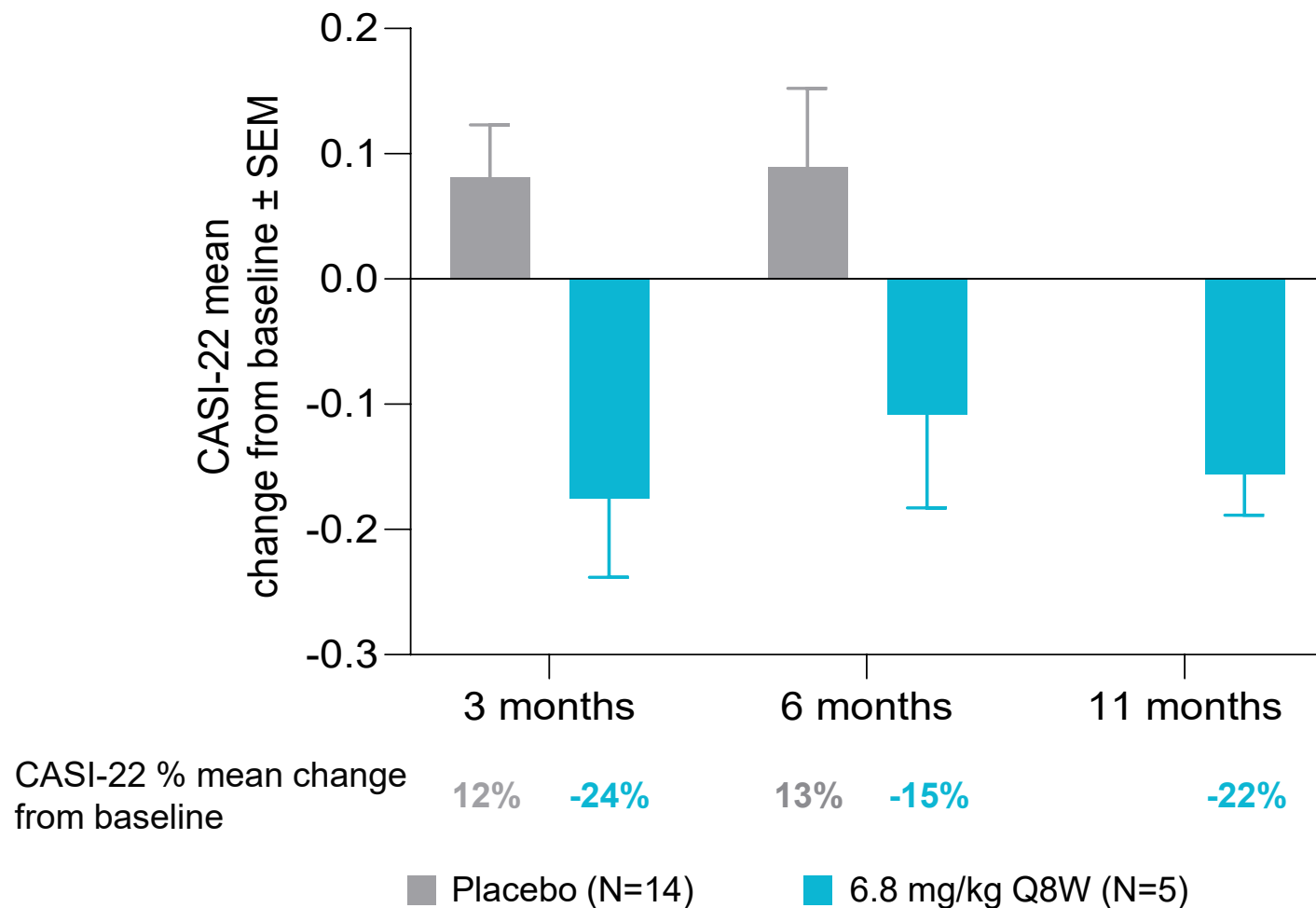


Zeleciment Basivarsen Targets the Underlying Cause of DM1 to Enable Functional Improvement in the Phase 1/2 ACHIEVE Trial

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Z-basivarsen at 6.8 mg/kg Q8W Led to Consistent Splicing Correction

