3.1	13
94	Dystrophin Threshold Level Necessary for Normalization of Neuronal Nitric Oxide Synthase, Inducible Nitric Oxide Synthase, and Ryanodine Receptor-Calcium Release Channel Type 1 Nitrosylation in Golden Retriever Muscular Dystrophy Dystrophinopathy. <i>Human Gene Therapy</i> , 2016, 27, 712-726.	2.7	12
95	ASC α 1 Is a Cell Cycle Regulator Associated with Severe and Mild Forms of Myopathy. <i>Annals of Neurology</i> , 2020, 87, 217-232.	5.3	12
96	Relationship between markers of disease activity and progression in skeletal muscle of GNE myopathy patients using quantitative nuclear magnetic resonance imaging and ^{31}P nuclear magnetic resonance spectroscopy. <i>Quantitative Imaging in Medicine and Surgery</i> , 2020, 10, 1450-1464.	2.0	12
97	Myostatin: a Circulating Biomarker Correlating with Disease in Myotubular Myopathy Mice and Patients. <i>Molecular Therapy - Methods and Clinical Development</i> , 2020, 17, 1178-1189.	4.1	12
98	Diagnosing X-linked Myotubular Myopathy – A German 20-year Follow Up Experience. <i>Journal of Neuromuscular Diseases</i> , 2021, 8, 79-90.	2.6	12
99	Hearing impairment in patients with myotonic dystrophy type 2. <i>Neurology</i> , 2018, 90, e615-e622.	1.1	11
100	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
101	Newborn screening of neuromuscular diseases. <i>Neuromuscular Disorders</i> , 2021, 31, 1070-1080.	0.6	9
102	Normative data on spontaneous stride velocity, stride length, and walking activity in a non-controlled environment. <i>Orphanet Journal of Rare Diseases</i> , 2021, 16, 318.	2.7	9
103	Improving Care and Empowering Adults Living with SMA: A Call to Action in the New Treatment Era. <i>Journal of Neuromuscular Diseases</i> , 2021, 8, 543-551.	2.6	9
104	Upper limb disease evolution in exon 53 skipping eligible patients with Duchenne muscular dystrophy. <i>Annals of Clinical and Translational Neurology</i> , 2021, 8, 1938-1950.	3.7	9
105	Evaluation of the serum matrix metalloproteinase-9 as a biomarker for monitoring disease progression in Duchenne muscular dystrophy. <i>Neuromuscular Disorders</i> , 2015, 25, 444-446.	0.6	8
106	RNA-Seq Analysis of an Antisense Sequence Optimized for Exon Skipping in Duchenne Patients Reveals No Off-Target Effect. <i>Molecular Therapy - Nucleic Acids</i> , 2018, 10, 277-291.	5.1	8
107	Genotype-related respiratory progression in Duchenne muscular dystrophy – A multicenter international study. <i>Muscle and Nerve</i> , 2022, 65, 67-74.	2.2	8
108	Home-based gait analysis as an exploratory endpoint during a multicenter phase 1 trial in limb girdle muscular dystrophy type 2 and facioscapulohumeral muscular dystrophy. <i>Muscle and Nerve</i> , 2022, 65, 237-242.	2.2	8

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109	Prognostic Factors and Treatment Effect Modifiers in Spinal Muscular Atrophy. <i>Clinical Pharmacology and Therapeutics</i> , 2021, 110, 1435-1454.	4.7	7
110	Reader response: Discrepancy in redetermination of <i>SMN2</i> copy numbers in children with SMA. <i>Neurology</i> , 2020, 95, 144-145.	1.1	7
111	Congenital muscular dystrophy phenotype with neuromuscular spindles excess in a 5-year-old girl caused by HRAS mutation. <i>Neuromuscular Disorders</i> , 2014, 24, 993-998.	0.6	6
112	250th ENMC International Workshop: Clinical trial readiness in nemaline myopathy 6-8 September 2019, Hoofddorp, the Netherlands. <i>Neuromuscular Disorders</i> , 2020, 30, 866-875.	0.6	6
