Supplementary Material 10

Results for Trial Dose Confirmatory Stage

Table S39: Grade 3 and higher adverse event (AE) information per patient in the dose-confirmation cohort.

Patient Number	Withdrawal Date	AE Category	AE Toxicity	AE Grade	AE Start Date	AE Stop Date	AE Ongoing?	DLT	Serious AE	Treatment Relatedness
1		Investigations	GGT increased	3	2015-07-14	2015-09-18	No	No	No	1-Unrelated
1		Respiratory, thoracic and mediastinal disorders	Cough	3	2015-11-05	2015-11-16	No	No	No	1-Unrelated
1		General disorders and administration site conditions	Pain	3	2015-12-17	2015-12-24	No	No	No	1-Unrelated
1		Investigations	GGT increased	3	2015-12-18		Yes	No	No	1-Unrelated
2	2015-12-10	General disorders and administration site conditions	Infusion related reaction	3	2015-12-09	2015-12-09	No	No	No	5-Definitely related
2	2015-12-10	Investigations	Alkaline phosphatase increased	3	2015-10-13		Yes	No	No	1-Unrelated
2	2015-12-10	Investigations	GGT increased	4	2015-10-13		Yes	No	No	1-Unrelated
3		Investigations	GGT increased	3	2015-11-18		Yes	No	No	1-Unrelated
4		Investigations	GGT increased	3	2016-01-07		Yes	No	No	1-Unrelated
4		Investigations	Alkaline phosphatase increased	3	2015-11-18	2016-01-18	No		No	1-Unrelated
4		Investigations	Alkaline phosphatase increased	3	2016-04-25	2016-05-18	No		No	1-Unrelated
5		Investigations	GGT increased	3	2015-12-16		Yes	No	No	1-Unrelated
5		Investigations	Alkaline phosphatase increased	3	2015-12-16		Yes	No	No	1-Unrelated
5		General disorders and administration site conditions	Infusion related reaction	3	2016-02-11	2016-02-11	No	No	Yes	5-Definitely related
5		Cardiac disorders	Other: Hypotension	3	2016-02-11	2016-02-11	No		No	1-Unrelated
6		Investigations	GGT increased	3	2016-04-11		Yes	No	No	1-Unrelated
7		Investigations	GGT increased	3	2016-05-11	2016-08-17	No	No	No	1-Unrelated
7		Investigations	GGT increased	3	2016-09-21	2016-11-16	No		No	1-Unrelated

Table S40: Descriptive statistics of circulating BTT1023 (μg/mL) across all treatment visits in the dose confirmatory population.

	Circulating BTT1023 (μg/mL)								
	Visit 3	**Visit 4**	**Visit 5**	**Visit 6**	**Visit 7**	**Visit 8**	**Visit 9**	**Visit 10**	**Visit 11**
N	6	6	6	6	6	6	6	6	6
Mean	0	7.12	4.07	8.36	13.83	24.85	22.65	6.7	0
Median	0	7.54	3.06	6.89	12.9	19.75	22.4	2.66	0
Range	(0,0)	(1.87,14.8)	(0,10.5)	(3.27,16.5)	(8.47,23.6)	(12.6,46.9)	(10.8,37.3)	(0.2,21)	(0,0)
IQR	(0,0)	(3.4,8.57)	(1.65,5.71)	(6.04,9.85)	(10.98,14.3)	(18.65,28.8)	(13.25,30.2)	(1.62,9.95)	(0,0)
ALQ (n)	1	0	0	0	0	0	0	0	0

Note:

ALQ(n) indicates the number of patients whose circulating BTT1023 levels were above the level of quantification.

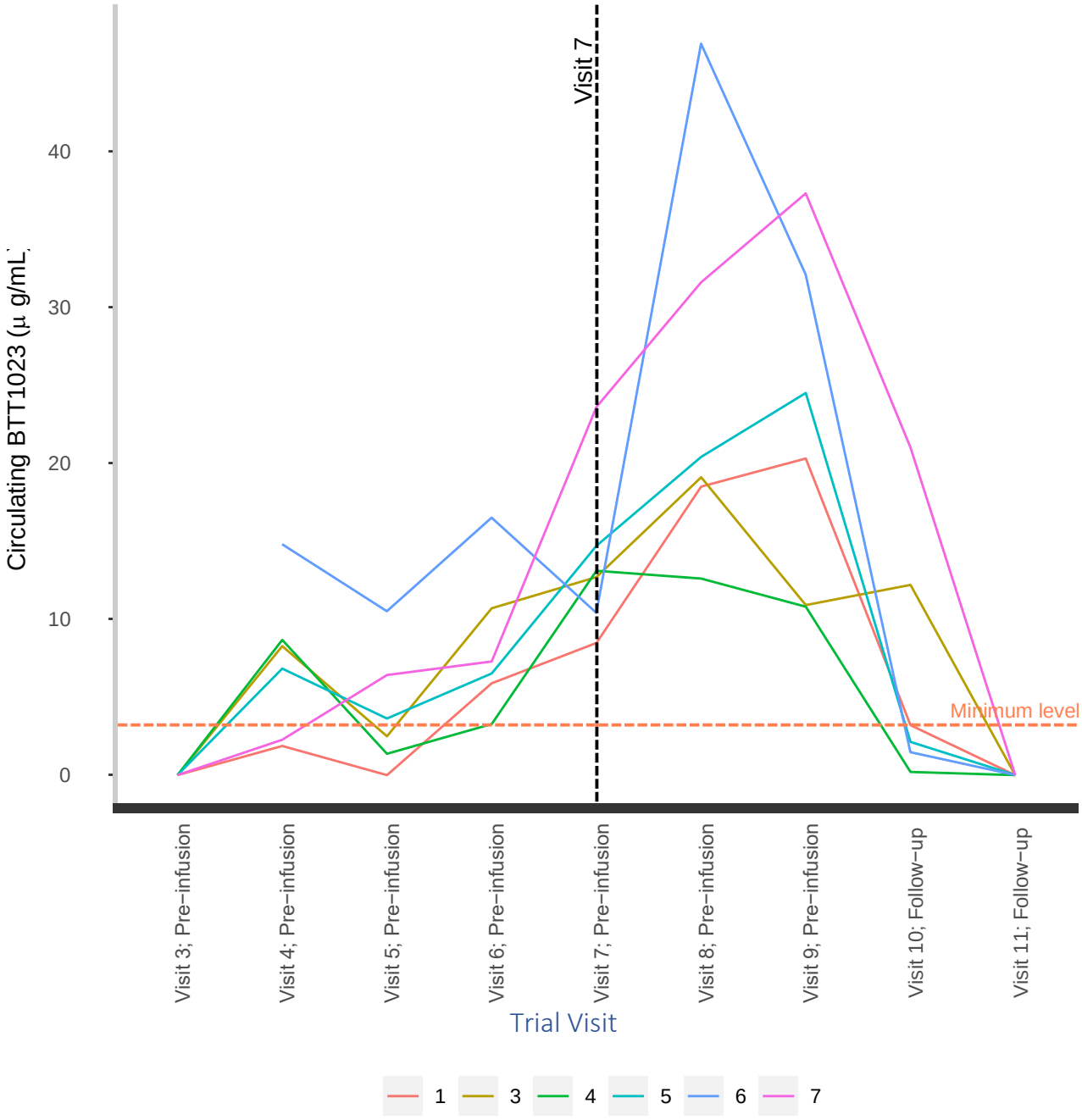

Figure S5: Repeated measures plot of circulating BTT1023 levels in the dose confirmatory population.