



## Clinical trial results:

### Duchenne muscular dystrophy: double-blind randomized trial to find optimum steroid regimen

#### Summary

EudraCT number	2010-023744-33
Trial protocol	GB IT
Global end of trial date	31 August 2023

#### Results information

Result version number	v1 (current)
This version publication date	19 June 2024
First version publication date	19 June 2024

#### Trial information

##### Trial identification

Sponsor protocol code	4.0
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##### Additional study identifiers

ISRCTN number	ISRCTN46102316
ClinicalTrials.gov id (NCT number)	NCT01603407
WHO universal trial number (UTN)	-

Notes:

##### Sponsors

Sponsor organisation name	University of Rochester
Sponsor organisation address	518 Hylan Building, Rochester, United States, NY14627
Public contact	Kim Hart, University of Rochester, 001 585 275 3767, Kim_Hart@URMC.Rochester.edu
Scientific contact	Kim Hart, University of Rochester, 001 585 275 3767, Kim_Hart@URMC.Rochester.edu

Notes:

##### Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

## Results analysis stage

Analysis stage	Final
Date of interim/final analysis	15 November 2019
Is this the analysis of the primary completion data?	Yes
Primary completion date	15 November 2019
Global end of trial reached?	Yes
Global end of trial date	31 August 2023
Was the trial ended prematurely?	No

Notes:

## General information about the trial

Main objective of the trial:

The proposed randomized controlled trial will compare three corticosteroid regimens to address the pragmatic hypothesis that daily corticosteroids (prednisone or deflazacort) will be of greater benefit in terms of function and subject/parent satisfaction than intermittent corticosteroids (prednisone).

Protection of trial subjects:

None

Background therapy:

None

Evidence for comparator:

The comparators used in the trial are daily prednisone, intermittent prednisone, and daily deflazacort. All three regimens are in common use in boys with Duchenne muscular dystrophy. Prednisone is a corticosteroid licenced for use in a wide range of conditions, and has been shown to improve muscle function in boys with DMD. Prednisone, rather than prednisolone, is the formulation of choice for the purposes of this trial. Prednisone is not currently licenced for use in DMD and will therefore be considered an Investigational Medicinal Product (IMP) for the purposes of this trial. Deflazacort is a glucocorticoid derived from prednisolone, and is licenced in Europe for use in a wide range of conditions. Deflazacort is not currently licenced for the indication of muscular dystrophy and there has been less exposure of children to deflazacort compared to prednisone in clinical trials. Moreover, deflazacort is not approved for any indication in the USA. Therefore for the purposes of this trial it will be considered an IMP. Despite the publication of consensus statements on the topic of the use of steroids to treat DMD, patient experience continues to be that widely different corticosteroid regimens remain in use.

Actual start date of recruitment	30 January 2013
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

## Population of trial subjects

### Subjects enrolled per country

Country: Number of subjects enrolled	United Kingdom: 59
Country: Number of subjects enrolled	Germany: 20
Country: Number of subjects enrolled	Italy: 22
Country: Number of subjects enrolled	United States: 82
Country: Number of subjects enrolled	Canada: 13
Worldwide total number of subjects	196
EEA total number of subjects	101

Notes:

<b>Subjects enrolled per age group</b>	
In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	196
Adolescents (12-17 years)	0
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details:

The recruitment period ran from 30 January 2013 until 17 September 2016, at 32 sites in the USA, Canada, the UK, Germany and Italy.

### Pre-assignment

Screening details:

All DMD patients' families were told of the trial and, if eligible, could consider participation. Clinical staff identified eligible patients via direct contact and electronic records. Potential participants were given brief trial information, and could agree to approach by research staff. Interested patients gave consent and were randomised.

### Period 1

Period 1 title	Baseline
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst, Carer, Assessor

### Arms

Are arms mutually exclusive?	Yes
<b>Arm title</b>	Daily prednisone

Arm description:

0.75 mg/kg/day prednisone, administered to participants daily, in the morning at home.

Arm type	Experimental
Investigational medicinal product name	Prednisone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.75 mg/kg/day

<b>Arm title</b>	Daily deflazacort
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Arm description:

0.9 mg/kg/day deflazacort, administered to participants daily, in the morning at home.

Arm type	Experimental
Investigational medicinal product name	Deflazacort
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.9 mg/kg/day

<b>Arm title</b>	Intermittent prednisone
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Arm description:

0.75 mg/kg/d prednisone for 10 days alternating with 10 days off, administered to participants daily, in the morning at home.

Arm type	Experimental
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Investigational medicinal product name	Prednisone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.75 mg/kg/day, 10 days on, 10 days off (placebo on 'off' days)

Number of subjects in period 1	Daily prednisone	Daily deflazacort	Intermittent prednisone
Started	65	65	66
Completed	65	65	66

## Period 2

Period 2 title	Months 3-36
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst, Carer, Assessor

## Arms

Are arms mutually exclusive?	Yes
<b>Arm title</b>	Daily prednisone

Arm description:

0.75 mg/kg/day prednisone, administered to participants daily, in the morning at home.

Arm type	Experimental
Investigational medicinal product name	Prednisone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.75 mg/kg/day

<b>Arm title</b>	Daily deflazacort
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Arm description:

0.9 mg/kg/day deflazacort, administered to participants daily, in the morning at home.

Arm type	Experimental
Investigational medicinal product name	Deflazacort
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.9 mg/kg/day

<b>Arm title</b>	Intermittent prednisone
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Arm description:

0.75 mg/kg/d prednisone for 10 days alternating with 10 days off, administered to participants daily, in the morning at home.

