Table 14.1.1: Patient Disposition (All Patients)

	Screen Failures	Low Dose	Proposed Therapeutic Dose
Screen Failures			
Patients Screened		3	10
Patients in the Enrolled Set		3	10
Patients in the ITT Set		0	0
Patients in the Safety Analysis Set		3 (100.0)	10 (100.0)
Patients in the Efficacy Completer Analysis Set		0	0
Patients Completed the Study		0	0
Patients Discontinued from the Study		0	0

Note: Percentages are based on the number of patients enrolled.
Program Name: t_sf_ds_sum.sas Output Generation: 17MAY2019 21:35

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

Parameter	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
Gestational age at birth (weeks)	n	2	8
	Mean (SD)	39.0 (1.41)	38.4 (1.51)
	Median	39.0	39.0
	Min, Max	38, 40	36, 40
Birth Weight (kg)	n	2	9
	Mean (SD)	3.2 (0.07)	3.4 (0.77)
	Median	3.2	3.2
	Min, Max	3, 3	2, 5
Birth length/height (cm)	n	1	2
	Mean (SD)	48.3 (-)	47.9 (0.57)
	Median	48.3	47.9
	Min, Max	48, 48	48, 48
Head circumference at birth (cm)	n	0	1
	Mean (SD)	0	35.0 (-)
	Median	0	35.0
	Min, Max	0	35, 35

Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA = spinal muscular atrophy.
* Age = (dose date - date of birth + 1).
Percentages are based on number of patients in Safety population for presented cohort.
Program Name: t_sf_dm_sum.sas Output Generation: 17MAY2019 21:35

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

Parameter	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
Age at Baseline (days)*	n	3	10
	Mean (SD)	1366.0 (70.76)	1011.4 (231.95)
	Median	1359.0	956.0
	Min, Max	1299, 1440	762, 1388
Age at Baseline (days)*	n	3	10
24 Months - 59 Months	n (%)	3 (100)	10 (100)
Weight at Baseline (kg)	n	3	10
	Mean (SD)	13.2 (1.37)	11.9 (1.34)
	Median	13.0	12.0
	Min, Max	12, 15	10, 15
Length/height at Baseline (cm)	n	3	10
	Mean (SD)	105.0 (9.63)	89.7 (3.70)
	Median	101.0	89.6
	Min, Max	98, 116	84, 96

Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA = spinal muscular atrophy.
* Age = (dose date - date of birth + 1).
Percentages are based on number of patients in Safety population for presented cohort.
Program Name: t_sf_dm_sum.sas Output Generation: 17MAY2019 21:35

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

Parameter	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
Gender	n	3	10
Female	n (%)	2 (66.7)	5 (50.0)
Male	n (%)	1 (33.3)	5 (50.0)
Ethnicity	n	3	10
Hispanic Or Latino	n (%)		1 (10.0)
Not Hispanic Or Latino	n (%)	3 (100)	9 (90.0)
Race	n	3	10
Other	n (%)		1 (10.0)
White	n (%)	3 (100)	9 (90.0)

Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA = spinal muscular atrophy.
* Age = (dose date - date of birth + 1).
Percentages are based on number of patients in Safety population for presented cohort.
Program Name: t_sf_dm_sum.sas Output Generation: 17MAY2019 21:35

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

Parameter	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
Familial history of SMA including affected siblings or parent carriers	n	3	10
Missing	n (%)		1 (10.0)
No	n (%)	2 (66.7)	6 (60.0)
Yes	n (%)	1 (33.3)	3 (30.0)
Parental history of SMA	n	1	3
Missing	n (%)	1 (33.3)	2 (20.0)
Both Are Carriers	n (%)		1 (10.0)
Siblings affected by SMA	n	1	3
Missing	n (%)		1 (10.0)
Siblings Affected	n (%)	1 (33.3)	2 (20.0)
Patient reported hospitalizations since birth	n	3	10
No	n (%)	1 (33.3)	6 (60.0)
Yes	n (%)	2 (66.7)	4 (40.0)

Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA = spinal muscular atrophy.
* Age = (dose date - date of birth + 1).
Percentages are based on number of patients in Safety population for presented cohort.
Program Name: t_sf_dm_sum.sas Output Generation: 17MAY2019 21:35

