

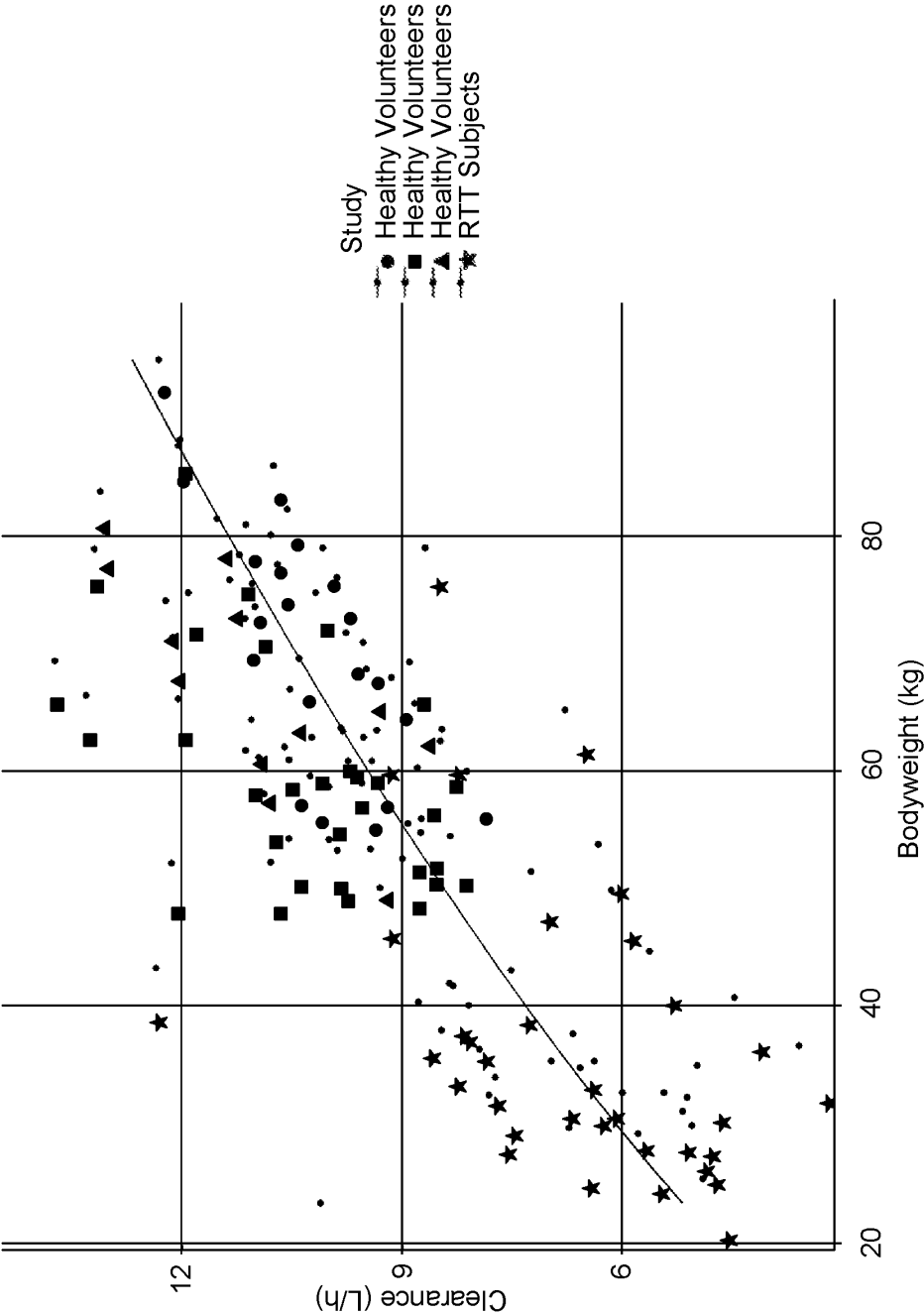


FIGURE 1

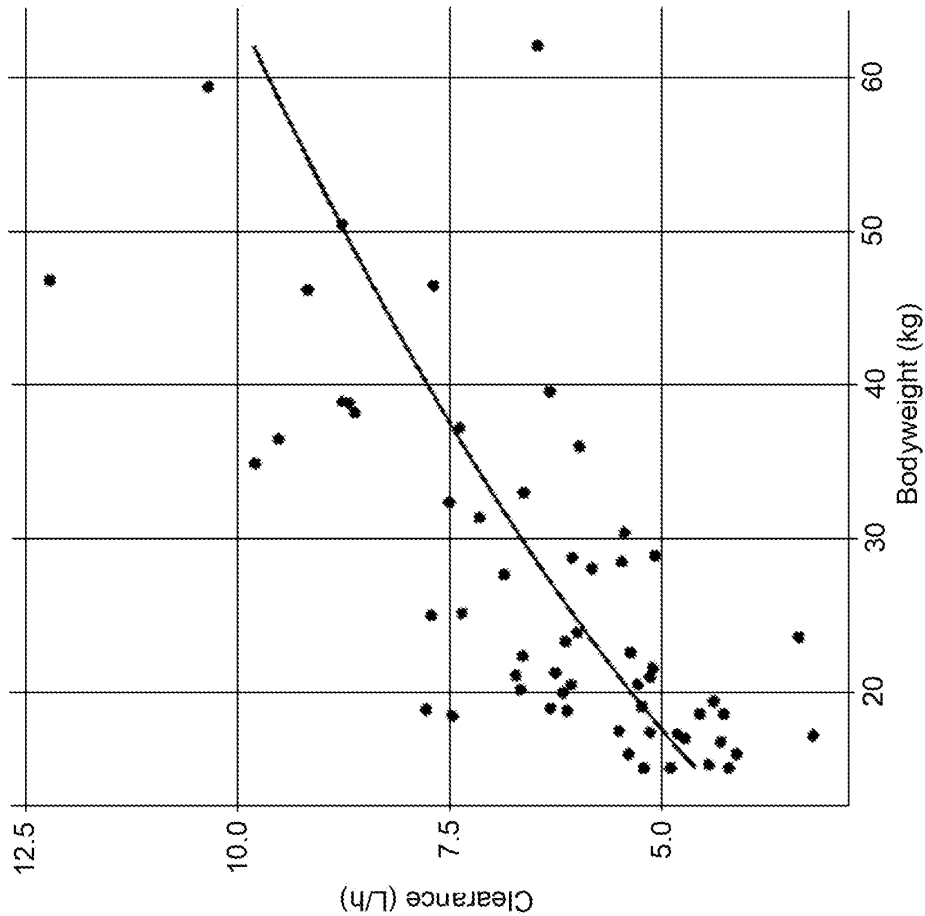


FIGURE 2

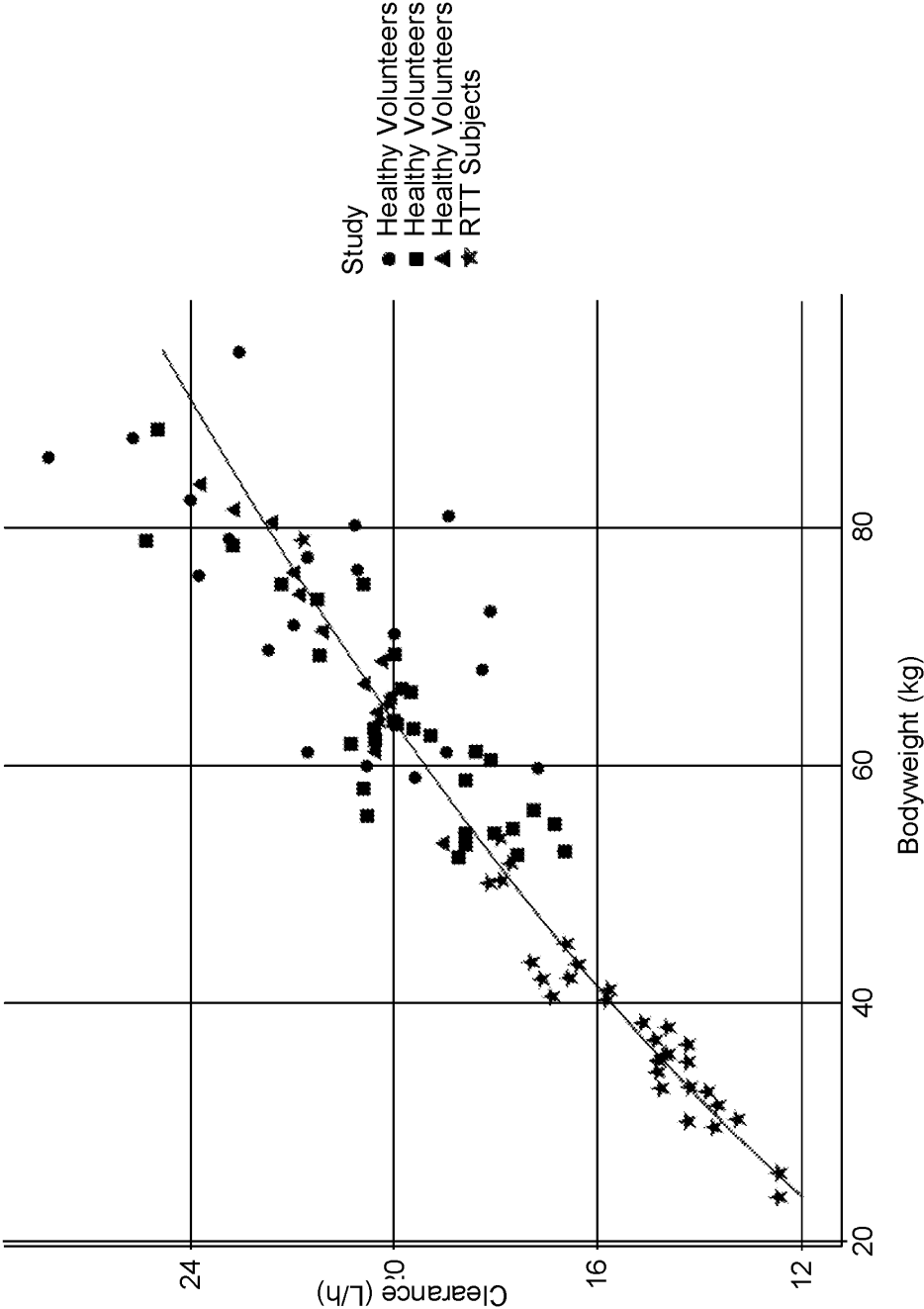