Arm type	Experimental
Investigational medicinal product name	Prednisone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Once daily administration, at 0.75 mg/kg/day, 10 days on, 10 days off (placebo on 'off' days)

<b>Number of subjects in period 2</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone
Started	65	65	66
Completed	54	54	56
Not completed	11	11	10
Consent withdrawn by subject	5	3	7
Adverse event, non-fatal	1	1	1
Unknown	1	-	-
Non-compliance with protocol	2	2	-
Entered a different trial	2	2	-
Lost to follow-up	-	3	2

## Baseline characteristics

### Reporting groups

Reporting group title	Daily prednisone
Reporting group description: 0.75 mg/kg/day prednisone, administered to participants daily, in the morning at home.	
Reporting group title	Daily deflazacort
Reporting group description: 0.9 mg/kg/day deflazacort, administered to participants daily, in the morning at home.	
Reporting group title	Intermittent prednisone
Reporting group description: 0.75 mg/kg/d prednisone for 10 days alternating with 10 days off, administered to participants daily, in the morning at home.	

Reporting group values	Daily prednisone	Daily deflazacort	Intermittent prednisone
Number of subjects	65	65	66
Age categorical Units: Subjects			
Children (2-11 years)	65	65	66
Age continuous Units: years arithmetic mean standard deviation	5.9 ± 1.0	5.8 ± 1.0	6.0 ± 1.1
Gender categorical Units: Subjects			
Female	0	0	0
Male	65	65	66
Country Units: Subjects			
USA	27	27	28
Canada	5	4	4
UK	19	20	20
Germany	6	7	7
Italy	8	7	7
Race Units: Subjects			
White	59	58	49
Black	2	0	1
Asian	1	2	6
Mixed	1	1	3
Other	1	2	4
Missing	1	2	3
Ethnicity Units: Subjects			
Non-hispanic	56	51	50
Hispanic	9	9	13
Missing	0	5	3
Mutation			

Units: Subjects			
No	1	1	0
Yes	64	64	66
Mother DMD Carrier			
Units: Subjects			
No	18	13	16
Yes	24	32	27
Missing	23	20	23
Family history of diabetes			
Units: Subjects			
No	42	34	36
Yes	21	30	29
Missing	2	1	1
Family history of tuberculosis			
Units: Subjects			
No	60	55	58
Yes	1	2	3
Missing	4	8	5
Speech and language difficulties			
Units: Subjects			
No	41	40	39
Yes	24	24	27
Missing	0	1	0
Presence of learning difficulties			
Units: Subjects			
No	47	43	41
Yes	13	17	20
Missing	5	5	5
Severity of learning difficulties			
Units: Subjects			
None	47	43	41
Mild	10	13	10
Moderate	2	2	10
Severe	1	0	0
Missing	5	7	5
Autism diagnosis			
Units: Subjects			
No	65	64	64
Yes	0	1	2
ADHD diagnosis			
Units: Subjects			
No	63	65	63
Yes	2	0	3
Previous surgery for DMD			
Units: Subjects			
No	63	60	64
Yes	2	5	2
Weight band			
Units: Subjects			
Band A	38	41	39
Band B	26	18	25



Band C	1	6	2
Initiation of Vitamin D			
Units: Subjects			
Yes	1	1	1
Missing	64	64	65
NSAA1 (Standing)			
Units: Subjects			
Unable	0	1	0
Modified	3	9	2
Able	60	55	62
Missing	2	0	2
NSAA2 (Walk (10 meters))			
Units: Subjects			
Modified	9	14	11
Able	54	51	53
Missing	2	0	2
NSAA3 (Stand up from chair)			
Units: Subjects			
Unable	0	1	0
Modified	22	20	20
Able	41	44	44
Missing	2	0	2
NSAA4 (Stand on one leg (Right))			
Units: Subjects			
Unable	3	3	3
Modified	37	43	40
Able	23	19	21
Missing	2	0	2
NSAA5 (Stand on one leg (Left))			
Units: Subjects			
Unable	2	3	2
Modified	38	41	40
Able	23	21	22
Missing	2	0	2
NSAA6 (Climb step (Right))			
Units: Subjects			
Unable	1	4	5
Modified	30	27	25
Able	32	34	34
Missing	2	0	2
NSAA7 (Climb step (Left))			
Units: Subjects			
Unable	3	5	2
Modified	33	28	35
Able	27	32	27
Missing	2	0	2
NSAA8 (Descend step (Right))			
Units: Subjects			
Unable	1	3	0
Modified	45	42	46
Able	17	20	18

Missing	2	0	2
NSAA9 (Descend step (Left))			
Units: Subjects			
Unable	2	3	3
Modified	40	46	47
Able	21	16	14
Missing	2	0	2
NSAA10 (Gets to sitting)			
Units: Subjects			
Unable	1	1	0
Modified	42	50	55
Able	20	14	9
Missing	2	0	2
NSAA11 (Rise from the floor)			
Units: Subjects			
Unable	2	1	3
Modified	59	64	58
Able	2	0	3
Missing	2	0	2
NSAA12 (Lifts head from supine)			
Units: Subjects			
Unable	15	16	20
Modified	34	37	37
Able	14	12	7
Missing	2	0	2
NSAA13 (Stand on heels)			
Units: Subjects			
Unable	10	17	16
Modified	33	37	35
Able	20	11	13
Missing	2	0	2
NSAA14 (Jump)			
Units: Subjects			
Unable	10	10	11
Modified	22	20	13
Able	31	35	40
Missing	2	0	2
NSAA15 (Hop (Right))			
Units: Subjects			
Unable	28	31	24
Modified	27	29	37
Able	8	5	3
Missing	2	0	2
NSAA16 (Hop (Left))			
Units: Subjects			
Unable	29	30	29
Modified	24	30	32
Able	10	5	3
Missing	2	0	2
NSAA17 (Walk/Run 10 meters)			
Units: Subjects			

