

Terence R Flotte

List of Publications by Year in descending order

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136
papers

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citations

36303

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all docs

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docs citations

140
times ranked

6701
citing authors

#	ARTICLE	IF	CITATIONS
1	Treatment of Leber Congenital Amaurosis Due to RPE65 Mutations by Ocular Subretinal Injection of Adeno-Associated Virus Gene Vector: Short-Term Results of a Phase I Trial. <i>Human Gene Therapy</i> , 2008, 19, 979-990.	2.7	880
2	Human gene therapy for RPE65 isomerase deficiency activates the retinoid cycle of vision but with slow rod kinetics. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2008, 105, 15112-15117.	7.1	639
3	Defective regulation of outwardly rectifying Cl ⁻ channels by protein kinase A corrected by insertion of CFTR. <i>Nature</i> , 1992, 358, 581-584.	27.8	433
4	Mutational Analysis of the Adeno-Associated Virus Type 2 (AAV2) Capsid Gene and Construction of AAV2 Vectors with Altered Tropism. <i>Journal of Virology</i> , 2000, 74, 8635-8647.	3.4	344
5	Phase 2 Clinical Trial of a Recombinant Adeno-Associated Viral Vector Expressing α -1-Antitrypsin: Interim Results. <i>Human Gene Therapy</i> , 2011, 22, 1239-1247.	2.7	297
6	Sustained transgene expression despite T lymphocyte responses in a clinical trial of rAAV1-AAT gene therapy. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2009, 106, 16363-16368.	7.1	295
7	A Phase I Study of an Adeno-Associated Virus-CFTR Gene Vector in Adult CF Patients with Mild Lung Disease. Johns Hopkins Children's Center, Baltimore, Maryland. <i>Human Gene Therapy</i> , 1996, 7, 1145-1159.	2.7	273
8	α -1 Antitrypsin Inhibits Caspase-3 Activity, Preventing Lung Endothelial Cell Apoptosis. <i>American Journal of Pathology</i> , 2006, 169, 1155-1166.	3.8	270
9	A Phase II, Double-Blind, Randomized, Placebo-Controlled Clinical Trial of tgAAVCF Using Maxillary Sinus Delivery in Patients with Cystic Fibrosis with Antrostomies. <i>Human Gene Therapy</i> , 2002, 13, 1349-1359.	2.7	239
10	Efficient and persistent gene transfer of AAV-CFTR in maxillary sinus. <i>Lancet</i> , The, 1998, 351, 1702-1703.	13.7	220
11	Recombinant Adeno-Associated Virus Gene Therapy in Light of Luxturna (and Zolgensma and Glybera): Where Are We, and How Did We Get Here?. <i>Annual Review of Virology</i> , 2019, 6, 601-621.	6.7	217
12	Phase I Trial of Intranasal and Endobronchial Administration of a Recombinant Adeno-Associated Virus Serotype 2 (rAAV2)-CFTR Vector in Adult Cystic Fibrosis Patients: A Two-Part Clinical Study. <i>Human Gene Therapy</i> , 2003, 14, 1079-1088.	2.7	213
13	Safety of Recombinant Adeno-Associated Virus Type 2 RPE65 Vector Delivered by Ocular Subretinal Injection. <i>Molecular Therapy</i> , 2006, 13, 1074-1084.	8.2	196
14	Safety and Biological Efficacy of an Adeno-Associated Virus Vector-Cystic Fibrosis Transmembrane Regulator (AAV-CFTR) in the Cystic Fibrosis Maxillary Sinus. <i>Laryngoscope</i> , 1999, 109, 266-274.	2.0	193
15	Results at 2 Years after Gene Therapy for RPE65-Deficient Leber Congenital Amaurosis and Severe Early-Childhood Onset Retinal Dystrophy. <i>Ophthalmology</i> , 2016, 123, 1606-1620.	5.2	184
16	Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus Serotype 2 α -1-Antitrypsin (AAT) Vector in AAT-Deficient Adults. <i>Human Gene Therapy</i> , 2006, 17, 1177-1186.	2.7	168
17	Gene Expression from Adeno-associated Virus Vectors in Airway Epithelial Cells. <i>American Journal of Respiratory Cell and Molecular Biology</i> , 1992, 7, 349-356.	2.9	167
18	CMV- β -Actin Promoter Directs Higher Expression from an Adeno-Associated Viral Vector in the Liver than the Cytomegalovirus or Elongation Factor 1 α Promoter and Results in Therapeutic Levels of Human Factor X in Mice. <i>Human Gene Therapy</i> , 2001, 12, 563-573.	2.7	163

