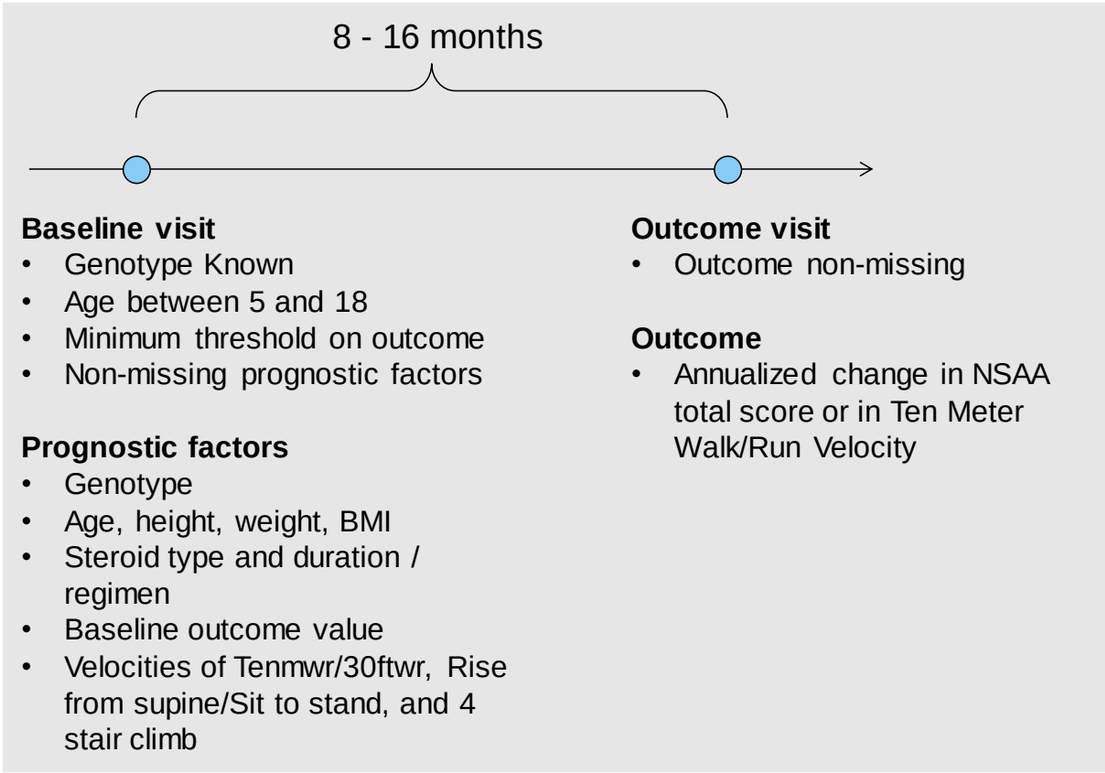
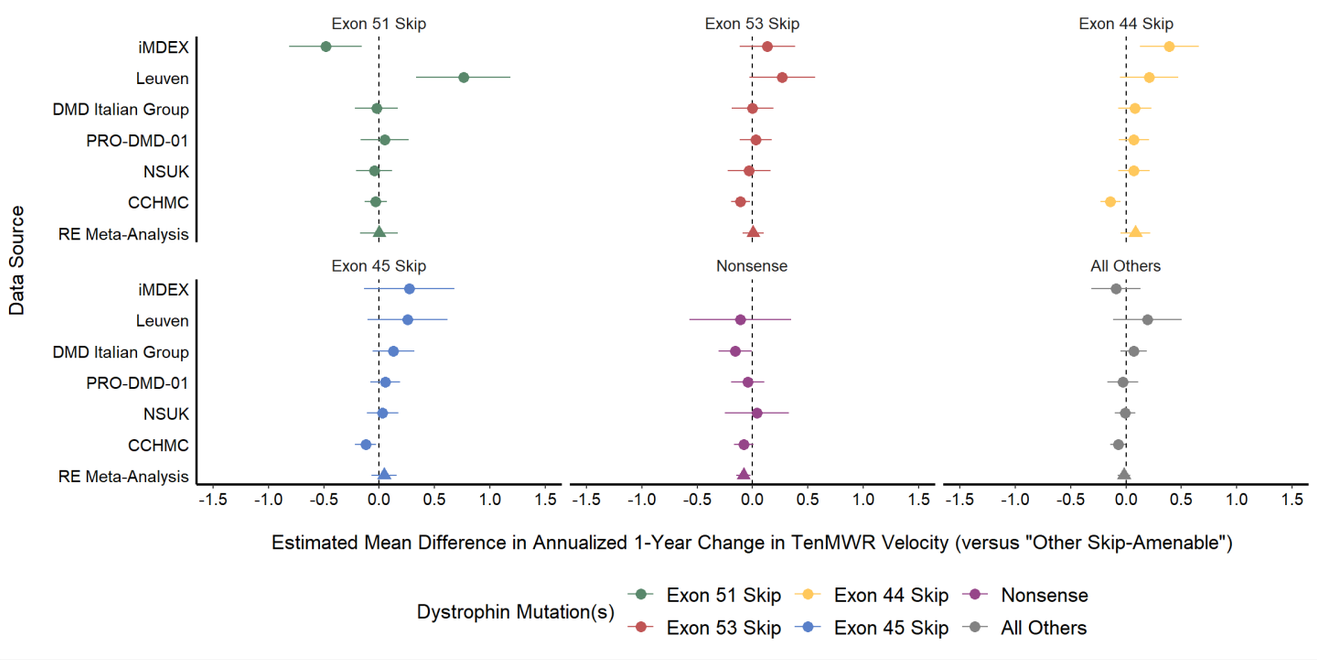
SUPPLEMENTARY FIGURES AND TABLES

