

Supplemental Table 4: Comparison with other cohorts

	Dragon-Durey <i>et al.</i> ²²	Sana <i>et al.</i> ²⁸	Sinha <i>et al.</i> ⁶	Lee <i>et al.</i> ²⁴	Geerdink <i>et al.</i> ²³	Noris <i>et al.</i> ²⁵	Abarrategui-Garrido <i>et al.</i> ¹⁹	Hofer <i>et al.</i> ¹⁵	Moore <i>et al.</i> ⁹	UK and Ireland
# Patients	45 (38 children)	4 children	138 children	15 children	6 children	8 (6 children)	7 children	25 children	13 children	17 children
Age (years)	Median 8.5 (children only)	Mean 6	Mean 8.4	Mean 8	1-7 years at onset in 83.3%	N/A	Mean 5.2	Mean 7.9	Median 8	Median 8
% Male	66% (children only)	N/A	72.5%	27%	50%	50%	N/A	N/A	46%	65%
Gastrointestinal symptoms	84% (diarrhoea 53%)	25%	Diarrhoea 9.4%	Diarrhoea 13%	83%	14.3%	N/A	87%	N/A	75% (diarrhoea 50%)
Extra-renal manifestations	50% hepatitis 23% pancreatitis 23.5% seizures	N/A	57.3% hepatic 40.6% seizures	7% hepatitis 7% pancreatitis 7% CNS	N/A	14.3% CNS	N/A	11% CNS 58% other	N/A	19% seizures 13% hepatitis 6% pancreatitis
Concomitant rare genetic variants	0/26	0	N/A	0/15	2/6 (33%): <i>CFI</i> , <i>C3</i>	2/8 (25%): <i>CFH</i> x2	0/7	1/8 (12.5%): <i>CFI</i>	5/13 (38%): <i>CFH</i> , <i>CFI</i> , <i>CD46</i> , <i>C3</i> x2	7/17 (41%): <i>CFH</i> , <i>CFI</i> x4, <i>CD46</i> , <i>C3</i>
Complement analysis	Low C3	58%	62%	67%	N/A	43%	57%	41%	27%	24%
	Low C4	0	N/A	N/A	N/A	14%	14%	15%	27%	29%
	Low FH	22%	N/A	N/A	N/A	29%	0	N/A	9%	12%
Treatment and evolution of first episode										
Supportive	6 (13% of total): ERF 1 (17%); relapses 2 (33%); TR 1 (17%); CKD 1 (17%)	0	N/A	N/A	2 (33%)	N/A	N/A	N/A	10 (77%)	2 (12% of total): ERF 2 (100%)
PI	6 (13% of total): relapses 5 (83%), TR 1 (17%)	0	N/A	91%	N/A	N/A	N/A	N/A	N/A	n/a
PEX	15 (33% of total): ERF 1 (7%); death 1 (7%); CKD 3 (20%); TR 3 (20%); relapses 6 (40%)	0	105 (76%) in total	69%	N/A	N/A	N/A	N/A	3 (23%)	11 (65% of total): ERF 4 (36%); relapses 5 (45%)
PEX or PI	N/A	0	N/A	N/A	4/6 (67%) (chronic in 3)	7/8 (87.5%)	5/7 (71%)	18/19 (95%)	N/A	n/a
PEX + Corticosteroids	0	0	N/A	N/A	N/A	3/8 (37.5%): remission in 2/3 (67%)	1	N/A	N/A	1
PEX + Immunosuppression	3 (6.7% of total)	4 (100%)	87 (63%) IS	7 (54%) steroids +/- IS	0	3/8 (37.5%)	2 (29%)	0	0	0
	Azathioprine	0	N/A	N/A	n/a	0	1	n/a	n/a	n/a
	Steroids + cyclophosphamide	2: no renal sequelae	4 (at relapse in patient treated with rituximab)	49	N/A	n/a	0	n/a	n/a	n/a
	Steroids + rituximab	0	(rituximab initially in 1)	18	N/A	n/a	0	n/a	n/a	n/a
	Steroids + MMF	1: no renal sequelae	0	N/A	N/A	n/a	0	0	n/a	n/a
Ecuzumab	0	0	0	0	0	0	0	0	0	4 (24% of total): 100% sustained remission
Maintenance Immunosuppression	N/A	n/a	47 (34%): steroids alone 21; plus azathioprine 8; plus MMF 18	N/A	n/a	N/A	N/A	n/a	n/a	0
Long term outcomes										
Sustained remission	N/A (25% no renal sequelae)	100% after cyclophosphamide	N/A	62% no sequelae	0	12% complete remission	N/A	N/A	N/A	n/a
Relapse	58%	25% (following rituximab in 1)	14 (11.5%); (4 whilst on IS)	31%	50%	37.5%	N/A	N/A	23%	29% overall
ERF	27%	0	32.8%	0	0	63%	43%	N/A	46%	35% overall
CKD (not including ERF)	39%	0	N/A	15%	N/A	N/A	N/A	N/A	N/A	CKD stage 3: 1 (6%)
Death	4 (8.9%)	0	20 (16.4%)	0	1 (16.7%)	0	1 (14.3%)	N/A	0	0
	Deaths: unknown; sudden death post dialysis; pulmonary hypertension; cardiac insufficiency	n/a	Deaths: complications of renal failure 16; sepsis 4	n/a	N/A	n/a	Death: myocarditis	N/A	n/a	n/a
Outcome of transplantation										
Recurrence	3 without specific management	n/a	0	n/a	0	1	0	N/A	0	1 early recurrence (<i>CFI</i> mutation) treated with Ecuzumab
No recurrence	3 with specific management	n/a	3 with specific management (PEX/IVIG/rituximab)	n/a	0	0	2	N/A	3	4 without specific management (1 graft loss rejection)

Abbreviations: CKD, chronic kidney disease; ERF, established renal failure; FH, factor H; IS, immunosuppression; IVIG, intravenous immunoglobulin; N/A, not available; n/a, not applicable; PEX, plasma exchange; PI, plasma infusion; TR, treatment resistance.