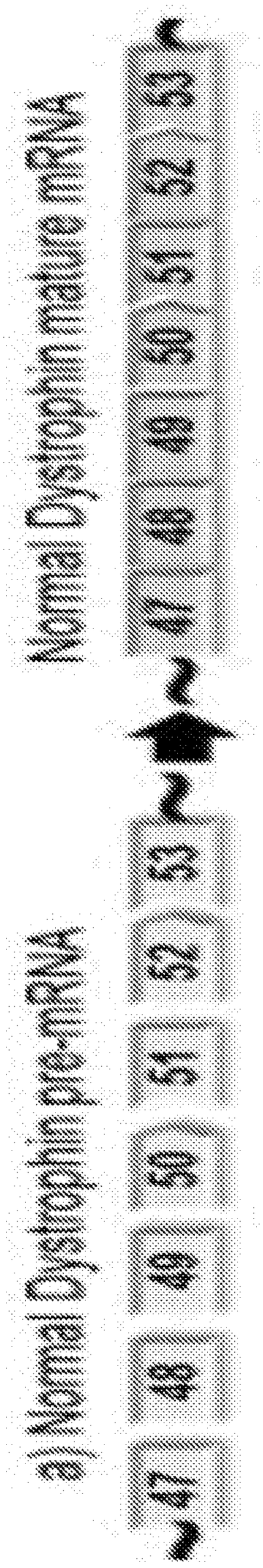
Figure 1


Nuclear/cytoplasmic tRNA
47
49
51
53
2
Cytoplasmic tRNA
53
51
49
2
Mitochondrial tRNA
49
47
2

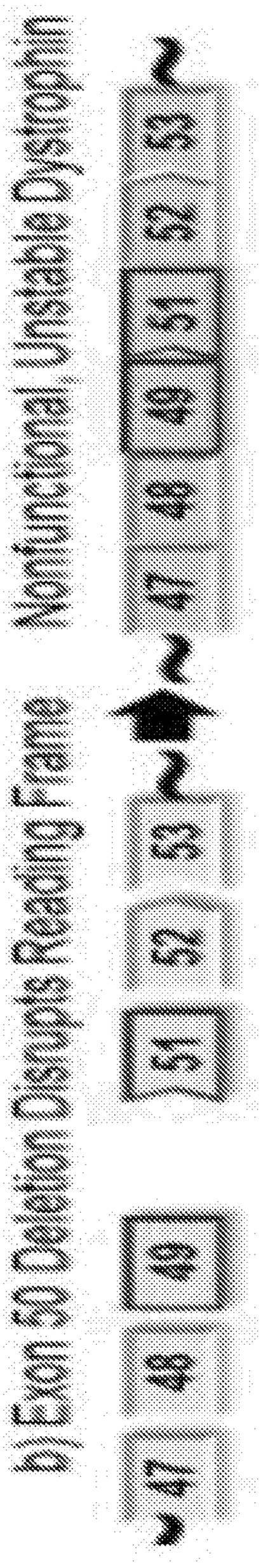
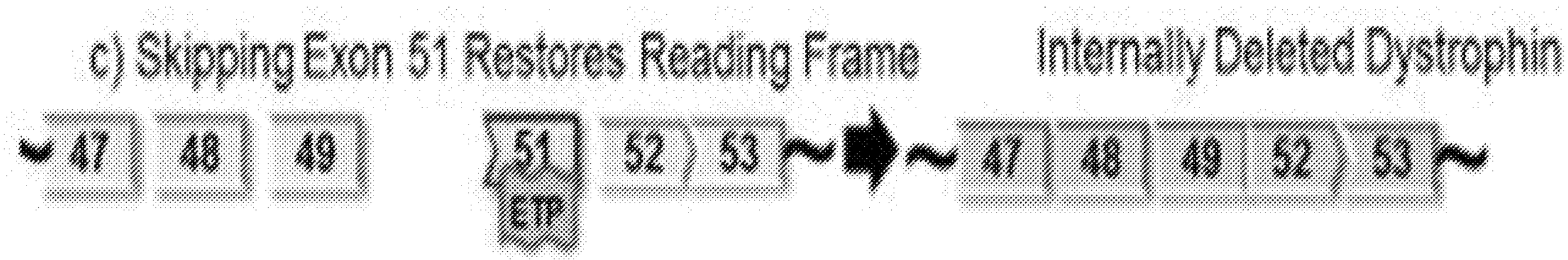

Non-Functional Unstable Dysfunctional
3D Collision Disrupts Reading Frame
Functional Protein
42
48
49
51
52
53
54
42
48
49
51
52
53
54

Figure 2

Figure 3



Source: Adapted from Kole 2012.

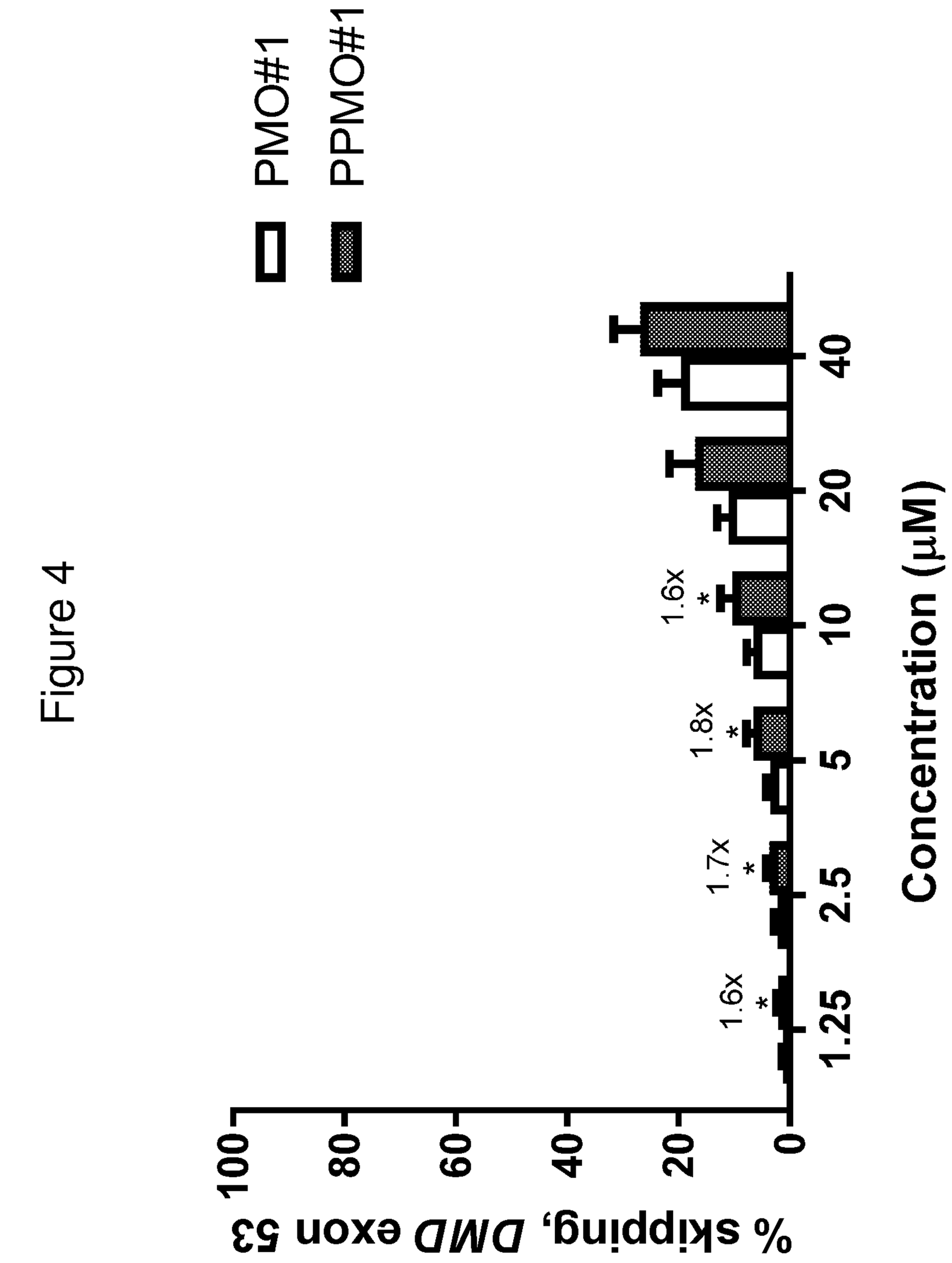

PMO#1
PPMO#1
1.6x
1.7x
1.8x
1.6x
*
*
*
*
1.25
2.5
5
10
20
40
Concentration (μM)
0
2
4
6
8
10
% skipping, DMD exon 53

