

## Publications

Listed below are citations for each publication. If you would like a copy of any of the publications, please contact the GTRP Clinical Coordinating Center ([gtrpccc@s-3.com](mailto:gtrpccc@s-3.com)).

### 2022

**Hepatocyte Rap1a contributes to obesity- and statin-associated hyperglycemia.** Wang Y, Spolitu S, Zadroga JA, Sarecha AK, Ozcan L. Cell Reports 2022 Aug 23;40(8)

### 2020

**Signalosome-Regulated Serum Response Factor Phosphorylation Determining Myocyte Growth in Width Versus Length as a Therapeutic Target for Heart Failure.** Li J, Tan Y, Passariello CL, Martinez EC, Kritzer MD, Li X, Li X, Li Y, Yu Q, Ohgi K, Thakur H, MacArthur Jr JW, Ivey JR, Woo YJ, Emter CA, Dodge-Kafka K, Rosenfeld MG, Kapiloff MS. Circulation 2020 Dec;142(22):2138-2154

**Calcineurin A $\beta$ -Specific Anchoring Confers Isoform-Specific Compartmentation and Function in Pathological Cardiac Myocyte Hypertrophy.** Li X, Li J, Martinez EC, Froese A, Passariello CL, Henshaw K, Rusconi F, Li Y, Yu Q, Thakur H, Nikolaev VO, Kapiloff MS. Circulation 2020 Sep 8;142(10): 948-962

**Systemic AAV9.LAMP2B injection reverses metabolic and physiologic multiorgan dysfunction in a murine model of Danon disease.** Manso AM, Hashem SI, Nelson BC, Gault E, Soto-Hermida A, Villarruel E, Brambatti M, Bogomolovas J, Bushway PJ, Chen C, Battiprolu P, Keravala A, Schwartz JD, Shah G, Gu Y, Dalton ND, Hammond K, Peterson K, Saftig P, Adler ED. Science Translational Medicine 2020 Mar 18;12(535)

**Lentiviral gene therapy for X-linked chronic granulomatous disease.** Kohn DB, Booth C, Kang EM, Pai SY, Shaw KL, Santilli G, Armant M, Buckland KF, Choi U, De Ravin SS, Dorsey MJ, Kuo CY, Leon-Rico D, Rivat C, Izotova N, Gilmour K, Snell K, Dip JX, Darwish J, Morris EC, Terrazas D, Wang LD, Bauser CA, Paprotka T, Kuhns DB, Gregg J, Raymond HE, Everett JK, Honnet G, Biasco L, Newburger PE, Bushman FD, Grez M, Gaspar HB, Williams DA, Malech HL, Galy A, Thrasher AJ, Net3CGD Consortium. Nature Medicine, 2020 Feb;26(2): 200-206

### 2019

**Non-immunogenic Utrophin Gene Therapy for the Treatment of Muscular Dystrophy Animal Models.** Song Y, Morales L, Malik AS, Mead AF, Greer CD, Mitchell MA, Petrov MT, Su LT, Choi ME, Roseblum ST, Lu X, VanBelzen DJ, Krishnakutty RK, Balzer FJ, Loro E, French R, Propert KJ, Zhou S, Kozyak BW, Nghiem PP, Khurana TS, Kornegay JN, Stedman HH. Nature Medicine, 25, 1505-1511 (2019)

**Osteopontin Promotes Left Ventricular Diastolic Dysfunction Through a Mitochondrial Pathway.** Yousefi K, Irion CI, Takeuchi LM, Ding W, Lambert G, Eisenberg T, Sukkar S, Granzier HL, Methawasin M, Lee DI, Hahn VS, Kass DA, Hatzistergos KE, Hare JM, Webster KA, Shehadeh LA. Journal of American College of Cardiology. 2019 Jun 4;73(21): 2705-2718

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### 2018

**Retinal AAV8-RS1 Gene Therapy for X-Linked Retinoschisis: Initial Findings from a Phase I/IIa Trial by Intravitreal Delivery.** Cukras C, Wiley HE, Jeffrey BG, Sen HN, Turriff A, Zeng Y, Vijayasathay C, Marangoni D, Ziccardi L, Kjellstrom S, Park TK, Hiriyanna S, Wright JF, Colosi P, Wu Z, Bush RA, Wei LL, Sieving PA. *Molecular Therapy*. 2018 Sep 5;26(9): 2282-2294

**Glucose promotes cell growth by suppressing branched-chain amino acid degradation.** Shao D, Villet O, Zhang Z, Choi SW, Yan J, Ritterhoff J, Gu H, Djukovic D, Christodoulou D, Kolwicz SC Jr, Raftery D, Tian R. *Nature Communications*. 2018 Jul 26;9(1): 2935

**Hypercholesterolemia induces T-cell expansion in humanized immune mice.** Proto JD, Doran AC, Subramanian M, Wang H, Zhang M, Sozen E, Rymond CC, Kuriakose G, D'Agati V, Winchester R, Sykes M, Yang YG, Tabas I. *Journal of Clinical Investigation*. 2018 Jun 1;128(6):2370-2375.

