

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

2023

- Morales ED, Yue Y, Watkins TB, Han J, Pan X, Gibson AM, HB, B-E O, Yao G, Makarewich CA, Babu GJ, **Duan D**. Dwarf open reading frame (DWORF) gene therapy ameliorated Duchenne muscular dystrophy cardiomyopathy in aged mdx mice. **Journal of American Heart Association (JAHA)**, 2023. In-press
- [Chamberlain JS, Robb M, Braun S, Brown KJ, Danos O, Ganot A, Gonzalez-Alegre P, Hunter N, McDonald C, Morris C, Tobolowsky M, Wagner KR, Ziolkowski O, Duan D](#). Micro-dystrophin expression as a surrogate endpoint for Duchenne muscular dystrophy clinical trials. **Human Gene Therapy** 2023. In-press.
- [Wasala LP, Watkin TB, Wasala NB, Burke MJ, Yue Y, Lai Y, Yao G, Duan D](#). The implication of hinge 1 and hinge 4 in micro-dystrophin gene therapy for Duchenne muscular dystrophy. **Human Gene Therapy** 2023. In-press
- [Wasala NB, Yue Y, Hu B, Shin J-H, Yao G, Duan D](#). Life-long outcomes of systemic AAV micro-dystrophin gene therapy in a murine Duchenne muscular dystrophy model. **Human Gene Therapy** 2023. In-press
- [Birch SM, Law MW, Conlon TJ, Guo L-J, Crudele JM, Hawkins EC, Nghiem PP, Ahn M, Meng H, Beatka MJ, Fickau BA, Prieto JC, Styner MA, Struharik MJ, Shanks C, Brown KJ, Golebiowski D, Bettis AK, Balog-Alvarez CJ, Clement N, Coleman KE, Corti M, Pan X, Hauschka SD, Gonzalez JP, Morris CA, Schneider JS, Duan D, Chamberlain JS, Byrne BJ, Kornegay JN](#). A blinded, placebo-controlled systemic gene therapy efficacy study in the GRMD model of Duchenne muscular dystrophy. **Science Translational Medicine** 15(677): eabo1815 2023.
- [Zalcman AR, Hakim CH, Lattimer J, Holland JR, Dodam JR, Duan D](#). MRI evaluation of gene therapy in the canine model of Duchenne muscular dystrophy. **Methods in Molecular Biology** 2587:339-352, 2023.
- [Hakim CH, Sandeep RP, Perez-Lopez D, Teixeira J, Herzog RW, Duan D](#). Assessment of the gene therapy immune response in the canine muscular dystrophy models. **Methods in Molecular Biology** 2587:353-375, 2023.

- [Hakim CH, Teixeira J, Leach SB, Duan D](#). *Physiological assessment of muscle, heart, and whole-body function in the canine model of Duchenne muscular dystrophy*. **Methods in Molecular Biology** 2587:67-103, **2023**.
- [Hakim CH, Perez-Lopez D, Burke M, Teixeira J, Duan D](#). *Molecular and biochemical assessment of gene therapy in the canine model of Duchenne muscular dystrophy*. **Methods in Molecular Biology** 2587:255-301, **2023**.
- [Hakim CH, Burke M, Teixeira J, Duan D](#). *Histological assessment of gene therapy in the canine DMD model*. **Methods in Molecular Biology** 2587:303-338, **2023**.

2022

- [Ebner J, Pan, X, Yue Y, Sideromenos S, Koenig X, Hilber K, Duan D](#). *No current rescue in dystrophic cardiac Purkinje fibers by micro-dystrophin*. **Circulation: Arrhythmia and Electrophysiology** 15(8): e011161, **2022**. PMID: 35917466; PMCID: PMC9396648; DOI: [10.1161/CIRCEP.122.011161](https://doi.org/10.1161/CIRCEP.122.011161).
- [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**, 29(6):333-345, 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* 145(18):e872-e873, 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* 33(9-10):518-528,, 2022. PMID: 35350865; DOI: [10.1089/hum.2021.130](https://doi.org/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, **2022**.

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- [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
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- [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

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