

# Appendix of Tables to Accompany “A Phase I/II Trial Design when Response is Unobserved in Subjects with Dose-Limiting Toxicity” by T.M. Braun

Table 1: Operating characteristics of the CRM in Setting 1 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	0.0	15.3	66.0	18.6	0.0	11.6	11.1	7.3
	B	0.1	20.9	54.8	24.3	0.0	11.8	9.1	9.1
	C	0.0	18.8	62.2	18.9	0.0	12.3	11.0	6.7
	D	0.0	20.0	59.3	20.7	0.0	12.0	10.5	7.5
	Mean				60.6				10.4
	SD				4.7				0.9
<i>Exp</i> (1)	A	0.0	14.6	68.1	17.3	0.0	11.1	12.3	6.5
	B	0.1	22.8	50.3	26.8	0.0	11.4	8.4	10.2
	C	0.0	22.8	71.9	5.2	0.0	15.8	13.3	0.8
	D	0.0	22.6	62.8	14.6	0.0	15.7	11.2	3.1
	Mean				63.3				11.3
	SD				9.4				2.1
$\mathcal{N}(0, 4)$	A	0.0	16.7	66.2	17.0	0.0	12.1	11.4	6.5
	B	0.1	24.4	48.4	27.0	0.0	12.5	8.2	9.3
	C	0.1	18.3	68.2	13.4	0.0	12.9	12.2	4.9
	D	2.1	20.3	56.6	21.0	0.6	12.4	10.1	6.9
	Mean				59.9				10.5
	SD				9.2				1.8
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.0	16.1	69.4	14.5	0.0	12.1	12.6	5.3
	B	0.0	21.9	52.1	25.9	0.0	11.4	9.3	9.4
	C	0.0	22.5	69.5	8.0	0.0	15.0	12.8	2.3
	D	0.0	24.5	58.5	16.9	0.0	15.1	10.1	4.8
	Mean				62.4				11.2
	SD				8.6				1.8

Table 2: Operating characteristics of the CRM in Setting 2 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	0.0	25.6	44.6	29.8	0.0	12.6	8.6	8.8
	B	0.0	29.7	35.3	34.9	0.0	12.8	6.5	10.8
	C	0.1	28.8	44.4	26.8	0.0	13.5	8.5	8.0
	D	0.0	29.6	39.9	30.5	0.0	13.5	7.5	9.0
	Mean				41.0				7.7
	SD				4.4				1.0
$Exp(1)$	A	0.0	23.6	45.7	30.7	0.0	11.6	9.2	9.2
	B	0.0	29.1	35.8	35.2	0.0	11.8	6.7	11.4
	C	0.1	34.9	49.9	15.0	0.0	16.1	10.1	3.8
	D	0.0	40.2	38.9	21.0	0.0	18.2	7.2	4.6
	Mean				42.6				8.3
	SD				6.4				1.6
$\mathcal{N}(0, 4)$	A	0.0	27.6	44.5	27.8	0.0	13.2	8.6	8.2
	B	0.0	32.0	35.6	32.4	0.0	13.4	6.5	10.1
	C	0.0	30.9	43.9	25.2	0.0	14.6	8.4	7.0
	D	2.1	33.7	37.4	26.8	0.6	14.7	6.8	7.9
	Mean				40.4				7.6
	SD				4.5				1.1
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.0	25.4	47.9	26.6	0.0	12.4	9.9	7.7
	B	0.0	29.7	36.5	33.9	0.0	11.6	7.4	11.0
	C	0.0	37.6	44.9	17.5	0.0	16.7	8.6	4.7
	D	0.0	40.8	37.6	21.5	0.0	17.7	6.9	5.4
	Mean				41.7				8.2
	SD				5.6				1.3

