OptiPrep™ Reference List RV02-1

GROUP II VIRUSES

- **PART A OF THIS REFERENCE LIST (pp 1-118) provides a comprehensive reference list of papers reporting the use of OptiPrep™ for rAAV purification.**
- **PART B of RV02-1 lists ALL other Group II virus papers (starting p118).**
- **IMPORTANT NOTE: The rAAV PAPERS RUN TO SEPT. 2018.**
- **THE COMPANION LIST (RV02-2) LISTS rAAV PAPERS AFTER SEPT. 2018.**
- **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 all OptiPrep™ technology.**

PART A

Adenovirus infections

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Adipose tissue delivery

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Liu, X., Magee, D., Wang, C., McMurphy, T., Slater, A., During, M. and Cao, L. (2014) *Adipose tissue insulin receptor knockdown via a new primate-derived hybrid recombinant AAV serotype* Mol. Ther. Meth. Clin. Dev., **1**: 8

Adrenal cortex delivery

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AdV receptor (see "Coxsackie virus")

Airway delivery (see "Respiratory system delivery")

Amyotrophic lateral sclerosis

Ceballos-Diaz, C., Rosario, A.M., Park, H-J., Chakrabarty, P., Sacino, A., Cruz, P.E., Siemienski, Z., Lara, N. et al (2015) *Viral expression of ALS-linked ubiquilin-2 mutants causes inclusion pathology and behavioral deficits in mice* Mol. Neurodegener., **10** :25

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Kubodera, T., Yamada, H., Anzai, M., Ohira, S., et al (2011) *In vivo application of an RNAi strategy for the selective suppression of a mutant allele* Hum. Gene. Ther., **22**, 27-34
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Uchida, A., Sasaguri, H., Kimura, N., Tajiri, M., et al (2012) *Non-human primate model of amyotrophiclateral sclerosis with cytoplasmic mislocalization of TDP-43* Brain, **135**, 833–846
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Anaphylaxis

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Anti-angiogenesis (cancer therapy); see also "Tumour cells, effect on"

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Anti-viral activity

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Apolipoproteins (see "Lipoprotein metabolism-related disorders")

Arsenic trioxide

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Arthritic joint delivery

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Atherosclerosis (see "Lipoprotein metabolism-related disorders")

Baculovirus expression system

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Bile salt metabolism

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Blood-brain barrier

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Body weight control (see "Obesity")

Bone disorders

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Caffeine, effect of

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Capsid – assembly, target specificity, structure, peptide ligand insertion

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Chen, Y.H., Claflin, K., Geoghegan, J.C. and Davidson, B.L. (2012) *Sialic acid deposition impairs the utility of AAV9, but not peptide-modified AAVs for brain gene therapy in a mouse model of lysosomal storage disease* Mol. Ther., **20**, 1393-1399
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Hippocampus dentate gyrus delivery

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Embryonic stem cells (see "Stem cell delivery")

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Hypercholesterolaemia (see Lipoprotein metabolism-related disorders")
Hyperoxaluria (see Hepatic/hepatocyte delivery")

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Inner ear delivery (see also "Cochlear delivery")

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Insulin growth factor etc, see "Diabetes and other pancreatic disorders"

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Intestinal delivery

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Intracellular interactions

Molecular interactions

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Xiao, W., Warrington, K.H., Hearing, P. Hughes, J. and Muzyczka, N. (2002) *Adenovirus-facilitated nuclear translocation of adeno-associated virus type 2* J. Virol., **76**, 11505-11517

Intramuscular injection

Benkhelifa-Ziyyat, S., Besse, A., Roda, M., Duque, S., Astord, S., Carcenac, R., Marais, T. and Barkats, M. (2013*) Intramuscular scAAV9-SMN injection mediates widespread gene delivery to the spinal cord and decreases disease severity in SMA mice* Mol. Ther., **21**, 282–290

Chew, W.L., Tabebordbar, M., Cheng, J.K.W., Mali, P., Wu, E.Y., Ng, A.H.M., Zhu, K., Wagers, A.J. and Church, G.M. (2016) *A multifunctional AAV–CRISPR–cas9 and its host response* Nat. Meth., **13**, 868-874

Van Lieshout, L.P., Soule, G., Sorensen, D., Frost, K.L., He, S., Tierney, K., Safronetz, D., Booth, S.A. (2018) *Intramuscular adeno-associated virus–mediated expression of monoclonal antibodies provides 100% protection against Ebola virus infection in mice* J. Infect. Dis., **217**, 917-925

