## Ciaran M Lee

## List of Publications by Citations

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#	Paper	IF	Citations
57	A high-fidelity Cas9 mutant delivered as a ribonucleoprotein complex enables efficient gene editing in human hematopoietic stem and progenitor cells. <i>Nature Medicine</i> , <b>2018</b> , 24, 1216-1224	50.5	344
56	CRISPR/Cas9-Based Genome Editing for Disease Modeling and Therapy: Challenges and Opportunities for Nonviral Delivery. <i>Chemical Reviews</i> , <b>2017</b> , 117, 9874-9906	68.1	287
55	COSMID: A Web-based Tool for Identifying and Validating CRISPR/Cas Off-target Sites. <i>Molecular Therapy - Nucleic Acids</i> , <b>2014</b> , 3, e214	10.7	219
54	CD7-edited T cells expressing a CD7-specific CAR for the therapy of T-cell malignancies. <i>Blood</i> , <b>2017</b> , 130, 285-296	2.2	183
53	The Neisseria meningitidis CRISPR-Cas9 System Enables Specific Genome Editing in Mammalian Cells. <i>Molecular Therapy</i> , <b>2016</b> , 24, 645-54	11.7	150
52	Streptococcus thermophilus CRISPR-Cas9 Systems Enable Specific Editing of the Human Genome. <i>Molecular Therapy</i> , <b>2016</b> , 24, 636-44	11.7	148
51	Nuclease Target Site Selection for Maximizing On-target Activity and Minimizing Off-target Effects in Genome Editing. <i>Molecular Therapy</i> , <b>2016</b> , 24, 475-87	11.7	87
50	Gene correction for SCID-X1 in long-term hematopoietic stem cells. <i>Nature Communications</i> , <b>2019</b> , 10, 1634	17.4	77
49	Engineered materials for in vivo delivery of genome-editing machinery. <i>Nature Reviews Materials</i> , <b>2019</b> , 4, 726-737	73.3	73
48	Efficient CRISPR/Cas9-Mediated Genome Editing Using a Chimeric Single-Guide RNA Molecule. <i>Frontiers in Plant Science</i> , <b>2017</b> , 8, 1441	6.2	72
47	AAV-CRISPR Gene Editing Is Negated by Pre-existing Immunity to Cas9. <i>Molecular Therapy</i> , <b>2020</b> , 28, 1432-1441	11.7	72
46	Highly efficient editing of the Iglobin gene in patient-derived hematopoietic stem and progenitor cells to treat sickle cell disease. <i>Nucleic Acids Research</i> , <b>2019</b> , 47, 7955-7972	20.1	67
45	Spatial control of in vivo CRISPR-Cas9 genome editing via nanomagnets. <i>Nature Biomedical Engineering</i> , <b>2019</b> , 3, 126-136	19	65
44	Correction of the £508 Mutation in the Cystic Fibrosis Transmembrane Conductance Regulator Gene by Zinc-Finger Nuclease Homology-Directed Repair. <i>BioResearch Open Access</i> , <b>2012</b> , 1, 99-108	2.4	64
43	A Self-Deleting AAV-CRISPR System for Genome Editing. <i>Molecular Therapy - Methods and Clinical Development</i> , <b>2019</b> , 12, 111-122	6.4	62
42	A Burden of Rare Variants Associated with Extremes of Gene Expression in Human Peripheral Blood. <i>American Journal of Human Genetics</i> , <b>2016</b> , 98, 299-309	11	61
41	Therapeutically relevant engraftment of a CRISPR-Cas9-edited HSC-enriched population with HbF reactivation in nonhuman primates. <i>Science Translational Medicine</i> , <b>2019</b> , 11,	17.5	59

