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Corresponding author: Chris Wier (medinfo@sarepta.com)

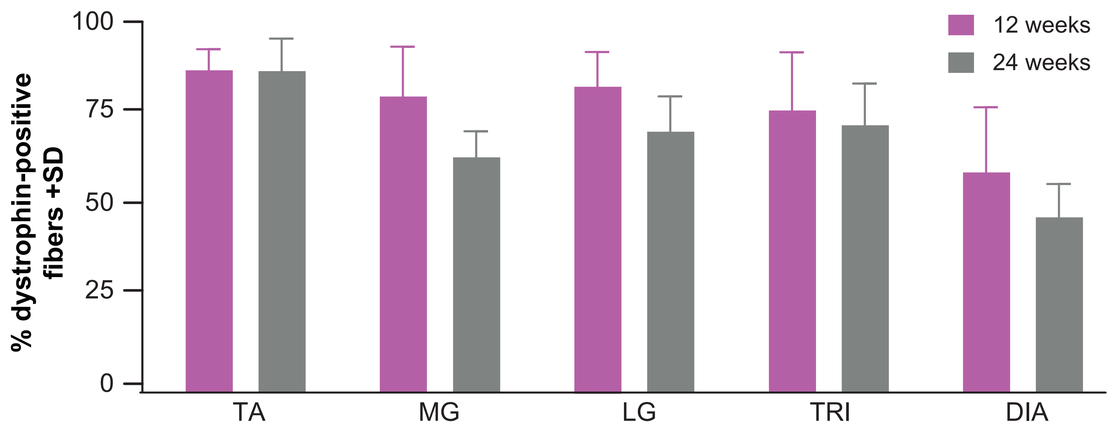
Evaluating pharmacology and efficacy of delandistrogene moxeparvovec in DMD^{mdx} rats

RA Potter,[1] C Wier,[1]* G Cooper-Olson,[1] E Wheeler,[1] ET Anderbery,[1] A Kempton,[1] L Clements,[1] K Adegboye,[1] A Haile,[1] E Peterson,[1] LR Rodino-Klapac[1]

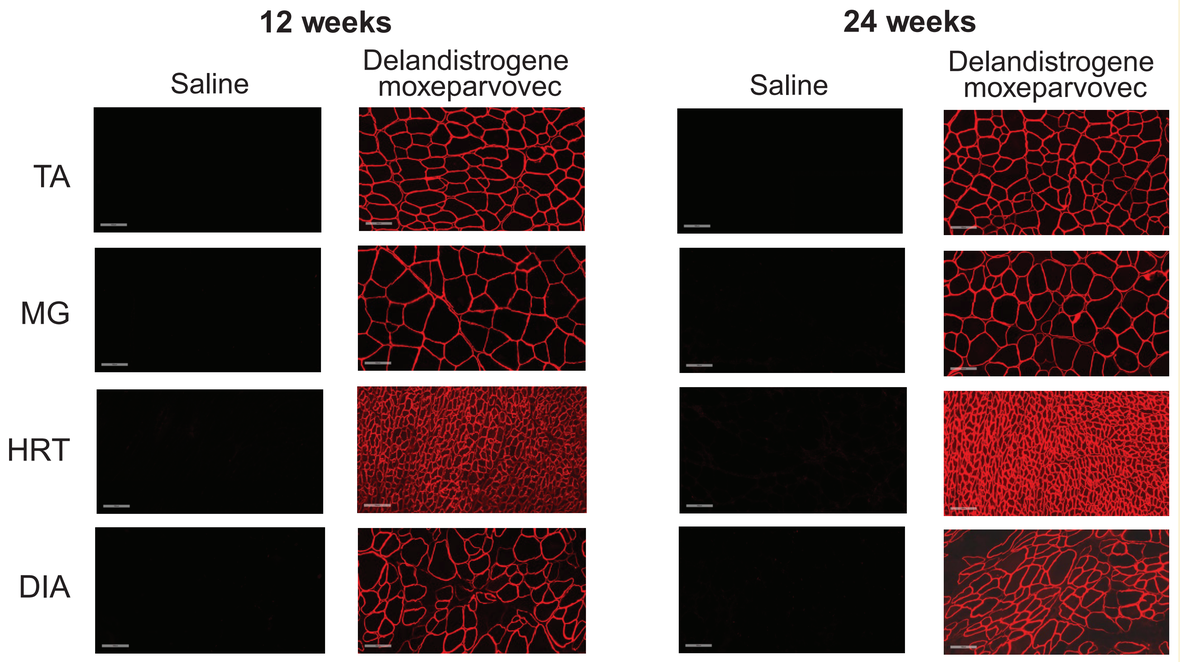
[1]Sarepta Therapeutics, Inc., Cambridge, MA, USA
*Presenter

