



**Recent References (2016-2020) on Gene Therapy Research  
Using ALZET® Osmotic Pumps**

**Q8586:** J. Kjell, *et al.* Defining the Adult Neural Stem Cell Niche Proteome Identifies Key Regulators of Adult Neurogenesis. *Cell Stem Cell* 2020;26(2):277-293 e8

**Agents:** Z-DON **Vehicle:** CSF, Artificial; DMSO; **Route:** CNS/CSF (intracerebral); IV; **Species:** Mice; **Pump:** 2001; **Duration:** 7 days;

**ALZET Comments:** Dose (100 µM); 0.4% DMSO used; Controls received mp w/ vehicle; animal info (male C57BL/6J mice, 8-10 weeks old); Z-DON aka Transglutaminase 2 inhibitor; ALZET brain infusion kit 2 used; Brain coordinates (1.2 mm laterally to and 0.5 mm posterior to the bregma (right side)); gene therapy;

**Q8557:** S. E. Joppe, *et al.* Genetic targeting of neurogenic precursors in the adult forebrain ventricular epithelium. *Life Sci Alliance* 2020;3(7):

**Agents:** Ara-C; **Vehicle:** Not stated; **Route:** CSF/CNS (intracerebral); IV; **Species:** Mice; **Pump:** 1007D; **Duration:** 7 days;

**ALZET Comments:** Controls received mp w/ vehicle; animal info (male mice); ALZET brain infusion kit 3 used; Brain coordinates (0 mm AP and -0.9 mm ML to the bregma); gene therapy;

**Q8486:** O. Gawrys, *et al.* Polyprenol-Based Lipofecting Agents for In Vivo Delivery of Therapeutic DNA to Treat Hypertensive Rats. *Biochem Genet* 2020;

**Agents:** vascular endothelial growth factor, DNA **Vehicle:** Glucose; **Route:** CSF/CNS (medula); **Species:** Rat; **Pump:** 2001; **Duration:** 1 week;

**ALZET Comments:** 5% glucose used; Controls received mp w/ vehicle; animal info (Male, adult spontaneously hypertensive rats, 16 weeks old, 299 + 4); Blood pressure measured via telemetry transmitters; vascular endothelial growth factor aka VEGF-A; ALZET brain infusion kit 2 used; gene therapy;

**Q8459:** S. Esteban, *et al.* Endothelial MT1-MMP targeting limits intussusceptive angiogenesis and colitis via TSP1/nitric oxide axis. *EMBO Mol Med* 2020;12(2):e10862

**Agents:** GDGRGDACK **Vehicle:** Dextran Sulfate; **Route:** SC; **Species:** Mice; **Pump:** 1003D; **Duration:** 3 days;

**ALZET Comments:** Dose (2.4 mg/mouse/day); 1% Dextran Sulfate used; animal info (C57BL/6 wild-type mice, 8-20 weeks old); peptides; gene therapy;

**Q8448:** S. Dominguez-Garcia, *et al.* A novel PKC activating molecule promotes neuroblast differentiation and delivery of newborn neurons in brain injuries. *Cell Death Dis* 2020;11(4):262

**Agents:** EOF2 **Vehicle:** PBS; DMSO; **Route:** CNS/CSF; **Species:** Mice; **Pump:** Not stated; **Duration:** 14 days;

**ALZET Comments:** Dose (5µM); 0.4% DMSO used; Controls received mp w/ vehicle; animal info (CD1 male mice, 2 months old); EOF2 aka plant derived diterpene; ALZET brain infusion kit II used; gene therapy;

**Q8434:** A. de Boer, *et al.* Environmental enrichment during the chronic phase after experimental stroke promotes functional recovery without synergistic effects of EphA4 targeted therapy. *Hum Mol Genet* 2020;29(4):605-617

**Agents:** APY-d3 **Vehicle:** CSF, Artificial; **Route:** CSF/CNS (intracerebral); IV; **Species:** Mice; **Pump:** 1002; **Duration:** 2 weeks;