Unable	4	4	3
Modified	33	38	42
Able	26	23	19
Missing	2	0	2
Number of brothers with DMD			
Units: Subjects			
None	43	48	56
One	8	9	8
Two	2	0	0
Missing	12	8	2
IOWA Conners I/O Subscale Score >= 10			
Units: Subjects			
No	57	58	55
Yes	4	7	8
Missing	4	0	3
IOWA Conners O/D Subscale Score >=9			
Units: Subjects			
No	58	58	56
Yes	3	7	6
Missing	4	0	4
PARS-III Total Score <72			
Units: Subjects			
No	61	62	57
Yes	1	3	6
Missing	3	0	3

<b>Reporting group values</b>	Total		
Number of subjects	196		
Age categorical			
Units: Subjects			
Children (2-11 years)	196		
Age continuous			
Units: years			
arithmetic mean			
standard deviation	-		
Gender categorical			
Units: Subjects			
Female	0		
Male	196		
Country			
Units: Subjects			
USA	82		
Canada	13		
UK	59		
Germany	20		
Italy	22		
Race			
Units: Subjects			
White	166		
Black	3		

Asian	9		
Mixed	5		
Other	7		
Missing	6		
Ethnicity Units: Subjects			
Non-hispanic	157		
Hispanic	31		
Missing	8		
Mutation Units: Subjects			
No	2		
Yes	194		
Mother DMD Carrier Units: Subjects			
No	47		
Yes	83		
Missing	66		
Family history of diabetes Units: Subjects			
No	112		
Yes	80		
Missing	4		
Family history of tuberculosis Units: Subjects			
No	173		
Yes	6		
Missing	17		
Speech and language difficulties Units: Subjects			
No	120		
Yes	75		
Missing	1		
Presence of learning difficulties Units: Subjects			
No	131		
Yes	50		
Missing	15		
Severity of learning difficulties Units: Subjects			
None	131		
Mild	33		
Moderate	14		
Severe	1		
Missing	17		
Autism diagnosis Units: Subjects			
No	193		
Yes	3		
ADHD diagnosis Units: Subjects			

No	191		
Yes	5		
Previous surgery for DMD Units: Subjects			
No	187		
Yes	9		
Weight band Units: Subjects			
Band A	118		
Band B	69		
Band C	9		
Initiation of Vitamin D Units: Subjects			
Yes	3		
Missing	193		
NSAA1 (Standing) Units: Subjects			
Unable	1		
Modified	14		
Able	177		
Missing	4		
NSAA2 (Walk (10 meters)) Units: Subjects			
Modified	34		
Able	158		
Missing	4		
NSAA3 (Stand up from chair) Units: Subjects			
Unable	1		
Modified	62		
Able	129		
Missing	4		
NSAA4 (Stand on one leg (Right)) Units: Subjects			
Unable	9		
Modified	120		
Able	63		
Missing	4		
NSAA5 (Stand on one leg (Left)) Units: Subjects			
Unable	7		
Modified	119		
Able	66		
Missing	4		
NSAA6 (Climb step (Right)) Units: Subjects			
Unable	10		
Modified	82		
Able	100		
Missing	4		
NSAA7 (Climb step (Left))			

Units: Subjects			
Unable	10		
Modified	96		
Able	86		
Missing	4		
NSAA8 (Descend step (Right))			
Units: Subjects			
Unable	4		
Modified	133		
Able	55		
Missing	4		
NSAA9 (Descend step (Left))			
Units: Subjects			
Unable	8		
Modified	133		
Able	51		
Missing	4		
NSAA10 (Gets to sitting)			
Units: Subjects			
Unable	2		
Modified	147		
Able	43		
Missing	4		
NSAA11 (Rise from the floor)			
Units: Subjects			
Unable	6		
Modified	181		
Able	5		
Missing	4		
NSAA12 (Lifts head from supine)			
Units: Subjects			
Unable	51		
Modified	108		
Able	33		
Missing	4		
NSAA13 (Stand on heels)			
Units: Subjects			
Unable	43		
Modified	105		
Able	44		
Missing	4		
NSAA14 (Jump)			
Units: Subjects			
Unable	31		
Modified	55		
Able	106		
Missing	4		
NSAA15 (Hop (Right))			
Units: Subjects			
Unable	83		
Modified	93		

Able	16		
Missing	4		
NSAA16 (Hop (Left))			
Units: Subjects			
Unable	88		
Modified	86		
Able	18		
Missing	4		
NSAA17 (Walk/Run 10 meters)			
Units: Subjects			
Unable	11		
Modified	113		
Able	68		
Missing	4		
Number of brothers with DMD			
Units: Subjects			
None	147		
One	25		
Two	2		
Missing	22		
IOWA Conners I/O Subscale Score >= 10			
Units: Subjects			
No	170		
Yes	19		
Missing	7		
IOWA Conners O/D Subscale Score >=9			
Units: Subjects			
No	172		
Yes	16		
Missing	8		
PARS-III Total Score <72			
Units: Subjects			
No	180		
Yes	10		
Missing	6		

## End points

### End points reporting groups

Reporting group title	Daily prednisone
Reporting group description: 0.75 mg/kg/day prednisone, administered to participants daily, in the morning at home.	
Reporting group title	Daily deflazacort
Reporting group description: 0.9 mg/kg/day deflazacort, administered to participants daily, in the morning at home.	
Reporting group title	Intermittent prednisone
Reporting group description: 0.75 mg/kg/d prednisone for 10 days alternating with 10 days off, administered to participants daily, in the morning at home.	
Reporting group title	Daily prednisone
Reporting group description: 0.75 mg/kg/day prednisone, administered to participants daily, in the morning at home.	
Reporting group title	Daily deflazacort
Reporting group description: 0.9 mg/kg/day deflazacort, administered to participants daily, in the morning at home.	
Reporting group title	Intermittent prednisone
Reporting group description: 0.75 mg/kg/d prednisone for 10 days alternating with 10 days off, administered to participants daily, in the morning at home.	