SUPPLEMENTARY MATERIALS

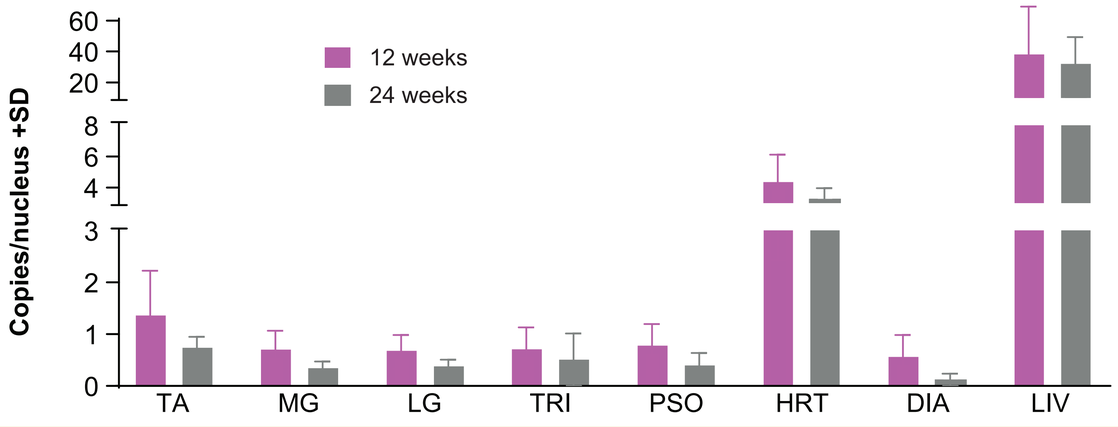
Quantification of SRP-9001 dystrophin-positive fibers showed no significant differences within the same tissue types between 12 and 24 weeks following treatment with delandistrogene moxeparvovec