## (2021-2019)

40	Optimization of CRISPR/Cas9 Delivery to Human Hematopoietic Stem and Progenitor Cells for Therapeutic Genomic Rearrangements. <i>Molecular Therapy</i> , <b>2019</b> , 27, 137-150	11.7	58
39	Somatic genome editing with CRISPR/Cas9 generates and corrects a metabolic disease. <i>Scientific Reports</i> , <b>2017</b> , 7, 44624	4.9	54
38	High-Efficiency, Selection-free Gene Repair in Airway Stem Cells from Cystic Fibrosis Patients Rescues CFTR Function in Differentiated Epithelia. <i>Cell Stem Cell</i> , <b>2020</b> , 26, 161-171.e4	18	50
37	Human genome-edited hematopoietic stem cells phenotypically correct Mucopolysaccharidosis type I. <i>Nature Communications</i> , <b>2019</b> , 10, 4045	17.4	44
36	Efficient fdCas9 Synthetic Endonuclease with Improved Specificity for Precise Genome Engineering. <i>PLoS ONE</i> , <b>2015</b> , 10, e0133373	3.7	42
35	Somatic Editing of Ldlr With Adeno-Associated Viral-CRISPR Is an Efficient Tool for Atherosclerosis Research. <i>Arteriosclerosis, Thrombosis, and Vascular Biology</i> , <b>2018</b> , 38, 1997-2006	9.4	41
34	CRISPR-based gene editing enables gene repair in IPEX patient cells. Science Advances, 2020, 6, eaaz057	114.3	38
33	Genome editing for inborn errors of metabolism: advancing towards the clinic. <i>BMC Medicine</i> , <b>2017</b> , 15, 43	11.4	30
32	In Vivo Ryr2 Editing Corrects Catecholaminergic Polymorphic Ventricular Tachycardia. <i>Circulation Research</i> , <b>2018</b> , 123, 953-963	15.7	29
31	Analysis of gene repair tracts from Cas9/gRNA double-stranded breaks in the human CFTR gene. <i>Scientific Reports</i> , <b>2016</b> , 6, 32230	4.9	21
30	Collagen-rich airway smooth muscle cells are a metastatic niche for tumor colonization in the lung. <i>Nature Communications</i> , <b>2019</b> , 10, 2131	17.4	20
29	Genome editing of donor-derived T-cells to generate allogenic chimeric antigen receptor-modified T cells: Optimizing IT cell-depleted haploidentical hematopoietic stem cell transplantation. <i>Haematologica</i> , <b>2021</b> , 106, 847-858	6.6	18
28	Examination of CRISPR/Cas9 design tools and the effect of target site accessibility on Cas9 activity. Experimental Physiology, <b>2018</b> , 103, 456-460	2.4	14
27	Controlled delivery of Eglobin-targeting TALENs and CRISPR/Cas9 into mammalian cells for genome editing using microinjection. <i>Scientific Reports</i> , <b>2015</b> , 5, 16031	4.9	14
26	Treating hemoglobinopathies using gene-correction approaches: promises and challenges. <i>Human Genetics</i> , <b>2016</b> , 135, 993-1010	6.3	12
25	Metabolic engineering generates a transgene-free safety switch for cell therapy. <i>Nature Biotechnology</i> , <b>2020</b> , 38, 1441-1450	44.5	12
24	Development of Eglobin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease. <i>Science Translational Medicine</i> , <b>2021</b> , 13,	17.5	12
23	Tools for experimental and computational analyses of off-target editing by programmable nucleases. <i>Nature Protocols</i> , <b>2021</b> , 16, 10-26	18.8	12

