DONGSHENG DUAN'S PUBLICATIONS

Books

- 2011 — Muscle Gene Therapy: Methods and Protocols. Humana Press, New York, NY (Duan, D. editor)
- 2010 — Muscle Gene Therapy, Springer, New York, NY (Duan, D. editor) All Publications

All Publications

2022

- Pan X, Yue Y, Boftis M, Wasala LP, Tran NT, Zhang K, Pintel DJ, Tai PWL, **Duan D**. Rational engineering of a functional CpG-free ITR for AAV gene therapy. **Gene Therapy,** online ahead of print **2022**. doi: 10.1038/s41434-021-00296-0

- **Duan D***, Flanigan KM, Aartsma-Rus A. *Regarding the article, "Therapeutic Exon Skipping via a CRISPR-guided Cytidine Deaminase Rescues Dystrophic Cardiomyopathy In Vivo"* **Circulation** in-press, **2022** (*, corresponding author)

- Wasala NB, Million ED, Watkins T, Wasala LP, Han J, Yue Y, Lu B, Chen SJ, Hakim CH, **Duan D.** *The gRNA vector level determines the outcome of systemic AAV CRISPR therapy for Duchenne muscular dystrophy.* **Human Gene Therapy** in-press, **2022**. PMID: 35350865; DOI: 10.1089/hum.2021.130

- Zhang X, Jenkins JG, Hakim CH, **Duan D***, Yao G*. *Four-limb wireless IMU sensor system for automatic gait detection in canines* (*****, co-corresponding author) **Scientific Reports** 12:4788**, 2022**.

- Zheng A, Arias EB, Wang H, Kwak SE, Pan X, **Duan D**, Cartee GD. The exercise-induced improvement in insulin-stimulated glucose uptake by rat skeletal muscle is absent in male AS160-knockout rats, partially restored by muscle expression of phosphomutated AS160, and fully restored by muscle expression of wildtype AS160. **Diabetes**, 72(2):219-232.

2021

- Hakim CH, Kumar SRP, Perez-Lopez DO, Wasala NB, Zhang D, Yue Y, Teixeira J, Pan X, Zhang K, Million ED, Nelson CE, Metzger S, Han J, Louderman JA, Schmidt F, Feng F, Grimm D, Smith BF, Yao G, Yang NN, Gersbach CA, Chen S-J, Herzog RW, **Duan D**. Cas9-specific immune responses compromise local and systemic AAV CRISPR therapy in multiple dystrophic canine models. Nature Communications 12(1),6769, 2021. (Commented in Molecular Therapy 30(1):10-122, 2022, https://pubmed.ncbi.nlm.nih.gov/34895501/; Commented in Human Gene Therapy 32(23-24):1430-1432, 2021. https://pubmed.ncbi.nlm.nih.gov/34935453/; Commented in Gene Therapy online ahead of print 2022. https://pubmed.ncbi.nlm.nih.gov/35194186/

- Hakim CH, Yang HT, Burke MJ, Teixeira J, Jenkins GJ, Yang NN, Yao G, **Duan D**. Contractile kinetic analysis reveals unexpected slow to fast myofiber type conversion in the extensor carpi ulnaris muscle of the canine DMD model. **Disease Models and Mechanisms** 14(12):dmm049006, **2021**.

- Kodippili K, Thorne PK, Laughlin MH, **Duan D**. Dystrophin deficiency impairs vascular structure and function in the canine model of Duchenne muscular dystrophy. **Journal of Pathology** 254(5):589-605, **2021.**

- **Duan D**. A cautiously optimistic outlook of a designer therapy for 1% Duchenne muscular dystrophy patients. **Human Gene Therapy** 32(17-18):872-874, 2021

- **Duan D**, Goemans N, Takeda S, Mercuri Eugenio, Aartsma-Rus A. *Duchenne muscular dystrophy.* **Nature Reviews Disease Primers** 7(1):13, **2021**. PMID: 33602943, DOI: 10.1038/s41572-021-00248-3

- Mareedu S, Million ED, **Duan D**, Babu GJ. *Abnormal Ca^{++} handling in Duchenne muscular dystrophy: mechanisms and potential therapies.* **Frontiers in Physiology** 12:647010, **2021**. PMID: 33897454, PMCID: PMC8063049, DOI: 10.3389/fphys.2021.647010

- Apkon S, Kinnett K, Cripe L, **Duan D**, Jackson JL, Kornegay JN, Mah ML, Nelson SF, Rao V, Scavina M, Wong BL, Flanigan KM. *Parent Project Muscular Dystrophy: females with dystrophinopathy conference. Orlando Florida, June 26-27, 2019.* **Journal of Neuromuscular Diseases** 8(2):315-322, **2021**. PMID: 33361607, DOI: 10.3233/JND-200555

- Fortin JS*, Hakim CH, Korte S, Johnson GC, **Duan D*.** *Widespread severe myodegeneration in a compound heterozygote female dog with dystrophin deficiency.* **Veterinary Medicine and Science** 7(3):654-659, **2021**. (*****, co-corresponding author) PMID: 33502125, PMCID: PMC8136971, DOI: 10.1002/vms3.433

- Kodippili K, Thorne PK, Laughlin MH, **Duan D.** *Dystrophin deficiency impairs vascular structure and function in the canine model of Duchenne muscular dystrophy.* **Journal of Pathology** 254(5):589-605, **2021**. PMID: 33999411, DOI: 10.1002/path.5704

- Wang H, Marrosu E, Brayson D, Wasala NB, Johnson EK, Scott CS, Yue Y, Hau K, Trask AJ, Zhang L, Froehner SC, Adams ME, **Duan D**, Montanaro F. *Biochemical characterization of micro-dystrophin reveals a role for cavins and ERK in DMD cardiomyopathy.* **Human Molecular Genetics,** 30(4):1321-1336, **2021**

2020

- Lyu P, Yoo, KW, Yadav MK, Atala A, Aartsma-Rus A, Putten MV, **Duan D**, Lu B. Sensitive and reliable evaluation of single-cut sgRNAs to restore dystrophin by a GFP-reporter assay. **PLoS One**15(9):e0239468, **2020**.

