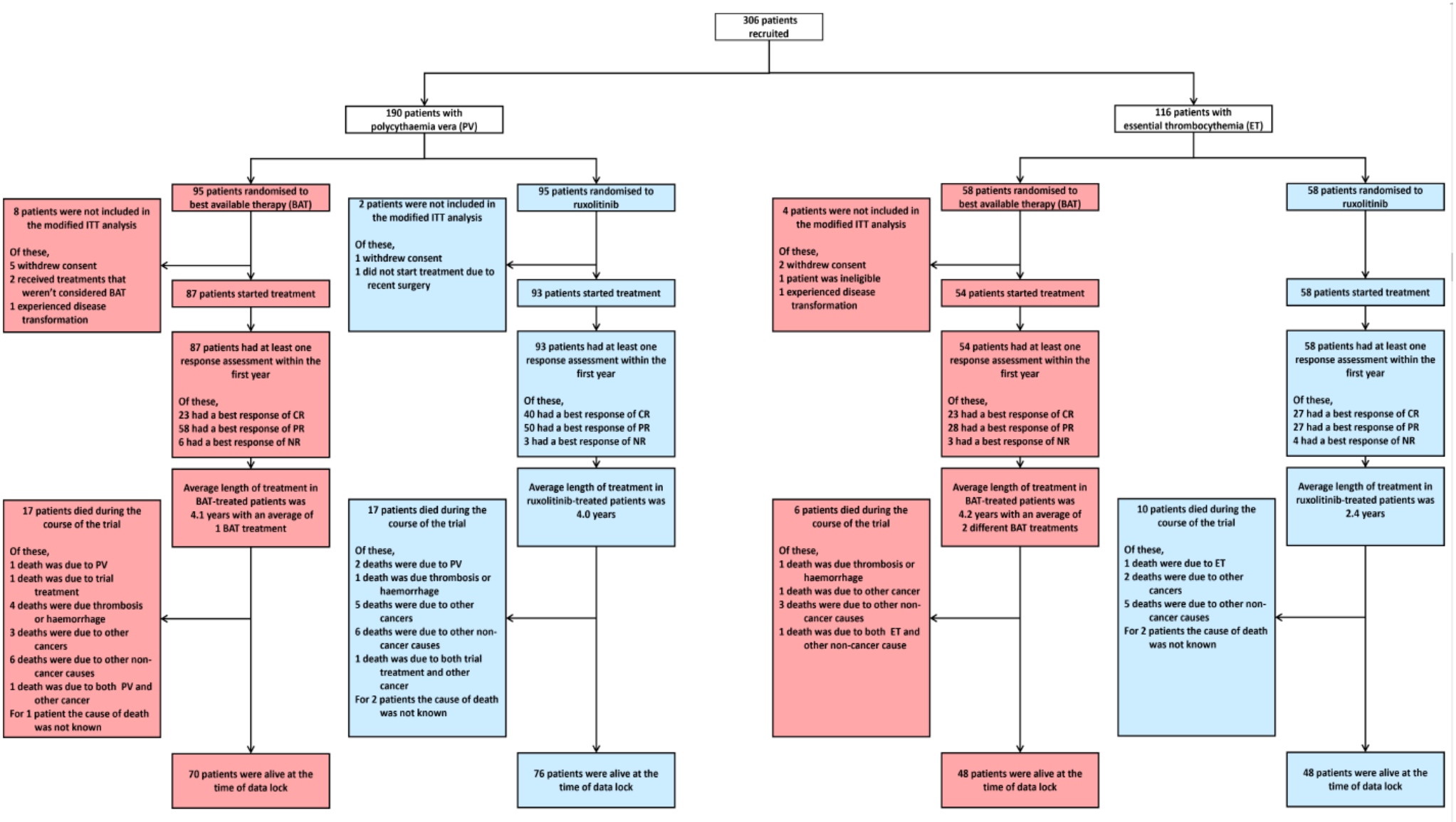
MAJIC Basic Results Summary

Participant Flow



Baseline Characteristics

Table 1: Time from diagnosis

	PV-BAT	PV-Ruxolitinib	All PV	ET-BAT	ET-Ruxolitinib	All ET
N	87	93	180	54	58	112
Mean (SD)	9 years (7)	9 years (7)	9 years (7)	7 years (6)	10 years (7)	9 years (6)
Median	8 years	8 years	8 years	5 years	10 years	7 years
Range	4 months, 32 years	6 days, 30 years	6 days, 32 years	5 months, 24 years	9 months, 31 years	5 months, 31 years

