FIGURE 1

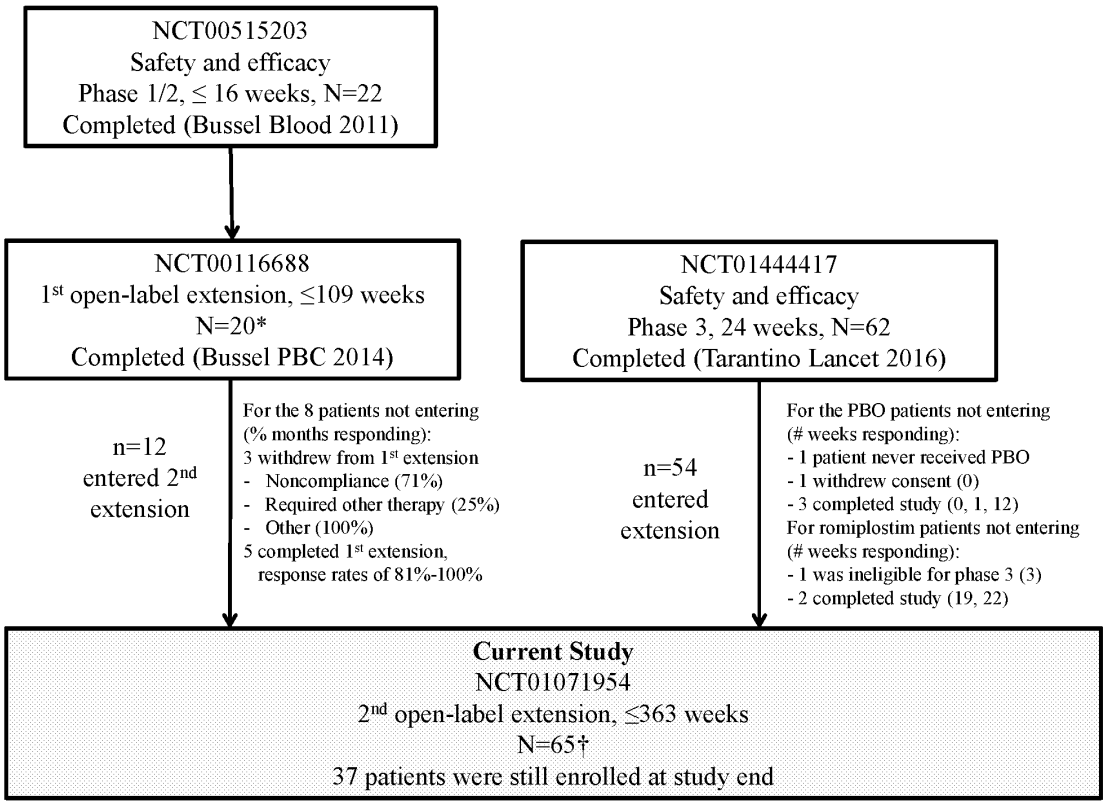

NCT00515203
Safety and efficacy
Phase 1/2, ≤ 16 weeks, N=22
Completed (Bussel Blood 2011)
NCT00116688
1st open-label extension, ≤109 weeks
N=20*
Completed (Bussel PBC 2014)
NCT01444417
Safety and efficacy
Phase 3, 24 weeks, N=62
Completed (Tarantino Lancet 2016)
n=12
entered 2nd
extension
For the 8 patients not entering
(% months responding):
3 withdrew from 1st extension
- Noncompliance (71%)
- Required other therapy (25%)
- Other (100%)
5 completed 1st extension,
response rates of 81%-100%
n=54
entered
extension
For the PBO patients not entering
(# weeks responding):
- 1 patient never received PBO
- 1 withdrew consent (0)
- 3 completed study (0, 1, 12)
For romiplostim patients not entering
(# weeks responding):
- 1 was ineligible for phase 3 (3)
- 2 completed study (19, 22)
Current Study
NCT01071954
2nd open-label extension, ≤363 weeks
N=65†
37 patients were still enrolled at study end

