Science Advancement & Outreach
A DIVISION OF PETA

1536 16th St. N.W., Washington, DC 20036

December 18, 2025

Dear Muscular Dystrophy Coordinating Committee members:

I am writing on behalf of Science Advancement and Outreach, the biomedical research policy division of People for the Ethical Treatment of Animals, to request that the priorities in the 2025 ten-year strategic plan for Muscular Dystrophy (MD) research align with NIH's April 29th policy directive[1] to prioritize human-based research technologies and reduce the use of animals in NIH-funded studies.

1. **Divest from using animals in muscular dystrophy research**

Currently, there is no cure available for any form of MD, and existing treatments can only slow disease progression.[2] Clearly, the current research paradigm is not delivering the outcomes MD patients urgently need. Yet, the Muscular Dystrophy Coordinating Committee's (MDCC) draft Action Plan included several priorities centered on the use of animal models to study human MD.

For decades, animals, particularly dogs, have been used in experiments to develop drugs for Duchenne muscular dystrophy (DMD). Despite this, only a small number of treatments with limited applicability and ongoing safety concerns for patients have been approved.[3,4,5,6] The scientific limitations of DMD experiments on animals are well documented. Species-specific differences in muscle fiber composition, metabolism, and function, along with disparities in lifespan and disease manifestations, fundamentally undermine the study of human MD in other species.[7,8,9]

To rectify this approach and align with agency-wide goals, the 2025 Action Plan must explicitly replace references to animal models with human-based, animal-free approaches, such as those that rely on human cells, tissues, or other human-derived data.

2. **Expand training and support for researchers using non-animal methods**

We commend the MDCC for incorporating non-animal methods (NAMs) throughout the draft priorities, many of which are already being applied successfully in MD research. Published studies using MD-derived induced pluripotent stem cell models,[10] 3D skeletal muscle organoids,[11] engineered skeletal muscle tissues,[12,13,14] advanced brain imaging techniques,[15] and machine learning–based diagnostic tools[16] demonstrate the value of these approaches in elucidating disease mechanisms and improving diagnosis.

However, NAM researchers in the MD field require more support to further develop and validate these new, animal-free methods. Moreover, many MD researchers have been trained primarily in

animal-based techniques and face significant barriers to learning and adopting new approaches. The MDCC's 2025 Action Plan can help address this gap by developing new training opportunities—particularly for students and early-career researchers—that are grounded in human-based MD research. While the inclusion of training in artificial intelligence and machine learning is an important step, the scope should be broadened to include other NAMs mentioned in the draft priorities and above. Existing resources can support these efforts.[17,18] Expanding training and institutional support for NAMs would equip researchers with the expertise needed to implement cutting-edge, human-relevant approaches, thereby improving the rigor, reproducibility, and overall quality of MD research.

By supporting these initiatives, the MDCC can help ensure that its next strategic plan delivers translational advances and tangible improvements in the lives of people living with MD.

Thank you for considering these recommendations. Our scientists are available to meet with the committee to discuss these requests and more.

