Table 14.1.1: Patient Disposition (All Patients)

¥0 5		
of this	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
Screen Failures Patients Screened Patients in the Enrolled Set Patients in the Safety Analysis Set Patients in the Efficacy Completer Analysis Set Patients Completed the Study Patients Discontinued from the Study Note: Percentages are based on the number of patients enrol: Program Name: t_sf_ds_sum.sas	Durbook and Subject to the Variations thereof	Output Generation: 17MAY2019 21:35 Anonymize

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

arameter	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
estational age at birth (weeks)	n	2	8
To the	Mean (SD)	39.0 (1.41)	38.4 (1.51)
77x 102	Median	39.0	39.0
C ₂ V _C	Min, Max	38, 40	36, 40
wirth Weight (kg)	n	2	9
3 6 6	Mean (SD)	3.2 (0.07)	3.4 (0.77)
δη, ο,	Median	3.2	3.2
	Min, Max	3, 3	2, 5
wirth length/height (cm)	n	1	2
Tx 0, 00	Mean (SD)	48.3 (-)	47.9 (0.57)
© 5, °C	Median	48.3	47.9
	Min, Max	48, 48	48, 48
On the	20		
ead circumference at birth (cm)	Sn	0	1
~ Y	Mean (SD)	0	35.0 (-)
	Median	0	35.0
	Min, Max	0	35, 35
circle weight (kg) dirth Weight (kg) dirth length/height (cm) dirth	there author	ħ.	
	ov ov	OF USE	
		appy has	
		10 0	
ote: cm = centimeters, kg = kilograms. SD = standard deviation. SMA = :	spinal muscular atrophy	7.	
Age = (dose date - date of birth + 1).	Transcarar acroping	10x	12
ercentages are based on number of patients in Safety population for pr	esented cohort.		Try.
rogram Name: t_sf_dm_sum.sas		Output Genera	tion: 17MAY2019 21:35
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			Anony
			,

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
Cz D	Mean (SD)	1366.0 (70.76)	1011.4 (231.95)
The Copy of the Co	Median	1359.0	956.0
Co. Co.	Min, Max	1299, 1440	762, 1388
Age at Baseline (days)*	n	3	10
2, 6, °C,			
24 Months - 59 Months	n (%)	3 (100)	10 (100)
and the second of the second o		_	
Weight at Baseline (kg)	n	3	10
C [*] ,0 , 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)	There is	2	1.0
Deligen/height at baseline (cm)	Mean (SD)	105 0 (9 63)	89 7 (3 70)
O ₂ , ~	Madian	103.0 (9.03)	89 6
L ₂	Min Max	98 116	84 96
Y Committee of the Comm	i dri Cr	50, 110	01, 00
Parameter Age at Baseline (days)* 24 Months - 59 Months Weight at Baseline (kg) Length/height at Baseline (cm) Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA * Age = (dose date - date of birth + 1). Percentages are based on number of patients in Safety population for Program Name: t_sf_dm_sum.sas	= spinal muscular atroph	Output Generat	.ion: 17MAY2019 21:35
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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
Town 1 o	(9.)	2 (66 7)	F (F0 0)
Female Male	n (%)	2 (00.7)	5 (50.0)
AUTC PARTY OF	11 (0)	1 (33.3)	3 (30.0)
Ethnicity	n	3	10
a, 6 Cr			
Hispanic Or Latino	n (%)		1 (10.0)
Not Hispanic Or Latino	n (%)	3 (100)	9 (90.0)
Race	n	3	1.0
C X	2.	3	10
Other	n (%)		1 (10.0)
White	n (%)	3 (100)	9 (90.0)
	thereof the duthorize	No.	
Parameter Gender Female Male Ethnicity Hispanic Or Latino Not Hispanic Or Latino Race Other White Note: cm = centimeters, kg = kilograms, SD = standard deviation, S * Age = (dose date - date of birth + 1). Percentages are based on number of patients in Safety population in Program Name: t_sf_dm_sum.sas	Ma - aninol mugaular otrophy	Cion do at hard	
* Age = (dose date - date of birth + 1).	om – sprnar muscurar acrophy	(O)	L
Percentages are based on number of patients in Safety population i	for presented cohort.		4/2:
Program Name: t_sf_dm_sum.sas		Output Genera	ation: 17MAY2019 21:35
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			Anonyr

