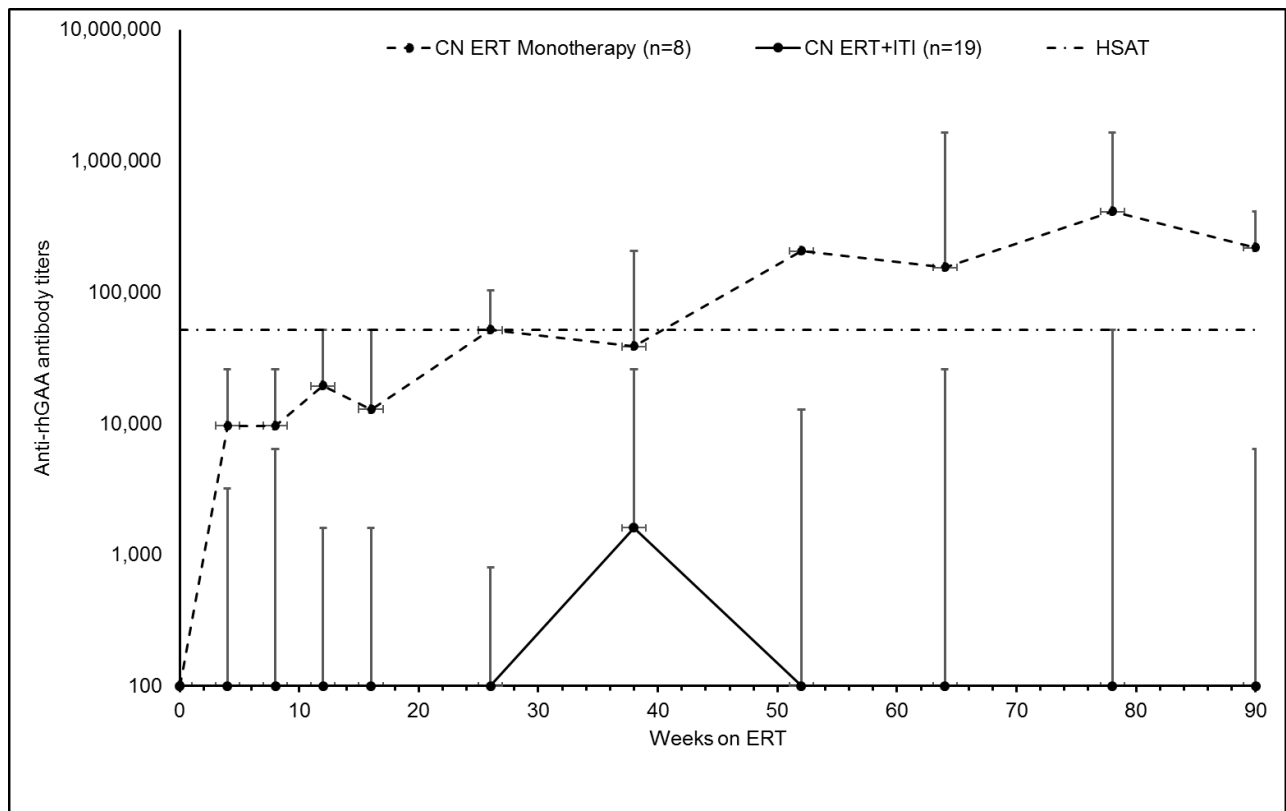
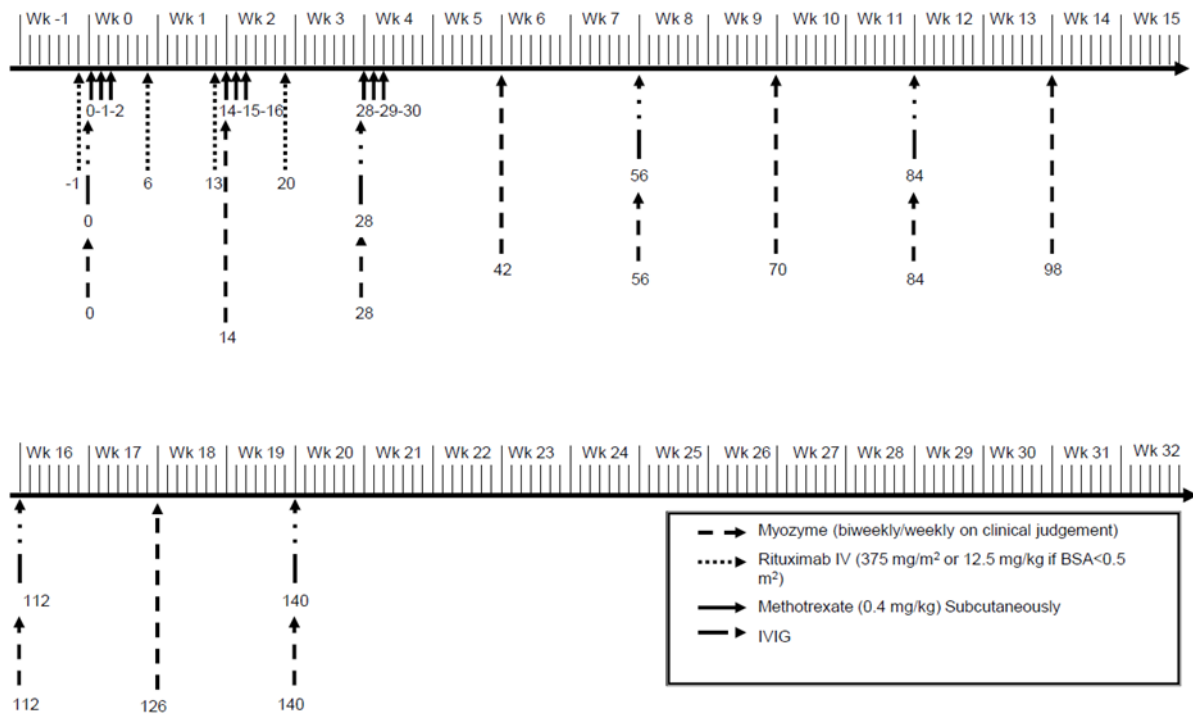


