

NEPA21 Publication List for Genome Editing Applications

Cell Culture Electroporation

(Organoids) *Gastroenterology*. 2018 Jul; 155(1):130-143.e15. van Rijn JM et al.

Intestinal Failure and Aberrant Lipid Metabolism in Patients With DGAT1 Deficiency.

(hiPSCs) *Stem Cell Res*. 2018 Apr; 28: 100-104. Y Tanaka et al.

Generation of D1-1 TALEN isogenic control cell line from Dravet syndrome patient iPSCs using TALEN-mediated editing of the SCN1A gene

(iPSCs) *Nat Commun*. 2018 Mar 5; 9(1):939. S Kim et al.

Microhomology-assisted scarless genome editing in human iPSCs

(HeLa) *Nat Commun*. 2018 Feb 6; 9(1):524 H Tsuchiya et al.

Ub-ProT reveals global length and composition of protein ubiquitylation in cells

(Primary mouse neurons) *J Biol Chem*. 2018 Feb 9; 293(6):2137-2148. Huang B et al.

OTX1 regulates cell cycle progression of neural progenitors in the developing cerebral cortex.

(MEF) *Cell Death Differ*. 2018 Feb; 25(2):444-452. V Abramowski et al.

PAXX and Xlf interplay revealed by impaired CNS development and immunodeficiency of double KO mice

(Bovine fibroblasts and goat fibroblasts) *Journal of Integrative Agriculture, Volume 17, Issue 2, February 2018* H LIU et al.

Comparing successful gene knock-in efficiencies of CRISPR/Cas9 with ZFNs and TALENs gene editing systems in bovine and dairy goat fetal fibroblasts

(iPSCs) *J Allergy Clin Immunol*. 2018 Jan; 141(1):339-349 S Takada et al.

Pluripotent stem cell models of Blau syndrome reveal an IFN- γ -dependent inflammatory response in macrophages

(CD4+ U87) *Mol Med Rep*. 2018 Jan; 17(1):243-249. Yu et al.

TALENs-mediated homozygous CCR5 Δ 32 mutations endow CD4+ U87 cells with resistance against HIV-1 infection

(Organoids) *Cell*. 2018 Jan 11; 172(1-2):373-386. Sachs N et al.

A Living Biobank of Breast Cancer Organoids Captures Disease Heterogeneity.

(iPSCs) *Sci Rep*. 2018 Jan 10; 8(1):310. Ishida K et al.

Site-specific randomization of the endogenous genome by a regulatable CRISPR-Cas9 piggyBac system in human cells.

(Bovine Fetal Fibroblast cells and HEK293T) *Sci Rep.* 2017 Dec 19;7(1):17827. Ikeda M et al.

Correction of a Disease Mutation using CRISPR/Cas9-assisted Genome Editing in Japanese Black Cattle.

(Primary human skin fibroblasts) *Oncotarget.* 2017 Dec 17;9(3):3779-3793. Lesport E et al.

Reduced recruitment of 53BP1 during interstrand crosslink repair is associated with genetically inherited attenuation of mitomycin C sensitivity in a family with Fanconi anemia.

(U251) *Biosci Rep.* 2017 Nov 6;37(6). pii: BSR20160523. Fu R et al.

TET1 exerts its tumor suppressor function by regulating autophagy in glioma cells

(iPSCs) *HepatoL Commun.* 2017 Nov;1(9):886-898. L Omer et al.

CRISPR correction of a homozygous low - density lipoprotein receptor mutation in familial hypercholesterolemia induced pluripotent stem cells

(iPSCs) University of Leicester <http://hdl.handle.net/2381/40397> 22 Sep 2017 NF Shankhi

Generation of Knockout Human iPSCs to Investigate Genes Associated with Telomere Length

(Organoids) University of Applied Sciences Technikum Wien

https://static1.squarespace.com/static/559921a3e4b02c1d7480f8f4/t/5a4613828165f559033ac2c6/1514541962900/Weidinger+Pia_747.PDF 1 Sep 2017 Pia Weidinger

Modeling lung tumorigenesis using CRISPR/Cas9-based genome editing in ex vivo 3D organoids

(hAMSCs) *Mol Ther Nucleic Acids.* 2017 Sep 15;8:395-403. Meca-Cortés et al.

