## Ciaran M Lee

## List of Publications by Year in descending order

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|          |                | 159585       | 161849         |
|----------|----------------|--------------|----------------|
| 56       | 4,436          | 30           | 54             |
| papers   | citations      | h-index      | g-index        |
|          |                |              |                |
|          |                |              |                |
| 63       | 63             | 63           | 6028           |
| all docs | docs citations | times ranked | citing authors |
|          |                |              |                |

| #  | Article   | IF   | CITATIONS |
|----|---|------|-----------|
| 1  | Targeted replacement of full-length CFTR in human airway stem cells by CRISPR-Cas9 for pan-mutation correction in the endogenous locus. Molecular Therapy, 2022, 30, 223-237.   | 8.2  | 24        |
| 2  | High-Throughput Imaging of CRISPR- and Recombinant Adeno-Associated Virus–Induced DNA Damage<br>Response in Human Hematopoietic Stem and Progenitor Cells. CRISPR Journal, 2022, 5, 80-94.  | 2.9  | 16        |
| 3  | Identification and Validation of CRISPR/Cas9 Off-Target Activity in Hematopoietic Stem and Progenitor Cells. Methods in Molecular Biology, 2022, 2429, 281-306.   | 0.9  | 1         |
| 4  | Genome editing of donor-derived T-cells to generate allogenic chimeric antigen receptor-modified T cells: Optimizing $\hat{l}\pm\hat{l}^2$ T cell-depleted haploidentical hematopoietic stem cell transplantation. Haematologica, 2021, 106, 847-858. | 3.5  | 46        |
| 5  | Tools for experimental and computational analyses of off-target editing by programmable nucleases.<br>Nature Protocols, 2021, 16, 10-26.  | 12.0 | 52        |
| 6  | Development of $\hat{l}^2$ -globin gene correction in human hematopoietic stem cells as a potential durable treatment for sickle cell disease. Science Translational Medicine, 2021, 13, .  | 12.4 | 82        |
| 7  | TNF-Î $\pm$ synergises with IFN-Î $^3$ to induce caspase-8-JAK1/2-STAT1-dependent death of intestinal epithelial cells. Cell Death and Disease, 2021, 12, 864.  | 6.3  | 54        |
| 8  | The TRACE-Seq method tracks recombination alleles and identifies clonal reconstitution dynamics of gene targeted human hematopoietic stem cells. Nature Communications, 2021, 12, 472.  | 12.8 | 23        |
| 9  | InÂvivo genome editing at the albumin locus to treat methylmalonic acidemia. Molecular Therapy -<br>Methods and Clinical Development, 2021, 23, 619-632.  | 4.1  | 10        |
| 10 | High-Efficiency, Selection-free Gene Repair in Airway Stem Cells from Cystic Fibrosis Patients Rescues CFTR Function in Differentiated Epithelia. Cell Stem Cell, 2020, 26, 161-171.e4.   | 11.1 | 97        |
| 11 | Metabolic engineering generates a transgene-free safety switch for cell therapy. Nature<br>Biotechnology, 2020, 38, 1441-1450.  | 17.5 | 39        |
| 12 | CRISPR-based gene editing enables <i>FOXP3</i> gene repair in IPEX patient cells. Science Advances, 2020, 6, eaaz0571.  | 10.3 | 84        |
| 13 | Fine-mapping within eQTL credible intervals by expression CROP-seq. Biology Methods and Protocols, 2020, 5, bpaa008.  | 2.2  | 8         |
| 14 | Pitfalls in Single Clone CRISPR-Cas9 Mutagenesis to Fine-Map Regulatory Intervals. Genes, 2020, 11, 504.  | 2.4  | 6         |
| 15 | Site-Specific Post-translational Surface Modification of Adeno-Associated Virus Vectors Using Leucine Zippers. ACS Synthetic Biology, 2020, 9, 461-467.   | 3.8  | 6         |
| 16 | AAV-CRISPR Gene Editing Is Negated by Pre-existing Immunity to Cas9. Molecular Therapy, 2020, 28, 1432-1441.  | 8.2  | 140       |
| 17 | Modulation of inhibitory signals in CAR T cells leads to improved activity against glioblastoma<br>Journal of Clinical Oncology, 2020, 38, 3031-3031.   | 1.6  | O         |
| 18 | Therapeutically relevant engraftment of a CRISPR-Cas9–edited HSC-enriched population with HbF reactivation in nonhuman primates. Science Translational Medicine, 2019, 11, .  | 12.4 | 88        |