FIGURE 3

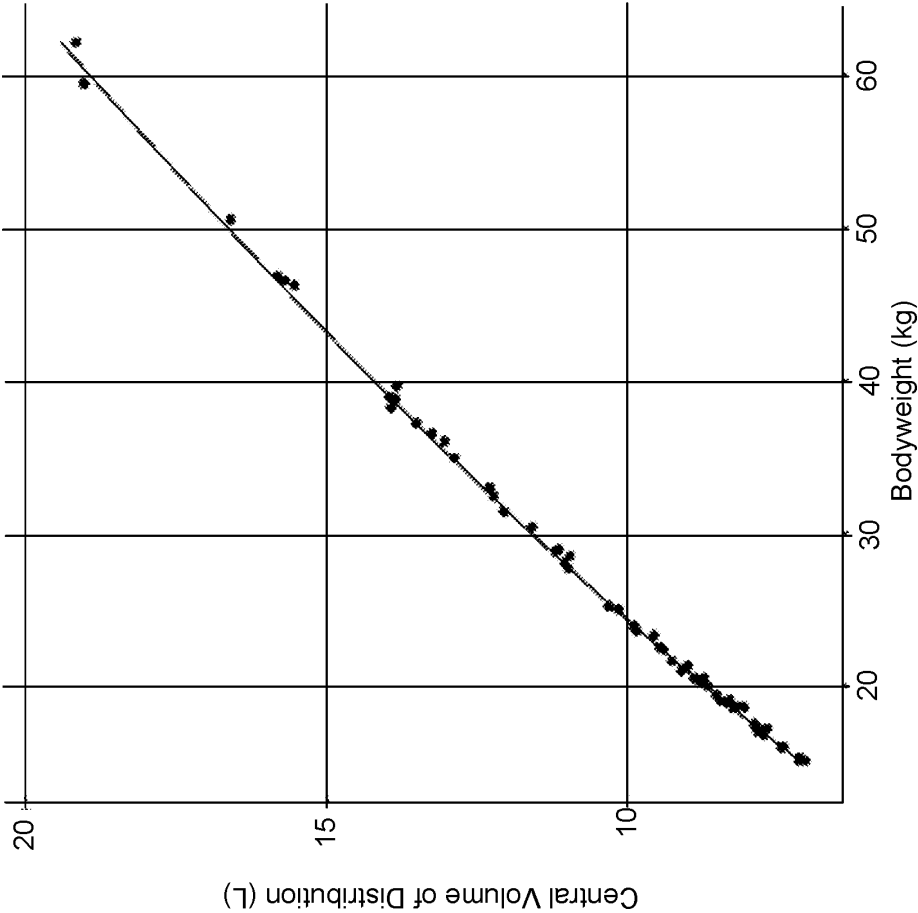


FIGURE 4

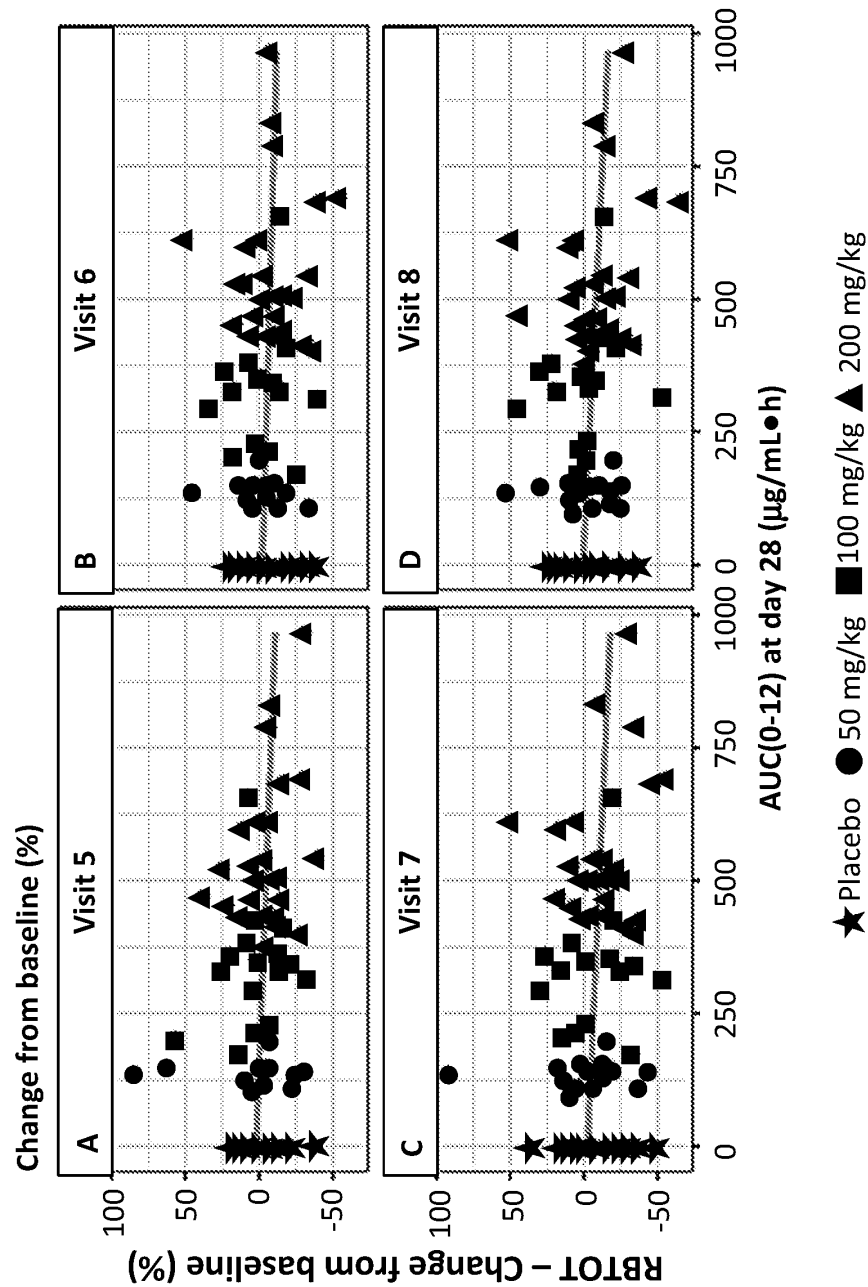


FIGURE 5A-D

