

S2 Table: Clinical trials in adults with polyarteritis nodosa

Study	Study population	Trial design	Years to recruit	Outcome	Adverse events	Conclusion
Ribi et al 2010 (A&R 62:1186-1197)	124 adults with PAN (n=58) or MPA (n=66) with FFS=0	Open-label RCT: 39 patients who failed corticosteroids randomised to receive CYC or AZA. Corticosteroids given to both groups.	12	<u>Remission:</u> 69% CYC 70% AZA <u>Death:</u> 20% 32% CYC 10% AZA	54% : equal in CYC or AZA	AZA or CYC reasonably effective for treating mild but corticosteroid resistant PAN/MPA
Guillevin et al 2003 (A&R 49: 93-100)	65 adults with PAN (n=18) or MPA (n=47) with FFS \geq 1	Open-label RCT of 6 vs 12 intravenous doses of CYC. Corticosteroids given to both groups. AZA not given on cessation of CYC.	6	<u>Remission:</u> 86% <u>Relapses:</u> 22% for 12 CYC 66% for 6 CYC <u>Death:</u> 22% 18% for 12 CYC 26% for 6 CYC	100% had nausea/vomiting 51% had other side effects	6 CYC pulses are less effective than 12 CYC pulses to treat severe PAN and MPA
Gayraud et al 1997 (Br J Rheum 36: 1290-1297)	25 adults with PAN (n=17) or CSS (n=8) with FFS=0	Open-label RCT of daily oral CYC vs monthly-IV CYC	4	<u>Remission:</u> 76% no difference between oral or IV CYC. Higher cumulative CYC dose in oral group <u>Relapses:</u> 16% similar in both groups <u>Death:</u> 4% 1 patient in IV CYC group died of sepsis.	72% <u>Oral CYC:</u> 27 side effects in 10/12 patients <u>IV CYC:</u> 14 side-effects in 8/13 patients	IV CYC recommended rather than oral CYC based on more favourable therapeutic index: overall lower cumulative dose with comparable efficacy.
Guillevin et al 1995 (A&R 38:1638-1645)	62 adults with PAN (n=48) or CSS (n=14) with FFS \geq 1	Non-blinded RCT of CS-CYC (n=28) versus CS-CYC plus plasma exchange (n=34)	5	<u>Remission:</u> 61% no difference +/- plasma exchange. <u>Death:</u> 18% 25% without plasma exchange 12% with plasma exchange (NS).	Infection: 15% Lymphoma: 1 patient	Additional plasma exchange is not superior to treatment with CS-CYC alone.

PAN: polyarteritis nodosa; MPA: microscopic polyangiitis; FFS: 5 factor score; RCT: randomised controlled trial; CYC: cyclophosphamide; AZA: azathioprine; CSS: Churg Strauss syndrome; CS: corticosteroid; NS: non-significant.