eFigure 1. Study design for 1-year changes in motor function



eFigure 2. Meta-analysis of adjusted genotype effects on 1-year Δ10MWR velocity vs. other skip-amenable genotypes across data sources



eTable 1: Data source characteristics

PRO-DMD-01	PRO-DMD-01 (NCT01753804) was a prospective observational study of disease progression in 269 boys with DMD from 16 centers worldwide. Data were provided by CureDuchenne, a 501(3)c DMD patient foundation. Study assessments were scheduled every 6 months, and primarily occurred between 2012 and 2015.
Data source type	Prospectively collected natural history data
Study identifier	NCT01753804
Study locations	16 sites across USA, South America, and Europe
Key inclusion & exclusion criteria	Genetically proven DMD; age 3 to 18 years; willing and able to comply with protocol requirements; life expectancy of at least 3 years; able to give informed assent and/or consent in writing signed by the subject and/or parent(s)/legal guardian
Typical standard of care, including glucocorticoid use and physical therapy	At baseline 208 subjects (78%) were using steroids for DMD, mainly in a continuous (56.2%) or intermittent (15.4%) regimen, and 59 (22.1%) used none (mostly younger boys).
Genotyping	Mutations in DMD genes were confirmed by DNA diagnostic techniques including but not limited to MLPA (Multiplex Ligation-dependent Probe Amplification), CGH (Comparative Genomic Hybridisation) of H-RMCA (High Resolution Melting Curve Analysis)
Leuven	Data were collected from boys with DMD during routine clinical practice at the Universitaire Ziekenhuizen pediatric neurology clinic in Leuven, Belgium. The database available for the present study included 155 boys with clinic visits occurring primarily from 2011-2016. Clinic visits occurred approximately every 6 months.
Data source type	Curated RWD from boys with DMD from routine clinical practice at the Universitaire Ziekenhuizen pediatric neurology clinic in Leuven, Belgium
Study identifier	-
Study locations	1 center in Belgium
Key inclusion & exclusion criteria	Genetically proven DMD; aged 4.5 to 17.5 years; no severe cognitive or behavioral disorder impairing compliance
Typical standard of care, including glucocorticoid use and physical therapy	Glucocorticoid usually prescribed from age of 4 to 6 years onwards; 90% received 0.90 mg/kg daily deflazacort; physical therapy advice for prevention of contractures
Genotyping	Genotyping was based on Multiplex Ligation-dependent Probe Amplification (MLPA) for deletions and duplications, with sequencing only when no alterations were detected with MLPA.
iMDEX	iMDEX (NCT02780492) was a natural history study in 87 boys with DMD from several centers in Europe. The iMDEX natural history study was funded by the Association Française contre les Myopathies. Study assessments occurred approximately every 6 months, and primarily occurred between 2013-2017.
Data source type	Prospective, longitudinal, multicenter observational study at neuromuscular centers
Study identifier	NCT02780492
Study locations	5 centers in Europe
Key inclusion & exclusion criteria	Diagnosis of DMD documented by MLPA or a standard genetic test for the disorder, genotypically confirmed to have an out-of-frame deletion(s) that could be corrected by skipping exon 51 or 53 or 45 or 44 or 46 or 50 or 52; ambulant children from 5 years old and teenagers with DMD; ability to walk independently for at least 75 meters in 6 minutes at recruitment; standard of care for DMD as recommended by the NorthStar UK and TREAT-NMD (i.e.: on glucocorticoids treatment); sufficiently preserved pulmonary function (FVC >30%) and absence of symptoms of cardiac failure
Typical standard of care, including glucocorticoid use and physical therapy	
Genotyping	Genotyping was based on Multiplex Ligation-dependent Probe Amplification (MLPA) for deletions and duplications, with sequencing only when no alterations were detected with MLPA.
North Star UK	The NSUK database contains clinical data for more than 500 ambulant boys who were treated according to current standards of care in the UK. Data were collected from 24 pediatric neuromuscular centers in the NorthStar clinical network. Clinic visits occurred approximately every 6 months and, in the data used for the present study, occurred primarily between 2005 and the present. Genotyping was based on Multiplex Ligation-dependent Probe Amplification (MLPA) for deletions and duplications, with sequencing only when no alterations were detected with MLPA.
Data source type	Prospective natural history study from specialist neuromuscular centers in the United Kingdom
Study identifier	-