### Primary: Reciprocal of time to rise from the floor averaged over months 3-36

End point title	Reciprocal of time to rise from the floor averaged over months 3-36
End point description:	
End point type	Primary
End point timeframe: Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	63	65	
Units: rises per second				
least squares mean (standard error)	0.2362 ( $\pm$ 0.008621)	0.2401 ( $\pm$ 0.008893)	0.1800 ( $\pm$ 0.008589)	

## Statistical analyses



<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7365
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.003939
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.02423
upper limit	0.0321

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.0562
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.08392
upper limit	-0.02848

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.06013
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.08807
upper limit	-0.0322

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**Primary: Forced Vital Capacity (FVC)**

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End point title	Forced Vital Capacity (FVC)
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End point description:

End point type	Primary
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End point timeframe:

Months 3-36

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End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	62	65	
Units: litre(s)				
least squares mean (standard error)	1.4374 (± 0.03103)	1.4015 (± 0.03191)	1.4608 (± 0.03097)	

**Statistical analyses**

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	123
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3904
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.03586
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.1362
upper limit	0.06451

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone

Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.569
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.02343
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.07551
upper limit	0.1224

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1518
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.05929
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.03995
upper limit	0.1585

### Primary: TSQM Global Satisfaction Subscale Score

End point title	TSQM Global Satisfaction Subscale Score
End point description:	
End point type	Primary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: Score				
least squares mean (standard error)	71.2278 ( $\pm$ 2.2508)	67.7708 ( $\pm$ 2.3229)	65.0729 ( $\pm$ 2.2550)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily deflazacort v Daily prednisone
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2456
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.457
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.6077
upper limit	3.6937

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0369
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-6.1549
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-13.2091
upper limit	0.8993

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone

Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3589
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.6979
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.7651
upper limit	4.3693

### Secondary: Forced Vital Capacity (FVC) %

End point title	Forced Vital Capacity (FVC) %
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	62	65	
Units: percent				
least squares mean (standard error)	96.7870 ( $\pm$ 1.8937)	98.0438 ( $\pm$ 1.9580)	92.7105 ( $\pm$ 1.8979)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	123
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.619
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.2568

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-4.8233
upper limit	7.3368

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1031
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-4.0765
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.0725
upper limit	1.9194

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0341
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-5.3333
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-11.3498
upper limit	0.6831

<b>Secondary: 10 meter walk/run velocity</b>	
End point title	10 meter walk/run velocity
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	63	65	
Units: second				
least squares mean (standard error)	0.2035 ( $\pm$ 0.004880)	0.2009 ( $\pm$ 0.005008)	0.1718 ( $\pm$ 0.004839)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6976
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.00262
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.01886
upper limit	0.01362

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.03165
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.04762
upper limit	-0.01567

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.02903
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.04515
upper limit	-0.0129

### Secondary: NSAA Total Score

End point title	NSAA Total Score
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	63	65	
Units: Score				
least squares mean (standard error)	23.7361 ( $\pm$ 0.6016)	24.0198 ( $\pm$ 0.6101)	20.7441 ( $\pm$ 0.5933)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7335
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.2836



Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.7216
upper limit	2.2889

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0004
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.992
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-4.9712
upper limit	-1.0129

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.2757
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.2581
upper limit	-1.2933

<b>Secondary: Six-minute Walk Test distance</b>	
End point title	Six-minute Walk Test distance
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	60	63	64	
Units: meter				
least squares mean (standard error)	384.95 ( $\pm$ 9.5798)	384.17 ( $\pm$ 9.6568)	346.81 ( $\pm$ 9.4059)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	123
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9532
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.7782
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-32.6647
upper limit	31.1083

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.004
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-38.1321
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-69.5948
upper limit	-6.6694

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0046
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-37.3538
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-68.7344
upper limit	-5.9733

### Secondary: TSQM Effectiveness Subscale

End point title	TSQM Effectiveness Subscale
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: Score				
least squares mean (standard error)	69.5998 ( $\pm$ 1.8774)	66.1078 ( $\pm$ 1.9212)	65.4464 ( $\pm$ 1.8872)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1618
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.492

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.4813
upper limit	2.4972

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0914
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-4.1534
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.0501
upper limit	1.7433

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7883
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.6614
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.5868
upper limit	5.2641

## Secondary: TSQM Side effects Subscale

End point title	TSQM Side effects Subscale
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	64	
Units: Score				
least squares mean (standard error)	85.3830 ( $\pm$ 2.1500)	83.1366 ( $\pm$ 2.2278)	85.9917 ( $\pm$ 2.1602)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4322
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.2463
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.124
upper limit	4.6313

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.829
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.6087
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.1734
upper limit	7.3909

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3135
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	2.8551
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.9524
upper limit	9.6625

### Secondary: Participant weight

End point title	Participant weight
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: kilogram(s)				
least squares mean (standard error)	26.3098 ( $\pm$ 0.3573)	24.8552 ( $\pm$ 0.3611)	26.2523 ( $\pm$ 0.3518)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0041
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.4545

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-2.6611
upper limit	-0.2479

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9076
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.05745
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.248
upper limit	1.1331

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0054
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.3971
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	0.2014
upper limit	2.5927

<b>Secondary: Participant height</b>	
End point title	Participant height
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: centimeter				
least squares mean (standard error)	116.81 ( $\pm$ 0.2584)	115.33 ( $\pm$ 0.2669)	119.87 ( $\pm$ 0.2617)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.48
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-2.3242
upper limit	-0.6358

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	3.054
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	2.2222
upper limit	3.8857

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.0001
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	4.534
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	3.6941
upper limit	5.3738

