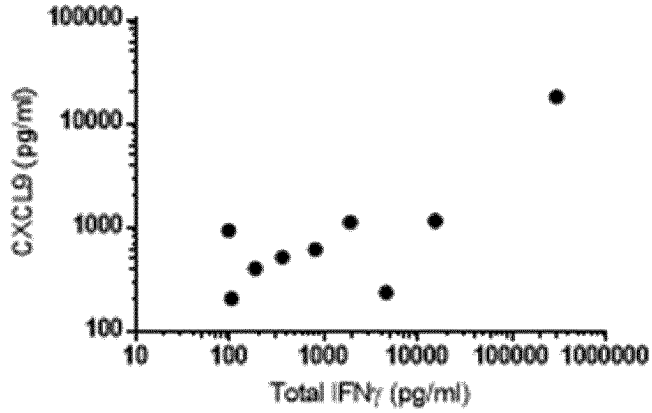
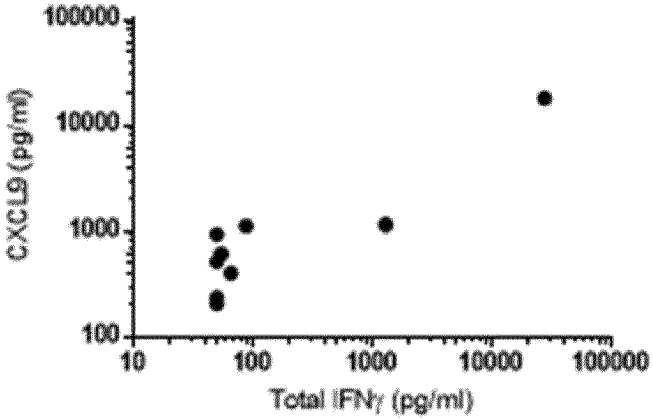
FIGURE 1



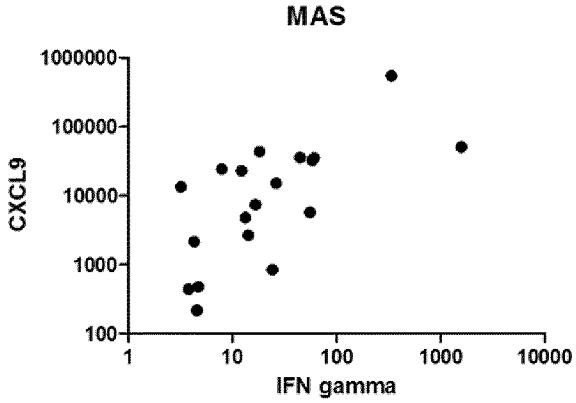
Number of XY pairs	9
Spearman r	0.5667
p-value (two-tailed)	0.1206

FIGURE 2



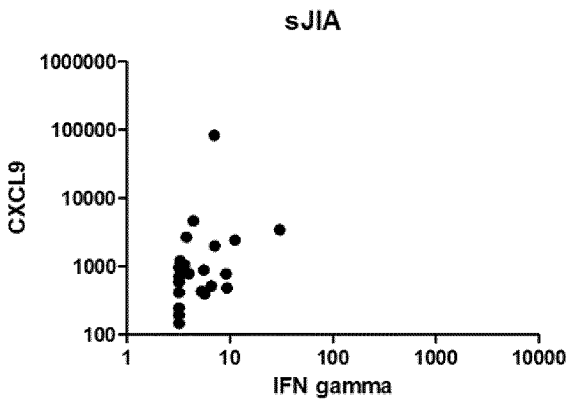
Number of XY pairs	9
Spearman r	0.7746
p-value (two-tailed)	0.0196

FIGURE 3A



Number of XY Pairs	19
Spearman r	0,6930
95% confidence interval	0.3356 - 0.8760
P value (two-tailed)	0,0010

FIGURE 3B



Number of XY Pairs	24
Spearman r	0,4469
95% confidence interval	0.04039 - 0.7265
P value (two-tailed)	0,0286

FIGURE 4A-1

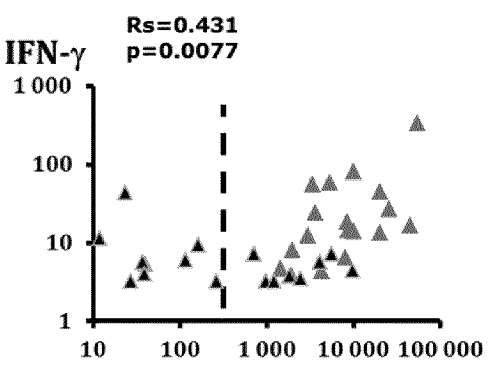
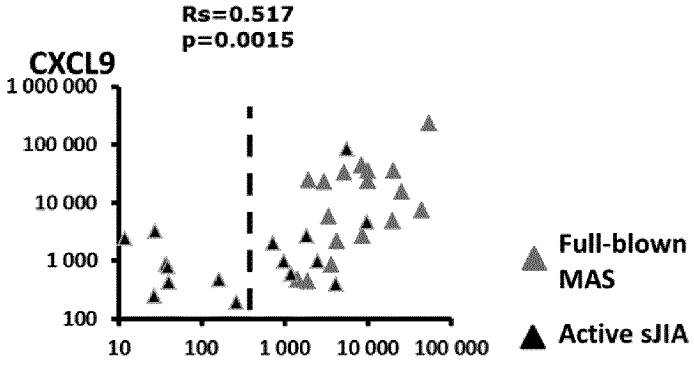


FIGURE 4A-2



Ferritin (ng/ml)

FIGURE 4B-1

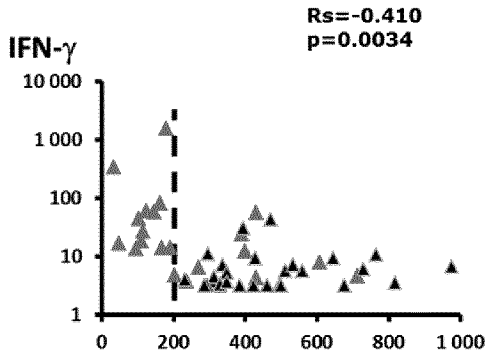
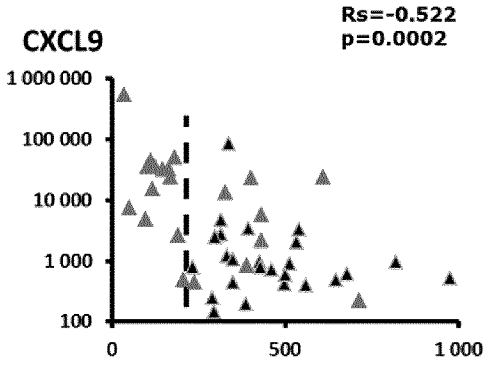


FIGURE 4B-2



PLT (x10^3/ml)