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19	Improved prime editors enable pathogenic allele correction and cancer modelling in adult mice. <i>Nature Communications</i> , 2021, 12, 2121.	12.8	155
20	Moving Forward After Two Deaths in a Gene Therapy Trial of Myotubular Myopathy. <i>Human Gene Therapy</i> , 2020, 31, 695-696.	2.7	145
21	Latent Adeno-Associated Virus Infection Elicits Humoral but Not Cell-Mediated Immune Responses in a Nonhuman Primate Model. <i>Journal of Virology</i> , 1999, 73, 8549-8558.	3.4	143
22	Safety in Nonhuman Primates of Ocular AAV2-RPE65, a Candidate Treatment for Blindness in Leber Congenital Amaurosis. <i>Human Gene Therapy</i> , 2006, 17, 845-858.	2.7	142
23	Human Treg responses allow sustained recombinant adeno-associated virus-mediated transgene expression. <i>Journal of Clinical Investigation</i> , 2013, 123, 5310-5318.	8.2	133
24	Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus Alpha 1-Antitrypsin (rAAV2-CB-hAAT) Gene Vector to AAT-Deficient Adults. <i>Human Gene Therapy</i> , 2004, 15, 93-128.	2.7	130
25	Lack of Cystic Fibrosis Transmembrane Conductance Regulator in CD3 ⁺ Lymphocytes Leads to Aberrant Cytokine Secretion and Hyperinflammatory Adaptive Immune Responses. <i>American Journal of Respiratory Cell and Molecular Biology</i> , 2011, 44, 922-929.	2.9	106
26	Gene therapy: The first two decades and the current state-of-the-art. <i>Journal of Cellular Physiology</i> , 2007, 213, 301-305.	4.1	105
27	Sustained miRNA-mediated Knockdown of Mutant AAT With Simultaneous Augmentation of Wild-type AAT Has Minimal Effect on Global Liver miRNA Profiles. <i>Molecular Therapy</i> , 2012, 20, 590-600.	8.2	105
28	A Phase I/II Study of tgAAV-CF for the Treatment of Chronic Sinusitis in Patients with Cystic Fibrosis. Stanford University, Stanford, California. <i>Human Gene Therapy</i> , 1998, 9, 889-909.	2.7	95
29	DNA-dependent PK inhibits adeno-associated virus DNA integration. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2004, 101, 2112-2116.	7.1	91
30	Immune Responses to Recombinant Adeno-Associated Virus Vectors: Putting Preclinical Findings into Perspective. <i>Human Gene Therapy</i> , 2004, 15, 716-717.	2.7	90
31	Repeated Delivery of Adeno-Associated Virus Vectors to the Rabbit Airway. <i>Journal of Virology</i> , 1999, 73, 9446-9455.	3.4	90
32	Intramuscular Administration of Recombinant Adeno-Associated Virus 2 \pm 1 Antitrypsin (rAAV-SERPINA1) Vectors in a Nonhuman Primate Model: Safety and Immunologic Aspects. <i>Molecular Therapy</i> , 2002, 6, 329-335.	8.2	87
33	Efficient Transduction of Vascular Endothelial Cells with Recombinant Adeno-Associated Virus Serotype 1 and 5 Vectors. <i>Human Gene Therapy</i> , 2005, 16, 235-247.	2.7	84
34	5 Year Expression and Neutrophil Defect Repair after Gene Therapy in Alpha-1 Antitrypsin Deficiency. <i>Molecular Therapy</i> , 2017, 25, 1387-1394.	8.2	84
35	A Single Intravenous rAAV Injection as Late as P20 Achieves Efficacious and Sustained CNS Gene Therapy in Canavan Mice. <i>Molecular Therapy</i> , 2013, 21, 2136-2147.	8.2	77
36	Efficient and Targeted Transduction of Nonhuman Primate Liver With Systemically Delivered Optimized AAV3B Vectors. <i>Molecular Therapy</i> , 2015, 23, 1867-1876.	8.2	73