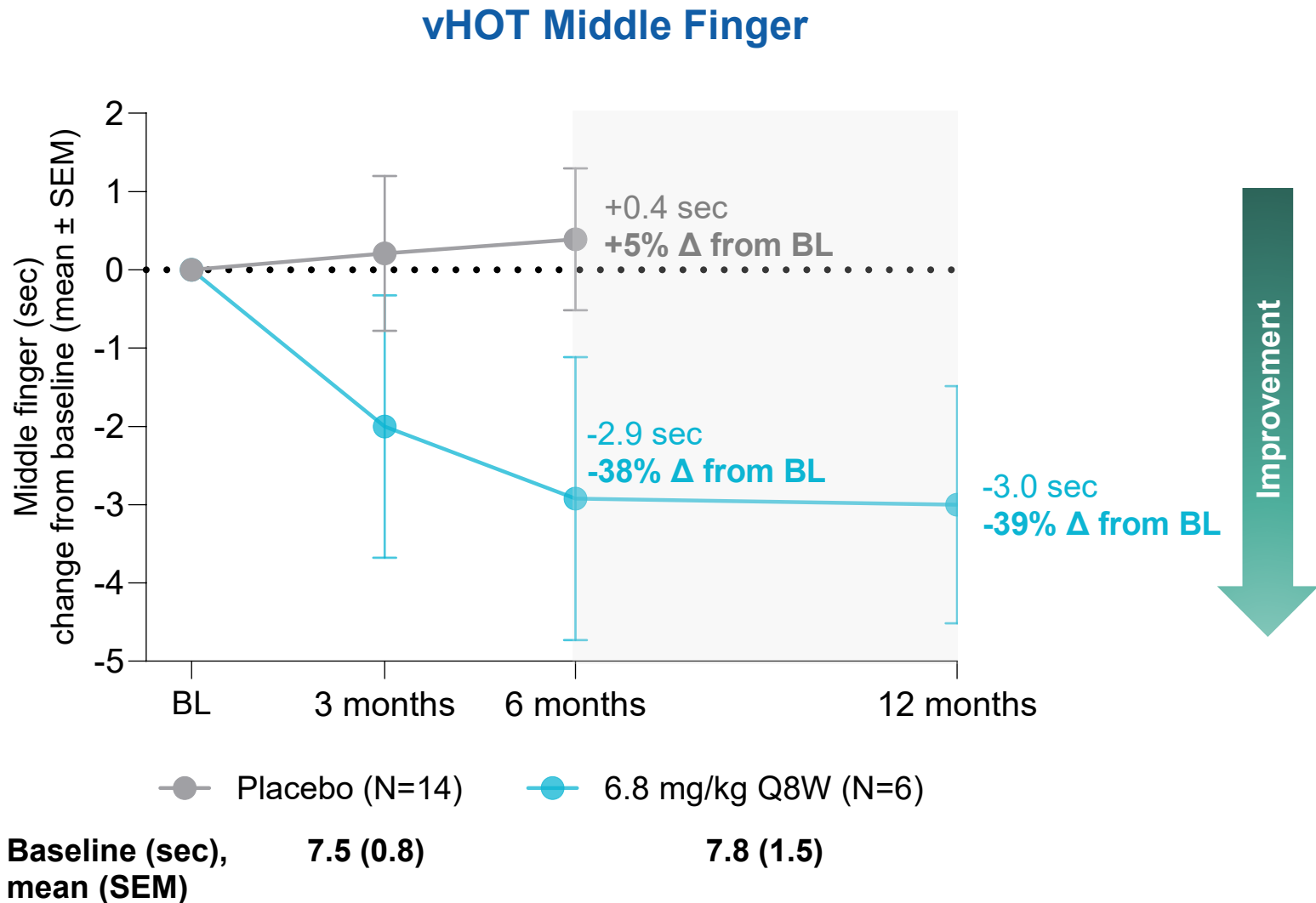


Zeleciment basivarsen "z-basivarsen" is an investigational medicine being evaluated in the ongoing ACHIEVE trial and has not received approval by the US FDA, EMA, or any other regulatory authorities.

CASI, composite alternative splicing index; QC, quality control; Q8W, every 8 weeks dosing.

All patients transitioned to treatment at Month 6. Multiple aliquot approach, all aliquots are tested and median taken across those with valid results, up to 4. One baseline sample in 6.8 mg/kg treatment groups not included within splicing assay as the sample did not meet QC criteria. Percent mean change, calculated as mean change from baseline divided by baseline mean. 3 months = 85 days; 6 months = 169 days; 11 months = 309 days.