Study locations	24 centers in the United Kingdom
Key inclusion & exclusion criteria	DMD diagnosis confirmed by genetic testing and/or a muscle biopsy
Typical standard of care, including glucocorticoid use and physical therapy	Treated according to current standards of care in the UK
Genotyping	Genotyping was based on Multiplex Ligation-dependent Probe Amplification (MLPA) for deletions and duplications, with sequencing only when no alterations were detected with MLPA.
CCHMC	Clinical practice data were obtained from the Comprehensive Neuromuscular Center at CCHMC. The database available for the present study included 600 boys diagnosed with DMD. Clinic visits occurred approximately every 6 to 12 months between 2008-2017 for the data used in the present study.
Data source type	Curated clinical data from electronic health records of boys with DMD from the Comprehensive Neuromuscular Center at CCHMC
Study identifier	-
Study locations	1 center in Cincinnati, USA
Key inclusion & exclusion criteria	Genetically proven DMD diagnosis
Typical standard of care, including glucocorticoid use and physical therapy	Standard care including prescribing glucocorticoid steroids usually starting from age 4 years and onwards; 88% received daily steroids; Stretching exercise and ankle foot orthoses use for prevention of contractures; Nutritional consultation for weight management and ensure good calcium and Vit D intakes
Genotyping	Multiplex PCR, Southern blot prior to availability of MLPA, CGH array, sequencing
DMD Italian Group	The database contains clinical data for 96 boys with DMD who were treated according to current standards of care in Italy. Data were collected during routine clinical practice in 13 neuromuscular clinical centers in the Italian Group registry and were curated at 12 month intervals for a total of 3 years during 2008-2013 for the data contributed to the present study.[47]
Data source type	Routine clinical practice data from neuromuscular clinical centers
Study identifier	-
Study locations	13 centers in Italy
Key inclusion & exclusion criteria	Genetically proven DMD diagnosis; age ≥5 years; walks independently ≥75 meters; no moderate or severe learning difficulties or behavioral problems; 3 yrs of annual (12 +/- 3 months) 6MWD assessment performed at the same center
Typical standard of care, including glucocorticoid use and physical therapy	92/96 patients on glucocorticoids; 80% on deflazacort; 42 on daily and 50 on intermittent
Genotyping	DNA diagnostic technique covering all DMD gene exons and/or a muscle biopsy. Mutations were classified according to the Leiden Muscular Dystrophy database.

eTable 2: Baseline characteristics included in fully adjusted models for change in NSAA and 10MWR velocity by data source (based on availability in each source)

Data Source	Baseline characteristics included in fully adjusted models
iMDEX	Genotype class, age, NSAA total score, 10MWR velocity, rise time velocity, 4SC velocity, current deflazacort, steroid duration, height, weight, BMI
Leuven	Genotype class, age, NSAA total score, 10MWR velocity, rise time velocity, 4SC velocity, current deflazacort, steroid duration, height, weight, BMI
DMD Italian Group	Genotype class, age, NSAA total score, 10MWR velocity, rise time velocity, steroid use (daily/intermittent/none), height
PRO-DMD-01	Genotype class, age, NSAA total score, 10MWR velocity, rise time velocity, 4SC velocity, current deflazacort, steroid duration, height, weight, BMI
NSUK	Genotype class, age, NSAA total score, 10MWR velocity, rise time velocity, current deflazacort, steroid duration, height, weight, BMI
CCHMC	Genotype class, age, NSAA total score, 30 foot walk/run velocity, 4SC velocity, sit to stand velocity, current deflazacort, steroid duration, height, weight, BMI

Base models in all data sources included only genotype class.
Intermediate models in all data sources included genotype class, age and NSAA total score.

eTable 3. Estimated associations between selected genotype classes and time from birth to 10MWR > 10s, and time from first 10MWR assessment to ambulatory milestone (10MWR > 10 seconds)