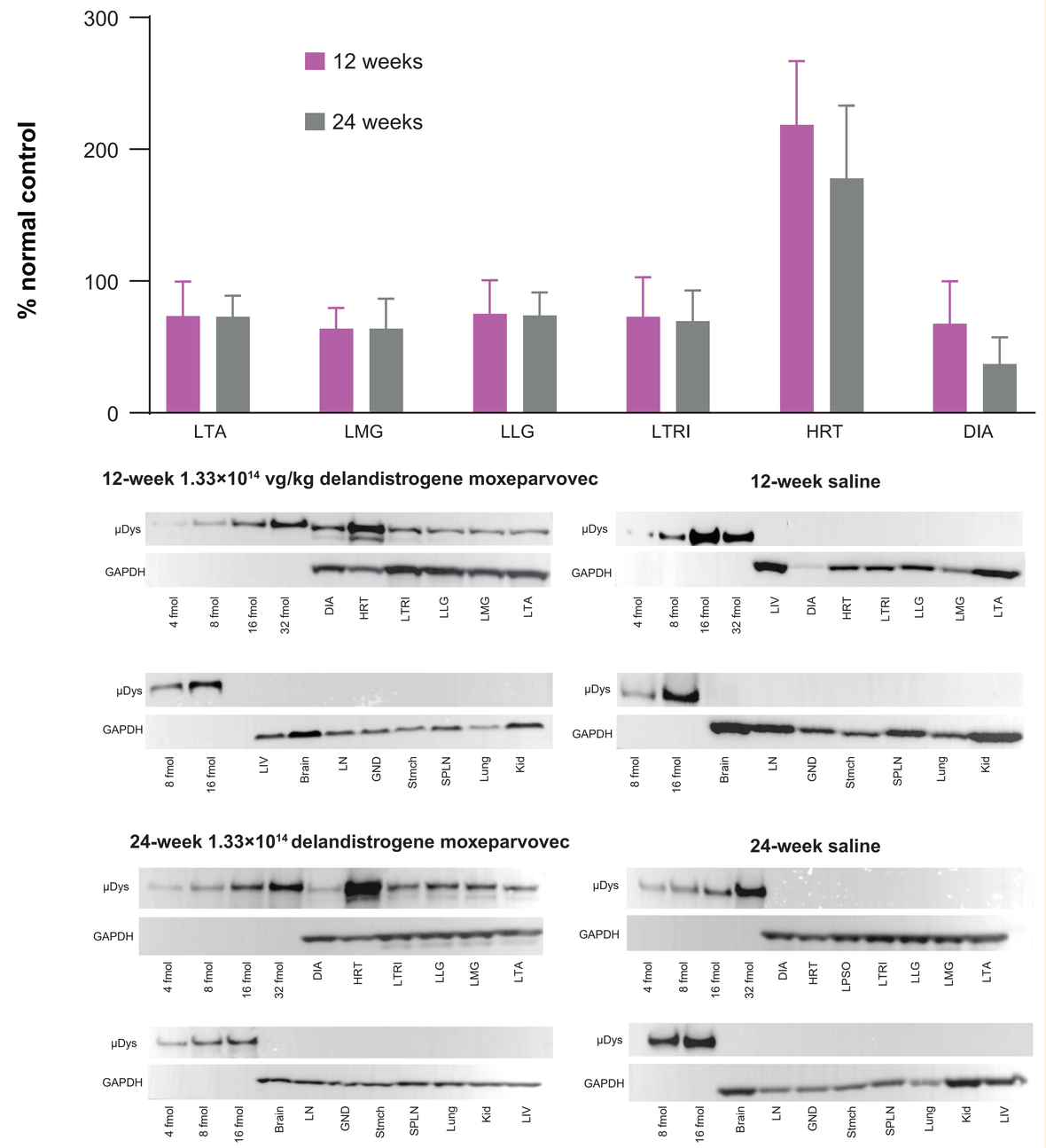
IF demonstrated SRP-9001 dystrophin localization in muscle at 12 and 24 weeks following treatment with delandistrogene moxeparvovec



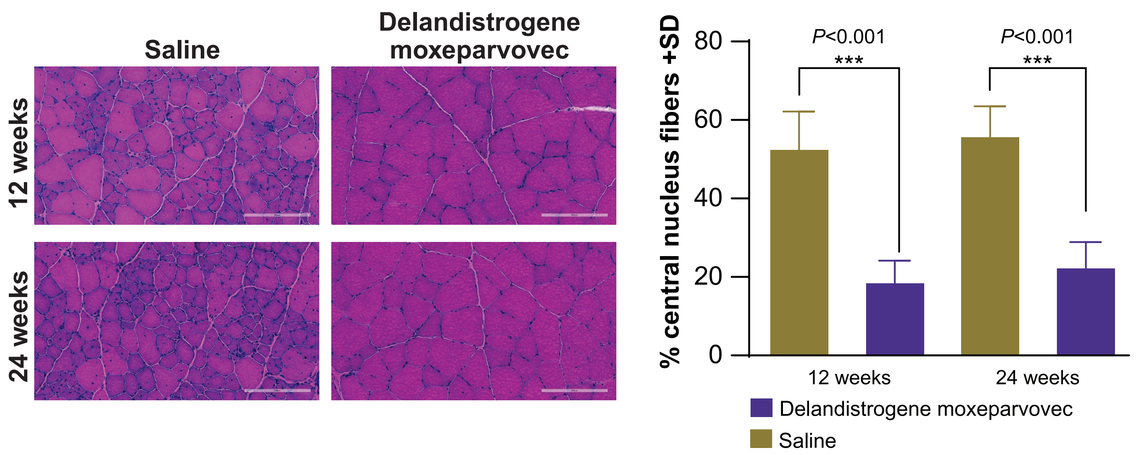
The SRP-9001 dystrophin transgene was broadly distributed across skeletal muscle, the diaphragm, and the heart in DMD^{mdx} rats (ddPCR)



Western blot quantification of SRP-9001 dystrophin protein expression in DMD^{mdx} rats