Table 14.3.1.1: Treatment Emergent Adverse Events (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term	Low Dose (N = 3) n (%) [m]	Proposed Therapeutic Dose (N = 10) n (%) [m]
Any TEAE	1 (33.3) [5]	5 (50.0) [11]
Cardiac disorders	1 (33.3) [1]	
Cardiac arrest	1 (33.3) [1]	
Infections and infestations	1 (33.3) [1]	3 (30.0) [4]
Bronchitis		1 (10.0) [1]
Gastroenteritis		1 (10.0) [1]
Pneumonia	1 (33.3) [1]	2 (20.0) [2]
Metabolism and nutrition disorders		2 (20.0) [3]
Dehydration		2 (20.0) [2]
Hypoglycaemia		1 (10.0) [1]
Respiratory, thoracic and mediastinal disorders	1 (33.3) [3]	3 (30.0) [4]
Acute respiratory failure		2 (20.0) [3]
Respiratory distress	1 (33.3) [2]	1 (10.0) [1]
Respiratory failure	1 (33.3) [1]	

n = Number of subjects; m = Number of events
Program Name: t_sf_ae_sum_01.sas Output Generation: 22MAY2019 17:03

Cut-off Date: 08 March 2019
Table 14.3.1.2: Onset Time and Duration of Treatment-Emergent Adverse Events by System Organ Class and Preferred Term - (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term	Low Dose (N = 3) Number of Events [m]	 Onset Day Median (Min-Max)	 Duration Median (Min-Max)
Any TEAE	5	1477 (1426 - 1529)	12 (1 - 15)
Cardiac disorders	1	1427 (1427 - 1427)	1 (1 - 1)
Cardiac arrest	1	1427 (1427 - 1427)	1 (1 - 1)
Infections and infestations	1	1482 (1482 - 1482)	10 (10 - 10)
Pneumonia	1	1482 (1482 - 1482)	10 (10 - 10)
Respiratory, thoracic and mediastinal disorders	3	1477 (1426 - 1529)	13 (12 - 15)
Respiratory distress	2	1503 (1477 - 1529)	13.5 (12 - 15)
Respiratory failure	1	1426 (1426 - 1426)	13 (13 - 13)

n = Number of subjects, m = Number of events
Program Name: t_sf_ae_sum_06.sas Output Generation: 22MAY2019 17:03

Cut-off Date: 08 March 2019
Table 14.3.1.2: Onset Time and Duration of Treatment-Emergent Adverse Events by System Organ Class and Preferred Term - (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term	Proposed Therapeutic Dose (N = 10) Number of Events [m]	 Onset Day Median (Min-Max)	 Duration Median (Min-Max)
Any TEAE	11	1106 (784 - 1351)	3 (3 - 16)
Infections and infestations	4	1042.5 (845 - 1106)	3 (3 - 6)
Bronchitis	1	1106 (1106 - 1106)	3 (3 - 3)
Gastroenteritis	1	979 (979 - 979)	6 (6 - 6)
Pneumonia	2	975.5 (845 - 1106)	3 (3 - 3)
Metabolism and nutrition disorders	3	1137 (784 - 1166)	3 (3 - 4)
Dehydration	2	960.5 (784 - 1137)	3.5 (3 - 4)
Hypoglycaemia	1	1166 (1166 - 1166)	3 (3 - 3)
Respiratory, thoracic and mediastinal disorders	4	1231 (873 - 1351)	5.5 (3 - 16)
Acute respiratory failure	3	1236 (1226 - 1351)	7 (4 - 16)
Respiratory distress	1	873 (873 - 873)	3 (3 - 3)

n = Number of subjects, m = Number of events
Program Name: t_sf_ae_sum_06.sas Output Generation: 22MAY2019 17:03

Table 14.3.1.3: Related Treatment Emergent Adverse Events (Safety Population)

No Subjects fulfill the criteria.

n = Number of subjects; m = Number of events
Program Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03