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37	Histone Deacetylase Inhibitor (HDAC) Suberoylanilide Hydroxamic Acid (SAHA)-mediated Correction of α 1-Antitrypsin Deficiency. <i>Journal of Biological Chemistry</i> , 2012, 287, 38265-38278.	3.4	72
38	Adeno-Associated Virus Type 2 and Hepatocellular Carcinoma?. <i>Human Gene Therapy</i> , 2015, 26, 779-781.	2.7	71
39	Viral Vector-mediated and Cell-based Therapies for Treatment of Cystic Fibrosis. <i>Molecular Therapy</i> , 2007, 15, 229-241.	8.2	67
40	Therapeutic level of functional human alpha 1 antitrypsin (hAAT) secreted from murine muscle transduced by adeno-associated virus (rAAV1) vector. <i>Journal of Gene Medicine</i> , 2006, 8, 730-735.	2.8	62
41	Survival Advantage of Both Human Hepatocyte Xenografts and Genome-Edited Hepatocytes for Treatment of α 1 Antitrypsin Deficiency. <i>Molecular Therapy</i> , 2017, 25, 2477-2489.	8.2	62
42	Editing out five <i>Serpina1</i> paralogs to create a mouse model of genetic emphysema. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2018, 115, 2788-2793.	7.1	62
43	Production of clinical-grade recombinant adeno-associated virus vectors. <i>Current Opinion in Biotechnology</i> , 2002, 13, 418-423.	6.6	61
44	Adeno-Associated Virus: A Ubiquitous Commensal of Mammals. <i>Human Gene Therapy</i> , 2005, 16, 401-407.	2.7	60
45	Alternate Translation Initiation Codons Can Create Functional Forms of Cystic Fibrosis Transmembrane Conductance Regulator. <i>Journal of Biological Chemistry</i> , 1995, 270, 11941-11946.	3.4	58
46	Preclinical Evaluation of a Recombinant Adeno-Associated Virus Vector Expressing Human Alpha-1 Antitrypsin Made Using a Recombinant Herpes Simplex Virus Production Method. <i>Human Gene Therapy</i> , 2011, 22, 155-165.	2.7	58
47	A Safe and Reliable Technique for CNS Delivery of AAV Vectors in the Cisterna Magna. <i>Molecular Therapy</i> , 2020, 28, 411-421.	8.2	58
48	Functional Characterization of a Recombinant Adeno-Associated Virus 5-Pseudotyped Cystic Fibrosis Transmembrane Conductance Regulator Vector. <i>Human Gene Therapy</i> , 2004, 15, 832-841.	2.7	57
49	In vivo post-transcriptional gene silencing of α 1 antitrypsin by adeno-associated virus vectors expressing siRNA. <i>Laboratory Investigation</i> , 2007, 87, 893-902.	3.7	57
50	<i>In Vivo</i> Genome Editing Partially Restores Alpha1-Antitrypsin in a Murine Model of AAT Deficiency. <i>Human Gene Therapy</i> , 2018, 29, 853-860.	2.7	54
51	Recent Developments in Recombinant AAV-Mediated Gene Therapy for Lung Diseases. <i>Current Gene Therapy</i> , 2005, 5, 361-366.	2.0	53
52	Efficient Hepatic Delivery and Expression from a Recombinant Adeno-associated Virus 8 Pseudotyped α 1-Antitrypsin Vector. <i>Molecular Therapy</i> , 2005, 12, 867-875.	8.2	53
53	Class I-restricted T-cell responses to a polymorphic peptide in a gene therapy clinical trial for α 1-antitrypsin deficiency. <i>Proceedings of the National Academy of Sciences of the United States of America</i> , 2017, 114, 1655-1659.	7.1	52
54	Dual Reporter Comparative Indexing of rAAV Pseudotyped Vectors in Chimpanzee Airway. <i>Molecular Therapy</i> , 2010, 18, 594-600.	8.2	49