### 2017

**Safety of Intradiaphragmatic Delivery of Adeno-Associated Virus-Mediated Alpha-Glucosidase (rAAV1-CMV-hGAA) Gene Therapy in Children Affected by Pompe Disease.** Corti M, Liberati C, Smith BK, Lawson LA, Tuna IS, Conlon TJ, Coleman KE, Islam S, Herzog RW, Fuller DD, Collins SW, Byrne BJ. *Human Gene Therapy Clinical Development*. 2017 Dec;28(4): 208-218

**A Preclinical Study in Rhesus Macaques for Cystic Fibrosis to Assess Gene Transfer and Transduction by AAV1 and AAV5 with a Dual-Luciferase Reporter System.** Guggino WB, Benson J, Seagrave J, Yan Z, Engelhardt J, Gao G, Conlon TJ, Cebotaru L. *Human Gene Therapy Clinical Development*. 2017 Sep;28(3): 145-156

**Use of Adeno-Associated Virus Vector for Cardiac Gene Delivery in Large-Animal Surgical Models of Heart Failure.** Katz MG, Fargnoli AS, Weber T, Hajjar RJ, Bridges CR. *Human Gene Therapy Clinical Development*. 2017 Sep;28(3): 157-164

**Motorneuron Glutamatergic Receptor Expression Following Recovery from Cervical Spinal Hemisection.** Gransee HM, Gonzalez-Porrás MA, Zhan WZ, Sieck GC, Mantilla CB. *Journal of Comparative Neurology*. 2017 Apr 1 525(5): 1192-1205

**Spacial and Temporal Patterns of Gene Expression After Cardiac MEK1 Gene Transfer Improve Post-Infarction Remodeling without Inducing Global Hypertrophy.** Fan Y, Yang YL, Yeh CC, Mann MJ. *Journal Cellular Biochemistry*. 2017 Apr; 118(4): 775-784

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#### **2017 cont'd**

**Inspiratory Muscle Conditioning Exercise and Diaphragm Gene Therapy in Pompe Disease: Clinical Evidence of Respiratory Plasticity.** Smith BK, Martin AD, Lawson LA, Vernot V, Marcus J, Islam S, Shafi N, Corti M, Collins SW, Byrne BJ. *Experimental Neurology*. 287 (2017) 216-224

**Current Methods in Cardiac Gene Therapy: Overview.** Ishikawa K and Hajjar RJ. *Methods in Molecular Biology*. 2017;1521: 3-14

**Generation of Efficient miRNA Inhibitors Using Tough Decoy Constructs.** Yoo J, Hajjar RJ, Jeong D. *Methods in Molecular Biology*. 2017;1521: 41-53

**Gene Transfer in Cardiomyocytes Derived from ES and iPS Cells.** Stillitano F, Karakikes I, Hajjar RJ. *Methods in Molecular Biology*. 2017;1521: 183-193

**Gene Transfer to Rodent Hearts In Vivo.** Del Monte F, Ishikawa K, Hajjar RJ. *Methods in Molecular Biology*. 2017;1521: 195-204

**Cardiac Gene Delivery in Large Animal Models: Antegrade Techniques.** Watanabe S, Leonardson L, Hajjar RJ, Ishikawa K. *Methods in Molecular Biology*. 2017;1521: 227-235

**Direct Myocardial Injection of Vectors.** Bonnet G, Ishikawa K, Hajjar RJ, Kawase Y. *Methods in Molecular Biology*. 2017;1521: 273-248

#### **2016**

**Smooth muscle-generated methylglyoxal impairs endothelial cell-mediated vasodilatation of cerebral microvessels in type 1 diabetic rats.** Alomar F, Singh J, Jang HS, Rozanzki GJ, Shao CH, Padanilam BJ, Mayhan WG, Bidasee KR. *British Journal of Pharmacology*. 2016 Dec;173(23): 3307-3326

**One-time injection of AAV8 encoding urocortin 2 provides long-term resolution of insulin resistance.** Gao MH, Giamouridis D, Lai NC, Walenta E, Paschoal VA, Kim YC, Miyanochara A, Guo T, Liao M, Liu L, Tan Z, Ciaraldi TP, Schenk S, Bhargava A, Oh DY, Hammond HK. *Journal Clinical Investigation Insight*. 2016 Sep 22;1(15)

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### 2016 cont'd

**Loss of Function of GALNT2 Lowers High-Density Lipoproteins in Humans, Nonhuman Primates, and Rodents.** Khetarpal SA, Schjoldager KT, Christoffersen C, Raghavan A, Edmondson AC, Reutter HM, Ahmed B, Ouazzani R, Peloso GM, Vitali C, Zhao W, Somasundara AV, Millar JS, Park Y, Fernando G, Livanov V, Choi S, Noé E, Patel P, Ho SP; Myocardial Infarction Exome Sequencing Study, Kirchgessner TG, Wandall HH, Hansen L, Bennett EP, Vakhrushev SY, Saleheen D, Kathiresan S, Brown CD, Abou Jamra R, LeGuern E, Clausen H, Rader DJ. *Cell Metabolism*. 2016 Aug 9;24(2): 234-45

**Vascular Regeneration in Ischemic Hindlimb by Adeno-Associated Virus Expressing Conditionally Silenced Vascular Endothelial Growth Factor.** Boden J, Lassance-Soares RM, Wang H, Wei Y, Spiga MG, Adi J, Layman H, Yu H, Vazquez-Padron RI, Andreopoulos F, Webster KA. *Journal of American Heart Association*. 2016 May 26;5(6)

**Cardiac *Stim1* Silencing Impairs Adaptive Hypertrophy and Promotes Heart Failure Through Inactivation of mTORC2/Akt Signaling.** Benard L, Gyun J, Cacheux M, Lee A, Nonnenmacher M, Matasic DS, Kohlbrenner E, Kho C, Pavoine C, Hajjar RJ, Hulot JB. *Circulation*. 2016;133: 1458-1471

**Intracoronary Gene Transfer of Adenylyl Cyclase 6 in Patients with Heart Failure: A Randomized Clinical Trial.** Hammond HK, Penny WF, Traverse JH, Henry TD, Watkins MW, Yancy CW, Swels RN, Adler ED, Patel AN, Murray DR, Ross RS, Bhargava V, Maisel A, Barnard DD, Lai NC, Dalton ND, Lee ML, Narayan SM, Blanchard DG, Gao MH. *JAMA Cardiology*. Online March 30, 2016.

