

## Safety and Efficacy of Apitegromab in Patients With Spinal Muscular Atrophy Types 2 and 3

### The Phase 2 TOPAZ Study

*Neurology*<sup>®</sup> 2024;103:e209519. doi:10.1212/WNL.0000000000209519

In the Research Article “Safety and Efficacy of Apitegromab in Patients With Spinal Muscular Atrophy Types 2 and 3: The Phase 2 TOPAZ Study” and its associated short-form version by Crawford et al,<sup>1</sup> some data were incorrectly reported in Tables 2–4 and the short-form table. In Table 2, mean age at symptom onset, y (min, max) for Cohort 1 (Apitegromab 20 mg/kg), the minimum should be 0.6. In Table 3 and its abbreviated version in the short form, the n (ITT population) number for Cohort 2 should be 14. In Table 4, ≥Grade 3 TEAEs for the Apitegromab 20 mg/kg group, the percentage should be 6.3.

The corrected tables follow. None of these changes affect the overall interpretation or conclusions of this article. The authors regret the errors.

### Reference

1. Crawford TO, Darras BT, Day JW, et al. Safety and efficacy of apitegromab in patients with spinal muscular atrophy types 2 and 3: the phase 2 TOPAZ study. *Neurology*. 2024;102(5):e209151. doi:10.1212/WNL.0000000000209151

**Table 2** Demographics and Baseline Characteristics

	Cohort 1		Cohort 2	Cohort 3	
	Ambulatory participants Age 5–21 y, RHS scores ≤63		Nonambulatory participants Age 5–21 y, HFMSE scores ≥10	Nonambulatory participants Age ≥2 y, HFMSE scores ≥10	
	Apitegromab 20 mg/kg	Apitegromab 20 mg/kg + nusinersen	Apitegromab 20 mg/kg + nusinersen	Apitegromab 2 mg/kg + nusinersen	Apitegromab 20 mg/kg + nusinersen
n (baseline population)	11	12	15	10	10
Mean age, y (min, max)	12.1 (7, 19)	13.1 (7, 21)	11.7 (8, 19)	4.1 (2, 6)	3.8 (2, 6)
Mean age at diagnosis, y (min, max)	5.9 (2, 15)	4.5 (2, 15)	3.1 (1, 16)	1.2 (1, 2)	1.2 (1, 3)
Mean age at symptom onset, y (min, max)	3.7 (0.6, 11)	3.0 (0.7, 14)	1.4 (0.5, 2)	0.9 (0.5, 1.2)	1.0 (0.5, 3.5)
Female (%)	73	58	53	30	50
SMN2 gene copies, n (%) <sup>a</sup>					
2	1 (9)	0	0	1 (10)	1 (10)
3	4 (36)	9 (75)	11 (73)	8 (80)	8 (80)
4	4 (36)	1 (8)	2 (13)	1 (10)	0
Mean nusinersen maintenance doses at baseline (min, max) <sup>b</sup>	N/A	3.9 (2, 6)	4.8 (2, 9)		4.8 (1, 7)
Total duration nusinersen treatment at baseline mo (min, max)	N/A	19.9 (12, 28)	24.2 (12, 39)		24.0 (10, 34)
Discontinued	0	1 <sup>c</sup>	0	0	0
Mean baseline RHS score (min, max)	47.6 (26, 63)	51.3 (43, 62)	N/A	N/A	N/A
Mean baseline HFMSE score (min, max)	N/A	N/A	22.7 (13, 39)	26.1 (12, 44)	23.5 (14, 42)
Mean baseline RULM score (min, max)	N/A	N/A	26.6 (19, 34)	25.0 (18, 34)	22.6 (15, 33)

Abbreviations: HFMSE = Hammersmith Functional Motor Scale Expanded; N/A = not applicable; RHS = Revised Hammersmith Scale; RULM = revised upper limb module.

<sup>a</sup> Data not available for all participants.

<sup>b</sup> Maintenance dose was used as a surrogate for duration of nusinersen exposure at screening.