Table 14.1.2: Demographics and Baseline Characteristics (Safety Population)

Parameter Familial history of SMA including affected siblings or parent carriers	Statistics	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 3)
Familial history of SMA including affected siblings or parent carriers	n	3	10
Ch the			
Missing	n (%)		1 (10.0)
No Co	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 (33.3)	1 (10.0)
Both Are Carriers	11 (%)		1 (10.0)
Siblings affected by SMA	n	1	3
Ch. The Co			
Missing	n (%)		1 (10.0)
Siblings Affected	n (%)	1 (33.3)	2 (20.0)
Detiant namental hamitalizations since binth	544	2	10
Patient reported hospitalizations since birth) II).	3	10
No	27 12 (25)	1 (33 3)	6 (60.0)
Yes	10(8)	2 (66 7)	4 (40.0)
103	20 1/2 1/2	2 (00.7)	1 (10.0)
Parameter Familial history of SMA including affected siblings or parent carriers Missing No Yes Parental history of SMA Missing Both Are Carriers Siblings affected by SMA Missing Siblings Affected Patient reported hospitalizations since birth No Yes Note: cm = centimeters, kg = kilograms, SD = standard deviation, SMA = sp * Age = (dose date - date of birth + 1). Percentages are based on number of patients in Safety population for presprogram Name: t_sf_dm_sum.sas	inal muscular atrophy	Output General	ation: 17MAY2019 21:35
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Table 14.3.1.1: Treatment Emergent Adverse Events (Safety Population)

MedDRA v21.0 System Organ Class Preferred Term Any TERE Cardiac disorders Cardiac arrest Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration Hypoglycaemia Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory distress Respiratory failure Respiratory failure Respiratory distress Program Name: t_sf_ae_sum_01.sas	1 (33.3) [5] 1 (33.3) [1] 1 (33.3) [1] 1 (33.3) [1]	5 (50.0) [11] 3 (30.0) [4] 1 (10.0) [1] 1 (10.0) [1] 2 (20.0) [2]
Cardiac disorders Cardiac arrest Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration	1 (33.3) [1] 1 (33.3) [1] 1 (33.3) [1] 1 (33.3) [1]	3 (30.0) [4] 1 (10.0) [1] 1 (10.0) [1] 2 (20.0) [2]
Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration	1 (33.3) [1]	3 (30.0) [4] 1 (10.0) [1] 1 (10.0) [1] 2 (20.0) [2]
Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration	1 (33.3) [1]	3 (30.0) [4] 1 (10.0) [1] 1 (10.0) [1] 2 (20.0) [2]
Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration	1 (33.3) [1]	1 (10.0) [1] 1 (10.0) [1] 2 (20.0) [2]
Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration	1 (33.3) [1]	1 (10.0) [1] 2 (20.0) [2]
Metabolism and nutrition disorders Dehydration	1 (33.3) [1]	2 (20.0) [2]
Metabolism and nutrition disorders Dehydration		
Dehydration Dehydration		2 (20 0) [3]
=2		2 (20.0) [2]
Hypoqlycaemia		1 (10.0) [1]
Op the aller		, , <u>.</u> .
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]	
Thereof of	String OF USC AF NAP	
<pre>n = Number of subjects; m = Number of events Program Name: t_sf_ae_sum_01.sas</pre>	Output Gen	eration: 22MAY2019 17:03 Anonymi

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Table 14.3.1.2: Onset Time and Duration of Treatment-Emergent Adverse Events by System Organ Class and Preferred Term - (Safety Population)