Intraperitoneal injection

Akbar, M.A., Cao, J.J., Lu, Y., Nardo, D., Chen, M-J., Elshikha, A.S., Ahamed, R., Brantly, M., Holliday, L.S. and Song, S. (2016) *Alpha-1 antitrypsin gene therapy ameliorates bone loss in ovariectomy-induced osteoporosis mouse model* Hum. Gene Ther., **27**, 679-686

Machida, A., Kuwahara, H., Mayra, A., Kubodera, T., et al (2013) *Intraperitoneal administration of AAV9-shRNA inhibits target gene expression in the dorsal root ganglia of neonatal mice* Mol. Pain, **9**:36
Medina-Echeverz, J., Fioravanti, J., Díaz-Valdés, N., Frank, K., et al (2014) *Harnessing high density lipoproteins to block transforming growth factor beta and to inhibit the growth of liver tumor metastases* PLoS One, **9**: e96799
Schultze, S.M., Dietrich, M., Hynx, D., Geier, A., Niessen, M., Spinas, G.A., Hemmings, B.A. and Tschopp, O. (2015) *Reduced hepatic lipid content in Pten-haplodeficient mice because of enhanced AKT2/PKBβ activation in skeletal muscle* Liver Int., **35**, 1354−1366
Shettigar, V., Zhang, B., Little, S.C., Salhi, H.E., Hansen, B.J., Li, N., Zhang, J., Roof, S.R., Ho, H-T., Brunello, L. et al (2016) *Rationally engineered Troponin C modulates in vivo cardiac function and performance in health and disease* Nat. Comm., **7**: 10794
Vrontou, S., Wong, A.M., Rau, K.K., Koerber, R. and Anderson, D.J. (2013) *Genetic identification of C fibres that detect massage-like stroking of hairy skin in vivo* Nature, **493**, 669-673

Intramuscular injection

Davis, A.S., Federici, T., Ray, W.C., Boulis, N.M., O'Connor, D., Clark, K.R. and Bartlett, J.S. (2015) *Rational design and engineering of a modified adeno-associated virus (AAV1)-based vector system for enhanced retrograde gene delivery* Neurosurgery, **76**, 216-225

Intrathecal injection

Huda, F., Konno, A., Matsuzaki, Y., Goenawan, H., Miyake, K., Shimada, T. and Hirai, H. (2014) *Distinct transduction profiles in the CNS via three injection routes of AAV9 and the application to generation of a neurodegenerative mouse model* Mol. Ther. Methods Clin. Dev., **1:** 14032
Ramsingh, A.I., Gray, S.J., Reilly, A., Koday, M., Bratt, D., Koday, M.T., Murnane, R., Smedley, J., Hu, Y., Messer, A. and Fuller, D.H. (2018) *Sustained AAV9-mediated expression of a non-self protein in the CNS of non-human primates after immunomodulation* PLoS One, 13: e0198154

Intrathoracic injection

Huang, J., Li, X., Coelho-dos-Reis, J.G.A., Wilson, J.M., Tsuji, M. (2014) *An AAV vector-mediated gene delivery approach facilitates reconstitution of functional human $CD8^+$ T cells in mice* PLoS One, **9**: e88205

Intratracheal injection

Vidović, D., Gijsbers, R., Quiles-Jimenez, A., Dooley, J., Van den Haute, C., Van der Perren, A., Liston, A., Baekelandt, V., Debyser, Z. and Carlon, M.S. (2016) *Noninvasive imaging reveals stable transgene expression in mouse airways after delivery of a nonintegrating recombinant adeno-associated viral vector* Hum. Gene Ther., **27**, 60-71

Intravenous injection

The vast number of papers reporting the use of intravenous injection in rAAV studies makes such a list unhelpful; these papers are therefore listed elsewhere according to target organ or cell and/or intracellular compartment, clinical condition and/or biological/biochemical/physiological studies.