**Megakaryocyte- and megakaryocyte precursor-related gene therapies.** Wilcox DA. *Blood* March 10 2016;127(10): 1260-1268

**Safety and biodistribution assessment of sc-rAAV2.5IL-1Ra administered via intra-articular injection in a mono-iodoacetate-induced osteoarthritis rat model.** Wang G, Evans CH, Benson JM, Hutt JA, Seagrave JC, Wilder JA, Grieger JC, Samulski RA, Terse PS. *Molecular Therapy – Methods & Clinical Development* 2016 Jan 13;3:15052

### 2015

**TrkB gene therapy by adeno-associated virus enhances recovery after cervical spinal cord injury.** Martinez-Galvez G, Zambrano JM, Diaz Soto JC, Zhan WZ, Gransee HM, Sieck GC, Mantilla CB. *Experimental Neurology* 2015 Dec 1; 276:31-40

**Safe and bodywide muscle transduction in young adult Duchenne muscular dystrophy dogs with adeno-associated virus.** Yue Y, Pan X, Hakim CH, Kodippili K, Zhang K, Shin JH, Yang HT, McDonald T, Duan D. *Human Molecular Genetics* 2015 Oct 15;24(20):5880-90

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### 2015 cont'd

**Delivery of Adeno-Associated Virus Gene Therapy by Intravascular Limb Infusion Methods.** Gruntman AM and Flotte TR. Human Gene Therapy Clinical Development. 2015 Sep;26(3): 159-64

**Evaluation of Readministration of a Recombinant Adeno-Associated Virus Vector Expressing Acid Alpha-Glucosidase in Pompe Disease: Preclinical to Clinical Planning.** Corti M, Cleaver B, Clément N, Conlon TJ, Faris KJ, Wang G, Benson J, Tarantal AF, Fuller D, Herzog RW, Byrne BJ. Human Gene Therapy Clinical Development 2015 Sep;26(3): 185-93

**Single and Compound Knock-outs of MicroRNA (miRNA)-155 and Its Angiogenic Gene Target *CCN1* in Mice Alter Vascular and Neovascular Growth in the Retina via Resident Microglia.** Yan L, Lee S, Lazzaro DR, Aranda J, Grant MB, Chaqour B. Journal of Biological Chemistry 2015 Sep 18;290(38):23264-81

**Progress with Recombinant Adeno-Associated Virus Vectors for Gene Therapy of Alpha-1 Antitrypsin Deficiency.** Gruntman AM, Flotte TR. Human Gene Therapy Methods. 2015 Jun;26(3):77-81

### 2014

**A needleless liquid jet injection delivery method for cardiac gene therapy: a comparative evaluation versus standard routes of delivery reveals enhanced therapeutic retention and cardiac specific gene expression.** Fagnoli AS, Katz MG, Williams RD, Margulies KB, Bridges CR. Journal of Cardiovascular Translational Research 2014 Nov;7(8): 756-67

**The Role of SUMO-1 in Cardiac Oxidative Stress and Hypertrophy.** Lee A, Jeong D, Mitsuyama S, Oh JG, Liang L, Ikeda Y, Sadoshima J, Hajjar R, Kho C. Antioxidants & Redox Signaling 2014 Nov 10;21(14): 1986-2001

**Developmental stage determines efficiency of gene transfer to muscle satellite cells by *in utero* delivery of adeno-associated virus vector serotype 2/9.** Stitelman D, Brazelton T, Bora A, Traas J, Joyeux L, Merianos D, Limberis MP, Davey MG, Flake AW. Molecular Therapy – Methods & Clinical Development (2014) 1, 14040

**Safety and efficacy of high-dose adeno-associated virus 9 encoding sarcoplasmic reticulum Ca (2+) adenosine triphosphatase delivered by molecular cardiac surgery with recirculating delivery in ovine ischemic cardiomyopathy.** Katz MG, Fagnoli AS, Williams RD, Steuerwald NM, Isidro A, Ivanina AV, Sokolova IM, Bridges CR. Journal of Thoracic Cardiovascular Surgery. 2014 Sep;148(3):1056-1073

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### **2014 cont'd**

**Phase I/II Trial of Diaphragm Delivery of Recombinant Adeno-Associated Virus Acid Alpha-Glucosidase (rAAV1-CMV-GAA) Gene Vector in Patients with Pompe Disease.** Byrne BJ, Smith B, Mah C, Lawson LA, Islam S, Corti M, Martin D, Dobija N, Irwin MV, Conlon T, Cleaver B, Clement N, Collins SW. *Human Gene Therapy Clinical Development* 25: 134-163 (September 2014)

**B-cell depletion is protective against anti-AAV capsid immune response: a human subject case study.** Corti M, Elder ME, Falk DJ, Lawson L, Smith BK, Nayak S, Conlon TJ, Clement N, Erger K, Lavassani E, Green MM, Doerfler PA, Herzog RW, Byrne BJ. *Molecular Therapy – Methods and Clinical Development* (2014) 1, 14033

**In Utero Lung Gene Transfer Using Adeno-Associated Viral and Lentiviral Vectors in Mice.** Joyeux L, Danzer E, Limberis MP, Zoltick PW, Radu A, Flake AW, Davey MG. *Human Gene Therapy Methods* 25:197-205 (June 2014)

**Assessment of toxicity and biodistribution of recombinant AAV8 vector-mediated immunomodulatory gene therapy in mice with Pompe disease.** Wang G, Young SP, Bali D, Hutt J, Li S, Benson J, Koeberl DD. *Molecular Therapy – Method & Clinical Development*. 2014 June 11: 1-10