Sincerely,

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[1] National Institutes of Health. NIH to prioritize human-based research technologies. April 29, 2025. Accessed December 15, 2025. https://www.nih.gov/news-events/news-releases/nih-prioritize-human-based-research-technologies
[2] La Pelusa A, Asuncion R, Kentris M. *Muscular Dystrophy*. Treasure Island (FL): StatPearls Publishing; 2024.
[3] Lowe D. Sarepta. Why? *Science*. June 21, 2024. Accessed December 15, 2025. https://www.science.org/content/blog-post/sarepta-why
[4] Liu A. FDA takes U-turn on Sarepta's Elevidys, backing Duchenne gene therapy again in ambulatory patients. July 28, 2025. Accessed December 15, 2025. https://www.fiercepharma.com/pharma/fda-takes-u-turn-sareptas-elevidys-backing-duchenne-gene-therapy-again-ambulatory-patients
[5] Basile LM. FDA's approval of Duchenne muscular dystrophy drug under scrutiny: clearance based on limited study, plus it can cost $1M yearly. August 25, 2023. Accessed December 15, 2025. https://www.mdlinx.com/article/fdas-approval-of-duchenne-muscular-dystrophy-drug-under-scrutiny-clearance-based-on-limited-study/24WdAGAT6thDLgWUBSpJ3
[6] U. S. Food & Drug Administration. FDA requests Sarepta Therapeutics suspend distribution of Elevidys and places clinical trials on hold for multiple gene therapy products following 3 deaths. Accessed December 15, 2025. https://www.fda.gov/news-events/press-announcements/fda-requests-sarepta-therapeutics-suspend-distribution-elevidys-and-places-clinical-trials-hold
[7] Dunayer E. Scientific problems with animal models of Duchenne muscular dystrophy. Perspectives on Animal Research. 1991. Accessed December 15, 2025. http://www.safermedicines.org/reports/Perspectives/vol_3_1991/duchenne_md.html
[8] Hu X, Charles JP, Akay T, Hutchinson JR, Blemker SS. Are mice good models for human neuromuscular disease? Comparing muscle excursions in walking between mice and humans. *Skelet Muscle*. 2017;7(1):26. doi:10.1186/s13395-017-0143-9
[9] McGreevy JW, Hakim CH, McIntosh MA, Duan D. Animal models of Duchenne muscular dystrophy: from basic mechanisms to gene therapy. *Dis Model Mech*. 2015;8(3):195-213. doi:10.1242/dmm.018424
[10] Eguchi A, Gonzalez AFGS, Torres-Bigio SI, et al. TRF2 rescues telomere attrition and prolongs cell survival in Duchenne muscular dystrophy cardiomyocytes derived from human iPSCs. *Proc Natl Acad Sci*. 2023;120(6):e2209967120. doi:10.1073/pnas.2209967120
[11] Shahriyari M, Islam MR, Sakib SM, et al. Engineered skeletal muscle recapitulates human muscle development, regeneration and dystrophy. *J Cachexia Sarcopenia Muscle*. 2022;13(6):3106-3121. doi:10.1002/jcsm.13094
[12] Shahriyari, *et al.*
[13] In 'T Groen SLM, Franken M, Bock T, Krüger M, De Greef JC, Pijnappel WWMP. A knock down strategy for rapid, generic, and versatile modelling of muscular dystrophies in 3D-tissue-engineered-skeletal muscle. *Skelet Muscle*. 2024;14(1):3. doi:10.1186/s13395-024-00335-5
[14] Franken M, Van Der Wal E, Zheng D, et al. Three-dimensional tissue engineered skeletal muscle modelling facioscapulohumeral muscular dystrophy. *Brain*. 2025;148(5):1723-1739. doi:10.1093/brain/awae379
[15] Maki H, Mori-Yoshimura M, Matsuda H, et al. Brain abnormalities in Becker muscular dystrophy: evaluation by voxel-based DTI and morphometric analysis. *Am J Neuroradiol*. 2023;44(12):1405-1410. doi:10.3174/ajnr.A8041
[16] Chen T, Zhu H, Hu Y, et al. Machine learning-based radiomics using MRI to differentiate early-stage Duchenne and Becker muscular dystrophy in children. *BMC Musculoskelet Disord*. 2025;26(1):287. doi:10.1186/s12891-025-08538-7
[17] Joint Research Centre (European Commission). EU Reference Laboratory for alternatives to animal testing (EURL ECVAM) - European Commission. 2025. Accessed December 15, 2025. https://joint-research-centre.ec.europa.eu/projects-and-activities/reference-and-measurement/european-union-reference-laboratories/eu-reference-laboratory-alternatives-animal-testing-eurl-ecvam_en
[18] Physicians Committee for Responsible Medicine. Engaging researchers in animal-free 21st century science. PCRM.org. 2025. Accessed December 15, 2025. https://www.pcrm.org/ethical-science/ethical-education-and-training/ERA21