<sup>c</sup> Participant discontinued the trial for reasons unrelated to study drug.

**Table 3** Primary Efficacy Analysis, ITT Population

	Cohort 1		Cohort 2	Cohort 3	
	Ambulatory participants Age 5–21 y (RHS)		Nonambulatory participants Age 5–21 y (HFMSE)	Nonambulatory participants Age ≥2 y (HFMSE)	
	Apitegromab 20 mg/kg	Apitegromab 20 mg/kg + nusinersen	Apitegromab 20 mg/kg + nusinersen	Apitegromab 2 mg/kg + nusinersen	Apitegromab 20 mg/kg + nusinersen
n (ITT population)	11	12 <sup>a</sup>	14 <sup>b</sup>	9 <sup>b</sup>	8 <sup>b</sup>
Primary efficacy end point	Mean change in baseline RHS scores		Mean change in baseline HFMSE scores	Mean change in baseline HFMSE scores	
Mean change in baseline score (SD) (95% CI) <sup>c</sup>	–0.4 (5.20) (–3.9–3.1)	–0.3 (2.67) (–2.0–1.4)	0.6 (3.50) (–1.4–2.7)	5.3 (8.93) (–1.5–12.2)	7.1 (6.42) (1.8–12.5)
Participants achieving ≥1-point increase, n (%) (95% CI)	4 (36.4) (10.93–69.21)	5 (41.7) (15.17–72.33)	9 (64.3) (35.14–87.24)	7 (77.8) (39.99–97.19)	7 (87.5) (47.35–99.68)
Participants achieving ≥3-point increase, n (%) (95% CI)	3 (27.3) (6.02–60.97)	2 (16.7) (2.09–48.41)	4 (28.6) (8.39–58.10)	5 (55.6) (21.20–86.30)	5 (62.5) (24.49–91.48)
Participants achieving ≥5-point increase, n (%) (95% CI)	1 (9.1) (0.23–41.28)	0 (0.00–26.46)	2 (14.3) (1.78–42.81)	5 (55.6) (21.20–86.30)	5 (62.5) (24.49–91.48)

Abbreviations: COVID-19 = coronavirus disease 2019; HFMSE = Hammersmith Functional Motor Scale Expanded; ITT = intent-to-treat; RHS = Revised Hammersmith Scale.

<sup>a</sup> One participant in cohort 1 received concomitant treatment with an acetylcholinesterase inhibitor before and during the trial and was excluded from the >per-protocol analysis because of this protocol violation.

<sup>b</sup> Four participants missed 3 doses of apitegromab during the 12-mo treatment period (cohort 2, n = 1; cohort 3, n = 3) because of COVID-19–related site access restrictions and were not included in the primary analysis.

<sup>c</sup> Mean change from baseline presented is to the month 12 end point.

**Table 4** Safety Results, All Participants

TEAE, n (%)	Apitegromab 2 mg/kg (n = 10)			Apitegromab 20 mg/kg (n = 48)			Total (N = 58)		
Any TEAE	9 (90.0)			44 (91.7)			53 (91.4)		
Serious TEAEs <sup>a</sup>	1 (10.0)			4 (8.3)			5 (8.6)		
TEAEs leading to discontinuation <sup>a</sup>	0			1 (2.1)			1 (1.7)		
≥Grade 3 TEAEs <sup>a</sup>	0			3 (6.3)			3 (5.2)		
Preferred term, n (%) <sup>b,c</sup>	Grade 1	Grade 2	Total <sup>d</sup>	Grade 1	Grade 2	Total <sup>d</sup>	Grade 1	Grade 2	Total <sup>d</sup>
Headache	2 (20.0)	0	2 (20.0)	11 (22.9)	1 (2.1)	12 (25.0)	13 (22.4)	1 (1.7)	14 (24.1)
Upper respiratory tract infection	2 (20.0)	1 (10.0)	3 (30.0)	7 (14.6)	3 (6.3)	10 (20.8)	9 (15.5)	4 (6.9)	13 (22.4)
Pyrexia	1 (10.0)	2 (20.0)	3 (30.0)	6 (12.5)	4 (8.3)	10 (20.8)	7 (12.1)	6 (10.3)	13 (22.4)
Cough	0	3 (30.0)	3 (30.0)	8 (16.7)	2 (4.2)	10 (20.8)	8 (13.8)	5 (8.6)	13 (22.4)
Nasopharyngitis	1 (10.0)	2 (20.0)	3 (30.0)	8 (16.7)	1 (2.1)	9 (18.8)	9 (15.5)	3 (5.2)	12 (20.7)

Abbreviation: TEAE = treatment-emergent adverse event.

