

OptiPrep™ Reference List RV02-2

GROUP II VIRUSES - rAAV

- ◆ **IMPORTANT NOTE: RV02-2 LISTS rAAV PAPERS PUBLISHED AFTER SEPT. 2018. THE COMPANION LIST (RV02-1) LISTS rAAV PAPERS PUBLISHED BEFORE SEPT. 2018.**
- ◆ **RV02-1 also lists publications on Group II viruses other than rAAV.**
- ◆ **References are divided alphabetically into “Site of delivery” and/or “Research topic” sections. References in each section are listed alphabetically according to first author; multiple references by the same first author are presented chronologically.**
- ◆ **To aid selection, key words are highlighted in blue.**
- ◆ **For detailed methodologies of Group II virus purifications see OptiPrep™ Application Sheets V14-V16. V06 is a methodological review of OptiPrep™ technology.**

AAV properties

AAV2 capsid structure

Tan, Y.Z., Aiyer, S., Mietzsch, M., Hull, J.A., McKenna, R., Grieger, J., Samulski, R.J., Baker, T.S., Agbandje-McKenna, M. and Lyumkis, D. (2018) *Sub-2 Å Ewald curvature corrected structure of an AAV2 capsid variant* Nat. Comm., **9**: 3628

AAVr3.45

Cho, M., Jung, K., Kim, S-H., Kim, I-S., Kim, M., Shin, M., Lee, H., Park, K.I. and Jang, J-H. (2019) *Safety and efficacy evaluations of an adeno-associated virus variant for preparing IL10-secreting human neural stem cell-based therapeutics* Gene Ther., **26**, 135–150

AAV biology

Wang, D., Tai, P.W.L. and Gao, G. (2019) *Adeno-associated virus vector as a platform for gene therapy delivery* Nat. Rev., **18**, 358-378

AAV libraries

Schmit, P.F., Pacouret, S., Zinn, E., Telford, E., Nicolaou, F., Broucque, F., Andres-Mateos, E., Xiao, R. et al (2020) *Cross-packaging and capsid mosaic formation in multiplexed AAV libraries* Mol. Ther: Meth. Clin. Devel., **17**, 107-121

AAV receptors

Zhang, R., Xu, G., Cao, L., Sun, Z., He, Y., Cui, M., Sun, Y., Li, S., Li, H., Qin, L. et al (2019) *Divergent engagements between adeno-associated viruses with their cellular receptor AAVR* Nat. Comm., **10**: 3760

Adipose tissue (incl. brown fat tissue)

Huang, W., Queen, N.J. and Cao, L. (2019) *rAAV-mediated gene delivery to adipose tissue* In Adeno-Associated Virus Vectors: Design and Delivery, Methods in Mol. Biol., **vol. 1950** (ed. Castle, M.J.), Springer Science+Business Media LLC New York, pp 389-405

Mouchiroud, M., Camiré, E., Aldow, M., Caron, A., Jubinville, E., Turcotte, L., Kaci, I., Beaulieu, M-J., Roy, C. et al (2019) *The Hepatokine TSK does not affect brown fat thermogenic capacity, body weight gain, and glucose homeostasis* Mol. Metab., **30**, 184-191

Age-related diseases

Davidsohn, N., Pezzone, M., Vernet, A., Graveline, A., Oliver, D., Slomovic, S., Punthambaker, S., Sun, X., Liao, R., Bonventre, J.V., and Church, G.M. (2019) *A single combination gene therapy treats multiple age-related diseases* Proc. Nat. Acad. Sci. USA, **116**, 23505–23511

Aldehyde dehydrogenase

Matsumura, Y., Stiles, K.M., Reid, J., Frenk, E.Z., Cronin, S., Pagovich, O.E. and Crystal, R.G. (2019) *Gene therapy correction of aldehyde dehydrogenase 2 deficiency* Mol. Ther: Meth. Clin. Devel., **15** 72-82

Amyloidosis

Colon-Perez, L.M., Ibanez, K.R., Suarez, M., Torroella, K., Acuna, K., Ofori, E., Levites, Y., Vaillancourt, D.E., Golde, T.E. et al (2019) *Neurite orientation dispersion and density imaging reveals white matter and hippocampal microstructure changes produced by Interleukin-6 in the TgCRND8 mouse model of amyloidosis* NeuroImage, **202**: 116138

