# Adrian J Thrasher

List of Publications by Year
in descending order



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|---|---|---|---|
| 466 papers | 37,080 citations | 2101 100 h-index | 4432 172 g-index |
| 478 all docs | 478 docs citations | 478 times ranked | 29952 citing authors |

| # | Article | IF | Citations |
|---|---|---|---|
| 1 | Overactive WASp in X-linked neutropenia leads to aberrant B-cell division and accelerated plasma cell generation. Journal of Allergy and Clinical Immunology, 2022, 149, 1069-1084. | 2.9 | 5 |
| 2 | Novel human liver-tropic AAV variants define transferable domains that markedly enhance the human tropism of AAV7 and AAV8. Molecular Therapy - Methods and Clinical Development, 2022, 24, 88-101. | 4.1 | 21 |
| 3 | Long-term safety and efficacy of lentiviral hematopoietic stem/progenitor cell gene therapy for Wiskottâ€“Aldrich syndrome. Nature Medicine, 2022, 28, 71-80. | 30.7 | 64 |
| 4 | Betibeglogene Autotemcel Gene Therapy for Nonâ€“Î² <sup>0</sup> /Î² <sup>0</sup> Genotype Î²-Thalassemia. New England Journal of Medicine, 2022, 386, 415-427. | 27.0 | 91 |
| 5 | AAV-p40 Bioengineering Platform for Variant Selection Based on Transgene Expression. Human Gene Therapy, 2022, 33, 664-682. | 2.7 | 16 |
| 6 | Critical role of WASp in germinal center tolerance through regulation of B cell apoptosis and diversification. Cell Reports, 2022, 38, 110474. | 6.4 | 4 |
| 7 | Gene therapy for Whiskottâ€“Aldrich syndrome: The latest news. Clinical and Translational Medicine, 2022, 12, e815. | 4.0 | 2 |
| 8 | Lentiviral Mediated ADA2 Gene Transfer Corrects the Defects Associated With Deficiency of Adenosine Deaminase Type 2. Frontiers in Immunology, 2022, 13, 852830. | 4.8 | 7 |
| 9 | Genome Editing With TALEN, CRISPR-Cas9 and CRISPR-Cas12a in Combination With AAV6 Homology Donor Restores T Cell Function for XLP. Frontiers in Genome Editing, 2022, 4, . | 5.2 | 8 |
| 10 | Gene therapy using haematopoietic stem and progenitor cells. Nature Reviews Genetics, 2021, 22, 216-234. | 16.3 | 151 |
| 11 | Gene Editing for the Treatment of Primary Immunodeficiency Diseases. Human Gene Therapy, 2021, 32, 43-51. | 2.7 | 23 |
| 12 | Gene and Cell Therapy for Inherited and Acquired Immune Deficiency. Human Gene Therapy, 2021, 32, 1-3. | 2.7 | 0 |
| 13 | ILâ€18: A potential inflammation biomarker in Wiskottâ€“Aldrich syndrome. European Journal of Immunology, 2021, 51, 1285-1288. | 2.9 | 1 |
| 14 | Long-term lymphoid progenitors independently sustain naÃ¯ve T and NK cell production in humans. Nature Communications, 2021, 12, 1622. | 12.8 | 2 |
| 15 | Autologous Ex Vivo Lentiviral Gene Therapy for Adenosine Deaminase Deficiency. New England Journal of Medicine, 2021, 384, 2002-2013. | 27.0 | 122 |
| 16 | Clonal expansion of T memory stem cells determines early anti-leukemic responses and long-term CAR T cell persistence in patients. Nature Cancer, 2021, 2, 629-642. | 13.2 | 59 |
| 17 | Predicting genotoxicity of viral vectors for stem cell gene therapy using gene expression-based machine learning. Molecular Therapy, 2021, 29, 3383-3397. | 8.2 | 25 |
| 18 | Safety and efficacy of an engineered hepatotropic AAV gene therapy for ornithine transcarbamylase deficiency in cynomolgus monkeys. Molecular Therapy - Methods and Clinical Development, 2021, 23, 135-146. | 4.1 | 21 |

| # | Article | IF | Citations |
|---|---|---|---|