FIGURE 2

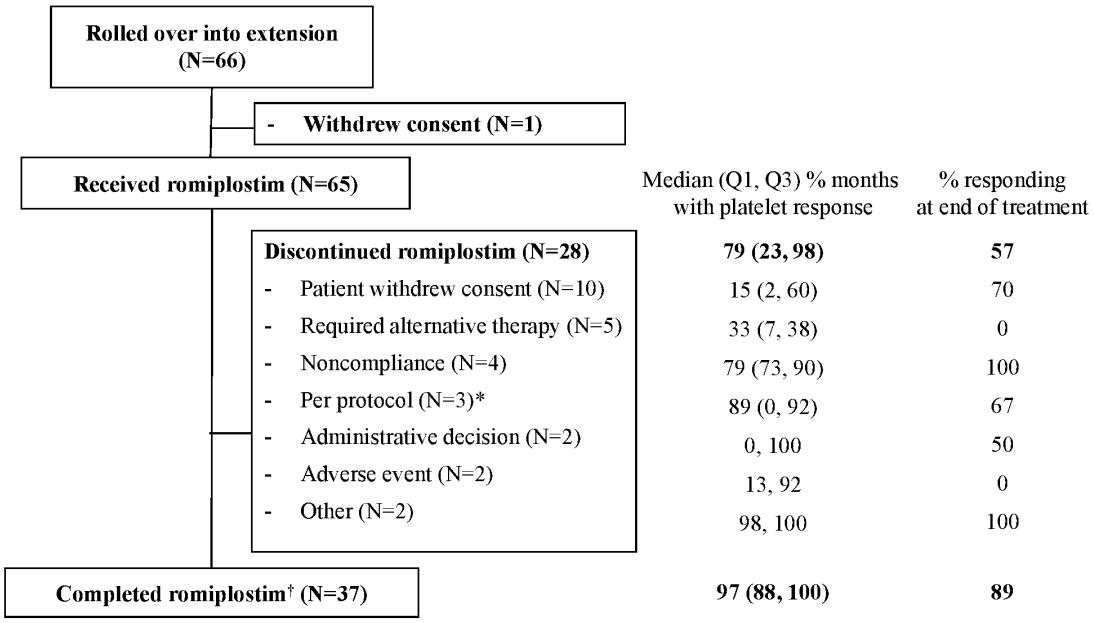

Rolled over into extension (N=66)
- Withdrew consent (N=1)
Received romiplostim (N=65)
Discontinued romiplostim (N=28)
- Patient withdrew consent (N=10)
- Required alternative therapy (N=5)
- Noncompliance (N=4)
- Per protocol (N=3)*
- Administrative decision (N=2)
- Adverse event (N=2)
- Other (N=2)
Completed romiplostim† (N=37)
Median (Q1, Q3) % months with platelet response
% responding at end of treatment
79 (23, 98)
57
15 (2, 60)
70
33 (7, 38)
0
79 (73, 90)
100
89 (0, 92)
67
0, 100
50
13, 92
0
98, 100
100
97 (88, 100)
89