FIGURE 4C-1

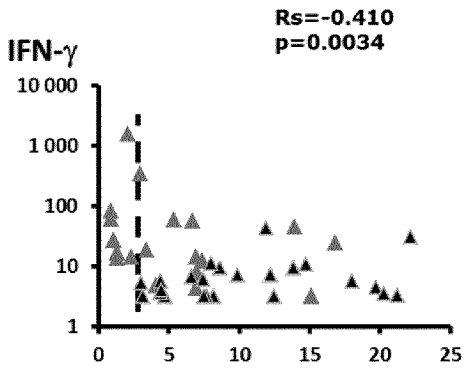
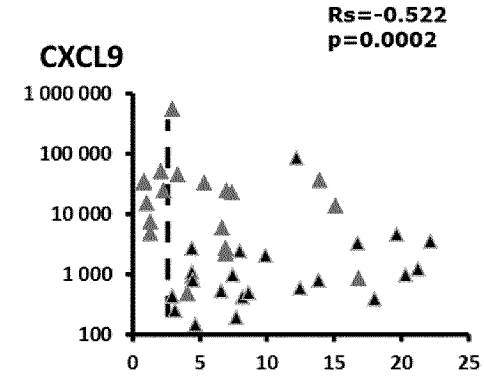


FIGURE 4C-2



Neu (x10^3/ml)

FIGURE 4D-1

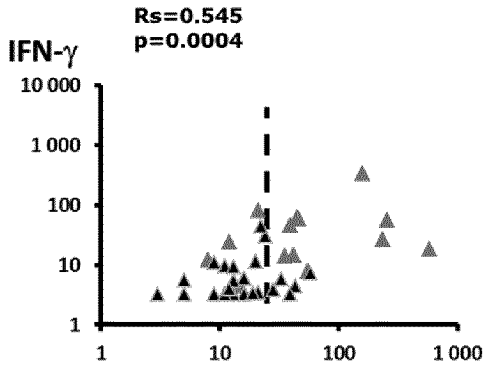
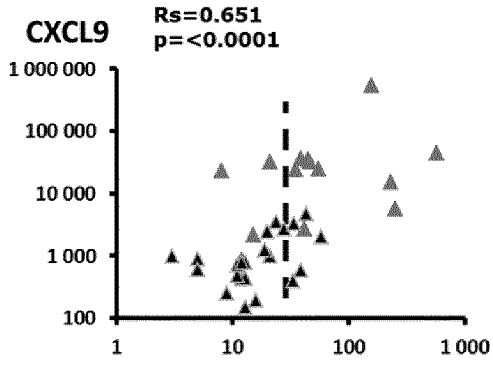


FIGURE 4D-2



ALT (IU/ml)