In utero administration

Chansel-Debordeaux, L., Bourdenx, M., Dovero, S., Grouthier, V., Dutheil, N., Espana, A., Groc, L., Jimenez, C., Bezard, E. and Dehay, B. (2017) *In utero delivery of rAAV2/9 induces neuronal expression of the transgene in the brain: towards new models of Parkinson's disease* Gene Ther., **24**, 801–809
Picconi, J.L., Muff-Luett, M.A., Wu, D., Bunchman, E., Schaefer, F. et al (2014) *Kidney-specific expression of GFP by in-utero delivery of pseudotyped adeno-associated virus 9* Mol. Ther. Meth. Clin. Dev., **1:** 14014

Joint delivery (see "Arthritic joint delivery")

Keratinocyte transduction

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Lung (see "Respiratory system delivery")

Locus coeruleus delivery

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Mitochondrial defects

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Multiple sclerosis

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Muscular dystrophy (see "Skeletal muscle delivery")

Mucopolysaccharidosis (see "Lysosomal storage diseases")

Myelin basic proteins

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Nephron segment: see “Kidney delivery”

Neutralizing antibodies

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Non-human primate administration

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Nuclear interactions/translocation

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Nucleus pulposus cells

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Obesity (including leptin therapy)

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Optical dysfunction

Achromatopsia

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Ancestral rAAVs, targeting of

Zinn, E., Pacouret, S., Khaychuk, V., Turunen, H.T., Carvalho, L.S., Andres-Mateos, E., Shah, S. Shelke, R. et al (2015) *In silico reconstruction of the viral evolutionary lineage yields a potent gene therapy vector* Cell Rep., **12**, 1056–1068

Angiogenesis

Joyal, J-S., Sun, Y., Gantner, M.L., Shao, Z., Evans, L.P., Saba, N., Fredrick, T., Burnim, S., Kim, J.S., Pate, G., Juan, A.M. et al (2016) *Retinal lipid and glucose metabolism dictates angiogenesis through the lipid sensor Ffar1* Nat. Med., 22, 439-445

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AIPL1 mutations

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Axonal degeneration/growth/misguidance

Koch, J.C., Tönges, L., Barski, E., Michel, U., et al (2014) *ROCK2 is a major regulator of axonal degeneration, neuronal death and axonal regeneration in the CNS* Cell Death Dis., **5**: e1225

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Planchamp, V., Bermel, C., Tönges, L., Ostendorf, T., et al (2008) *BAG1 promotes axonal outgrowth and regeneration in vivo via Raf-1 and reduction of ROCK activity* Brain **131**, 2606-2619

Bardet-Biedl syndrome

Seo, S., Mullins, R.F., Dumitrescu, A.V., Bhattarai, S., et al (2013) *Subretinal gene therapy of mice with Bardet-Biedl syndrome type 1* Invest. Ophthalmol. Vis. Sci., **54**, 6118–6132

Bipolar cells

Cronin, T., Vandenberghe, L.H., Hantz, P., Juttner, J., Reimann, A., Kacso, A.E., Huckfeldt, R.M., Busskamp. V., Kohler, H., Lagali, P.S. et al (2014) *Efficient transduction and optogenetic stimulation of retinal bipolar cells by a synthetic adeno-associated virus capsid and promoter* EMBO Mol. Med., **6**, 1175-1190

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Ciliary neurotrophic factor

Pernet, V., Joly, S., Dalkara, D., Jordi, N., Schwarz, O., Christ, F., Schaffer, D.V., Flannery, J.G. and Schwab, M.E. (2013) *Long-distance axonal regeneration induced by CNTF gene transfer is impaired by axonal misguidance in the injured adult optic nerve* Neurobiol. Dis., **51**, 202–213

Channel rhodopsin 2

Sengupta, A., Chaffiol, A., Macé, E., Caplette, R., Desrosiers, M., Lampič, M., Forster, V., Marre, O. Lin, J.Y. et al (2016) *Red-shifted channel rhodopsin stimulation restores light responses in blind mice, macaque retina, and human retina* EMBO Mol. Med., **8**, 148-1264

Choroideremia

Patrício, M.I., Barnard, A.R., Cox, C.I., Blue, C. and MacLaren, R.E. (2018) *The biological activity of AAV vectors for choroideremia gene therapy can be measured by in vitro prenylation of RAB6A* Mol. Ther. Meth. Clin. Dev., **9**, 288-295

Cone vision/opsins/promoters/viability factor

Li, Q., Timmers, A.M., Guy, J., Pang, J. and Hauswirth, W.W. (2008) *Cone-specific expression using a human red opsin promoter in recombinant AAV* Vision Res., **48**, 332-338

Masseck, O.A., Spoida, K., Dalkara, D., Maejima, T., et al (2014) *Vertebrate cone opsins enable sustained and highly sensitive rapid control of Gi/o signaling in anxiety circuitry* Neuron **81**, 1263–1273