FIGURE 3A

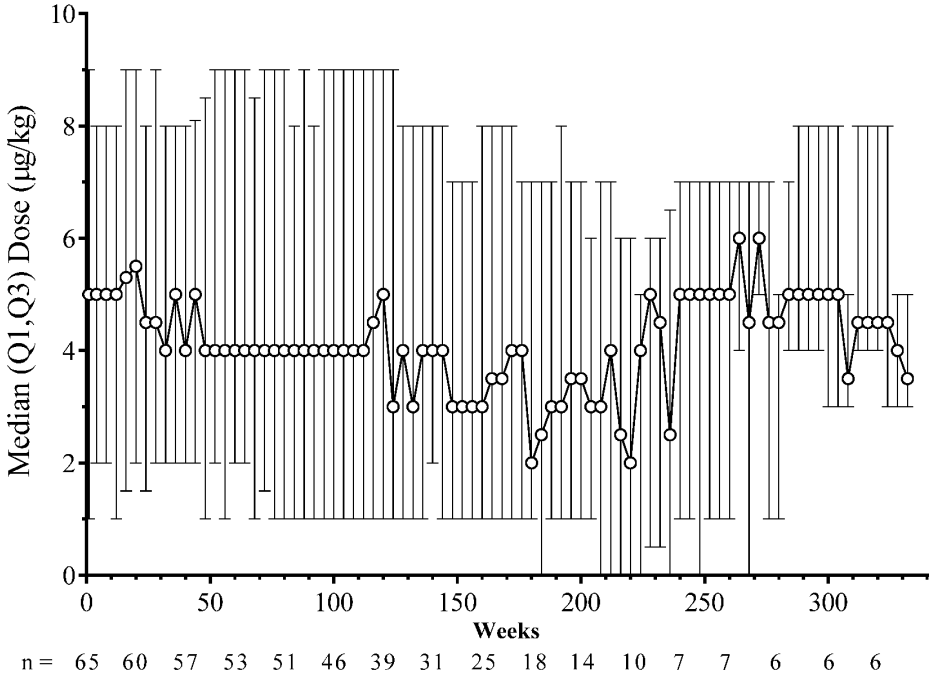

Median (Q1,Q3) Dose (µg/kg)
10
8
6
4
2
0
0
50
100
150
200
250
300
Weeks
n = 65 60 57 53 51 46 39 31 25 18 14 10 7 7 6 6 6

FIGURE 3B

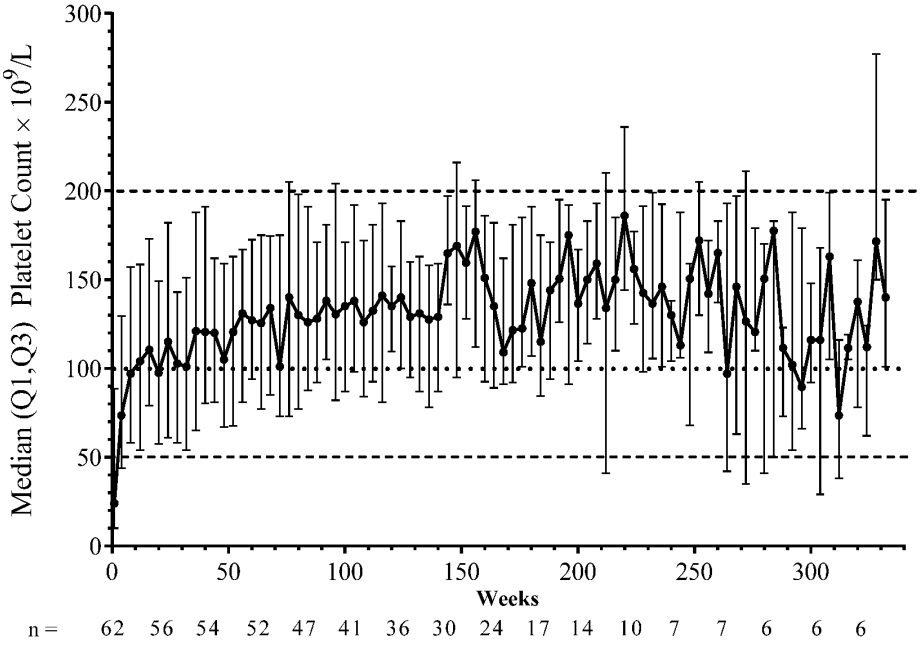

Median (Q1,Q3) Platelet Count × 10^9/L
300
250
200
150
100
50
0
0
50
100
150
200
250
300
Weeks
n = 62 56 54 52 47 41 36 30 24 17 14 10 7 7 6 6 6