<sup>a</sup> Determined to be unrelated to treatment.

<sup>b</sup> Medical dictionary for regulatory activities.

<sup>c</sup> Most frequently reported.

<sup>d</sup> Combined, pooled, prespecified, and post hoc.

# Neurologic Deterioration in Patients With Acute Ischemic Stroke or Transient Ischemic Attack

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In the Article “Neurologic Deterioration in Patients With Acute Ischemic Stroke or Transient Ischemic Attack” by Park et al.,<sup>1</sup> the following link should have been provided to access the supplementary materials: [datadryad.org/stash/dataset/doi:10.5061/dryad.3tx95x6cb](https://datadryad.org/stash/dataset/doi:10.5061/dryad.3tx95x6cb). The authors regret the omission.

## Reference

1. Park TH, Lee J-K, Park M-S, et al. Neurologic deterioration in patients with acute ischemic stroke or transient ischemic attack. *Neurology*. 2020;95(16):e2178-e2191. doi:10.1212/WNL.0000000000010603

## Corrections to Preprint Server Information

*Neurology*® 2024;103:e209573. doi:10.1212/WNL.0000000000209573

Because of an error in the *Neurology* editorial office’s content management system, several papers were published without their preprint server information. The preprint information, which should have been listed in the Publication History section, is provided for each paper below.

- “Association Between Diseases and Symptoms Diagnosed in Primary Care and the Subsequent Specific Risk of Multiple Sclerosis” by Guinebretiere et al.<sup>1</sup>: Previously published in MedRxiv (doi.org/10.1101/2022.11.16.22282386).
- “Association of Blood-Based DNA Methylation Markers With Late-Onset Alzheimer Disease: A Potential Diagnostic Approach” by Acha et al.<sup>2</sup>: Previously published in Research Square (doi.org/10.21203/rs.3.rs-2385191/v1).
- “Association of Poor Oral Health With Neuroimaging Markers of White Matter Injury in Middle-Aged Participants in the UK Biobank” by Rivier et al.<sup>3</sup>: Previously published in MedRxiv (doi.org/10.1101/2023.03.18.23287435).
- “Association of PM2.5 Exposure and Alzheimer Disease Pathology in Brain Bank Donors—Effect Modification by APOE Genotype” by Christensen et al.<sup>4</sup>: Previously published in MedRxiv (doi.org/10.1101/2023.04.07.23288288).
- “Comparative Effectiveness of Natalizumab, Fingolimod, and Injectable Therapies in Pediatric-Onset Multiple Sclerosis: A Registry-Based Study” by Spelman et al.<sup>5</sup>: Previously published in MedRxiv (doi.org/10.1101/2022.10.12.22280969).
- “Evaluation of ATNPD Framework and Biofluid Markers to Predict Cognitive Decline in Early Parkinson Disease” by Cousins et al.<sup>6</sup>: Previously published in MedRxiv (doi.org/10.1101/2023.04.21.23288930v1).
- “Interplay Between Chronic Kidney Disease, Hypertension, and Stroke: Insights From a Multivariable Mendelian Randomization Analysis” by Kelly et al.<sup>7</sup>: Previously published in MedRxiv (doi.org/10.1101/2022.09.14.22279923).
- “Single Nucleotide Polymorphisms Associated With Motor Recovery in Patients With Nondisabling Stroke:GWAS Study” by Aldridge et al.<sup>8</sup>: Previously published in MedRxiv (doi.org/10.1101/2023.02.16.23286040).
- “Trends in Cognitive Function Before and After Diabetes Onset: The China Health and Retirement Longitudinal Study” by Chen et al.<sup>9</sup>: Previously published in MedRxiv (doi.org/10.1101/2023.07.02.23292154).