**Attenuation of Monocrotaline-Induced Pulmonary Hypertension by Luminal Adeno-Associated Virus Serotype 9 Gene Transfer of Prostacyclin Synthase.** Gubrij IB, Martin SR, Pangle AK, Kurten R, Johnson LG. *Human Gene Therapy*. 2014.Jun25(6):498-505

**Targeted Delivery of TrkB Receptor to Phrenic Motorneurons Enhances Functional Recovery of Rythmic Phrenic Activity After Cervical Spinal Hemisection.** Gransee HM, Zhan WZ, Sieck GC, Mantilla CB. *PLoS One*. 2014 May 28;8(5):1-10

**Inhibition of miR-25 improves cardiac contractility in the failing heart.** Wahlquist C, Jeong D, Rojas-Muñoz A, Kho C, Lee A, Mitsuyama S, van Mil A, Park WJ, Sluijter JP, Doevendans PA, Hajjar RJ, Mercola M. *Nature*. 2014 Apr 24;508(7497):531-5

**Preclinical Demonstration of Lentiviral Vector-mediated Correction of Immunological and Metabolic Abnormalities in Models of Adenosine Deaminase Deficiency.** Carbonaro DA, Zhang L, Jin X, Montiel-Equihua C, Geiger S, Carmo M, Cooper A, Fairbanks L, Kaufman ML, Sebire NJ, Hollis RP, Blundell MP, Senadheera S, Fu P, Sahaghian A, Chan RY, Wang X, Cornetta K, Thrasher AJ, Kohn DB, Gaspar HB. *Molecular Therapy*. 2014 Mar; 22(3):607-22

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### 2014 cont'd

**What is suppression of anti-Adeno-associated virus capsid T-cells achieving?** Flotte TR, Mueller C. Human Gene Therapy. 2014 Mar;25(3):178-9

### 2013

**Intraleural Administration of an AAVrh.10 Vector Coding for Human  $\alpha$ 1-Antitrypsin for the Treatment of  $\alpha$ 1-Antitrypsin Deficiency.** Chiuchiolo MJ, Kaminsky SM, Sondhi D, Hackett NR, Rosenberg JB, Frenk EZ, Hwang Y, Van de Graaf BG, Hutt JA, Wang G, Benson J, Crystal RG. Human Gene Therapy Clinical Development. 2013 Dec;24(4):161-73

**Human Treg responses allow sustained recombinant Adeno-associated virus-mediated transgene expression.** Mueller C, Chulay JD, Trapnell BC, Humphries M, Carey B, Sandhaus RA, McElvaney NG, Messina L, Tang Q, Rouhani FN, Campbell-Thompson M, Fu AD, Yachnis A, Knop DR, Ye GJ, Brantly M, Calcedo R, Somanathan S, Richman LP, Vonderheidi RH, Hulme MA, Brusko TM, Wilson JM, Flotte TR. Journal of Clinical Investigation. 2013 Dec;123(12):5310-8

**SUMO-1 gene transfer improves cardiac function in a large-animal model of heart failure.** Tilemann L, Lee A, Ishikawa K, Aguero J, Rapti K, Santos-Gallego C, Kohlbrenner E, Fish KM, Kho C, Hajjar RJ. Science Translational Medicine. 2013 Nov 3;5(211):211ra159

**Retinol-binding protein 4 and its membrane receptor STRA6 control adipogenesis by regulating cellular retinoid homeostasis and retinoic acid receptor  $\alpha$  activity.** Muenzner M, Tuvia N, Deutschmann C, Witte N, Tolkachov A, Valai A, Henze A, Sander LE, Raila J, Schupp M. Molecular and Cellular Biology. 2013 Oct;33(20):4068-82

**Adeno-associated virus serotype 9 efficiently targets ischemic skeletal muscle following systemic delivery.** Katwal AB, Konkalmatt PR, Piras BA, Hazarika S, Li SS, Lye R, John R, Sanders JM, Ferrante EA, Yan Z, Annex BH, French BA. Gene Therapy. 2013 Sep;20(9):930-8

**Cardiac surgical delivery of the sarcoplasmic reticulum calcium ATPase rescues myocytes in ischemic heart failure.** Fargnoli AS, Katz MG, Yarnall C, Isidro A, Petrov M, Steuerwald N, Ghosh S, Richardville KC, Hillesheim R, Williams RD, Kohlbrenner E, Stedman HH, Hajjar RJ, Bridges CR. Annals of Thoracic Surgery. 2013 Aug;96(2):586-95.

**Phase I/II Trial of Adeno-Associated Virus-Mediated Alpha-Glucosidase Gene Therapy to the Diaphragm for Chronic Respiratory Failure in Pompe Disease: Initial Safety and Ventilatory Outcomes.** Smith BK, Collins SW, Conlon TJ, Mah CS, Lawson LA, Martin AD, Fuller DD, Cleaver BD, Clément N, Phillips D, Islam S, Dobjia N, Byrne BJ. Human Gene Therapy. 2013 Jun;24(6):630-40

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### 2013 cont'd

**Microdystrophin ameliorates muscular dystrophy in the canine model of Duchenne Muscular Dystrophy.** Shin JH, Pan X, Hakim CH, Yang HT, Yue Y, Zhang K, Terjung RL, Duan D. *Molecular Therapy*. 2013 Apr;21(4):750-57

**Molecular mechanisms responsible for the differential effects of ApoE3 and ApoE4 on plasma lipoprotein – cholesterol levels.** Li H, Dhanasekaran P, Alexander ET, Rader DJ, Phillips MC, Lund-Katz S. *Arteriosclerosis, Thrombosis, and Vascular Biology*. 2013 Apr;33(4):687-93

