



# Long-term Safety and Tolerability of Delandistrogene Moxeparvec in Duchenne Muscular Dystrophy: Phase 1 to Phase 3 Clinical Trials

Jerry R. Mendell<sup>1</sup>; Craig M. McDonald<sup>2</sup>; Eugenio M. Mercuri<sup>3</sup>; Francesco Muntoni<sup>4</sup>; Craig M. Zaidman<sup>5</sup>; Sachi Dharia<sup>1</sup>; Stefanie Mason<sup>1</sup>; Jianfeng Meng<sup>1</sup>; Alexander P. Murphy<sup>6</sup>; Emanuel Palatinsky<sup>1</sup>; Carol Reid<sup>6</sup>; Christoph Wandel<sup>7</sup>; Crystal Proud<sup>8</sup>

<sup>1</sup>Sarepta Therapeutics, Inc., Cambridge, MA, USA; <sup>2</sup>UC Davis, Davis, CA, USA; <sup>3</sup>Pediatric Neurology, Catholic University, Rome, Italy; <sup>4</sup>Dubowitz Neuromuscular Centre and the Genetic Therapy Accelerator Centre, NIHR Great Ormond Street Hospital Biomedical Research Centre, Great Ormond Street Institute of Child Health, University College London, and Great Ormond Street Hospital Trust, London, UK; <sup>5</sup>Washington University in Saint Louis, Saint Louis, MO, USA; <sup>6</sup>Roche Products Ltd, Welwyn Garden City, UK; <sup>7</sup>F. Hoffmann-La Roche Ltd, Basel, Switzerland; <sup>8</sup>Pediatric Neurologist and Director of Neurology, Children's Hospital of The King's Daughters, Norfolk, VA, USA