Analysis / Genotype class	HR*	95% CI	P-value
Measured as time from birth to milestone (age at milestone) and adjusting for data source (N=647)			
Skip 44 vs. all other skip-amenable	0.69	(0.47, 1.01)	0.06
Skip 51 vs. all other skip-amenable	1.62	(1.18, 2.22)	<0.01
Measured as time from baseline to milestone and adjusting for data source (N=590)			
Skip 44 vs. all other skip-amenable	0.79	(0.51, 1.21)	0.27
Skip 51 vs. all other skip-amenable	1.08	(0.73, 1.59)	0.69
Measured as time from baseline to milestone, adjusting for data source, age and 10MWR velocity (N=590)			
Skip 44 vs. all other skip-amenable	0.88	(0.57, 1.36)	0.56
Skip 51 vs. all other skip-amenable	1.03	(0.70, 1.52)	0.88

*Cox proportional hazards model; no departures from the proportional hazards assumption were detected.

eTable 4. Patient characteristics and outcomes by genotype class in a) age at 10MWR > 10s and b) time from baseline to 10MWR > 10s analyses

Characteristic	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All Others
Age at 10MWR > 10s analyses							
Number of patients	122	137	165	100	124	65	249
Number of patients with 10MWR > 10s	32	46	60	32	35	17	72
Median age (years) at first 10MWR > 10*	13.8	12.9	12.2	13.5	14.0	14.7	14.1
Time from baseline to 10MWR > 10s analyses							
Number of patients	114	126	137	94	120	62	217
Baseline characteristics							
Age (years)	7.87 ± 2.56	7.82 ± 2.40	7.14 ± 1.98	7.86 ± 2.53	7.78 ± 2.28	7.75 ± 2.70	7.41 ± 2.18
Age (years), median	7.31	7.13	6.61	7.13	7.53	7.4	6.7
10MWR (m/s)	5.22 ± 1.51	5.55 ± 1.59	5.75 ± 1.70	5.89 ± 1.65	5.19 ± 1.60	5.18 ± 1.45	4.99 ± 1.35
NSAA	23.64 ± 6.20	23.04 ± 6.44	21.52 ± 7.00	22.77 ± 6.88	23.33 ± 5.76	21.32 ± 7.73	22.66 ± 6.12
Deflazacort	43.8%	51.8%	38.8%	45.7%	47.3%	69.2%	62.6%
Daily steroids	74.2%	77.0%	62.5%	69.5%	76.3%	71.4%	90.2%
Outcomes							
Number of Patients with post-baseline 10MWR > 10s	26	35	36	23	31	16	51
Median time from baseline (years) to first 10MWR*	6.5	5.9	5.8	5.9	7.0	8.0	6.2

*Based on Kaplan-Meier estimates

eTable 5. Numbers of 1-year intervals and unique patients included in analyses of 1-year ΔNSAA

	All genotypes	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All others
Number of 1-year intervals	**1668**	**192**	**158**	**124**	**147**	**195**	**134**	**718**
iMDEX	52	23	7	2	8	5	0	7
Leuven	76	8	7	2	7	9	2	41
Italian Group	160	23	4	17	23	16	1	76
PRO-DMD-01	310	44	56	13	39	36	38	84
NSUK	505	46	42	43	25	43	13	293
CCHMC	565	48	42	47	45	86	80	217
Number of unique patients	**793**	**87**	**81**	**64**	**69**	**92**	**59**	**341**
iMDEX	31	11	5	2	5	4	0	4
Leuven	33	3	3	2	2	5	2	16
Italian Group	70	11	2	7	10	7	1	32
PRO-DMD-01	166	22	30	9	20	21	20	44
NSUK	276	21	25	25	17	23	6	159
CCHMC	217	19	16	19	15	32	30	86

eTable 6. Summary statistics for baseline age (years) by data source and genotype class (NSAA analysis sample)

