Protocol AVXS-101-CL-3030

Database Lock Date: 12 Dec 2019

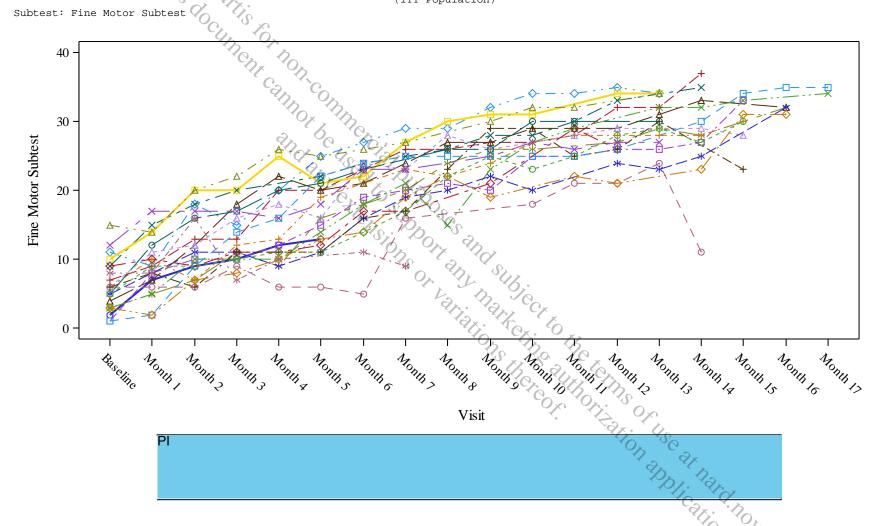
Final Run

Figure 14.2.4.2.2

Spaghetti Plot of Bayley Scale (Monthly) By Visit

(ITT Population)

(ITT Population)



Program Name: f_ef_qs_02.sas Output Generation: 06JAN2020 10:41

ges 2 to smoved as , Public Release data listings).

And an order of the property of the control Pages 2 to 35 of CSR Appendix 16.2 have been removed as per Health Canada Guidance on

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> Table 14.2.3.2-1 $\mathcal{O}_{ extstyle extstyl$ ITT Population

Visit Statistics Actual Charge from Reseline Actual Charge from Reseline 1 22	C, C		1.1E	1.1E14 vg/kg	
Mean (SD) (A.2 (3.65) (A.2 (3.	The corp.		(N	= 22)	
Mean (SD) (A.2 (3.65) (A.2 (3.	Visit	tatistics		Change from Baseline	
Mean (SD) (A.2 (3.65) (A.2 (3.	Baseline	2.			
Post-baseline value for the visit with maximum CFB Observed value Mean SD	M	ean (SD)			
Post-baseline value for the visit with maximum CFB Observed value Mean (SD)					
	M	in, Max	1, 15		
	Post-baseline value for the visit with maximum CFB Observed value	1 de la	22	22	
	М	ean (SD)	30.1 (5.90)	23.9 (6.60)	
	M	edian	31.5	24.5	
	М	in, Max	13, 37	8, 34	
An any main and Dama 2	CFB = Change From Baseline Program Name: t_ef_qs_bly_chg_01.sas	On On Nariana Inana	thereof the lettins of use at hat on application output Ger	neration: 30DEC2019 10:51	
Ananymizar Para 3				Anonymized Page 3	

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> Table 14.2.3.2-2 Change from Baseline to the Maximum in the Bayley Raw Gross Motor Scales ITT Population

## 1.5 (1.87) Mean (SD)	1.1E14 vg/kg		1		C. S.	
Mean (SD) 1.5 (1.87) Median 1.0	(N = 22)				The Contract of the Contract o	
Mean (SD) 1.5 (1.87) Median 1.0	rom Baseline	Change		Statistics	Visit	
Mean (SD) 1.5 (1.87) Median 1.0				10	Baseline	
		7)		Mean (SD)		
Post-baseline value for the visit with maximum CFB Observed value Mean SD 17.5 (9.84) 16.0 (9.84) 18.5 17.						
Post-baseline value for the visit with maximum CFB Observed value Mean SD			0, 7	Min, Max		
Median (SD) (17.5 (9.84) 16.0 (9 18.5 17. Min, Max (17.5 (9.84)) 17.5 (9.84) 16.0 (9 18.5 17. Min, Max (17.5 (9.84)) 17.5 (9.84) 18.5 17. Min, Max (17.5 (9.84)) 18.5 17. Min, Max (17.5 (9.84	22		22	alay Cliscial		
Median Min, Max On 18.5 17. 0, 46 0, 4	0 (9.19)	34) 1	17.5 (9.84)	Mean (SD)		
CFB = Change From Baseline Program Name: t_ef_qs_bly_chg_01.sas O, 46 O, 46 O, 46 O, 46 O, 46 Output Generation: 30DEC	17.0		18.5	Median		
CFB = Change From Baseline Program Name: t_ef_qs_bly_chg_01.sas Output Generation: 30DEC	0, 42		0, 46	Min, Max		
	ODEC2019 10:51	put Generation:	Ottication application output	Ons or variable of to the original thereof.		
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Page 1 of 1

Final Run

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> Table 14.3.4.14.1 Patients Reported Non-invasive Ventilatory Support after Dosing ITT Population

Number of Patients Independent of NIV Ventilatory Support [1] Number of Patients opted for NIV Ventilatory Support [2] Through CRF Collect through SD card Number of Patients in Permanent Ventilation	AVXS-101 (N = 22) n (%) 15(68.2) 7(31.8) 3(13.6) 5(22.7)
Number of Patients opted for NIV Ventilatory Support [2] Through CRF	n (%) 15(68.2) 7(31.8) 3(13.6)
Number of Patients opted for NIV Ventilatory Support [2] Through CRF	15(68.2) 7(31.8) 3(13.6)
umber of Patients opted for NIV Ventilatory Support [2] Through CRF	7(31.8) 3(13.6)
Through CRF	3(13.6)
Grillant through GD and	5(00.7)
Collect through SD card Tumber of Patients in Permanent Ventilation	5(22.7)
number of Patients in Permanent Ventilation	1/4 [)
and a use cial	1(4.5)
Catensi Apport	never opted for NIV ventilation after the treatment start date. in both ways and some patients only reported through one of two

Output Generation: 07JAN2020 07:57 Program Name: t_sf_vt_sum_02.sas

^[2] Due to the way of data collection, some patients reported data in both ways and some patients only reported through one of two ways.

Pages 39 to 767 of CSR Appendix 16.2 have been removed as per Health Canada **Guidance on Public Release of Clinical** Anton to the list of the letter of the fellow and subject to the fello PMA.

OCUMPOR FOR TROPIC COMPRISE OF THE STANDARD STANDAR Information (Patient data listings).