- Zhao J, Yue Y, Patel A, Wasala LP, Karp JF, Zhang K, **Duan D***, Lai Y*. High-Resolution Histological Landscape of AAV DNA Distribution in Cellular Compartments and Tissues following Local and Systemic Injection. **Molecular Therapy-Methods & Clinical Development** 18:856-868, **2020**. (*, co-corresponding author).
- White Z, Hakim CH, Theret M, Yang NN, Francis G, Cox D, Straub V, Rossi F, **Duan D***, Panagiotopoulos C, Bernatchez P*. High prevalence of plasma lipid abnormalities in human and canine Duchenne and Becker Muscular Dystrophies depicts a new type of primary genetic dyslipidemia. **Journal of Clinical Lipidology. 2020** May 29; S1993-2874 (20)30196-3. Doi:10.1016/j.jacl.2020.05.098 Online ahead of print. PMID: 32593511 (*, co-corresponding author)
- **Duan D**. Laying the foundation for neuromuscular disease gene therapy. **Human Gene Therapy** 31(15-16):785-786, **2020**.
- Hakim CH, Clement N, Wasala LP, Yang HT, Yue Y, Zhang K, Kodippili K, Adamson-Small L, Pan X, Schneider JS, Yang NN, Chamberlain JS, Byrne BJ, **Duan D.** Micro-dystrophin AAV vectors generated by transient transfection and herpesvirus system are similarly effective in protecting muscle disease in dystrophic mice. **Molecular Therapy-Methods & Clinical Development** 18:664-678, **2020**. PMCID: PMC7403893 PMID: 32775499
- Nance ME, Ravanfar M, Messler M, **Duan D***, Yao G*. PSOCT imaging of muscle degeneration and regeneration in a canine muscle xenograft model. **Biomedical Optics Express 2020** Apr 6;11(5):2383-2393. doi: 10.1364/BOE.390936. eCollection 2020 May 1.PMID: 32499931 (*, co-corresponding author)
- Wasala NB, Chen S-J, **Duan D.** Duchenne muscular dystrophy animal models for high-throughput drug discovery and precision medicine. **Expert Opinion in Drug Discovery** 15(4):443-456, **2020**.
- Story BD, Miller ME, Bradbury AM, Million ED, **Duan D,** Taghian T, Fernau D, Beecy SJ, Gray-Edwards HL. Canine models of inherited musculoskeletal and neurodegenerative diseases. **Frontiers in Veterinary Science** 7:80, **2020**. https://doi.org/10.3389/fvets.2020.00080
- Yao G, **Duan D**. High-resolution 3D tractography of fibrous tissue based on polarization-sensitive optical coherence tomography. **Experimental Biology and Medicine** 245(4):273-281, **2020**.
- Chiao YA, Zhang H, Sweetwyne M, Whitson J, Ting YS, Basisty N, Pino L, Quarles E, Nguyen NT, Campbell M, Zhang T, Gaffrey MJ, Merrihew G, Wang L, Yue Y, **Duan D**, Granzier H, Szeto HH, Qian W-J, Marcinek D, MacCoss MJ, Rabinovitch PS. Late-life restoration of mitochondrial function reverses cardiac dysfunction in old mice. **Elife** 9:e55513, **2020**.
- Wasala NB, Yue Y, Lostal W, Wasala LP, Niranjan N, Hajjar RJ, Babu G, **Duan D**. Single SERCA2a Therapy Ameliorated Dilated Cardiomyopathy for 18 Months in a Mouse Model of Duchenne Muscular Dystrophy. **Mol Ther.** 2020 Mar 4;28(3):845-854, doi: 10.1016/j.ymthe.2019.12.011. Epub **2020** Jan 10.

2019

- Zhang D, Hurst T, **Duan D**, Chen S-J. Unified energetics analysis unravels SpCas9 cleavage activity for optimal gRNA design. **Proceedings of National Academy of Science,** 116(18):8693-8698. **2019**.

- Wasala NB, Hakim CH, Chen S-J, Yang NN, **Duan D.** Questions answered and unanswered by the first CRISPR editing study in the canine model of Duchenne muscular dystrophy. **Human Gene Therapy,** 30(5):535-543. **2019**.

- Patel A, Zhao J, **Duan D**, Lai Y. Design of AAV vectors for Delivery of Large or Multiple Transgenes. **Methods in Molecular Biology**, 1950:19-33., **2019**.

- Nance ME, **Duan D.** Development of next generation muscle gene therapy vectors. **Muscle Gene Therapy 2nd edition** (Publisher: Springer.) Duan D and Mendel JR (Ed.), In-press, **2019**.

- **Duan D.** Considerations on preclinical muscle gene therapy studies. **Muscle Gene Therapy 2nd edition** (Publisher: Springer.) Duan D and Mendel JR (Ed.), In-press, **2019**.

- Lai Y, **Duan D**. Design of muscle gene therapy expression cassette. **Muscle Gene Therapy 2nd edition** (Publisher: Springer.) Duan D and Mendel JR (Ed.), In-press, **2019**.

- Wasala LP, Hakim CH, Yue Y, Yang NN, **Duan D.** Systemic delivery of adeno-associated viral vectors in mice and dogs**. Methods in Molecular Biology** 1937:281-294, **2019**.

2018

- Hakim CH, Wasala NB, Nelson CE, Wasala LP, Yue Y, Louderman JA, Lessa TB, Zhang K, Jenkins GJ, Nance ME, Pan X, Kodippili K, Yang NN, Chen S-J, Gersbach CA, **Duan D.** AAV CRISPR editing rescues cardiac and muscle function for 18 months. **JCI Insight** 3(23): e124297, **2018**.

- **Duan D.** CRISPR alleviates muscular dystrophy in dogs. **Nature Biomedical Engineering,** 2(11):795-796**, 2018**

- **Duan D.** Systemic AAV micro-dystrophin gene therapy for Duchenne muscular dystrophy. **Molecular Therapy** 26(10):2337-2356**, 2018**

- **Duan D.** Micro-dystrophin gene therapy goes systemic in Duchenne muscular dystrophy patients. **Human Gene Therapy** 29(7):733-736, **2018**

- Kodippili K, Hakim CH, Yang HT, Pan X, Yang NN, Laughlin MH, Terjung RL, **Duan D.** Nitric oxide dependent attenuation of norepinephrine-induced vasoconstriction is impaired in the canine model of Duchenne muscular dystrophy. **Journal of Physiology** 596(21):5199-5216**, 2018**

- Kodippili K, **Duan D.** Expressing full-length dystrophin using adeno-associated virus. **Gene Therapy in Neurological Disorders** Elsevier (Publisher) Ed: Mingjie Li and Joy Snider Chapter 13, 259-276, **2018**

- Jenkins JG, Hakim CH, Yang NN, Yao G, **Duan D**. Automatic characterization of stride parameters in canines with a single wearable inertial sensor. **PLoS One**13(6): e0198893, **2018**.