FIGURE 4A

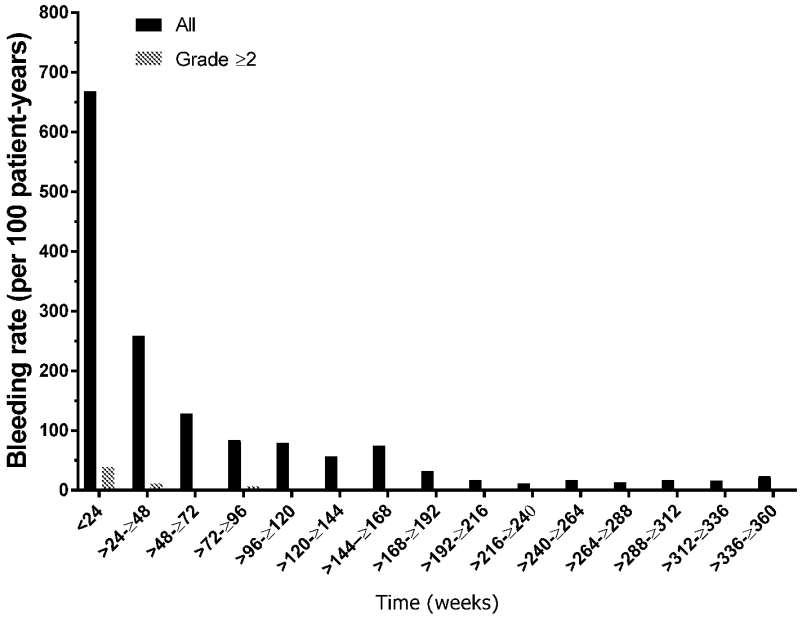
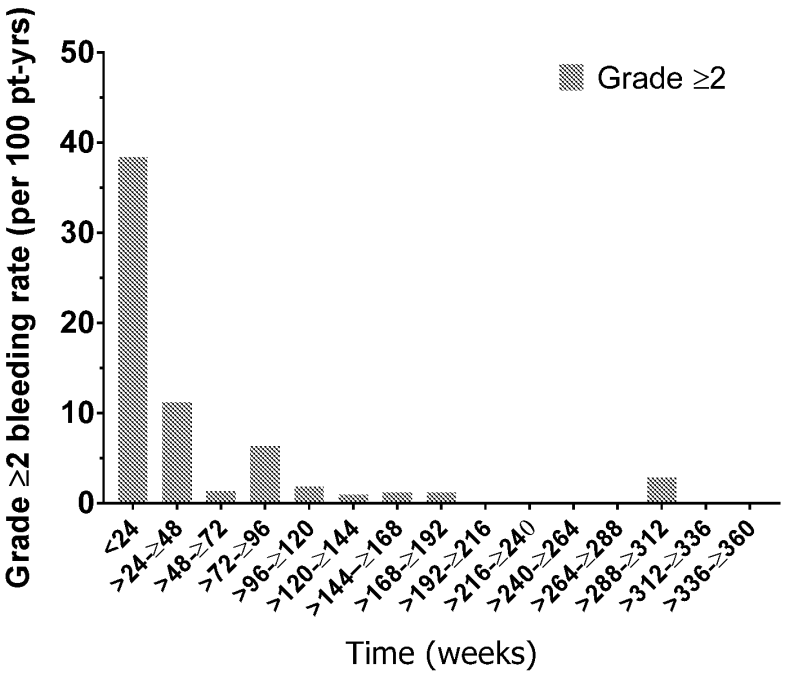
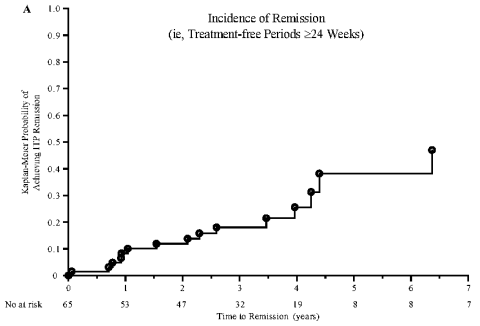
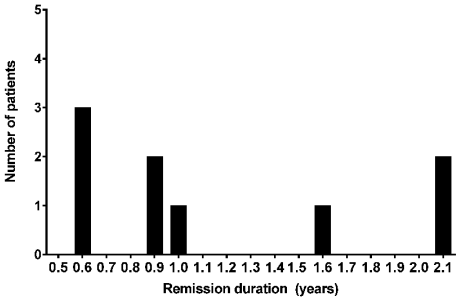

All
Grade ≥2
Bleeding rate (per 100 patient-years)
800
700
600
500
400
300
200
100
0
<24
>24-≥48
>48-≥72
>72-≥96
>96-≥120
>120-≥144
>144-≥168
>168-≥192
>192-≥216
>216-≥240
>240-≥264
>264-≥288
>288-≥312
>312-≥336
>336-≥360
Time (weeks)