FIGURE 5

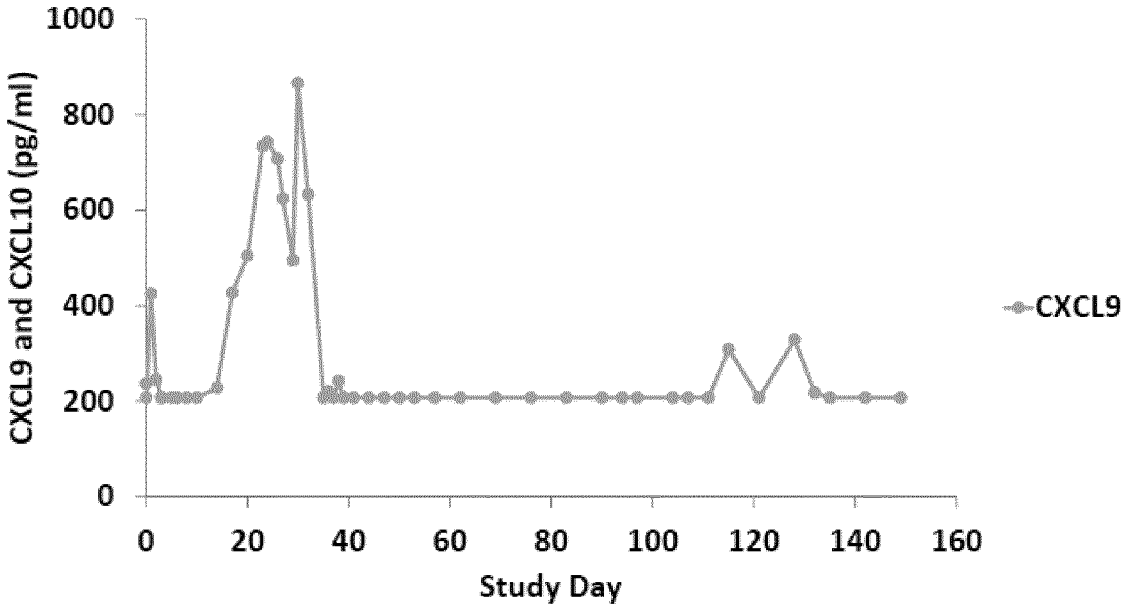


FIGURE 6

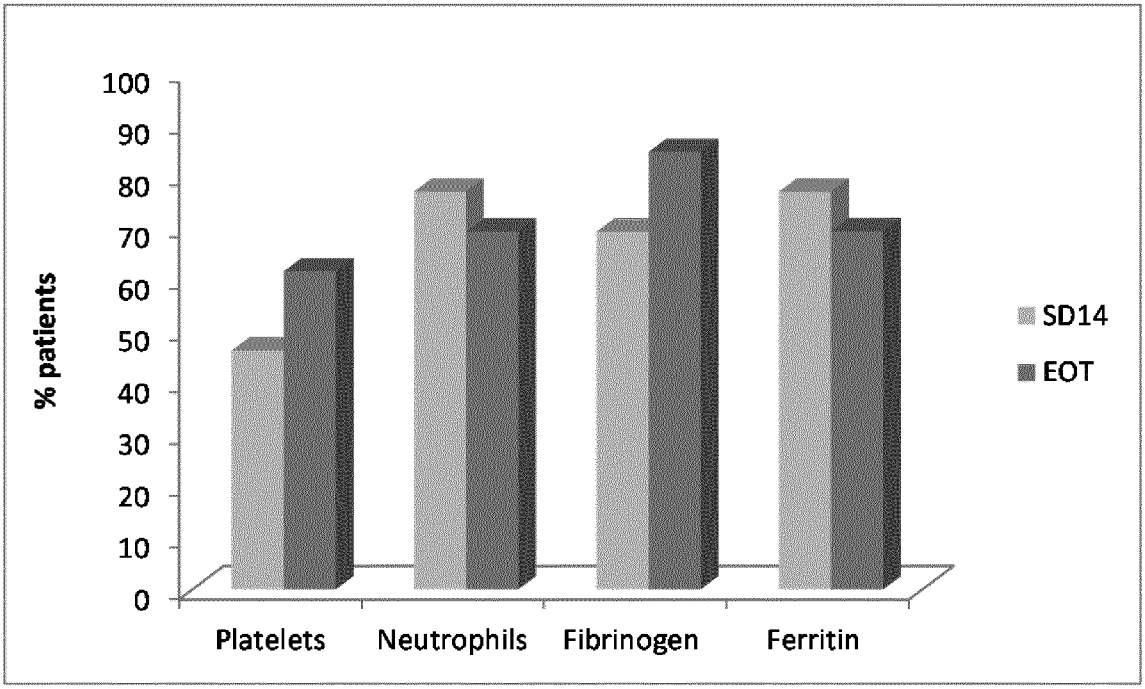


FIGURE 7A

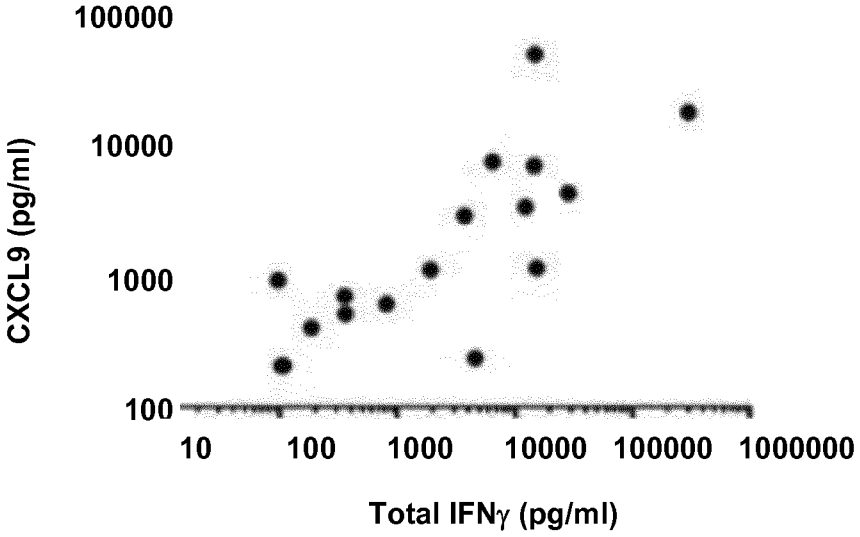
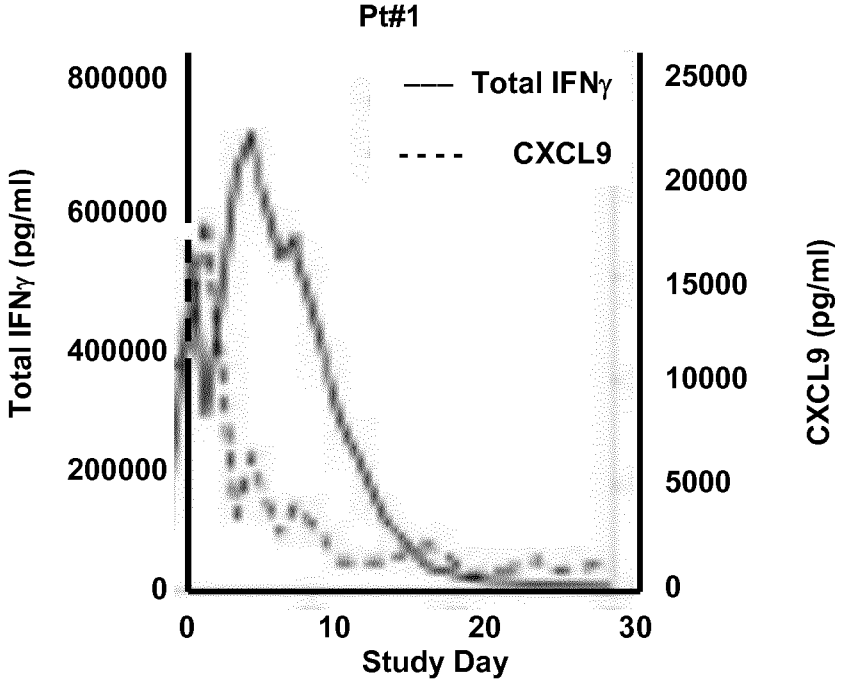


FIGURE 7B



Number of XY pairs	17
Spearman r	0.7885
95% CI	0.4839 – 0.9227
p-value (two tailed)	0.0003