- Wasala NB, Shin J-H, Lai Y, Yue Y, **Duan D.** Cardiac specific expression of ΔH2-R15 mini-dystrophin normalized all ECG abnormalities and the end-diastolic volume in a 23-m-old mouse model of Duchenne dilated cardiomyopathy. **Human Gene Therapy** 29(7):737-748**, 2018** (Journal cover image)

- Patel A, Zhao J, Yue Y, Zhang K, **Duan D***, Lai Y*. Dystrophin R16/17-syntrophin PDZ fusion protein restores sarcolemmal nNOSμ. **Skeletal Muscle** 8:36, **2018**. https://doi.org/10.1186/s13395-018-0182-x (*, co-corresponding author).

- Nelson DM, Lindsay A, Judge LM, **Duan D**, Chamberlain JS, Lowe DA, Ervasti JM. Variable rescue of microtubule and physiological phenotypes in mdx muscle expressing different miniaturized dystrophins. **Human Molecular Genetics** 27(12):2090-2100**, 2018**.

- Nance ME, **Duan D.** Gene therapy: use of viruses as vectors. **Reference Module in Biomedical Sciences** Elsevier. 10-Apr-2018 doi:10.1016/B978-0-12-801238-3.95711-8**, 2018**. https://www.sciencedirect.com/science/article/pii/B9780128012383957118?via%3Dihub

- Wang Y, Ravanfar M, Zhang K, **Duan D**, Yao G. Automatic quantification of microscopic heart damage in a mouse model of Duchenne muscular dystrophy using optical polarization tractography. **Journal of Biophotonics** 11(4):e201700284, **2018**.

- Gordish-Dressman H, Willmann R, Dalle Pazze L, Kreibich A, van Putten M, Heydemann A, Bogdanik L, Lutz C, Davies K, Demonbruen AR, **Duan D**, Elsey D, Fukada SI, Girgenrath M, Patrick Gonzalez J, Grounds MD, Nichols A, Partridge T, Passini M, Sanarica F, Schnell FJ, Wells DJ, Yokota T, Young CS, Zhong Z, Spurney C, Spencer M, De Luca A, Nagaraju K, Aartsma-Rus A. "Of Mice and Measures": A Project to Improve How We Advance Duchenne Muscular Dystrophy Therapies to the Clinic (First Workshop Report: Examining current findings and opportunities around the emerging D2.B10-Dmd^{mdx}/J (D2/mdx) model in context of the classic C57BL/10ScSn-Dmd^{mdx}/J (Bl10/mdx)). **Journal of Neuromuscular Diseases** 5(4):407-417**, 2018**.

- Kodippili K, Pan X, Yang HT, Hakim CH, Yue Y, Zhang Y, Shin J-H, Yang NN, **Duan D.** Duan AAV gene therapy for Duchenne muscular dystrophy with a 7-kb mini-dystrophin gene in the canine model. **Human Gene Therapy** 29(3):299-311**, 2018** (Journal cover image)

- Nance ME, Hakim CH, Yang NN and **Duan D.** Nanotherapy for Duchenne muscular dystrophy. **WIREs Nanomedicine and Nanobiotechnology** 18(2):e1472**, 2018** (Journal cover image)

2017

- Wang Y, Zhang K, **Duan D**, Yao G. Heart structure remodeling in the mdx4cv mouse model of Duchenne cardiomyopathy revealed using optical polarization tractography. Biomedical Optics Express 8(3):1271-1276**, 2017**.
- **Duan D.** A new kid in the playground of CRISPR DMD therapy. Human Gene Therapy Clinical Development 28(2):62-64**, 2017**.
- Voit A, Patel V, Pachon R, Shah V, Bakhutma M, Kohlbrenner E, McArdle J, Dell'Italia LJ, Mendell JR, Xie L-H, Hajjar RJ, **Duan D**, Fraidenraich D, Babu GJ. Reducing sarcolipin expression mitigates Duchenne muscular dystrophy and associated cardiomyopathy in mice. Nature Communication 8:1068, **2017.**
- Hakim CH, Wasala NB, Pan X, Kodippili K, Yue Y, Zhang K, Yao G, Haffner B, Duan XS, Schneider JS, Yang NN, Chamberlain JS, **Duan D.** A five-repeat micro-dystrophin gene ameliorated dystrophic phenotype in the severe DBA/2J-mdx model of Duchenne muscular dystrophy. Molecular Therapy-Methods & Clinical Development 6:216-230**, 2017.**
- Azinfar L, Ravanfar M, Wang Y, Zhang K, **Duan D**, Yao G. High resolution imaging of the fibrous microstructure of the bovine carotid artery with optical polarization tractography. Journal of Biophotonics 10(2):231-241, **2017**
- Xu X, **Duan D**, Chen S. CRISPR-Cas9 cleavage efficiency correlates strongly with target-sgRNA folding stability: from physical mechanism to off-target assessment. Scientific Reports 7:143, **2017**.
- Hakim CH, Mijailovic A, Lessa TB, Coates JR, Rutkove SB, **Duan D.** Non-invasive evaluation of muscle disease in the canine model of Duchenne muscular dystrophy by electrical impedance myography. PLoS One, 12(3):e0173557**, 2017**.
- Wasala NB, Yue Y, Jenna Vance, **Duan D.** Uniform low-level dystrophin expression in the heart partially preserved cardiac function in an aged mouse model of Duchenne cardiomyopathy. Journal of Molecular and Cellular Cardiology 102:45-52, **2017.**

2016

- Nelson CE, Hakim CH, Ousterout DG, Thakore P, Moreb EA, Castellanos R, Madhavan S, Pan X, Asokan A, Zhang F, **Duan D** and Gersbach CA. Genome editing with CRISPR/Cas9 restores dystrophin expression and improves muscle function in an adult mouse model of Duchenne muscular dystrophy. Science 6271:403-407, **2016**.
- **Duan D**, Rafael-Fortney JA, Blain A, Kass DA, McNally EM, Metzger JM, Spurney CF, Kinnett K. Standard operating procedures (SOPs) for preclinical Duchenne muscular dystrophy cardiomyopathy studies. Journal of Cardiovascular Translational Research 9(1):85-86, **2016**.

