NEPA21 Publication List for Genome Editing Applications

**Cell Culture Electroporation**

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

Microhomology-assisted scarless genome editing in human iPSCs

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

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

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

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

TALENs-mediated homozygous CCR5Δ32 mutations endow CD4+ U87 cells with resistance against HIV-1 infection

A Living Biobank of Breast Cancer Organoids Captures Disease Heterogeneity.

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

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

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

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.


(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.

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

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

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

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

Calcium dysregulation contributes to neurodegeneration in FTLD patient iPS cell-derived neurons

ANGPTL8/betatrophin alleviates insulin resistance via the Akt-GSK3β or Akt-FoxO1 pathway in HepG2 cells

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

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

Efficient modification of the myostatin gene in porcine somatic cells and generation of knockout piglets.
Inducible Transgene Expression in Human iPS Cells Using Versatile All-in-One piggyBac Transposons.

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

Efficient genetic engineering of human intestinal organoids using electroporation.

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

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.

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

Zygote Electroporation

Successful production of genome-edited rats by the rGONAD method

Electroporation of mice zygotes with dual guide RNA/Cas9 complexes for simple and efficient cloning-free genome editing
Generation of gene-edited rats by delivery of CRISPR/Cas9 protein and donor DNA into intact zygotes using electroporation

Highly efficient RNA-guided base editing in mouse embryos

Genome Editing in Mouse and Rat by Electroporation

Targeted mutagenesis in mice by electroporation of Cpf1 ribonucleoproteins

Simple Genome Editing of Rodent Intact Embryos by Electroporation

Simple knockout by electroporation of engineered endonucleases into intact rat embryos

**In Utero Electroporation**

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.

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

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

CRISPR/Cas9-Mediated Gene Knock-Down in Post-Mitotic Neurons
In Vivo Pancreas Electroporation

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

In Vivo Muscle Electroporation

IGFN1_v1 is required for myoblast fusion and differentiation.

Algae Electroporation

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