Anaesthesia

Zhou, B., Chen, L., Liao, P., Huang, L., Chen, Z., Liao, D., Yang, L., Wang, J. et al (2019) *Astroglial dysfunctions drive aberrant synaptogenesis and social behavioral deficits in mice with neonatal exposure to lengthy general anaesthesia* PLoS Biol., **17**: e3000086

Angiogenesis

Lee, S., Ahad, A., Luu, M., Moon, S., Caesar, J.A., Cardoso, W.V., Grant, M.B. and Chaqoura, B. (2019) *CCN1–Yes-associated protein feedback loop regulates physiological and pathological angiogenesis* Mol. Cell. Biol., **39**: e00107-19

Atherosclerosis

Zhou, M., Learned, R.M., Rossi, S.J., Tian, H., DePaoli, A.M. and Ling, L. (2019) *Therapeutic FGF19 promotes HDL biogenesis and trans-hepatic cholesterol efflux to prevent atherosclerosis* J. Lipid Res., **60**, 550–565

Auditory function

Al-Moyed, H., Cepeda, A.P., Jung, S.Y., Moser, T., Kügler, S. and Reisinger, E. (2019) *A dual-AAV approach restores fast exocytosis and partially rescues auditory function in deaf otoferlin knock-out mice* EMBO Mol. Med., **11**: e9396

Autoimmune disorders

Vasquez, M., Consuegra-Fernández, M., Aranda, F., Jimenez, A., Tenesaca, S., Fernandez-Sendin, M., Gomar, C., Ardaiz, N., Di Trani, C.A. (2019) *Treatment of experimental autoimmune encephalomyelitis by sustained delivery of low-dose IFN-α* J. Immunol., **203**, 696–704

Autophagy

Wu, H., Chen, H., Zheng, Z., Li, J., Ding, J., Huang, Z., Jia, C., Shen, Z., Bao, G., Wu, L., Al Mamun, A. et al (2019) *Trehalose promotes the survival of random-pattern skin flaps by TFEB mediated autophagy enhancement* Cell Death Dis., **10**: 483

Baculovirus expression vector

Sandro, Q., Relizani, K. and Benchaouir, R. (2019) *AAV production using baculovirus expression vector system* In Viral Vectors for Gene Therapy: Methods and Protocols, Methods in Mol. Biol., **1937** (ed. Manfredsson F.P. and Benskey, M.J.), Springer Science+Business Media LLC New York, pp 91-99

Basolateral amygdalae

Huang, T-N., Hsu, T-T., Lin, M-H., Chuang, H-C., Hu, H-T., Sun, C-P., Tao, M-H., Lin, J.Y. and Hsueh, Y-P. (2019) *Interhemispheric connectivity potentiates the basolateral amygdalae and regulates social interaction and memory* Cell Reports 29, 34–48

Biomaterial-based delivery

Kim, S-H., Lee, S., Lee, H., Cho, M., Schaffer, D.V. and Jang, J-H. (2019) *AAVR-displaying interfaces: serotype-independent adeno-associated virus capture and local delivery systems* Mol. Ther: Meth. Clin. Devel., **15**, 432-443

Bocaviruses

Fakhiri, J., Schneider, M.A., Puschhof, J., Stanifer, M., Schildgen, V., Holderbach, S., Voss, Y., El Andari, J., Schildgen, O. et al (2019) *Novel chimeric gene therapy vectors based on adeno-associated virus and four different mammalian bocaviruses* Mol. Ther. Meth. Clin. Devel., **12**, 202-222

Bone targeting

Alméciga-Díaz, C.J., Montaña, A.M., Barrera, L.A. and Tomatsu, S. (2018) *Tailoring the AAV2 capsid vector for bone-targeting* Pediatr. Res., **84**, 545–551

Bronchial epithelial cells - Cystic fibrosis

Lopes-Pacheco, M., Kitoko, J.Z., Morales, M.M., Petrs-Silva, H. and Rocco, P.R.M. (2018) *Self-complementary and tyrosine-mutant rAAV vectors enhance transduction in cystic fibrosis bronchial epithelial cells* Exp. Cell Res., **372**, 99–107