### Secondary: Body Mass Index (BMI)

End point title	Body Mass Index (BMI)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: kilogram(s)/square meter				
least squares mean (standard error)	18.9337 ( $\pm$ 0.2564)	18.3132 ( $\pm$ 0.2592)	18.0577 ( $\pm$ 0.2524)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0853
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.6205

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.4845
upper limit	0.2434

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0143
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.876
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.7292
upper limit	-0.02285

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4731
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.2555
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.1117
upper limit	0.6007

<b>Secondary: IOWA Conners Total Score</b>	
End point title	IOWA Conners Total Score
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	60	62	62	
Units: Score				
least squares mean (standard error)	8.0970 ( $\pm$ 0.6029)	8.5777 ( $\pm$ 0.6173)	9.2830 ( $\pm$ 0.6070)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	122
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5456
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.4807
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.4327
upper limit	2.394

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	122
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1358
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.186
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.7215
upper limit	3.0936

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Intermittent prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3713
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.7053
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.1914
upper limit	2.6021

### Secondary: PARS-III Total Score

End point title	PARS-III Total Score
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	62	63	
Units: Score				
least squares mean (standard error)	85.8447 ( $\pm$ 0.9183)	85.0151 ( $\pm$ 0.9406)	84.8066 ( $\pm$ 0.9340)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	123
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4933
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.8296

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.7417
upper limit	2.0825

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3917
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.0381
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.9519
upper limit	1.8757

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.8627
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.2085
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.1099
upper limit	2.6929

<b>Secondary: SDQ Total Difficulties Score</b>	
End point title	SDQ Total Difficulties Score
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	60	64	
Units: Score				
least squares mean (standard error)	11.5469 ( $\pm$ 0.5082)	12.1498 ( $\pm$ 0.5303)	11.7749 ( $\pm$ 0.5108)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	122
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3742
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.6029
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.0281
upper limit	2.2338

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7314
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.228
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.3704
upper limit	1.8264

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5776
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.3749
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.9944
upper limit	1.2446

### Secondary: Revised Rutter Scale Score

End point title	Revised Rutter Scale Score
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	60	62	65	
Units: Score				
least squares mean (standard error)	3.5921 ( $\pm$ 0.2799)	3.7675 ( $\pm$ 0.2866)	3.9236 ( $\pm$ 0.2780)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	122
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6371
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.1754

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.7192
upper limit	1.0701

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3657
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.3315
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.5494
upper limit	1.2125

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6692
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.1561
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-0.7229
upper limit	1.0351

## Secondary: Systolic Blood Pressure

End point title	Systolic Blood Pressure
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	



<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	65	
Units: mm Hg				
least squares mean (standard error)	105.22 ( $\pm$ 0.8427)	105.40 ( $\pm$ 0.8747)	103.47 ( $\pm$ 0.8461)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.8758
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.174
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-2.5056
upper limit	2.8536

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1099
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.755
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-4.3868
upper limit	0.8769

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.081
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.929
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-4.5772
upper limit	0.7193

## Secondary: Diastolic Blood Pressure

End point title	Diastolic Blood Pressure
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	62	64	
Units: mm Hg				
least squares mean (standard error)	63.3740 ( $\pm$ 0.7083)	61.6113 ( $\pm$ 0.7324)	61.9813 ( $\pm$ 0.7144)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0616
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.7628

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-4.0205
upper limit	0.495

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1316
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.3927
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.6085
upper limit	0.823

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.691
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.37
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-1.8699
upper limit	2.61

<b>Secondary: Pulse</b>	
End point title	Pulse
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	65	
Units: bpm				
least squares mean (standard error)	98.8095 ( $\pm$ 1.1039)	99.6571 ( $\pm$ 1.1453)	97.0617 ( $\pm$ 1.1040)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5605
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.8476
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-2.6545
upper limit	4.3496

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2252
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.7478
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.2092
upper limit	1.7135

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	129
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0735
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.5954
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.0693
upper limit	0.8785

### Secondary: PedsQL Physical functioning (Parent)

End point title	PedsQL Physical functioning (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	60.552123 ( $\pm$ 2.456210)	56.667290 ( $\pm$ 2.438880)	53.787578 ( $\pm$ 2.448008)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2376
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.884834

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-11.7375
upper limit	3.9678

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0362
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-6.764545
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-14.4738
upper limit	0.94469

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3583
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.879712
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.3624
upper limit	4.60301

<b>Secondary: PedsQL Emotional functioning (Parent)</b>	
End point title	PedsQL Emotional functioning (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	71.123574 ( $\pm$ 2.183412)	69.699984 ( $\pm$ 2.212084)	69.480768 ( $\pm$ 2.191095)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6251
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.42359
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.37755
upper limit	5.53037

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.569
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.642806
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.52941
upper limit	5.2438

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9382
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.219216
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.97003
upper limit	6.53159

### Secondary: PedsQL Social functioning (Parent)

End point title	PedsQL Social functioning (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	63.581032 ( $\pm$ 1.927473)	66.024577 ( $\pm$ 1.967497)	60.028922 ( $\pm$ 1.954941)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3372
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	2.443545



Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.6344
upper limit	8.5215

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.163
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.55211
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.6311
upper limit	2.52689

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0168
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-5.995655
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-11.983
upper limit	-0.00827

<b>Secondary: PedsQL School functioning (Parent)</b>	
End point title	PedsQL School functioning (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	68.097830 ( $\pm$ 1.900524)	66.653218 ( $\pm$ 1.914070)	66.397809 ( $\pm$ 1.953206)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5652
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.444611
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.44113
upper limit	4.5519

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5022
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.700021
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.74901
upper limit	4.34897

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9178
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.25541
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.16229
upper limit	5.65148

### Secondary: PedsQL Psychological health (Parent)

End point title	PedsQL Psychological health (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	67.442758 ( $\pm$ 1.652715)	67.495323 ( $\pm$ 1.659289)	65.522673 ( $\pm$ 1.679620)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9806
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.052566