| 19 | Preclinical Optimization and Safety Studies of a New Lentiviral Gene Therapy for p47<sup>phox</sup>-Deficient Chronic Granulomatous Disease. Human Gene Therapy, 2021, 32, 949-958. | 2.7 | 4 |
| 20 | Restoring Iron Homeostasis in Pts Who Achieved Transfusion Independence after Treatment with Betibeglogene Autotemcel Gene Therapy: Results from up to 7 Years of Follow-up. Blood, 2021, 138, 573-573. | 1.4 | 2 |
| 21 | A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results. Blood, 2021, 138, 2932-2932. | 1.4 | 5 |
| 22 | Improvement in Health-Related Quality of Life Following Treatment with Betibeglogene Autotemcel in Patients with Transfusion-Dependent Î²-Thalassemia Enrolled in Phase 3 Studies. Blood, 2021, 138, 3085-3085. | 1.4 | 3 |
| 23 | Gene Therapy for Fanconi Anemia [Group A]: Interim Results of RP-L102 Clinical Trials. Blood, 2021, 138, 3968-3968. | 1.4 | 1 |
| 24 | Gene therapy and genome editing for primary immunodeficiency diseases. Genes and Diseases, 2020, 7, 38-51. | 3.4 | 26 |
| 25 | Correction of both immunodeficiency and hypoparathyroidism by thymus transplantation in complete DiGeorge syndrome. American Journal of Transplantation, 2020, 20, 1447-1450. | 4.7 | 9 |
| 26 | Lentiviral Vector Production Titer Is Not Limited in HEK293T by Induced Intracellular Innate Immunity. Molecular Therapy - Methods and Clinical Development, 2020, 17, 209-219. | 4.1 | 22 |
| 27 | Differential Transgene Silencing of Myeloid-Specific Promoters in the AAVS1 Safe Harbor Locus of Induced Pluripotent Stem Cell-Derived Myeloid Cells. Human Gene Therapy, 2020, 31, 199-210. | 2.7 | 31 |
| 28 | Lentiviral Hematopoietic Stem Cell Gene Therapy Rescues Clinical Phenotypes in a Murine Model of Pompe Disease. Molecular Therapy - Methods and Clinical Development, 2020, 18, 558-570. | 4.1 | 11 |
| 29 | Targeted gene correction of human hematopoietic stem cells for the treatment of Wiskott -ÂAldrich Syndrome. Nature Communications, 2020, 11, 4034. | 12.8 | 87 |
| 30 | WAS Promoter-Driven Lentiviral Vectors Mimic Closely the Lopsided WASP Expression during Megakaryocytic Differentiation. Molecular Therapy - Methods and Clinical Development, 2020, 19, 220-235. | 4.1 | 4 |
| 31 | Restoring the natural tropism of AAV2 vectors for human liver. Science Translational Medicine, 2020, 12, . | 12.4 | 41 |
| 32 | Gene Editing and Genotoxicity: Targeting the Off-Targets. Frontiers in Genome Editing, 2020, 2, 613252. | 5.2 | 31 |
| 33 | An intronic deletion in megakaryoblastic leukemia 1 is associated with hyperproliferation of B cells in triplets with Hodgkin lymphoma. Haematologica, 2020, 105, 1339-1350. | 3.5 | 13 |
| 34 | Whole-genome sequencing of a sporadic primary immunodeficiency cohort. Nature, 2020, 583, 90-95. | 27.8 | 148 |
| 35 | Lentiviral gene therapy rescues p47phox chronic granulomatous disease and the ability to fight Salmonella infection in mice. Gene Therapy, 2020, 27, 459-469. | 4.5 | 11 |
| 36 | Whole-genome sequencing of patients with rare diseases in a national health system. Nature, 2020, 583, 96-102. | 27.8 | 338 |

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|---|---|---|---|
| 37 | Attenuation of Heparan Sulfate Proteoglycan Binding Enhances InÂVivo Transduction of Human Primary Hepatocytes with AAV2. Molecular Therapy - Methods and Clinical Development, 2020, 17, 1139-1154. | 4.1 | 29 |
| 38 | Gene therapy for X-linked severe combined immunodeficiency: Historical outcomes and current status. Journal of Allergy and Clinical Immunology, 2020, 146, 258-261. | 2.9 | 19 |