To dix.		Low Dose (N = 3)	
MedDRA v21.0 System Organ Class Preferred Term	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)
MedDRA v21.0 System Organ Class Preferred Term Any TEAE Cardiac disorders Cardiac arrest Infections and infestations Pneumonia Respiratory, thoracic and mediastinal disorders Respiratory failure n = Number of subjects, m = Number of events Program Name: t_sf_ae_sum_06.sas	Tarkotino the thorong thereof.	Output General	ration: 22MAY2019 17:03

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Table 14.3.1.2: Onset Time and Duration of Treatment-Emergent Adverse Events by System Organ Class and Preferred Term - (Safety Population)

The strain of th	Proposed Therapeutic Dose (N = 10)		
MedDRA v21.0 System Organ Class Preferred Term	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)
MedDRA v21.0 System Organ Class Preferred Term Any TEAE Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration Hypoglycaemia Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory distress n = Number of subjects, m = Number of events Program Name: t_sf_ae_sum_06.sas	thereof of	Ciths of Use at hard	ŽO.
<pre>n = Number of subjects, m = Number of events Program Name: t_sf_ae_sum_06.sas</pre>		Output Gene	ration: 22MAY2019 17:03 Anonymize

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n = Number of subjects; m = Number of events Program Name: t_sf_ae_sum_02.sas