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.11134
upper limit	5.21647

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3807
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.920085
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.1495
upper limit	3.30933

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3616
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.972651
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.13475
upper limit	3.18944

<b>Secondary: PedsQL Total Score (Parent)</b>	
End point title	PedsQL Total Score (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	62	64	64	
Units: Score				
least squares mean (standard error)	64.878617 ( $\pm$ 1.671003)	63.711112 ( $\pm$ 1.690016)	61.334318 ( $\pm$ 1.689153)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5947
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.167505
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.40628
upper limit	4.07127

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily prednisone
Number of subjects included in analysis	126
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1098
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.544299
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.83474
upper limit	1.74614

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	128
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2803
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.376794
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.63231
upper limit	2.87872

### Secondary: PedsQL Physical functioning (Child)

End point title	PedsQL Physical functioning (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	54	52	49	
Units: Score				
least squares mean (standard error)	64.802642 ( $\pm$ 2.818842)	61.626840 ( $\pm$ 2.569129)	60.024621 ( $\pm$ 2.669149)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	106
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3501
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.175801

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-11.2954
upper limit	4.94375

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	103
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1923
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-4.778021
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-13.5339
upper limit	3.97783

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6332
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.602219
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.6192
upper limit	6.41478

<b>Secondary: PedsQL Emotional functioning (Child)</b>	
End point title	PedsQL Emotional functioning (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	54	52	47	
Units: Score				
least squares mean (standard error)	68.783278 ( $\pm$ 2.707352)	65.931150 ( $\pm$ 2.645838)	70.129170 ( $\pm$ 2.703500)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	106
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.396
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.852128
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.8762
upper limit	5.17198

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6982
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.345892
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.9442
upper limit	9.63594

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	99
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2158
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	4.19802
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-3.8992
upper limit	12.2952

### Secondary: PedsQL Social functioning (Child)

End point title	PedsQL Social functioning (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	54	52	47	
Units: Score				
least squares mean (standard error)	66.183571 ( $\pm$ 2.734419)	67.903617 ( $\pm$ 2.701055)	67.103568 ( $\pm$ 2.639465)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily deflazacort v Daily prednisone
Number of subjects included in analysis	106
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6174
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.720046

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.50173
upper limit	9.94182

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7909
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.919997
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.36573
upper limit	9.20572

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily deflazacort
Number of subjects included in analysis	99
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.8141
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.800049
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.92431
upper limit	7.32421

<b>Secondary: PedsQL School functioning (Child)</b>	
End point title	PedsQL School functioning (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	53	52	47	
Units: Score				
least squares mean (standard error)	66.997459 ( $\pm$ 2.571557)	64.376071 ( $\pm$ 2.561826)	65.459647 ( $\pm$ 2.537007)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	105
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.4193
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.621388
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.3734
upper limit	5.13058

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.639
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.537811
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.3671
upper limit	6.29145

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	99
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.7334
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.083577
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.5135
upper limit	8.68066

### Secondary: PedsQL Psychological health (Child)

End point title	PedsQL Psychological health (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	54	52	47	
Units: Score				
least squares mean (standard error)	68.030401 ( $\pm$ 2.220309)	66.535611 ( $\pm$ 2.172845)	67.787745 ( $\pm$ 2.291333)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	106
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.5906
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.49479

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.13045
upper limit	5.14088

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9353
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.242656
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.38778
upper limit	6.90247

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	99
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6666
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	1.252134
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.69057
upper limit	8.19483

<b>Secondary: PedsQL Total Score (Child)</b>	
End point title	PedsQL Total Score (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	54	52	47	
Units: Score				
least squares mean (standard error)	67.393595 ( $\pm$ 2.163314)	64.964480 ( $\pm$ 2.167527)	65.074246 ( $\pm$ 2.173933)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	106
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3875
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.429115
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.14448
upper limit	4.28625

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.424
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.319348
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.2513
upper limit	4.6126

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	99
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9683
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.109766
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.48556
upper limit	6.70509

### Secondary: PedsQL Neuromuscular Disease (NMD) (Parent)

End point title	PedsQL Neuromuscular Disease (NMD) (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	63	64	
Units: Score				
least squares mean (standard error)	80.451773 ( $\pm$ 1.297970)	78.932563 ( $\pm$ 1.341342)	77.958755 ( $\pm$ 1.329188)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.38
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.51921

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.65055
upper limit	2.61213

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.1474
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.493018
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.60089
upper limit	1.61485

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.574
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.973808
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.10986
upper limit	3.16224

<b>Secondary: PedsQL Communication (Parent)</b>	
End point title	PedsQL Communication (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	



<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	63	64	
Units: Score				
least squares mean (standard error)	70.761931 ( $\pm$ 2.905224)	70.401908 ( $\pm$ 2.991088)	67.041237 ( $\pm$ 3.072087)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.9256
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.360024
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-9.5599
upper limit	8.83985

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3416
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.720694
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-13.0598
upper limit	5.61846

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3832
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.360671
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-12.5607
upper limit	5.83933

### Secondary: PedsQL Family resources (Parent)

End point title	PedsQL Family resources (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	63	64	
Units: Score				
least squares mean (standard error)	81.638630 ( $\pm$ 1.948499)	79.053570 ( $\pm$ 2.008589)	74.842625 ( $\pm$ 1.988352)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.3145
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-2.58506

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-8.7193
upper limit	3.5492

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0079
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-6.796005
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-12.9005
upper limit	-0.69148

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Intermittent prednisone v Daily deflazacort
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0977
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-4.210945
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-10.2804
upper limit	1.8585

<b>Secondary: PedsQL Neuromuscular Module Total (Parent)</b>	
End point title	PedsQL Neuromuscular Module Total (Parent)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