**ALZET Comments:** Dose (5 µM); animal info (In-bred C57BL/6J male mice, 10–12 weeks of age); behavioral testing (accelerating rotarod; horizontal ladder task); APY-d3 aka peptide solution, β APYCVYR β ASWSC; peptides; ALZET brain infusion kit 3 used; Brain coordinates (0.1 mm caudal and 1.0 mm lateral of bregma); cyanoacrylate adhesive; gene therapy;

**Q7565:** C. S. Morrow, *et al.* Stem Cell Aging? Blame It on the Niche. *Cell Stem Cell* 2019;24(3):353-354

**Agents:** Antibody neutralizing inflammatory cytokine CXCL10 **Vehicle:** Not Stated; **Route:** SC; **Species:** Mice; **Pump:** Not Stated; **Duration:** Not Stated;

**ALZET Comments:** animal info (7 months old); gene therapy;

**Q8289:** F. M. Mir, *et al.* Paired Utility of Aza-Amino Acyl Proline and Indolizidinone Amino Acid Residues for Peptide Mimicry: Conception of Prostaglandin F2alpha Receptor Allosteric Modulators That Delay Preterm Birth. *J Med Chem* 2019;62(9):4500-4525



**Agents:** Azapeptide 2a and 2b **Vehicle:** Saline; **Route:** SC; **Species:** Mice; **Pump:** Not stated; **Duration:** Not stated;  
**ALZET Comments:** Dose (20 mg/day/animal); Controls received mp w/ vehicle; animal info (CD-1, Female, Pregnant); peptides; gene therapy;

**Q8287:** Z. Min, *et al.* Asymmetrical methyltransferase PRMT3 regulates human mesenchymal stem cell osteogenesis via miR-3648. *Cell Death Dis* 2019;10(8):581

**Agents:** Protein arginine methyltransferase 3 inhibitor **Vehicle:** DMSO; **Route:** SC; **Species:** Mice; **Pump:** 2002; **Duration:** 2, 4, or 6 weeks;

**ALZET Comments:** Dose (20 mg/kg/day); animal info (8 weeks old, C57BL/6); Multiple pumps per animal (1, 2, or 3); Protein arginine methyltransferase 3 inhibitor aka SGC707; enzyme inhibitor (Protein arginine methyltransferase 3 inhibitor); gene therapy;

**Q6966:** S. Kroller-Schon, *et al.* Endothelial alpha1AMPK modulates angiotensin II-mediated vascular inflammation and dysfunction. *Behavior Genetics* 2019;114(2):8

**Agents:** Angiotensin II **Vehicle:** Saline; **Route:** SC; **Species:** Mice; **Pump:** 1007D; **Duration:** 7 days;

**ALZET Comments:** Dose (0.5 mg/kg/day); 0.9% % NaCl used; Controls received mp w/ vehicle; animal info (6 week old mice); gene therapy;

**Q8043:** S. Ikeda, *et al.* Blockade of L-type Ca(2+) channel attenuates doxorubicin-induced cardiomyopathy via suppression of CaMKII-NF-kappaB pathway. *Sci Rep* 2019;9(1):9850

**Agents:** Nifedipine **Vehicle:** Saline; **Route:** SC; **Species:** Mice; **Pump:** 2002; **Duration:** 14 days;

**ALZET Comments:** Dose (10 mg/kg/day); Controls received mp w/ vehicle; animal info (9-10 weeks, C57BL/6J); Nifedipine aka L-type Ca2+ blocker ; gene therapy;

**R0372:** J. Hong, *et al.* Relaxin gene therapy: A promising new treatment option for various diseases with aberrant fibrosis or irregular angiogenesis. *Mol Cell Endocrinol* 2019;

**Agents:** Relaxin, human recomb. **Vehicle:** Not Stated; **Route:** SC; **Species:** Rat (pregnant); **Pump:** Not Stated; **Duration:** Not Stated;