Table 2: Patient characteristics

Disease type/Treatment arm	PV-BAT (87)	PV-Ruxolitinib (93)	All PV (180)	ET-BAT (54)	ET-Ruxolitinib (58)	All ET (112)
Age, years						
N	87	93	180	54	58	112
Mean (sd)	64 (11)	64 (11)	64 (11)	65 (14)	62 (12)	64 (13)
Median	66	67	66	67	65	67
Range	28, 85	34, 88	28, 88	37, 85	34, 90	34, 90
Gender (N (%))						
Male	49 (56)	56 (60)	105 (58)	23 (43)	22 (38)	45 (40)
Female	38 (44)	37 (40)	75 (42)	31 (57)	36 (62)	67 (60)
Total	87 (100)	93 (100)	180 (100)	54 (100)	58 (100)	112 (100)
Resistant or intolerent to hydroxycarbomide (N (%))						
Resistant	23 (26)	31 (33)	54 (30)	10 (19)	19 (33)	29 (26)
Intolerant	37 (43)	43 (46)	80 (44)	28 (52)	30 (52)	58 (52)
Both	27 (31)	19 (20)	46 (26)	16 (30)	9 (16)	25 (22)
Total	87 (100)	93 (100)	180 (100)	54 (100)	58 (100)	112 (100)
JAK status (N (%))						
Negative	2 (2)	4 (4)	6 (3)	27 (50)	30 (52)	57 (51)
Positive	85 (98)	89 (96)	174 (97)	27 (50)	28 (48)	55 (49)
Total	87 (100)	93 (100)	180 (100)	54 (100)	58 (100)	112 (100)

Outcome Measures

Primary Outcomes:

Table 3: Complete response within a year

Disease type/Treatment arm	PV-BAT (87)	PV-Ruxolitinib (93)	All PV (180)	ET-BAT (54)	ET-Ruxolitinib (58)	All ET (112)
Best response within a year						
No or partial response	64 (74)	53 (57)	117 (65)	31 (57)	31 (53)	62 (55)
Complete response	23 (26)	40 (43)	63 (35)	23 (43)	27 (47)	50 (45)

Secondary Outcomes:

Table 4: Best response within a year

Disease type/Treatment arm	PV-BAT (87)	PV-Ruxolitinib (93)	All PV (180)	ET-BAT (54)	ET-Ruxolitinib (58)	All ET (112)
Best response within a year						
NR	6 (7)	3 (3)	9 (5)	3 (6)	4 (7)	7 (6)
PR	58 (67)	50 (54)	108 (60)	28 (52)	27 (47)	55 (49)
CR	23 (26)	40 (43)	63 (35)	23 (43)	27 (47)	50 (45)
Best overall response within a year						
NR	6 (7)	3 (3)	9 (5)	3 (6)	4 (7)	7 (6)
Overall response (PR+CR)	81 (93)	90 (97)	171 (95)	51 (94)	54 (93)	105 (94)

NR = no response; PR = partial response; CR = complete response

Table 5- Summary of response rates within the first year of the trial:

Response	PV-BAT	PV-Ruxolitinib	All PV	ET-BAT	ET-Ruxolitinib	All ET
Complete response	26% (95% CI: 18, 37)	43% (95% CI: 33, 54)	35% (95% CI: 28, 42)	43% (95% CI: 29, 57)	47% (95% CI: 33, 60)	45% (95% CI: 35, 54)
Overall response	93% (95% CI: 86, 97)	97% (95% CI: 91, 99)	95% (95% CI: 91, 98)	94% (95% CI: 85, 99)	93% (95% CI: 83, 98)	94% (95% CI: 88, 97)

Table 6: Summary of events, median survival and survival proportions for PFS and OS

			PV-BAT	PV-Ruxolitinib	All PV	ET-BAT	ET-Ruxolitinib	All ET
PFS	Number of events		27	22	49	9	17	26
	Median survival		Not reached	Not reached	Not reached	Not reached	Not reached	Not reached
	Survival percentage at	1 year	95% (95% CI: 88, 98)	96% (95% CI: 89, 98)	95% (95% CI: 91, 98)	98% (95% CI: 87, 100)	91% (95% CI: 80, 96)	95% (95% CI: 88, 97)
		3 years	75% (95% CI: 63, 83)	84% (95% CI: 74, 90)	79% (95% CI: 72, 85)	90% (95% CI: 78, 96)	78% (95% CI: 64, 87)	84% (95% CI: 75, 90)
		5 years	64% (95% CI: 51, 74)	76% (95% CI: 65, 84)	70% (95% CI: 62, 77)	81% (95% CI: 67, 90)	55% (95% CI: 26, 77)	71% (95% CI: 59, 81)
OS	Number of events		17	17	34	6	10	16
	Median survival		Not reached	Not reached	Not reached	Not reached	Not reached	Not reached
	Survival percentage at	1 year	99% (95% CI: 92, 100)	100%	99% (95% CI: 96, 100)	98% (95% CI: 87, 100)	98% (95% CI: 88, 100)	98% (95% CI: 93, 100)
		3 years	87% (95% CI: 77, 93)	88% (95% CI: 79, 93)	87% (95% CI: 81, 92)	96% (95% CI: 85, 99)	89% (95% CI: 76, 95)	93% (95% CI: 86, 97)
		5 years	75% (95% CI: 61, 84)	83% (95% CI: 72, 89)	79% (95% CI: 71, 85)	87% (95% CI: 73, 94)	64% (95% CI: 28, 85)	80% (95% CI: 66, 88)

