

**Marfan Data Codebook**  
**Form A122: Study Drug Uptitration Cycles – Study Drugs A and B**  
**(raw data)**

**DATE CREATED 13SEP18**

**Number of Observations: 1819**

**Number of Variables: 37**

**Organization of file: Up to Six Records per Patient**

Variable Label (VAR)	Variable Name	Category	N(%)	N(Missing)	Mean(SD)	Median(Q1,Q3)	Range of Values
A1. Random identification number	RANDOM_ID			1819(0)			144508.00-145870.00
A2. Study drug assigned	STUDYDRUG122	Study Drug A	931(51.2)				
		Study Drug B	888(48.8)				
A3a. Type of cycle: Study drug A	CYCLEA122	End of cycle 1b	313(17.2)				
		End of cycle 2	258(14.2)				
		End of cycle 3	204(11.2)				
		End of cycle 4	156(8.6)				
		Skipped	888(48.8)				
A3b. Type of cycle: Study drug B	CYCLEB122	End of cycle 1	307(16.9)				
		End of cycle 2	300(16.5)				
		End of cycle 3	281(15.4)				
		Skipped	931(51.2)				
A3c. Repeated cycle?	REPEAT_CYC	NO	1737(95.5)				
		YES	82(4.5)				

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A4. Prescription number	RX_NUM122	1	329(18.1)				
		2	542(29.8)				
		3	470(25.8)				
		4	237(13.0)				
		5	158(8.7)				
		6	48(2.6)				
		7	18(1.0)				
		8	13(0.7)				
		9	2(0.1)				
		10	2(0.1)				
B1. Age at check-up call	CALL122_AGE			1819(0)	10.98(6.20)	10.40(5.90,15.40)	0.60-26.30
B2. Study drug intolerances?	INTOL122	NO	1720(94.6)				
		YES	99(5.4)				
B2a. Intolerances: Dizziness?	DIZZ122	NO	79(4.3)				
		SKIPPED	1720(94.6)				

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		YES	20(1.1)				
B2b. Intolerances: Fatigue?	FATIGUE122	NO	63(3.5)				
		SKIPPED	1720(94.6)				
		YES	36(2.0)				
B2c. Intolerances: Syncope?	SYNCOPE122	NO	96(5.3)				
		SKIPPED	1720(94.6)				
		YES	3(0.2)				
B2d. Intolerances: Shortness of breath?	SOB122	NO	85(4.7)				
		SKIPPED	1720(94.6)				
		YES	14(0.8)				
B2e. Intolerances: Other symptoms	OTHSX122	NO	57(3.1)				
		SKIPPED	1720(94.6)				
		YES	42(2.3)				
B2f. Intolerances: Other reason?	OTHS122	NO	80(4.4)				

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		SKIPPED	1720(94.6)				
		YES	19(1.0)				
B3. Subject < 18 months?	AGE18MO	NO	1763(96.9)				
		YES	56(3.1)				
B3a. Systolic blood pressure	SBP_122			44(1775)	93.84(14.61)	93.50(80.00,102.50)	72.00-134.00
B3b. Diastolic blood pressure	DBP_122			39(1780)	56.95(9.69)	56.00(50.00,62.00)	38.00-84.00
B3c. Systolic blood pressure <2%tile for age?	SBP_PCT122	<i>Missing</i>	10(0.5)				
		NO	46(2.5)				
		SKIPPED	1763(96.9)				
B4. Safety labs obtained?	LABS_122	NO	759(41.7)				
		SKIPPED	1041(57.2)				
		YES	19(1.0)				
B4a. Age at safety labs	LABAGE_122			19(1800)	1.07(0.31)	1.10(0.80,1.40)	0.60-1.50
B4b. Safety labs within normal limits for age?	LABSNORM_122	Abnormal - below threshold for automatically decreasein	1(0.1)				

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		All values normal	18(1.0)				
		Skipped	1800(99.0)				
C0. Was an ECG done?	ECG_DONE	<i>Missing</i>	764(42.0)				
		NO	59(3.2)				
		YES	996(54.8)				
C1. Age at ECG	ECG_AGE122			1752(67)	10.97(6.18)	10.40(6.00,15.40)	0.60-26.40
C2. 24 hour average heart rate	AVG_HR122			1748(71)	82.85(14.71)	81.00(72.00,92.00)	48.00-150.00
C2a. 24 hour average heart rate, above threshold?	THRESHOLD122			1819(0)	0.84(0.96)	1.00(1.00,1.00)	-9.00-2.00
C2b. Current heart rate below target?	CUR_HR122	<i>Missing</i>	1(0.1)				
		NO	339(18.6)				
		SKIPPED	1426(78.4)				
		YES	53(2.9)				
D1. Outcome of assessment	OUTCOME122	Adjust dose	54(3.0)				
		Continue uptitration	1176(64.7)				

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Variable Label (VAR)	Variable Name	Category	N(%)	N(Missing)	Mean(SD)	Median(Q1,Q3)	Range of Values
		End uptitration	589(32.4)				
E1. Age at uptitration prescription written	UPDOSE122_AGE			1176(643)	10.70(6.15)	9.90(5.80,15.10)	0.60-26.30
E2. Uptitration: Dose prescribed	UP_RX122			1173(646)	1.86(1.01)	1.40(0.90,2.80)	0.40-4.10
E3. Uptitration: Frequency of administration	UP_FREQ122	Daily	1049(57.7)				
		Skipped	643(35.3)				
		Twice a day	127(7.0)				
E4. Uptitration: Daily dose	UP_DAY122			1819(0)	44.24(54.09)	25.00(-2.00,75.00)	-2.00-250.00
F1. Age at adjustment prescription written	ADJUST122_AGE			54(1765)	12.11(5.98)	12.15(6.50,16.40)	2.00-24.60
F2. Adjustment: Action taken	ACTION122	Changed to BID administration	5(0.3)				
		Decreased current dose	15(0.8)				
		Maintained current dose	28(1.5)				
		Other	4(0.2)				
		Permanently discontinued study drug	2(0.1)				
		Skipped	1765(97.0)				

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F3. Adjustment: Dose prescribed	ADJ_RX122			52(1767)	1.46(0.86)	1.10(0.90,2.00)	0.00-3.50
F4. Adjustment: Daily dose	ADJ_DAY122			52(1767)	59.26(34.68)	56.25(37.50,75.00)	0.00-150.00
G1. Age at maintenance dose reached	MAINT_AGE			589(1230)	11.39(6.27)	11.00(6.30,15.60)	0.60-26.40
Form Version	VER_ID	A		94(5.2)			
		B		670(36.8)			
		C		99(5.4)			
		D		956(52.6)			