- Yue Y, Binalsheikh IM, Leach SB, Domeier TL, **Duan D**. Prospect of gene therapy for cardiomyopathy in hereditary muscular dystrophy. Expert Opinion on Orphan Drugs 4(2):169-183, **2016**.
- **Duan D.** Dystrophin gene replacement and gene repair therapy for Duchenne muscular dystrophy in 2016. Human Gene Therapy Clinical Development. 27(1):9-18, **2016**.
- Zhao J, Kodippili K, Yue Y, Hakim CH, Wasala L, Pan X, Zhang K, Yang NN, **Duan D**, Lai Y. Dystrophin contains multiple independent membrane-binding domains. Human Molecular Genetics 25(10):3647-3653, **2016**. (DD and YL as co-corresponding authors).
- Wasala NB, Lai Y, Shin J-H, Zhao J, Yue Y, **Duan D.** Genomic removal of a therapeutic mini-dystrophin gene from adult mice elicits a Duchenne muscular dystrophy-like phenotype. Human Molecular Genetics 25(13):2633-2644, **2016**.
- Yue Y, Wasala NB, Bostick B, **Duan D.** 100-fold but not 50-fold dystrophin overexpression aggravates electrocardiographic defects in the mdx model of Duchenne muscular dystrophy. Molecular Therapy-Methods & Clinical Development. 3:16045, 2016.
- **Duan D.** Systemic delivery of adeno-associated viral vectors. Current Opinion in Virology 21:16-25, **2016**.
- Wang Y, Ravanfar M, Zhang K, **Duan D**, Yao G. Mapping 3D fiber orientation in tissue using dual-angle optical polarization tractography. Biomedical Optics Express. 7(10):3855-3870, **2016**.
- Yao X, Wang Y, Ravanfar M, Pfeiffer FM, **Duan D**, Yao G. Imaging fiber structure in cartilage using optical polarization tractography. Journal of Biomedical Optics**,** 21(11):116004, **2016**.

2015

- McGreevy JW, Hakim CH, McIntosh M, **Duan D.** Animal models for Duchenne muscular dystrophy: from basic mechanisms to gene therapy. Disease Model and Mechanism 8(3):195-213, **2015**.
- Wasala NB, Zhang K, Wasala PL, Hakim CH, **Duan D**. FVB background does not dramatically alter dystrophic phenotype in mdx mice. PLoS Currents Muscular Dystrophy. 7. pii: ecurrents.md.28266819ca0ec5fefcac767ea9a3461c, **2015**.
- Pan X, Yue Y, Zhang K, Hakim CH, Kodippili K, McDonald T, **Duan D**. AAV-8 is more efficient than AAV-9 in transducing neonatal dog heart. Human Gene Therapy Methods. 26(4):54-61, **2015**.
- Cheever TR, Berkley D, Braun S, Brown RH, Byrne BJ, Chamberlain JS, Cwik V, **Duan D**, Federoff HJ, High KA, Kaspar BK, Klinger KW, Larkindale J, Lincecum J, Mavilio F, McDonald CL, McLaughlin J, McLeod BW, Mendell JR, Nuckolls G, Stedman HH, Tagle DA,

Vandenberghe LH, Wilson JM, Wernett PJ, Wang H, Porter JD, Gubitz AK . Perspectives on best practices for gene therapy programs. Human Gene Therapy 26(3):127-133, **2015**.

- **Duan D**, Hakim CH, Ambrosio C, Smith B, Sweeney L. Early loss of ambulation is not a representative clinical feature in Duchenne muscular dystrophy dogs. Disease Model and Mechanism 8(3):193-194, **2015**.
- McNally EM, Kaltman JR, Benson DW, Canter CE, Cripe LH, **Duan D**, Finder JD, Hoffman EP, Judge DP, Kertesz N, Kinnett K, Kirsch R, Metzger JM, Pearson GD, Rafael-Fortney JA, Raman SV, Spurney CF, Targum SL, Wagner KR, Markham LW. Contemporary Cardiac Issues in Duchenne Muscular Dystrophy. Circulation 131:1590-1598, **2015**.
- Wang Y, Zhang K, Wasala NB, **Duan D**, Yao G. Optical polarization tractography revealed significant fiber disarray in skeletal muscle of a mouse model for Duchenne muscular dystrophy. Biomedical Optics Express. 6(2):347-352, 2015.
- **Duan D**. Duchenne muscular dystrophy gene therapy in the canine model. Human Gene Therapy Clinical Development. 26(3):157-169, **2015**.
- Yue Y, Pan X, Hakim CH, Kodippili K, Zhang K, Shin J-H, Yang HS, McDonald T, **Duan D**. Safe and bodywide muscle transduction in young adult Duchenne muscular dystrophy dogs with adeno-associated virus. Human Molecular Genetics. 24(20):5880-5890, 2015.
(highlighted in Human Gene Therapy Clinical Development 26(4):213-214, **2015**. Also reported in Fox News, Forbes and NIH Research Matters).
- Hakim CH, Peters AA, Feng F, Yao G, **Duan D**. Night activity reduction is a signature physiological biomarker for Duchenne muscular dystrophy dogs. Journal of Neuromuscular Diseases. 2(4):397-407, **2015**.
- Nance ME and **Duan D**. Perspective on adeno-associated virus (AAV) capsid modification for Duchenne muscular dystrophy gene therapy. Human Gene Therapy 16(12):786-800, **2015**.