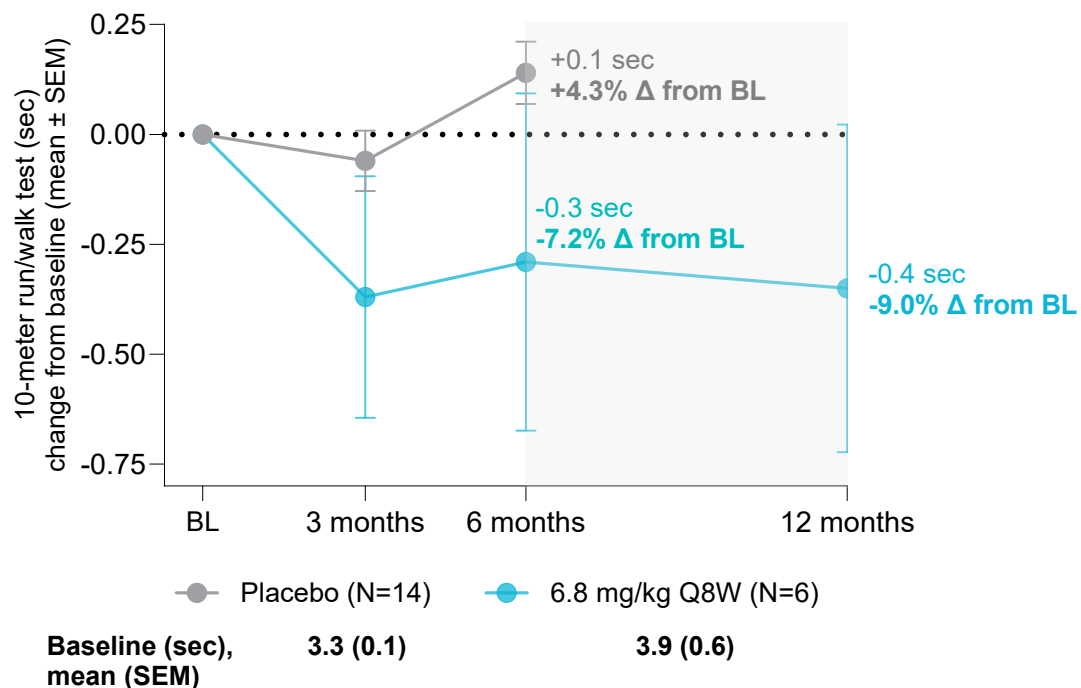
Sustained vHOT Improvement at 6 and 12 Months



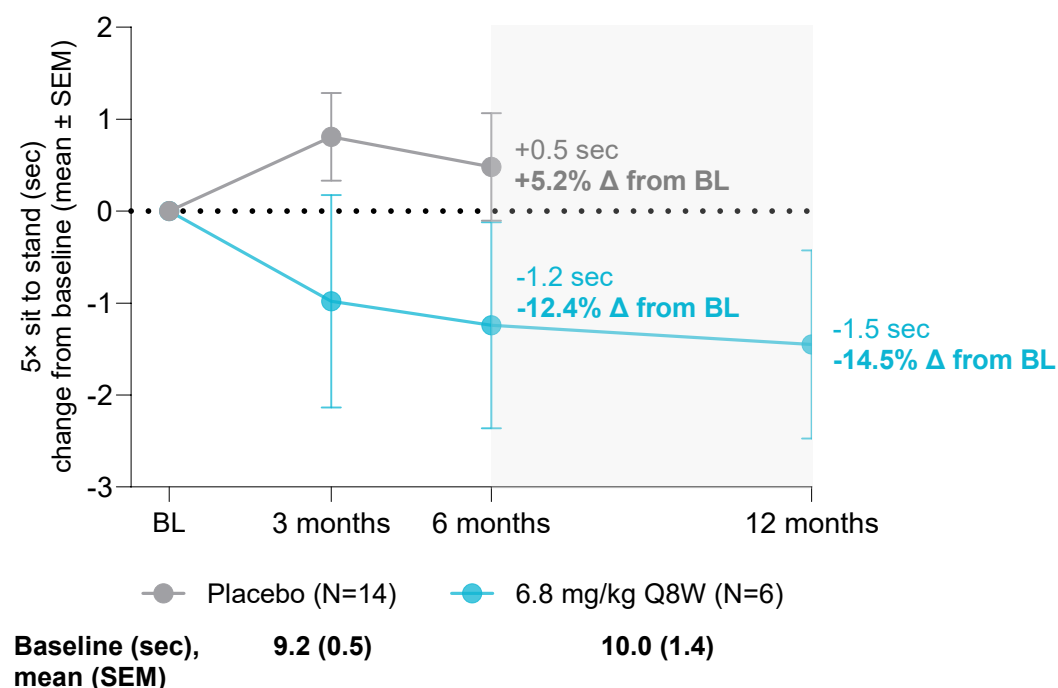
Z-basivarsen is an investigational medicine being evaluated in the ongoing **ACHIEVE** trial and has not received approval by the US FDA, EMA, or any other regulatory authorities.
BL, baseline; Q8W, every 8 weeks dosing; sec, second; SEM, standard error of the mean; vHOT, video hand opening time.
vHOT Middle Finger (sec) is the average of all myotonia trials for an individual participant in **ACHIEVE**. 3 months = 85 days; 6 months = 169 days; 12 months = 337 days.