FIGURE 8A

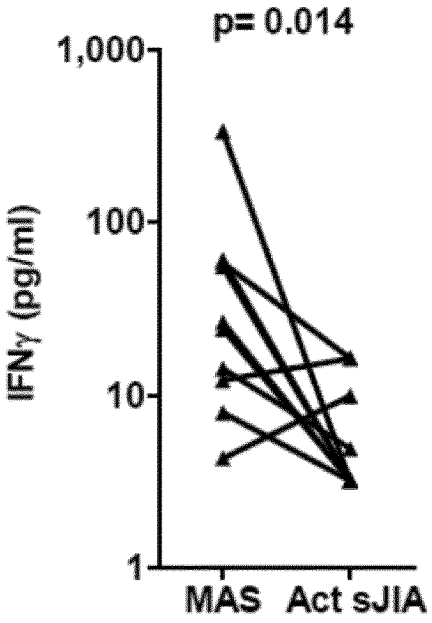


FIGURE 8B

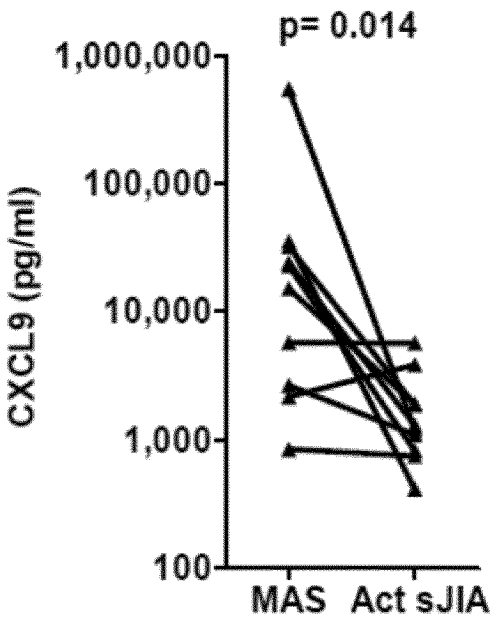


FIGURE 8C

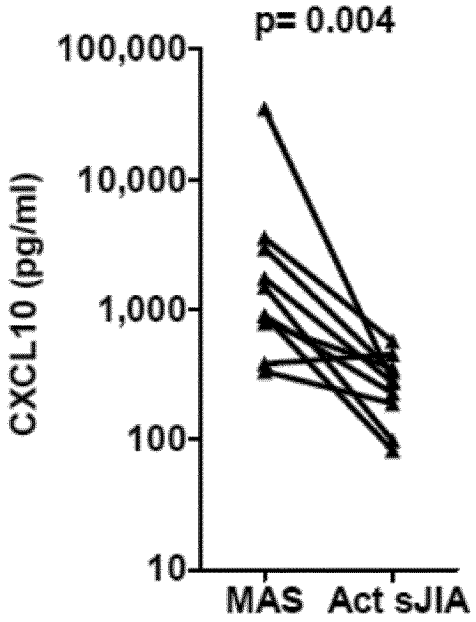


FIGURE 8D

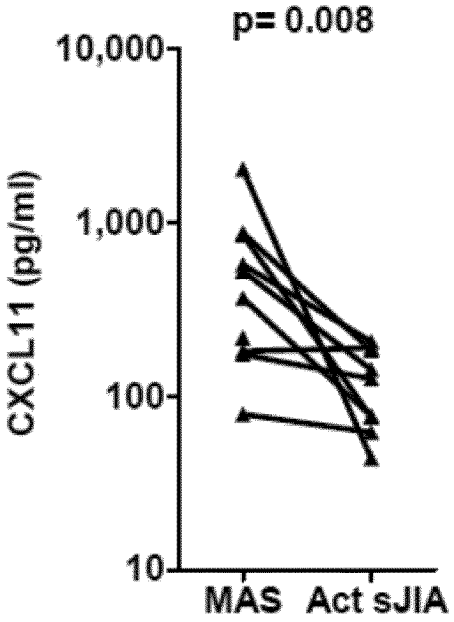


FIGURE 9A

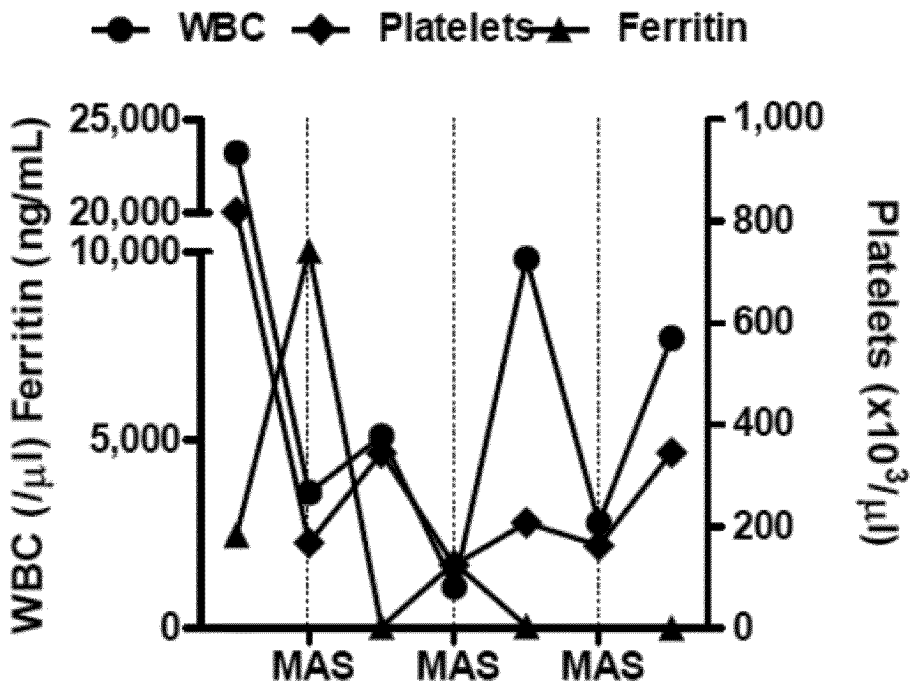


FIGURE 9B

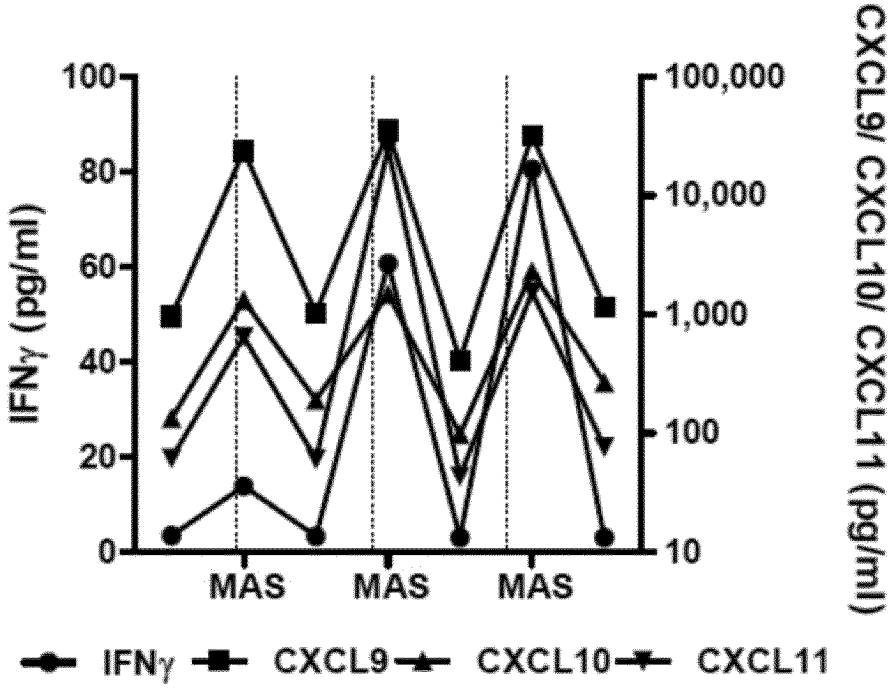


FIGURE 10A

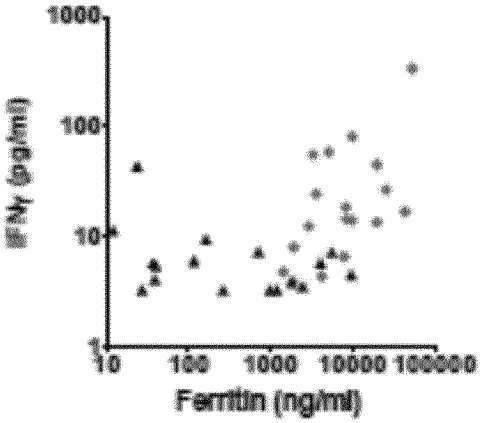


FIGURE 10B

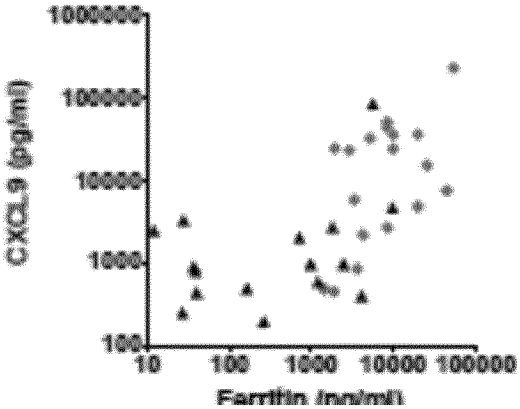


FIGURE 10C

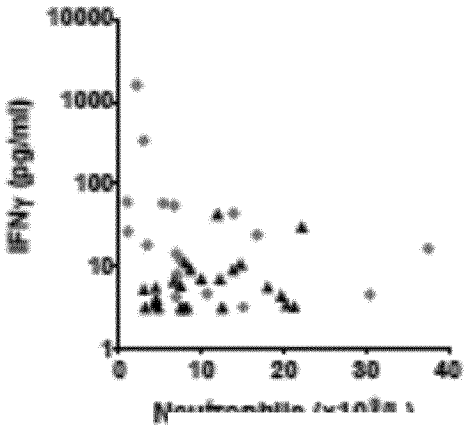


FIGURE 10D

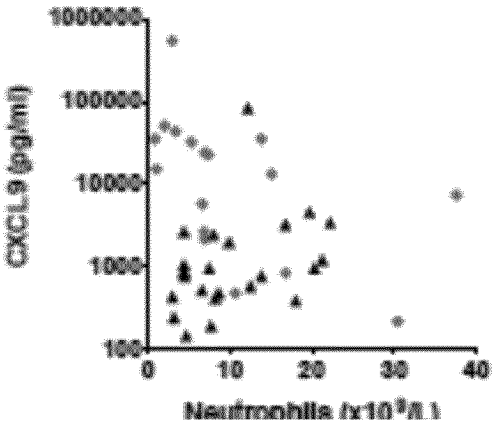


FIGURE 10E