2014

- Lai Y, Zhao J, Yue Y, Wasala NB, **Duan D**. Partial restoration of cardiac function with ΔPDZ nNOS in aged mdx model of Duchenne cardiomyopathy. Human Molecular Genetics, 23(12):3189-3199, **2014**. PMCID:PMC4030774.
- Kodippili K, Vince L, Shin J-H, Yue Y, Mooris G, McIntosh MA, **Duan D**. Characterization of 65 epitope-specific dystrophin monoclonal antibodies in canine and murine models of Duchenne muscular dystrophy by immunostaining and western blot. PLoS One, 9(2):e88280, **2014**.
- Hakim CH, Yue Y, Shin J-H, Williams RR, Zhang K, Smith BF, **Duan D**. Systemic gene transfer reveals distinctive muscle transduction profile of tyrosine mutant AAV-1, -6 and -9 in neonatal dogs. Molecular Therapy-Methods & Clinical Development. 1:14002,
- Lostal W, Kodippili K, Yue Y, **Duan D**. Full-length dystrophin reconstitution with adeno-associated viral vectors. Human Gene Therapy 25(6):552-62, **2014**.

- Wang Y, Zhang K, Wasala NB, Yao X, **Duan D**, Yao G. Histology validation of mapping depth-resolved cardiac fiber orientation in fresh mouse heart using optical polarization tractography. Biomedical Optics Express 5(8):2843-55, **2014**. PMCID: PMC4133011

2013

- Hakim C, Burkin D, **Duan D**. 2013. Alpha7 integrin preserves the function of the extensor digitorum longus muscle in dystrophin-null mice. Journal of Applied Physiology 115(9):1388-1392.
- Zhang Y, Yue Y, Li L, Hakim C, Zhang K, Thomas GD, Duan D. 2013. Systemic dual AAV delivery of a 6 kb nNOS-binding mini-dystrophin gene ameliorates exercised-induced muscle injury and functional ischemia in murine DMD models. Human Molecular Genetics 22(18):3720-3729.
- Pan X, Yue Y, Zhang K, Lostal W, Shin J-H, **Duan D**. 2013. Long-term robust transduction of the dog heart from a peripheral vein by adeno-associated virus serotype-8. Human Gene Therapy 24(6):584-594.
- Hakim C, **Duan D**. 2013. Truncated dystrophins reduce muscle stiffness in mdx extensor digitorum longus muscle. Journal of Applied Physiology 114(4):482-9. [Epub 2012 Dec 6].
- Shin J-H, Pan X, Hakim C, Yang HT, Yue Y, Zhang K, Terjung RL, **Duan D**. 2013. Micro- dystrophin ameliorates muscular dystrophy in dogs. Molecular Therapy Jan 15. doi: 10.1038/mt.2012.283. [Epub ahead of print].
- Wasala N, Bostick B, Yue Y, **Duan D**. 2013. Exclusive skeletal muscle correction does not modulate dystrophic heart disease in the aged mdx model of Duchenne cardiomyopathy. Human Molecular Genetics Mar 15. [Epub ahead of print].
- Lai Y, Zhao J, Yue Y, **Duan D**. 2013. α2 and α3 helices of dystrophin R16 and R17 frame a micro-domain in the α1 helix of dystrophin R17 for nNOS binding. Proceedings of National Academy of Science 110(2):525-530. (commented in Proceedings of National Academy of Science 110(2):387-388, 2013).
- Shin J-H, Greer B, Hakim C, Zhou Z, Chung Y, Duan Y, He Z, **Duan D**. 2013. Quantitative phenotyping of Duchenne muscular dystrophy dog by comprehensive gait analysis and overnight activity monitoring. PLoS One 8(3):e59875.
- Li D, **Duan D**. 2013. Mitochondria-targeted anti-aging gene therapy with adeno-associated viral vectors. Methods in Molecular Biology 1048:161-80.
- Hakim C, Wasala N, **Duan D**. 2013. Ex vivo evaluation of the EDL muscle contractile and passive properties and in situ evaluation of the TA muscle contractility. Journal of Visualized Experiments (72):pii:50183. doi:10.3791/50183.

2012

- Hakim C, **Duan D.** 2012. Marginal level dystrophin expression improves limb muscle passive mechanic properties in dystrophin-null mice. Muscle & Nerve 46(6):943-7.
- Yang HT, Shin J-H, Hakim C, Pan X, Terjung RL, **Duan D.** 2012. Dystrophin deficiency compromises force production of the extensor carpi ulnaris muscle in the canine model of Duchenne muscular dystrophy. PLoS One 7(9):e44438.
- Bostick B, Shin J-H, Yue Y, Wasala N, Lai Y, **Duan D.** 2012. AAV micro-dystrophin therapy alleviates stress-induced cardiac death but does not reduce myocardial fibrosis in >21- m-old mdx mice. Journal of Molecular and Cellular Cardiology 53(2):217-222.
- Hakim C, Grange R, **Duan D**. 2012. Myofiber branching, dystrophin deficiency and muscle tearing (Reply to Head). Journal of Applied Physiology 112:332.
- Lai Y, **Duan D.** 2012. Progress in gene therapy of dystrophic heart disease. Gene Therapy 19(6):678-85. [Epub ahead of print].
- Shin J-H, Yue Y, Smith B, **Duan D.** 2012. Humoral immunity to AAV-6, 8 and 9 in normal and dystrophic dogs. Human Gene 23(2):287-94. [Epub 2011 Dec 14].
- Shin J-H, Yue Y, Srivastava A, Smith B, **Duan D**. 2012. A simplified immune suppression scheme leads to sustained micro-dystrophin expression in Duchenne muscular dystrophy dogs. Human Gene Therapy 23(2):202-9. [Epub 2011 Dec 14].
- Bacman S, Williams S, **Duan D**, Moraes C. 2012. Manipulation of mtDNA heteroplasmy in muscles of newborn mice by AAV9 expressing a mitochondria targeted restriction endonuclease. Gene Therapy [Epub 2011 Dec 1].
- Hakim C, **Duan D.** 2012. Gender differences in contractile and passive properties of mdx extensor digitorum longus muscle. Muscle & Nerve 45(2):250-256.
- Zhang Y, **Duan D.** 2012. Novel mini-dystrophin gene dual AAV vectors restore nNOS expression at the sarcolemma. Human Gene Therapy 23(1):98-103
- Shin J-H, Yue Y, **Duan D.** 2012. Recombinant AAV production and purification. Methods in Molecular Biology 798:267-284.