Benefit Across Multiple Timed Function Tests Sustained at 6 and 12 Months

10-Meter Walk/Run Test



5 Times Sit to Stand



Improvement

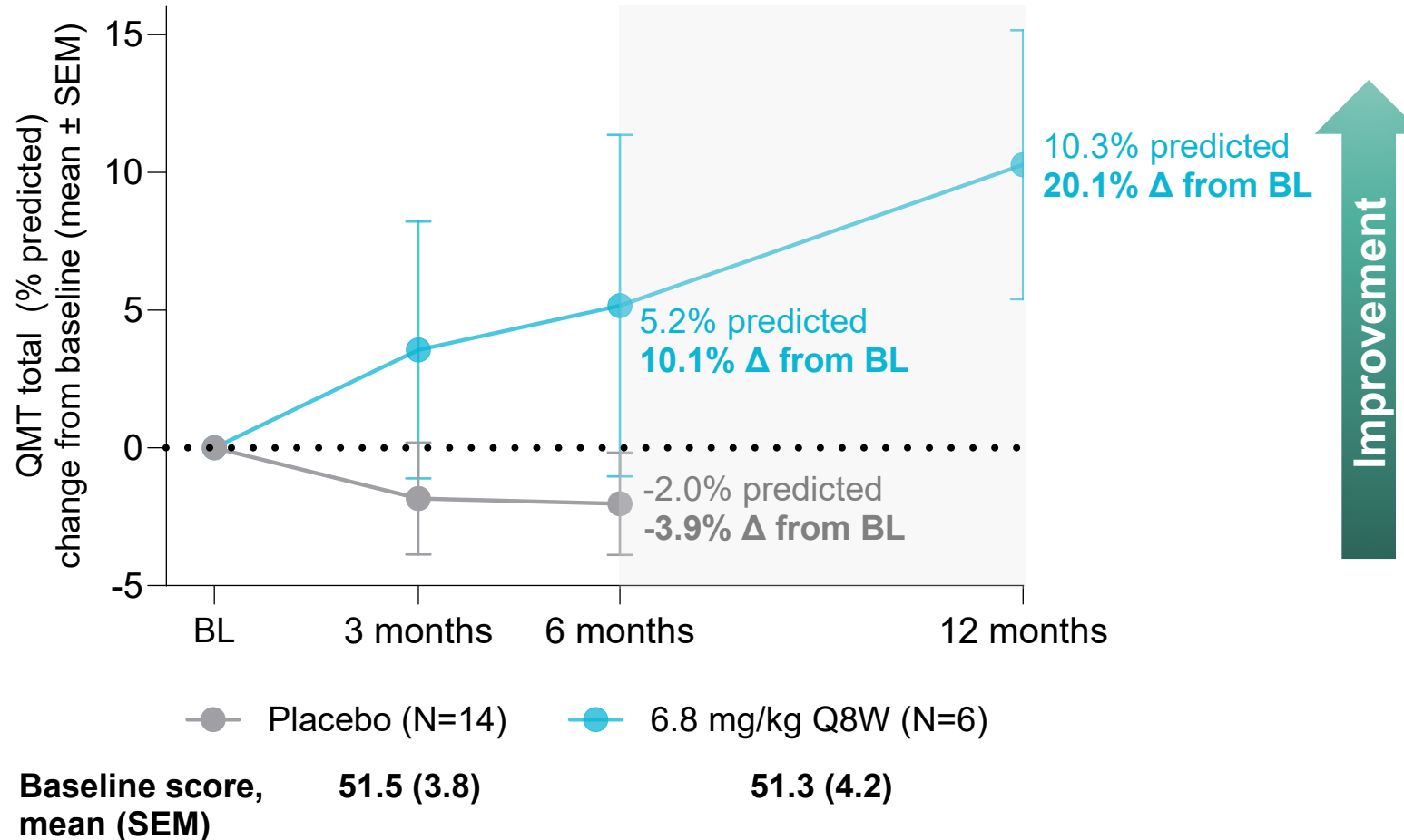
