SUPPLEMENTARY MATERIAL

Caregiver Global Impression observations from EMBARK: A Phase 3 study evaluating delandistrogene moxeparvovec in ambulatory patients with Duchenne Muscular Dystrophy

Craig M. McDonald[1], Jacob S. Elkins[2], Sai Dharmarajan[2], Katherine Gooch[2], Teofil Ciobanu[3], Claire J. Lansdall[3], Alexander P. Murphy[4], Fiona McDougall[5], Eugenio M. Mercuri[6], Ivana Audhya[2] and the EMBARK Study Group

[1]University of California, Sacramento, CA 95819, USA
[2]Sarepta Therapeutics, Inc., Cambridge, MA 02142, USA
[3]F. Hoffmann-La Roche Ltd., Basel, Switzerland
[4]F. Hoffmann-La Roche Ltd, Welwyn Garden City, UK
[5]Genentech, South San Francisco, CA 94080, USA
[6]Pediatric Neurology Institute, Catholic University and Nemo Pediatrico, Fondazione Policlinico Gemelli IRCCS, Rome, Italy

Institutional review boards and ethics committees list

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
USA		
Anne Connolly	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Katherine Mathews	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Carmen Leon Astudillo	WCG IRB WCG North America: 212 Carnegie Center, Suite 301, Princeton, NJ 08540 +1 609-945-0101	Sharad Adekar, MD, PhD, CIP, Chair, Signature Authority Kelly FitzGerald, PhD, CIP, Executive Chair, Signature Authority
Susan Iannaccone	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Partha Ghosh	Boston Children's Hospital's Institutional Review Board 300 Longwood Avenue, Mailstop BCH 3164, Boston, MA 02115 (617) 355-7052	Tina Young Poussaint, MD, Radiology, IRB Chair Mark Alexander, MD, Cardiology, IRB Vice Chair Carlo Brugnara, MD, Laboratory Medicine, IRB Vice Chair
Emma Ciafaloni	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Susan Apkon	WCG IRB WCG North America: 212 Carnegie Center, Suite 301, Princeton, NJ 08540 +1 609-945-0101	Sharad Adekar, MD, PhD, CIP, Chair, Signature Authority Kelly FitzGerald, PhD, CIP, Executive Chair, Signature Authority

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
Craig Zaidman	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Craig McDonald	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Erika Finanger	OHSU Institutional Review Board 3181 SW Sam Jackson Park Road - L106RI, Portland, OR 97239-3098 (503) 494-7887	Kathryn Schuff, MD, MCR, Chair, Designated Reviewer Terri Barret, PhD, Vice Chair, Designated Reviewer
Nancy Kuntz	The Institutional Review Boards of the Ann & Robert H. Lurie Children's Hospital of Chicago Stanley Manne Children's Research Institute 225 East Chicago Avenue, Box 205, Chicago, IL 60611 (312) 503-7060	Jennifer Rubin, MD
Russell Butterfield	University of Utah Institutional Review Board 75 South 2000 East Salt Lake City, UT 84112 (801) 581-3655	Mark Eliason, PhD, Dermatology, Chair
Chamindra Laverty	University of California San Diego Institutional Review Board Administration 9500 Gilman Drive, Mail Code 0052, La Jolla, CA 92093-0990 (858) 246-4777	Ben Mooso, Director
Carolina Tesi Rocha	Stanford University Administrative Panel on Human Subjects in Medical Research, Palo Alto, CA 94306 (650) 724-7541[a]	Hendrikus J.M. Lemmens, MD, PhD, Professor, Co-chair David D. Oakes, MD, Professor, Emeritus, Co-chair
Michio Hirano	Columbia Research Human Research Protection Office Institutional Review Boards 154 Haven Avenue, 1st Floor, New York, NY 10032	Pamela Freda, MD, Endocrinology, IRB Chair