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55	AAV gene therapy for Tay-Sachs disease. <i>Nature Medicine</i> , 2022, 28, 251-259.	30.7	49
56	Expression of a Truncated Cystic Fibrosis Transmembrane Conductance Regulator with an AAV5-pseudotyped Vector in Primates. <i>Molecular Therapy</i> , 2007, 15, 756-763.	8.2	48
57	Preclinical Characterization of A Recombinant Adeno-Associated Virus Type 1-Pseudotyped Vector Demonstrates Dose-Dependent Injection Site Inflammation And Dissemination of Vector Genomes to Distant Sites. <i>Human Gene Therapy</i> , 2007, 18, 245-256.	2.7	48
58	Gene Therapy for Cystic Fibrosis. <i>Clinical Reviews in Allergy and Immunology</i> , 2008, 35, 164-178.	6.5	48
59	Recombinant AAV Serotype and Capsid Mutant Comparison for Pulmonary Gene Transfer of α -1-Antitrypsin Using Invasive and Noninvasive Delivery. <i>Molecular Therapy</i> , 2009, 17, 81-87.	8.2	48
60	Results at 5 Years After Gene Therapy for RPE65-Deficient Retinal Dystrophy. <i>Human Gene Therapy</i> , 2018, 29, 1428-1437.	2.7	48
61	Gene-Based Therapy for Alpha-1 Antitrypsin Deficiency. <i>COPD: Journal of Chronic Obstructive Pulmonary Disease</i> , 2013, 10, 44-49.	1.6	47
62	Adeno-Associated Virus-Based Gene Therapy for Inherited Disorders. <i>Pediatric Research</i> , 2005, 58, 1143-1147.	2.3	45
63	CAR T-Cell Therapy: Progress and Prospects. <i>Human Gene Therapy Methods</i> , 2017, 28, 61-66.	2.1	45
64	Gene therapy for alpha-1 antitrypsin deficiency. <i>Human Molecular Genetics</i> , 2011, 20, R87-R92.	2.9	43
65	Effect of Cigarette Smoke Exposure and Structural Modifications on the α -1 Antitrypsin Interaction with Caspases. <i>Molecular Medicine</i> , 2012, 18, 445-454.	4.4	43
66	Successful transgene expression with serial doses of aerosolized rAAV2 vectors in rhesus macaques. <i>Molecular Therapy</i> , 2003, 8, 918-926.	8.2	42
67	A Rationally Engineered Capsid Variant of AAV9 for Systemic CNS-Directed and Peripheral Tissue-Detargeted Gene Delivery in Neonates. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 9, 234-246.	4.1	42
68	Accelerated Graduation and the Deployment of New Physicians During the COVID-19 Pandemic. <i>Academic Medicine</i> , 2020, 95, 1492-1494.	1.6	41
69	Functional Characterization of a Recombinant Adeno-Associated Virus 5-Pseudotyped Cystic Fibrosis Transmembrane Conductance Regulator Vector. <i>Human Gene Therapy</i> , 2004, 15, 832-841.	2.7	41
70	Recombinant Adeno-Associated Virus Integration Sites in Murine Liver After Ornithine Transcarbamylase Gene Correction. <i>Human Gene Therapy</i> , 2013, 24, 520-525.	2.7	40
71	Adeno-Associated Virus Neutralizing Antibodies in Large Animals and Their Impact on Brain Intraparenchymal Gene Transfer. <i>Molecular Therapy - Methods and Clinical Development</i> , 2018, 11, 65-72.	4.1	38
72	Ex Vivo transduced liver progenitor cells as a platform for gene therapy in mice. <i>Hepatology</i> , 2004, 40, 918-924.	7.3	37