**ALZET Comments:** Dose (2000 ng/h); Resultant plasma level (RLX level close to 0.5 ng/mL); gene therapy;

**Q8017:** S. Gupta, *et al.* Analysis of the Qatari R336C cystathionine beta-synthase protein in mice. *J Inherit Metab Dis* 2019;42(5):831-838

**Agents:** Bortezomib **Vehicle:** Not stated; **Route:** SC; **Species:** Mice; **Pump:** Not stated; **Duration:** 2 days;

**ALZET Comments:** Dose (0.49 mg/kg/day); animal info (Tg-R336C Cbs-/-); gene therapy;

**Q7455:** R. E. De la Vega, *et al.* Specific, Sensitive, and Stable Reporting of Human Mesenchymal Stromal Cell Chondrogenesis. *Tissue Eng Part C Methods* 2019;25(3):176-190

**Agents:** FK-506, SEW2871 **Vehicle:** Saline; **Route:** SC; **Species:** Rat; **Pump:** 2002; **Duration:** 14 days;

**ALZET Comments:** Dose (0.15 mg/kg/day- FK-506, 0.075 mg/kg/day- SEW2871); Controls received mp w/ vehicle; animal info (Male, 14 weeks old, Fischer-344); gene therapy;

**Q7242:** Y. Takeda, *et al.* Epigenetic Regulation of Aldosterone Synthase Gene by Sodium and Angiotensin II. *J Am Heart Assoc* 2018;7(10):

**Agents:** Angiotensin II, Candesartan **Vehicle:** Not Stated; **Route:** SC; **Species:** Rat; **Pump:** Not Stated; **Duration:** 4 weeks;

**ALZET Comments:** Dose (Ang II 200 ng/kg/min, Candesartan 1mg/kg/day); animal info (Male, Wistar, 6 weeks old); Candesartan aka Ang II type 1 receptor antagonist; gene therapy;

**Q7266:** K. Taguchi, *et al.* RAGE-aptamer attenuates deoxycorticosterone acetate/salt-induced renal injury in mice. *Sci Rep* 2018;8(1):2686

**Agents:** DNA aptamer (RAGE) **Vehicle:** Not Stated; **Route:** SC; **Species:** Mice; **Pump:** 2006; **Duration:** 21 days;

**ALZET Comments:** Dose (2 x10<sup>-4</sup> ug/day); Controls received mp w/ vehicle; animal info (8 week old, male, C57BL/6J ); RAGE-apt is a DNA aptamer against the advanced glycation end products receptor; gene therapy;



**Q7223:** J. Lu, *et al.* CIC-2 knockdown prevents cerebrovascular remodeling via inhibition of the Wnt/beta-catenin signaling pathway. *Cellular & Molecular Biology Letters* 2018;23(29)

**Agents:** Angiotensin II **Vehicle:** Saline; **Route:** SC; **Species:** Mice; **Pump:** 1002; **Duration:** Not Stated;

**ALZET Comments:** Dose (1.5 mg/kg/day); Controls received mp w/ vehicle; animal info (Male, 12 weeks old, 20-25 g, C57BL/6); gene therapy;

**Q8078:** F. Li, *et al.* Loss of renal SNX5 results in impaired IDE activity and insulin resistance in mice. *Diabetologia* 2018;61(3):727-737

**Agents:** SNX5-specific or non silencing snRNA **Vehicle:** Not stated; **Route:** SC; **Species:** Mice; **Pump:** 1007D; **Duration:** 7 days;

**ALZET Comments:** Dose (3 ug/day); animal info (Male, C57BL/6J, 1 year old); SNX5-specific aka sorting nexin 5 ; gene therapy;

**Q8058:** A. Kuroda, *et al.* Minocycline Directly Enhances the Self-Renewal of Adult Neural Precursor Cells. *Neurochem Res* 2018;43(1):219-226