The editorial office regrets the omissions.

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## Corrections to Received Date Information

*Neurology*® 2024;103:e209596. doi:10.1212/WNL.0000000000209596

Due to an error in the *Neurology* editorial office's content management system, several papers were published with incorrect received date information. The correct received dates, which should have been listed in the Publication History section, are provided for each paper below.

- “Association Between Hippocampal Volumes and Cognition in Cerebral Amyloid Angiopathy” by Perosa et al.<sup>1</sup>: Received by *Neurology* July 8, 2022.
- “Association of Blood-Based DNA Methylation Markers With Late-Onset Alzheimer Disease” by Acha et al.<sup>2</sup>: Received by *Neurology* August 28, 2022.
- “Association of Glycemic Variability With Imaging Markers of Vascular Burden,  $\beta$ -Amyloid, Brain Atrophy, and Cognitive Impairment” by Jang et al.<sup>3</sup>: Received by *Neurology* January 17, 2023.
- “Association of Motor Function With Cognitive Trajectories and Structural Brain Differences” by Wang et al.<sup>4</sup>: Received by *Neurology* December 17, 2022.
- “Association of Neighborhood-Level Socioeconomic Factors With Delay to Hospital Arrival in Patients With Acute Stroke” by Forman et al.<sup>5</sup>: Received by *Neurology* January 10, 2023.
- “Association of Spinal Cord Atrophy and Brain Paramagnetic Rim Lesions With Progression Independent of Relapse Activity in People With MS” by Cagol et al.<sup>6</sup>: Received by *Neurology* January 19, 2023.
- “Clinical Reasoning: A 74-Year-Old Woman Presenting With Monocular Ptosis and Binocular Diplopia” by Liu et al.<sup>7</sup>: Received by *Neurology* November 16, 2022.
- “Clinical Reasoning: A Woman With Progressive Painless Sequential Monocular Vision Loss” by Ditrapani et al.<sup>8</sup>: Received by *Neurology* August 8, 2022.
- “Comparison of 2 Methods for Estimating Multiple Sclerosis–Related Mortality” by Rollot et al.<sup>9</sup>: Received by *Neurology* October 20, 2022.
- “Cumulative Use of Proton Pump Inhibitors and Risk of Dementia: The Atherosclerosis Risk in Communities Study” by Northuis et al.<sup>10</sup>: Received by *Neurology* January 4, 2023.
- “Effectiveness of Yoga Intervention in Reducing Felt Stigma in Adults With Epilepsy: A Randomized Controlled Trial” by Kaur et al.<sup>11</sup>: Received by *Neurology* October 14, 2022.
- “[18F]DPA-714 PET Imaging in the Presurgical Evaluation of Patients With Drug-Resistant Focal Epilepsy” by Cheval et al.<sup>12</sup>: Received by *Neurology* November 26, 2022.
- “Fine Particulate Matter and Parkinson Disease Risk Among Medicare Beneficiaries” by Krzyzanowski et al.<sup>13</sup>: Received by *Neurology* October 30, 2022.
- “Interplay Between Chronic Kidney Disease, Hypertension, and Stroke” by Kelly et al.<sup>14</sup>: Received by *Neurology* September 14, 2022.
- “Measures of Aging Biology in Saliva and Blood as Novel Biomarkers for Stroke and Heart Disease in Older Adults” by Waziry et al.<sup>15</sup>: Received by *Neurology* November 28, 2022.