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73	Recombinant Adeno-Associated Virus-Mediated Global Anterograde Delivery of Glial Cell Line-Derived Neurotrophic Factor to the Spinal Cord: Comparison of Rubrospinal and Corticospinal Tracts in the Rat. <i>Human Gene Therapy</i> , 2008, 19, 71-82.	2.7	36
74	Correlation Between DNA Transfer and Cystic Fibrosis Airway Epithelial Cell Correction After Recombinant Adeno-Associated Virus Serotype 2 Gene Therapy. <i>Human Gene Therapy</i> , 2005, 16, 921-928.	2.7	35
75	Delayed Expression of Adeno-Associated Virus Vector DNA. <i>Intervirology</i> , 1999, 42, 213-220.	2.8	33
76	Recombinant Adeno-Associated Virus Vector-Based Gene Transfer for Defects in Oxidative Metabolism. <i>Human Gene Therapy</i> , 2000, 11, 2067-2078.	2.7	33
77	Cystic Fibrosis Transmembrane Regulator Missing the First Four Transmembrane Segments Increases Wild Type and $\Delta F508$ Processing*. <i>Journal of Biological Chemistry</i> , 2008, 283, 21926-21933.	3.4	33
78	The rapidly evolving state of gene therapy. <i>FASEB Journal</i> , 2018, 32, 1733-1740.	0.5	33
79	Localized Gene Expression Following Administration of Adeno-associated Viral Vectors via Pancreatic Ducts. <i>Molecular Therapy</i> , 2005, 12, 519-527.	8.2	30
80	Enhanced IgE allergic response to <i>Aspergillus fumigatus</i> in CFTR $\Delta F508/\Delta F508$ mice. <i>Laboratory Investigation</i> , 2006, 86, 130-140.	3.7	29
81	Partial correction of the CFTR $\Delta F508$ -dependent ABPA mouse model with recombinant adeno-associated virus gene transfer of truncated CFTR gene. <i>Journal of Gene Medicine</i> , 2008, 10, 51-60.	2.8	29
82	Current status of gene therapy for α_1 -1 antitrypsin deficiency. <i>Expert Opinion on Biological Therapy</i> , 2015, 15, 329-336.	3.1	29
83	CRISPR/Cas-Dependent and Nuclease-Free <i>In Vivo</i> Therapeutic Gene Editing. <i>Human Gene Therapy</i> , 2021, 32, 275-293.	2.7	26
84	[53] Adeno-associated virus vectors for gene therapy of cystic fibrosis. <i>Methods in Enzymology</i> , 1998, 292, 717-732.	1.0	24
85	Muscle-Directed Delivery of an AAV1 Vector Leads to Capsid-Specific T Cell Exhaustion in Nonhuman Primates and Humans. <i>Molecular Therapy</i> , 2020, 28, 747-757.	8.2	23
86	Recombinant Adeno-Associated Virus Gene Therapy for Cystic Fibrosis and α_1 -Antitrypsin Deficiency. <i>Chest</i> , 2002, 121, 98S-102S.	0.8	21
87	Long-term Correction of Very Long-chain Acyl-CoA Dehydrogenase Deficiency in Mice Using AAV9 Gene Therapy. <i>Molecular Therapy</i> , 2012, 20, 1131-1138.	8.2	20
88	Development of rAAV2-CFTR: History of the First rAAV Vector Product to be Used in Humans. <i>Human Gene Therapy Methods</i> , 2016, 27, 49-58.	2.1	19
89	Recombinant Adeno-Associated Virus Vector Genomes Take the Form of Long-Lived, Transcriptionally Competent Episomes in Human Muscle. <i>Human Gene Therapy</i> , 2016, 27, 32-42.	2.7	18
90	Systemic Correction of a Fatty Acid Oxidation Defect by Intramuscular Injection of a Recombinant Adeno-Associated Virus Vector. <i>Human Gene Therapy</i> , 2006, 17, 71-80.	2.7	17