CRISPR/Cas9-Mediated Knockin Application in Cell Therapy: A Non-viral Procedure for Bystander Treatment of Glioma in Mice

(iPSCs) *Regenerative Therapy Volume 6, June 2017, Pages 15-20* Horie H et al.

Impairment of the transition from proliferative stage to prehypertrophic stage in chondrogenic differentiation of human induced pluripotent stem cells harboring the causative mutation of achondroplasia in fibroblast growth factor receptor

(CMK11-5 & F-36P-MPL cells) *Leukemia.* 2017 May;31(5):1136-1144. K Shide et al.

Calreticulin mutant mice develop essential thrombocythemia that is ameliorated by the JAK inhibitor ruxolitinib

(iPSCs) *The FASEB Journal Vol. 31, 909.2, 1 Apr 2017* L Omer et al.

CRISPR/Cas9 Genome Editing to Repair Receptor-Mediated Endocytosis in Homozygous Familial Hypercholesterolemia Induced Pluripotent Stem Cells

(iPSCs) *Mol Brain*. 2017 Feb 15;10(1):7. N Murakami et al.

Proteasome impairment in neural cells derived from HMSN-P patient iPSCs

(HEK293) *Sci Rep*. 2017 Feb 14;7:42476 Hirose M et al.

CRISPR/Cas9-mediated genome editing in wild-derived mice: generation of tamed wild-derived strains by mutation of the α (nonagouti) gene

(Spermatogonial stem cells) *Cell Res*. 2017 Feb;27(2):241-252. CH Li et al.

Long-term propagation of tree shrew spermatogonial stem cells in culture and successful generation of transgenic offspring

(iPSCs) *Cell Rep*. 2017 Jan 3;18(1):68-81. Hosoya M et al.

Cochlear Cell Modeling Using Disease-Specific iPSCs Unveils a Degenerative Phenotype and Suggests Treatments for Congenital Progressive Hearing Loss

(iPSCs) *Hum Mol Genet*. 2016 Dec 1;25(23):5188-5197. T Ishikawa et al.

Genetic and pharmacological correction of aberrant dopamine synthesis using patient iPSCs with BH4 metabolism disorders

(iPSCs) *Sci Rep*. 2016 Oct 10;6:34904. K Imamura et al.

Calcium dysregulation contributes to neurodegeneration in FTLD patient iPSC-derived neurons

(PLB985 cells, WR19L cells) *Proc Natl Acad Sci U S A*. 2016 Aug 23;113(34):9509-14. J Suzuki et al.

Xkr8 phospholipid scrambling complex in apoptotic phosphatidylserine exposure.

(HepG2) *Exp Cell Res*. 2016 Jul 15;345(2):158-67. Rong Guo X et al.

ANGPTL8/betatrophin alleviates insulin resistance via the Akt-GSK3 β or Akt-FoxO1 pathway in HepG2 cells.

(iPSCs) *J Am Soc Nephrol*. 2016 Jun;27(6):1778-91. Sharmin S et al.

Human Induced Pluripotent Stem Cell-Derived Podocytes Mature into Vascularized Glomeruli upon Experimental Transplantation.

(iPSCs) *Methods*. 2016 May 15;101:27-35 Li, HL et al.

Efficient genomic correction methods in human iPS cell using CRISPR–Cas9 system

(Primary porcine dermal fibroblasts, kidney fibroblasts and myoblasts) *Mol Reprod Dev*. 2016 Jan;83(1):61-70. Rao S et al.

Efficient modification of the myostatin gene in porcine somatic cells and generation of knockout piglets.

(iPSCs) *Methods Mol Biol*. 2016;1357:111-31. Ocegüera-Yanez F et al.

Inducible Transgene Expression in Human iPS Cells Using Versatile All-in-One piggyBac Transposons.

(Ba/F3) *Mol Cell Biol.* 2015 Dec 14;36(4):645-59. S Gyobu et al.

A Role of TMEM16E Carrying a Scrambling Domain in Sperm Motility.

(Organoids) *Nat Protoc.* 2015 Oct;10(10):1474-85 Fujii M et al.

Efficient genetic engineering of human intestinal organoids using electroporation.

(iPSCs) *Exp Hematol.* 2015 Oct;43(10):849-57. Iizuka H et al.

Targeted gene correction of RUNX1 in induced pluripotent stem cells derived from familial platelet disorder with propensity to myeloid malignancy restores normal megakaryopoiesis.