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
	(212) 305-5883	Pietro Canetta, MD, Nephrology, IRB Vice Chair
Perry Shieh	University of California Los Angeles Institutional Review Board (UCLA IRB) 10889 Wilshire Blvd, Suite 830, Los Angeles, CA 90095-1406 (310) 825-7122	James McGough, MD, Psychiatry and Biobehavioral Sciences, Chair, Regular Voting Leia Nghiemphu, MD, Neurology, Vice Chair, Regular Voting
John Brandsema	Children's Hospital of Philadelphia's (CHOP) institutional Review Board (CHOP's IRB) 2716 South Street, Suite 4321, Philadelphia, PA 19416 (215) 590-2830	Members selected based on their willingness
Mai ElMallah	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
William Burnette	Vanderbilt Human Research Protection Program (HRPP) Health Sciences Committee 1 - Institutional Review Board[a] 1313 21st Ave., South, Suite 505, Nashville, TN 37232	Michael G O'Connor, MD, Chair Jo Wilson, MD, MPH, Vice Chair
Crystal Proud	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
Matthew Harmelink	Medical College of Wisconsin Institutional Review Board (MCW IRB)	Board #1: Sue Kehl, PhD, Non-MD Scientist, Chair Danny Thomas, MD, MPH, Physician, Chair Board #2: Nathan Thompson, MD, PharmD, Physician, Pharmacist, Chair

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
Jessica Nance	Johns Hopkins Medicine Institutional Review Board (JHM IRB) 1620 McElderry Street, Reed Hall, Suite B-130, Baltimore, MD 21205-1911 (410) 955-3008	B. Douglas Smith, MD, Professor, Oncology, Chair Craig Hendrix, MD, Professor, Clinical Pharmacology, Co-chair
Aravindhan Veerapandiyan	ADVARRA IRB 6100 Merriweather Dr., Suite 600, Columbia, MD 21044 410-884-2900	Luke Gelinas, Executive Chair Susan Ebert, Executive Vice Chair
UK		
Francesco Muntoni	South Central - Oxford A Research Ethics Committee Ground Floor Temple Quay House 2 The Square, Bristol, BS1 6PN	Dr Hugh Davies
Volker Straub	South Central - Oxford A Research Ethics Committee Ground Floor Temple Quay House 2 The Square, Bristol, BS1 6PN	Dr Hugh Davies
Laurent Servais	South Central - Oxford A Research Ethics Committee Ground Floor Temple Quay House 2 The Square, Bristol, BS1 6PN	Dr Hugh Davies
Taiwan		
Yuh-Jyh Jong	Institutional Review Board of Kaohsiung Medical University Chung-Ho Memorial Hospital	Hsueh-Wei Yen
Yin-Hsiu Chien	Research Ethics Committee of National Taiwan University Hospital	Daniel Fu-Chang Tsai
Spain		
Dr. Núria Muelas Gómez	CEIm Parc Taulí Edificio Santa Fe, ala izquierda, 2ª planta Parc Taulí, 1 08208 Sabadell, Barcelona	Chair: Miquel Àngel Seguí Palmer, MD Secretary: Coloma Moreno Quiroga, MD
Dr. Andrés Eduardo Nascimiento Osorio	CEIm Parc Taulí Edificio Santa Fe, ala izquierda, 2ª planta Parc Taulí, 1 08208 Sabadell, Barcelona	Chair: Miquel Àngel Seguí Palmer, MD Secretary: Coloma Moreno Quiroga, MD
Italy		