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91	Cell and Gene Therapy for Genetic Diseases: Inherited Disorders Affecting the Lung and Those Mimicking Sudden Infant Death Syndrome. <i>Human Gene Therapy</i> , 2012, 23, 548-556.	2.7	17
92	Birth of a New Therapeutic Platform: 47 Years of Adeno-associated Virus Biology From Virus Discovery to Licensed Gene Therapy. <i>Molecular Therapy</i> , 2013, 21, 1976-1981.	8.2	16
93	Modulating Immune Responses to AAV by expanded polyclonal T-regulatory cells and capsid specific chimeric antigen receptor T-regulatory cells. <i>Molecular Therapy - Methods and Clinical Development</i> , 2021, 23, 490-506.	4.1	16
94	CFTR mutations impart elevated immune reactivity in a murine model of cystic fibrosis related diabetes. <i>Cytokine</i> , 2008, 44, 154-159.	3.2	15
95	Adeno-Associated Virus-Mediated Gene Transfer for Lung Diseases. <i>Human Gene Therapy</i> , 2005, 16, 643-648.	2.7	13
96	Gene Transfer in the Lung Using Recombinant Adeno-associated Virus. <i>Current Protocols in Microbiology</i> , 2012, 26, Unit14D.2.	6.5	13
97	Progress with Recombinant Adeno-Associated Virus Vectors for Gene Therapy of Alpha-1 Antitrypsin Deficiency. <i>Human Gene Therapy Methods</i> , 2015, 26, 77-81.	2.1	12
98	Retro-Orbital Venous Sinus Delivery of rAAV9 Mediates High-Level Transduction of Brain and Retina Compared with Temporal Vein Delivery in Neonatal Mouse Pups. <i>Human Gene Therapy</i> , 2017, 28, 228-230.	2.7	12
99	Two-Plasmid Packaging System for Recombinant Adeno-Associated Virus. <i>BioResearch Open Access</i> , 2020, 9, 219-228.	2.6	12
100	Common pathways to Dean of Medicine at U.S. medical schools. <i>PLoS ONE</i> , 2021, 16, e0249078.	2.5	12
101	Real time laryngoscopy with olfactory challenge for diagnosis of psychogenic stridor. <i>Pediatric Pulmonology</i> , 1993, 16, 259-262.	2.0	10
102	Biochemical Correction of Short-Chain Acyl-Coenzyme A Dehydrogenase Deficiency After Portal Vein Injection of rAAV8-SCAD. <i>Human Gene Therapy</i> , 2008, 19, 579-588.	2.7	10
103	Charting a Clear Path: The ASGCT Standardized Pathways Conference. <i>Molecular Therapy</i> , 2014, 22, 1235-1238.	8.2	10
104	Bridging from Intramuscular to Limb Perfusion Delivery of rAAV: Optimization in a Non-human Primate Study. <i>Molecular Therapy - Methods and Clinical Development</i> , 2019, 13, 233-242.	4.1	10
105	Recent developments in the protection of pediatric research subjects. <i>Journal of Pediatrics</i> , 2006, 149, 285-286.e2.	1.8	9
106	Therapeutics: Gene Therapy for Alpha-1 Antitrypsin Deficiency. <i>Methods in Molecular Biology</i> , 2017, 1639, 267-275.	0.9	9
107	Towards a rAAV-based gene therapy for ADA-SCID: from ADA deficiency to current and future treatment strategies. <i>Pharmacogenomics</i> , 2008, 9, 947-968.	1.3	8
108	N-Glycosylation Augmentation of the Cystic Fibrosis Epithelium Improves <i>Pseudomonas aeruginosa</i> Clearance. <i>American Journal of Respiratory Cell and Molecular Biology</i> , 2011, 44, 824-830.	2.9	8