(HEK293) *Exp Anim.* 2015;64(1):31-7 Honda et al.

Single-step generation of rabbits carrying a targeted allele of the tyrosinase gene using CRISPR/Cas9

(Organoids) *Nat Med.* 2015 Mar;21(3):256-62. Matano M et al.

Dissociated intestinal organoids 2015 Modeling colorectal cancer using CRISPR-Cas9-mediated engineering of human intestinal organoids.

(iPSCs) *Stem Cell Reports.* 2015 Jan 13;4(1):143-54. Li, HL et al.

Precise Correction of the Dystrophin Gene in Duchenne Muscular Dystrophy Patient Induced Pluripotent Stem Cells by TALEN and CRISPR-Cas9

(HeLa) *Oncotarget.* 2014 Sep 30;5(18):8393-401. Ding Y et al.

TALEN-mediated Nanog disruption results in less invasiveness, more chemosensitivity and reversal of EMT in Hela cells

(Jurkat) *Sci Rep.* 2014 May 23;4:5043 Matsubara Y et al.

Transcription activator-like effector nuclease-mediated transduction of exogenous gene into IL2RG locus

Zygote Electroporation

BMC Biotechnol. 2018 Apr 2;18(1):19. Matsuyama M et al.

Successful production of genome-edited rats by the rGONAD method

Sci Rep. 2018 Jan 11;8(1):474. Marie Teixeira et al.

Electroporation of mice zygotes with dual guide RNA/Cas9 complexes for simple and efficient cloning-free genome editing

Sci Rep. 2017 Nov 29;7(1):16554. Séverine Remy et al.

Generation of gene-edited rats by delivery of CRISPR/Cas9 protein and donor DNA into intact zygotes using electroporation

Nat Biotechnol. 2017 May;35(5):435-437. Kim K et al.

Highly efficient RNA-guided base editing in mouse embryos

Methods Mol Biol. 2017;1630:81-89. Kaneko T

Genome Editing in Mouse and Rat by Electroporation

Nat Biotechnol. 2016 Aug;34(8):807-8. Hur JK et al.

Targeted mutagenesis in mice by electroporation of Cpf1 ribonucleoproteins

PLoS One. 2015 Nov 10;10(11):e0142755. Kaneko T et al.

Simple Genome Editing of Rodent Intact Embryos by Electroporation

Sci Rep. 2014 Oct 1;4:6382. Kaneko T et al.

Simple knockout by electroporation of engineered endonucleases into intact rat embryos

[In Utero Electroporation](#)

Acta Neuropathol Commun. 2017 Jun 6;5(1):44. Lecuyer M et al.

PLGF, a placental marker of fetal brain defects after in utero alcohol exposure.

Nature. 2016 Dec 1;540(7631):144-149. Saito A et al.

In vivo genome editing via CRISPR/Cas9 mediated homology-independent targeted integration.

Nat Biotechnol. 2016 Oct;34(10):1060-1065. Morita S et al.

Targeted DNA demethylation in vivo using dCas9-peptide repeat and scFv-TET1 catalytic domain fusions

Development. 2016 Sep 1;143(17):3216-22. Tsunekawa Y et al.

Developing a *de novo* targeted knock-in method based on *in utero* electroporation into the mammalian brain

Cell. 2016 Jun 16;165(7):1803-1817. Mikuni T et al.

High-Throughput, High-Resolution Mapping of Protein Localization in Mammalian Brain by In Vivo Genome Editing

PLoS One. 2014 Aug 20;9(8):e105584. Straub C et al.

CRISPR/Cas9-Mediated Gene Knock-Down in Post-Mitotic Neurons

[In Vivo Pancreas Electroporation](#)

Nat Commun. 2016 Feb 26;7:10770. Maresch R et al.

Multiplexed pancreatic genome engineering and cancer induction by transfection-based CRISPR/Cas9 delivery in mice

[In Vivo Muscle Electroporation](#)

PLoS One. 2017 Jun 30;12(6):e0180217. Li X et al.

IGFN1_v1 is required for myoblast fusion and differentiation.

[Algae Electroporation](#)

Plant Cell. 2017 Oct;29(10):2498-2518. Andre Greiner et al.

Targeting of Photoreceptor Genes in *Chlamydomonas reinhardtii* via Zincfinger Nucleases and CRISPR/Cas9