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|----|---|------|-----------|
| 19 | Human genome-edited hematopoietic stem cells phenotypically correct Mucopolysaccharidosis type I. Nature Communications, 2019, 10, 4045.  | 12.8 | 88        |
| 20 | Engineered materials for in vivo delivery of genome-editing machinery. Nature Reviews Materials, 2019, 4, 726-737.  | 48.7 | 139       |
| 21 | Highly efficient editing of theÂβ-globin gene in patient-derived hematopoietic stem and progenitor cells to treat sickle cell disease. Nucleic Acids Research, 2019, 47, 7955-7972.   | 14.5 | 110       |
| 22 | Collagen-rich airway smooth muscle cells are a metastatic niche for tumor colonization in the lung. Nature Communications, 2019, 10, 2131.  | 12.8 | 27        |
| 23 | Gene correction for SCID-X1 in long-term hematopoietic stem cells. Nature Communications, 2019, 10, 1634.   | 12.8 | 140       |
| 24 | Spatial control of in vivo CRISPR–Cas9 genome editing via nanomagnets. Nature Biomedical Engineering, 2019, 3, 126-136.   | 22.5 | 107       |
| 25 | Optimization of CRISPR/Cas9 Delivery to Human Hematopoietic Stem and Progenitor Cells for Therapeutic Genomic Rearrangements. Molecular Therapy, 2019, 27, 137-150.   | 8.2  | 97        |
| 26 | A Self-Deleting AAV-CRISPR System for InÂVivo Genome Editing. Molecular Therapy - Methods and Clinical Development, 2019, 12, 111-122.  | 4.1  | 93        |
| 27 | Examination of CRISPR/Cas9 design tools and the effect of target site accessibility on Cas9 activity. Experimental Physiology, 2018, 103, 456-460.  | 2.0  | 20        |
| 28 | In Vivo <i>Ryr</i> 2 Editing Corrects Catecholaminergic Polymorphic Ventricular Tachycardia. Circulation Research, 2018, 123, 953-963.  | 4.5  | 63        |
| 29 | Somatic Editing of <i>Ldlr</i> With Adeno-Associated Viral-CRISPR Is an Efficient Tool for Atherosclerosis Research. Arteriosclerosis, Thrombosis, and Vascular Biology, 2018, 38, 1997-2006.                                 | 2.4  | 63        |
| 30 | A high-fidelity Cas9 mutant delivered as a ribonucleoprotein complex enables efficient gene editing in human hematopoietic stem and progenitor cells. Nature Medicine, 2018, 24, 1216-1224.                                   | 30.7 | 573       |
| 31 | Abstract 032: Somatic Editing of Ldlr with AAV-CRISPR for Atherosclerosis Studies. Arteriosclerosis, Thrombosis, and Vascular Biology, 2018, 38, .  | 2.4  | 0         |
| 32 | Sickle Human Umbilical Cord Derived Erythroid Progenitor Cells (S-HUDEP2): An Ideal in-Vitro System for Screening Anti-Sickling Compounds for Sickle Cell Disease. Blood, 2018, 132, 3675-3675.                               | 1.4  | 0         |
| 33 | Engineered Human Umbilical Cord Derived Erythroid Progenitor Cells (HUDEP2) with Sickle or β-Thalassemia Mutation: An in-Vitro System for Testing Pharmacological Induction of Fetal Hemoglobin. Blood, 2018, 132, 3478-3478. | 1.4  | 1         |
| 34 | Persistence of CRISPR/Cas9-Edited Hematopoietic Stem and Progenitor Cells and Reactivation of Fetal Hemoglobin in Nonhuman Primates. Blood, 2018, 132, 806-806.   | 1.4  | 0         |
| 35 | Highly Efficient Editing of the Beta-Globin Gene in Patient Derived Hematopoietic Stem and Progenitor Cells to Treat Sickle Cell Disease. Blood, 2018, 132, 2192-2192.  | 1.4  | 1         |
| 36 | Genome editing for inborn errors of metabolism: advancing towards the clinic. BMC Medicine, 2017, 15, 43.   | 5.5  | 42        |