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
Prof. Eugenio Maria Mercuri Dr. Claudio Bruno Prof. Giacomo Pierto Comi	COMITATO ETICO TERRITORIALE LAZIO AREA 3 Fondazione Policlinico Universitario Agostino Gemelli IRCCS Università Cattolica del Sacro Cuore Largo Francesco Vito, 1 00168, Rome comitatoetico.lazioarea3@policlinicogemelli.it	Chairman: Andrea Bacigalupo
Prof. Eugenio Maria Mercuri Dr. Claudio Bruno Prof. Giacomo Pierto Comi	COMITATO ETICO TERRITORIALE LAZIO AREA 3 Fondazione Policlinico Universitario Agostino Gemelli IRCCS Università Cattolica del Sacro Cuore Largo Francesco Vito, 1 00168 Rome comitatoetico.lazioarea3@policlinicogemelli.it	Chairman: Andrea Bacigalupo
Prof. Eugenio Maria Mercuri Dr. Claudio Bruno Prof. Giacomo Pierto Comi	COMITATO ETICO TERRITORIALE LAZIO AREA 3 Fondazione Policlinico Universitario Agostino Gemelli IRCCS Università Cattolica del Sacro Cuore Largo Francesco Vito, 1 00168 Rome comitatoetico.lazioarea3@policlinicogemelli.it	Chairman: Andrea Bacigalupo
Belgium		
Prof. Nicolas Deconinck	UZ Gent Commissie voor Medische Ethiek UZ Gent C. Heymanslaan 10, 9000 Gent	Prof. Dr. P. Deron
Germany		
Prof. Dr. med. Wolfgang Mueller-Felber (NC)	Ethikkommission der Medizinischen Fakultät der LMU München Pettenkoferstr. 8a, 80336 Munich	Prof. Dr. Rudolf M. Huber
Dr. med. Andrea Gangfuss	Ethikkommission der Medizinischen Fakultät der Universität Duisburg-Essen Universitätsklinikum Essen	Prof. Dr. med. Ulrike Schara-Schmidt

Name of principal investigator	Name/address of IEC/IRB	Name of IEC/IRB Chairperson
	Robert-Koch-Str. 9-11, 45147 Essen	
Hong Kong		
Dr. Chan, Hoi Shan Sophelia	Central Institutional Review Board Address: Research Office, 8/F, Tower A, Hong Kong Children's Hospital, 1 Shing Cheong Road, Kowloon, Hong Kong	Dr. Yuen, Vivian Man-Ying
Japan		
Ryosuke Bo	Institutional Review Board of Kobe University Hospital 7-5-2 Kusunoki-cho, Chuo-ku, Kobe-shi, Hyogo	Kandai Nozu
Hirofumi Komaki	National Center of Neurology and Psychiatry Institutional Review Board 4-1-1 Ogawa-Higashi, Kodaira, Tokyo	Yuji Takahashi
Keiko Ishigaki	Tokyo Women's Medical University Institutional Review Board 8-1 Kawada-cho, Shinjuku-ku, Tokyo	Katsuji Nishimura
Yuichi Abe	National Center for Child Health and Development Institutional Review Board 2-10-1 Okura, Setagaya-ku, Tokyo	Mitsuru Kubota

CaGI-C scoring

The first four items assessing change in patient's health from baseline to week 52 utilize a 7-point categorical response options ranging from very much worse (7), much worse (6), minimally worse (5), no change (4), minimally improved (3), much improved (2), very much improved (1).

CaGI-S scoring

CaGI-S items are rated using 5-level categorical response options with lower values indicating less severity: symptoms, very mild (1), mild (2), moderate (3), severe (4), and very severe (5); physical ability, very mild impairment (1), mild impairment (2), moderate impairment (3), severe impairment (4), and very severe impairment (5); daily activity performance, very mild impact/impairment (1), mild impact/impairment (2), moderate impact/impairment (3), severe impact/impairment (4), and very severe impact/impairment (5); and overall health summary, very mildly impaired/impacted (1), mildly impaired/impacted (2), moderately impaired/impacted

(3), severely impaired/impacted (4), and very severely impaired/impacted (5). Change in severity was derived as the difference in severity between the week 52 and baseline (change from baseline) and could range from -4 to 4 with lower (negative) scores indicate improvement. Response could be categorized as Improvement (score change of -1 or less), No change (score change of 0), or Worsening (score change of +1 or more).

SUPPLEMENTAL RESULTS

Supplemental Table 1. Treatment group counts and percentages for CaGI-S responses by age group (mITT population; Part 1, ad hoc all patients).