Capsid structure/capsid variants/libraries

Cabanes-Creus, M., Ginn, S.L., Amaya, A.K., Liao, S.H.Y., Westhaus, A., Hallwirth, C.V., Wilmott, P., Ward, J., Dilworth, K.L. (2019) *Codon-optimization of wild-type adeno-associated virus capsid sequences enhances DNA family shuffling while conserving functionality* Mol. Ther. Meth. Clin. Devel., **12**, 71-84

Challis, R.C., Kumar, S.R., Chan, K.Y., Challis, C., Beadle, K., Jang, M.J., Kim, H.M., Rajendran, P.S., Tompkins, J.D et al (2019) *Systemic AAV vectors for widespread and targeted gene delivery in rodents* Nat. Protoc., **14**, 379–414

Hanlon, K.S., Meltzer, J.C., Buzhdygan, T., Cheng, M.J., Sena-Esteves, M., Bennett, R.E., Sullivan, T.P., Razmpour, R. et al (2019) *Selection of an efficient AAV vector for robust CNS transgene expression* Mol. Ther. Meth. Clin. Devel. **15**, 320-332

Mary, B., Maurya, S., Arumugam, S., Kumar, V. and Jayandharan, G.R. (2019) *Post-translational modifications in capsid proteins of recombinant adeno-associated virus (AAV) 1-rh10 serotypes* FEBS J., **286**, 4964–4981

Shinohara, Y., Konno, A., Nitta, K., Matsuzaki, Y., Yasui, H., Suwa, J., Hiromura, K. and Hirai, H. (2019) *Effects of neutralizing antibody production on AAV-PHP.B-mediated transduction of the mouse central nervous system* Mol. Neurobiol., **56**, 4203–4214

Thadani, N.N., Dempsey, C., Zhao, J., Vasquez, S.M. and Suh, J. (2018) *Reprogramming the activatable peptide display function of adeno-associated virus nanoparticles* ACS Nano, **12**, 1445–1454

Cardiac disorders

Cardiac gene delivery

Adamiak, M., Liang, Y., Mathiyalagan, P., Agarwal, M., Jha, D., Kohlbrenner, E., Chepurko, E., Jeong, D. et al (2018) *Robust cardiac gene delivery and evasion of neutralizing antibodies by extracellular vesicle-associated AAV vectors* Circulation, **138**, Suppl.1, abstr. 16378

Guenther, C.M., Brun, M.J., Bennett, A.D., Ho, M.L., Chen, W., Zhu, B., Lam, M., Yamagami, M., Kwon, S., Bhattacharya, N. et al. (2019) *Protease-activatable adeno-associated virus vector for gene delivery to damaged heart tissue* Mol. Ther., **27**, 611-622

Lopez-Gordo, E., Kohlbrenner, E., Katz, M.G. and Weber, T. (2019) *AAV vectors for efficient gene delivery to rodent hearts* In Adeno-Associated Virus Vectors: Design and Delivery, Methods in Mol. Biol., vol. **1950** (ed. Castle, M.J.), Springer Science+Business Media LLC New York, pp 311-332

Yoo, J., Kohlbrenner, E., Kim, O., Hajjar, R.J. and Jeong, D. (2018) *Enhancing atrial-specific gene expression using a calsequestrin cis-regulatory module 4 with a sarcolipin promoter* J. Gene Med., **20**: e3060

Cardiac failure/hypertrophy/functional protection

Gao, J., Guo, Y., Chen, Y., Zhou, J., Liu, Y. and Su, P. (2019) *Adeno-associated virus 9-mediated RNA interference targeting SOCS3 alleviates diastolic heart failure in rats* Gene, **697**, 11–18

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Mi-Mi, L., Farman, G.P., Mayfield, R.M., Strom, J., Chu, M., Pappas, C.T. and Gregorio, C.C. (2020) *In vivo elongation of thin filaments results in heart failure* PLoS One, **15**: e0226138