| 39 | Key diagnostic markers for autoimmune lymphoproliferative syndrome with molecular genetic diagnosis. Blood, 2020, 136, 1933-1945. | 1.4 | 24 |
| 40 | Safety of Autologous Hematopoietic Stem Cell Transplantation with Gene Addition Therapy for Transfusion-Dependent Î²-Thalassemia, Sickle Cell Disease, and Cerebral Adrenoleukodystrophy. Biology of Blood and Marrow Transplantation, 2020, 26, S38-S39. | 2.0 | 3 |
| 41 | Lentiviral gene therapy for X-linked chronic granulomatous disease. Nature Medicine, 2020, 26, 200-206. | 30.7 | 175 |
| 42 | High-Throughput In Vitro, Ex Vivo, and In Vivo Screen of Adeno-Associated Virus Vectors Based on Physical and Functional Transduction. Human Gene Therapy, 2020, 31, 575-589. | 2.7 | 65 |
| 43 | Interim Results from the Phase 3 Hgb-207 (Northstar-2) and Hgb-212 (Northstar-3) Studies of Betibeglogene Autotemcel Gene Therapy (LentiGlobin) for the Treatment of Transfusion-Dependent Î²-Thalassemia. Biology of Blood and Marrow Transplantation, 2020, 26, S87-S88. | 2.0 | 8 |
| 44 | Characterization of the clinical and immunologic phenotype and management of 157 individuals with 56 distinct heterozygous NFKB1 mutations. Journal of Allergy and Clinical Immunology, 2020, 146, 901-911. | 2.9 | 78 |
| 45 | Successful Preclinical Development of Gene Therapy for Recombinase-Activating Gene-1-Deficient SCID. Molecular Therapy - Methods and Clinical Development, 2020, 17, 666-682. | 4.1 | 37 |
| 46 | Clonal tracking in gene therapy patients reveals a diversity of human hematopoietic differentiation programs. Blood, 2020, 135, 1219-1231. | 1.4 | 50 |
| 47 | Wiskott Aldrich syndrome protein regulates non-selective autophagy and mitochondrial homeostasis in human myeloid cells. ELife, 2020, 9, . | 6.0 | 18 |
| 48 | Bleeding and splenectomy in Wiskott-Aldrich syndrome: A single-centre experience. Journal of Allergy and Clinical Immunology: in Practice, 2019, 7, 1042-1044.e1. | 3.8 | 10 |
| 49 | Age-Related Seroprevalence of Antibodies Against AAV-LK03 in a UK Population Cohort. Human Gene Therapy, 2019, 30, 79-87. | 2.7 | 51 |
| 50 | In Utero Gene Therapy (IUGT) Using GLOBE Lentiviral Vector Phenotypically Corrects the Heterozygous Humanised Mouse Model and Its Progress Can Be Monitored Using MRI Techniques. Scientific Reports, 2019, 9, 11592. | 3.3 | 15 |
| 51 | Gene therapy for primary immunodeficiency. Human Molecular Genetics, 2019, 28, R15-R23. | 2.9 | 55 |
| 52 | Generation and Clinical Application of Gene-Modified Autologous Epidermal Sheets in Netherton Syndrome: Lessons Learned from a Phase 1 Trial. Human Gene Therapy, 2019, 30, 1067-1078. | 2.7 | 27 |
| 53 | Enhancing Lentiviral and Alpharetroviral Transduction of Human Hematopoietic Stem Cells for Clinical Application. Molecular Therapy - Methods and Clinical Development, 2019, 14, 134-147. | 4.1 | 37 |
| 54 | Loss of Janus Associated Kinase 1 Alters Urothelial Cell Function and Facilitates the Development of Bladder Cancer. Frontiers in Immunology, 2019, 10, 2065. | 4.8 | 9 |

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|---|---|---|---|
| 55 | Targeted Repair of p47-CGD in iPSCs by CRISPR/Cas9: Functional Correction without Cleavage in the Highly Homologous Pseudogenes. Stem Cell Reports, 2019, 13, 590-598. | 4.8 | 20 |
| 56 | Loss of the interleukin-6 receptor causes immunodeficiency, atopy, and abnormal inflammatory responses. Journal of Experimental Medicine, 2019, 216, 1986-1998. | 8.5 | 153 |