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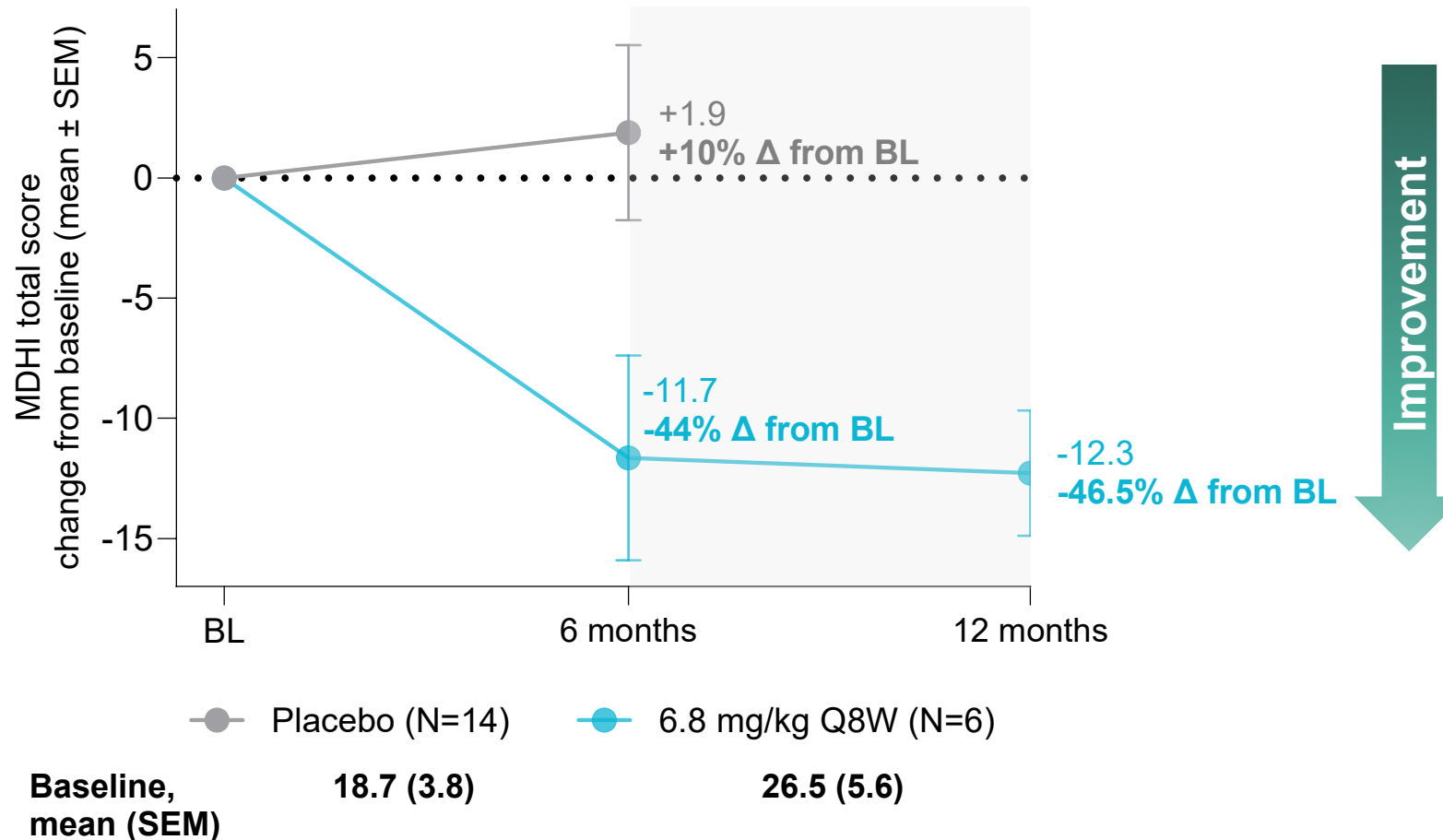
Strength Continued to Improve from Month 6 to Month 12