Table 14.3.1.4: Serious Treatment Emergent Adverse Events (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term	Low Dose (N = 3) n (%) [m]	Proposed Therapeutic Dose (N = 10) n (%) [m]
Any Serious TEAE	1 (33.3) [5]	5 (50.0) [11]
Cardiac disorders	1 (33.3) [1]	
Cardiac arrest	1 (33.3) [1]	
Infections and infestations	1 (33.3) [1]	3 (30.0) [4]
Bronchitis		1 (10.0) [1]
Gastroenteritis		1 (10.0) [1]
Pneumonia	1 (33.3) [1]	2 (20.0) [2]
Metabolism and nutrition disorders		2 (20.0) [3]
Dehydration		2 (20.0) [2]
Hypoglycaemia		1 (10.0) [1]
Respiratory, thoracic and mediastinal disorders	1 (33.3) [3]	3 (30.0) [4]
Acute respiratory failure		2 (20.0) [3]
Respiratory distress	1 (33.3) [2]	1 (10.0) [1]
Respiratory failure	1 (33.3) [1]	

n = Number of subjects; m = Number of events

Table 14.3.1.5: Serious Related Treatment Emergent Adverse Events (Safety Population)

No Subjects fulfill the criteria.

n = Number of subjects; m = Number of events
Program Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03

Cut-off Date: 08 March 2019
Table 14.3.1.1.8: Treatment-Emergent Adverse Events Leading to Premature Discontinuation from Study by System Organ Class and Preferred Term - (Safety Population)

No Subjects fulfill the criteria.

n = Number of subjects; m = Number of events
Program Name: t_sf_ae_sum_03.sas Output Generation: 22MAY2019 17:03

Cut-off Date: 08 March 2019
Table 14.3.1.9: Treatment-Emergent Adverse Events of Grade 3 or Grade 4 Severity by System Organ Class and Preferred Term - (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term	Low Dose (N = 3) n (%) [m]	Proposed Therapeutic Dose (N = 10) n (%) [m]
Any TEAE with CTCAE Grade 3 or Grade 4	1 (33.3) [5]	5 (50.0) [11]
Cardiac disorders	1 (33.3) [1]	
Cardiac arrest	1 (33.3) [1]	
Infections and infestations	1 (33.3) [1]	3 (30.0) [4]
Bronchitis		1 (10.0) [1]
Gastroenteritis		1 (10.0) [1]
Pneumonia	1 (33.3) [1]	2 (20.0) [2]
Metabolism and nutrition disorders		2 (20.0) [3]
Dehydration		2 (20.0) [2]
Hypoglycaemia		1 (10.0) [1]
Respiratory, thoracic and mediastinal disorders	1 (33.3) [3]	3 (30.0) [4]
Acute respiratory failure		2 (20.0) [3]
Respiratory distress	1 (33.3) [2]	1 (10.0) [1]
Respiratory failure	1 (33.3) [1]	

n = Number of subjects; m = Number of events
Program Name: t_sf_ae_sum_04.sas Output Generation: 22MAY2019 17:03

Table 14.3.2.6: Treatment-Emergent Adverse Events of Special Interest by Standardized MedDRA Query and Preferred Term - (Safety Population)

No Subjects fulfill the criteria.

n = Number of subjects; m = Number of events
Note: TEAEs are classified by adverse events of special interest and preferred term (PT) using Medical Dictionary for Regulatory Activities (MedDRA, version 21.0).
Program Name: t_sf_ae_sum_05.sas Output Generation: 22MAY2019 17:03

Cut-off Date: 08 March 2019

Table 14.3.4.5: Summary of Patients Meeting Potential Hepatotoxicity Criteria Based on Laboratory Data - (Safety Population)

No Subjects fulfill the criteria.

Onset day when an event is identified if the post-baseline laboratory values meet the criteria regardless of the baseline laboratory value. Duration Days is (first corresponding date of not-meeting criteria after meeting the criteria – the first date of the meeting criteria or the baseline day if the baseline values meet the criteria + 1)

Program Name: t_sf_lb_sum_11.sas Output Generation: 20MAY2019 14:13

Cut-off Date: 08 March 2019

Table 14.3.4.6: Summary of Patients Meeting Potential Hematology Criteria Based on Laboratory Data - (Safety Population)

No Subjects fulfill the criteria.

Onset day when an event is identified if the post-baseline laboratory values meet the criteria regardless of the baseline laboratory value. Duration Days is (first corresponding date of not-meeting criteria after meeting the criteria – the first date of the meeting criteria or the baseline day if the baseline values meet the criteria + 1)

Program Name: t_sf_lb_sum_12.sas Output Generation: 20MAY2019 14:13

Pages 17 to 50 of CSR Appendix 16.2 have been removed as per Health Canada Guidance on Public Release of Clinical Information (Patient data listings).