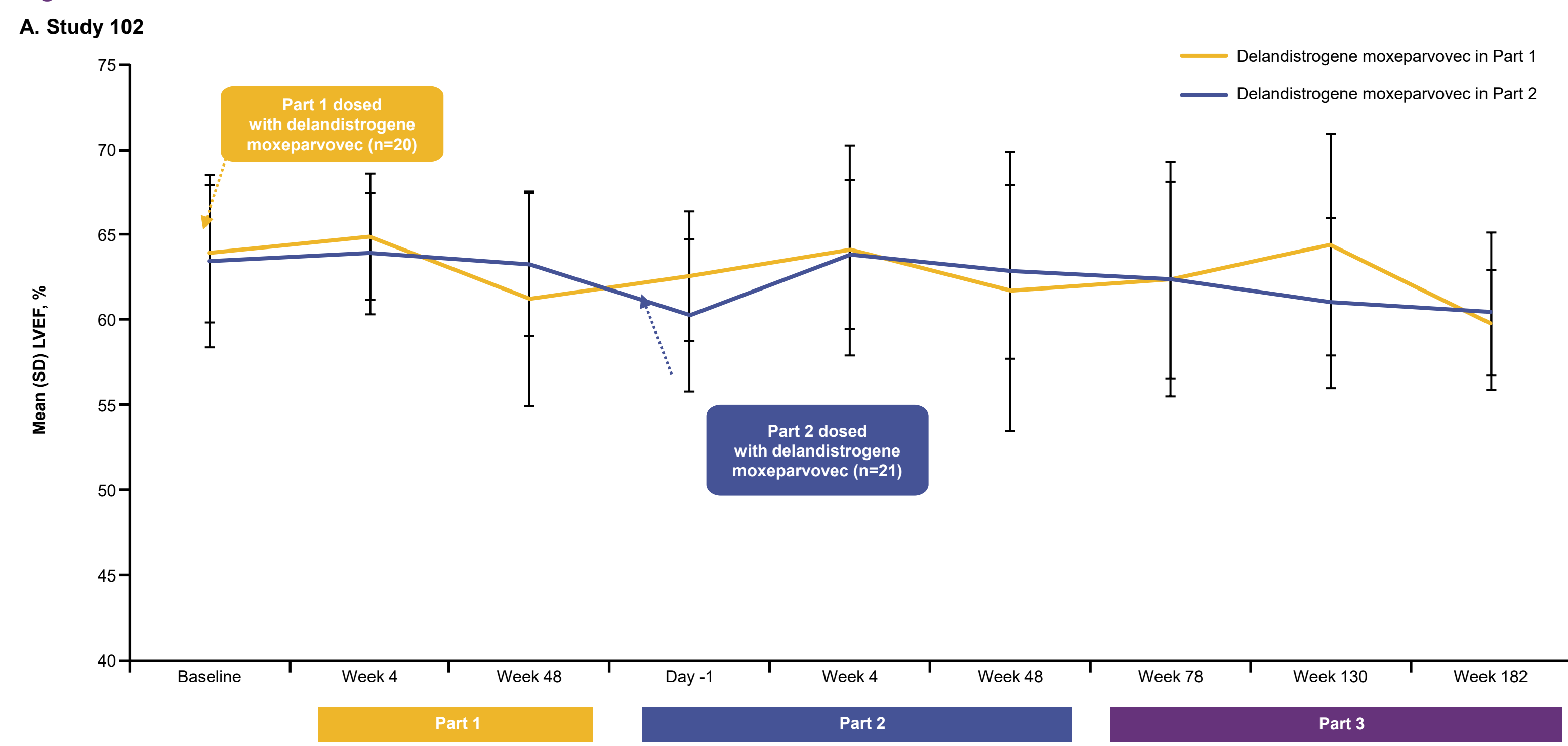
## SUPPLEMENTARY INFORMATION

Table S1. Baseline Liver Function Test Values Across Clinical Trials<sup>a</sup>

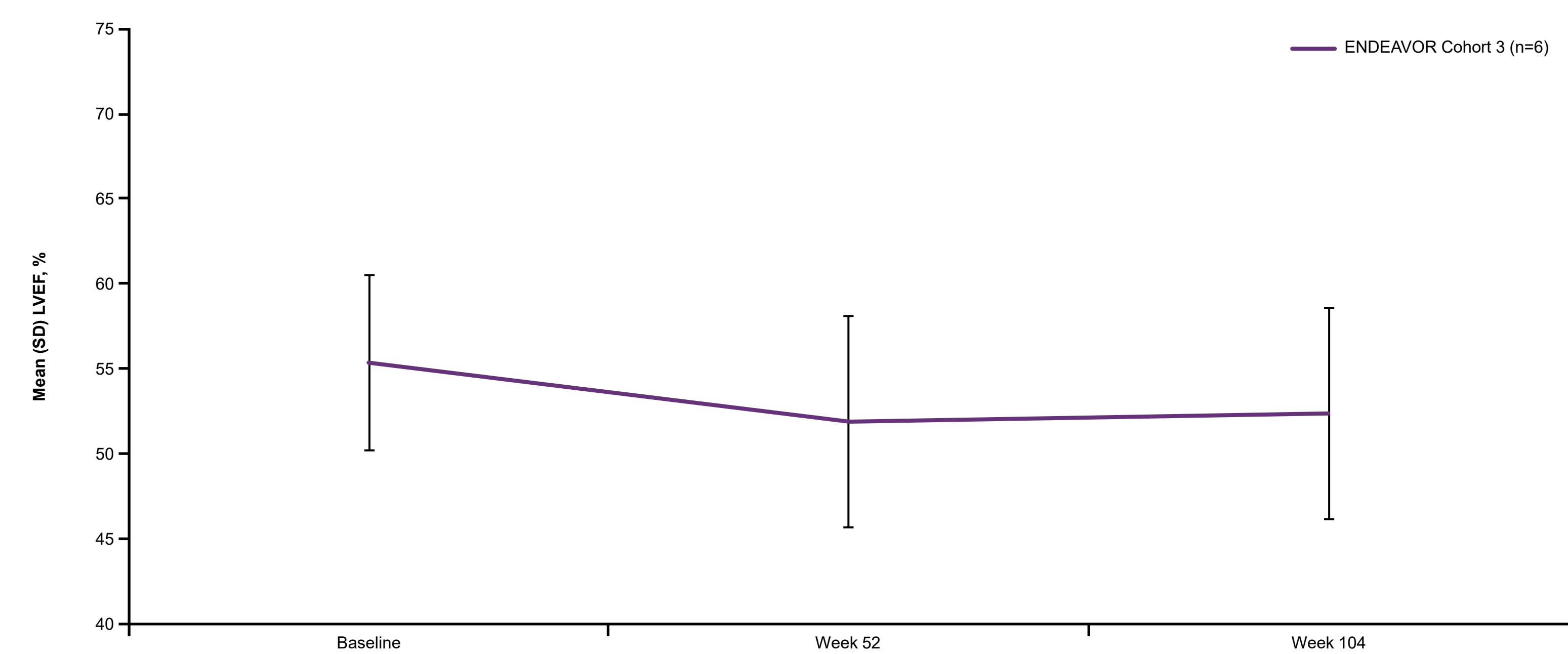
	Study 101 n=4	Study 102 n=41	Study 103 (ENDEAVOR) n=46						Study 301 (EMBARK) Part 1 n=63
			Cohort 1 n=20	Cohort 2 n=7	Cohort 3 n=6	Cohort 4 n=7	Cohort 5a n=6	Cohort 5b n=2	
GGT mean (range), U/L	5.0 (5-5)	10.8 <sup>b</sup> (5-17)	8.7 (5-21)	10.1 (7-20)	17.8 (7-41)	6.7 (6-8)	9.8 (7-14)	13.0 (10-16)	8.1 (4-17)
ALP mean (range), U/L	106 (85-120)	85 <sup>c</sup> (41-132)	121 (70-192)	89 (38-226)	100 (64-135)	176 (119-225)	114 (85-159)	70 (41-100)	100 (53-295)
ALT mean (range), U/L	558 (387-801)	525 <sup>d</sup> (47-1242)	580 (251-999)	310 (177-532)	93 (42-147)	442 (220-733)	544 (336-878)	124 (107-141)	498 <sup>e</sup> (251-986)
Total bilirubin mean (range), mg/dL	0.30 (0.2-0.4)	0.32 <sup>f</sup> (0.1-0.9)	0.26 (0.1-1)	0.40 (0.2-0.9)	0.18 (0.1-0.3)	0.21 (0.1-0.5)	0.20 (0.1-0.4)	0.15 (0.1-0.2)	0.26 (0.09-1.14)