### 2012

**Lentiviral Vector Gene Transfer to Porcine Airways.** Sinn PL, Cooney AL, Oakland M, Dylla DE, Wallen TJ, Pezzulo AA, Chang EH, McCray PB Jr. *Molecular Therapy-Nucleic Acids*. 2012 Nov 27

**Connective tissues growth factor regulates retinal neovascularization through p53 protein-dependent transactivation of the Matrix Metalloproteinase (MMP)-2.** Chintala H, Liu H, Parmar R, Kamalska M, Kim YJ, Lovett D, Grant MB, Chaqour B. *Journal of Biological Chemistry*. 2012 Nov 23;287(48):40570-85

**Hepatic sortilin regulates both apolipoprotein B secretion and LDL catabolism.** Strong A, Ding Q, Edmondson AC, Millar JS, Sachs KV, Li X, Kumaravel A, Wang MY, Ding A, Guo L, Alexander ET, Nguyen D, Lund-Katz S, Phillips MC, Morales CR, Tall AR, Kathiresan S, Fisher EA, Musunuru K, Rader DJ. *Journal of Clinical Investigation*. 2012 August 1;122(8); 2807-2816

**Successful transfection of genes using AAV-2/9 vector in swine coronary and peripheral arteries.** Pankajakshan D, Makinde TO, Gaurav R, Del Core M, Hatzoudis G, Pipinos I, Agrawal DK. *Journal of Surgical Research*. 2012 Jun 1;175(1):169-175

**Characterization of an HIV-targeted transcriptional gene-silencing RNA in primary cells.** Turner AMW, Ackley AM, Matrone MA, Morris KV. *Human Gene Therapy*. 2012 May;23:473-483

**An AAV2/5 vector enhances safety of gene transfer to the mouse salivary gland.** Geguchadze RN, Machen L, Zourelas L, Gallo PH, Passineau MJ. *Journal of Dental Research*. 2012 Apr;91(4):382-6

**Humoral immunity to AAV-6, 8, and 9 in normal and dystrophic dogs.** Shin JH, Yue Y, Smith B, Duan D. *Human Gene Therapy*. 2012 March;23:287-294

**Analysis of particle content of recombinant adeno-associated virus serotype 8 vectors by ion-exchange chromatography.** Lock M, Alvira MR, Wilson JM. *Human Gene Therapy Methods*. 2012 Feb;23(1):56-64

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### 2012 cont'd

**A simplified immune suppression scheme leads to persistent micro-dystrophin expression in Duchenne Muscular Dystrophy dogs.** Shin JH, Yue Y, Srivastava A, Smith B, Duan D. Human Gene Therapy. 2012 Feb;23:202-209

**The effects of apolipoprotein F deficiency on high density lipoprotein cholesterol metabolism in mice.** Lagor WR, Fields DW, Khetarpal SA, Kumaravel A, Lin W, Weintraub N, Wu K, Hamm-Alvarez SF, Drazul-Schrader D, de la Llera-Moya M, Rothblat GH, Rader DJ. PLoS ONE. 2012 Feb;7(2):e31616

**Developing novel lentiviral vectors into clinical products.** Leath A and Cornetta K. Methods in Enzymology. 2012;507:89-108

### 2011

**Phase 2 clinical trial of a recombinant adeno-associated viral vector expressing  $\alpha$ 1-antitrypsin: interim results.** Flotte TR, Trapnell BC, Humphries M, Carey B, Calcedo R, Rouhani F, Campbell-Thompson M, Yachnis AT, Sandhaus RA, McElvaney NG, Mueller C, Messina LM, Wilson JM, Brantly M, Knop DR, Ye GJ, Chulay JD. Human Gene Therapy. 2011 Oct;22(10):1239-47

**Myocardial gene delivery using molecular cardiac surgery with recombinant adeno-associated virus vectors in vivo.** White JD, Thesier DM, Swain JBD, Katz MG, Tomasulo C, Henderson A, Wang L, Yarnall C, Fargnoli A, Sumaroka M, Isidro A, Petrov M, Holt D, Nolen-Walston R, Koch WJ, Stedman HH, Rabinowitz J, Bridges CR. Gene Therapy (2011) 18; 546-552

**PEG-modulated column chromatography for purification of recombinant adeno-associated virus serotype 9.** Zhou J, Yang X, Wright JF, High KA, Couto L, Qu G. Journal of Virology Methods. 2011 Apr;173(1):99-107.

**The matricellular protein cysteine-rich protein 61 (CCN1/Cyr61) enhances physiological adaptation of retinal vessels and reduces pathological neovascularization associated with ischemic retinopathy.** Hasan A, Pokeza N, Shaw L, Lee HS, Lazzaro D, Chintala H, Rosenbaum D, Grant MB, Chaqour B. Journal of Biological Chemistry. March 18, 2011;286(11):9542-9554

**Preclinical evaluation of a recombinant adeno-associated virus vector expressing human alpha-1 antitrypsin made using a recombinant herpes simplex virus production method.** Chulay JD, Ye GJ, Thomas DL, Knop DR, Benson JM, Hutt JA, Wang G, Humphries M, Flotte TR. Human Gene Therapy. 2011 Feb;22:155-165

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### 2011 cont'd

**Vector characterization methods for quality control testing of recombinant adeno-associated viruses.**

Wright JF and Zelenia O. *Methods in Molecular Biology*. 2011;737:247-78

### 2010

**Gene therapy during cardiac surgery: role of surgical technique to minimize collateral organ gene expression.**

Katz MG, Swain JD, Fagnoli AS, Bridges CR. *Interactive Cardiovascular and Thoracic Surgery*. 2010 Dec;11(6):727-31

**Trib1 is a lipid- and myocardial infarction-associated gene that regulates hepatic lipogenesis and VLDL production in mice.**