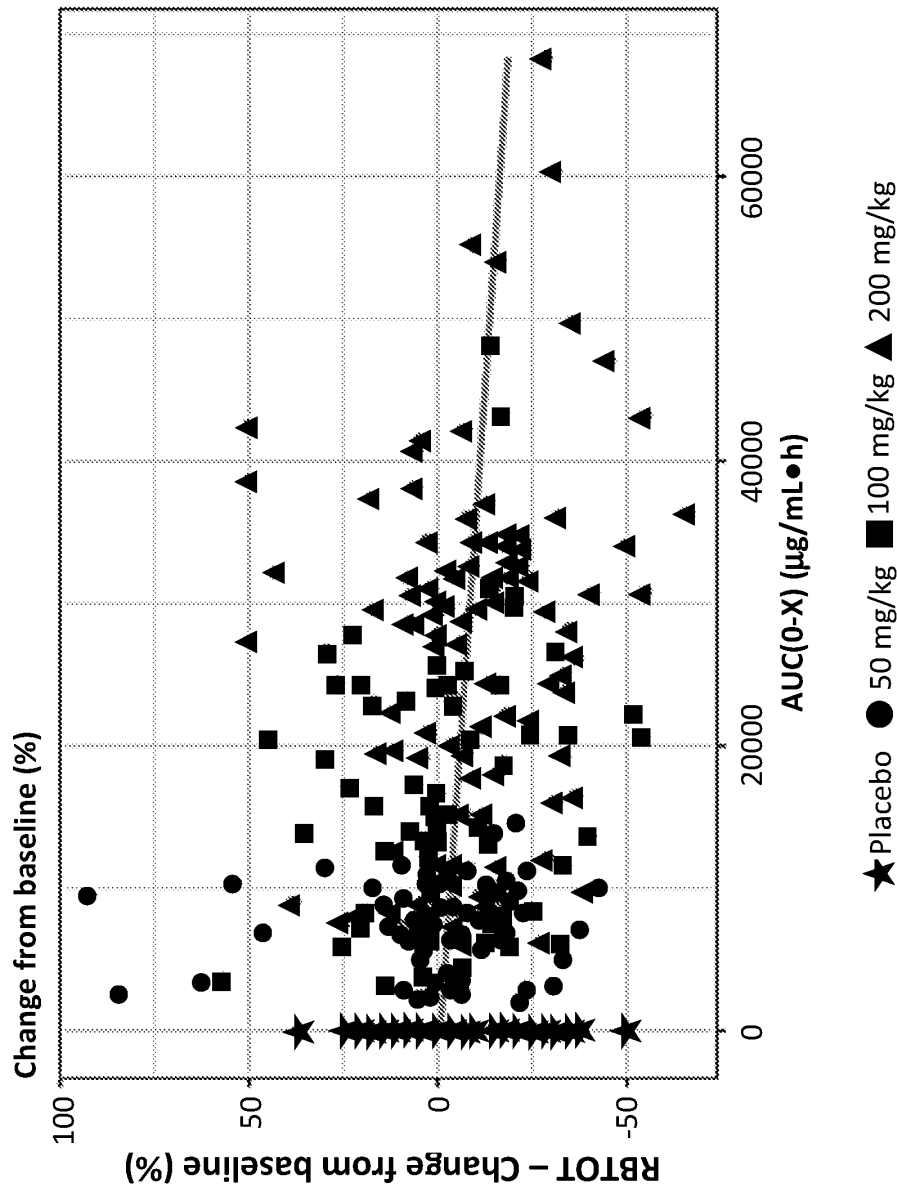


FIGURE 6

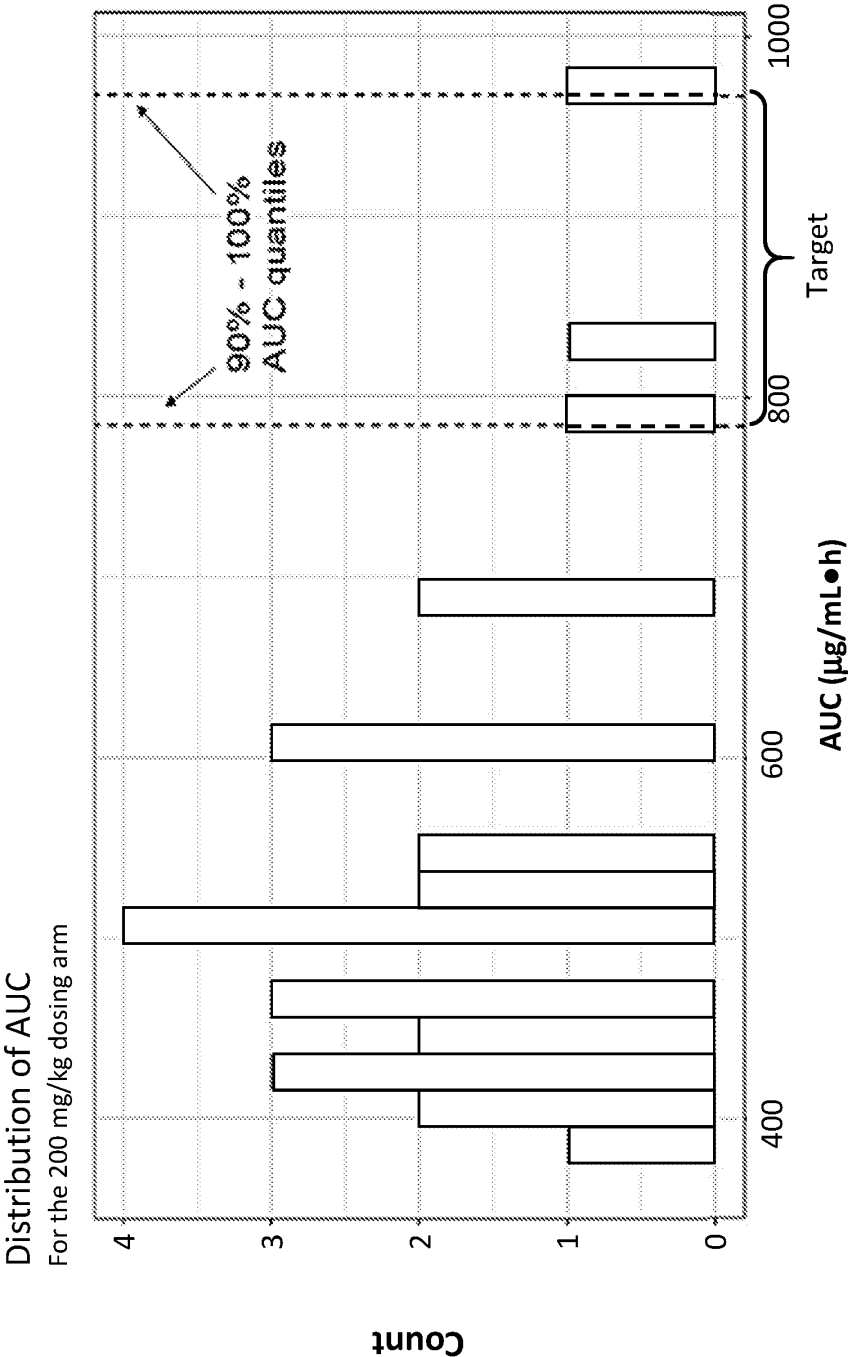


FIGURE 7

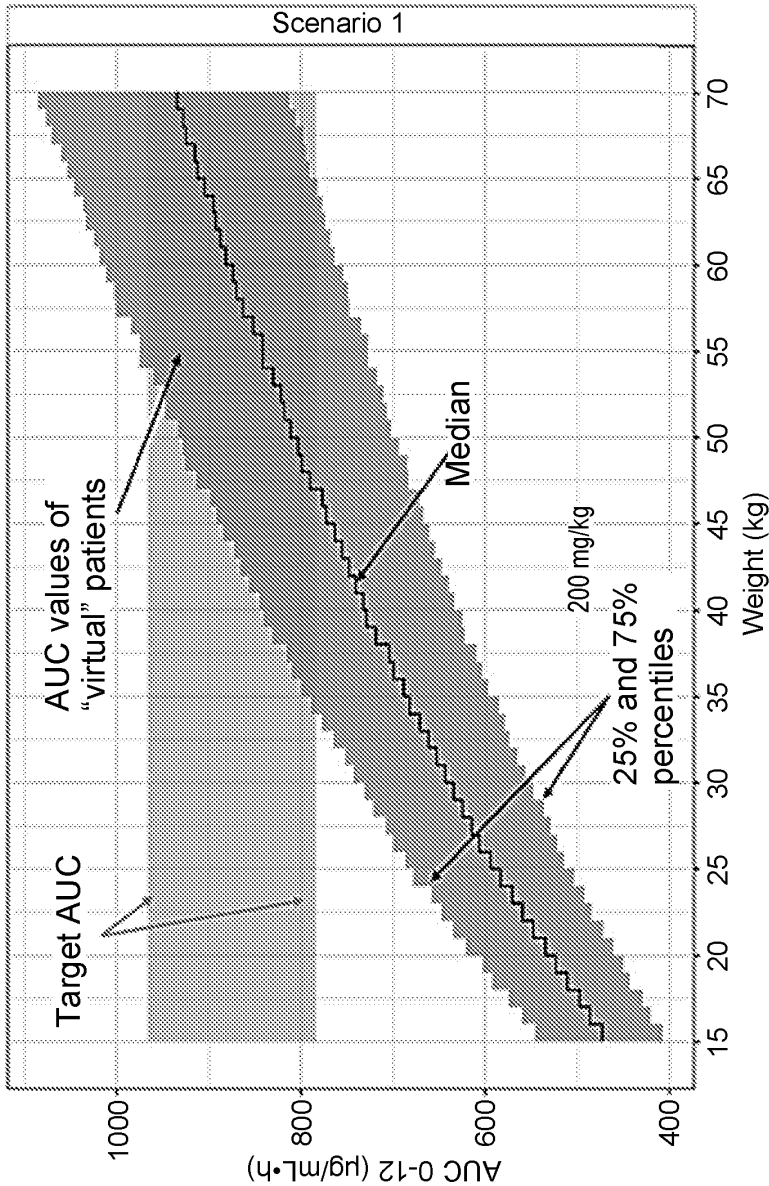


FIGURE 8

	Timing of assessment					Assessed by:	
	SC		Treatment period, Week			Caregiver	Clinician
	SC	BL	SC	BL	12		
Co-primary Endpoints							
RSBQ	☐	☐	☐	☐	☐	☐	
CGI-I			☐	☐	☐		☐
Key Secondary Endpoint							
CSBS-DP-IT		☒	☒	☒	☒	☒	
Other Secondary Endpoints							
CGI-S	☒	☒	☒	☒	☒	☒	☒
ICND including QoL		☒			☒	☒	
RTT-CBI		☒			☒	☒	
RTT-HF		☒		☒	☒		☒
RTT-AMB		☒	☒	☒	☒		☒
RTT-COMC		☒	☒	☒	☒		☒
RTT-VCOM		☒	☒	☒	☒		☒