2011

- Bostick B, Shin J-H, Yue Y, **Duan D.** 2011. AAV-microdystrophin therapy improves cardiac performance in aged female mdx mice. Molecular Therapy 19 (10):1826-1832
- Shin J-H, Bostick B, Yue Y, Hajjar R, **Duan D.** 2011. SERCA2a gene transfer improves electrocardiographic performance in aged mdx mice. Journal of Translational Medicine 9:132
- **Duan D.** 2011. Gene delivery to the heart: an updated review on the vectors and methods. (Editorial). Journal of Gene Medicine 13:556
- Wasala N, Shin J, **Duan D**. 2011. The evolution of heart gene delivery vectors. Journal of Gene Medicine 13:557-565

- Li D, Shin J-H, **Duan D**. 2011. iNOS ablation does not improve specific force of the extensor digitorum longus muscle in dystrophin-deficient mdx4cv mice. PLoS One 6(6):e21618.
- Hakim C, Grange R, **Duan D**. 2011. The Passive mechanical properties of the extensor digitorum longus muscle are compromised in 2 to 20-month-old mdx mice. Journal of Applied Physiology 110(6):1656-1663
- Fine D, Shin J-H, Yue Y, Volkmann D, Leach SB, Smith BF, McIntosh M, **Duan D**. 2011. Age-matched comparison reveals early ECG and echocardiography changes in dystrophin-deficient dogs. Neuromuscular Disorder 21(7):453-461.
- **Duan D**. 2011. Duchenne muscular dystrophy gene therapy: Lost in translation? Research and Report in Biology 2:31-42.
- Bagher P, **Duan D**, Segal SS. 2011. Impaired Neurovascular Transmission in a Mouse Model of Duchenne Muscular Dystrophy. Journal of Applied Physiology 110:601-609.
- Smith B, Yue Y, Kornegay JN, Shin J-H, Williams RR, **Duan D**. 2011. LINE-1 element insertion results in an independent canine model for Duchenne muscular dystrophy in the corgi breed. Laboratory Investigation 91(2):216-231.
- Shin J-H, Hakim C, Zhang K, **Duan D**. 2011. Genotyping mdx, mdx3cv, and mdx4cv mice by primer competition PCR. Muscle & Nerve, Feb;43(2):283-6. doi:10.1002/mus.21873. Epub 2010 Dec 9.
- Ghosh A, Yue Y, **Duan D**. 2011. Efficient transgene reconstitution with hybrid dual AAV vectors carrying the minimized bridging sequences. Human Gene Therapy 22(1):77-83.
- Yue Y, Shin J-H, **Duan D**. 2011. Whole body skeletal muscle transduction in neonatal dogs with AAV-9. Method in Molecular Biology 709:313-329.
- Bostick B, Yue Y, **Duan D**. 2011. Phenotyping cardiac gene therapy in mice. Method in Molecular Biology 709:91-104.
- Hakim C, Li D, **Duan D**. 2011. Monitoring murine skeletal muscle function for muscle gene therapy. Method in Molecular Biology 709:75-89.
- Li D, Yue Y, Lai Y, Hakim C, **Duan D**. 2011. Nitrosative stress elicited by nNOSm delocalization inhibits muscle force in dystrophin-null mice. Journal of Pathology 223 (1):88-98.
- **Duan D** (editor) Muscle Gene Therapy: Methods and Protocols. Humana Press. (Publisher) (2011) ISBN: 978-1-61737-981-9.

2010

- Li D, Yue Y, **Duan D**. 2010. Minimal dystrophin expression improves clinical outcome of dystrophin/utrophin double knockout mice. PLoS ONE 5(12):e15286.
- Qiao C, Wei Z, Yuan Z, Shin J, Li J, Jayandharan G, Zhong L, Srivastava A, Xiao X, **Duan D**. 2010. AAV6 capsid tyrosine to phenylalanine mutations improve gene transfer to skeletal muscle. Human Gene Therapy 21(10):1343-1348.

- Bostick B, Yue Y, **Duan D.** 2010. Gender influences cardiac function in the mdx model of Duchenne cardiomyopathy. Muscle & Nerve 42(4):600-603.
- Li D, Bareja A, Judeg L, Yue Y, Lai Y, Fairclough R, Davies KE, Chamberlain JS, **Duan D**. 2010. Sarcolemmal nNOS anchoring reveals a qualitative difference between dystrophin and utrophin. Journal of Cell Science 123:2008-13.
- **Duan D** (editor) Muscle Gene Therapy Springer. (Publisher) (2010) ISBN:978-1-4419-1205-3.
- Lai Y, Yue Y, Bostick B, **Duan D**. 2010. Delivering large therapeutic gene to muscle. In Muscle Gene Therapy Springer. (Publisher)
- Duan D (Ed.) Shin J-H, Bostick B, Yue Y, **Duan D**. 2010. Duchenne cardiomyopathy gene therapy. In Muscle Gene Therapy Springer. (Publisher) Duan D (Ed.)
- Lai Y, Yue Y, **Duan D.** 2010. Evidence for the failure of adeno-associated virus serotype-5 to package a viral genome equal or larger than 8.2 kb. Molecular Therapy 18(1):75-79. (Commented in Molecular Therapy 18:6-7, 2010).
- Lei B, Zhang K, Yue Y, Ghosh A, **Duan D.** 2010. Adeno-associated virus serotype-9 mediated retinal outer plexiform layer transduction is mainly through the photoreceptors. In Retinal Degenerative Diseases. Springer. (Publisher), Anderson, Hollyfield, and LaVail (Ed.) Advances in Experimental Biology and Medicine 664:671-678.

2009

- Ghosh A, Yue Y, Shin JH, **Duan D.** 2009. Systemic trans-splicing AAV delivery efficiently transduces the heart of adult mdx mouse, a model for Duchenne muscular dystrophy. Human Gene Therapy 20:1319-1328 (Commented in Human Gene Therapy 20:1224- 1225, 2009).
- Li D, Lai Y, Yue Y, Rabinovitch PS, Hakim C, **Duan D.** 2009. Ecotopic catalase expression in mitochondria by adeno-associated virus enhances exercise performance in mice. PLoS ONE 4(8):e6673.
- Lei B, Zhang K, Yue Y, Ghosh A, **Duan D**. 2009. Adeno-associated virus serotype-9 efficiently transduces the retinal outer plexiform layer. Molecular Vision 15:1374-1382.
- Li D, Long C, Yue Y, **Duan D.** 2009. Sub-physiological sarcoglycan expression contributes to compensatory muscle protection in mdx mice. Human Molecular Genetics 18(7):1209-1220.
- Lai Y, Thomas GD, Yue Y, Yang HT, Li D, Long C, Judge L, Bostick B, Chamberlain JS, Terjung R, **Duan D.** 2009. Dystrophins carrying spectrin-like repeats 16/17 anchor nNOS to the sarcolemma and enhance exercise performance. Journal of Clinical Investigation 119(3):624-635. (Commented in JCI 119:484-486, 2009).
- Bostick B, Yue Y, Long C, Marchalk N, Fine D, **Duan D.** 2009.Cardiac expression of a mini-dystrophin that normalizes skeletal muscle force only partially restores heart function in aged mdx mice. Molecular Therapy 17(2):253-261.