-Progression free survival(PFS) is defined as the time from date of registration to the date of progression or date of death from any cause

-Overall survival (OS) is defined as the as the time from date of registration to the date of death from any cause

Table 7: Haemorrhagic and thromboembolic events

Events (Patients affected, %*)	PV-BAT	PV-Ruxolitinib	All PV	ET-BAT	ET-Ruxolitinib	All ET
Haemorrhagic event	18 (16; 18%)	15 (12; 13%)	33 (28; 16%)	7 (7; 13%)	3 (3; 5%)	10 (10; 9%)
Major haemorrhagic event	11 (10; 11%)	3 (2; 2%)	14 (12; 7%)	4 (4; 7%)	1 (1; 2%)	5 (5; 4%)
Thromboembolic event	22 (17; 20%)	14 (10; 11%)	36 (27; 15%)	10 (7; 13%)	11 (10; 17%)	21 (17; 15%)
Major thromboembolic event	22 (17; 20%)	13 (9; 10%)	35 (26; 14%)	6 (5; 9%)	8 (7; 12%)	14 (12; 11%)

Adverse Events

Table 8: Summary of AE toxicities (CTCAE grade 3 and above) occurring in at least 5% of patients

	Events (patients, %)
PV	
BAT	
Sepsis	7 (5, 6%)
Thromboembolic event	6 (6, 7%)
Ruxolitinib	
Anemia	12 (7, 8%)
ET	
Ruxolitinib	
Anemia	24 (14, 24%)
Dyspnea	3 (3, 5%)
Fall	3 (3, 5%)
Headache	3 (3, 5%)
Hyperkalemia	3 (3, 5%)
Hypertension	3 (3, 5%)
Lung infection	5 (5, 9%)
Thromboembolic event	5 (4, 7%)

Table 9: Summary of AE toxicities (CTCAE grade 3 and above) considered related to treatment occurring in at least 5% of patients

	Events (patients, %)
PV	
Ruxolitinib	
Anemia	11 (6, 6%)
ET	
Ruxolitinib	
Anemia	22 (12, 21%)
Lung infection	3 (3, 5%)

Table 10: SAE Categorisation Summary

Disease type/Treatment arm	PV-BAT (97)	PV-Ruxolitinib (135)	All PV (232)	ET-BAT (38)	ET-Ruxolitinib (75)	All ET (113)
SAE Categorisation (N (%))						
Unrelated SAE	89 (92)	96 (71)	185 (80)	36 (95)	50 (67)	86 (76)
SAR	1 (1)	28 (21)	29 (13)	0 (0)	20 (27)	20 (18)
Non fatal/life-threatening SUSAR	0 (0)	8 (6)	8 (3)	0 (0)	4 (5)	4 (4)
Fatal/life-threatening SUSAR	0 (0)	3 (2)	3 (1)	0 (0)	1 (1)	1 (1)
No recorded categorisation	7 (7)	0 (0)	7 (3)	2 (5)	0 (0)	2 (2)
Total	97 (100)	135 (100)	232 (100)	38 (100)	75 (100)	113 (100)

N = number of events

Table 11: Summary table of admitting toxicities including SUSARS and SARs

Events (Patients, %)	PV-BAT	PV-Ruxolitinib	ET-Ruxolitinib
Abdominal pain	0 (0, 0%)	1 (1, 1%)	1 (1, 2%)
Anemia	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Atrial fibrillation	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Basal cell carcinoma	0 (0, 0%)	3 (3, 3%)	0 (0, 0%)
Chest infection	0 (0, 0%)	0 (0, 0%)	2 (2, 3%)
Dental caries	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Diarrhea	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Dyspnea	0 (0, 0%)	2 (2, 2%)	3 (3, 5%)
EBV reactivation	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Encephalitis infection	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Esophageal infection	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Fever	0 (0, 0%)	1 (1, 1%)	1 (1, 2%)
Gum infection	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Headache	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Heart failure	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Hematoma	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Lower gastrointestinal hemorrhage	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Lung infection	0 (0, 0%)	2 (2, 2%)	4 (4, 7%)
Lymph gland infection	0 (0, 0%)	2 (2, 2%)	0 (0, 0%)
Myocardial infarction	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Myoclonic jerk	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Neck edema	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Neoplasm benign, malignant and unspecified	1 (1, 1%)	4 (2, 2%)	0 (0, 0%)
Otitis media	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Platelet count decreased	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Pleuritic pain	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Pulmonary thromboembolus	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Soft tissue infection	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Squamous cell carcinoma	0 (0, 0%)	6 (5, 5%)	1 (1, 2%)
Sudden death NOS	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Surgical procedure	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
TB	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Thromboembolic event	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Transformation to Myelodysplastic syndrome	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)
Transformation to Myelofibrosis	0 (0, 0%)	1 (1, 1%)	2 (2, 3%)
Upper respiratory infection	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
Urinary tract infection	0 (0, 0%)	1 (1, 1%)	0 (0, 0%)
small bowel necrosis	0 (0, 0%)	0 (0, 0%)	1 (1, 2%)