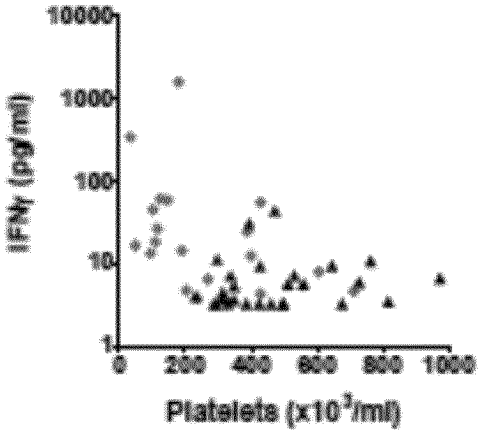


FIGURE 10F

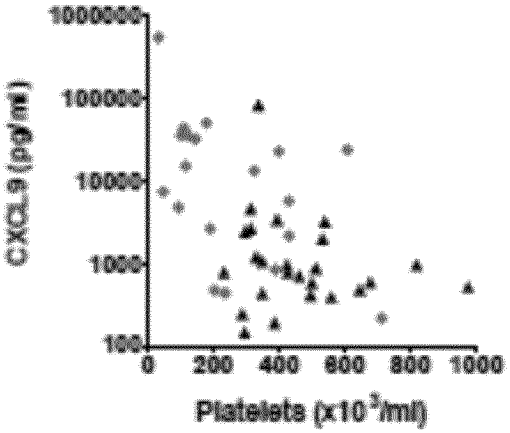


FIGURE 10G

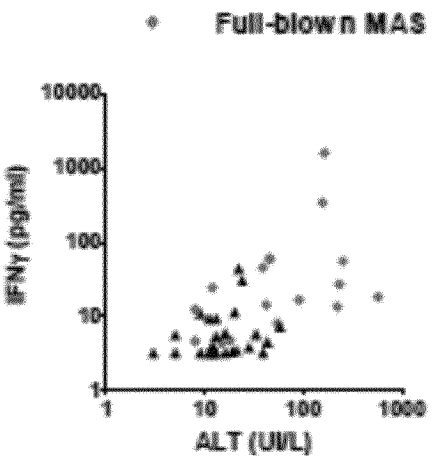


FIGURE 10H

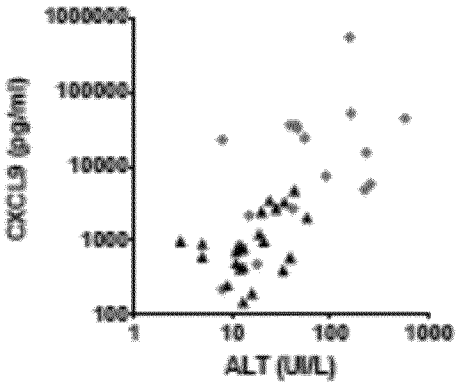


FIGURE 10I

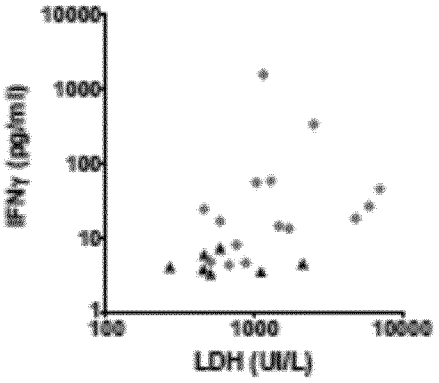


FIGURE 10J

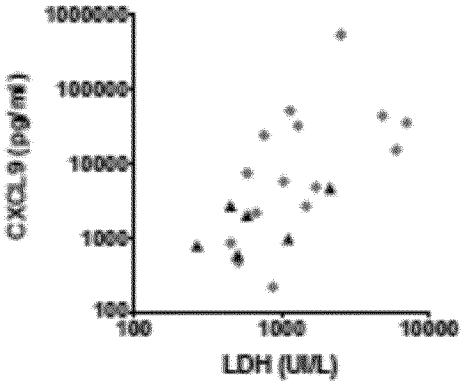


FIGURE 11A

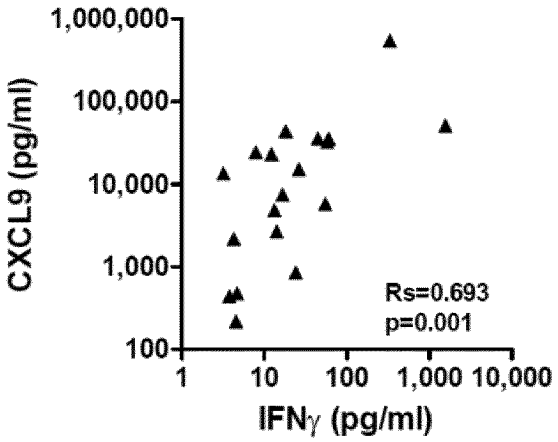


FIGURE 11B

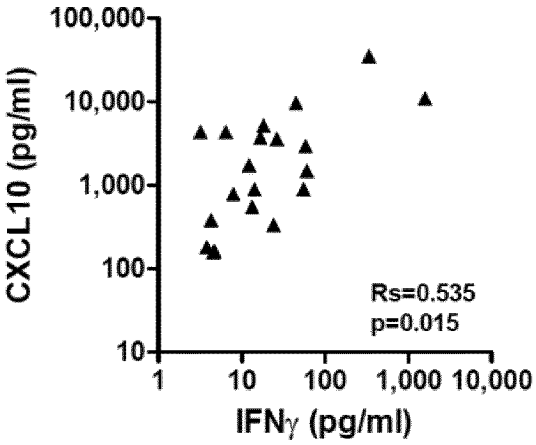


FIGURE 11C

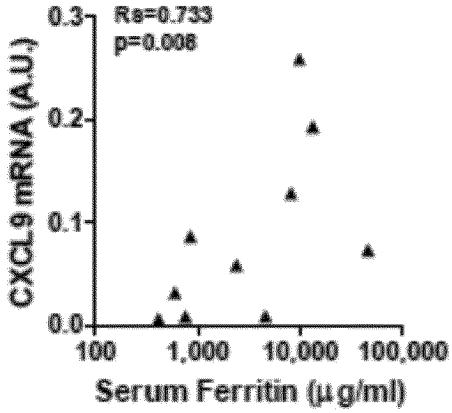


FIGURE 11D

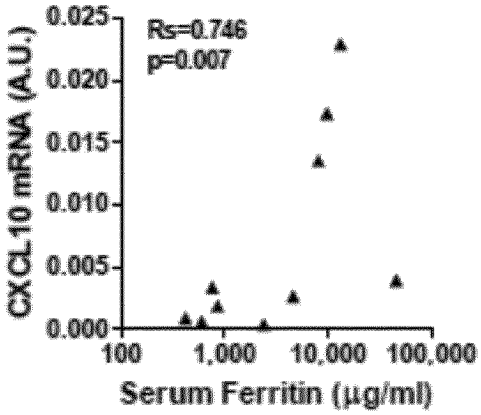


FIGURE 11E