Data source	All Genotypes	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All others
iMDEX								
Mean ± SD	8.36 ± 1.99	8.37 ± 1.31	8.06 ± 3.28	10.02 ± 4.01	8.00 ± 1.49	8.33 ± 2.33	-	8.57 ± 2.55
Median	7.8	7.93	6.57	10.02	7.59	7.88	-	7.66
Range	(5.61, 13.44)	(6.48, 11.05)	(5.68, 13.44)	(7.18, 12.85)	(6.25, 10.73)	(6.25, 12.14)	-	(5.61, 11.89)
Leuven								
Mean ± SD	9.51 ± 2.58	10.12 ± 1.60	7.36 ± 2.38	5.79 ± 0.81	12.68 ± 1.57	11.38 ± 1.20	11.25 ± 1.60	8.91 ± 2.41
Median	9.43	10.65	6.36	5.79	12.8	11.04	11.25	8.69
Range	(5.21, 15.72)	(7.32, 11.97)	(5.41, 12.14)	(5.21, 6.36)	(10.06, 14.94)	(9.76, 13.02)	(10.12, 12.38)	(5.61, 15.72)
DMD Italian Group								
Mean ± SD	8.79 ± 1.94	9.55 ± 2.43	10.24 ± 2.96	9.67 ± 1.24	8.88 ± 2.04	8.31 ± 2.30	7.00 ± -	8.39 ± 1.61
Median	8.7	9.9	10.49	9.83	8.6	7.75	7	8.4
Range	(5.00, 13.60)	(5.00, 13.33)	(6.80, 13.17)	(7.33, 12.20)	(6.00, 13.60)	(5.58, 12.83)	(7.00, 7.00)	(5.00, 11.70)
PRO-DMD-01								
Mean ± SD	8.93 ± 2.42	9.83 ± 3.02	8.70 ± 2.18	8.46 ± 2.57	8.71 ± 2.23	9.67 ± 2.58	8.23 ± 1.99	8.80 ± 2.25
Median	8.55	9.23	8.46	8.2	7.99	9.75	7.85	8.53
Range	(5.04, 17.97)	(5.04, 17.97)	(5.18, 14.84)	(5.34, 13.17)	(5.59, 13.16)	(5.07, 16.40)	(5.06, 13.59)	(5.25, 15.47)
NSUK								
Mean ± SD	8.33 ± 2.24	8.69 ± 2.27	8.01 ± 2.07	8.39 ± 1.75	8.65 ± 1.72	8.17 ± 2.53	8.44 ± 1.84	8.30 ± 2.35
Median	7.94	8.2	7.76	8.3	8.85	7.53	8.76	7.74
Range	(5.01, 16.79)	(5.03, 14.12)	(5.03, 13.50)	(5.37, 11.68)	(5.88, 11.44)	(5.14, 15.95)	(5.62, 11.64)	(5.01, 16.79)
CCHMC								
Mean ± SD	9.51 ± 2.63	9.49 ± 3.22	9.00 ± 2.04	8.90 ± 2.27	9.05 ± 1.92	10.52 ± 3.29	9.25 ± 2.51	9.54 ± 2.45
Median	9.22	8.79	9.09	8.76	9.05	10.17	9.21	9.43
Range	(5.01, 17.86)	(5.05, 17.86)	(5.04, 12.77)	(5.03, 13.31)	(5.01, 13.03)	(5.10, 17.72)	(5.06, 15.10)	(5.10, 17.02)

eTable 7. Summary statistics for baseline NSAA total score by data source and genotype class (NSAA analysis sample)

Data source	All Genotype classes	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All others
iMDEX								
Mean ± SD	25.06 ± 6.34	27.57 ± 4.05	22.43 ± 6.53	22.00 ± 14.14	22.38 ± 8.67	26.60 ± 7.64	-	22.29 ± 4.79
Median	25.5	28	23	22	23	31	-	21
Range	(12.00, 34.00)	(21.00, 34.00)	(15.00, 31.00)	(12.00, 32.00)	(12.00, 32.00)	(16.00, 33.00)	-	(17.00, 31.00)
Leuven								
Mean ± SD	25.43 ± 5.90	28.75 ± 4.89	23.43 ± 5.06	29.00 ± 1.41	26.00 ± 4.97	18.22 ± 4.15	20.50 ± 12.02	26.68 ± 5.31
Median	26.5	31	23	29	26	19	20.5	28
Range	(12.00, 33.00)	(18.00, 33.00)	(16.00, 29.00)	(28.00, 30.00)	(18.00, 31.00)	(12.00, 25.00)	(12.00, 29.00)	(14.00, 33.00)
DMD Italian Group								
Mean ± SD	25.44 ± 5.75	24.30 ± 6.83	24.25 ± 3.40	22.35 ± 5.06	25.52 ± 5.62	24.81 ± 5.58	17.00 ± -	26.76 ± 5.43
Median	27	24	23.5	24	27	27	17	28
Range	(12.00, 34.00)	(13.00, 33.00)	(21.00, 29.00)	(13.00, 31.00)	(12.00, 33.00)	(14.00, 31.00)	(17.00, 17.00)	(12.00, 34.00)
PRO-DMD-01								
Mean ± SD	24.37 ± 6.20	24.89 ± 6.66	24.05 ± 6.74	21.23 ± 6.80	22.79 ± 5.71	25.00 ± 6.36	25.26 ± 5.23	24.85 ± 5.98
Median	26	26.5	26	19	24	26	27	25
Range	(12.00, 34.00)	(12.00, 33.00)	(13.00, 34.00)	(14.00, 34.00)	(12.00, 33.00)	(12.00, 33.00)	(12.00, 32.00)	(12.00, 34.00)
NSUK								
Mean ± SD	23.55 ± 5.79	25.02 ± 5.91	23.55 ± 5.48	24.84 ± 5.33	20.80 ± 5.28	23.81 ± 5.37	27.85 ± 4.88	23.14 ± 5.88
Median	24	26	24	24	20	24	28	23
Range	(12.00, 34.00)	(12.00, 34.00)	(14.00, 34.00)	(14.00, 33.00)	(12.00, 32.00)	(15.00, 34.00)	(19.00, 33.00)	(12.00, 34.00)
CCHMC								
Mean ± SD	24.41 ± 5.88	23.83 ± 5.30	24.74 ± 5.36	22.96 ± 5.68	24.20 ± 5.59	27.85 ± 4.89	24.80 ± 5.98	23.33 ± 6.04
Median	25	23.5	26	24	25	29	26	23
Range	(12.00, 34.00)	(15.00, 33.00)	(13.00, 34.00)	(13.00, 32.00)	(14.00, 34.00)	(12.00, 34.00)	(12.00, 34.00)	(12.00, 34.00)