| 57 | FAS mutations are an uncommon cause of immune thrombocytopenia in children and adults without additional features of immunodeficiency. British Journal of Haematology, 2019, 186, e163-e165. | 2.5 | 6 |
| 58 | Genome editing for blood disorders: state of the art and recent advances. Emerging Topics in Life Sciences, 2019, 3, 289-299. | 2.6 | 4 |
| 59 | In Utero Transplantation of Expanded Autologous Amniotic Fluid Stem Cells Results in Long-Term Hematopoietic Engraftment. Stem Cells, 2019, 37, 1176-1188. | 3.2 | 13 |
| 60 | How I manage patients with Wiskott Aldrich syndrome. British Journal of Haematology, 2019, 185, 647-655. | 2.5 | 37 |
| 61 | The European Society for Immunodeficiencies (ESID) Registry Working Definitions for the ClinicalÂDiagnosis of Inborn Errors of Immunity. Journal of Allergy and Clinical Immunology: in Practice, 2019, 7, 1763-1770. | 3.8 | 381 |
| 62 | Lentiglobin Gene Therapy for Transfusion-Dependent Î²-Thalassemia: Outcomes from the Phase 1/2 Northstar and Phase 3 Northstar-2 Studies. Biology of Blood and Marrow Transplantation, 2019, 25, S66-S67. | 2.0 | 3 |
| 63 | 26â€...What tests are useful for ALPS?. , 2019, , . | | 0 |
| 64 | Codon-Optimization of Wild-Type Adeno-Associated Virus Capsid Sequences Enhances DNA Family Shuffling while Conserving Functionality. Molecular Therapy - Methods and Clinical Development, 2019, 12, 71-84. | 4.1 | 22 |
| 65 | EROS/CYBC1 mutations: Decreased NADPH oxidase function and chronic granulomatous disease. Journal of Allergy and Clinical Immunology, 2019, 143, 782-785.e1. | 2.9 | 59 |
| 66 | Safety and early efficacy outcomes for lentiviral fibroblast gene therapy in recessive dystrophic epidermolysis bullosa. JCI Insight, 2019, 4, . | 5.0 | 56 |
| 67 | Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Results in an Expanded Cohort. Blood, 2019, 134, 3345-3345. | 1.4 | 12 |
| 68 | Northstar-2: Updated Safety and Efficacy Analysis of Lentiglobin Gene Therapy in Patients with Transfusion-Dependent Î²-Thalassemia and Non-Î²0/Î²0 Genotypes. Blood, 2019, 134, 3543-3543. | 1.4 | 13 |
| 69 | Clonal Dynamics of Early Responder and Long-Term Surviving CAR-T Cells in Humans. Blood, 2019, 134, 52-52. | 1.4 | 2 |
| 70 | Long-Term Hematopoietic Engraftment of Congenic Amniotic Fluid Stem Cells After in Utero Intraperitoneal Transplantation to Immune Competent Mice. Stem Cells and Development, 2018, 27, 515-523. | 2.1 | 10 |
| 71 | Autonomous role of Wiskott-Aldrich syndrome platelet deficiency in inducing autoimmunity and inflammation. Journal of Allergy and Clinical Immunology, 2018, 142, 1272-1284. | 2.9 | 28 |
| 72 | Prevalence and clinical challenges among adults with primary immunodeficiency and recombination-activating gene deficiency. Journal of Allergy and Clinical Immunology, 2018, 141, 2303-2306. | 2.9 | 40 |

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|---|---|---|---|
| 73 | Non-Clinical Efficacy and Safety Studies on G1XCGD, a Lentiviral Vector for Ex Vivo Gene Therapy of X-Linked Chronic Granulomatous Disease. Human Gene Therapy Clinical Development, 2018, 29, 69-79. | 3.1 | 31 |
| 74 | Leukocyte adhesion deficiency-I: A comprehensive review of all published cases. Journal of Allergy and Clinical Immunology: in Practice, 2018, 6, 1418-1420.e10. | 3.8 | 85 |
| 75 | Preclinical Development of a Lentiviral Vector for Gene Therapy of X-Linked Severe Combined Immunodeficiency. Molecular Therapy - Methods and Clinical Development, 2018, 9, 257-269. | 4.1 | 38 |