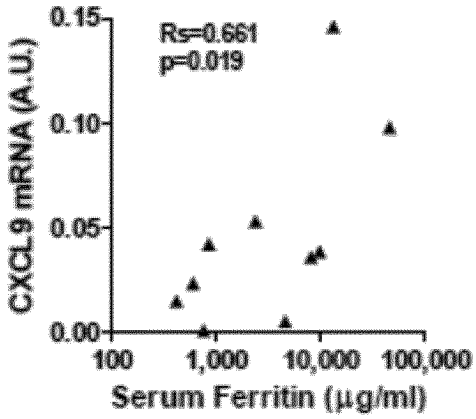


FIGURE 11F

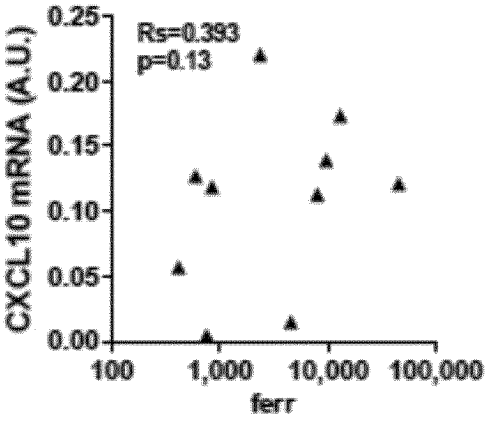


FIGURE 12

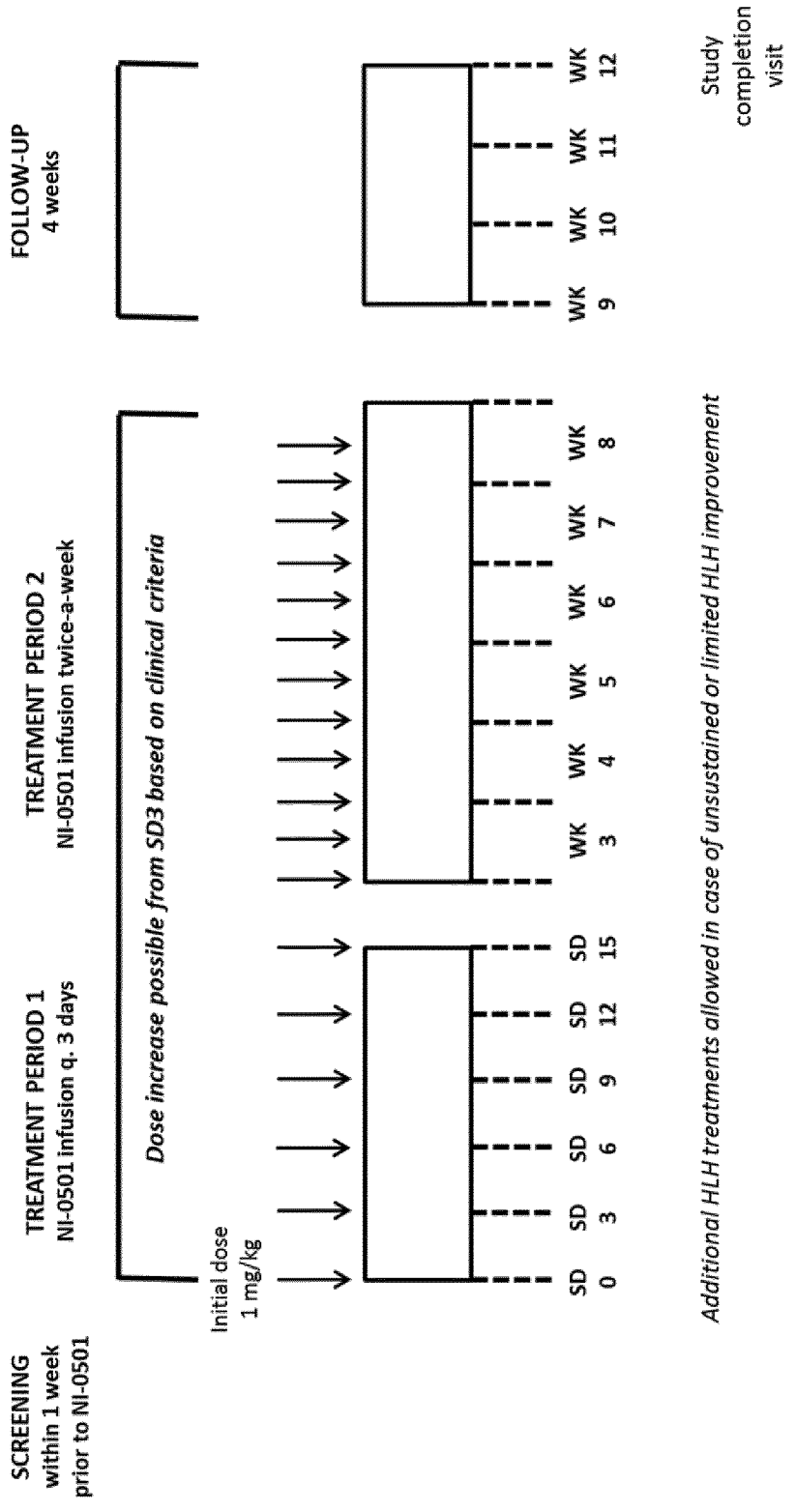

SCREENING
within 1 week
prior to NI-0501
TREATMENT PERIOD 1
NI-0501 infusion q. 3 days
TREATMENT PERIOD 2
NI-0501 infusion twice-a-week
FOLLOW-UP
4 weeks
Dose increase possible from SD3 based on clinical criteria
Initial dose
1 mg/kg
SD 0
SD 3
SD 6
SD 9
SD 12
SD 15
WK 3
WK 4
WK 5
WK 6
WK 7
WK 8
WK 9
WK 10
WK 11
WK 12
Study completion visit
Additional HLH treatments allowed in case of unsustained or limited HLH improvement