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

Amy Serious TEAE 1 (33.3) [5] 5 (50.0) [11] Cardiac disorders Cardiac arrest 1 (33.3) [1] Infections and infestations Bronchitis Fronchitis 1 (33.3) [1] 3 (30.0) (4) Exprehence of the control	MedDRA v21.0 System Organ Class Preferred Term	Low Dose (N = 3)	Proposed Therapeutic Dose (N = 10) n (%) [m]
Cardiac disorders Cardiac arrest 1 (33.3) [1] Infections and infeatations 1 (33.3) [1] 3 (30.0) [4] Bronchitis Cantreonteritis 1 (10.0) [1] Gastroenteritis 1 (33.3) [1] 2 (20.0) [2] Metabolism and nutrition disorders 2 (20.0) [3] 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) [3] 3 (30.0) [4] Acute respiratory failure 2 (20.0) [3] Respiratory failure 1 (33.3) [1] Anonymetabolism and nutrition disorders Cutput Generation: 22MAY2019 17:03 Anonymetabolism and nutrition disorders Cutput Generation: 22MAY2019 17:03 Anonymetabolism and nutrition disorders Anonymetabolism and nutrition disorders Cutput Generation: 22MAY2019 17:03 Anonymetabolism and nutrition disorders Cutput Generation: 22MAY2019 17:03	Any Serious TEAE	1 (33.3) [5]	5 (50.0) [11]
Cardiac disorders Cardiac arrest Car	This serious This	1 (33.3, [3]	3 (30.0 / [11]
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] Wetabolism and nutrition disorders 2 (20.0) [3] Dehydration 2 (20.0) [2] Wetabolism and mediastinal disorders 1 (10.0) [1] Respiratory, thoracic and mediastinal disorders 2 (20.0) [3] Respiratory failure 2 (20.0) [3] Respiratory failure 2 (20.0) [3] Respiratory failure 1 (33.3) [3] 3 (30.0) [4] Respiratory failure 2 (20.0) [3] Respiratory failure 1 (33.3) [1] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3] Anonymetr f subjects; m = Number of events 2 (20.0) [3]	Cardiac disorders	1 (33.3) [1]	
Infections and infestations Bronchitis Gastroenteritis I (10.0) [1] Gastroenteritis I (10.0) [1] Pneumonia Recabolism and nutrition disorders Dehydration Eyosalycaemia Acute respiratory failure Respiratory distress Respiratory failure Pay 1 (33.3) [2] 1 (10.0) [1] Respiratory failure Power of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAWY2019 17:03	Cardiac arrest	1 (33.3) [1]	
Threations and infestations 1 (33,3) [1] 3 (30.0) [4]	and the contraction of the contr		
Respiratory failure	Infections and infestations	1 (33.3) [1]	3 (30.0) [4]
Gastroenteritis Pneumonia 1 (33.3) [1] 2 (20.0) [2] Wetabolism and nutrition disorders 2 (20.0) [3] Behydration Hypoglycaemia 2 (20.0) [2] Hypoglycaemia 2 (20.0) [2] Hypoglycaemia 2 (20.0) [2] Hypoglycaemia 3 (30.0) [4] Respiratory failure Respiratory failure 1 (33.3) [3] 3 (30.0) [4] 2 (20.0) [3] Respiratory distress Respiratory distress Respiratory failure 1 (33.3) [1] **Number of subjects: n = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03	Bronchitis		1 (10.0) [1]
Pneumonia 1 (33.3) (1) 2 (20.0) (2) Metabolism and nutrition disorders Phypoglycaemia 2 (20.0) (3) 2 (20.0) (1) Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory failure Respiratory failure Respiratory failure Respiratory failure Pneumonia 1 (33.3) (3) 3 (30.0) (4) 2 (20.0) (3) 3 (30.0) (4) 4 (33.3) (2) 1 (10.0) (1) Respiratory failure Pneumonia 1 (33.3) (3) 3 (30.0) (4) 2 (20.0) (3) 3 (30.0) (4) 4 (33.3) (1) Respiratory failure Pneumonia 1 (33.3) (3) 3 (30.0) (4) 2 (20.0) (3) 3 (30.0) (4) 4 (33.3) (1) Respiratory failure Pneumonia 1 (33.3) (3) 3 (30.0) (4) 2 (20.0) (3) 3 (30.0) (4) 4 (33.3) (1) Respiratory failure Pneumonia Pneumonia	Gastroenteritis		1 (10.0) [1]
Detabolism and nutrition disorders Debydration Bypoglycaemia 2 (20.0) [3] Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory distress Respiratory failure 1 (33.3) [3] 3 (30.0) [4] 2 (20.0) [3] Respiratory distress 1 (33.3) [2] 1 (10.0) [1] Respiratory failure Pumber of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03	Pneumonia	1 (33.3) [1]	2 (20.0) [2]
### Metabolism and nutrition disorders Dehydration 2 (20.0) [3] 2 (20.0) [2] Mypoglycaemia 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 failure 1 (33.3) [2] 1 (10.0) [1] Respiratory failure 1 (33.3) [1] Another respiratory failure 1 (33.3) [2] 1 (10.0) [1] Respiratory failure 1 (33.3) [1] Another respiratory failure 1 (33.3) [2] 1 (10.0) [1] Another respiratory failure 1 (33.3) [2] 1 (10.0) [1] Another respiratory failure 1 (33.3) [2] 1 (10.0) [1] Another respiratory failure 2 (20.0) [3] Another respiratory failure 3 (30.0) [4] Another respiratory failure 4 (30.0) [4] Another respiratory failure 4 (30.0) [4] Another r			
Dehydration 2 (20.0) [2] 1 (10.0) [1] Respiratory, thoracic and mediastinal disorders 1 (33.3) [3] 3 (30.0) [4] 2 (20.0) [3] Respiratory distress 1 (33.3) [1] 1 (10.0) [1] Respiratory failure 1 (33.3) [1] 1 (10.0) [1] 1 1 (33.3) [1] 1 (10.0) [1] 1 1 (33.3) [1] 1 (33.3) [1] 1	Metabolism and nutrition disorders		2 (20.0) [3]
Hypoglycaemia	Dehydration		2 (20.0) [2]
Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory distress Respiratory failure 1 (33.3) [2] 1 (10.0) [1] 1 (33.3) [2] 1 (10.0) [1] 1 (33.3) [1] - Number of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas Cutput Generation: 22MAY2019 17:03	Hypoglycaemia		1 (10.0) [1]
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure	Respiratory, thoracic and mediastinal disorders	1 (33.3) [3]	3 (30.0) [4]
Respiratory distress Respiratory failure 1 (33.3) [2] 1 (10.0) [1] 1 (33.3) [1] 1 (33.3) [2] 1 (10.0) [1] 1 (33.3) [1] - Number of subjects: m = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03 Anonym	Acute respiratory failure	\$2.	2 (20.0) [3]
= Number of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas 1 (33.3) [1] Output Generation: 22MAY2019 17:03	Respiratory distress	1 (33.3) [2]	1 (10.0) [1]
= Number of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03	Respiratory failure	1 (33.3) [1]	
= Number of subjects; m = Number of events rogram Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03		VZ Z	
Number of subjects; m = Number of events Program Name: t_sf_ae_sum_02.sas Output Generation: 22MAY2019 17:03 Anonym		thereof authorization applies	Ada
Anonym	n = Number of subjects; m = Number of events Program Name: t_sf_ae_sum_02.sas	Outpu	t Generation: 22MAY2019 17:03
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n = Number of subjects; m = Number of events Program Name: t_sf_ae_sum_02.sas