2008

- Yue Y, Ghosh A, Long C, Bostick B, Smith B, Kornegay J, **Duan D**. 2008. A single intravenous injection of adeno-associated virus serotype-9 leads to whole body skeletal muscle transduction in dogs. Molecular Therapy 16(12):1944-1952. (Commented in LabAnimals).
- Lai Y, Li D, Yue Y, **Duan D**. 2008. Design of trans-splicing adeno-associated viral vectors for Duchenne muscular dystrophy gene therapy. Method in Molecular Biology 433(1):259-275.
- **Duan D**. 2008. Dystrophin knockdown mice suggest that early, transient dystrophin expression might be enough to prevent later pathology. Neuromuscular Disorder 18(11):904-905.
- Bostick B, Yue Y, Lai Y, Long C, Li D, **Duan D**. 2008. AAV-9 micro-dystrophin genetherapy ameliorates electrocardiographic abnormalities in mdx mice. Human Gene Therapy 19(8):851-856. (Journal Cover).
- Yue Y, Ghosh A, Long C, Bostick B, Smith B, Kornegay J, **Duan D**. 2008. A single intravenous injection of adeno-associated virus serotype-9 leads to whole body skeletal muscle transduction in dogs. Molecular Therapy Online Sep 30.
- Li D, Yue Y, **Duan D**. 2008. Preservation of Muscle Force in Mdx3cv Mice Correlates with Low-level Expression of a Near Full-length Dystrophin Protein. American Journal of Pathology 172(5):1332-1341.
- Lai Y, Li D, Yue Y, **Duan D**. 2008. Design of trans-splicing adeno-associated viral vectors for Duchenne muscular dystrophy gene therapy. Method in Molecular Biology 433(1):259-275.
- **Duan D**. 2008. Drug profile: Myodys, a full-length dystrophin plasmid vector for Duchenne and Becker muscular dystrophy gene therapy. Current Opinion in Molecular Therapeutics 10(1):86-94.
- Bostick B, Yue Y, Long C, **Duan D**. 2008. Prevention of Dystrophin-deficient Cardiomyopathy in 21-month-old Carrier Mice by Mosaic Dystrophin Expression or Complementary Dystrophin/Utrophin Expression. Circulation Research in press. (Online publication 10/25/2007). (Journal Cover).
- Ghosh A, Yue Y, Lai Y, **Duan D**. 2008. A hybrid vector system expands adenoassociated viral vector packaging capacity in a transgene-independent manner. MolecularTherapy 16(1):124-130.

2007

- Bostick B, Ghosh A, Yue Y, Long C, **Duan D**. 2007. Systemic AAV-9 transduction in mice is influenced by animal age but not by the route of administration. Gene Therapy 14(22):1605-09.
- https://www.ncbi.nlm.nih.gov/pubmed?Db=pubmed&Cmd=ShowDetailView&TermToSearch=17696155&ordinalpos=6&itool=EntrezSystem2.PEntrez.Pubmed.Pubmed_ResultsPanel.Pubmed_RVDocSum 2007. Catalase Over-expression does not Impair EDL Muscle Function in Normal Mice. Muscle & Nerve 24:165-177.

- Ghosh A, **Duan D**. 2007. Expanding adeno-associated viral vector capacity: a tale of two vectors. Biotechnology and Genetic Engineering Reviews 24:165-177. (Journal Cover).
- Ghosh A, Yue Y, Chun Long, Brian Bostick, **Duan D**. 2007. Efficient whole body transduction with trans-splicing adeno-associated viral vectors. Molecular Therapy 15(4):750-755.

2006

- **Duan D**. 2006. From the smallest virus to the biggest gene: marching towards gene therapy of Duchenne muscular dystrophy gene therapy. Discovery Medicine 6(33):103-108. (Journal Cover).
- **Duan D**. 2006. Challenges and opportunities in dystrophin-deficient cardiomyopathy gene therapy. Human Molecular Genetics 15 (Suppl 2):R253-261.
- Lai Y, Yue Y, Liu M, **Duan D**. 2006. Synthetic Intron Improves Transduction Efficiency of the Trans-splicing Adeno-associated Viral Vectors. Human Gene Therapy 17(10):1036- 1042. (Journal Cover).
- Yue Y, Liu M, **Duan D**. 2006. C-terminal truncated Microdystrophin Recruitis Dystrobrevin and Syntrophin to the Dystrophin-associated Glycoprotein Complex and Reduces Muscular Dystrophy in sympotomatic Utrophin/dystrophin Double Knockout Mice. Molecular Therapy 14(1):79-87.
- Ghosh A, Yue Y, **Duan D**. 2006. Viral Serotype and the Transgene Sequence Influence Overlapping Adeno-associated Viral (AAV) Vector-Mediated Gene Transfer in Skeletal Muscle. Journal of Gene Medicine, 8(3):298-305 (2006).
- **Duan D**, Yan Z, Engelhardt, JF. 2006. "Expanding the Capacity of AAV Vectors" in Parvoviruses Hodder Arnold Inc. (Publisher) M.E. Bloom, S.F. Cotmore, R.M. Linden, C.R. Parrish, and J.R. Kerr (Ed.) pp 525-532. (DD as corresponding author).
- Yan Z, **Duan D**, Engelhardt, JF. 2006."Mechanisms of recombiant adeno-associated virus transduction" in Parvoviruses, Hodder Arnold Inc. (Publisher) M.E. Bloom, S.F. Cotmore, R.M. Linden, C.R. Parrish, and J.R. Kerr (Ed.) pp 511-524.