| 76 | T-cell gene therapy for perforin deficiency correctsÂcytotoxicity defects and prevents hemophagocytic lymphohistiocytosis manifestations. Journal of Allergy and Clinical Immunology, 2018, 142, 904-913.e3. | 2.9 | 44 |
| 77 | WASP-mediated regulation of anti-inflammatory macrophages is IL-10 dependent and is critical for intestinal homeostasis. Nature Communications, 2018, 9, 1779. | 12.8 | 40 |
| 78 | Loss-of-function nuclear factor Î¹B subunit 1 (NFKB1) variants are the most common monogenic cause of common variable immunodeficiency in Europeans. Journal of Allergy and Clinical Immunology, 2018, 142, 1285-1296. | 2.9 | 185 |
| 79 | Lancet Commission: Stem cells and regenerative medicine. Lancet, The, 2018, 391, 883-910. | 13.7 | 184 |
| 80 | One hundred percent survival after transplantation of 34 patients with Wiskott-Aldrich syndrome over 20Âyears. Journal of Allergy and Clinical Immunology, 2018, 142, 1654-1656.e7. | 2.9 | 39 |
| 81 | Molecular Evidence of Genome Editing in a Mouse Model of Immunodeficiency. Scientific Reports, 2018, 8, 8214. | 3.3 | 6 |
| 82 | De Novo Truncating Mutations in WASF1 Cause Intellectual Disability with Seizures. American Journal of Human Genetics, 2018, 103, 144-153. | 6.2 | 36 |
| 83 | Transfer of gene-corrected T cells corrects humoral and cytotoxic defects in patients with X-linked lymphoproliferative disease. Journal of Allergy and Clinical Immunology, 2018, 142, 235-245.e6. | 2.9 | 31 |
| 84 | Lentiglobin Gene Therapy for Patients with Transfusion-Dependent Î²-Thalassemia (TDT): Results from the Phase 3 Northstar-2 and Northstar-3 Studies. Blood, 2018, 132, 1025-1025. | 1.4 | 13 |
| 85 | Dendritic cell-expressed common gamma-chain recruits IL-15 for trans-presentation at the murine immunological synapse. Wellcome Open Research, 2018, 3, 84. | 1.8 | 7 |
| 86 | Dendritic cell-expressed common gamma-chain recruits IL-15 for trans-presentation at the murine immunological synapse. Wellcome Open Research, 2018, 3, 84. | 1.8 | 4 |
| 87 | A New Chapter on Targeted Gene Insertion for X-CGD: Do Not Skip the Intro(n). Molecular Therapy, 2017, 25, 307-309. | 8.2 | 3 |
| 88 | Limiting Thymic Precursor Supply Increases the Risk of Lymphoid Malignancy in Murine X-Linked Severe Combined Immunodeficiency. Molecular Therapy - Nucleic Acids, 2017, 6, 1-14. | 5.1 | 20 |
| 89 | Molecular remission of infant B-ALL after infusion of universal TALEN gene-edited CAR T cells. Science Translational Medicine, 2017, 9, . | 12.4 | 707 |
| 90 | Natural killer cells differentiated in vitro from cord blood CD34 + cells are more advantageous for use as an immunotherapy than peripheral blood and cord blood natural killer cells. Cytotherapy, 2017, 19, 710-720. | 0.7 | 10 |

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|---|---|---|---|
| 91 | Thymus transplantation for complete DiGeorge syndrome: European experience. Journal of Allergy and Clinical Immunology, 2017, 140, 1660-1670.e16. | 2.9 | 108 |
| 92 | Evolving Gene Therapy in Primary Immunodeficiency. Molecular Therapy, 2017, 25, 1132-1141. | 8.2 | 66 |
| 93 | Human Amniocytes Are Receptive to Chemically Induced Reprogramming to Pluripotency. Molecular Therapy, 2017, 25, 427-442. | 8.2 | 10 |
| 94 | Development of a pCCLChim Lentiviral Vector for Gene Therapy of Patients with Chronic Granulomatous Disease (CGD) due to p47-phox Deficiency. Journal of Allergy and Clinical Immunology, 2017, 139, AB186. | 2.9 | 0 |