Table 3: Operating characteristics of the CRM in Setting 3 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	0.2	22.6	61.9	15.2	0.0	10.0	12.1	7.8
	B	0.8	20.5	63.7	14.9	0.2	9.8	12.7	7.3
	C	0.6	19.1	66.4	13.9	0.2	9.2	13.6	7.0
	D	0.1	21.2	65.2	13.6	0.0	9.4	13.6	6.9
	Mean				64.3				13.0
	SD				1.9				0.7
$Exp(1)$	A	0.3	27.5	52.6	19.6	0.1	9.7	10.2	10.1
	B	0.5	23.3	60.8	15.3	0.1	8.8	12.7	8.4
	C	0.4	18.6	67.5	13.5	0.1	8.7	14.3	6.9
	D	0.4	19.6	67.0	12.9	0.1	9.3	14.5	6.1
	Mean				62.0				12.9
	SD				7.0				2.0
$\mathcal{N}(0, 4)$	A	0.5	29.0	52.3	18.2	0.1	11.5	10.0	8.3
	B	0.5	25.6	60.7	13.2	0.1	10.8	12.2	6.8
	C	0.8	21.6	64.5	13.0	0.2	10.2	13.1	6.4
	D	6.5	19.8	61.4	12.3	1.9	9.1	12.7	6.3
	Mean				59.7				12.0
	SD				5.2				1.4
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.0	25.8	54.7	19.3	0.0	8.9	10.9	10.2
	B	0.0	19.2	66.0	14.8	0.0	6.9	13.7	9.4
	C	0.0	19.9	67.2	12.8	0.0	9.6	14.3	6.0
	D	0.3	21.7	66.5	11.5	0.1	10.3	14.1	5.5
	Mean				63.6				13.3
	SD				6.0				1.6

Table 4: Operating characteristics of the CRM in Setting 5 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	0.9	25.2	43.3	30.5	0.2	10.3	8.7	10.8
	B	1.8	24.1	45.7	28.4	0.5	10.3	9.3	9.9
	C	1.9	22.5	45.0	30.6	0.5	10.0	9.5	10.1
	D	0.3	23.5	47.4	28.7	0.1	10.2	9.8	10.0
	Mean				45.5				9.3
	SD				1.7				0.5
$Exp(1)$	A	1.3	25.2	34.1	39.3	0.4	9.1	6.8	13.8
	B	1.3	25.5	43.1	30.1	0.4	9.0	9.2	11.4
	C	1.7	22.4	45.8	30.1	0.4	9.7	10.0	9.9
	D	1.5	22.9	47.7	28.0	0.4	10.2	10.4	9.0
	Mean				42.7				9.1
	SD				6.0				1.6
$\mathcal{N}(0, 4)$	A	1.8	29.0	34.2	34.9	0.5	11.4	6.9	11.2
	B	1.4	29.9	42.3	26.5	0.4	11.4	9.0	9.2
	C	2.3	25.9	44.4	27.4	0.6	11.3	9.1	9.0
	D	11.1	22.5	39.9	26.5	3.2	9.4	8.6	8.8
	Mean				40.2				8.4
	SD				4.4				1.0
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.0	24.7	34.9	40.5	0.0	8.3	7.4	14.4
	B	0.0	19.7	47.3	33.0	0.0	6.5	10.1	13.4
	C	0.2	26.0	46.9	27.0	0.0	11.0	10.3	8.7
	D	1.6	26.7	46.6	25.2	0.4	11.5	10.1	7.9
	Mean				43.9				9.5
	SD				6.0				1.4

Table 5: Operating characteristics of the CRM in Setting 6 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	0.0	12.8	68.7	18.5	0.0	8.4	13.1	8.5
	B	0.1	18.3	58.2	23.4	0.0	10.1	11.1	8.8
	C	0.1	11.8	70.9	17.2	0.0	8.5	13.7	7.7
	D	0.0	12.1	66.9	21.1	0.0	8.7	13.1	8.1
	Mean				66.2				12.8
	SD				5.6				1.1
$Exp(1)$	A	0.0	11.0	69.4	19.6	0.0	7.1	13.6	9.3
	B	0.1	19.5	57.2	23.2	0.0	9.7	10.9	9.4
	C	0.1	10.6	71.7	17.5	0.0	8.8	15.1	6.1
	D	0.1	12.5	70.6	16.8	0.0	9.9	14.9	5.1
	Mean				67.2				13.6
	SD				6.7				1.9
$\mathcal{N}(0, 4)$	A	0.0	11.7	71.1	17.2	0.0	8.2	13.7	8.0
	B	0.1	20.1	56.3	23.5	0.0	10.8	10.7	8.5
	C	0.1	11.6	69.8	18.4	0.0	9.0	13.7	7.3
	D	2.0	13.2	66.4	18.4	0.6	9.4	13.1	6.9
	Mean				65.9				12.8
	SD				6.7				1.4
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.0	10.0	71.4	18.6	0.0	7.1	14.4	8.5
	B	0.0	18.6	58.7	22.7	0.0	9.0	11.6	9.4
	C	0.0	13.1	71.1	15.9	0.0	9.7	14.4	5.9
	D	0.1	13.8	68.4	17.8	0.0	10.7	13.6	5.7
	Mean				67.4				13.5
	SD				6.0				1.3