Figure 4

Figure 5A

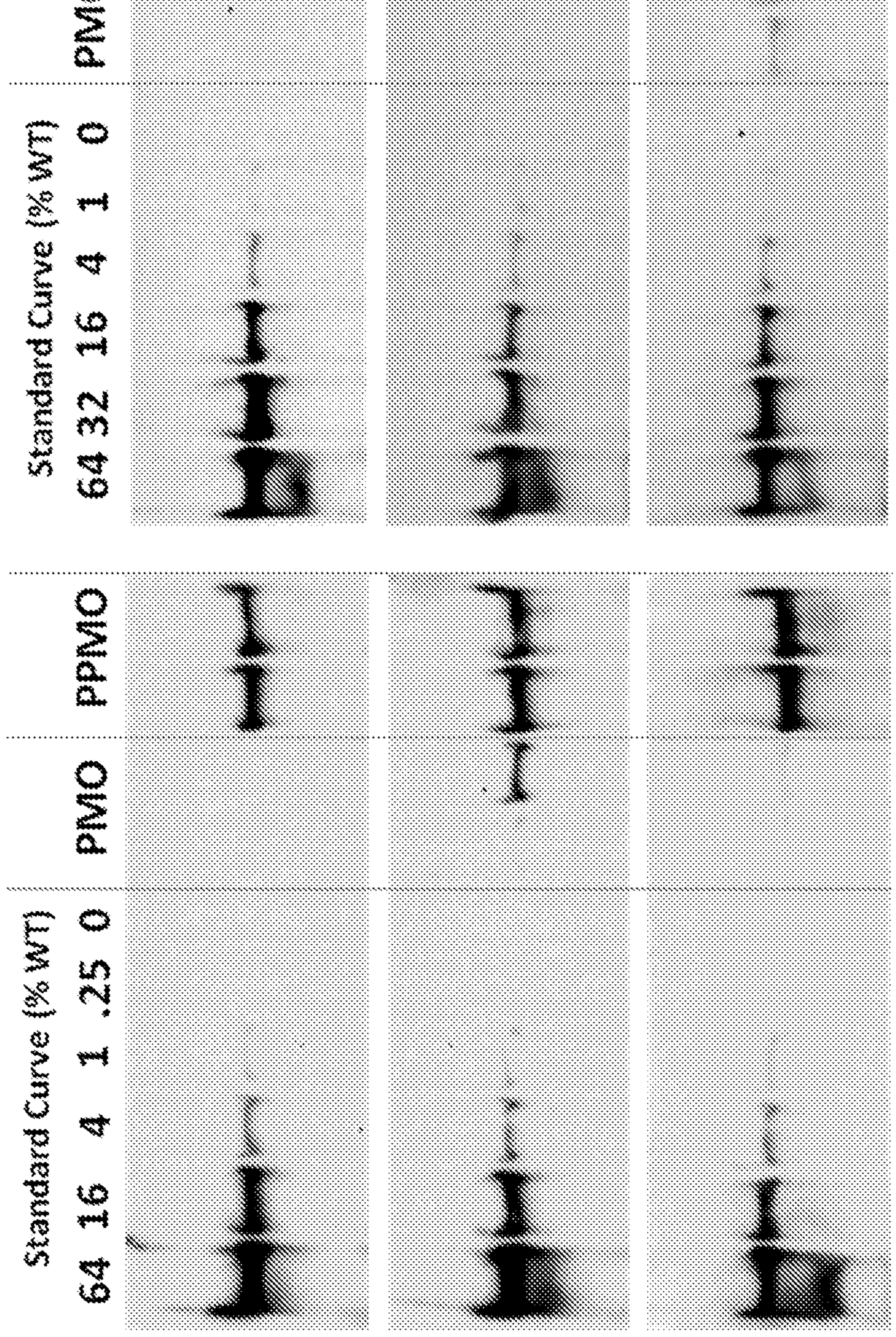


Figure 5B

Figure 5D

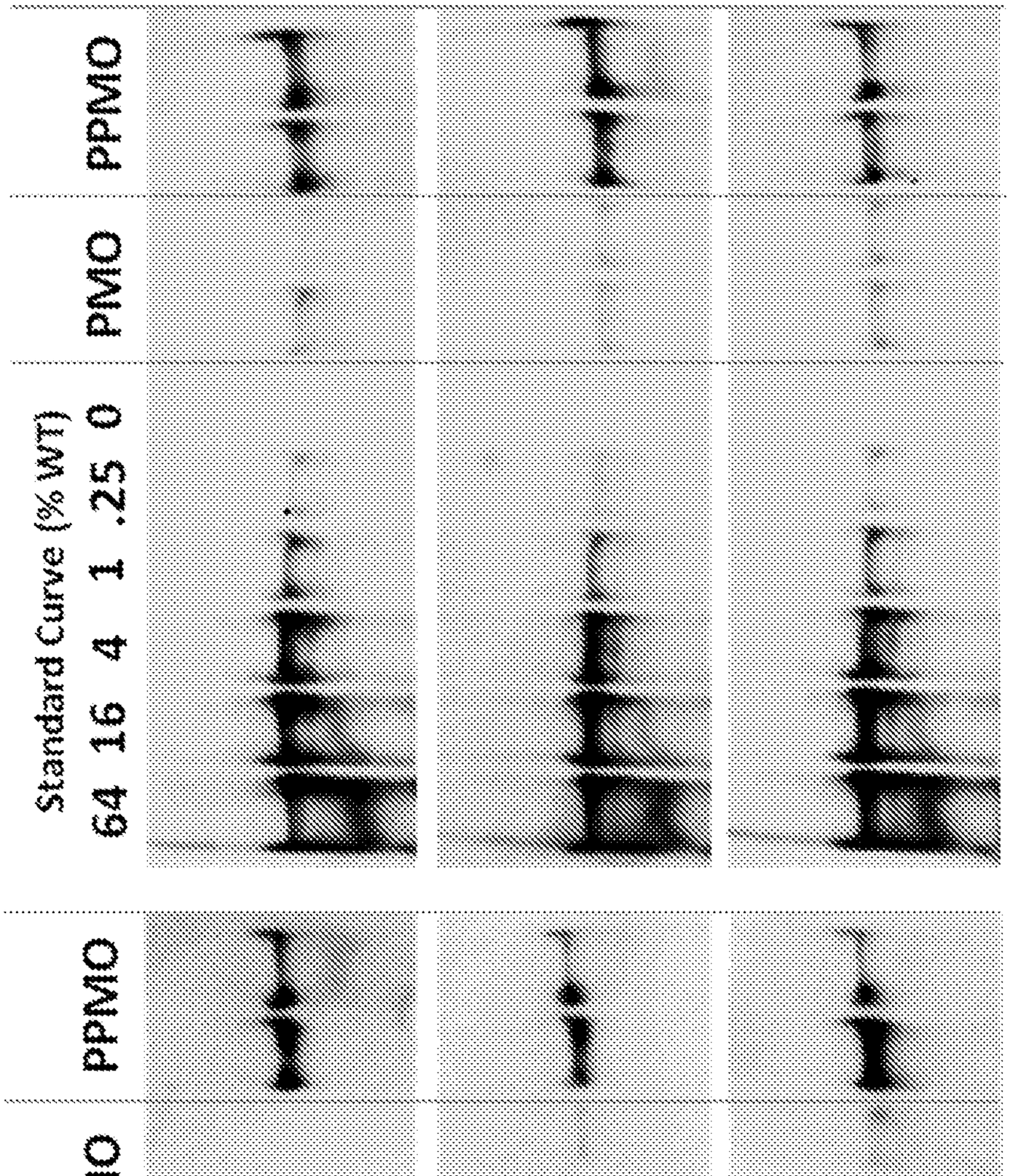


Figure 5C

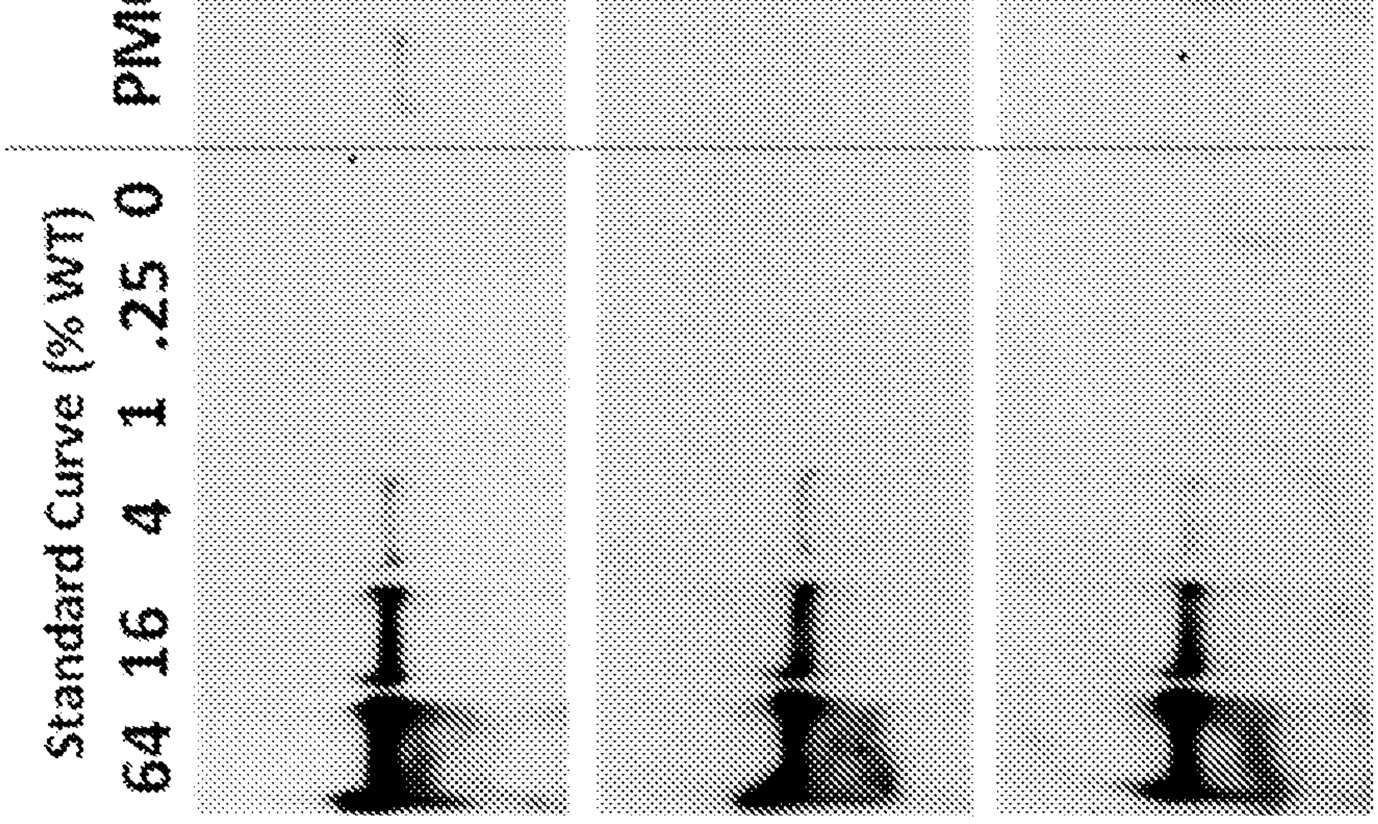


Figure 6A

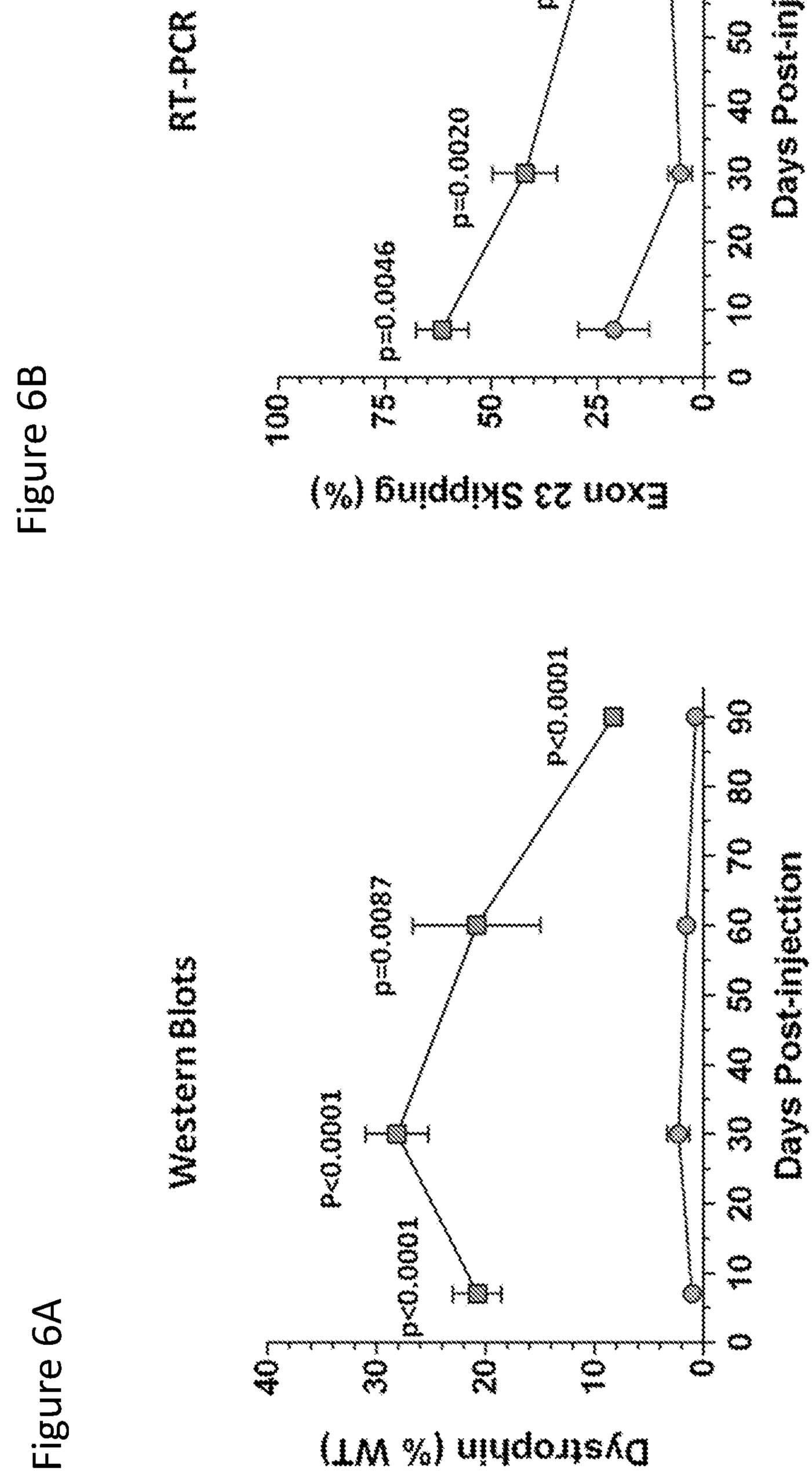


Figure 6B