			Difference at Week 52 from Week 0								
	All Patients	**Missing Response**	**-4**	**-3**	**-2**	**-1**	**0**	**1**	**2**	**3**	**4**
Age Group – 4-5 years											
CaGI-S1: Severity of DMD symptoms, n (%)											
All Patients	60 (100)	7 (12)	0 (0)	0 (0)	1 (2)	18 (30)	27 (45)	5 (8)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	30 (100)	5 (17)	0 (0)	0 (0)	0 (0)	11 (37)	12 (40)	2 (7)	0 (0)	0 (0)	0 (0)
Placebo	30 (100)	2 (7)	0 (0)	0 (0)	1 (3)	7 (23)	15 (20)	3 (10)	2 (7)	0 (0)	0 (0)
CaGI-S2: Severity of impairment, n (%)											
All Patients	60 (100)	7 (12)	0 (0)	0 (0)	1 (2)	20 (33)	27 (45)	5 (8)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	30 (100)	5 (17)	0 (0)	0 (0)	0 (0)	14 (47)	11 (37)	0 (0)	0 (0)	0 (0)	0 (0)
Placebo	30 (100)	2 (7)	0 (0)	0 (0)	1 (3)	6 (20)	16 (53)	5 (17)	0 (0)	0 (0)	0 (0)
CaGI-S3: Ability of daily activities, n (%)											
All Patients	60 (100)	7 (12)	0 (0)	0 (0)	1 (2)	16 (27)	27 (45)	9 (15)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	30 (100)	5 (17)	0 (0)	0 (0)	0 (0)	11 (37)	10 (33)	4 (13)	0 (0)	0 (0)	0 (0)
Placebo	30 (100)	2 (7)	0 (0)	0 (0)	1 (3)	5 (17)	17 (57)	5 (17)	0 (0)	0 (0)	0 (0)
CaGI-S6: Severity of overall health, n (%)											
All Patients	60 (100)	7 (12)	0 (0)	0 (0)	1 (2)	14 (23)	29 (48)	8 (13)	1 (2)	0 (0)	0 (0)

	All Patients	Missing Response	Difference at Week 52 from Week 0								
			-4	-3	-2	-1	0	1	2	3	4
Delandistrogene moxeparvovec	30 (100)	5 (17)	0 (0)	0 (0)	1 (3)	9 (30)	12 (40)	3 (10)	0 (0)	0 (0)	0 (0)
Placebo	30 (100)	2 (7)	0 (0)	0 (0)	0 (0)	5 (17)	17 (57)	5 (17)	1 (3)	0 (0)	0 (0)
Age Group – 6-7 years											
CaGI-S1: Severity of DMD symptoms, n (%)											
All Patients	65 (100)	7 (11)	0 (0)	0 (0)	1 (2)	14 (22)	30 (46)	12 (18)	1 (2)	0 (0)	0 (0)
Delandistrogene moxeparvovec	33 (100)	5 (15)	0 (0)	0 (0)	0 (0)	10 (30)	14 (42)	4 (12)	0 (0)	0 (0)	0 (0)
Placebo	32 (100)	2 (6)	0 (0)	0 (0)	1 (3)	4 (13)	16 (50)	8 (25)	1 (3)	0 (0)	0 (0)
CaGI-S2: Severity of impairment, n (%)											
All Patients	65 (100)	7 (11)	0 (0)	0 (0)	3 (5)	12 (18)	33 (51)	8 (12)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	33 (100)	5 (15)	0 (0)	0 (0)	2 (6)	6 (18)	18 (55)	2 (6)	0 (0)	0 (0)	0 (0)
Placebo	32 (100)	2 (6)	0 (0)	0 (0)	1 (3)	6 (19)	15 (47)	6 (19)	2 (6)	0 (0)	0 (0)
CaGI-S3: Ability of daily activities, n (%)											
All Patients	65 (100)	7 (11)	0 (0)	0 (0)	1 (2)	15 (23)	31 (48)	9 (14)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	33 (100)	5 (15)	0 (0)	0 (0)	0 (0)	7 (21)	17 (52)	4 (12)	0 (0)	0 (0)	0 (0)
Placebo	32 (100)	2 (6)	0 (0)	0 (0)	1 (3)	8 (25)	14 (44)	5 (16)	2 (6)	0 (0)	0 (0)
CaGI-S6: Severity of overall health, n (%)											
All Patients	65 (100)	7 (11)	0 (0)	0 (0)	3 (5)	9 (14)	36 (55)	8 (12)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	33 (100)	5 (15)	0 (0)	0 (0)	1 (3)	6 (18)	20 (61)	1 (3)	0 (0)	0 (0)	0 (0)
Placebo	32 (100)	2 (6)	0 (0)	0 (0)	2 (6)	3 (9)	16 (50)	7 (22)	2 (6)	0 (0)	0 (0)