Burkhardt R, Toh SA, Lagor WR, Birkeland A, Levin M, Li X, Robblee M, Fedorov VD, Yamamoto M, Satoh T, Akira S, Kathiresan S, Breslow JL, Rader DJ. *The Journal of Clinical Investigation*. 2010;120(12):4410-4414

**Specific disruption of astrocytic Ca<sup>2+</sup> signaling pathway in vivo by adeno-associated viral transduction.**

Xie Y, Wang T, Sun GY, Ding S. *Neuroscience*. 2010 Nov 10;170(4):992-1003

**Rapid, simple, and versatile manufacturing of recombinant Adeno-associated viral vectors at scale.**

Lock M, Alvira M, Vandenberghe LH, Samanta A, Toelen J, Debyser Z, Wilson JM. *Human Gene Therapy*. 2010 Oct;21(10):1259-1271

**Efficient serotype-dependent release of functional vector into the culture medium during Adeno-associated virus manufacturing.**

Vandenberghe LH, Xiao R, Lock M, Lin J, Korn M, Wilson JM. *Human Gene Therapy*. 2010 Oct;21(10):1251-1257

**From noncoding variant to phenotype via SORT1 at the 1p13 cholesterol locus.**

Musunuru K, Strong A, Frank-Kamenetsky M, Lee NE, Ahfeldt T, Sachs KV, Li X, Li H, Kuperwasser N, Ruda VM, Pirruccello JJ, Muchmore B, Prokunina-Olsson L, Hall JL, Schadt EE, Morales CR, Lund-Katz S, Phillips MC, Wong J, Cantley W, Racie T, Ejebe KG, Orho-Melander M, Melander O, Kotliansky V, Fitzgerald K, Krauss RM, Cowan CA, Kathiresan S, Rader DJ. *Nature*. 2010 Aug 5; 466(7307):714-9

### 2009

**Synergistic effect of HIF-1 $\alpha$  gene therapy and HIF-1-activated bone marrow-derived angiogenic cells in a mouse model of limb ischemia.**

Rey S, Lee K, Wang CJ, Gupta K, Chen S, McMillan A, Bhise N, Levchenko A, Semenza GL. *Proceedings of the National Academy of Sciences USA*. 2009 Dec 1;106(48):20399-404

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#### **2009 cont'd**

**Liver-specific deficiency of serine palmitoyltransferase (SPT) subunit 2 decreases plasma sphingomyelin and increases Apolipoprotein E levels.** Li Z, Li Y, Chakraborty M, Fan Y, Bui HH, Peake DA, Kuo MS, Xiao X, Cao G, Jiang XC. *Journal of Biological Chemistry*. 2009 Sep 25;284(39):27010-9

**Overexpression of Apolipoprotein F reduces HDL cholesterol levels in vivo.** Lagor WR, Brown RJ, Toh S, Millar JS, Fuki IV, de la Llera-Moya M, Yuen T, Rothblat G, Billheimer JT, Rader DJ. *Arteriosclerosis, Thrombosis, and Vascular Biology*. 2009 Jan 29(1):40-6

## Presentations

Listed below are citations for each presentation. If you would like a copy of any of the presentations, please contact the GTRP Clinical Coordinating Center ([gtrpccc@s-3.com](mailto:gtrpccc@s-3.com)).

### 2022

**Preclinical Development of Gene Therapy for Post-operative Atrial Fibrillation.** Donahue JK. American Society of Gene and Cell Therapy Annual Meeting, May 2022 (slides)

### 2021

**A New Mouse Model of Myocardial Lipids, Fibrosis, Arrhythmia and Diastolic Dysfunction Induced by Hyperlipidemia and Cardiac LDLR.** Williams M, Iron C, Condor J, Lambert G, Seo G, Goldberger J, Shehadeh L. American Heart Association Basic Cardiovascular Scientific Session in August 2021. (slides)

### 2019

**Outcome of Hematopoietic Stem Cell Gene Therapy for Wiskott-Aldrich Syndrome.** Labrosse R, Chu J, Armant MA, van der Spek J, Miggelbrink A, Fong J, Everett JK, Raymond H, Kessler L, Dansereau C, Mackinnon B, Koo S, Morris E, London WB, Ozen A, Baris S, Despotovic JM, Forbes L, Saitoh A, Takachi T, King A, Nguyen T, Vu V, Bushman FD, Galy A, Notarangelo LD, Williams DA, Pai SY. American Society of Hematology Annual Meeting, December 2019 (poster)

**Intratracheal AAV1.SERCA2a Gene Therapy Interrupts the Formation of Plexiform Lesions and Rescues from Heart Failure in Severe Pulmonary Hypertension.** Fargnoli AS. Heart Failure Society of America, 2019 September. (slides)

**Osteopontin Regulates Adult Cardiomyocyte Division in a Mouse Model of Pressure Overload Induced Heart Failure.** Irion CI, John-William K, Chahdi A, Yousefi K, Fernandez YR, Hatzistergos KE, Hare JM, Webster K, Shehadeh LA. Basic Cardiovascular Scientific Sessions 2019 Basic Cardiovascular Sciences Scientific Sessions, July 29–August 1, 2019, Boston, MA. (slides)

**AAV9.LAMP-2B Reverses Metabolic and Physiologic Multiorgan Dysfunction in a Murine Model of Danon Disease.** Manso AM, Hashem S, Nelson B, Gault E, Villarruel E, Soto-Hermida A, Battiprolu P, Keravala A, Schwartz JD, Shah G, Gu Y, Dalton ND, Hammond K, Peterson K, Adler ED. American College of Cardiology, March 18, 2019 (poster)