Quantitative Muscle Testing (QMT) Total Score

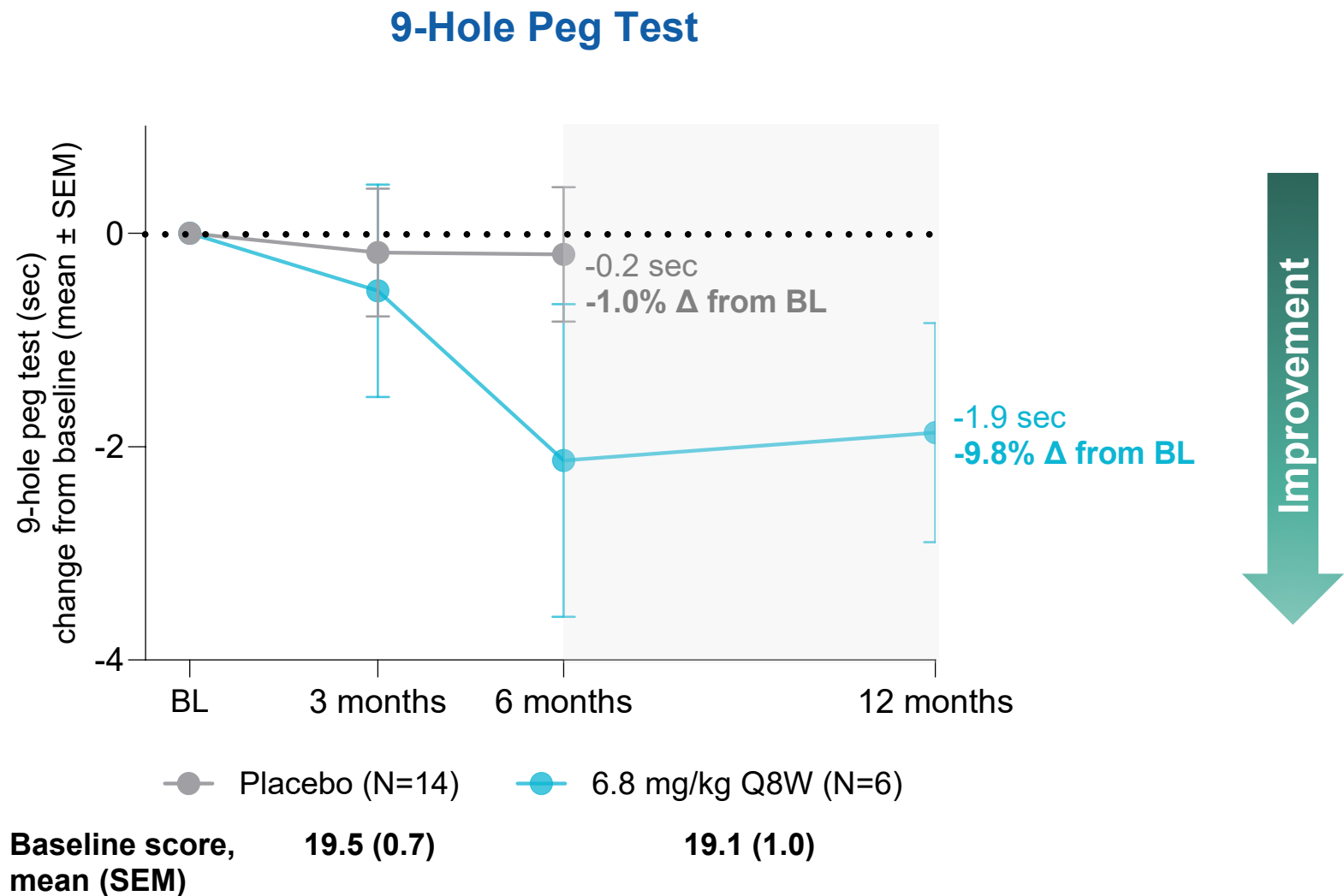


Improvement in Patient Reported Outcome Sustained at 6 and 12 Months

Myotonic Dystrophy Health Index (MDHI) Total Score

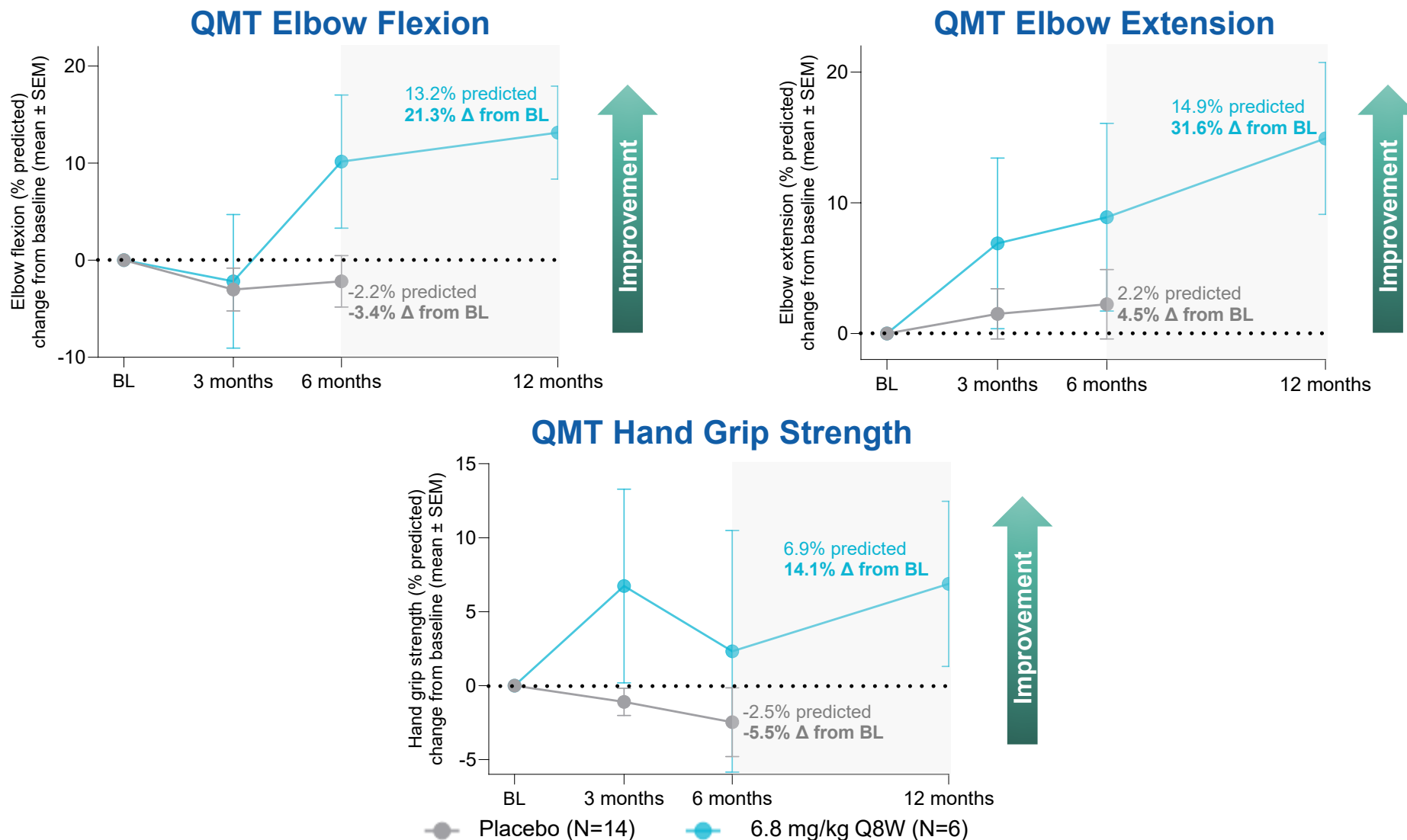


Meaningful Improvement in 9-Hole Peg Test Associated with Improved Upper Extremity Strength, Myotonia, Other Function



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3 months = 85 days; 6 months = 169 days; 12 months = 337 days.