eTable 8. Summary statistics for annualized changes in NSAA total score by data source and genotype class (NSAA analysis sample)

Data source	All Genotype classes	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All others
iMDEX								
N (intervals)	52	23	7	2	8	5	0	7
Mean ± SD	-2.94 ± 4.48	-2.47 ± 4.57	-1.43 ± 4.79	-5.49 ± 0.56	-3.75 ± 3.63	-2.44 ± 2.38		-4.73 ± 6.44
Median	-3.00	-1.99	-3.01	-5.49	-4.05	-1.94		-3.96
Range	(-15.63, 6.52)	(-15.63, 6.02)	(-6.96, 6.52)	(-5.89, -5.10)	(-7.95, 3.11)	(-6.42, 0.00)		(-11.53, 5.92)
Leuven								
N (intervals)	76	8	7	2	7	9	2	41
Mean ± SD	-1.49 ± 4.08	-1.45 ± 3.54	1.34 ± 5.44	1.76 ± 2.49	-3.48 ± 3.83	-5.00 ± 3.35	-5.41 ± 1.51	-0.83 ± 3.64
Median	-1.54	-1.08	1.73	1.76	-5.07	-4.00	-5.41	0.00
Range	(-11.06, 7.09)	(-7.20, 4.91)	(-8.51, 6.71)	(0.00, 3.52)	(-8.02, 2.42)	(-11.06, -1.82)	(-6.48, -4.34)	(-10.78, 7.09)
DMD Italian Group								
N (intervals)	160	23	4	17	23	16	1	76
Mean ± SD	-2.13 ± 4.06	-1.99 ± 3.22	0.75 ± 1.89	-4.98 ± 4.63	-3.25 ± 4.89	-0.93 ± 2.76	-10.96 ± -	-1.48 ± 3.76
Median	-1.49	-1.00	1.49	-3.99	-3.99	-1.49	-10.96	-1.00
Range	(-13.95, 6.98)	(-8.97, 2.99)	(-1.99, 1.99)	(-13.95, 1.00)	(-13.95, 5.98)	(-4.98, 3.99)	(-10.96, -10.96)	(-13.95, 6.98)
PRO-DMD-01								
N (intervals)	310	44	56	13	39	36	38	84
Mean ± SD	-2.61 ± 4.40	-1.83 ± 3.64	-2.11 ± 4.30	-4.20 ± 5.59	-2.75 ± 4.37	-3.21 ± 4.36	-2.37 ± 5.04	-2.90 ± 4.40
Median	-2.03	-1.92	-2.06	-3.17	-2.79	-2.02	-1.53	-2.05
Range	(-17.00, 10.59)	(-12.00, 8.62)	(-10.93, 10.59)	(-16.31, 7.85)	(-12.48, 4.99)	(-17.00, 2.94)	(-13.25, 7.00)	(-15.72, 6.79)
NSUK								
N (intervals)	505	46	42	43	25	43	13	293
Mean ± SD	-2.42 ± 5.12	-1.08 ± 5.62	-2.33 ± 4.92	-3.87 ± 6.03	-3.73 ± 4.99	-2.22 ± 4.56	-3.09 ± 5.96	-2.31 ± 4.95
Median	-1.90	-0.93	-1.78	-3.19	-3.56	-1.97	-0.91	-1.94
Range	(-21.00, 10.68)	(-17.76, 9.72)	(-13.16, 10.22)	(-19.06, 7.47)	(-11.59, 9.16)	(-13.78, 7.67)	(-15.83, 2.69)	(-21.00, 10.68)
CCHMC								
N (intervals)	565	48	42	47	45	86	80	217
Mean ± SD	-2.23 ± 3.99	-1.76 ± 4.12	-2.25 ± 3.91	-2.30 ± 4.43	-2.16 ± 4.18	-0.99 ± 3.35	-2.45 ± 4.16	-2.75 ± 3.94
Median	-1.85	-1.01	-1.47	-2.85	-1.86	-0.93	-2.15	-2.29
Range	(-21.36, 9.21)	(-11.19, 8.67)	(-11.19, 9.21)	(-12.72, 6.98)	(-12.46, 6.04)	(-12.00, 5.58)	(-21.36, 5.78)	(-17.99, 7.44)