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Najate, A., Fridlich, R., Millet-Pue, G., Clerin, E., Delalande, F., Jaillard, C., Blond, F., Perrocheau, L., Reichman, S. et al (2015) *Rod-derived cone viability factor promotes cone survival by stimulating aerobic glycolysis* Cell, **161**, 817–832
Szikra, T., Trenholm, S., Drinnenberg, A., Jüttner, J., Raics, Z., Farrow, K., Biel, M. et al (2014) *Rods in daylight act as relay cells for cone-driven horizontal cell–mediated surround inhibition* Nat. Neurosci., **17, 1728-1735**
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Corneal disorders/transduction

Gruenert, A.K., Czugala, M., Mueller, C., Schmeer, M., Schleef, M., Kruse, F.E. and Fuchsluger, T.A. (2016) *Self-complementary adeno-associated virus vectors improve transduction efficiency of corneal endothelial cells* PLoS One, **11**: e0152589
Hippert, C., Ibanes, S., Serratrice, N., Court, F., et al V. (2012) *Corneal transduction by intra-stromal injection of AAV vectors in vivo in the mouse and ex vivo in human explants* PLoS One **7**: e35318
Mohan, R.R., Sinha, S., Tandon, A., Gupta, R., et al (2011) *Efficacious and safe tissue-selective controlled gene therapy approaches for the cornea* PloS One, **6**: e18771
Mohan, R.R., Tandon, A., Sharma, A., Cowden, J.W. et al (2011) *Significant inhibition of corneal scarring in vivo with tissue-selective, targeted AAV5 decorin gene therapy* Invest. Ophthalmol. Vis. Sci., **52**, 4833–4841
Mohan, R.R., Tovey, J.C.K., Sharma, A., Schultz, G.S., et al (2011) *Targeted decorin gene therapy delivered with adeno-associated virus effectively retards corneal neovascularization in vivo* PLoS One **6**: e26432
Pinto, B.S., Saxena, T., Oliveira, R., Méndez-Gómez, H.R., Cleary, J.D., Denes, L.T., McConnell, O. et al (2017) *Impeding transcription of expanded microsatellite repeats by deactivated Cas9* Mol. Cell **68**, 479-490

Delivery procedures (cells, serotypes, tissues)

Boye, S.L., Bennett, A., Scalabrino, M.L., McCullough, K.T., Van Vliet, K., Choudhury, S., Ruan, Q., Peterson, J., Agbandje-McKenna, M. and Boye, S.E. (2016) *Impact of heparan sulfate binding on transduction of retina by recombinant adeno-associated virus vectors* J. Virol., **90**, 4215-4231
Buck, T.M., Pellissier, L.P., Vos, R.M., van Dijk E.H.C., Boon, C.J.F. and Wijnholds, J. (2018) *AAV serotype testing on cultured human donor retinal explants* In Retinal Gene Therapy: Methods and Protocols, Methods in Molecular Biology, **vol. 1715** (eds. Boon, C.J.F. and Wijnholds, J.) Springer Science+Business Media, LLC New York 275-288
Buie, L.K., Rasmussen, C.A., Porterfield, E.C., Ramgolam, V.S., (2010) *Self-complementary AAV virus (scAAV) safe and long-term gene transfer in the trabecular meshwork of living rats and monkeys* Invest. Ophthalmol. Vis. Sci., **51**, 236–248
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Intracardiac administration (scAAV2/9 and scAAV2/rh10 variants)

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Myocardial dysfunction (incl. fibrosis/heart failure/hypertension/infarction etc)

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Pericardial cavity delivery (see "Actin filaments")

Phospholamban suppression
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Vestibular hair cells (see "Inner ear delivery" and "Cochlear delivery")

Vexosome (exosome) gene delivery

György, B., Fitzpatrick, Z., Crommentuijn, M.H.W., Mu, D. and Maguire, C.A. (2014) *Naturally enveloped AAV vectors for shielding neutralizing antibodies and robust gene delivery in vivo* Biomaterials, **35**, 7598-7609
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PART B

Anneloviridae

Torque teno virus

Bzhalava, D., Ekströmb, J., Lysholm, F., Hultin, E., Faust, H., Persson, B., Lehtinen, M., Villiers, E-M. and Dillner, J. (2012) *Phylogenetically diverse TT virus viremia among pregnant women* Virology, **432**, 427–434

Dependovirus

Avian adeno-associated virus

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Parvoviridae

Densovirinae (Penaeus stylirostris)

Kaufmann, B., Bowman, V.D., Li, Y., Szelei, J., Waddell, P.J., Tijssen, P. and Rossmann, M.G. (2010) *Structure of Penaeus stylirostris densovirus, a shrimp pathogen* J. Virol., **84**, 11289-11296

Parvovirinae

Bocavirus

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Minute virus (of mice)

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