Figure 7B
30 Days

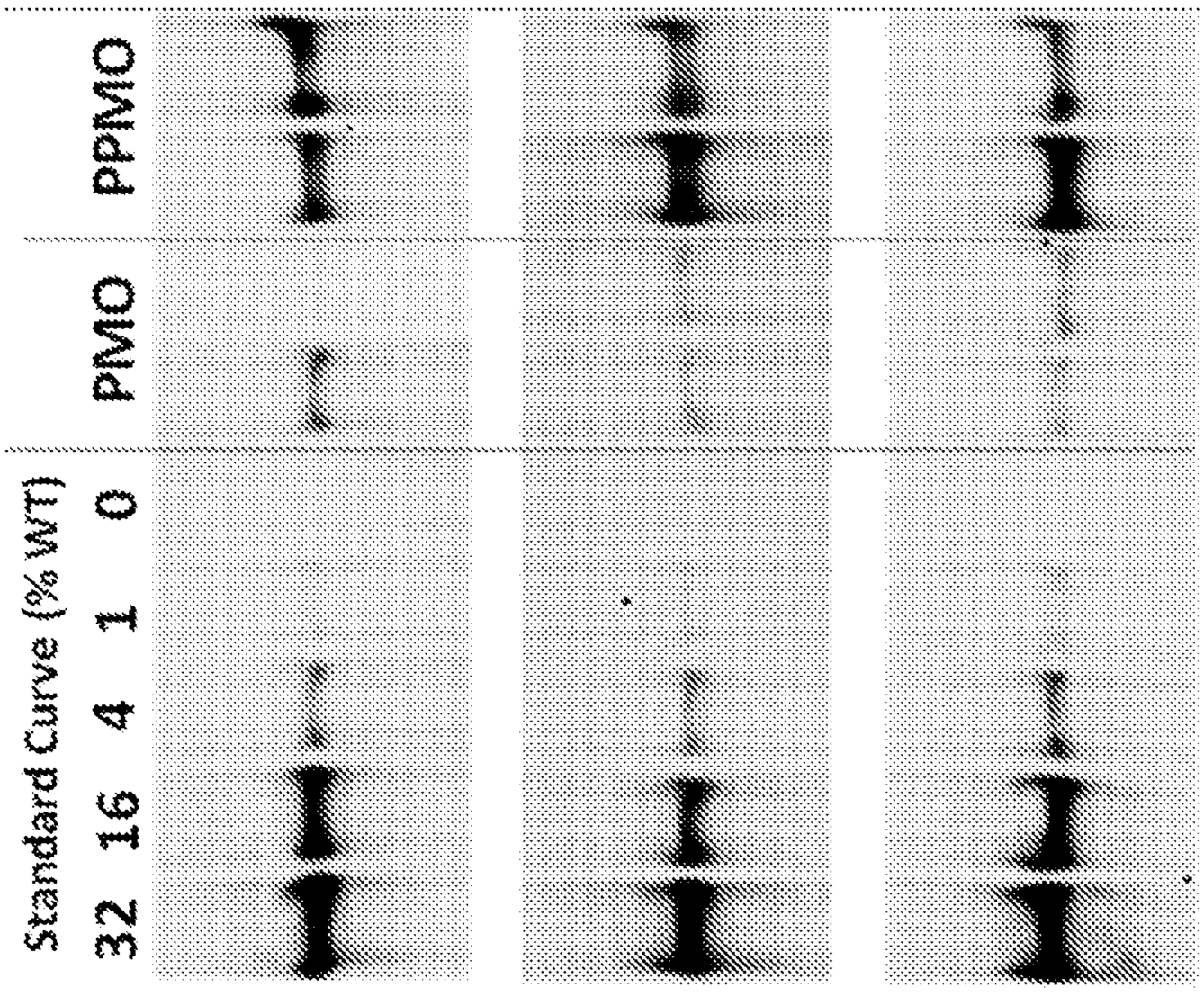


Figure 7A
7 Days

Figure 7D

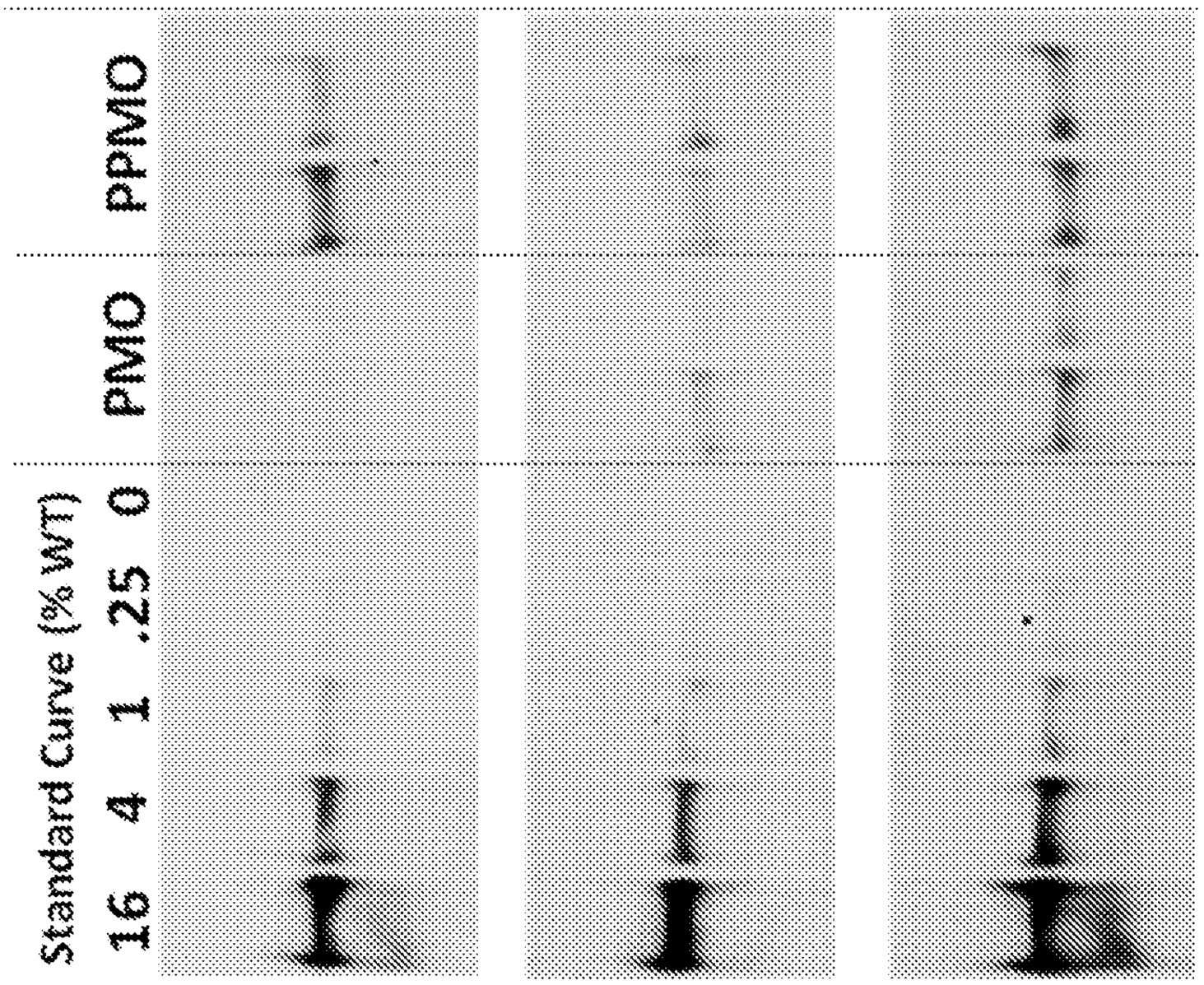


Figure 7C

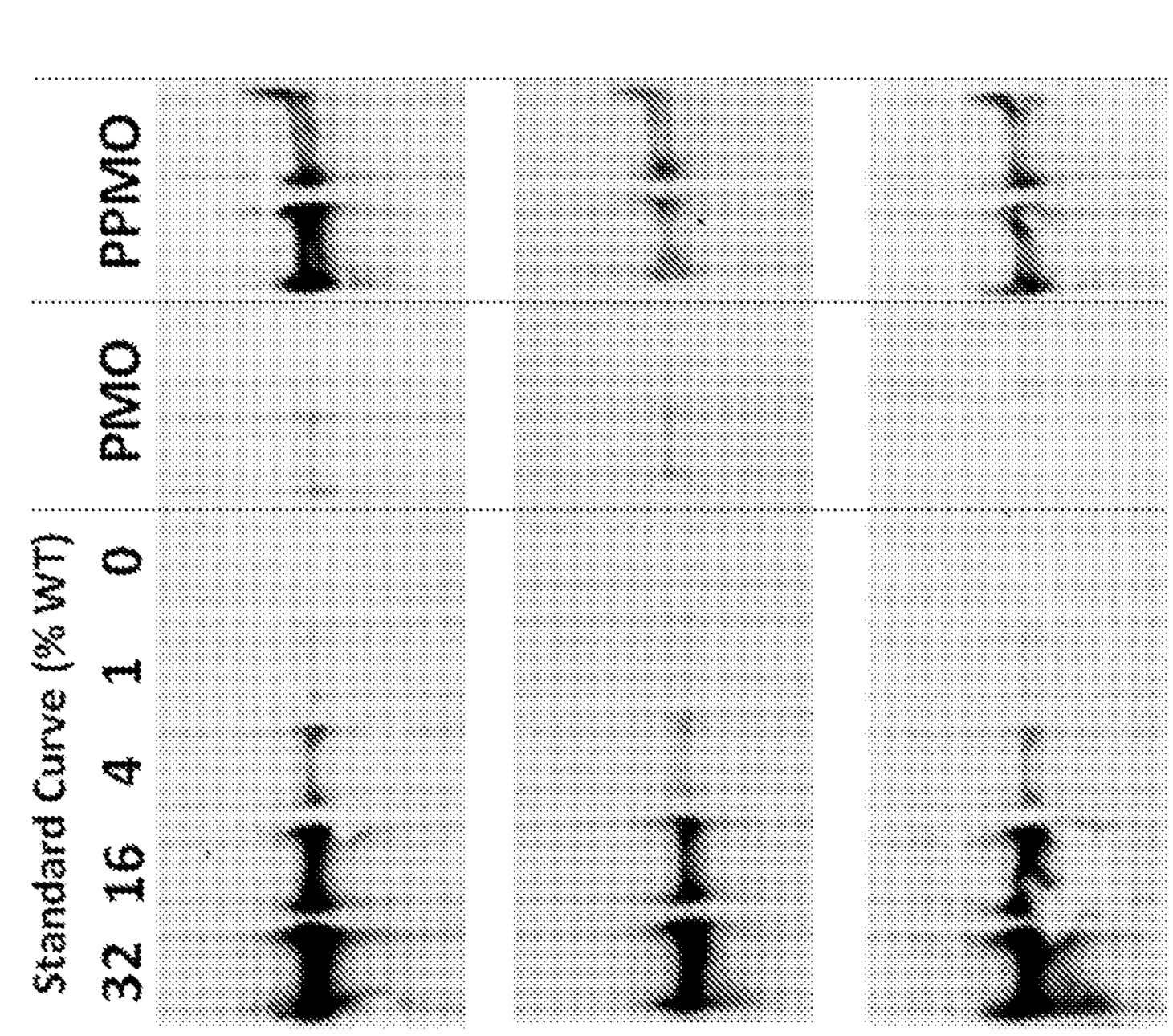


Figure 8A

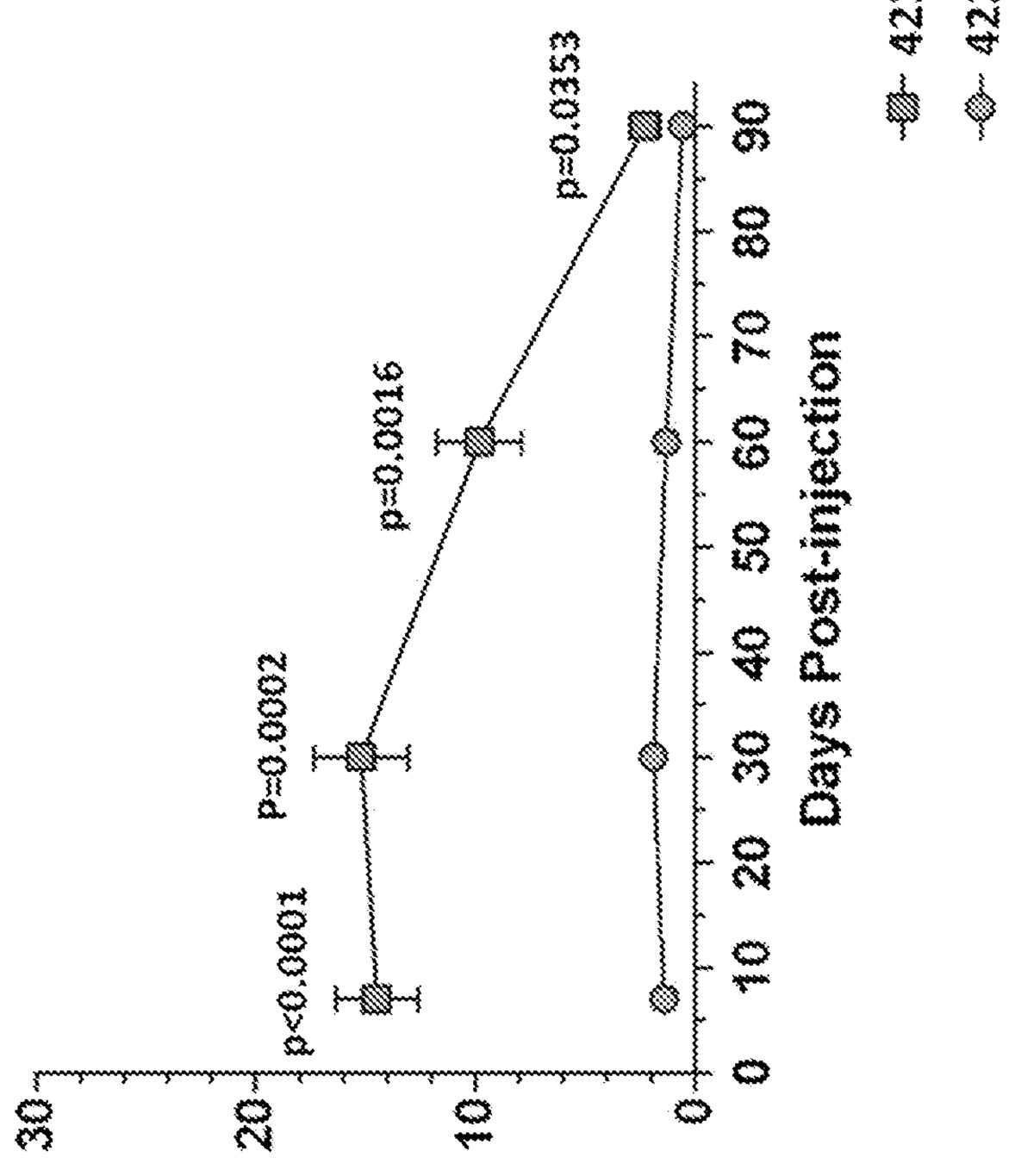


Figure 8B