AMB, Ambulation and Gross Motor Skills; BL, Baseline; CBI, Caregiver Burden Inventory; CGI, Clinical Global Impressions-Improvement; CGI-S, Clinical Global Impressions-Severity; COMC, Ability to Communicate Choices; CSBS-DP-IT, Communication and Symbolic Behavior Scales Developmental Profile™ Infant-Toddler Checklist; HF, Hand Function; ICND, Impact of Childhood Neurologic Disability Scale; QoL, quality of life; RSBQ, Rett Syndrome Behavior Questionnaire; SC, screening; VCOM, Verbal Communication

FIGURE 9

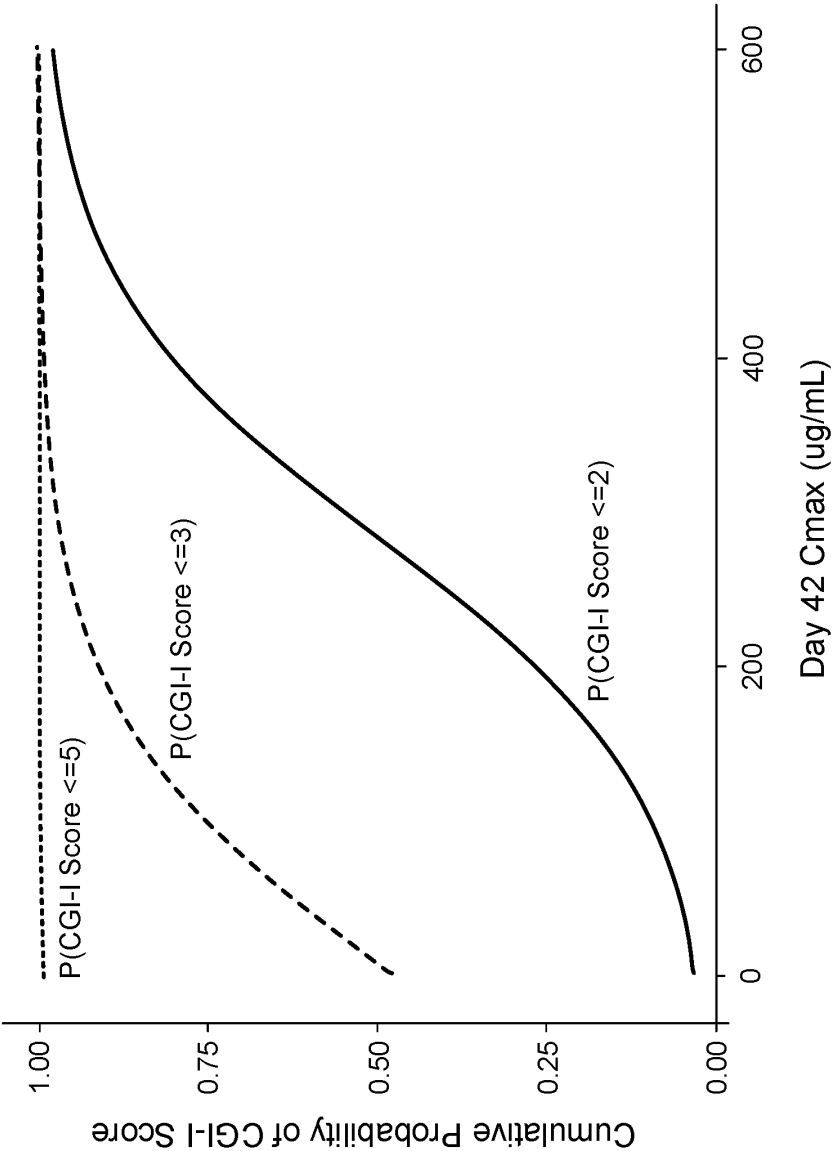


FIGURE 10

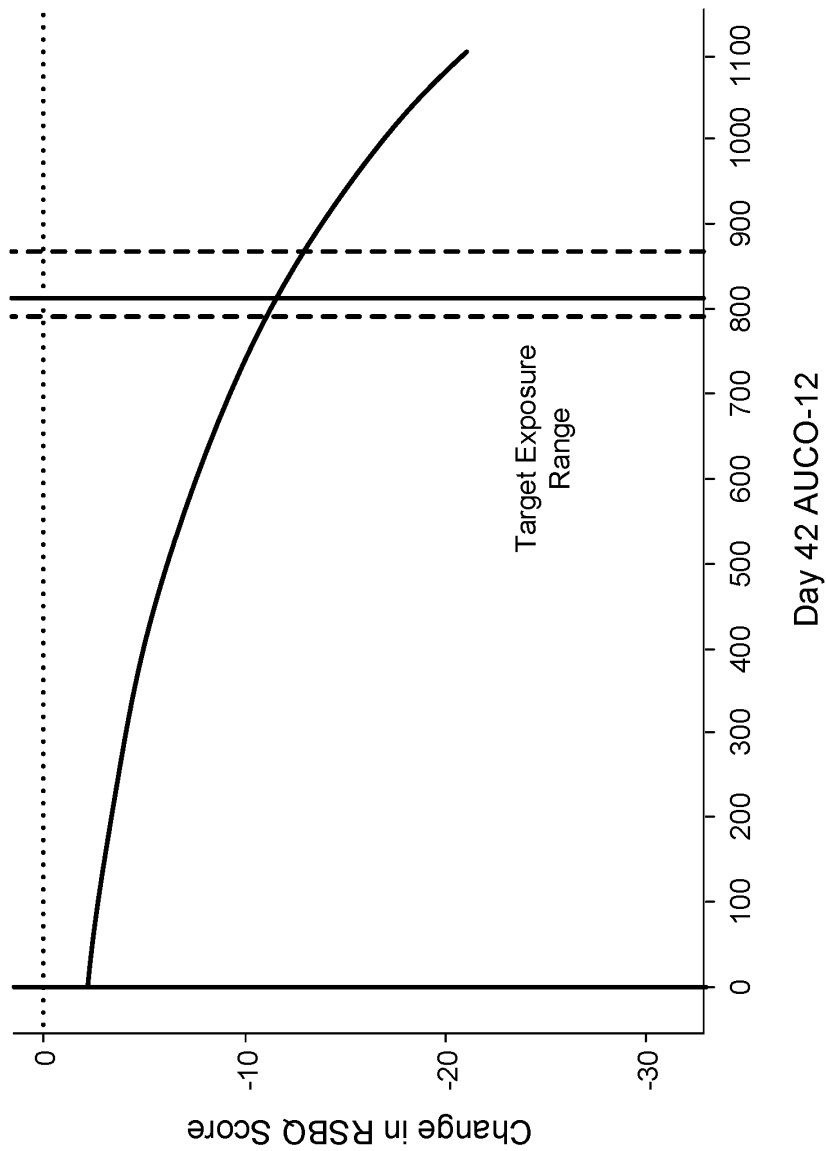


FIGURE 11

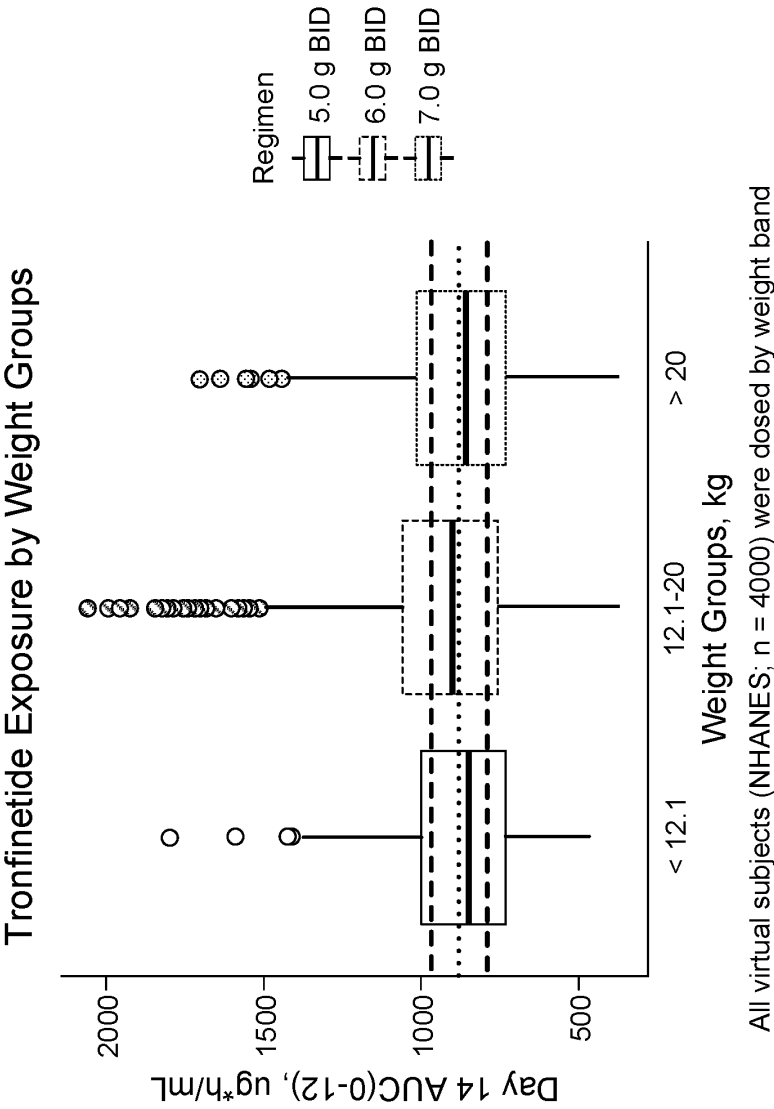


FIGURE 12