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Corrigendum to “Randomized phase 2 trial and open-label extension of domagrozumab in Duchenne muscular dystrophy” [Neuromuscular Disorders, Vol. 30 (6) 2020, 492-502] (Neuromuscular Disorders (2020) 30(6) (492–502), (S0960896620301188), (10.1016/j.nmd.2020.05.002))

Kathryn R. Wagner
Johns Hopkins School of Medicine

Hoda Z. Abdel-Hamid
University of Pittsburgh

Jean K. Mah
Cumming School of Medicine

Craig Campbell
Western University, craig.campbell@lhsc.on.ca

Michela Guglieri
The Newcastle Upon Tyne Hospitals NHS Foundation Trust

See next page for additional authors

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Corrigendum

Corrigendum to "Randomized phase 2 trial and open-label extension of domagrozumab in Duchenne muscular dystrophy" [Neuromuscular Disorders, Vol. 30 (6) 2020, 492-502]

Kathryn R. Wagner[a,*], Hoda Z. Abdel-Hamid[b], Jean K. Mah[c], Craig Campbell[d], Michela Guglieri[e], Francesco Muntoni[f], Yasuhiro Takeshima[g], Craig M. McDonald[h], Anna Kostera-Pruszczyk[i], Peter Karachunski[j], Russell J. Butterfield[k], Eugenio Mercuri[l,m], Chiara Fiorillo[n], Enrico S. Bertini[o], Cuixia Tian[p], Jeffery Statland[q], Alesia B. Sadosky[r], Vivek S. Purohit[s], Sarah P. Sherlock[s], Jeffrey P. Palmer[s], Michael Binks[s], Lawrence Charnas[s], Shannon Marraffino[s], Brenda L. Wong[t]

[a] *Center for Genetic Muscle Disorders, Baltimore, Kennedy Krieger Institute, Departments of Neurology and Neuroscience, Johns Hopkins School of Medicine, Baltimore, MD, USA*
[b] *University of Pittsburgh, Pittsburgh, PA, USA*
[c] *Cumming School of Medicine, University of Calgary, Alberta Children's Hospital, Calgary, AB, Canada*
[d] *Department of Pediatrics, Clinical Neurological Sciences and Epidemiology, University of Western Ontario, Children's Hospital London Health Sciences Centre, London, ON, Canada*
[e] *The John Walton Muscular Dystrophy Research Centre, Newcastle University and Newcastle Hospitals NHS Foundation Trust, Newcastle upon Tyne, UK*
[f] *NIHR Great Ormond Street Hospital Biomedical Research Centre, Great Ormond Street Institute of Child Health, University College London, & Great Ormond Street Hospital Trust, London, UK*
[g] *Hyogo College of Medicine, Nishinomiya, Hyogo, Japan*
[h] *Lawson Health Research Institute: Children's Hospital, London, ON, Canada*
[i] *Department of Neurology, Medical University of Warsaw, Warsaw, Poland*
[j] *University of Minnesota, Minneapolis, MN, USA*
[k] *University of Utah School of Medicine, Salt Lake City, UT, USA*
[l] *Paediatric Neurology, Catholic University, Rome, Italy*
[m] *Centro Clinico Nemo, Fondazione Policlinico Universitario Agostino Gemelli IRCCS, Rome, Italy*
[n] *IRCCS Giannina Gaslini, Genova, Italy*
[o] *Bambino Gesu Children's Research Hospital IRCCS, Rome, Italy*
[p] *Cincinnati Children's Hospital Medical Center; College of Medicine University of Cincinnati, Cincinnati, OH, USA*
[q] *University of Kansas Medical Center, Kansas City, KS, USA*
[r] *Pfizer Inc, New York, NY, USA*
[s] *Pfizer Inc, Cambridge, MA, USA*
[t] *University of Massachusetts Medical School, Worcester, MA, USA*

This article reported on the results from a phase 2 trial of domagrozumab and its open-label extension in patients with Duchenne muscular dystrophy (Clinicaltrials.gov identifiers: NCT02310763 and NCT02907619). The manuscript also provided results on two secondary endpoints for magnetic resonance imaging (MRI), muscle volume and muscle volume index.

The authors regret that, following publication of the results and in preparation for a separate publication on MRI results from this trial, the MRI images were reviewed and segmentation errors were identified. As a result, the team worked to (1) Perform a rigorous quality inspection of all analysed data; (2) Identify cases where there were incorrect segmentations; (3) correct segmentation errors; (4) Re-analyse all data with correct segmentation.

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* Corresponding author: Center for Genetic Muscle Disorders, Kennedy, Krieger Institute, 707 N Broadway, Baltimore, MD 21205, USA.
E-mail address: wagnerk@kennedykrieger.org (K.R. Wagner).

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Using the updated MRI data, the MMRM analysis showed there was a change in the significance of secondary endpoints evaluating *Thigh Muscle Volume* and *Muscle Volume Index*. No significant differences between treatment groups in muscle volume measures were found in the original analysis.

These results have not altered the overall interpretation of the study results but do necessitate revisions to the article. These data confirm that the trial design and execution adequately tested the hypothesis that myostatin inhibition would slow or delay the loss of function in patients with Duchenne muscular dystrophy (DMD). The increase in muscle volume observed by MRI in patients with DMD treated with domagrozumab is in accordance with mechanism of action for domagrozumab, which targets myostatin, a negative regulator of muscle growth. The increase in muscle volume did not lead to a clinical benefit in patients with DMD. The primary endpoint (4 stair climb) did not meet statistical significance, nor did the other functional tests. The study was terminated due to lack of efficacy.

Full details of the needed revisions are as follows:

1. In the results section 3.6 (page 8, second paragraph), we reported no significant differences in mean percent change from baseline between domagrozumab and placebo for both muscle volume and muscle volume index.

This paragraph was replaced with the following text:

"There was a significant difference between domagrozumab and placebo in the mean percent change from baseline in thigh muscle volume at Week 17 (difference 2.945%, P=0.0087) and Week 49 (differences 4.087%, P=0.0298), and in muscle volume index at Week 33 (difference 2.612%, P=0.0376) and Week 49 (differences3.208%, P=0.0411)."

2. In the discussion (page 9), the following sentence, "Although neither muscle volume nor muscle volume index measures were statistically significant in this study, they are both consistent with a potential anabolic effect." was replaced with,

"The increase in muscle volume observed on MRI in patients with DMD treated with domagrozumab, is in accordance with mechanism of action for this compound which targets myostatin, a negative regulator of muscle growth. However, the increase in muscle volume did not lead to a clinical benefit (improved function) in patients with DMD."

3. In view of the correction to the Results section, this is now reflected in the abstract which has changed to read: "There were no significant between-group differences in secondary clinical endpoints, except for the thigh muscle volume and muscle volume index measures (P<0.05)."

The authors would like to apologise for any inconvenience caused.