Strength Improvement Noted In Both Proximal and Distal Muscles of Upper Body

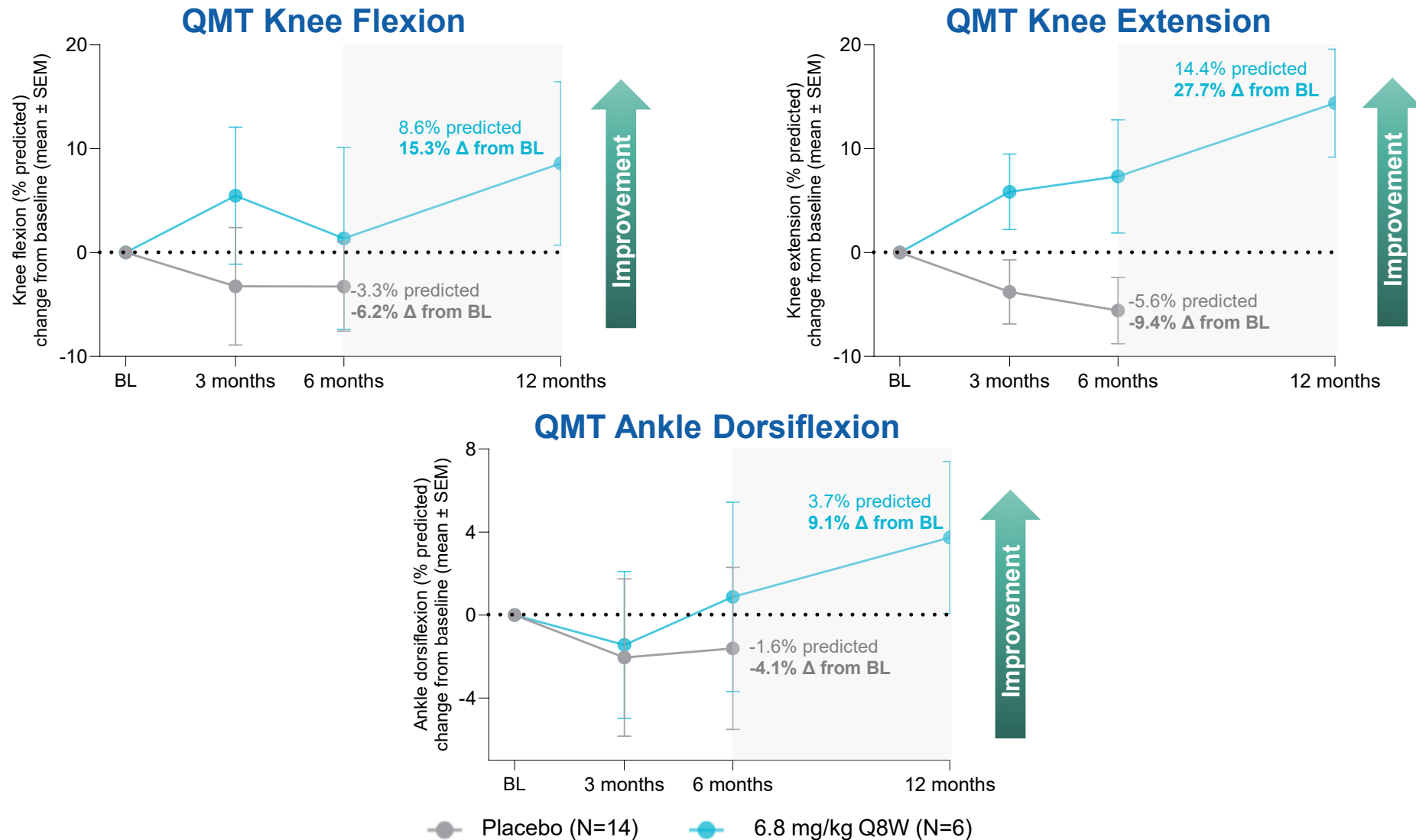


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BL, baseline; QMT, quantitative muscle testing; Q8W, every 8 weeks dosing; SEM, standard error of the mean.

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Strength Improvement Noted In Lower Body Through 12 Months



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