CaGI-S, CaGI-S, Caregiver Global Impression of Severity; mITT, modified intention-to-treat population

Supplemental Table 2. Treatment group counts and percentages for CaGI-C responses by adverse event (mITT population; Part 1, ad hoc all patients).

	All Patients	Missing Response	Very much improved	Much improved	Minimally improved	No change	Minimally worse	Much worse	Very much worse
Nausea or vomiting in the first 2 weeks after treatment									
CaGI-C1: Changes in symptoms, n (%)									
All Patients	45 (100)	2 (4)	10 (22)	11 (24)	14 (31)	4 (9)	3 (7)	1 (2)	0 (0)
Delandistrogene moxeparvovec	38 (100)	2 (5)	8 (21)	10 (26)	13 (34)	4 (11)	1 (3)	0 (0)	0 (0)
Placebo	7 (100)	0 (0)	2 (29)	1 (14)	1 (14)	0 (0)	2 (29)	1 (14)	0 (0)
CaGI-C2: Changes in physical ability, n (%)									
All Patients	45 (100)	1 (2)	11 (24)	13 (29)	13 (29)	4 (9)	2 (4)	1 (2)	0 (0)
Delandistrogene moxeparvovec	38 (100)	1 (3)	9 (24)	12 (32)	12 (32)	4 (11)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	0 (0)	2 (29)	1 (14)	1 (14)	0 (0)	2 (29)	1 (14)	0 (0)
CaGI-C3: Changes in activities ability, n (%)									
All Patients	45 (100)	1 (2)	9 (20)	11 (24)	13 (29)	9 (20)	2 (4)	0 (0)	0 (0)
Delandistrogene moxeparvovec	38 (100)	1 (3)	7 (18)	10 (26)	12 (32)	8 (21)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	0 (0)	2 (29)	1 (14)	1 (14)	1 (14)	2 (29)	0 (0)	0 (0)
CaGI-C4A: Changes in overall health, n (%)									
All Patients	45 (100)	1 (2)	10 (22)	13 (29)	8 (18)	10 (22)	2 (4)	1 (2)	0 (0)
Delandistrogene moxeparvovec	38 (100)	1 (3)	8 (21)	12 (32)	8 (21)	9 (24)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	0 (0)	2 (29)	1 (14)	0 (0)	1 (14)	2 (29)	1 (14)	0 (0)
No nausea or vomiting in the first 2 weeks after treatment									
CaGI-C1: Changes in symptoms, n (%)									
All Patients	80 (100)	4 (5)	6 (8)	18 (23)	18 (23)	27 (34)	7 (9)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	3 (12)	9 (36)	5 (20)	3 (12)	2 (8)	0 (0)	0 (0)