**Agents:** Minocycline **Vehicle:** Saline; **Route:** CSF/CNS (Lateral ventricle); **Species:** Mice; **Pump:** 1007; **Duration:** 7 days;

**ALZET Comments:** "Dose (0.6 uL/h); Controls received mp w/ vehicle; animal info (20-30 weeks old, Male); Brain coordinates (0.3 mm posterior to Bregma, 0.8 mm lateral, and 2.7 mm below the surface of the skull); bilateral cannula used; cyanoacrylate adhesive; gene therapy; "

**Q7149:** K. Kamio, *et al.* Resolution of bleomycin-induced murine pulmonary fibrosis via a splenic lymphocyte subpopulation. *Respir Res* 2018;19(1):71

**Agents:** Bleomycin **Vehicle:** Saline; **Route:** SC; **Species:** Mice; **Pump:** 2001; **Duration:** 7 days;

**ALZET Comments:** Dose (100 mg/kg/day); Controls received mp w/ vehicle; animal info (C57BL/6); gene therapy;

**Q7139:** Y. Gao, *et al.* The histone methyltransferase DOT1L inhibits osteoclastogenesis and protects against osteoporosis. *Cell Death & Disease* 2018;9(2):33

**Agents:** EPZ5676 **Vehicle:** DMSO, Water; **Route:** SC; **Species:** Mouse; **Pump:** 2006, 2002; **Duration:** 8 weeks;

**ALZET Comments:** Dose (1.6 mg/d); 50% DMSO used; Controls received mp w/ vehicle; animal info (Eight-week-old, female, FVB/N); Model 2006 pumps replaced with Model 2002 after six weeks; long-term study; EPZ5676 aka small molecule inhibitor; gene therapy;

**Q7138:** S. Galic, *et al.* AMPK signaling to acetyl-CoA carboxylase is required for fasting- and cold-induced appetite but not thermogenesis. *eLife Journal* 2018;7(**Agents:** Ghrelin, N-octanoylated **Vehicle:** Ghrelin, N-octanoylated; **Route:** SC; **Species:** Mouse; **Pump:** 2002; **Duration:** 14 days;

**ALZET Comments:** Dose (30 ug/day); Controls received mp w/ vehicle; animal info (Male, ACC DKI); Peptide, recombinant protein aka N-octanoylated murine ghrelin; gene therapy;

**Q7137:** K. Fujita, *et al.* Targeting Tyro3 ameliorates a model of PGRN-mutant FTLT-TDP via tau-mediated synaptic pathology. *Nat Commun* 2018;9(1):433

**Agents:** Gö6976 **Vehicle:** PBS; **Route:** CSF/CNS (intrathecal); **Species:** Mouse; **Pump:** 2006; **Duration:** 2 weeks;

**ALZET Comments:** Dose (0.15 uL/h); animal info (PGRN-KI and C57BL/6J, 10-12 weeks old); behavioral testing (Morris water maze test, Fear-conditioning test, Probe test, Rotarod test, Open-field test, Light-dark box test ); enzyme inhibitor (PKC inhibitor); gene therapy;

**Q7109:** S. Chakraborty, *et al.* Dexamethasone-induced Intra-Uterine Growth Restriction impacts NOSTRIN and its downstream effector genes in the rat mesometrial uterus. *Sci Rep* 2018;8(1):8342

**Agents:** Dexamethasone **Vehicle:** Ethanol; **Route:** SC; **Species:** Rat; **Pump:** 2ML1; **Duration:** 7 days;

**ALZET Comments:** Dose (200 ug/kg/day); 10% ethanol used; Controls received mp w/ vehicle; animal info (Sprague Dawley, 8-10 weeks old); gene therapy;



**Q5940:** Y. Zou, *et al.* Prenatal levonorgestrel exposure induces autism-like behavior in offspring through ERbeta suppression in the amygdala. *Mol Autism* 2017;8(46)