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Table 14.3.1.8: Treatment Emergent Adverse Events Leading to Premature Discontinuation from Study by System Organ Class and Preferred Term - (Safety Population)

n = Number of subjects; m = Number of events Program Name: t_sf_ae_sum_03.sas

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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 Any TEAE with CTCAE Grade 3 or Géade 40. Cardiac disorders Cardiac arrest Infections and infestations Bronchitis Gastroenteritis Pneumonia Metabolism and nutrition disorders Dehydration Hypoglycaemia Respiratory, thoracic and mediastinal disorders Acute respiratory failure Respiratory distress Respiratory failure **Respiratory failure** **Respiratory failure** **Infections and infestations Bronchitis Gastroenteritis Dehydration Hypoglycaemia **Respiratory thoracic and mediastinal disorders Acute respiratory distress Respiratory distress Respiratory failure **Infections and infestations **Infections and infestations Bronchitis Gastroenteritis Program Name: t_sf_ae_sum_04.sas	Low Dose	Proposed Therapeutic Dose
System Organ Class	(N = 3)	(N = 10)
Preferred Term	n (%) [m]	n (%) [m]
Any TEAE with CTCAE Grade 3 or Grade 4	1 (33.3) [5]	5 (50.0) [11]
	1 / 22 2) [1]	
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]
Filediionia Cy D.	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]	
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	7	2
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	10	1. P.
	*	(C) (4)
		95: 'Qo.
		102 F3
n = Number of subjects; m = Number of events		47.
Program Name: t_si_ae_sum_04.sas	Out	tput Generation: 22MAY2019 17:03
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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.

Regulatory Activities (MedDRA, version 21.0).

Program Name: t_sf_ae_sum_05.sas

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Output Generation: 22MAY2019 17:03

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

Table 14.3.4.5: Summary of Patients Meeting Potential Hepatotoxicity Criteria Based on Laboratory Data - (Safety Popula Nonest 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 this criteria - the first date of the meeting criteria or the baseline values meet the criteria after meeting this criteria - the first date of the meeting criteria or the baseline values meet the criteria - 1) brownen Name: t sf_lb_sum_ll.sas Output Generation: e criteria + 1)
Output Generation: 20MAY2019 14:13 Program Name: t_sf_lb_sum_11.sas

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

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

No Subjects fulfill the criteria.

No Subjects fulfill the criteria.

Onset day when an event is identified if the post-baseline laboratory values meet the criteria regardless of the Daseline laboratory value, Duration Days is (first corresponding date of not-meeting criteria after meeting this criteria - the first date of the meeting criteria or the baseline day if the baseline values meet the criteria - 1) Drownan Name: t sf_lb_sum_12.sas

Output Generation: e criteria + 1)
Output Generation: 20MAY2019 14:13 Program Name: t_sf_lb_sum_12.sas

ges 17 to 50 of cen removed as per in Public Release of C. (Patient data listings). Pages 17 to 50 of CSR Appendix 16.2 have been removed as per Health Canada Guidance