eTable 9. Numbers of 1-year intervals and unique patients included in analyses of 1-year Δ10MWR velocity

	All genotype classes	Skip 44	Skip 45	Skip 51	Skip 53	Other skip amen.	Non-sense	All others

Number of 1-year intervals	**1631**	**188**	**163**	**120**	**142**	**197**	**139**	**682**
iMDEX	51	21	7	2	8	6	0	7
Leuven	84	8	8	3	7	11	2	45
Italian Group	122	15	3	13	17	13	1	60
PRO-DMD-01	332	47	60	16	44	40	40	85
NSUK	442	46	38	33	19	41	12	253
CCHMC	600	51	47	53	47	86	84	232
Number of unique patients	**792**	**85**	**84**	**61**	**67**	**94**	**65**	**336**
iMDEX	32	11	5	2	5	5	0	4
Leuven	38	3	4	3	2	5	2	19
Italian Group	61	8	2	6	8	6	1	30
PRO-DMD-01	173	22	32	9	22	22	22	44
NSUK	256	21	24	20	15	24	6	146
CCHMC	232	20	17	21	15	32	34	93

eTable 10. Estimated genotype effects on 1-year Δ10MWR velocity

	Unadjusted				Adjusted			
	Estimated effect on 1-year change in 10MWR (m/s) (95% CI)	T	SE		Estimated effect on 1-year change in 10MWR (m/s) (95% CI)	T	SE	
Effects vs. other skip-amenable								
Skip 44	0.04 (-0.04, 0.12)	0.07	0.04		0.08 (-0.05, 0.22)	0.15	0.07	
Skip 45	0.06 (-0.06, 0.18)	0.10	0.06		0.05 (-0.07, 0.16)	0.10	0.06	
Skip 51	-0.03 (-0.10, 0.04)	0	0.04		0.00 (-0.17, 0.17)	0.18	0.09	
Skip 53	-0.00 (-0.07, 0.06)	0	0.03		0.01 (-0.09, 0.10)	0.08	0.05	
Nonsense	-0.11 (-0.19, -0.03)	0.06	0.04	*	-0.08 (-0.15, -0.02)	0	0.03	*
Effects vs. all others								
Skip 44	0.03 (-0.02, 0.08)	0	0.03		0.07 (-0.04, 0.18)	0.12	0.06	
Skip 45	0.02 (-0.04, 0.08)	0.02	0.03		0.03 (-0.04, 0.10)	0.04	0.04	
Skip 51	-0.02 (-0.10, 0.07)	0.06	0.04		0.00 (-0.16, 0.16)	0.17	0.08	
Skip 53	0.00 (-0.05, 0.06)	0	0.03		0.01 (-0.06, 0.07)	0.04	0.03	
Other skip-amenable	0.00 (-0.05, 0.05)	0	0.03		0.02 (-0.04, 0.07)	0.03	0.03	
Nonsense	-0.09 (-0.21, 0.02)	0.11	0.06		-0.08 (-0.19, 0.04)	0.10	0.06	

*p-value < 0.05;
T = the standard deviation of the mean across data sources
SE = standard error, a measure of uncertainty in the population mean

eTable 11. Estimated adjusted data-source specific effects and random-effects meta-analysis weights for genotype classes relative to the other skip-amenable group on 1-year ΔNSAA