	All Patients	Missing Response	Very much improved	Much improved	Minimally improved	No change	Minimally worse	Much worse	Very much worse
Placebo	55 (100)	1 (2)	3 (5)	9 (16)	13 (24)	24 (44)	5 (9)	0 (0)	0 (0)
CaGI-C2: Changes in physical ability, n (%)									
All Patients	80 (100)	4 (5)	8 (10)	16 (20)	22 (28)	19 (24)	10 (13)	1 (1)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	5 (20)	7 (28)	6 (24)	2 (8)	2 (8)	0 (0)	0 (0)
Placebo	55 (100)	1 (2)	3 (5)	9 (16)	16 (29)	17 (31)	8 (15)	1 (2)	0 (0)
CaGI-C3: Changes in activities ability, n (%)									
All Patients	80 (100)	4 (5)	6 (8)	12 (15)	27 (34)	27 (34)	4 (5)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	4 (16)	7 (28)	6 (24)	5 (20)	0 (0)	0 (0)	0 (0)
Placebo	55 (100)	1 (2)	2 (4)	5 (9)	21 (38)	22 (40)	4 (7)	0 (0)	0 (0)
CaGI-C4A: Changes in overall health, n (%)									
All Patients	80 (100)	4 (5)	5 (6)	16 (20)	24 (30)	24 (30)	7 (9)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	2 (8)	9 (36)	7 (28)	2 (8)	2 (8)	0 (0)	0 (0)
Placebo	55 (100)	1 (2)	3 (5)	7 (13)	17 (31)	22 (40)	5 (9)	0 (0)	0 (0)

CaGI-C, Caregiver Global Impression of Change; mITT, modified intention-to-treat population

Supplemental Table 3. Treatment group counts and percentages for CaGI-S responses by adverse event (mITT population; Part 1, ad hoc all patients).

			Difference at Week 52 from Week 0								
	All Patients	**Missing Response**	**-4**	**-3**	**-2**	**-1**	**0**	**1**	**2**	**3**	**4**
Nausea or vomiting in the first 2 weeks after treatment											
CaGI-S1: Severity of DMD symptoms, n (%)											
All Patients	45 (100)	8 (18)	0 (0)	0 (0)	0 (0)	10 (22)	22 (49)	5 (11)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	38 (100)	7 (18)	0 (0)	0 (0)	0 (0)	10 (26)	17 (45)	4 (11)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	1 (14)	0 (0)	0 (0)	0 (0)	0 (0)	5 (71)	1 (14)	0 (0)	0 (0)	0 (0)
CaGI-S2: Severity of impairment, n (%)											
All Patients	45 (100)	8 (18)	0 (0)	0 (0)	1 (2)	14 (31)	19 (42)	3 (7)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	38 (100)	7 (18)	0 (0)	0 (0)	1 (3)	13 (34)	16 (42)	1 (3)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	1 (14)	0 (0)	0 (0)	0 (0)	1 (14)	3 (43)	2 (29)	0 (0)	0 (0)	0 (0)
CaGI-S3: Ability of daily activities, n (%)											
All Patients	45 (100)	8 (18)	0 (0)	0 (0)	0 (0)	9 (20)	21 (47)	7 (16)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	38 (100)	7 (18)	0 (0)	0 (0)	0 (0)	8 (21)	18 (47)	5 (13)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	1 (14)	0 (0)	0 (0)	0 (0)	1 (14)	3 (43)	2 (29)	0 (0)	0 (0)	0 (0)
CaGI-S6: Severity of overall health, n (%)											
All Patients	45 (100)	8 (18)	0 (0)	0 (0)	1 (2)	8 (18)	22 (49)	6 (13)	0 (0)	0 (0)	0 (0)
Delandistrogene moxeparvovec	38 (100)	7 (18)	0 (0)	0 (0)	1 (3)	7 (18)	19 (50)	4 (11)	0 (0)	0 (0)	0 (0)
Placebo	7 (100)	1 (14)	0 (0)	0 (0)	0 (0)	1 (14)	3 (43)	2 (29)	0 (0)	0 (0)	0 (0)