Figure 9A

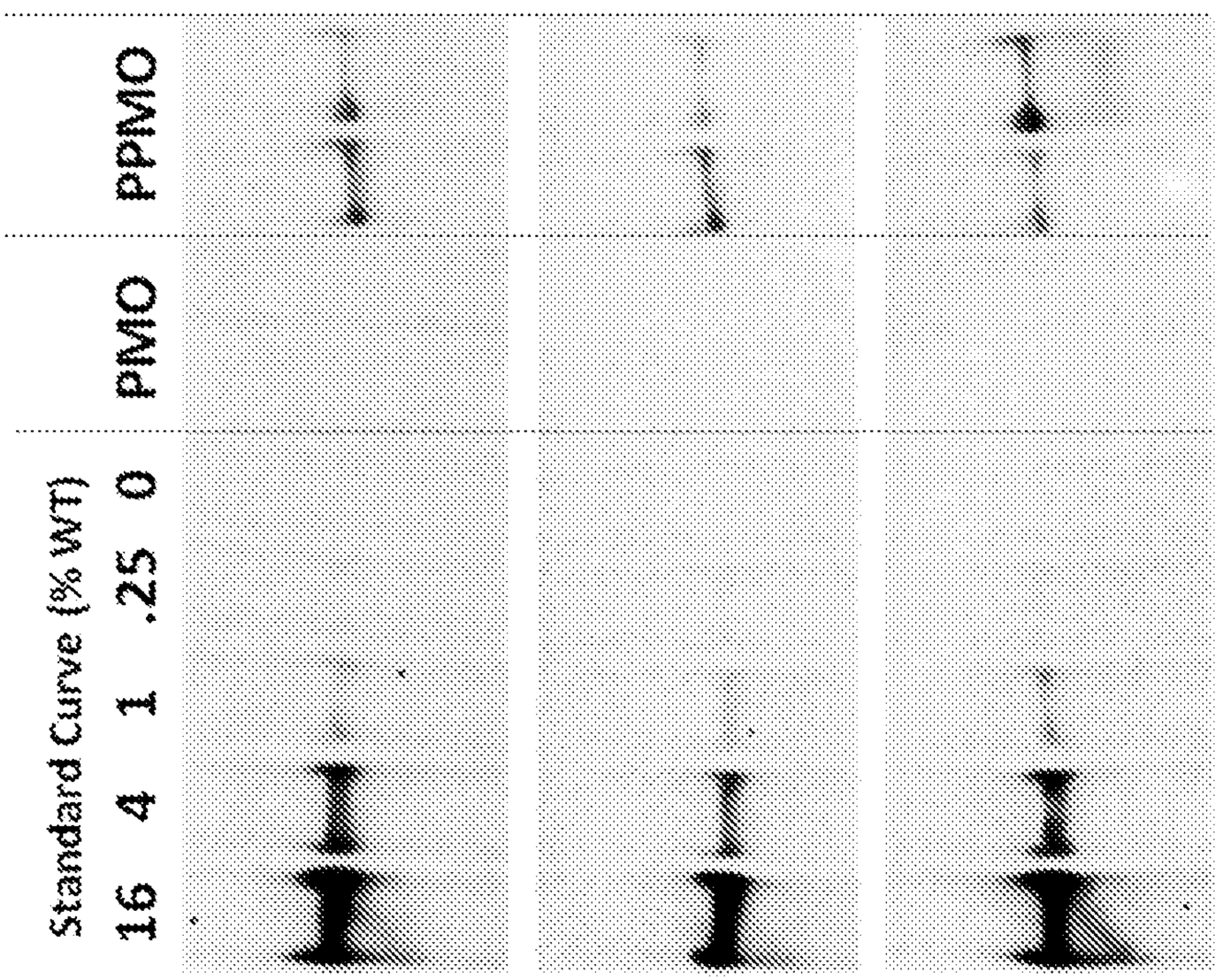


Figure 9B

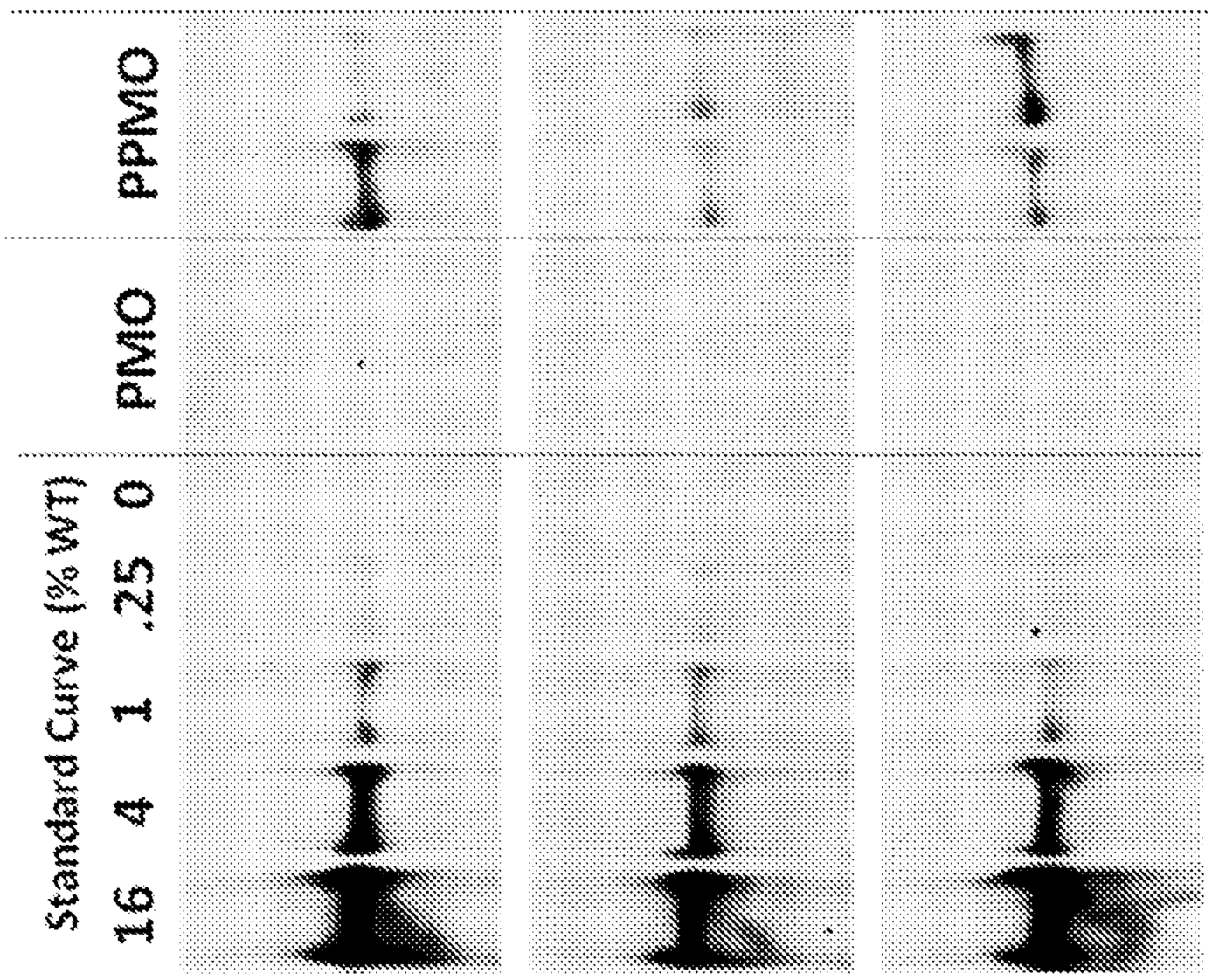


Figure 9C

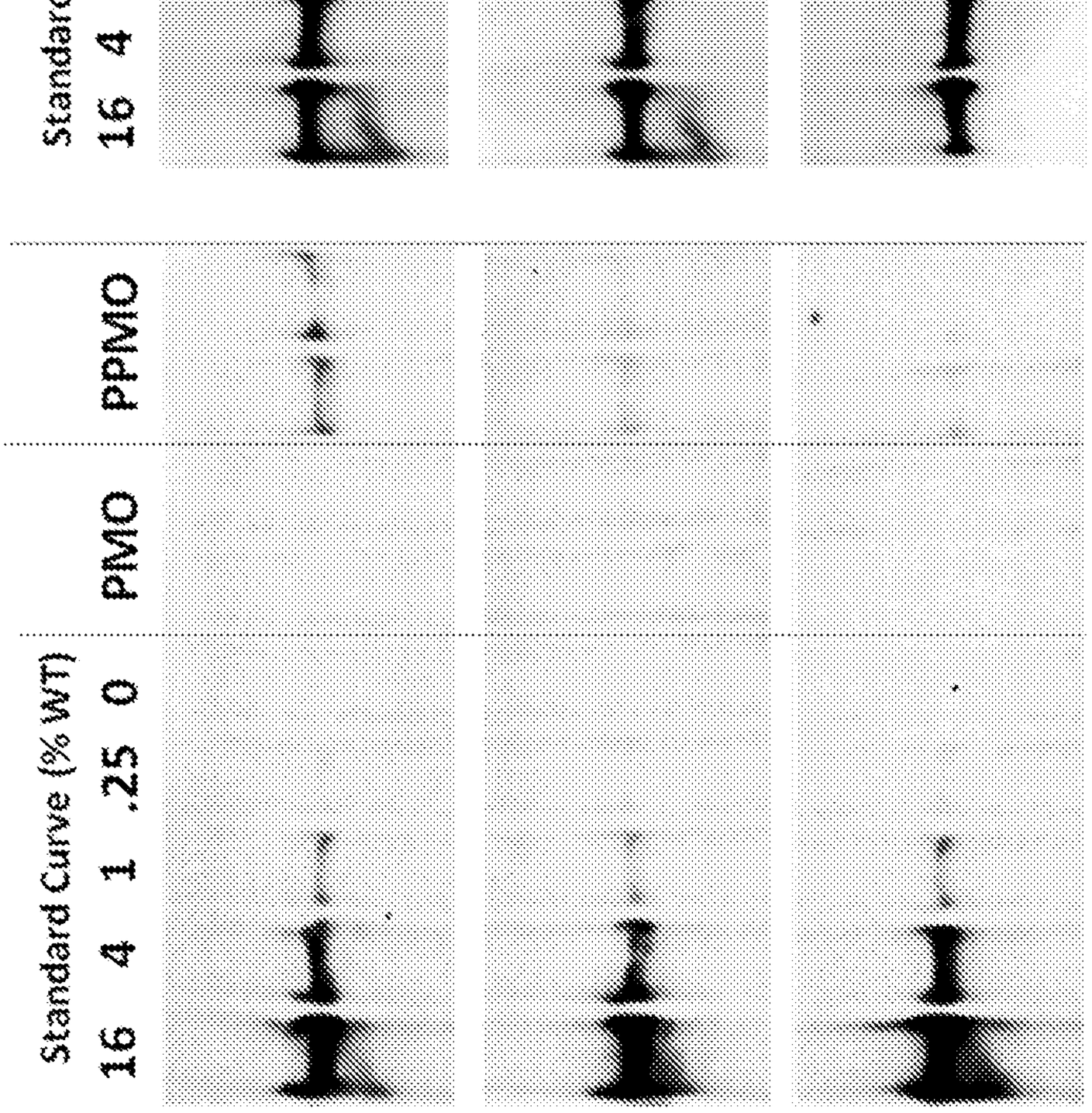


Figure 9D

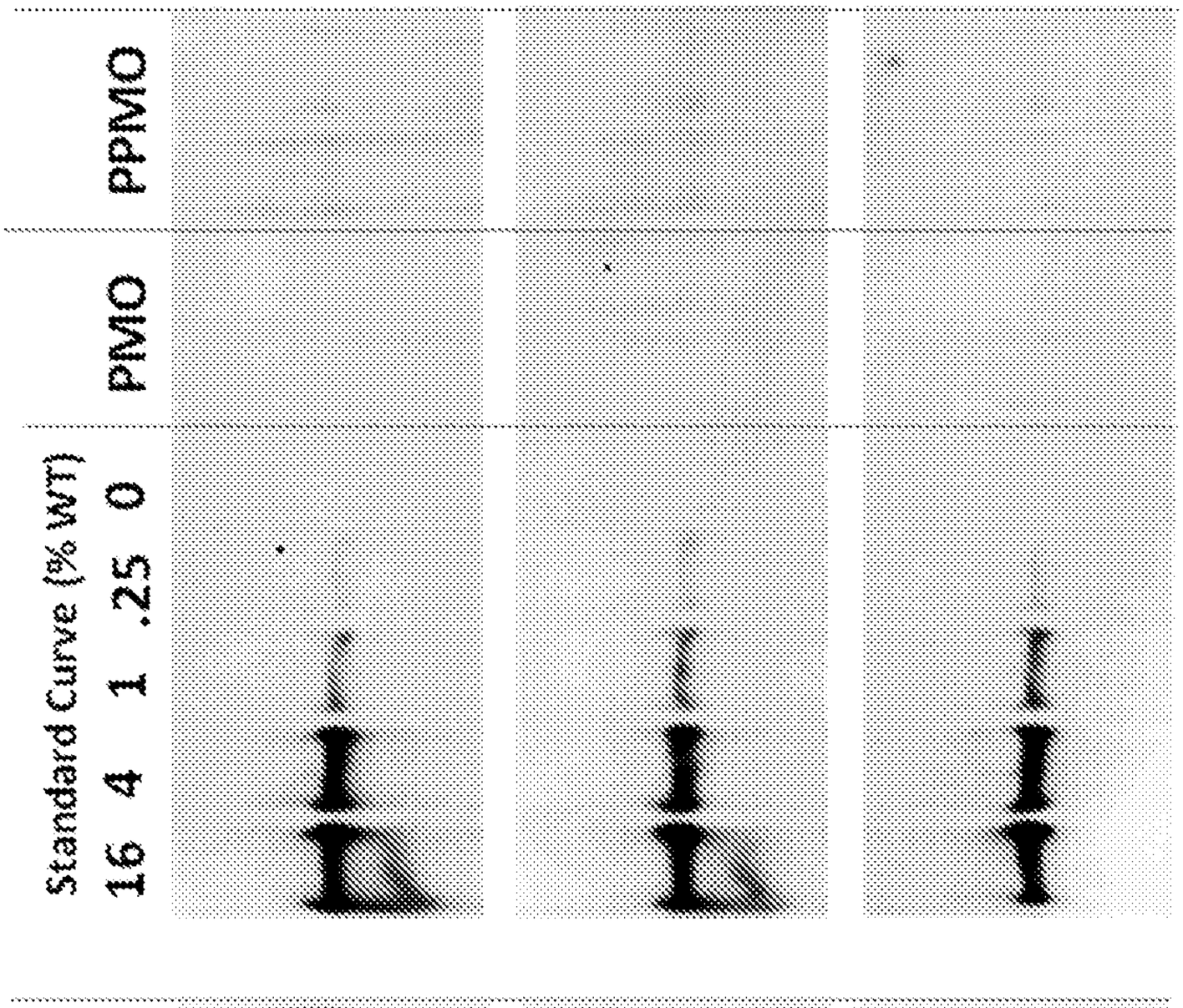


Figure 10A