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|----|---|------|-----------|
| 37 | CRISPR/Cas9-Based Genome Editing for Disease Modeling and Therapy: Challenges and Opportunities for Nonviral Delivery. Chemical Reviews, 2017, 117, 9874-9906.                | 47.7 | 418       |
| 38 | CD7-edited T cells expressing a CD7-specific CAR for the therapy of T-cell malignancies. Blood, 2017, 130, 285-296.   | 1.4  | 326       |
| 39 | Somatic genome editing with CRISPR/Cas9 generates and corrects a metabolic disease. Scientific Reports, 2017, 7, 44624.   | 3.3  | 76        |
| 40 | Design and Validation of CRISPR/Cas9 Systems for Targeted Gene Modification in Induced Pluripotent Stem Cells. Methods in Molecular Biology, 2017, 1498, 3-21.                | 0.9  | 10        |
| 41 | Efficient CRISPR/Cas9-Mediated Genome Editing Using a Chimeric Single-Guide RNA Molecule. Frontiers in Plant Science, 2017, 8, 1441.  | 3.6  | 107       |
| 42 | 131. Chromatin-Dependent Loci Accessibility Affects CRISPR-Cas9 Targeting Efficiency. Molecular Therapy, 2016, 24, S54.   | 8.2  | 1         |
| 43 | Analysis of gene repair tracts from Cas9/gRNA double-stranded breaks in the human CFTR gene. Scientific Reports, 2016, 6, 32230.  | 3.3  | 26        |
| 44 | Treating hemoglobinopathies using gene-correction approaches: promises and challenges. Human Genetics, 2016, 135, 993-1010.   | 3.8  | 13        |
| 45 | The Neisseria meningitidis CRISPR-Cas9 System Enables Specific Genome Editing in Mammalian Cells.<br>Molecular Therapy, 2016, 24, 645-654.                                    | 8.2  | 190       |
| 46 | Streptococcus thermophilus CRISPR-Cas9 Systems Enable Specific Editing of the Human Genome. Molecular Therapy, 2016, 24, 636-644.   | 8.2  | 204       |
| 47 | A Burden of Rare Variants Associated with Extremes of Gene Expression in Human Peripheral Blood.<br>American Journal of Human Genetics, 2016, 98, 299-309.                    | 6.2  | 84        |
| 48 | Nuclease Target Site Selection for Maximizing On-target Activity and Minimizing Off-target Effects in Genome Editing. Molecular Therapy, 2016, 24, 475-487.                   | 8.2  | 100       |
| 49 | Therapeutic Crispr/Cas9 Genome Editing for Treating Sickle Cell Disease. Blood, 2016, 128, 4703-4703.   | 1.4  | 13        |
| 50 | Re-Creating Hereditary Persistence of Fetal Hemoglobin (HPFH) to Treat Sickle Cell Disease (SCD) and $\hat{l}^2$ -Thalassemia. Blood, 2016, 128, 4708-4708.                   | 1.4  | 2         |
| 51 | Controlled delivery of $\hat{l}^2$ -globin-targeting TALENs and CRISPR/Cas9 into mammalian cells for genome editing using microinjection. Scientific Reports, 2015, 5, 16031. | 3.3  | 20        |
| 52 | 331. Development of Neisseria meningitidis CRISPR/Cas9 Systems for Efficient and Specific Genome Editing. Molecular Therapy, 2015, 23, S132-S133.                             | 8.2  | 4         |
| 53 | Gene Editing with Crispr-Cas9 for Treating Beta-Hemoglobinopathies. Blood, 2015, 126, 3376-3376.  | 1.4  | 4         |
| 54 | Efficient fdCas9 Synthetic Endonuclease with Improved Specificity for Precise Genome Engineering. PLoS ONE, 2015, 10, e0133373.   | 2.5  | 46        |

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|----|---|-----|-----------|
| 55 | COSMID: A Web-based Tool for Identifying and Validating CRISPR/Cas Off-target Sites. Molecular Therapy - Nucleic Acids, 2014, 3, e214.  | 5.1 | 315       |
| 56 | Correction of the î"F508 Mutation in the Cystic Fibrosis Transmembrane Conductance Regulator Gene by Zinc-Finger Nuclease Homology-Directed Repair. BioResearch Open Access, 2012, 1, 99-108. | 2.6 | 74        |