	All Patients	Missing Response	Difference at Week 52 from Week 0								
			-4	-3	-2	-1	0	1	2	3	4
No nausea or vomiting in the first 2 weeks after treatment											
CaGI-S1: Severity of DMD symptoms, n (%)											
All Patients	80 (100)	6 (8)	0 (0)	0 (0)	2 (3)	22 (28)	35 (44)	12 (15)	3 (4)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	0 (0)	0 (0)	0 (0)	11 (44)	9 (36)	2 (8)	0 (0)	0 (0)	0 (0)
Placebo	55 (100)	3 (5)	0 (0)	0 (0)	2 (4)	11 (20)	26 (47)	10 (18)	3 (5)	0 (0)	0 (0)
CaGI-S2: Severity of impairment, n (%)											
All Patients	80 (100)	6 (8)	0 (0)	0 (0)	3 (4)	18 (23)	41 (51)	10 (13)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	0 (0)	0 (0)	1 (4)	7 (28)	13 (52)	1 (4)	0 (0)	0 (0)	0 (0)
Placebo	55 (100)	3 (5)	0 (0)	0 (0)	2 (4)	11 (20)	28 (51)	9 (16)	2 (4)	0 (0)	0 (0)
CaGI-S3: Ability of daily activities, n (%)											
All Patients	80 (100)	6 (8)	0 (0)	0 (0)	2 (3)	22 (28)	37 (46)	11 (14)	2 (3)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	0 (0)	0 (0)	0 (0)	10 (40)	9 (36)	3 (12)	0 (0)	0 (0)	0 (0)
Placebo	55 (100)	3 (5)	0 (0)	0 (0)	2 (4)	12 (22)	28 (51)	8 (15)	2 (4)	0 (0)	0 (0)
CaGI-S6: Severity of overall health, n (%)											
All Patients	80 (100)	6 (8)	0 (0)	0 (0)	3 (4)	15 (19)	43 (54)	10 (13)	3 (4)	0 (0)	0 (0)
Delandistrogene moxeparvovec	25 (100)	3 (12)	0 (0)	0 (0)	1 (4)	8 (32)	13 (52)	0 (0)	0 (0)	0 (0)	0 (0)
Placebo	55 (100)	3 (5)	0 (0)	0 (0)	2 (4)	7 (13)	30 (55)	10 (18)	3 (5)	0 (0)	0 (0)

CaGI-S, Caregiver Global Impression of Severity; mITT, modified intention-to-treat population

Supplemental Table 4. Sensitivity analysis where responses were imputed for patients with missing responses at week 52 and week 0 (for CaGI-S).

	Imputed Models				Complete Cases Model
	Estimated Odds	Delandistrogene moxeparvovec N (N Imputed)	Placebo N (N Imputed)	Prop Odds Tests N Fails / N Models	Estimated Odds
CaGI-C					
Rate any changes in symptoms	4.0 (2.0-7.8)	63 (5)	62 (1)	0 / 50	4.0 (2.0-8.0)
Rate any changes in physical ability	4.9 (2.4-9.8)	63 (4)	62 (1)	0 / 50	4.9 (2.5-10.0)
Rate any changes in activities ability	4.0 (2.0-7.9)	63 (4)	62 (1)	0 / 50	4.0 (2.0-8.0)
Rate any changes in overall health	3.8 (1.9-7.5)	63 (4)	62 (1)	0 / 50	3.8 (1.9-7.6)
CaGI-S					
Rate the severity of DMD symptoms	3.6 (1.6-7.9)	63 (10)	62 (4)	10 / 50	3.9 (1.8-8.8)
Rate the severity of impairment	4.4 (2.0-9.8)	63 (10)	62 (4)	5 / 50	4.4 (2.0-10.2)
Rate the ability of daily activities	2.2 (1.0-4.7)	63 (10)	62 (4)	15 / 50	2.2 (1.0-4.9)
Rate the severity of overall health	3.8 (1.7-8.5)	63 (10)	62 (4)	10 / 50	3.6 (1.7-8.3)

CaCI-C, CaGI-S, Caregiver Global Impression of Change; CaGI-S, Caregiver Global Impression of Severity; DMD, Duchenne muscular dystrophy