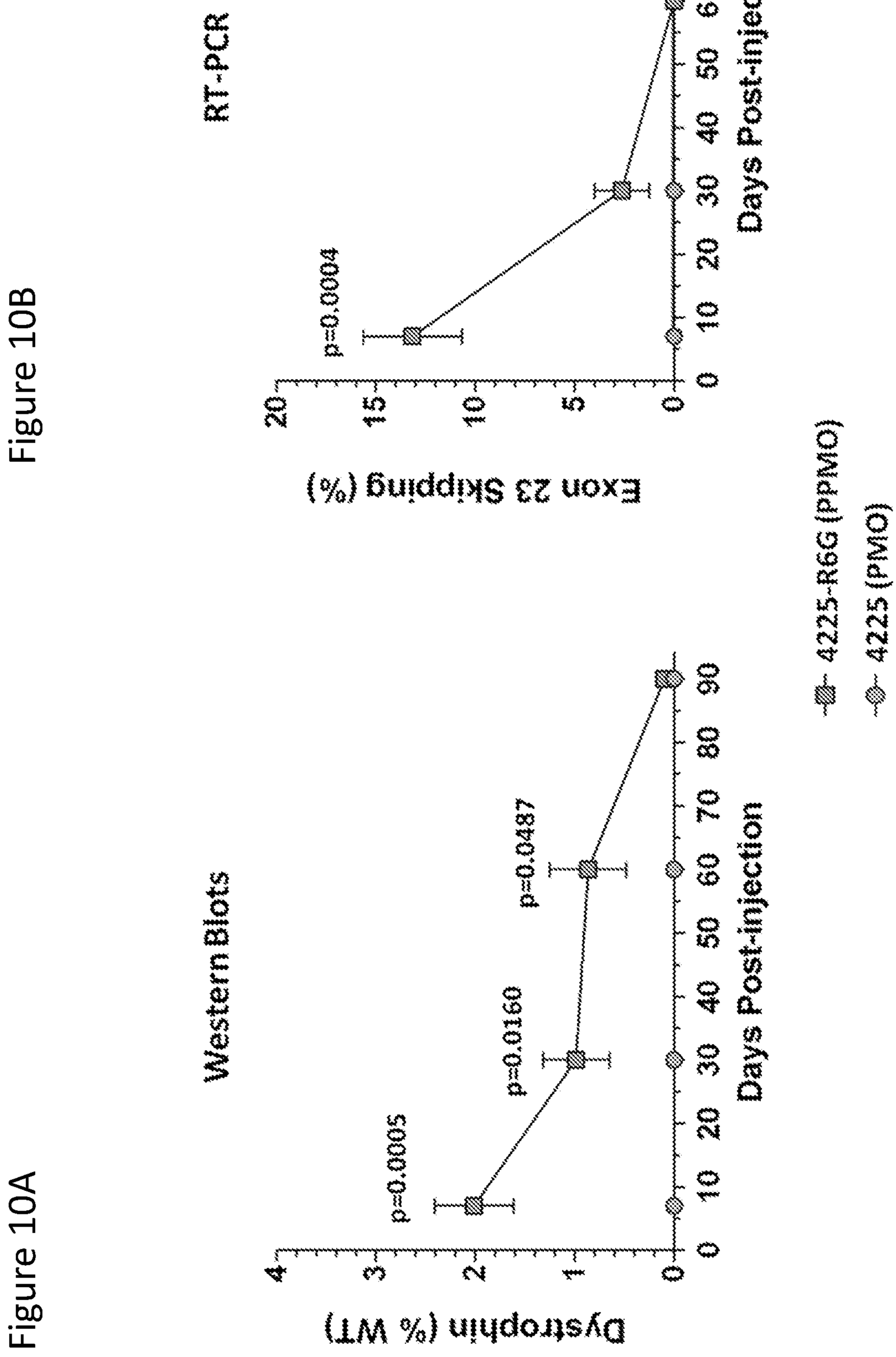


Figure 10B

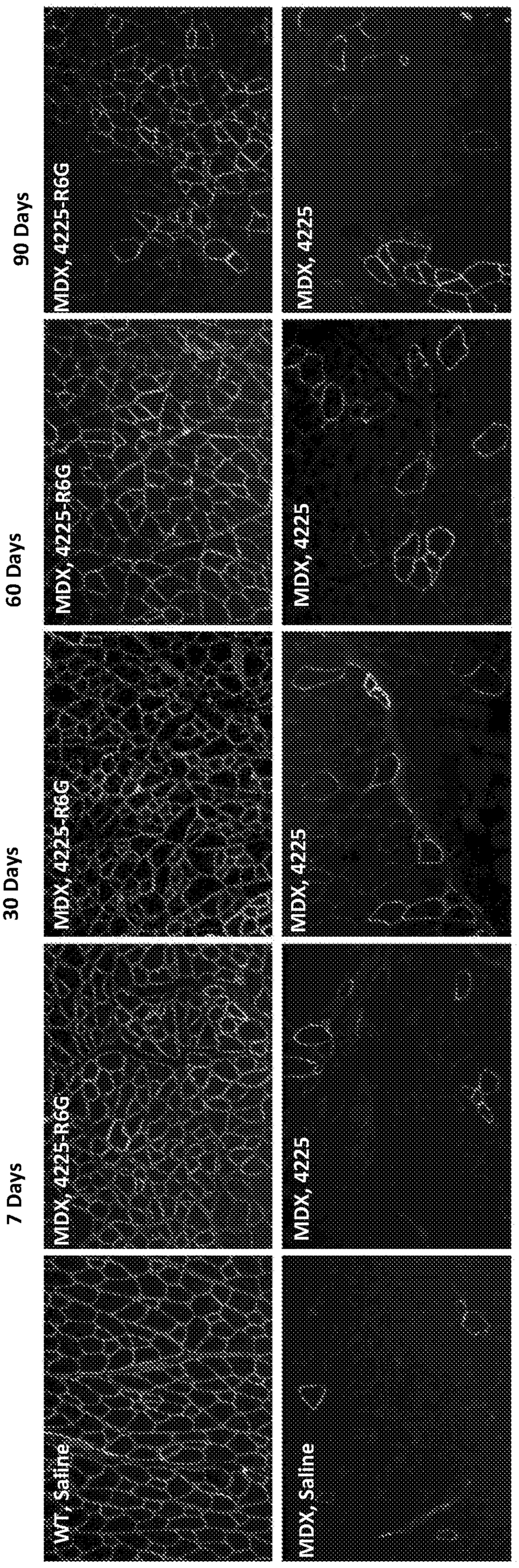

90 Days
MDX, 4225-R6G
MDX, 4225
60 Days
MDX, 4225-R6G
MDX, 4225
30 Days
MDX, 4225-R6G
MDX, 4225
7 Days
MDX, 4225-R6G
MDX, 4225
WT, Saline
MDX, Saline

Figure 11

Figure 12A