FIGURE 13A

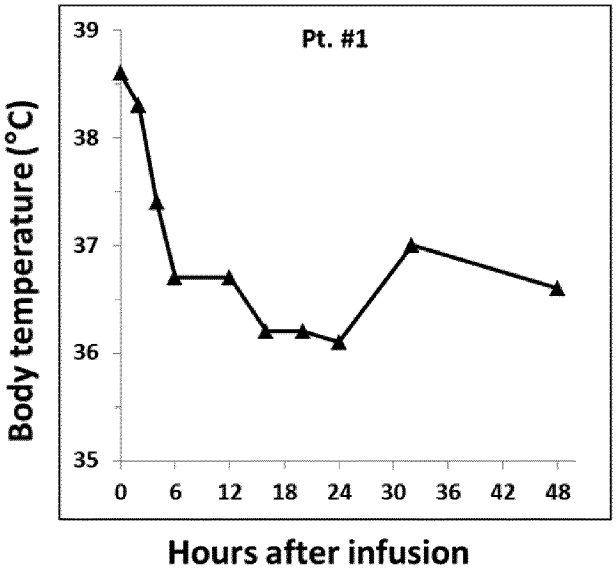


FIGURE 13B

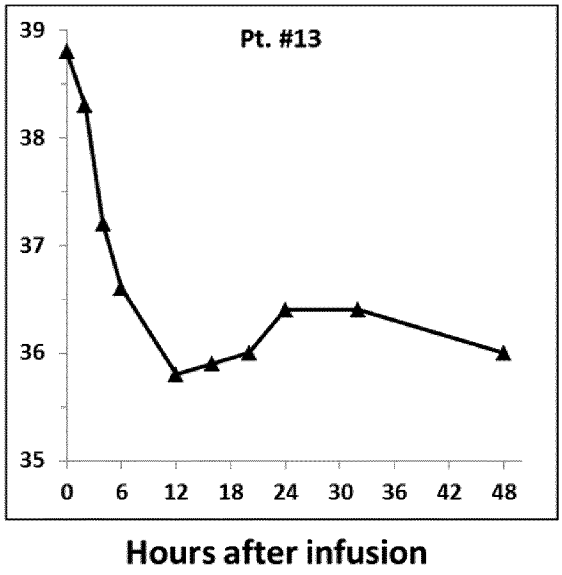
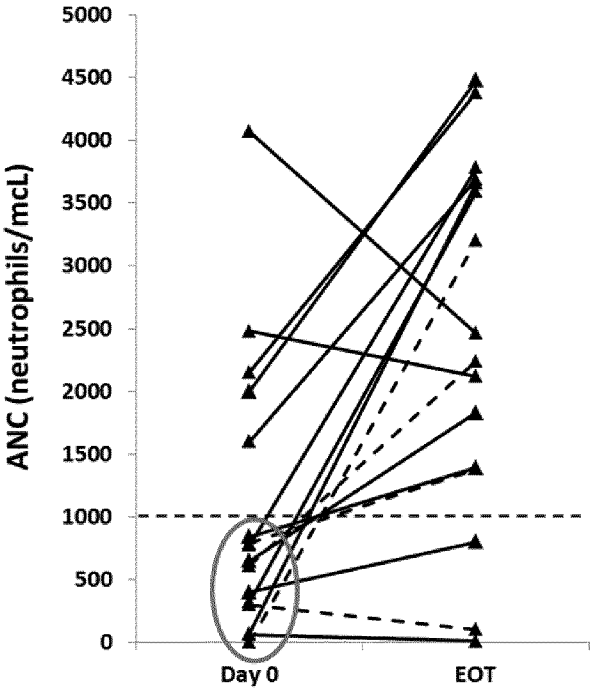


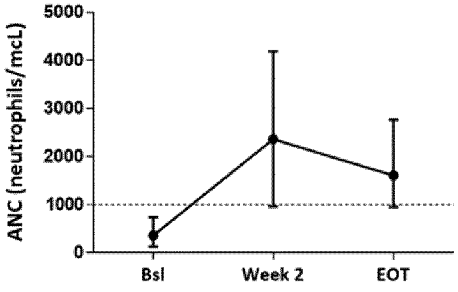
FIGURE 14



EOT = end of treatment with NI-0501
Solid lines = pts. with favorable response
Dashed lines = pts. with insufficient responses

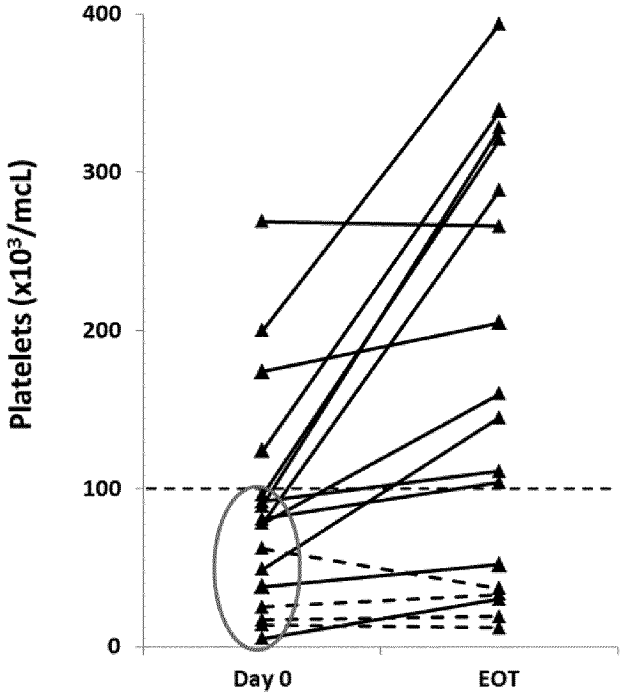
No. of patients with ANC < 1000/mcl	
Baseline (SD0)	End of Treatment
11/18	3/18
p < 0.001 (*)	

* One-sided single proportion test



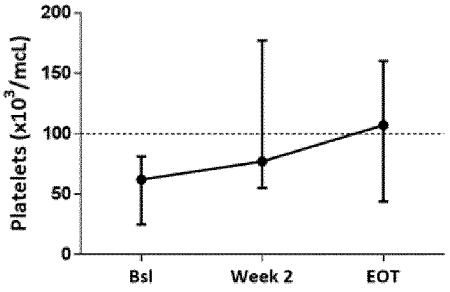
Median (Q1;Q3)

FIGURE 15



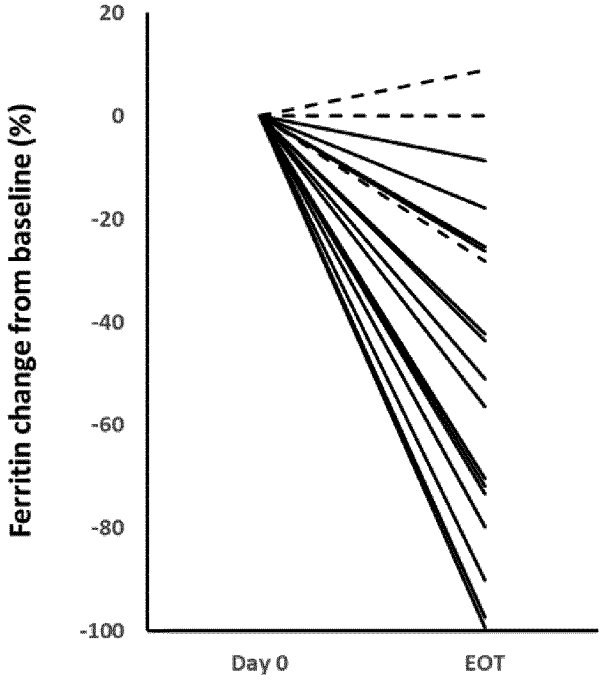
No. of patients with PLT < 100 x10³/mcl	
Baseline (SD0)	End of Treatment
13/18	6/18
p < 0.001 (*)	

* One-sided single proportion test



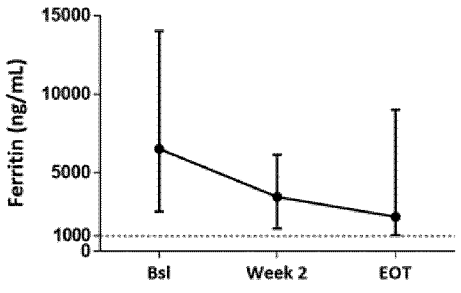
Median (Q1;Q3)