	Skip 44			Skip 45			Skip 51			Skip 53			Nonsense			All other		
Data source	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**
iMDEX	0.06	(-3.72, 3.85)	4.2	-4.82	(-10.48, 0.84)	5.9	-1.73	(-7.10, 3.64)	3.3	-4.27	(-8.43, -0.10)	5.1				-2.10	(-5.94, 1.73)	3.8
Leuven	2.34	(-0.32, 5.00)	7.9	4.96	(2.06, 7.85)	13.3	3.21	(-1.05, 7.47)	5.0	3.19	(-0.37, 6.75)	6.7	-1.77	(-5.40, 1.85)	17.3	1.75	(-0.96, 4.46)	7.0
DMD Italian Group	-0.12	(-1.77, 1.52)	17.2	1.59	(-0.14, 3.32)	18.8	-3.03	(-5.47, -0.59)	13.5	-1.53	(-3.64, 0.58)	15.2	-9.32	(-11.26, -7.38)	20.5	-0.42	(-1.83, 0.99)	18.1
PRO-DMD-01	0.29	(-1.06, 1.64)	22.4	-0.20	(-1.49, 1.10)	20.8	-0.92	(-3.47, 1.62)	12.6	-0.92	(-2.40, 0.56)	23.0	0.16	(-1.44, 1.75)	21.0	-0.78	(-2.11, 0.55)	19.3
NSUK	1.27	(-0.14, 2.69)	21.2	-0.06	(-1.60, 1.48)	19.7	-1.24	(-3.05, 0.56)	21.5	-0.77	(-2.35, 0.81)	21.6	-1.01	(-3.47, 1.45)	19.6	-0.16	(-1.29, 0.98)	22.8
CCHMC	-0.64	(-1.79, 0.51)	27.2	-1.32	(-2.44, -0.20)	21.6	-1.49	(-2.44, -0.53)	44.1	-1.17	(-2.35, -0.00)	28.4	-1.61	(-2.60, -0.62)	21.7	-1.57	(-2.41, -0.74)	29.0
Random effects meta-analysis	**0.33**	**(-0.47, 1.12)**		**0.34**	**(-1.23, 1.91)**		**-1.34**	**(-2.33, -0.35)**		**-0.95**	**(-1.95, 0.05)**		**-2.73**	**(-5.95, 0.49)**		**-0.68**	**(-1.46, 0.11)**	
p-value	0.042			0.67			0.079			0.063			0.10			0.091		

eTable 12. Estimated adjusted data-source specific effects and random-effects meta-analysis weights for genotype classes relative to the other skip-amenable group on 1-year Δ10MWR velocity

	Skip 44			Skip 45			Skip 51			Skip 53			Nonsense			All other		
Data source	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**	**Mean difference**	**95% CI**	**Weight %**
iMDEX	0.39	(0.13, 0.66)	12.3	0.27	(-0.13, 0.68)	6.2	-0.48	(-0.81, -0.16)	12.7	0.13	(-0.12, 0.38)	10.8				-0.09	(-0.31, 0.13)	6.1
Leuven	0.21	(-0.05, 0.47)	12.3	0.26	(-0.11, 0.62)	7.5	0.76	(0.34, 1.19)	9.6	0.27	(-0.03, 0.57)	8.3	-0.11	(-0.57, 0.35)	2.0	0.19	(-0.12, 0.50)	3.2
DMD Italian Group	0.08	(-0.07, 0.23)	17.9	0.13	(-0.06, 0.32)	16.9	-0.02	(-0.22, 0.17)	18.5	0.00	(-0.19, 0.19)	15.7	-0.16	(-0.30, -0.01)	19.1	0.07	(-0.05, 0.19)	17.8
PRO-DMD-01	0.07	(-0.07, 0.21)	18.5	0.05	(-0.08, 0.19)	22.1	0.05	(-0.17, 0.27)	17.3	0.03	(-0.12, 0.17)	20.5	-0.04	(-0.19, 0.11)	18.8	-0.03	(-0.17, 0.11)	13.7
NSUK	0.07	(-0.07, 0.21)	18.3	0.03	(-0.11, 0.17)	21.3	-0.04	(-0.21, 0.12)	19.8	-0.03	(-0.23, 0.16)	15.1	0.04	(-0.25, 0.33)	5.0	-0.01	(-0.10, 0.09)	24.7
CCHMC	-0.14	(-0.23, -0.05)	20.7	-0.12	(-0.22, -0.03)	26.0	-0.03	(-0.13, 0.07)	22.2	-0.11	(-0.19, -0.02)	29.5	-0.08	(-0.17, 0.01)	55.1	-0.07	(-0.14, -0.00)	34.4
Random effects meta-analysis	**0.08**	**(-0.05, 0.22)**		**0.05**	**(-0.07, 0.16)**		**0.00**	**(-0.17, 0.17)**		**0.01**	**(-0.09, 0.10)**		**-0.08**	**(-0.15, -0.02)**		**-0.02**	**(-0.07, 0.04)**	
p-value	0.22			0.44			1			0.91			0.014			0.54		