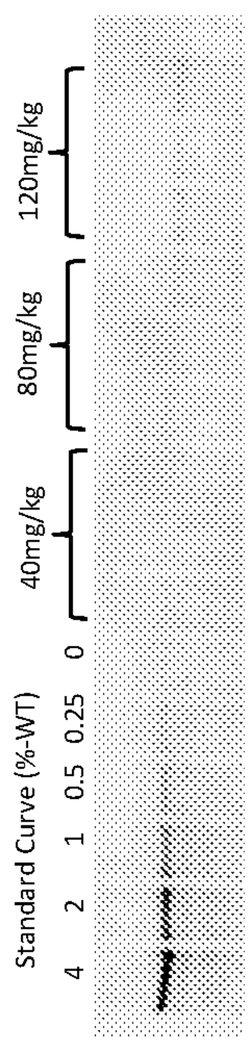
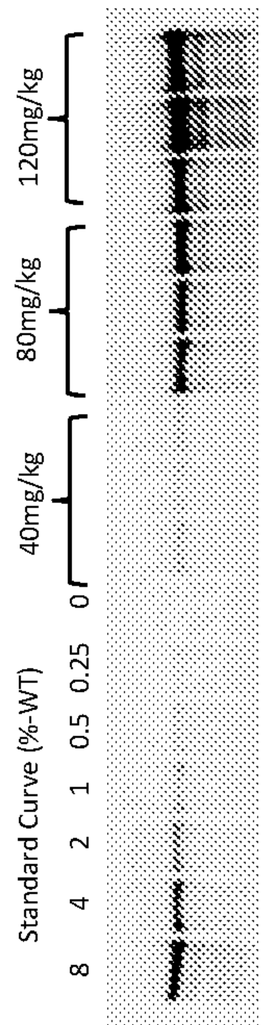


Figure 12B



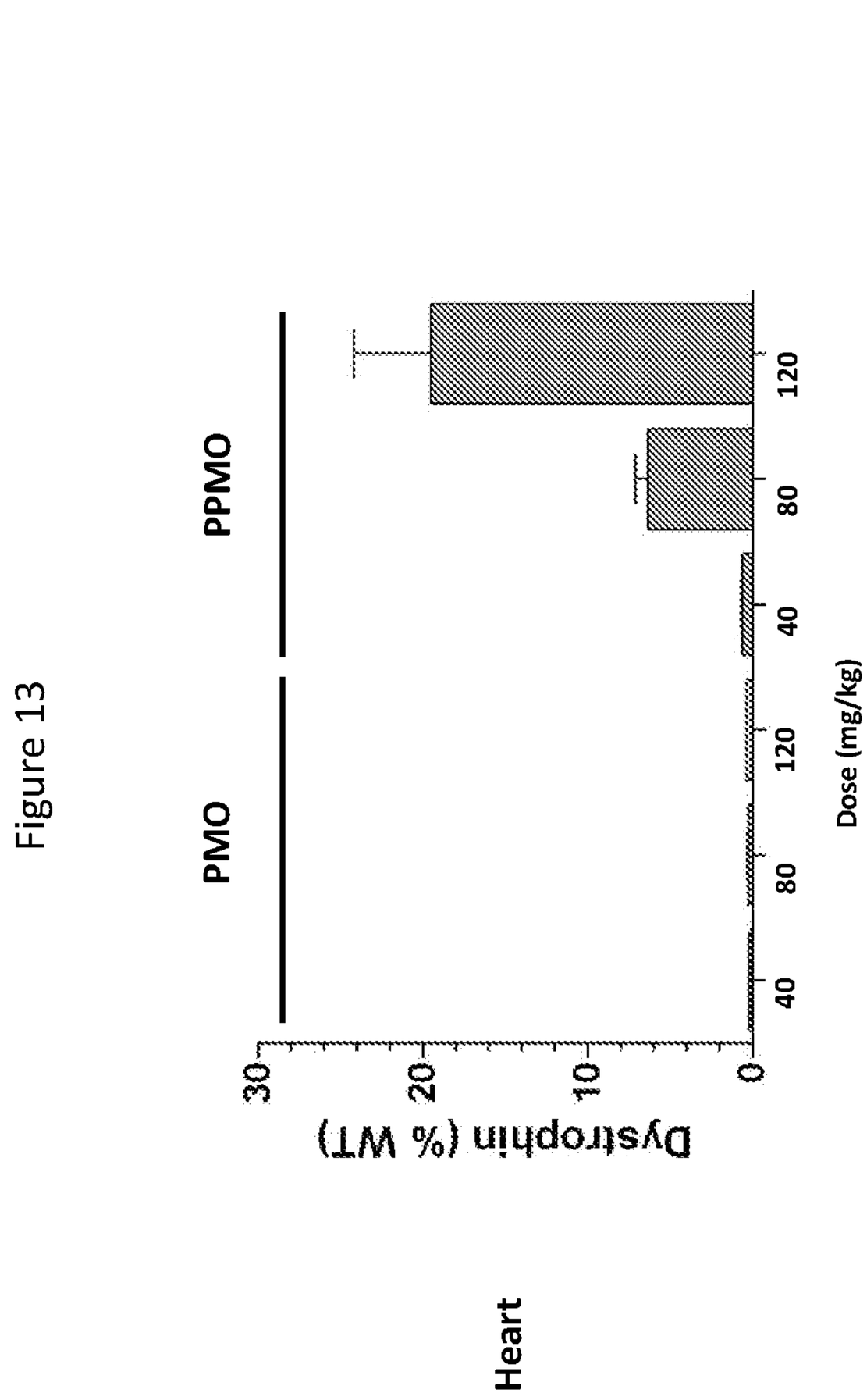


Figure 13

Figure 14A

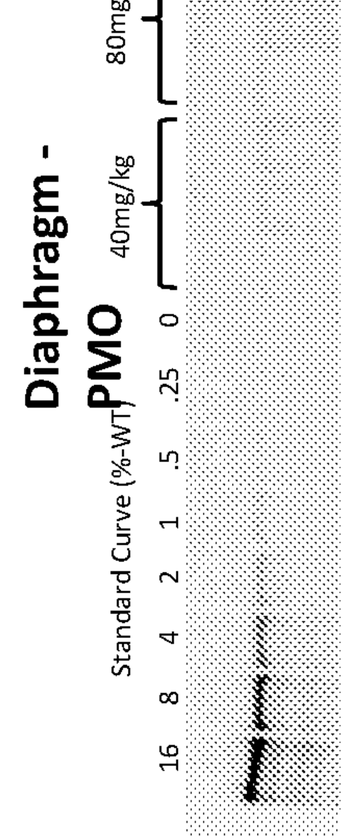
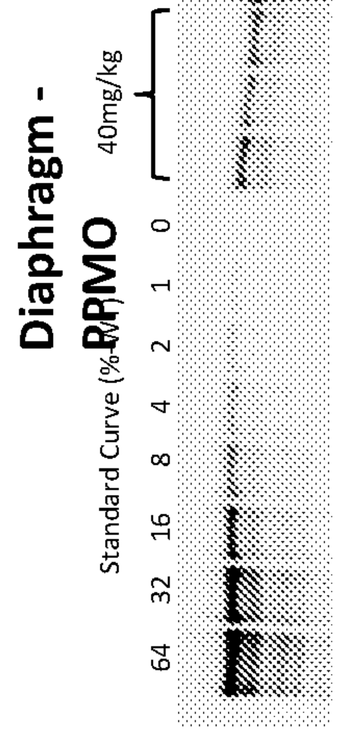


