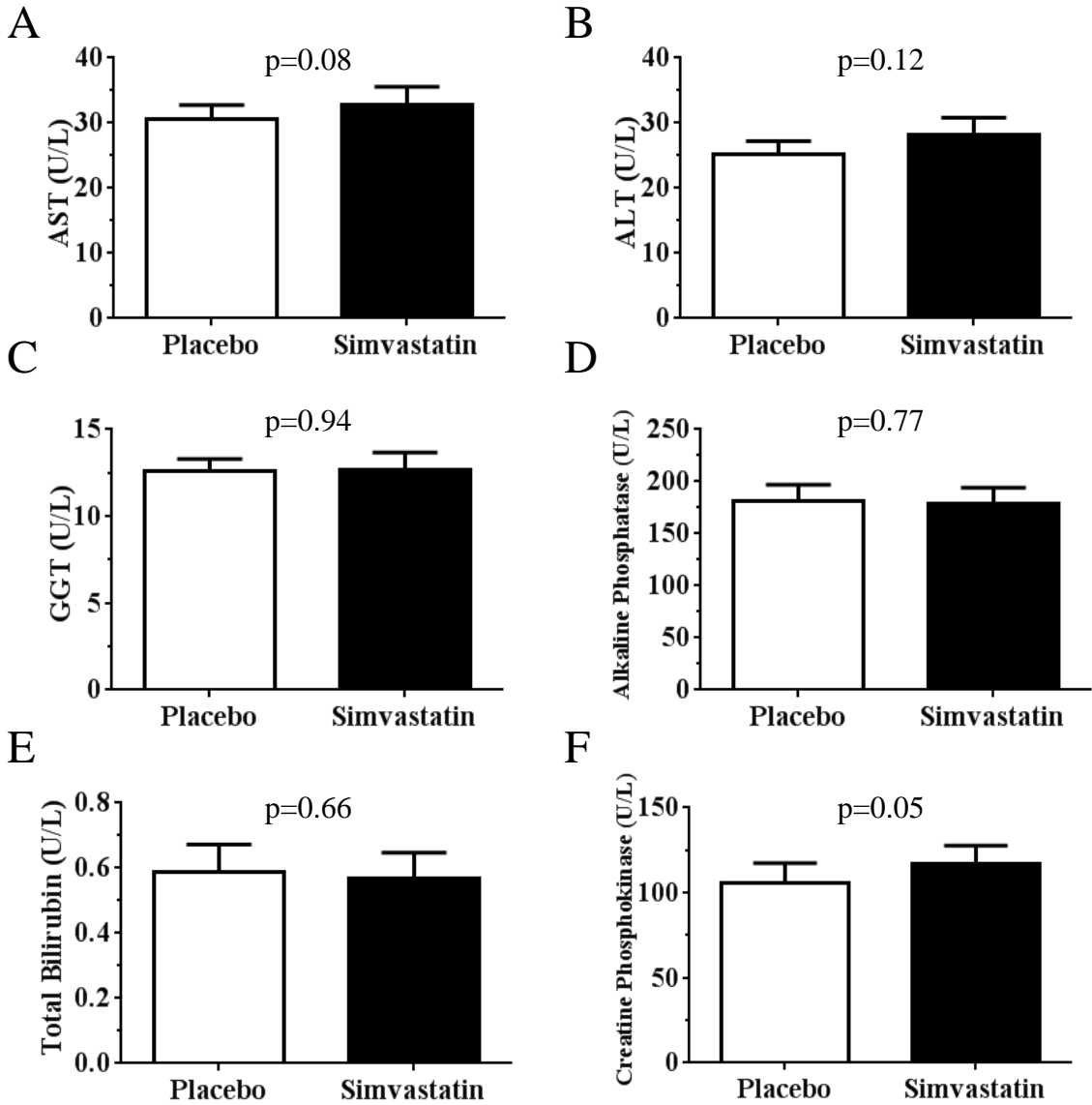
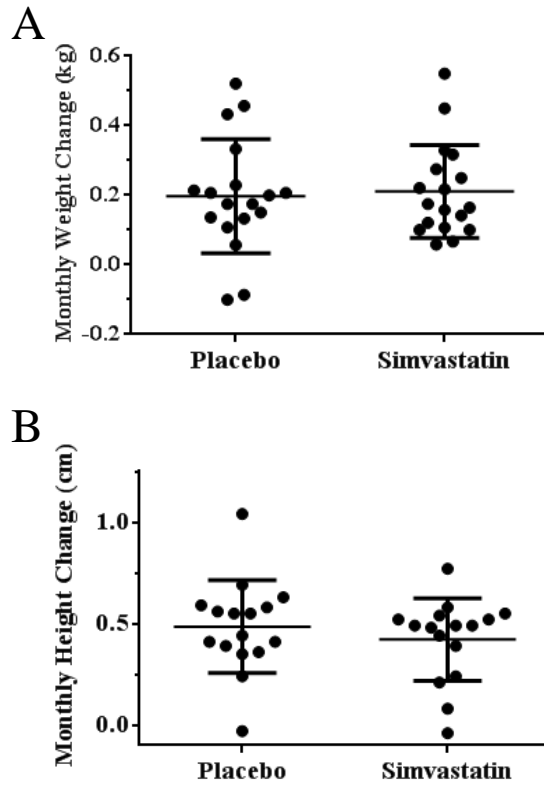