| 95 | Lentiviral vectors can be used for full-length dystrophin gene therapy. Scientific Reports, 2017, 7, 79. | 3.3 | 41 |
| 96 | Lentiviral vectors can be used for full-length dystrophin gene therapy. Scientific Reports, 2017, 7, 44775. | 3.3 | 29 |
| 97 | Autoinflammatory periodic fever, immunodeficiency, and thrombocytopenia (PFIT) caused by mutation in actin-regulatory gene WDR1 . Journal of Experimental Medicine, 2017, 214, 59-71. | 8.5 | 117 |
| 98 | Targeted genome editing restores T cell differentiation in a humanized X-SCID pluripotent stem cell disease model. Scientific Reports, 2017, 7, 12475. | 3.3 | 9 |
| 99 | Hematopoietic Stem-Cell Gene Therapy for Cerebral Adrenoleukodystrophy. New England Journal of Medicine, 2017, 377, 1630-1638. | 27.0 | 412 |
| 100 | Phenotypic Characterization of EIF2AK4 Mutation Carriers in a Large Cohort of Patients Diagnosed Clinically With Pulmonary Arterial Hypertension. Circulation, 2017, 136, 2022-2033. | 1.6 | 111 |
| 101 | Characterization of a core region in the A2UCOE that confers effective anti-silencing activity. Scientific Reports, 2017, 7, 10213. | 3.3 | 9 |
| 102 | Gene therapy for Wiskott-Aldrich syndrome in a severely affected adult. Blood, 2017, 130, 1327-1335. | 1.4 | 83 |
| 103 | Wiskottâ€Aldrich syndrome protein: Emerging mechanisms in immunity. European Journal of Immunology, 2017, 47, 1857-1866. | 2.9 | 72 |
| 104 | Wiskott-Aldrich syndrome protein regulates autophagy and inflammasome activity in innate immune cells. Nature Communications, 2017, 8, 1576. | 12.8 | 50 |
| 105 | Primary immunodeficiencies due to abnormalities of the actin cytoskeleton. Current Opinion in Hematology, 2017, 24, 16-22. | 2.5 | 29 |
| 106 | Absence of Î³-Chain in Keratinocytes Alters Chemokine Secretion, Resulting in Reduced Immune Cell Recruitment. Journal of Investigative Dermatology, 2017, 137, 2120-2130. | 0.7 | 12 |
| 107 | A Personal Reflection from London. Human Gene Therapy, 2017, 28, 959-959. | 2.7 | 0 |
| 108 | Wiskottâ€“Aldrich Syndrome, Leukocyte Adhesion Deficiency, and Other Migration Defects in Human Primary Immunodeficiency. , 2016, , 416-425. | | 0 |

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|---|---|---|---|
| 109 | Automated manufacturing of chimeric antigen receptor T cells for adoptive immunotherapy using CliniMACS Prodigy. Cytotherapy, 2016, 18, 1002-1011. | 0.7 | 174 |
| 110 | Autologous skeletal muscle derived cells expressing a novel functional dystrophin provide a potential therapy for Duchenne Muscular Dystrophy. Scientific Reports, 2016, 6, 19750. | 3.3 | 29 |
| 111 | 250. A Phase 2/3 Study of the Efficacy and Safety of Ex Vivo Gene Therapy with Lenti-D TM Lentiviral Vector for the Treatment of Cerebral Adrenoleukodystrophy. Molecular Therapy, 2016, 24, S98-S99. | 8.2 | 2 |
| 112 | 690. Development of a Clinical Lentiviral Vector for Gene Therapy of SCID-X1. Molecular Therapy, 2016, 24, S273-S274. | 8.2 | 0 |
| 113 | Common variable immunodeficiency and natural killer cell lymphopenia caused by Ets-binding site mutation in the IL-2 receptor Î³ (IL2RG) gene promoter. Journal of Allergy and Clinical Immunology, 2016, 137, 940-942.e4. | 2.9 | 14 |
| 114 | Lentiviral Vector-Mediated Correction of a Mouse Model of Leukocyte Adhesion Deficiency Type I. Human Gene Therapy, 2016, 27, 668-678. | 2.7 | 21 |
| 115 | N-WASP is required for B-cellâ€“mediated autoimmunity in Wiskott-Aldrich syndrome. Blood, 2016, 127, 216-220. | 1.4 | 24 |