Figure 14B



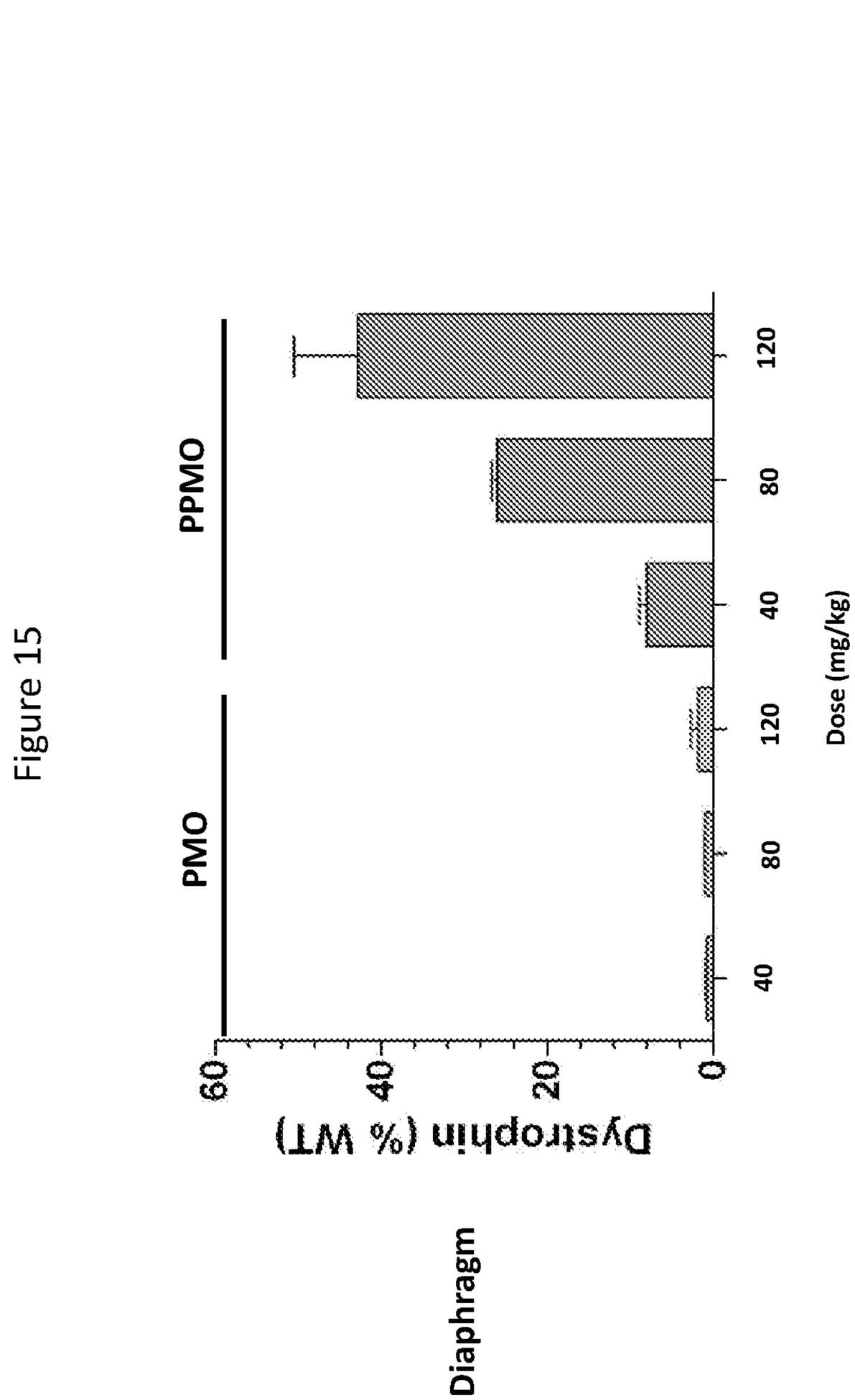

Diaphragm
Dystrophin (% WT)
0
20
40
60
PMO
PPMO
40
80
120
Dose (mg/kg)

Figure 15

Figure 16A

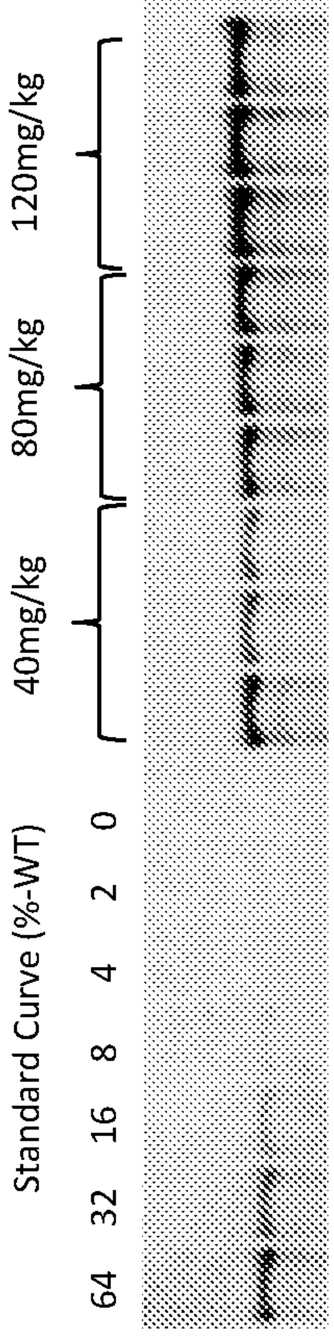


Figure 16B

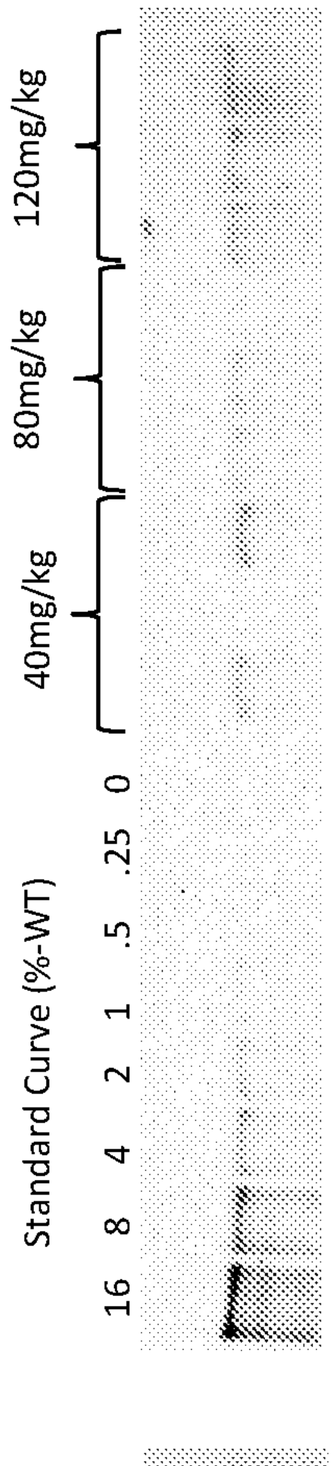
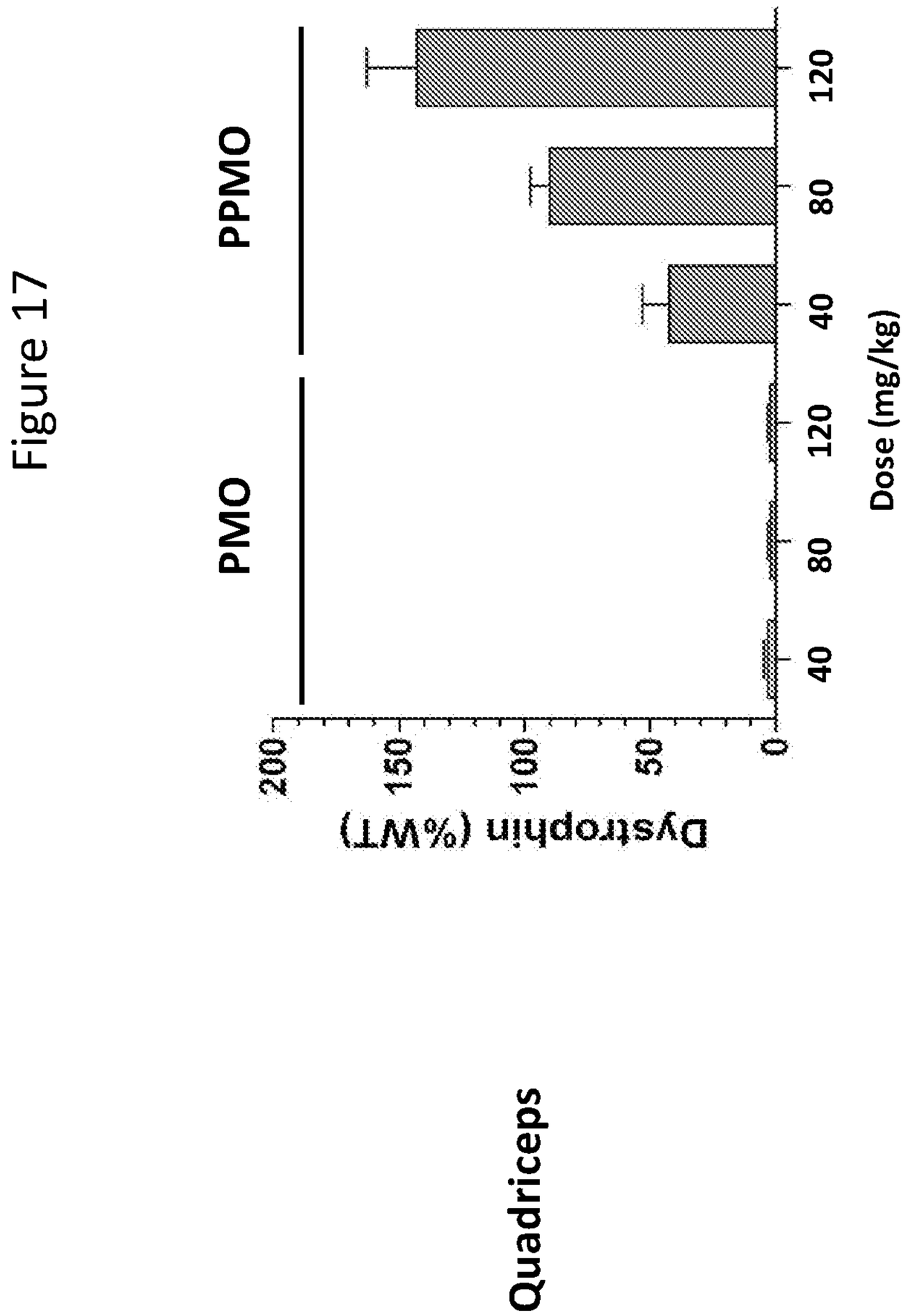


Figure 17


PPMO
PMO
Dystrophin (%WT)
200
150
100
50
0
120
80
40
120
80
40
Dose (mg/kg)
Quadricep

Cycle No.: Subunit (SU)	Pre-coupling Treatment				Coupling Cycle		Post-Coupling Treatment	
	1	2	3	4			1	2
	30% TFE/DCM Wash	CYTFA Solution[1]	Neutralization Solution	DCM Wash	Quantity SU (g) NEM (L) DMI (L)	RT Coupling Time (Hrs.)	DCM Wash	30% TFE/DCM Wash
1:DPG	5.5L	a) 5,5L b) 5.5L, 122ml	3x5.5L	5.5L	536.7g; 195 ml NEM; 3.2L DMI	5	5.5L	2x5.5L
2:T	7.0L	a) 7L b) 7L, 158ml	3x7L	2x7L	468.2g and 195ml NEM 3.2L DMI	4.25	7L	2x7L
3:T	8L	a) 8L b) 8L, 182ml	3x8L	2x8L	536.7g; 195ml NEM; 3.4L DMI	4.25	8L	2x8L
4:DPG	9L	a) 9L b) 9L, 206ml	3x9L	2x9L	536.7g; 195ml NEM; 3.6L DMI	4.25	9L	2x9L
5:C	9.5L	a) 9.5L b) 9.5L, 220ml	3x9.5L	2x9.5L	555.2g; 195ml NEM; 3.4L DMI	4.25	9.5L	2x9.5L
6:C	10L	a) 10L b) 10L, 232ml	3x10L	2x10L	555.2g; 195ml NEM; 3.45L DMI	4.25	10L	2x10L
7:T	11L	a) 11L b) 11L, 256ml	3x11L	2x11L	536.7g; 195ml NEM;	4.25	11L	2x11L

Figure 18

[1] ml indicates the amount of 1:1 NEM/DCM

Cycle No.: Subunit (SU)	Pre-coupling Treatment				Coupling Cycle		Post-Coupling Treatment	
	1	2	3	4			1	2
	30% TFE/DCM Wash	CYTFA Solution[1]	Neutralization Solution	DCM Wash	Quantity SU (g) NEM (L) DMI (L)	RT Coupling Time (Hrs.)	DCM Wash	30% TFE/DCM Wash
					3.57L DMI			
8:C	11L	a) 11L b) 11L, 256ml	3x11L	2x11L	555.2g; 195ml NEM; 3.64L DMI	4.25	11L	2x11L
9:C	11.5L	a) 11.5L b) 11.5L 268ml	3x11.5L	2x 11.5L	468.2g; 195ml NEM; 3.72L DMI	4.25	11.5L	2x11.5L
10:DPG	12L	a) 12L b) 12L, 280ml	3x12L	2x12L	536.7g; 195ml NEM; 3.96L DMI	4.25	12L	2x12L
11:DPG	13.5L	a) 13.5L b) 13.5L, 204ml	3x13.5L	2x 13.5L	721.7g; 253ml NEM; 4.02L DMI	4.25	13.5L	2x13.5L
12:T	13.5L	a) 13.5L b) 13.5L, 204ml	3x13.5L	2x 13.5L	721.7g; 253ml NEM; 4.02L DMI	4.25	13.5L	2x13.5L
13:T	14L	a) 14L b) 14L, 216ml	3x14L	2x14L	941.9g; 253ml NEM; 4.02L DMI	4.25	14L	2x14L
14:C	14.5L	a) 14.5L b) 14.5L, 228ml	3x14.5L	2x 14.5L	941.9g; 253ml NEM; 4.1L DMI	4.25	14.5L	2x14.5L