2005

- Lai Y, Yue Y, Liu M, Ghosh A, Engelhardt JF, Chamberlain JS, **Duan D**. 2005. Efficient Expression of the 6kb ΔH2-R19 Mini-dystrophin Gene by Trans-Splicing Adeno-Associated Viral Vectors Ameliorates Muscular Dystrophy in Mdx Mice. Nature Biotechnology 23:1435-1439. (2005).
- Liu M, Yue Y, Harper S, Grange R, Chamberlain JS, **Duan D**. 2005. Adeno-associated Virus-mediated Micro-dystrophin Expression Protects Young Mdx Muscle from Contraction-induced injury. Molecular Therapy 11(2):245-256.

2004

- Rex TS, Tsui I, Hahn P, Maguire AM, **Duan D**, Bennett J, Dunaief JL. 2004. Adenovirus-mediated Delivery of Catalase to RPE Cells Protects Neighboring Photoreceptors from Photo-oxidative Stress. Human Gene Therapy 15(10):960-967.
- Xu Z, Yue Y, Lai Y, Zhang C, Qiu J, Pintel DJ, **Duan D**. 2004. Trans-splicing Adenoassociated Viral Vector-medicated Gene Therapy is Limited by the Accumulation of Spliced mRNA but not by Dual Vector Co-infection Efficiency. Human Gene Therapy 15(9): 896-905.
- Yue Y, Skimming JW, Liu M, Strawn T, **Duan D**. 2004. Full-length Dystrophin Expression in Half of the Heart Cells Ameliorates b-Isoproterenol-induced Cardiomyopathy in mdx mice. Human Molecular Genetics 13(15): 1669-1675.

2003

- Yue Y, Li, Z, Harper SQ, Davisson RL, Chamberlain JS, **Duan D**. 2003. Microdystrophin Gene Therapy of Cardiomyopathy Restores Dystrophin-Glycoprotein Complex and Improves Sarcolemma Integrity in the Mdx Mouse Heart. Circulation 108(13):1626-32. (Journal Cover).
- Yue Y, **Duan D**. 2003. Double Strand Interaction is the Predominant Pathway for Intermolecular Recombination of Adeno-associated Viral Genome. Virology 313(1):1-7.
- Li Z, Sharm R, **Duan D**, Davisson RL. 2003. Adenovirus-Mediated Gene Transfer to Adult Mouse Cardiomyocytes is Selectively Influenced by Culture Medium. Journal of Gene Medicine 5(9):765-772.
- **Duan D**, Yue Y, and Engelhardt JF. 2003. Consequences of DNA-Dependent Protein Kinase Catalytic Subunit Deficiency On Recombinant Adeno-associated Virus Genome Circularization and Heterodimerization in Muscle. Journal of Virology 77:4751-4759. (DD as corresponding author).
- **Duan D**, Yue Y, Yan Z, Engelhardt, JF. 2003. "Trans-splicing Vectors Expand the Packaging Limits of Adeno-associated Virus for Gene Therapy Applications" in Methods in Molecular Medicine. Viral Vectors for Gene Therapy Methods and Protocols Humana Press Inc. Ed: Curtis A. Machida 76:287-307.(DD as corresponding author).
- **Duan D**, Yue Y, Engelhardt, JF. 2003. " Dual vector expansion of the recombinant AAV packaging capacity" in Methods in Molecular Biology. Cardiac Gene Transfer: Protocols, Principles and Applications Humana Press Inc. Ed: Joseph M. Metzger 219:29-51.

2002

- **Duan D**, Yue Y, Engelhardt, JF. 2002. "Adeno-associated Virus" in Gene Therapy in Lung Diseases (Marcel Dekker, Inc. Publisher) (Ed: Steven M. Albelda pp51-92)(DD and JE as corresponding authors).
- Yue Y, **Duan D**. 2002. Development of multiple cloning cis-vectors for recombinant adeno-associated virus production. Biotechniques 33(3):672-678.

- Harper S, Hause M, DelloRusso C, **Duan D**, Crawford R, Phelps S, Harper H, Robinson A, Engelhardt J, Brooks S, Chamberlain J. Modular Flexibility of Dystrophin: Implications for Gene Therapy of Duchenne Muscular dystrophy. Nature Medicine 8(3):253-261(2002).

2001

- **Duan D**, Engelhardt, JF. 2001 "Stretching it: Expanded Applications of Recombinant Adeno-associated Viral Vectors for Gene Therapy" in Biotech Lab International. 6(2):10- 12.
- **Duan D**, Yue Y, and Engelhardt JF. 2001. Expanding AAV packaging capacity with trans-splicing or overlapping vectors: a quantitative comparison. Molecular Therapy 4:383-391. (DD as corresponding author).
- **Duan D**, Yan Z, Yue Y, Wei D, and Engelhardt JF. 2001. Enhancement Of Muscle Gene Therapy By Pseudotyped AAV-5 Correlates With Myoblast Differentiation Journal of Virology 75:7662-7671. (DD as corresponding author).

2000

- **Duan D**, Yue Y, Yan Z, Yang J, and Engelhardt JF. 2000. Endosomal Processing Limits Gene Transfer to Polarized Airway Epithelia by Adeno-Associated Virus. Journal of Clinical Investigation 105:1573-1587. (Cover Image)
- Yan Z, Zhang Y, **Duan D**, and Engelhardt JF. 2002. Trans-Splicing Vectors Expand the Utility of Adeno-Associated Virus for Gene Therapy. Proceeding of National Academy of Science 97:6716-6721. (*commentary on this paper appeared in Proceeding of National Academy of Science 97(12):6239-6241, 2000).
- **Duan D**, Yue Y, Yan Z, and Engelhardt JF. 2000. A New Dual Vector Approach to Enhance Recombinant AAV Mediated Gene Expression Through Intermolecular Cisactivation. Nature Medicine. 6:595-598 2000). (*commentary on this paper appeared in Nature Biotechnology 18(5):497-498.
- Walters RW, **Duan D**, Engelhardt JF, and Welsh MJ. 2000. Incorporation of AdenoAssociated Virus in a Calcium Phosphate Coprecipitate Improves Gene Transfer to Airway Epithelia In Vitro and In Vivo. Journal of Virology 74:535-540.