22	Therapeutic Crispr/Cas9 Genome Editing for Treating Sickle Cell Disease. <i>Blood</i> , <b>2016</b> , 128, 4703-4703	2.2	7
21	Targeted replacement of full-length CFTR in human airway stem cells by CRISPR-Cas9 for pan-mutation correction in the endogenous locus. <i>Molecular Therapy</i> , <b>2021</b> ,	11.7	7
20	The TRACE-Seq method tracks recombination alleles and identifies clonal reconstitution dynamics of gene targeted human hematopoietic stem cells. <i>Nature Communications</i> , <b>2021</b> , 12, 472	17.4	7
19	Fine-mapping within eQTL credible intervals by expression CROP-seq. <i>Biology Methods and Protocols</i> , <b>2020</b> , 5, bpaa008	2.4	6
18	TNF-Bynergises with IFN-Ito induce caspase-8-JAK1/2-STAT1-dependent death of intestinal epithelial cells. <i>Cell Death and Disease</i> , <b>2021</b> , 12, 864	9.8	6
17	Design and Validation of CRISPR/Cas9 Systems for Targeted Gene Modification in Induced Pluripotent Stem Cells. <i>Methods in Molecular Biology</i> , <b>2017</b> , 1498, 3-21	1.4	5
16	331. Development of Neisseria meningitidis CRISPR/Cas9 Systems for Efficient and Specific Genome Editing. <i>Molecular Therapy</i> , <b>2015</b> , 23, S132-S133	11.7	4
15	Highly Efficient Repair of the <b>E</b> 508 Mutation in Airway Stem Cells of Cystic Fibrosis Patients with Functional Rescue of the Differentiated Epithelia		3
14	Pitfalls in Single Clone CRISPR-Cas9 Mutagenesis to Fine-map Regulatory Intervals. <i>Genes</i> , <b>2020</b> , 11,	4.2	2
13	Site-Specific Post-translational Surface Modification of Adeno-Associated Virus Vectors Using Leucine Zippers. <i>ACS Synthetic Biology</i> , <b>2020</b> , 9, 461-467	5.7	2
12	Gene Editing with Crispr-Cas9 for Treating Beta-Hemoglobinopathies. <i>Blood</i> , <b>2015</b> , 126, 3376-3376	2.2	2
11	Re-Creating Hereditary Persistence of Fetal Hemoglobin (HPFH) to Treat Sickle Cell Disease (SCD) and EThalassemia. <i>Blood</i> , <b>2016</b> , 128, 4708-4708	2.2	2
10	Targeted replacement of full-length CFTR in human airway stem cells by CRISPR/Cas9 for pan-mutation correction in the endogenous locus		2
9	TRACE-Seq Reveals Clonal Reconstitution Dynamics of Gene Targeted Human Hematopoietic Stem Cell	s	1
8	Gene Correction for SCID-X1 in Long-Term Hematopoietic Stem Cells		1
7	genome editing at the albumin locus to treat methylmalonic acidemia <i>Molecular Therapy - Methods and Clinical Development</i> , <b>2021</b> , 23, 619-632	6.4	O
6	Engineered Human Umbilical Cord Derived Erythroid Progenitor Cells (HUDEP2) with Sickle or EThalassemia Mutation: An in-Vitro System for Testing Pharmacological Induction of Fetal Hemoglobin. <i>Blood</i> , <b>2018</b> , 132, 3478-3478	2.2	0
5	Highly Efficient Editing of the Beta-Globin Gene in Patient Derived Hematopoietic Stem and Progenitor Cells to Treat Sickle Cell Disease. <i>Blood</i> , <b>2018</b> , 132, 2192-2192	2.2	Ο

## LIST OF PUBLICATIONS

4	Sickle Human Umbilical Cord Derived Erythroid Progenitor Cells (S-HUDEP2): An Ideal in-Vitro System for Screening Anti-Sickling Compounds for Sickle Cell Disease. <i>Blood</i> , <b>2018</b> , 132, 3675-3675	2.2
3	Persistence of CRISPR/Cas9-Edited Hematopoietic Stem and Progenitor Cells and Reactivation of Fetal Hemoglobin in Nonhuman Primates. <i>Blood</i> , <b>2018</b> , 132, 806-806	2.2
2	Modulation of inhibitory signals in CAR T cells leads to improved activity against glioblastoma <i>Journal of Clinical Oncology</i> , <b>2020</b> , 38, 3031-3031	2.2
1	Identification and Validation of CRISPR/Cas9 Off-Target Activity in Hematopoietic Stem and Progenitor Cells <i>Methods in Molecular Biology</i> , <b>2022</b> , 2429, 281-306	1.4