<b>End point values</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	61	63	64	
Units: Score				
least squares mean (standard error)	79.573350 ( $\pm$ 1.388228)	77.934436 ( $\pm$ 1.442132)	76.000414 ( $\pm$ 1.422186)	

## Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily prednisone v Daily deflazacort
Number of subjects included in analysis	124
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.37
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.638914
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.0025
upper limit	2.72468

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	125
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0521
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-3.572935
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-7.96408
upper limit	0.81821

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
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Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	127
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.2927
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-1.934022
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.32235
upper limit	2.4543

### Secondary: PedsQL Neuromuscular Disease (Child)

End point title	PedsQL Neuromuscular Disease (Child)
End point description:	
End point type	Secondary
End point timeframe:	
Months 3-36	

End point values	Daily prednisone	Daily deflazacort	Intermittent prednisone	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	52	49	48	
Units: Score				
least squares mean (standard error)	80.418850 ( $\pm$ 1.802410)	79.495976 ( $\pm$ 1.785707)	79.977143 ( $\pm$ 1.754817)	

### Statistical analyses

<b>Statistical analysis title</b>	MMRM (daily prednisone vs daily deflazacort)
Comparison groups	Daily deflazacort v Daily prednisone
Number of subjects included in analysis	101
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.6849
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.922874

Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-6.35415
upper limit	4.5084

<b>Statistical analysis title</b>	MMRM (daily prednisone vs intermittent prednisone)
Comparison groups	Daily prednisone v Intermittent prednisone
Number of subjects included in analysis	100
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.8478
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	-0.441707
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.93813
upper limit	5.05472

<b>Statistical analysis title</b>	MMRM (daily deflazacort vs intermittent prednisone)
Comparison groups	Daily deflazacort v Intermittent prednisone
Number of subjects included in analysis	97
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.8348
Method	Mixed models analysis
Parameter estimate	Mean difference (net)
Point estimate	0.481167
Confidence interval	
level	Other: 98.3 %
sides	2-sided
lower limit	-5.02832
upper limit	5.99066

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

All non-SAEs/SARs occurring during drug treatment were reported on the eCRF system within four weeks of the form being due.

Adverse event reporting additional description:

All Adverse Events were recorded. PIs were responsible for managing all AEs/ARs according to local protocols.

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	2.1
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### Reporting groups

Reporting group title	Daily prednisone
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Reporting group description:

Participants receiving daily prednisone.

Reporting group title	Daily deflazacort
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Reporting group description:

Participants receiving daily deflazacort.

Reporting group title	Intermittent prednisone
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Reporting group description:

Participants receiving prednisone, 10 days on, 10 days off.

Serious adverse events	Daily prednisone	Daily deflazacort	Intermittent prednisone
Total subjects affected by serious adverse events			
subjects affected / exposed	7 / 65 (10.77%)	10 / 65 (15.38%)	5 / 66 (7.58%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Fracture			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Fall			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Head injury			

subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Surgical and medical procedures			
Adenotonsillectomy			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Tentomy			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Umbilical hernia repair			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Circumcision			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Orchidopexy			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Lymphadenopathy			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0



General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vomiting			
subjects affected / exposed	0 / 65 (0.00%)	2 / 65 (3.08%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Appendicitis			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infection			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	2 / 66 (3.03%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Incontinence			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Constipation			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Abdominal pain			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Respiratory, thoracic and mediastinal disorders			
Infection			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Epistaxis			
subjects affected / exposed	0 / 65 (0.00%)	0 / 65 (0.00%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Chromaturia			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Myoglobinuria			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hypercalciuria			
subjects affected / exposed	1 / 65 (1.54%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Rhabdomyolysis			
subjects affected / exposed	1 / 65 (1.54%)	2 / 65 (3.08%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 3	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Joint pain			
subjects affected / exposed	0 / 65 (0.00%)	1 / 65 (1.54%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intervertebral disc compression			

subjects affected / exposed	2 / 65 (3.08%)	0 / 65 (0.00%)	0 / 66 (0.00%)
occurrences causally related to treatment / all	2 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
<b>Infections and infestations</b>			
Infection			
subjects affected / exposed	1 / 65 (1.54%)	4 / 65 (6.15%)	1 / 66 (1.52%)
occurrences causally related to treatment / all	0 / 1	0 / 5	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Frequency threshold for reporting non-serious adverse events: 5 %

<b>Non-serious adverse events</b>	Daily prednisone	Daily deflazacort	Intermittent prednisone
Total subjects affected by non-serious adverse events			
subjects affected / exposed	61 / 65 (93.85%)	58 / 65 (89.23%)	55 / 66 (83.33%)
<b>Injury, poisoning and procedural complications</b>			
Fall			
subjects affected / exposed	10 / 65 (15.38%)	7 / 65 (10.77%)	12 / 66 (18.18%)
occurrences (all)	15	9	16
<b>Nervous system disorders</b>			
Headache			
subjects affected / exposed	9 / 65 (13.85%)	9 / 65 (13.85%)	6 / 66 (9.09%)
occurrences (all)	25	17	8
<b>General disorders and administration site conditions</b>			
Pyrexia			
subjects affected / exposed	12 / 65 (18.46%)	11 / 65 (16.92%)	10 / 66 (15.15%)
occurrences (all)	21	12	13
<b>Gastrointestinal disorders</b>			
Vomiting			
subjects affected / exposed	19 / 65 (29.23%)	16 / 65 (24.62%)	15 / 66 (22.73%)
occurrences (all)	34	25	21
Diarrhoea			
subjects affected / exposed	9 / 65 (13.85%)	7 / 65 (10.77%)	10 / 66 (15.15%)
occurrences (all)	11	8	11
Abdominal pain			