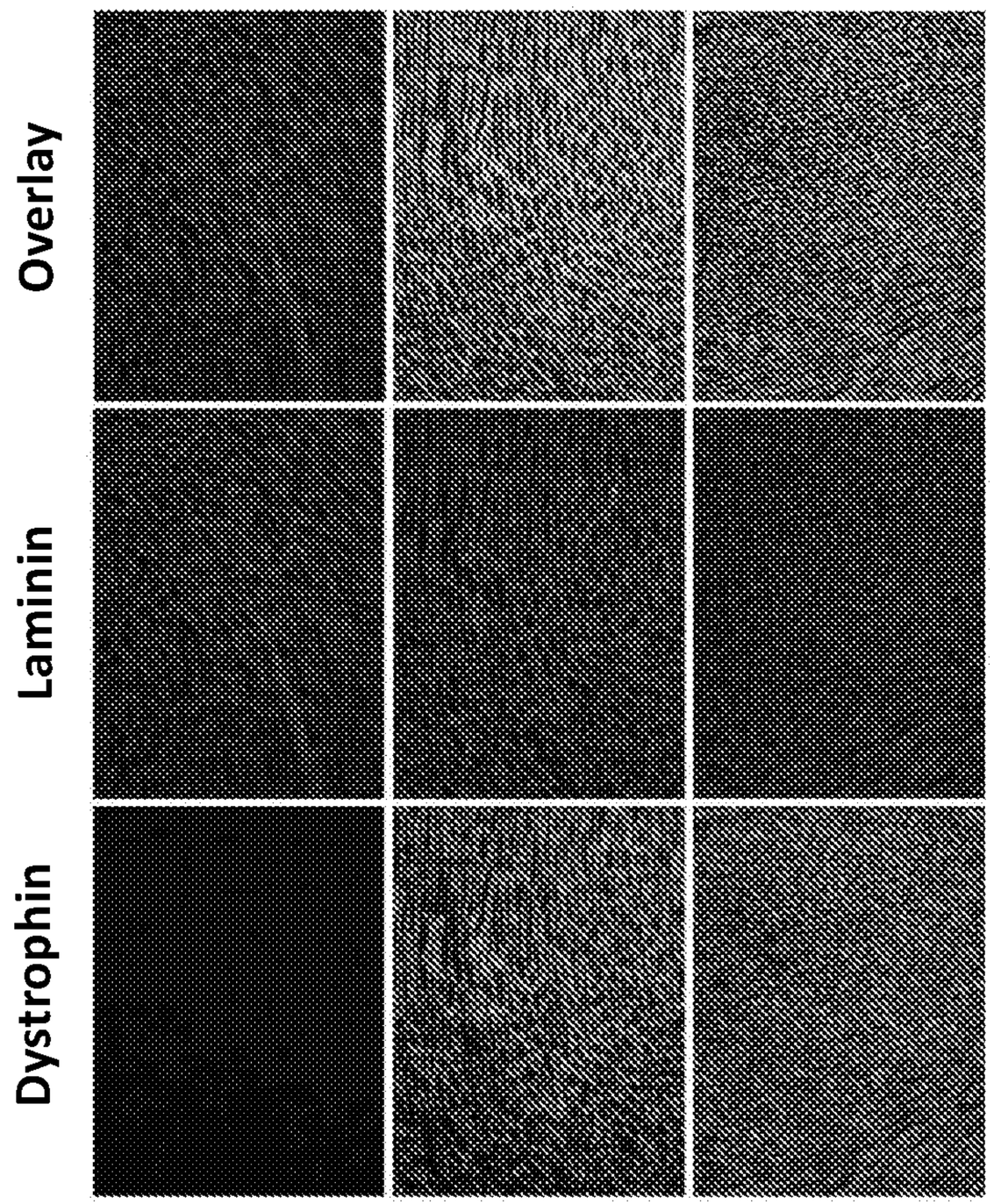
Figure 18 (continued)

Cycle No.: Subunit (SU)	Pre-coupling Treatment				Coupling Cycle		Post-Coupling Treatment	
	1	2	3	4			1	2
	30% TFE/DCM Wash	CYTFA Solution[1]	Neutralization Solution	DCM Wash	Quantity SU (g) NEM (L) DMI (L)	RT Coupling Time (Hrs.)	DCM Wash	30% TFE/DCM Wash
15:T	15.5L	a) 15.5L b) 15.5L, 254ml	3x15.5L	2x 15.5L	721.7g; 253ml NEM; 4.26L DMI	4.25	15.5L	2x15.5L
16:DPG	15.5L	a) 15.5L b) 15.5L, 254ml	3x15.5L	2x 15.5L	721.7g; 253ml NEM; 4.26L DMI	4.25	15.5L	2x15.5L
17:A	16L	a) 16L b) 16L, 366ml	3x16L	2x16L	941.9g; 253ml NEM; 4.4L DMI	4.75	16L	2x16L
18:A	16.5L	a) 16.5L b) 16.5L, 378ml	3x16.5L	2x 16.5L	721.7g; 253ml NEM; 4.4L DMI	4.25	16.5L	2x16.5L
19: DPG	16.5L	a) 16.5L b) 16.5L, 378ml	3x16.5L	2x 16.5L	608.7g; 253ml NEM; 4.57L DMI	4.25	16.5L	2x16.5L
20:DPG	17L	a) 17L b) 17L, 390ml	3x17L	2x17L	941.9g; 253ml NEM; 4.57L DMI	4.75	17L	2x17L
21:T	17L	a) 17L b) 17L, 390ml	3x17L	2x17L	1159.2g; 311ml NEM; 4.72L DMI	4.25	17L	2x17L
22: DPG	17.5L	a) 17.5L b) 17.5L, 402ml	3x17.5L	2x 17.5L	858.7g; 311ml NEM;	4.75	17.5L	2x17.5L

Figure 18 (continued)

Cycle No.: Subunit (SU)	Pre-coupling Treatment				Coupling Cycle		Post-Coupling Treatment	
	1	2	3	4			1	2
	30% TFE/DCM Wash	CYTFA Solution[1]	Neutralization Solution	DCM Wash	Quantity SU (g) NEM (L) DMI (L)	RT Coupling Time (Hrs.)	DCM Wash	30% TFE/DCM Wash
					4.72L DMI			
23:T	17.5L	a) 17.5L b) 17.5L, 402ml	3x17.5L	2x 17.5L	888.3g; 311ml NEM; 4.88L DMI	4.25	17.5L	2x17.5L
24:T	18L	a) 18L b) 18L, 414ml	3x18L	2x18L	749.1g; 311ml NEM; 4.95L DMI	4.25	18L	2x18L
25:C	18L	a) 18L b) 18L, 414ml	3x18L	2x18L	749.1g; 311ml NEM; 5.1L DMI	4.25	18L	2x18L

Figure 18 (continued)


Heart
Overlay
Laminin
Dystrophin

Diaphragm
Overlay
Laminin
MDX Saline
MDX PPMO
WT Saline
Dystrophin

Figure 19

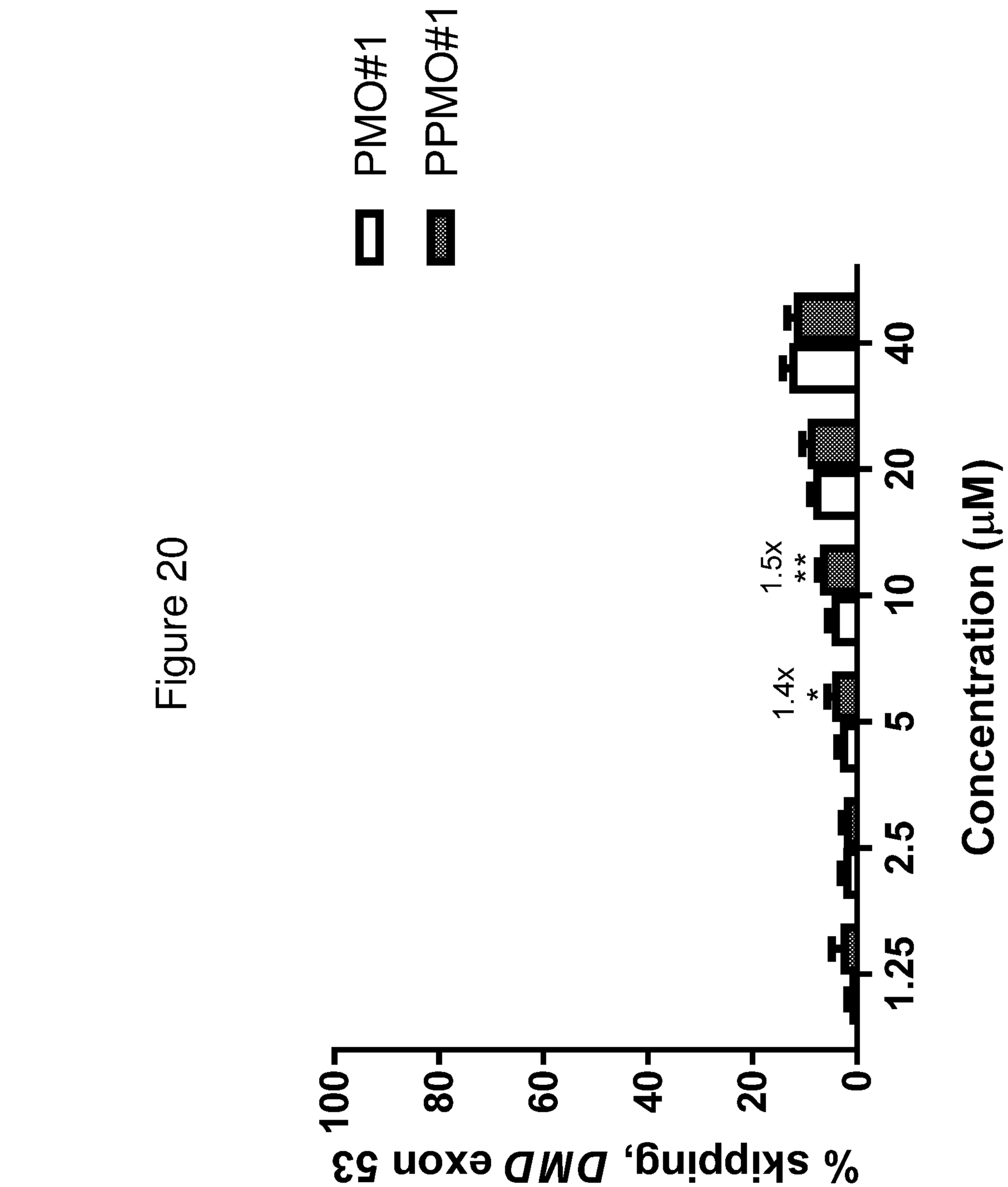

PMO#1
PMO#1
1.5x
**
1.4x
*
0
20
40
60
80
100
1.25
2.5
5
10
20
40
% skipping, DMD exon 53
Concentration (μM)

Figure 20