| 116 | Debate on Germline Gene Editing. Human Gene Therapy Methods, 2016, 27, 135-142. | 2.1 | 8 |
| 117 | Impact of BREXIT on UK Gene and Cell Therapy: The Need for Continued Pan-European Collaboration. Human Gene Therapy, 2016, 27, 653-655. | 2.7 | 3 |
| 118 | Deletion of Wiskottâ€“Aldrich syndrome protein triggers Rac2 activity and increased cross-presentation by dendritic cells. Nature Communications, 2016, 7, 12175. | 12.8 | 31 |
| 119 | Treating Immunodeficiency through HSC Gene Therapy. Trends in Molecular Medicine, 2016, 22, 317-327. | 6.7 | 96 |
| 120 | Hyperinflammation in patients with chronic granulomatous disease leads to impairment of hematopoietic stem cell functions. Journal of Allergy and Clinical Immunology, 2016, 138, 219-228.e9. | 2.9 | 74 |
| 121 | Lentiviral Engineered Fibroblasts Expressing Codon-Optimized COL7A1 Restore Anchoring Fibrils in RDEB. Journal of Investigative Dermatology, 2016, 136, 284-292. | 0.7 | 42 |
| 122 | WASp-dependent actin cytoskeleton stability at the dendritic cell immunological synapse is required for extensive, functional T cell contacts. Journal of Leukocyte Biology, 2016, 99, 699-710. | 3.3 | 54 |
| 123 | FOXP3+ Tregs require WASP to restrain Th2-mediated food allergy. Journal of Clinical Investigation, 2016, 126, 4030-4044. | 8.2 | 53 |
| 124 | New Molecular Surrogate Assay for Genotoxicity Assessment of Gene Therapy Vectors (SAGA). Blood, 2016, 128, 4710-4710. | 1.4 | 4 |
| 125 | a Diversity of Human Hematopoietic Differentiation Programs Identified through In Vivo Tracking of Hematopoiesis in Wiskott-Aldrich Syndrome Patients. Blood, 2016, 128, 3871-3871. | 1.4 | 0 |
| 126 | Preclinical Development of Gene Therapy for X-Linked Severe Combined Immunodeficiency (SCID-X1). Blood, 2016, 128, 4705-4705. | 1.4 | 1 |

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|---|---|---|---|
| 127 | Gene therapy: progress and predictions. Proceedings of the Royal Society B: Biological Sciences, 2015, 282, 20143003. | 2.6 | 108 |
| 128 | Sheep CD34+ Amniotic Fluid Cells Have Hematopoietic Potential and Engraft After Autologous In Utero Transplantation. Stem Cells, 2015, 33, 122-132. | 3.2 | 26 |
| 129 | Autologous Transplant/Gene Therapy for Adenosine Deaminase-Deficient Severe Combined Immune Deficiency. Biology of Blood and Marrow Transplantation, 2015, 21, S102. | 2.0 | 1 |
| 130 | Immunodeficiency and severe susceptibility to bacterial infection associated with a loss-of-function homozygous mutation of MKL1. Blood, 2015, 126, 1527-1535. | 1.4 | 66 |
| 131 | C-8. Immunological and Metabolic Correction After Lentiviral Vector Gene Therapy for ADA Deficiency. Molecular Therapy, 2015, 23, S102-S103. | 8.2 | 8 |
| 132 | 243. Pre-Clinical Development of Lentiviral Gene Therapy for X-Linked Severe Combined Immunodeficiency. Molecular Therapy, 2015, 23, S95. | 8.2 | 0 |
| 133 | 612. Site-Specific Gene Editing of COL7A1 Restores Type VII Collagen in RDEB iPSCs. Molecular Therapy, 2015, 23, S243. | 8.2 | 0 |
| 134 | Bâ€cell intrinsic TLR7 signals promote depletion of the marginal zone in a murine model of Wiskottâ€“Aldrich syndrome. European Journal of Immunology, 2015, 45, 2773-2779. | 2.9 | 19 |
| 135 | Gene therapy for monogenic disorders of the bone marrow. British Journal of Haematology, 2015, 171, 155-170. | 2.5 | 35 |