FIGURE 16



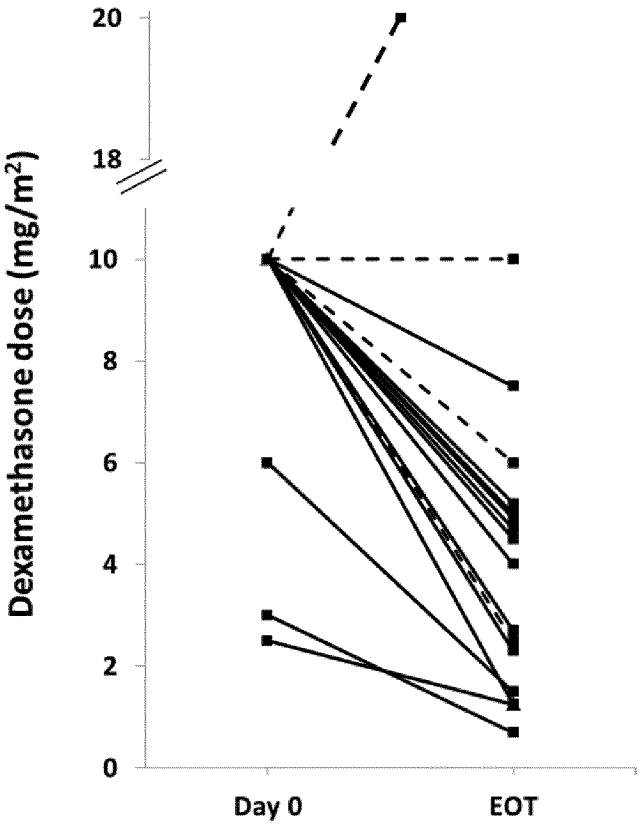
Median serum levels (pts. with baseline > 1000 ng/ml; n=13)	
Baseline (SD0)	End of Treatment
5880 ng/ml	1909 ng/ml
p = 0.006 (*)	

* Paired t-test on log transformed data



Median (Q1;Q3)

FIGURE 17



Median dose	
Baseline (SD0)	End of Treatment
10.0 mg/m²	4.5 mg/m²
p = 0.02 (*)	
No. of pts with tapering ≥ 50%:	
13/17^	

* two-sided Wilcoxon Signed rank test
^ data for 1 patient not yet available

FIGURE 18

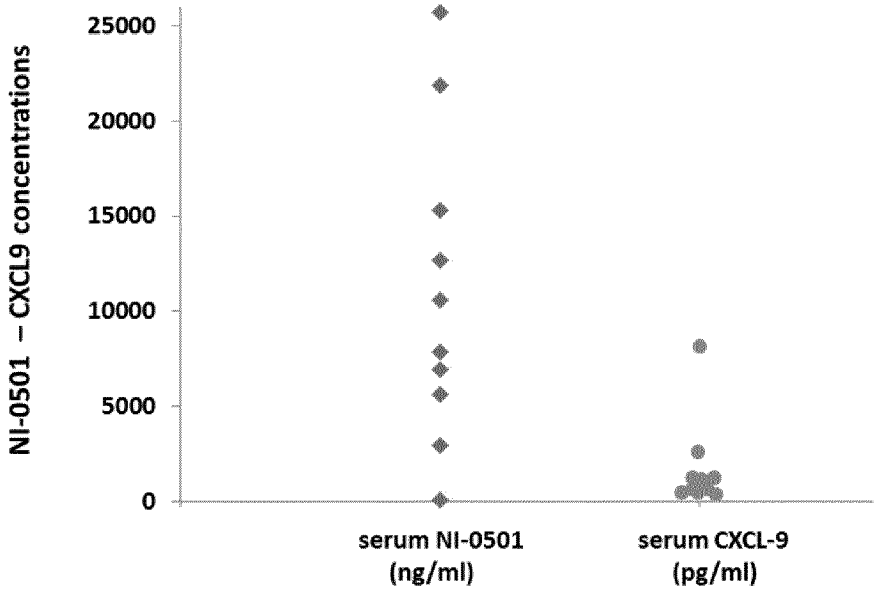
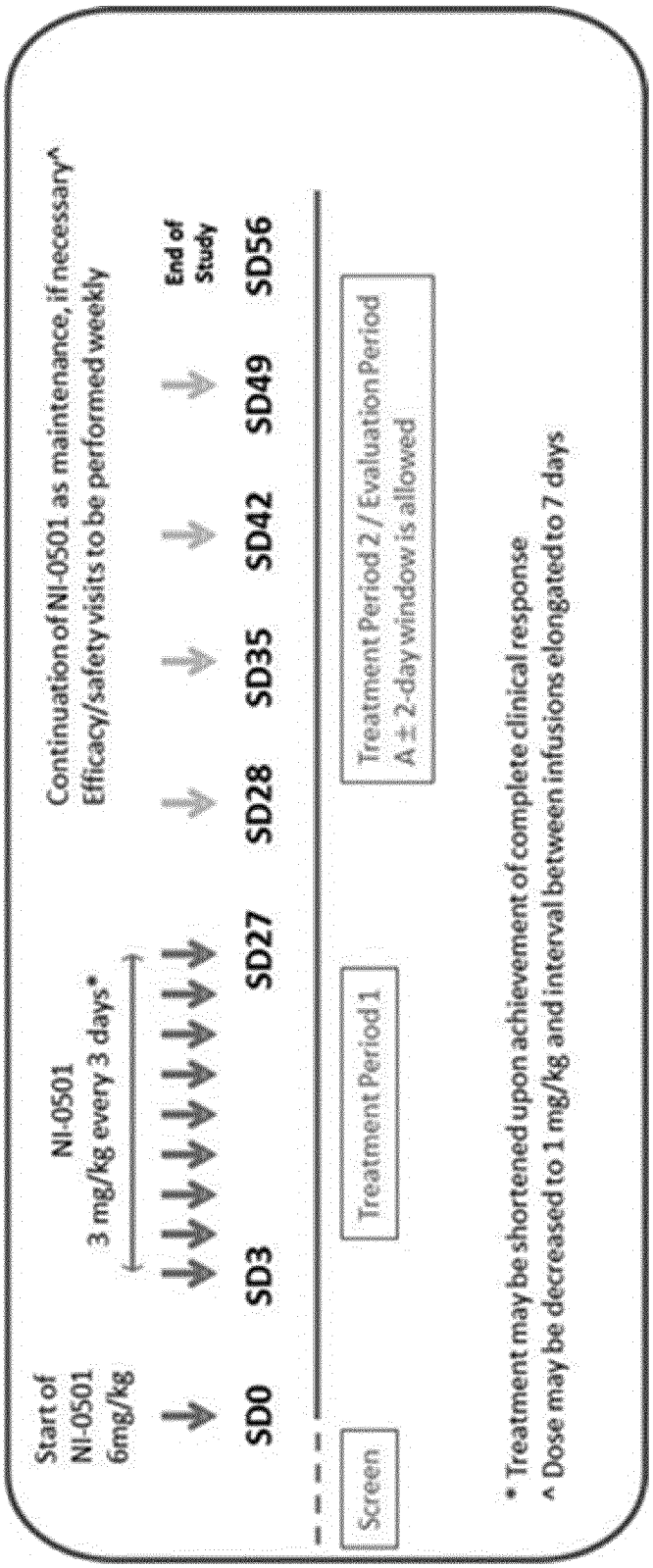


FIGURE 19


Start of NI-0501 6mg/kg
NI-0501 3 mg/kg every 3 days*
Continuation of NI-0501 as maintenance, if necessary^ Efficacy/safety visits to be performed weekly
SD0
SD3
SD27
SD28
SD35
SD42
SD49
SD56
End of Study
Screen
Treatment Period 1
Treatment Period 2 / Evaluation Period A ± 2-day window is allowed
* Treatment may be shortened upon achievement of complete clinical response
^ Dose may be decreased to 1 mg/kg and interval between infusions elongated to 7 days