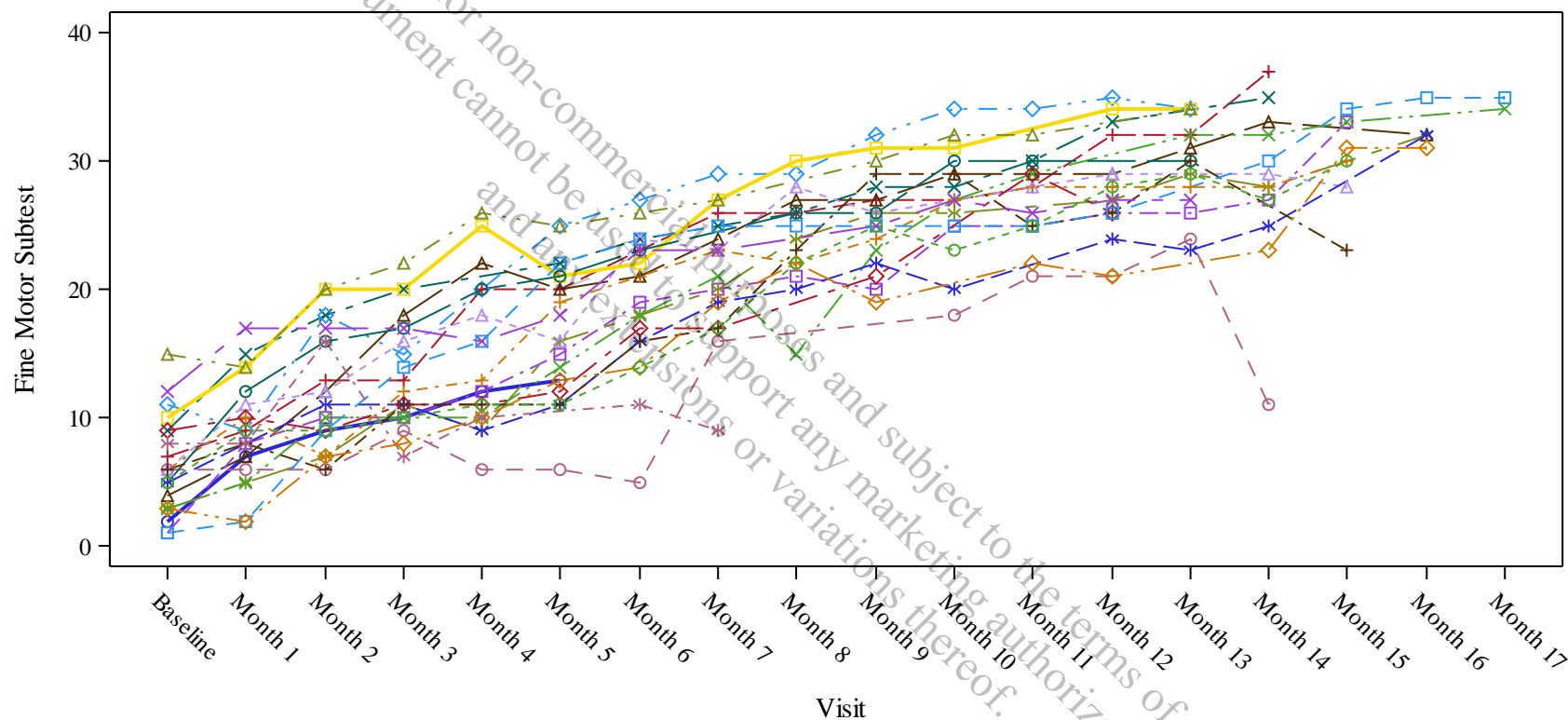


Figure 14.2.4.2.2
Spaghetti Plot of Bayley Scale (Monthly) By Visit
(ITT Population)

Subtest: Fine Motor Subtest



PI

Pages 2 to 35 of CSR Appendix 16.2 have been removed as per Health Canada Guidance on Public Release of Clinical Information (Patient data listings).

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Table 14.2.3.2-1
Change from Baseline to the Maximum in the Bayley Raw Fine Motor Scales
ITT Population

Visit	Statistics	1.1E14 vg/kg (N = 22)	
		Actual	Change from Baseline
Baseline	n	22	
	Mean (SD)	6.2 (3.65)	
	Median	6.0	
	Min, Max	1, 15	
Post-baseline value for the visit with maximum CFB Observed value	n	22	22
	Mean (SD)	30.1 (5.90)	23.9 (6.60)
	Median	31.5	24.5
	Min, Max	13, 37	8, 34

CFB = Change From Baseline
Program Name: t_ef_qs_bly_chg_01.sas

Output Generation: 30DEC2019 10:51

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

Visit	Statistics	1.1E14 vg/kg (N = 22)	
		Actual	Change from Baseline
Baseline	n	22	
	Mean (SD)	1.5 (1.87)	
	Median	1.0	
	Min, Max	0, 7	
Post-baseline value for the visit with maximum CFB Observed value	n	22	22
	Mean (SD)	17.5 (9.84)	16.0 (9.19)
	Median	18.5	17.0
	Min, Max	0, 46	0, 42

CFB = Change From Baseline
Program Name: t_ef_qs_bly_chg_01.sas

Output Generation: 30DEC2019 10:51

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

	AVXS-101 (N = 22) n (%)
Number of Patients Independent of NIV Ventilatory Support [1]	15(68.2)
Number of Patients opted for NIV Ventilatory Support [2]	7(31.8)
Through CRF	3(13.6)
Collect through SD card	5(22.7)
Number of Patients in Permanent Ventilation	1(4.5)

[1] Independence of ventilation support is defined as subjects who never opted for NIV ventilation after the treatment start date.
[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.

Program Name: t_sf_vt_sum_02.sas

Output Generation: 07JAN2020 07:57

**Pages 39 to 767 of CSR Appendix 16.2
have been removed as per Health Canada
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