- “Molecular and Phenotypic Characterization of the RORB-Related Disorder” by Gokce-Samar et al.<sup>16</sup>: Received by *Neurology* January 5, 2023.
- “Mortality and Causes of Death in Children With Cerebral Palsy With Scoliosis Treated With and Without Surgery” by Ahonen et al.<sup>17</sup>: Received by *Neurology* February 13, 2023.
- “Movement Disorders in Patients With Genetic Developmental and Epileptic Encephalopathies” by van der Veen et al.<sup>18</sup>: Received by *Neurology* February 17, 2023.
- “MRI vs CT for Baseline Imaging Evaluation in Acute Large Artery Ischemic Stroke: A Subanalysis of the SWIFT-DIRECT Trial” by Fladt et al.<sup>19</sup>: Received by *Neurology* February 13, 2023.
- “Muscle MRI in Patients With Oculopharyngeal Muscular Dystrophy” by Kroon et al.<sup>20</sup>: Received by *Neurology* November 30, 2022.
- “Pearls & Oy-sters: Adult-Onset Craniopharyngioma Presenting With Cognitive Dysfunction and Obstructive Hydrocephalus” by Schroeder et al.<sup>21</sup>: Received by *Neurology* February 3, 2023.
- “Pearls & Oy-sters: Mesial Temporal Seizures in the Absence of the Mesial Temporal Lobe” by Patel et al.<sup>22</sup>: Received by *Neurology* February 2, 2023.
- “Performance of a [<sup>18</sup>F]Flortaucipir PET Visual Read Method Across the Alzheimer Disease Continuum and in Dementia With Lewy Bodies” by Coomans et al.<sup>23</sup>: Received by *Neurology* November 19, 2022.
- “Prehospital Detection of Large Vessel Occlusion Stroke With Electroencephalography: Results of the ELECTRA-STROKE Study” by van Stigt et al.<sup>24</sup>: Received by *Neurology* February 3, 2023.
- “Pretreatment Neurofilament Light Chain Serum Levels, Early Disease Severity, and Treatment Response in Pediatric Multiple Sclerosis” by Huppke et al.<sup>25</sup>: Received by *Neurology* February 8, 2023.
- “Prevalence, Clinical Features, Neuroimaging, and Genetic Findings in Children With Ataxic Cerebral Palsy in Europe” by Horber et al.<sup>26</sup>: Received by *Neurology* February 14, 2023.
- “Prevalence, Demographic, and Clinical Factors Associated With Cognitive Dysfunction in Patients With Neuromyelitis Optica Spectrum Disorder” by Vlahovic et al.<sup>27</sup>: Received by *Neurology* January 23, 2023.
- “Quality of Life in Patients With Confirmed and Suspected Spinal CSF Leaks” by Liaw et al.<sup>28</sup>: Received by *Neurology* November 15, 2022.
- “Relationship Between Neonatal Brain Injury and Objective Measures of Head Trauma” by Dunbar et al.<sup>29</sup>: Received by *Neurology* February 2, 2023.
- “Role of Daytime Continuous Polysomnography in the Diagnosis of Pediatric Narcolepsy Type 1” by Pizza et al.<sup>30</sup>: Received by *Neurology* November 19, 2022.
- “Role of Deployment History on the Association Between Epilepsy and Traumatic Brain Injury in Post-9/11 Era US Veterans” by Henion et al.<sup>31</sup>: Received by *Neurology* January 30, 2023.
- “Simultaneous Comparisons of 25 Acute Migraine Medications Based on 10 Million Users’ Self-Reported Records From a Smartphone Application” by Chiang et al.<sup>32</sup>: Received by *Neurology* February 17, 2023.
- “Survival Analysis of Immunotherapy Effects on Relapse Rate in Pediatric and Adult Autoimmune Encephalitis” by Yang et al.<sup>33</sup>: Received by *Neurology* December 21, 2022.
- “Utility of Acute and Subacute Blood Biomarkers to Assist Diagnosis in CT Negative Isolated Mild Traumatic Brain Injury” by Reyes et al.<sup>34</sup>: Received by *Neurology* January 23, 2023.
- “Utilizing <sup>18</sup>F-AV-133 VMAT2 PET Imaging to Monitor Progressive Nigrostriatal Degeneration in Parkinson Disease” by Beauchamp et al.<sup>35</sup>: Received by *Neurology* September 28, 2022.

The editorial office regrets the errors.

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