| 136 | Coherence analysis discriminates between retroviral integration patterns in CD34+ cells transduced under differing clinical trial conditions. Molecular Therapy - Methods and Clinical Development, 2015, 2, 15015. | 4.1 | 1 |
| 137 | Natural Killer Cells Improve Hematopoietic Stem Cell Engraftment by Increasing Stem Cell Clonogenicity In Vitro and in a Humanized Mouse Model. PLoS ONE, 2015, 10, e0138623. | 2.5 | 11 |
| 138 | Adoptive T-Cell Therapy for Cancer in the United Kingdom: A Review of Activity for the British Society of Gene and Cell Therapy Annual Meeting 2015. Human Gene Therapy, 2015, 26, 276-285. | 2.7 | 17 |
| 139 | Platelet actin nodules are podosome-like structures dependent on Wiskottâ€“Aldrich syndrome protein and ARP2/3 complex. Nature Communications, 2015, 6, 7254. | 12.8 | 86 |
| 140 | Patching up hematopoietic stem cells. Nature Biotechnology, 2015, 33, 1236-1238. | 17.5 | 2 |
| 141 | Immunotherapy of HCC metastases with autologous T cell receptor redirected T cells, targeting HBsAg in a liver transplant patient. Journal of Hepatology, 2015, 62, 486-491. | 3.7 | 160 |
| 142 | Construction of stable packaging cell lines for clinical lentiviral vector production. Scientific Reports, 2015, 5, 9021. | 3.3 | 74 |
| 143 | Site- and allele-specific polycomb dysregulation in T-cell leukaemia. Nature Communications, 2015, 6, 6094. | 12.8 | 47 |
| 144 | Current and emerging treatment options for Wiskottâ€“Aldrich syndrome. Expert Review of Clinical Immunology, 2015, 11, 1015-1032. | 3.0 | 59 |

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|---|---|---|---|
| 145 | Outcomes Following Gene Therapy in Patients With Severe Wiskott-Aldrich Syndrome. JAMA - Journal of the American Medical Association, 2015, 313, 1550. | 7.4 | 327 |
| 146 | Altered BCR and TLR signals promote enhanced positive selection of autoreactive transitional B cells in Wiskott-Aldrich syndrome. Journal of Experimental Medicine, 2015, 212, 1663-1677. | 8.5 | 67 |
| 147 | Editorial overview: New technologies. Current Opinion in Pharmacology, 2015, 24, vii-viii. | 3.5 | 0 |
| 148 | Perforin Gene Transfer Into Hematopoietic Stem Cells Improves Immune Dysregulation in Murine Models of Perforin Deficiency. Molecular Therapy, 2015, 23, 737-745. | 8.2 | 41 |
| 149 | Lentivirus technologies for modulation of the immune system. Current Opinion in Pharmacology, 2015, 24, 119-127. | 3.5 | 11 |
| 150 | Automated Lentiviral Transduction of T Cells with Cars Using the Clinimacs Prodigy. Blood, 2015, 126, 2043-2043. | 1.4 | 3 |
| 151 | First Clinical Application of Talen Engineered Universal CAR19 T Cells in B-ALL. Blood, 2015, 126, 2046-2046. | 1.4 | 75 |
| 152 | Frozen Cord Blood Hematopoietic Stem Cells Differentiate into Higher Numbers of Functional Natural Killer Cells In Vitro than Mobilized Hematopoietic Stem Cells or Freshly Isolated Cord Blood Hematopoietic Stem Cells. PLoS ONE, 2014, 9, e87086. | 2.5 | 28 |
| 153 | Exacerbated experimental arthritis in Wiskottâ€“Aldrich syndrome protein deficiency: Modulatory role of regulatory B cells. European Journal of Immunology, 2014, 44, 2692-2702. | 2.9 | 22 |
| 154 | â€œDarwinianâ€•Tumor-Suppression Model Unsupported in Clinical Experience. Molecular Therapy, 2014, 22, 1562-1563. | 8.2 | 6 |
| 155 | Preclinical Demonstration of Lentiviral Vector-mediated Correction of Immunological and Metabolic Abnormalities in Models of Adenosine Deaminase Deficiency. Molecular Therapy, 2014, 22, 607-622. | 8.2 | 77 |
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