**Effective Lentiviral Gene Therapy for X-Linked Chronic Granulomatous Disease (X-CGD).** Malech HM, Booth C, Kang EM, Pai S-Y, Shaw KL, Santilli G, Armant M, Buckland KF, Choi U, De Ravin SS, Dorsey MJ, Kuo CY, Leon-Rico D, Rivat C, Snell K, Xu-Bayford J, Morris EC, Terrazas D, Wang LD, Honnet G, Newburger P, Bushman FD, Grez M, Gaspar HB, Williams DA, Galy A, Kohn DB, and Thrasher AJ. Transplantation & Cellular Therapy Meetings of ASBMT & CIBMTR 2019, February 24, 2019. (slides)

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### 2018 cont'd

**AAV8-Mediated hLDLR Gene Transfer in Patients with Homozygous Familial Hypercholesterolemia: Interim Analysis of the Safety Profile in the First 6 Subjects.** Cuchel M, Carr R, Bajaj A, Brent C, Duell B, Tardif JC, Linton M, Fiorentino R, Yoo S, Meagher E, Rader DJ. The American Society of Human Genetics; October 16-20, 2018 (poster)

**Gene Therapy for Sickle Cell Anemia Using a Modified Gamma Globin Lentivirus Vector and Reduced Intensity Conditioning Transplant Shows Promising Correction of the Disease Phenotype.** Malik P, Grimley M, Quinn CT, Shova A, Little C, Lutzko C, Kalfa TA, Niss O, Metha PA, Chandra S, Grassman E, Van der Loo JCM, Witting S, Nordling D, Shreshta A, Felker S, Terrell C, Reeves L, Pillis D, Anastacia L, Bushman FD, Knight-Madden J, Kalinyak K, Davies SM, Asnani M. 2018 American Society of Hematology Meeting (slides)

### 2017

**Osteopontin Deficiency Ameliorates Heart Failure with Preserved Ejection Fraction (HFpEF) Pathology by Upregulating Mitochondrial 2-Oxoglutarate Dehydrogenase Like (OGDHL) Enzyme.** Yousefi K, Ding W, Lambert G, Shehadeh L. Basic Cardiovascular Sciences (BCVS) 2017 Scientific Sessions, 2017 July 10-14 (poster)

**Molecular Cardiac Surgery Mediated SERCA2a Overexpression Demonstrates Enhanced Cardiac Function and Reduced Borderzone Apoptosis in a 6 Month Ovine Ischemic Cardiomyopathy Model.** Fagnoli A, Katz M, Hajjar R, Bridges C. American Society of Gene and Cell Therapy Annual Meeting, 2017 May (poster)

**Enhancing Plasmid Based Therapy Using a Nanoparticle Formulation of Anti-inflammatory Glucocorticoids in Acute Myocardial Infarction: A Drug/Gene Combination Approach for the Heart.** Fagnoli A, Katz M, Hajjar R, Bridges C. American Society of Gene and Cell Therapy Annual Meeting, 2017 May (poster)

### 2016

**LV Unloading Using Impella CP Improves LA Pressure, Function, and Stiffness.** Watanabe S, Fish K, Leonardson L, Hajjar RJ, Ishikawa K. Acute Cardiac Unloading and Recovery 2016 Symposium , 2016 August 26 (poster)

**LV Unloading Using Impella Reduces LA Pressure and Volume Overload in Chronic Mitral Regurgitation.** Watanabe S, Fish K, Leonardson L, Hajjar RJ, Ishikawa K. Acute Cardiac Unloading and Recovery 2016 Symposium , 2016 August 26 (poster)

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### 2016 cont'd

**LV Unloading Using an Impella CP Reduces Wall Stress and Improves Coronary Flow and Perfusion in Infarcted Myocardium.** Ishikawa K, Watanabe S, Leonardson L, Fish K, Hajjar RJ. Acute Cardiac Unloading and Recovery 2016 Symposium, 2016 August 26 (poster)

**Gene Therapy Reduces Angioplasty induced Coronary Restenosis in a High Cholesterol Swine Model.** Agrawal DK et al. European Atherosclerosis Society. 2016 May 29 – June 1 (slides)

**A Characterization of Transgene-Specific “Proteomes” in Lentiviral Vector Clinical Production Lots Identifies Differences Between Vectors with High and Low Infectious Titers.** Witting S, Reeves L, Witzmann F. American Society of Cell and Gene Therapy Annual Meeting. 2016 May 4-7 (poster)

### 2015

**Gene Therapy Using a Self-Inactivating Lentiviral Vector Improves Clinical and Laboratory Manifestations of Wiskott-Aldrich Syndrome.** Chu JI, Henderson LA, Armant M, Male F, Dansereau CH, MacKinnon B, Burke CJ, Cavanaugh ME, London WB, Barlan IB, Ozen A, Baris S, Karakoc-Aydiner E, Kiykim A, Hanson IC, Despotovic JM, Saitoh A, Takachi T, Imai K, King A, Arredondo S, Ritz J, Bushman FD, Galy A, Daniele L, Williams DA, Pai SY. American Society of Hematology Annual Meeting. 2015 December 6 (slides)

**Mesenchymal stem cells in the prevention of thrombosis and intimal hyperplasia.** Agrawal DK et al. Great Wall – International Congress of Cardiology. 2015 October 29 – November 1 (slides)

**Gene Therapy to Prevent Restenosis Following Coronary Intervention.** Agrawal DK et al. International Congress of Cardiology. 2015 October 22-24 (slides)

**Differentiation of Bone Marrow Stromal Cells Into Endothelial Cells and Stem Cell Therapy in Coronary Artery Disease.** Agrawal DK et al. International Academy of Cardiovascular Sciences – European section. 2015 October 7-10 (slides)

**AC6 Gene Transfer for Clinical CHF.** Hammond HK. 2015 European Society of Cardiology Congress. 2015 September (slides)