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Stroik, D.R., Ceholski, D.K., Bidwell, P.A., Mleczko, J., Thanel, P.F., Kamdar, F., Autry, J.M., Cornea, R.L. and Thomas, D.D. (2020) *Viral expression of a SERCA2a-activating PLB mutant improves calcium cycling and synchronicity in dilated cardiomyopathic hiPSC-CMs* J. Mol. Cell. Cardiol., **138**, 59–65

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Cardiomyocyte proliferation

Haginiwa, S., Sadahiro, T., Kojima, H., Isomi, M., Tamura, F., Kurotsu, S., Tani, H., Muraoka, N. et al (2019) *Tbx6 induces cardiomyocyte proliferation in postnatal and adult mouse hearts* Biochem. Biophys. Res. Comm., **513**, 1041-1047

Cardiomyopathy

Chatzifrangkeskou, M., Yadin, D., Marais, T., Chardonnet, S., Cohen-Tannoudji, M., Mougnot, N., Schmitt, A., Crasto, S., Di Pasquale, E. et al (2018) *Cofilin-1 phosphorylation catalyzed by ERK1/2 alters cardiac actin dynamics in dilated cardiomyopathy caused by lamin A/C gene mutation* Hum. Mol. Genet., **27**, 3060-3078

Liao, D., Chen, W., Tan, C.Y., Wong, J.X., Chan, P.S., Tan, L.W., Foo, R. and Jiang, J. (2019) *Upregulation of Y1l suppresses dilated cardiomyopathy caused by Ttn insufficiency* Sci. Rep., **9**: 16330

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Xu, R., Jia, Y., Zygmunt, D.A. and Martin, P.T. (2019) *rAAVrh74.MCK.GALGT2 protects against loss of hemodynamic function in the aging mdx mouse heart* Mol. Ther., **27**, 636-649

Yadav, S., Yuan, C-C., Kazmierczak, K., Liang, J., Huang, W., Takeuchi, L.M., Kanashiro-Takeuchi, R.M. and Szczesna-Cordary, D. (2019) *Therapeutic potential of AAV9-S15D-RLC gene delivery in humanized MYL2 mouse model of HCM* J. Mol. Med., **97**, 1033–1047

Heart rate control

Rajendran, P.S., Challis, R.C., Fowlkes, C.C., Hanna, P., Tompkins, J.D., Jordan, M.C., Hiyari, S., Gabris-Weber, B.A., Greenbaum, A. et al (2019) *Identification of peripheral neural circuits that regulate heart rate using optogenetic and viral vector strategies* Nature Comm., **10**: 1944

Myocardial infarction

Garikipati, V.N.S., Verma, S.K., Cheng, Z., Liang, D., Truongcao, M.M., Cimini, M., Yue, Y., Huang, G., Wang, C. et al (2019) *Circular RNA CircFncd3b modulates cardiac repair after myocardial infarction via FUS/VEGF-A axis* Nat. Comm., **10**: 4317

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Motloch, L.J., Cacheux, M., Ishikawa, K., Xie, C., Hu, J., Agüero, J., Fish, K.M., Hajjar, R.J. and Akar, F.G. (2018) *Primary effect of SERCA2a gene transfer on conduction reserve in chronic myocardial infarction* J. Am. Heart Assoc., **7**: e009598

Tian, S., Lei, I., Gao, W., Liu, L., Guo, Y., Creech, J., Herron, T.J., Xian, S., Ma, P.X., Chen, Y.E., Li, Y., Alam, H.B. and Wang, Z. (2019) *HDAC inhibitor valproic acid protects heart function through Foxm1 pathway after acute myocardial infarction* Ebio Med., **39**, 83–94

Transcription factors

Akerberg, B.N., Gu, F., VanDusen, N.J., Zhang, X., Dong, R., Li, K., Zhang, B., Zhou, B., Sethi, I., Ma, Q. et al (2019) *A reference map of murine cardiac transcription factor chromatin occupancy identifies dynamic and conserved enhancers* Nat. Comm., **10**: 4907

Cell-specific gene transfer

Reul, J., Muik, A. and Buchholz, C.J. (2019) *Ligand coupling to the AAV capsid for cell-specific gene transfer* In Adeno-Associated Virus Vectors: Design and Delivery, Methods in Mol. Biol., vol. **1950** (ed. Castle, M.J.), Springer Science+Business Media LLC New York, pp 35-50