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109	Recombinant adeno-associated virus-mediated gene delivery of long chain acyl coenzyme A dehydrogenase (LCAD) into LCAD-deficient mice. <i>Journal of Gene Medicine</i> , 2008, 10, 1113-1123.	2.8	7
110	Apparently Nonspecific Enzyme Elevations After Portal Vein Delivery of Recombinant Adeno-Associated Virus Serotype 2 Vector in Hepatitis C Virus-Infected Chimpanzees. <i>Human Gene Therapy</i> , 2008, 19, 681-689.	2.7	7
111	Modulation of Exaggerated-IgE Allergic Responses by Gene Transfer-mediated Antagonism of IL-13 and IL-17e. <i>Molecular Therapy</i> , 2010, 18, 511-518.	8.2	7
112	Large-scale molecular epidemiological analysis of AAV in a cancer patient population. <i>Oncogene</i> , 2021, 40, 3060-3071.	5.9	7
113	Gene Therapy for Rare Neurological Disorders. <i>Clinical Pharmacology and Therapeutics</i> , 2022, 111, 743-757.	4.7	7
114	Recombinant Adeno-Associated Virus-Mediated Gene Transfer for the Potential Therapy of Adenosine Deaminase-Deficient Severe Combined Immune Deficiency. <i>Human Gene Therapy</i> , 2011, 22, 935-949.	2.7	6
115	Ethical Implications of the Cost of Molecularly Targeted Therapies. <i>Human Gene Therapy</i> , 2015, 26, 573-574.	2.7	6
116	In vivo gene editing works in humans: Results of a phase 1 clinical trial for TTR amyloidosis. <i>Molecular Therapy</i> , 2021, 29, 2633-2634.	8.2	6
117	AAV9 gene replacement therapy for respiratory insufficiency in very long chain acyl-CoA dehydrogenase deficiency. <i>Journal of Inherited Metabolic Disease</i> , 2019, 42, 870-877.	3.6	5
118	Volume and Infusion Rate Dynamics of Intraparenchymal Central Nervous System Infusion in a Large Animal Model. <i>Human Gene Therapy</i> , 2020, 31, 617-625.	2.7	5
119	Liver-directed SERPINA1 gene therapy attenuates progression of spontaneous and tobacco smoke-induced emphysema in α 1-antitrypsin null mice. <i>Molecular Therapy - Methods and Clinical Development</i> , 2022, 25, 425-438.	4.1	5
120	Delivery of Adeno-Associated Virus Gene Therapy by Intravascular Limb Infusion Methods. <i>Human Gene Therapy Clinical Development</i> , 2015, 26, 159-164.	3.1	4
121	Engraftment of Human Hepatocytes in the PiZ-NSG Mouse Model. <i>Methods in Molecular Biology</i> , 2020, 2164, 75-85.	0.9	4
122	Preclinical Study Design for rAAV. <i>Methods in Molecular Biology</i> , 2012, 807, 317-337.	0.9	3
123	What Is Suppression of Anti-Adeno-Associated Virus Capsid T-Cells Achieving?. <i>Human Gene Therapy</i> , 2014, 25, 178-179.	2.7	3
124	Supporting Families Considering Participation in a Clinical Trial: Parent-Provider Perspectives. <i>Pediatrics</i> , 2021, 147, .	2.1	3
125	Adeno-Associated Viral Vectors for CF Gene Therapy. , 2002, 70, 599-608.		2
126	Moving Forward Toward a Cure for Parkinson's: Neuropathology of the Nigrostriatal Pathway Determines the Location of Growth Factor Delivery. <i>Molecular Therapy</i> , 2011, 19, 827-829.	8.2	2

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127	Quantification of Total Human Alpha-1 Antitrypsin by Sandwich ELISA. <i>Methods in Molecular Biology</i> , 2017, 1639, 211-216.	0.9	2
128	The Gene Therapy Resource Program: A Decade of Dedication to Translational Research by the National Heart, Lung, and Blood Institute. <i>Human Gene Therapy Clinical Development</i> , 2017, 28, 178-186.	3.1	2
129	DNA Vaccination in 2018: An Update. <i>Human Gene Therapy</i> , 2018, 29, 963-965.	2.7	1
130	The signal and the trap: Targeted delivery and retention of proteins in the mitochondrion. <i>Molecular Therapy</i> , 2003, 7, 715-716.	8.2	0
131	In Utero Efficacy of Cystic Fibrosis Gene Therapy: Difficult Studies, Positive or Negative. <i>Molecular Therapy</i> , 2008, 16, 806-807.	8.2	0
132	Getting Tough on Capsid Screening: Tough Decoys Enable Barcoding of Vectors Capable of both Entry and Expression. <i>Human Gene Therapy</i> , 2019, 30, 919-920.	2.7	0
133	Gene and Cell Therapy for Inherited and Acquired Immune Deficiency. <i>Human Gene Therapy</i> , 2021, 32, 1-3.	2.7	0
134	Liver targeting with rAAV7: balancing tropism with immune profiles. <i>Gene Therapy</i> , 2021, 28, 115-116.	4.5	0
135	In Reply to Ramotshwana et al. <i>Academic Medicine</i> , 2021, 96, e15-e16.	1.6	0
136	Muscle-Directed Gene Therapy for Alpha-1 Antitrypsin Deficiency. , 2019, , 775-786.		0