Table 6: Operating characteristics of the CRM in Setting 9 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	66.0	0.0	0.0	33.9	12.5	0.0	0.0	17.5
	B	71.0	0.0	0.0	29.0	14.5	0.0	0.0	15.5
	C	73.7	0.0	0.0	26.3	14.9	0.0	0.0	15.1
	D	70.8	0.0	0.0	29.2	11.9	0.0	0.0	18.1
	Mean	70.4				13.5			
	SD	3.2				1.5			
$Exp(1)$	A	63.8	0.0	0.0	36.2	12.4	0.0	0.0	17.6
	B	59.2	0.0	0.0	40.8	11.7	0.0	0.0	18.3
	C	62.2	0.0	0.0	37.8	12.5	0.0	0.0	17.5
	D	66.6	0.0	0.0	33.5	13.6	0.0	0.0	16.4
	Mean	63.0				12.6			
	SD	3.1				0.8			
$\mathcal{N}(0, 4)$	A	67.8	0.0	0.0	32.2	14.0	0.0	0.0	16.0
	B	65.6	0.0	0.0	34.4	13.6	0.0	0.0	16.4
	C	68.2	0.0	0.0	31.8	14.4	0.0	0.0	15.6
	D	76.9	0.0	0.0	23.1	18.2	0.0	0.0	11.8
	Mean	69.6				15.1			
	SD	5.0				2.1			
$\mathcal{N}(0, \sigma_{LI}^2)$	A	39.0	0.0	0.0	61.0	5.2	0.0	0.0	24.8
	B	20.4	0.0	0.0	79.7	2.1	0.0	0.0	27.9
	C	55.1	0.0	0.0	44.9	9.7	0.0	0.0	20.3
	D	64.5	0.0	0.0	35.4	12.8	0.0	0.0	17.2
	Mean	44.8				7.5			
	SD	19.3				4.7			

Table 7: Operating characteristics of the CRM in Setting 10 with four different prior distributions for  $\beta$  and four different skeletons.

Prior	Skeleton	Probability of Selecting MTD				Percentage of Patients Assigned			
		None	Below MTD	At MTD	Above MTD	None	Below MTD	At MTD	Above MTD
UM	A	4.7	66.4	14.5	14.4	1.1	22.5	2.8	3.6
	B	8.6	53.5	14.9	22.9	2.2	19.3	2.9	5.6
	C	8.9	66.0	11.7	13.4	2.2	22.1	2.5	3.2
	D	4.6	65.4	13.6	16.5	0.9	22.3	2.8	4.0
	Mean				13.7			2.7	
	SD				1.4			0.2	
$Exp(1)$	A	6.5	60.1	18.8	14.5	1.7	21.1	3.8	3.5
	B	6.4	51.5	15.0	27.1	1.7	18.5	3.1	6.8
	C	6.8	77.6	13.1	2.6	1.8	25.1	2.8	0.4
	D	8.2	73.9	11.7	6.3	2.1	24.6	2.2	1.1
	Mean				14.7			3.0	
	SD				3.1			0.6	
$\mathcal{N}(0, 4)$	A	8.8	62.7	14.3	14.2	2.2	21.5	2.8	3.4
	B	8.0	57.4	10.8	23.9	2.0	20.0	2.4	5.6
	C	10.2	66.5	13.6	9.8	2.6	22.2	2.7	2.5
	D	23.9	50.2	12.2	13.9	6.7	17.6	2.3	3.3
	Mean				12.7			2.6	
	SD				1.6			0.2	
$\mathcal{N}(0, \sigma_{LI}^2)$	A	0.5	70.9	16.5	12.0	0.1	23.9	3.2	2.8
	B	0.0	55.7	17.5	26.7	0.0	19.9	3.6	6.4
	C	2.5	80.5	11.9	5.2	0.6	25.7	2.6	1.1
	D	7.3	73.5	9.9	9.2	1.8	24.3	1.8	2.0
	Mean				13.9			2.8	
	SD				3.6			0.8	