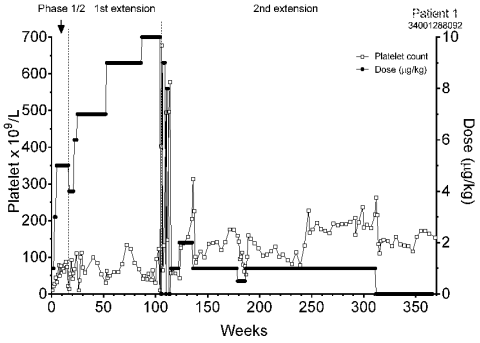
FIGURE 4B


Grade ≥2 bleeding rate (per 100 pt-yrs)
50
40
30
20
10
0
Grade ≥2
<24
>24-≥48
>48-≥72
>72-≥96
>96-≥120
>120-≥144
>144-≥168
>168-≥192
>192-≥216
>216-≥240
>240-≥264
>264-≥288
>288-≥312
>312-≥336
>336-≥360
Time (weeks)

FIGURES 5A-5D







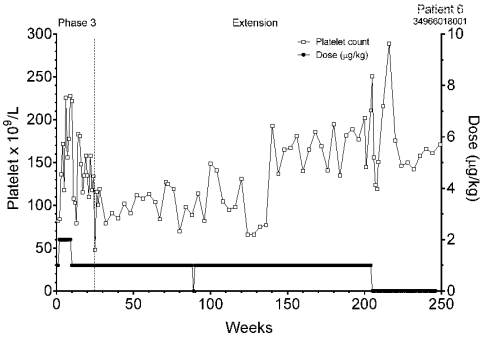
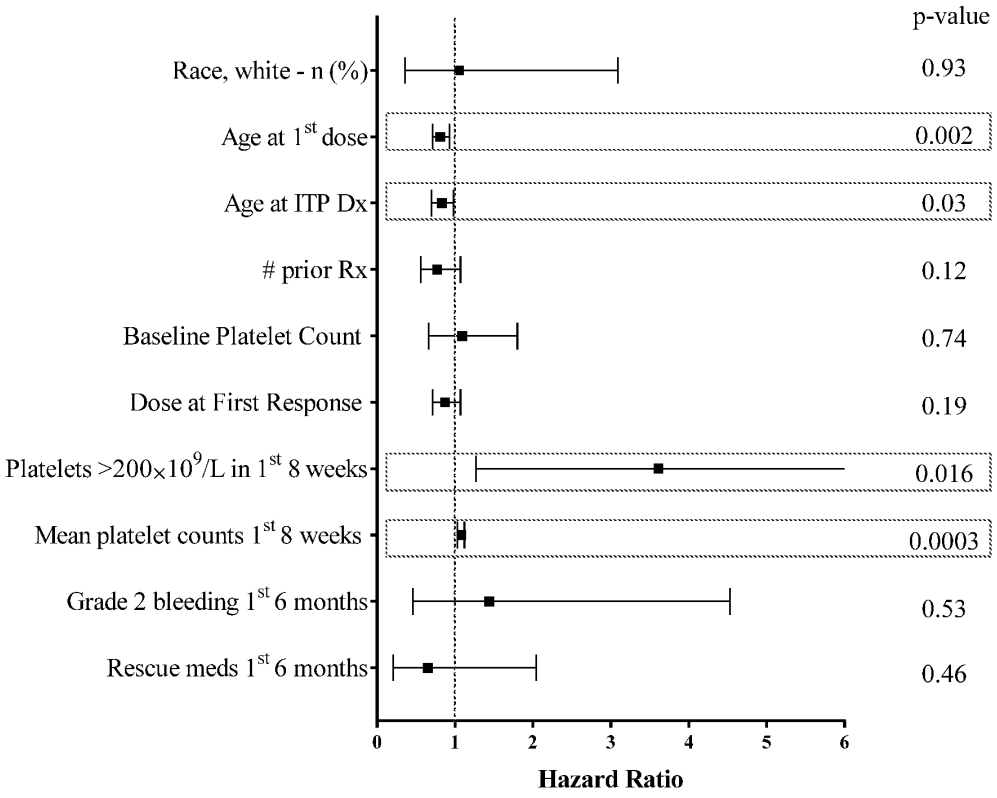