113	North Star Ambulatory Assessment changes in ambulant Duchenne boys amenable to skip exons 44, 45, 51, and 53: A 3 year follow up. <i>PLoS ONE</i> , 2021, 16, e0253882.	2.5	6
114	Crowdfunding for neuromuscular disease treatment: the ethical implications. <i>Lancet Neurology</i> , The, 2021, 20, 788-789.	10.2	6
115	FIREFISH Part 2: Efficacy and safety of risdiplam (RG7916) in infants with Type 1 spinal muscular atrophy (SMA)., 2020, , .		6
116	Response of plasma microRNAs to nusinersen treatment in patients with SMA. <i>Annals of Clinical and Translational Neurology</i> , 2022, 9, 1011-1026.	3.7	6
117	Abnormal Glycosylation Profile and High Alpha-Fetoprotein in a Patient with Twinkle Variants. <i>JIMD Reports</i> , 2016, 29, 109-113.	1.5	5
118	Multidisciplinary care allowing uneventful vaginal delivery in a woman with Pompe disease. <i>Neuromuscular Disorders</i> , 2016, 26, 610-613.	0.6	5
119	EGR2 mutation enhances phenotype spectrum of Dejerine Sottas syndrome. <i>Journal of Neurology</i> , 2016, 263, 1456-1458.	3.6	5
120	DMD and West syndrome. <i>Neuromuscular Disorders</i> , 2017, 27, 911-913.	0.6	5
121	Randomisation versus prioritisation in a managed access programme: Lessons from spinal muscular atrophy. <i>Neuromuscular Disorders</i> , 2020, 30, 267-269.	0.6	5
122	Respiratory management of spinal muscular atrophy type 1 patients treated with Nusinersen. <i>Pediatric Pulmonology</i> , 2022, 57, 1505-1512.	2.0	5
123	INCEPTUS Natural History, Run-in Study for Gene Replacement Clinical Trial in X-Linked Myotubular Myopathy. <i>Journal of Neuromuscular Diseases</i> , 2022, 9, 503-516.	2.6	5
124	Novel free-circulating and extracellular vesicle-derived miRNAs dysregulated in Duchenne muscular dystrophy. <i>Epigenomics</i> , 2020, 12, 1899-1915.	2.1	4
125	Muscular Dystrophy. <i>New England Journal of Medicine</i> , 2014, 371, e35.	27.0	3
126	Improved Muscular Weakness During Asthma Exacerbation. <i>JAMA Neurology</i> , 2017, 74, 353.	9.0	2

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127	“The Times They Are a-Changin’”™. In reply to El-Zaidy et al.: AVXS-101 (Onasemnogene Apeparvovec) for SMA1: Comparative Study with a Prospective Natural History Cohort. <i>Journal of Neuromuscular Diseases</i> , 2019, 6, 319-320.	2.6	2
128	Correspondence on: “Discrepancy in Spinal Muscular Atrophy Incidence findings in newborn screening programs: the influence of carrier screening?” by Kay et al. <i>Genetics in Medicine</i> , 2020, 22, 1913-1914.	2.4	2
129	E-Health & Innovation to Overcome Barriers in Neuromuscular Diseases. Report from the 1st eNMD Congress: Nice, France, March 22-23, 2019. <i>Journal of Neuromuscular Diseases</i> , 2021, 8, 743-754.	2.6	2
130	Video games to measure outcome for children with neuromuscular disorders. <i>Developmental Medicine and Child Neurology</i> , 2020, 62, 266-266.	2.1	1
131	Response to letter: A decision for life “ Treatment decisions in newly diagnosed families with spinal muscular atrophy. <i>European Journal of Paediatric Neurology</i> , 2021, 30, 103-104.	1.6	1
132	Odalisque's Position as a Geste Antagoniste in a Variant Phenotype of Ataxia-Telangiectasia. <i>Movement Disorders Clinical Practice</i> , 2019, 6, 413-414.	1.5	0
133	Leveraging Natural History Data in One- and Two-Arm Hierarchical Bayesian Studies of Rare Disease Progression. <i>Statistics in Biosciences</i> , 0, , 1.	1.2	0
134	23 Seksualiteit en verstandelijke beperking. , 2009, , 535-551.		0