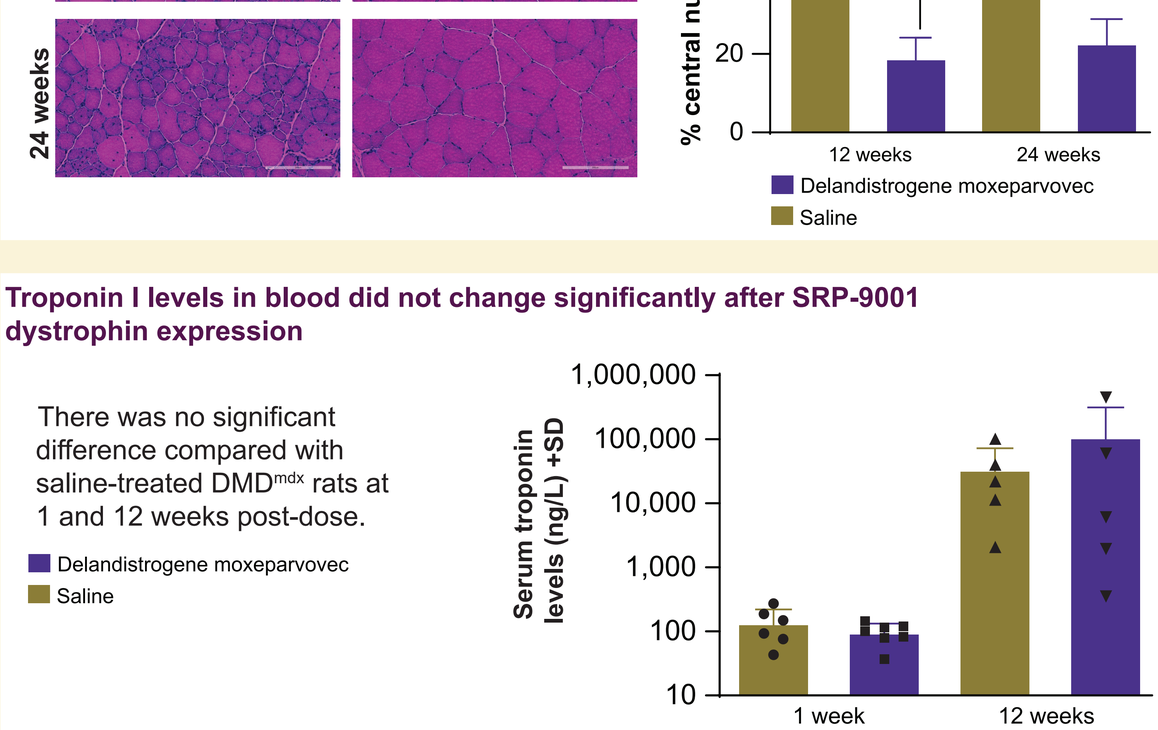


H&E demonstrated improved muscle histology (decreased central nucleation) in the gastrocnemius following treatment with delandistrogene moxeparvovec



Troponin I levels in blood did not change significantly after SRP-9001 dystrophin expression

There was no significant difference compared with saline-treated DMD^{mdx} rats at 1 and 12 weeks post-dose.



ABBREVIATIONS

µDys, microdystrophin; ddPCR, droplet digital polymerase chain reaction; DIA, diaphragm; DMD, Duchenne muscular dystrophy; GAPDH, glyceraldehyde-3-phosphate dehydrogenase; GND, gonad; H&E, hematoxylin and eosin; HRT, heart; IF, immunofluorescence; Kid, kidney; LG, left gastrocnemius; LIV, liver; LLG, left lateral gastrocnemius; LMG, left medial gastrocnemius; LN, lymph node; LPSO, left psoas; LTA, left tibialis anterior; LTRI, left tricep; mdx, muscular dystrophy X-linked; MG, medial gastrocnemius; PSO, psoas; SD, standard deviation; SPLN, spleen; Stmch, stomach; TA, tibialis anterior; TRI, tricep.

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This research used DMD^{mdx} rats, which were generated and characterized in the following publication: Larcher T, et al. Characterization of dystrophin deficient rats: a new model for Duchenne muscular dystrophy. *PLoS One.* 2014; 9:e110371. These data are an encore of data first presented by RA Potter at the 27th International Annual Congress of the World Muscle Society (WMS) 2022.