subjects affected / exposed occurrences (all)	8 / 65 (12.31%) 14	8 / 65 (12.31%) 10	8 / 66 (12.12%) 10
Abdominal pain upper subjects affected / exposed occurrences (all)	10 / 65 (15.38%) 11	4 / 65 (6.15%) 4	10 / 66 (15.15%) 12
Constipation subjects affected / exposed occurrences (all)	7 / 65 (10.77%) 9	9 / 65 (13.85%) 11	7 / 66 (10.61%) 9
Nausea subjects affected / exposed occurrences (all)	5 / 65 (7.69%) 5	4 / 65 (6.15%) 4	1 / 66 (1.52%) 1
Respiratory, thoracic and mediastinal disorders			
Cough subjects affected / exposed occurrences (all)	13 / 65 (20.00%) 20	12 / 65 (18.46%) 14	11 / 66 (16.67%) 22
Nasopharyngitis subjects affected / exposed occurrences (all)	9 / 65 (13.85%) 23	9 / 65 (13.85%) 18	11 / 66 (16.67%) 18
Upper respiratory tract infection subjects affected / exposed occurrences (all)	11 / 65 (16.92%) 19	11 / 65 (16.92%) 11	7 / 66 (10.61%) 10
Rhinitis subjects affected / exposed occurrences (all)	4 / 65 (6.15%) 5	5 / 65 (7.69%) 6	6 / 66 (9.09%) 8
Skin and subcutaneous tissue disorders			
Hypertrichosis subjects affected / exposed occurrences (all)	5 / 65 (7.69%) 7	7 / 65 (10.77%) 7	0 / 66 (0.00%) 0
Psychiatric disorders			
Abnormal behaviour subjects affected / exposed occurrences (all)	12 / 65 (18.46%) 14	17 / 65 (26.15%) 20	16 / 66 (24.24%) 20
Mood altered subjects affected / exposed occurrences (all)	4 / 65 (6.15%) 4	7 / 65 (10.77%) 8	2 / 66 (3.03%) 2
Aggression			

subjects affected / exposed occurrences (all)	4 / 65 (6.15%) 5	4 / 65 (6.15%) 4	2 / 66 (3.03%) 3
Renal and urinary disorders Pollakiuria subjects affected / exposed occurrences (all)	4 / 65 (6.15%) 8	2 / 65 (3.08%) 3	5 / 66 (7.58%) 6
Musculoskeletal and connective tissue disorders Pain in extremity subjects affected / exposed occurrences (all)  Back pain subjects affected / exposed occurrences (all)  Arthralgia subjects affected / exposed occurrences (all)	8 / 65 (12.31%) 9  5 / 65 (7.69%) 5  3 / 65 (4.62%) 3	10 / 65 (15.38%) 15  6 / 65 (9.23%) 6  6 / 65 (9.23%) 8	5 / 66 (7.58%) 7  8 / 66 (12.12%) 8  2 / 66 (3.03%) 2
Infections and infestations Ear infection subjects affected / exposed occurrences (all)  Influenza subjects affected / exposed occurrences (all)	4 / 65 (6.15%) 6  8 / 65 (12.31%) 10	7 / 65 (10.77%) 7  1 / 65 (1.54%) 1	8 / 66 (12.12%) 10  2 / 66 (3.03%) 2
Metabolism and nutrition disorders Abnormal weight gain subjects affected / exposed occurrences (all)  Vitamin D deficiency subjects affected / exposed occurrences (all)  Cushingoid subjects affected / exposed occurrences (all)	9 / 65 (13.85%) 12  7 / 65 (10.77%) 9  6 / 65 (9.23%) 7	4 / 65 (6.15%) 4  6 / 65 (9.23%) 9  6 / 65 (9.23%) 6	5 / 66 (7.58%) 5  5 / 66 (7.58%) 7  3 / 66 (4.55%) 3

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
12 June 2012	<p>Changes to:</p> <p>Study protocol Dear Colleagues EU Letter FAQ FOR DMD – EU GP Notification Letter Parent Consent Form Child PIS age four to five Parent PIS</p> <p>New documents:</p> <p>Instructions about study drug for families Letter for patients' associations/registries</p> <p>Document changed since previous submission:</p> <p>IOWA-Conners questionnaire</p> <p>Biobank information:</p> <p>Biobank FOR DMD Information Biobank Consent Form Adult Parents Biobank Information Sheet (adult) Biobank Information Sheet (child 10-16) Biobank Information Sheet (child &lt;10)</p>
09 December 2015	Substantial Amendment to set up Addenbrooke's Hospital, Cambridge, as a trial site.
24 August 2016	<p>Substantial Amendment covering:</p> <ul style="list-style-type: none"><li>• Protocol changes, as detailed in the enclosed document, covering:<ul style="list-style-type: none"><li>o A change to allow FOR-DMD trial participants to take part in other trials, after 36 months' participation in FOR-DMD</li><li>o A change to allow FOR-DMD trial participants to take part in FOR-DMD for a minimum of 36 rather than 60 months, with the option to remain on the trial to 60 months</li><li>o Only primary outcome measures and safety variables will be tracked at years four and five</li><li>o Participants will be unblinded at 60 months, with unblinding after 36 months only to allow the participant to take part in another trial, or for clinical need, rather than other reasons eg request of participant or his family</li><li>o Removal of some tests after 36 months' participation</li><li>o Changes to the Schedule of Evaluations, and screening procedures</li><li>o Changes regarding documentation of concomitant medication</li><li>o A decrease in the sample size from 300 to 225 participants, at 35, rather than 40, trial sites</li><li>o An increase in the recruitment period, from two and a half years to three years</li><li>o Clarification of terms, update of an address, and correction of typos</li></ul></li><li>• A change to the Reference Safety Information (RSI) for the trial From SmPC for deflazacort, 22 July 2009, and prednisone, 30 April 2009.</li></ul> <p>To Section 4.8 of SmPCs for deflazacort, 17 May 2015, and prednisone, 4 March 2013</p>

