Table 1 Demographic and other baseline characteristics (FAS)

	SA-ER (N=10)	Placebo (N=4)	Total (N=14)
Sex (n [%])			
Male	3 (30.0)	1 (25.0)	4 (28.6)
Female	7 (70.0)	3 (75.0)	10 (71.4)
Age (years) [a)]			
Mean (SD)	41.3 (6.1)	37.0 (6.9)	40.1 (6.4)
Median	40.5	39.5	40.5
Min, Max	32, 50	27, 42	27, 50
Weight (kg) [b)]			
Mean (SD)	58.22 (10.18)	55.93 (14.84)	57.56 (11.12)
Median	56.90	49.05	52.75
Min, Max	48.3, 78.3	47.5, 78.1	47.5, 78.3
Duration of illness (years) [c)]			
Mean (SD)	10.8 (2.6)	10.5 (2.6)	10.7 (2.5)
Median	10.5	11.0	10.5
Min, Max	6, 15	7, 13	6, 15
Age of first GNE myopathy symptom (years) [d)]			
Mean (SD)	31.4 (6.0)	27.5 (5.1)	30.3 (5.8)
Median	30.5	28.5	30.5
Min, Max	20, 41	21, 32	20, 41
UEC score at baseline (Pattern: Grip strength of 4 kg or less treated as 0 kg)			
Mean (SD)	32.77 (12.37)	26.55 (8.74)	30.99 (11.50)
Median	27.53	27.03	27.53
Min, Max	19.60, 55.40	15.45, 36.70	15.45, 55.40

GNEM-FAS upper extremity score			
Mean (SD)	27.4 (2.8)	28.0 (2.2)	27.6 (2.5)
Median	26.5	27.5	27.0
Min, Max	24, 32	26, 31	24, 32
Prior SA-ER administrated (n [%])			
Yes	4 (40.0)	1 (25.0)	5 (35.7)

GNEM-FAS, GNE myopathy-Functional Activity Scale; Max, maximum; Min, minimum; N, number of subjects in the treatment group; SA-ER, sialic acid extended-release; SD, standard deviation; UEC, upper extremity composite.

a) Obtained informed consent

b) Screening

c) Duration of illness (years) = Year of informed consent - Year first symptom noted

d) Age of first GNE myopathy symptom (years) = Year first symptom noted - Year of birth

Table 2 Linear mixed effect model of change from baseline in UEC score (FAS)

Treatment group	Analysis visit	n	LSM	Standard error	Difference in LSM [a)]	95% CI for difference in LSM [a)]		
						Lower		Upper
SA-ER	Week 12	10	0.935	1.091	2.035	-2.195	-	6.265
	Week 24	10	0.840	1.091	1.653	-2.577	-	5.882
	Week 36	10	0.615	1.091	3.365	-0.865	-	7.595
	Week 48	10	-0.115	1.091	2.510	-1.720	-	6.740
Placebo	Week 12	4	-1.100	1.726	-	-	-	-
	Week 24	4	-0.813	1.726	-	-	-	-
	Week 36	4	-2.750	1.726	-	-	-	-
	Week 48	4	-2.625	1.726	-	-	-	-

CI, confidence interval; FAS, full analysis set; LSM, least square mean; n, number of subjects analyzed; SA-ER, sialic acid extended-release; UEC, upper extremity composite.

The linear mixed effect model includes treatment group, visit, treatment group*visit as fixed effects and subject as random effect.

Degrees of freedom estimated using Kenward-Roger method.

Not imputed, because there are not missing values at Week 48.

a) SA-ER – Placebo

Table 3 Efficacy rate for investigator's integrated efficacy assessment (FAS)

Treatment Group	N	n	Effective	Ineffective	Undeter-minable	Efficacy Rate (%)	95% CI for Rate		
							Lower		Upper
SA-ER	10	10	7	3	0	70.0	34.75	-	93.33
Placebo	4	4	2	2	0	50.0	6.76	-	93.24

CI, confidence interval; FAS, full analysis set; N, number of subjects in the treatment group; n, number of subjects assessed; SA-ER, sialic acid extended-release.

Table 4 Summary of adverse events (safety population)

	SA-ER (N=10) n (%) [Number of Events]	Placebo (N=4) n (%) [Number of Events]
Any adverse events	9 (90.0) [55]	4 (100.0) [17]
Any adverse drug reactions	0 (0.0) [0]	0 (0.0) [0]
Serious adverse events	1 (10.0) [1]	1 (25.0) [1]
Serious adverse drug reactions	0 (0.0) [0]	0 (0.0) [0]
Adverse events leading to death	0 (0.0) [0]	0 (0.0) [0]
Adverse events leading to drug withdrawn	0 (0.0) [0]	0 (0.0) [0]
Adverse events occurring in 2 or more subjects		
Eye disorders	2 (20.0) [2]	0 (0.0) [0]
Dry eye	2 (20.0) [2]	0 (0.0) [0]
Gastrointestinal disorders	6 (60.0) [19]	2 (50.0) [5]
Diarrhoea	2 (20.0) [11]	1 (25.0) [1]
Gastrooesophageal reflux disease	2 (20.0) [2]	0 (0.0) [0]
General disorders and administration site conditions	2 (20.0) [2]	2 (50.0) [2]
Pyrexia	2 (20.0) [2]	2 (50.0) [2]
Immune system disorders	3 (30.0) [4]	1 (25.0) [1]
Immunisation reaction	3 (30.0) [4]	1 (25.0) [1]
Infections and infestations	3 (30.0) [5]	2 (50.0) [2]
Nasopharyngitis	2 (20.0) [3]	2 (50.0) [2]

N, number of subjects in the treatment group; MedDRA, Medical Dictionary for Regulatory Activities; n, number of subjects with events; SA-ER, sialic acid extended-release.

Subjects with more than one event within a Preferred Term or System Organ Class are counted once for each category.

Adverse events were encoded according to MedDRA Ver. 25.0.