Cellular communication network factor 1 (CCN1)

Lee, S., Ahad, A., Luu, M., Moon, S., Caesar, J.A., Cardoso, W.V., Grant, M.B. and Chaqoura, B. (2019) *CCN1–Yes-associated protein feedback loop* regulates physiological and pathological *angiogenesis* *Mol. Cell Biol.*, **39**: e00107-19

Cholestasis

Aronson, S.J., Bakker, R.S., Shi, X., Duijst, S., ten Bloemendaal, L., de Waart, D.R., Verheij, J., Ronzitti, G. et al (2019) *Liver-directed gene therapy results in long-term correction of progressive familial intrahepatic cholestasis type 3 in mice* *J. Hepatol.*, **71**, 153–162

Circular RNAs

Meganck, R.M., Borchardt, E.K., Castellanos Rivera, R.M., Scalabrino, M.L., Wilusz, J.E., Marzluff, W.F. and Asokan, A. (2018) *Tissue-dependent expression and translation of circular RNAs with recombinant AAV vectors in vivo* *Mol. Ther. Nucleic Acids*, **13**, 89-98

Clinical application (AAV vector choice)

He, T., Itano, M.S., Earley, L.F., Hall, N.E., Riddick, N., Samulski, R.J. and Li, C. (2019) *The influence of murine genetic background in adeno-associated virus transduction of the mouse brain* *Human Gene Ther. Clin. Devel.*, **30**, 169-181

Cre recombinase gene

Meador, K., Wysoczynski, C.L., Norris, A.J., Aoto, J., Bruchas, M.R. and Tucker, C.L. (2019) *Achieving tight control of a photoactivatable Cre recombinase gene switch: new design strategies and functional characterization in mammalian cells and rodent* *Nucl. Acids Res.*, **47**: e97

CRISPR/Cas9

Chen, S., Sun, S., Moonen, D., Lee, C., Lee, A.Y-F., Schaffer, D.V. and He, L. (2019) *CRISPR-READI: efficient generation of knockin mice by CRISPR RNP electroporation and AAV donor infection* *Cell Rep.*, **27**, 3780–3789

D'Amico, D., Mottis, A., Potenza, F., Sorrentino, V., Li, H., Romani, M., Lemos, V., Schoonjans, K. et al (2019) *The RNA-binding protein PUM2 impairs mitochondrial dynamics and mitophagy during aging* *Mol. Cell* **73**, 775–787

Ekman, F.K., Ojala, D.S., Adil, M.M., Lopez, P.A., Schaffer, D.V. and Gaj, T. (2019) *CRISPR-Cas9-mediated genome editing increases lifespan and improves motor deficits in a Huntington's disease mouse model* *Mol. Ther: Nucleic Acids*, **17**, 829-839

Fakhiri, J., Nickl, M. and Grimm, D. (2019) *Rapid and simple screening of CRISPR guide RNAs (gRNAs) in cultured cells using adeno-associated viral (AAV) vectors* In *CRISPR Gene Editing: Methods and Protocols*, *Methods in Mol. Biol.*, vol. 1961 (ed. Luo, Y.), Springer Science+Business Media LLC New York, pp 111-126

Gao, Y., Hisey, E., Bradshaw, T.W.A., Erata, E., Brown, W.E., Courtland, J.L., Uezu, A., Xiang, Y., Diao, Y. and Soderling, S.H. (2019) *Plug-and-play protein modification using homology-independent universal genome engineering* *Neuron*, **103**, 583–597

Ideno, N., Yamaguchi, H., Okumura, T., Huang, J., Brun, M.J., Ho, M.L., Suh, J., Gupta, S., Maitra, A. and Ghosh, B. (2019) *A pipeline for rapidly generating genetically engineered mouse models of pancreatic cancer using in vivo CRISPR-Cas9-mediated somatic recombination* *Lab. Invest.*, **99**, 1233–1244

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Pavel-Dinu, M., Wiebking, V., Dejene, B.T., Srifa, W., Mantri, S., Nicolas, C.E., Lee, C., Bao, G. and Kildebeck, E.J. (2019) *Gene correction for SCID-X1 in long-term hematopoietic stem cells* *Nat. Comm.*, **10**: 1634