Supplemental Figure 1



Supplemental Figure 2



Supplemental Table 1: Randomization Demographics

	Cohort		
	<u>Placebo First</u>	<u>Simvastin First</u>	
Number of subjects	12	10	
Gender (Male/Female)	7/5	6/4	p=1.0 ^b
Age (years)	8.6 ± 1.3 ^a	7.8 ± 0.7	p=0.60
Severity Score	13.1 ± 2.0	13.4 ± 2.2	p=0.91
Residual cholesterol synthesis	0.41 ± 0.06	0.33 ± 0.04	p=0.31
DHC/Total Sterol Ratio			
Plasma (%)	9.1 ± 1.9	9.1 ± 2.9	p=0.99
CSF (%)	7.3 ± 1.2	5.9 ± 1.4	p=0.47

^aMean ± SEM, unpaired t-test

^bFischer's exact test

Supplemental Table 2. Adverse Events

<u>Subject</u>	<u>Phase</u>	<u>Issue</u>
S03	Simv	Urinary tract infection
	Simv	Waking up attributed to evening dose and abdominal gas
	Placebo	Episode of limping
	OLE	H1N1 infection
	OLE	Sun sensitivity
S04	Placebo	Muscle pain and increasing CPK levels*
S05	Baseline	Gastroenteritis and leg pain after lumbar puncture*
S06	Simv	Post spinal tap headache*
	OLE	Transient elevation of total bilirubin
S08	Placebo	Diagnosed with glaucoma*
	Simv	Shingles
	Simv	Dengue fever
S10	Placebo	Flushing episode related to CRH infusion
	OLE	Increased aggression and self-injurious behavior
S11	Placebo	Periorbital cellulitis*
	Simv	Diarrhea
S12	Placebo	Diarrhea
	Placebo	Scarlet fever
	Placebo	Episode of jerking and dazed appearance one day after starting this phase.
	OLE	H1N1 infection
	OLE	Two crying episodes and refusal to walk
S13	Placebo	Emesis two days after starting this phase
	OLE	Episode of abdominal pain
S16	Placebo	Diarrhea
	Simv	Diarrhea
	Simv	Corneal abrasion after ophthalmological evaluation
S17	Simv	Salmonella gastroenteritis
S19	Simv	Parvovirus B19 infection
S20	Simv	Pneumonia
S21	Placebo	Dx with seizure disorder
	Simv	H1N1 infection
S23	Baseline	Hematuria
	Simv	Corneal abrasion after ophthalmological evaluation

*SAE