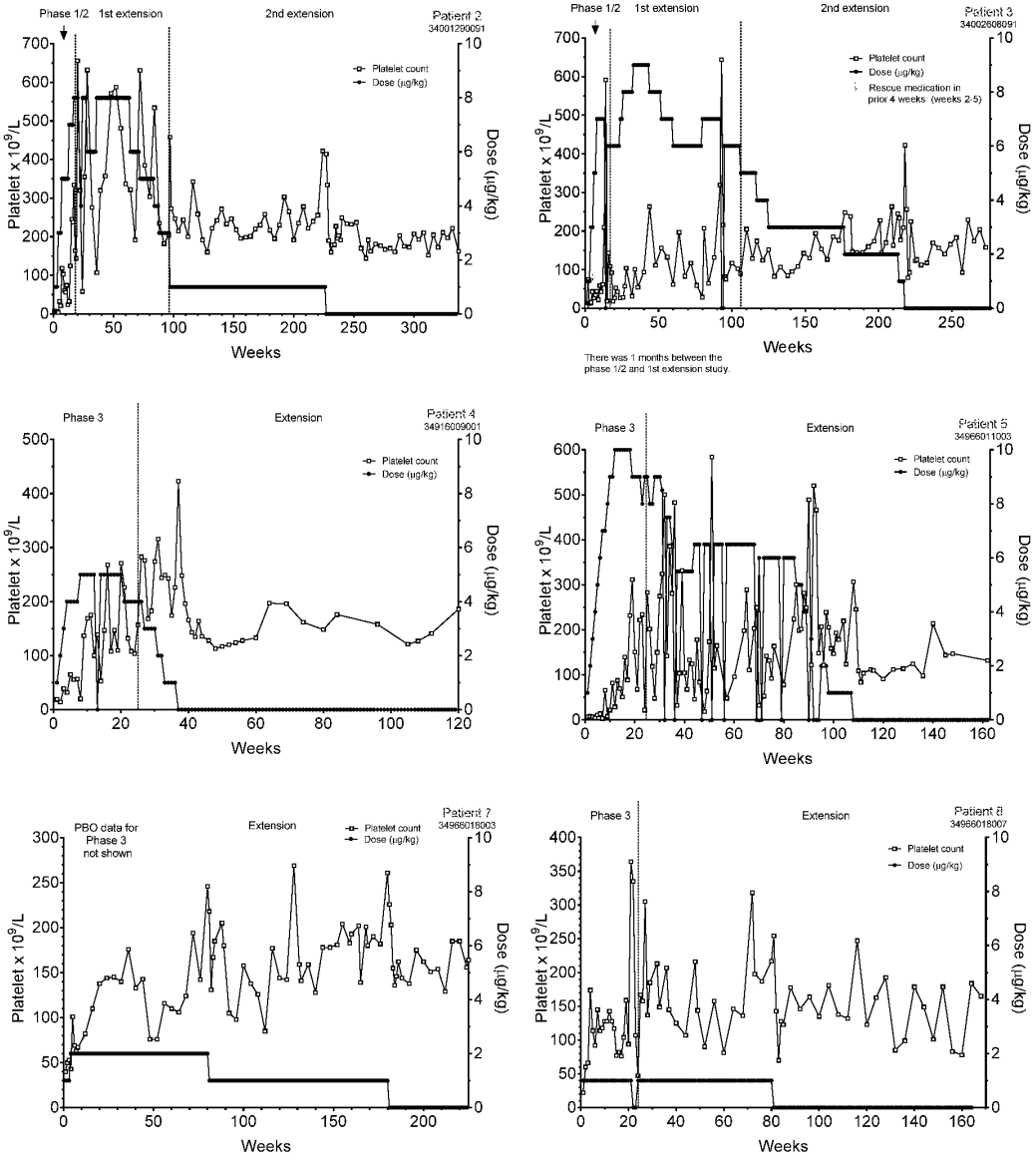


FIGURE 6


p-value
Race, white - n (%)
0.93
Age at 1st dose
0.002
Age at ITP Dx
0.03
prior Rx
0.12
Baseline Platelet Count
0.74
Dose at First Response
0.19
Platelets >200x10^9/L in 1st 8 weeks
0.016
Mean platelet counts 1st 8 weeks
0.0003
Grade 2 bleeding 1st 6 months
0.53
Rescue meds 1st 6 months
0.46
0
1
2
3
4
5
6
Hazard Ratio

FIGURES 7A-7F



FIGURES 7G-7M