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Tran, N.T., Sommermann, T., Graf, R., Trombke, J., Pempe, J., Petsch, K., K€uhn, R., Rajewsky, K. and Chu, V.T. (2019) *Efficient CRISPR/Cas9-mediated gene knockin in mouse hematopoietic stem and progenitor cells* Cell Reports **28**, 3510- 3522

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Cytosine/adenine base editing

Levy, J.M., Yeh, W-H., Pendse, N., Davis, J.R., Hennessey, E., Butcher, R., Koblan, L.W., Comander, J., Liu, Q. and Liu, D.R. (2020) *Cytosine and adenine base editing of the brain, liver, retina, heart and skeletal muscle of mice via adeno-associated viruses* Nat. Biomed. Engineer., **4**, 97–110

Dendritic cell targeting

Krotova, K., Day, A. and Aslanidi, G. (2019) *An engineered AAV6-based vaccine induces high cytolytic anti-tumor activity by directly targeting DCs and improves Ag presentation* Mol. Ther. Oncolytics, **15**, 166-177

Rossi, A., Dupaty, L, Aillot, L, Zhang, L, Gallien, C., Hallek, M., Odenthal, M., Adriouch, S et al (2019) *Vector uncoating limits adenoassociated viral vector-mediated transduction of human dendritic cells and vector immunogenicity* Sci. Rep., **9**: 3631

Diabetic disorders

Gan, S.U., Fu, Z., Sia, K.C., Kon, O.L., Calne, R. and Lee, K.O. (2019) *Development of a liver-specific Tet-off AAV8 vector for improved safety of insulin gene therapy for diabetes* J. Gene Med. **21**: 3067

Guay, C., Kruit, J.K., Rome, S., Menoud, V., Mulder, N.L., Jurdzinski, A., Mancarella, F., Sebastiani, G., Donda, A. and Gonzalez, B.J. (2019) *Lymphocyte-derived exosomal microRNAs promote pancreatic β cell death and may contribute to type 1 diabetes development* Cell Metab., **28**, 348-361

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Li, T., Li, H., Li, W., Chen, S., Feng, T., Jiao, W., Wu, C., Dong, J. et al (2019) *Interleukin-37 sensitize the elderly type 2 diabetic patients to insulin therapy through suppressing the gut microbiota dysbiosis* Mol. Immunol., **112**, 322–329

DNA shuffling

Herrmann, A-K., Bender, C., Kienle, E., Gross, S., El Andari, J., Botta, J., Schürmann, N., Wiedtke, E., Niopek, D. and Grimm, D. (2019) *A robust and all-inclusive pipeline for shuffling of adeno-associated viruses* ACS Synth. Biol., **8**, 194–206

Endothelial cells

Zhang, L., Rossi, A., Lange, L., Meumann, N., Koitzsch, U., Christie, K., Nesbit, M.A., Moore, C.B.T., Hacker, U.T. et al (2019) *Capsid engineering overcomes barriers toward adeno-associated virus vector-mediated transduction of endothelial cells* Hum. Gene Ther., **30**. 1284-1296

Enteric nervous system

Boesmans, W., Hao, M.M., Fung, C., Li, Z., Van den Haute, C., Tack, J., Pachnis, V. and Vanden Berghe, P. (2019) *Structurally defined signaling in neuro-glia units in the enteric nervous system* Glia, **67**, 1167–1178

Epidermal growth factor

Cramer, M.L., Xu, R. and Martin, P.T. (2019) *Soluble heparin binding epidermal growth factor-like growth factor is a regulator of GALGT2 expression and GALGT2-dependent muscle and neuromuscular phenotypes* Mol. Cell. Biol., **39**: e00140-19

Epilepsy

Snowball, A., Chabrol, E., Wykes, R.C., Shekh-Ahmad, T., Cornford, J.H., Lieb, A., Hughes, M.P., Massaro, G., Rahim, A.A. (2019) *Epilepsy gene therapy using an engineered potassium channel* J. Neurosci., **39**, 3159 – 3169

Food-seeking behaviour

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Haemophilia

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