**Agents:** Lentivirus, ER beta **Vehicle:** CSF, artificial; **Route:** CSF/CNS (amygdala); **Species:** Rat; **Pump:** 2002; **Duration:** 2 weeks;

**ALZET Comments:** Controls received mp w/ empty lentivirus; animal info (male, Sprague Dawley, 8 weeks old); functionality of mp verified by India ink injection; behavioral testing (marbles burying test, social interaction, elevated plus maze, open-field test); gene therapy; Used Plastics One cannula;

**Q5829:** N. Martinez-Sanchez, *et al.* Thyroid hormones induce browning of white fat. *J Endocrinol* 2017;232(2):351-362

**Agents:** Thyroxin, L-, Adenovirus vector; Gene, green fluorescent protein; Gene, AMP-activated protein kinase **Vehicle:** Saline; **Route:** CSF/CNS (hypothalamus); **Species:** Rat; **Pump:** 1007D; **Duration:** 7, 21 days;

**ALZET Comments:** bilateral cannula used; animal info (200-250g); gene therapy; Therapeutic indication (Browning, thyroid hormones);

**Q5052:** G. Murlidharan, *et al.* CNS-restricted Transduction and CRISPR/Cas9-mediated Gene Deletion with an Engineered AAV Vector. *Mol Ther Nucleic Acids* 2016;5(7):

**Agents:** Viral vector, adeno-associated (AAV9, AAV2g0); Gene, CBh-ScGFP **Vehicle:** Not Stated; **Route:** CSF/CNS (intrathecal); **Species:** Mice; **Pump:** 2001D; **Duration:** 24 hrs;

**ALZET Comments:** animal info (8 weeks old C57/Bl6 male mice); comparison of IT bolus injections vs mp; ALZET mouse intrathecal catheter used (lumbar cannulation); gene therapy;

**Q5158:** M. Gujrati, *et al.* Multifunctional pH-Sensitive Amino Lipids for siRNA Delivery. *Bioconjugate Chemistry* 2016;27(1):19-35

**Agents:** RNA, small interfering/EHCO; PEGylated EHCO **Vehicle:** Not Stated; **Route:** Not Stated; **Species:** Mice (nude); **Pump:** Not Stated; **Duration:** 14 days;

**ALZET Comments:** Controls received treated with nonspecific PEGylated EHCO/siGFP nanoparticles (PEGGFP) and non-PEGylated EHCO/HIF-1 $\alpha$ ; cancer; gene therapy, RNA nanoparticle infusion; peptides; "These results indicate that PEGylation can significantly improve the stability of EHCO/siRNA nanoparticles during storage in solution, possibly by preventing the aggregation of the nanoparticles and providing better protection to the siRNA cargo from degradation" (pg 31);

**Q5312:** K. Caviness, *et al.* Complex Interplay of the UL136 Isoforms Balances Cytomegalovirus Replication and Latency. *MBio* 2016;7(2):e01986

**Agents:** Granulocyte-colony stimulating factor; AMD3100 **Vehicle:** Not Stated; **Route:** IP; **Species:** Mice (NOD/SCID); **Pump:** 1007D; **Duration:** 1 week;

**ALZET Comments:** animal info (NOD-scid humanized (huNSG) mice); gene therapy; immunology; Engraftment of human CD45+ cells; viral persistence; Dose (300mg/ml Colony-stim; 125 ug AMD3100);

**Q5625:** J. Benoit, *et al.* Epigenetic stability in the adult mouse cortex under conditions of pharmacologically induced histone acetylation. *Brain Structure and Function* 2016;221(8):3963-3978

**Agents:** Trichostatin A **Vehicle:** DMSO, water; **Route:** SC; **Species:** Mice; **Pump:** 2002; **Duration:** 14 days, 28 days;

**ALZET Comments:** Controls received mp w/ vehicle; animal info (6 months old); Subset of pumps replaced every 2 weeks; gene therapy;