<sup>a</sup>Data in this table do not comprehensively represent all ongoing trials. <sup>b</sup>n=20. <sup>c</sup>n=82.

Figure S1. LVEF Values Over Time

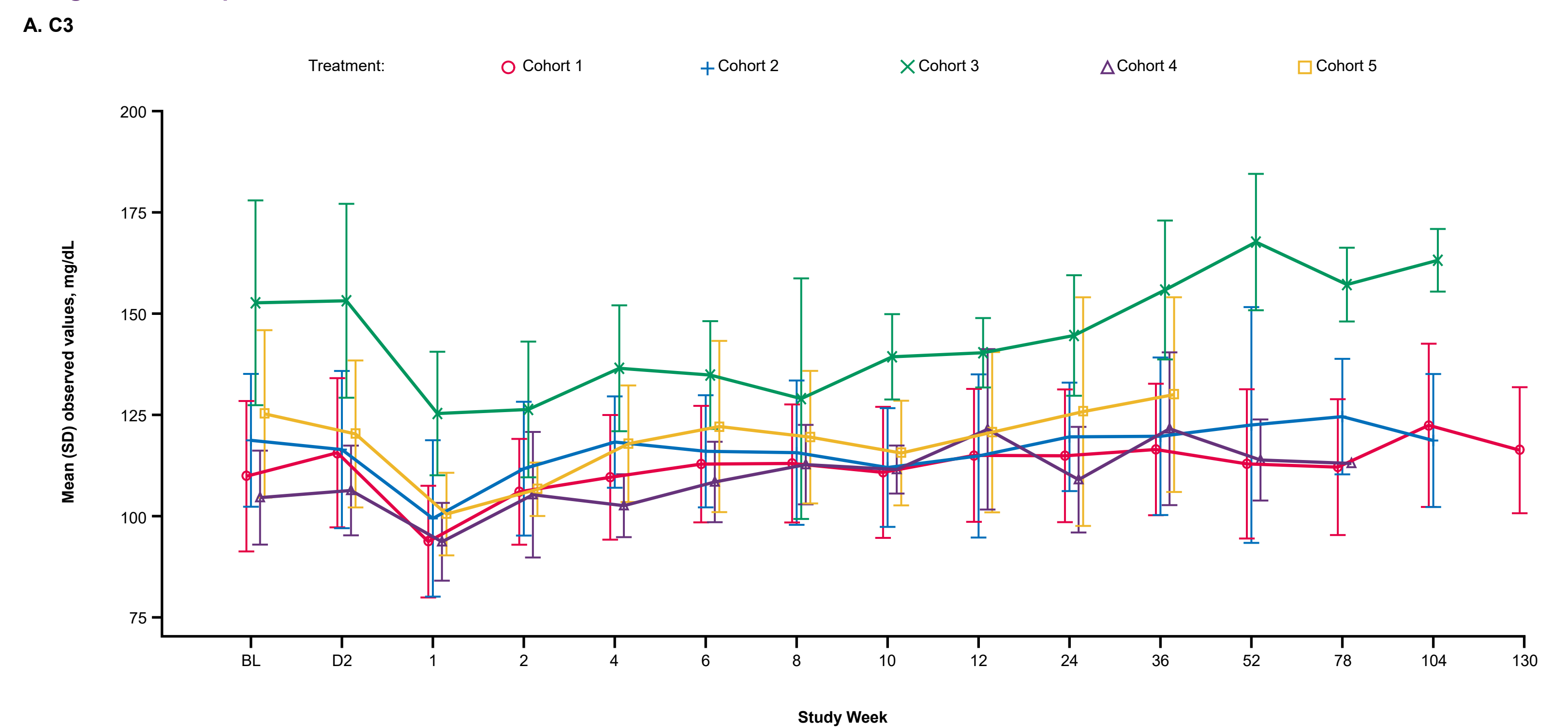


B. ENDEAVOR Cohort 3

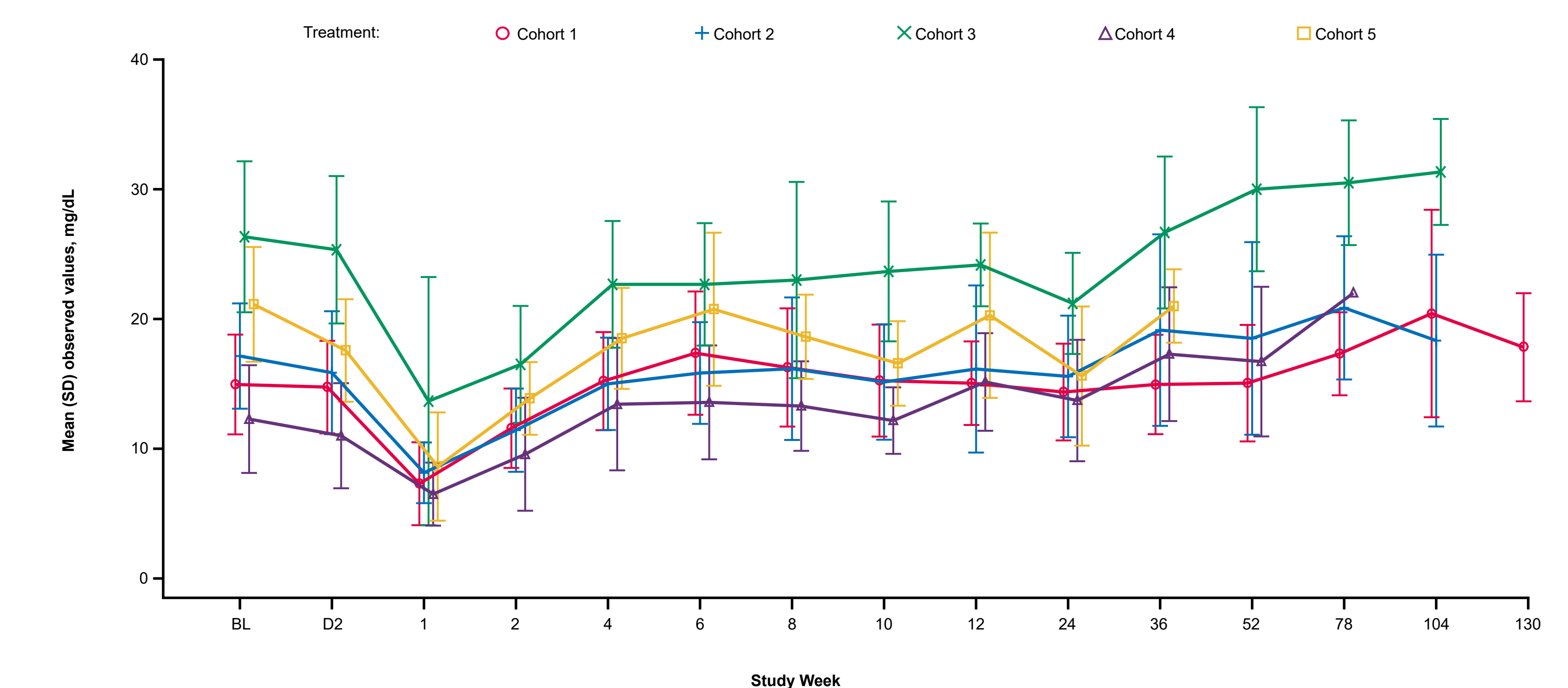


LVEF was measured using an echocardiogram. Normal LVEF ranges from 50% to 70%.

Figure S2. Complement Levels in Patients From ENDEAVOR Cohorts 1-5b



B. C4



### Abbreviations

ALP, alkaline phosphatase; ALT, alanine aminotransferase; BL, baseline; C, complement; D, day; GGT, gamma-glutamyl transferase; LVEF, left ventricular ejection fraction.