**Gene Therapy to Prevent Restenosis Following Intervention in CAD.** Agrawal DK et al. 8th International Congress on Cardiovascular Disease. 2015 August 13-15 (slides)

**Gene Therapy to Prevent Restenosis Following Coronary Intervention.** Agrawal DK et al. Invited Presentation at the University of Missouri. 2015 May 12 (slides)

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### 2015 cont'd

**Stem Cell Therapy in Coronary Artery Disease.** Agrawal DK et al. Invited Presentation at the Regenerative Medicine Symposium. 2015 April 17 (slides)

**Gene Therapy to Prevent Restenosis Following Coronary Intervention.** Agrawal DK et al. Invited Presentation at the University of Nebraska Medical Center. 2015 January 26 (slides)

### 2014

**An Innovative Needleless Liquid Jet Injection Delivery Method for Heart Failure Gene Therapy.** Fargnoli AS. 2014 Heart Failure Society Meeting. 2014 November (slides)

**Safety and Efficacy of High Dose SERCA2a Gene Therapy Delivered by Molecular Cardiac Surgery with Recirculating Delivery (MCARD).** Bridges C. American Association of Thoracic Surgery Annual Meeting. 2014 April (slides)

**Targeted Glyoxalase-1 gene transfer prevents cardiac dysfunction in diabetes.** Bidasee K. Experimental Biology Meeting. 2014 April (slides)

**Targeted Glyoxalase-1 gene transfer to prevent cerebral vascular dysfunction and cognitive decline in rates with type-1 diabetes.** Bidasee K. Experimental Biology Meeting. 2014 April (poster)

### 2013

**Gene therapy for  $\alpha$ 1-antitrypsin deficiency with AAV intrapleural administration.** Crystal R. American Society of Gene and Cell Therapy Annual Meeting. Salt Lake City, UT. 2013 May (slides)

### 2012

**ApoE3 and ApoE4 polymorphism: Mechanistic basis for differential isoform effects on serum cholesterol levels and lipoprotein profiles.** Lund-Katz S. Recent Advances in Cardiovascular Disease Assessment, XVI International Symposium on Atherosclerosis, Sydney, Australia. 2012 March (slides)

### 2011

**Normalization of angiogenesis and vasculogenesis by CCN1: Implications in ocular neovascular diseases.** Chaqour B. The International CCN Society: Targets 2011, Vancouver, BC, Canada. 2011 September 24-27 (slides)

**Transductional and transcriptional targeting of AAV to the pulmonary vasculature.** Passineau MJ, Zourelis L, Machen L, Benza RL. American Thoracic Society 2011, Denver, Colorado. 2011 May 13-18 (poster)

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### 2011 cont'd

**Comparison of the structure-function relationships of human ApoE3 and ApoE4: From in vitro structural studies to in vivo mouse studies.** Lund-Katz S. American Heart Association Workshop on HDL, Chicago, Illinois. 2011 April (slides)

### 2010

**Adeno-associated virus 9 vector in the gene therapy of occlusive vascular diseases.** Agrawal DK, Pankajakshan D, Del Core M, Pipinos I, Hatzoudis G. European Atherosclerosis Society Congress 2010, Hamburg, Germany. 2010 June 20-23 (poster)

**The next step from GWAS and eQTL candidate gene discovery: An in vivo follow up of novel genes identified through the Global Lipids Genetics Consortium.** Stylianou IM, Fitzgerald K, Kathiresan S, Rader DJ. 9th Annual Meeting of the Complex Trait Community, Chicago, Illinois. 2010 May 10 (slides)

**Characterization of GALNT2, a novel GWAS locus associated with HDL-C.** Edmondson AC, Khera A, Khetarpal SA, Kathiresan S, Rader DJ. Arteriosclerosis, Thrombosis and Vascular Biology (ATVB) 2010 Scientific Sessions, San Francisco, California. 2010 April 8-10 (poster)

**Sortilin is a novel modulator of low-density lipoprotein cholesterol.** Strong A, Musunuru K, Frank-Kamenetsky M, Sachs KV, Li X, Li H, Lund-Katz S, Phillips M, Wong J, Cantley W, Kotliansky V, Fitzgerald K, Krauss RM, Kathiresan S, Rader DJ. Arteriosclerosis, Thrombosis and Vascular Biology (ATVB) 2010 Scientific Sessions, San Francisco, California. 2010 April 8 (slides)

### 2009

**AAV9-Wnt11 gene therapy improves cardiac recovery after myocardial infarction by modulating the inflammatory response.** Kobayashi K, Klyachko E, Thorne T, Kentaro J, Tongers J, Misener S, Losordo DW. American Heart Association Meeting, Orlando, Florida. 2009 November 14-18 (slides)

**Hemoxygenase-1 gene modification enhances the regenerative efficacy of CD34+ cell-therapy after myocardial infarction.** Tongers J, Kentaro J, Roncalli JG, Renault MA, Kamide CE, Klyachko E, Kobayashi K, Motlagh D, Losordo DW. American Heart Association Meeting, Orlando, Florida. 2009 November 14-18 (slides)

**Functional characterization of GALNT2, a novel GWAS locus associated with HDL-C.** Edmondson A, Khera A, Khetarpal S, Kathiresan S, Rader DJ. 59th Annual Meeting of the American Society of Human Genetics, Honolulu, Hawaii. 2009 October 24 (slides)

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#### **2009 cont'd**

**Functional characterization of GALNT2, a novel GWAS locus associated with HDL-C.** Edmondson A, Khera A, Khetarpal S, Kathiresan S, Rader D. Gordon Research Conference, Human Genetics and Genomics, Biddeford, Maine. 2009 July 19-24 (poster)