Supplementary Figure 1. Comparison of longitudinal IgG antibody titers in CRIM-negative IPD patients treated with ERT+ITI versus treatment with ERT monotherapy



High and sustained rhGAA IgG antibody titers (HSAT) defined as titers of $\geq 51,200$ at or beyond six months on ERT (shown by a horizontal dashed line).

Supplementary Figure 2. ITI treatment regimen which includes rituximab, methotrexate, and intravenous immunoglobulin (IVIg)



Supplementary Table 1. *GAA* variant data on CRIM-negative IPD patients on ERT+IT

CRIM-negative patients treated with ITI (alive at the time of database lock)				
Pt.	<i>GAA</i> Allele 1	<i>GAA</i> Allele 2	Allele 1 type	Allele 2 type
1	c.341insT	c.341insT	Frameshift	Frameshift
3	c.2608C>T	c.2608C>T	Nonsense	Nonsense
4	c.546+2T>C	c.546+2T>C	Splice site	Splice site
5	c.236_246del	c.236_246del	Frameshift	Frameshift
6	c.525delT	c.2560C>T	Frameshift	Nonsense
10	c.2560C>T	c.1292_1295dupTGCA	Nonsense	Frameshift
11	c.2560C>T	c.2560C>T	Nonsense	Nonsense
13	c.2560C>T	c.2560C>T	Nonsense	Nonsense
14	c.258dupC	c.2227C>T	Frameshift	Nonsense
15	c.1754+2T>A	c.1822C>T	Splice site	Nonsense
16	c.2237G>A	c.437delT	Nonsense	Frameshift
17	c.2560C>T	c.2560C>T	Nonsense	Nonsense
18	c.2560C>T	c.2560C>T	Nonsense	Nonsense
19	c.2560C>T	c.525delT	Nonsense	Frameshift
Deceased CRIM-negative patients treated with ITI				
Pt.	<i>GAA</i> Allele 1	<i>GAA</i> Allele 2	Allele 1 type	Allele 2 type
2	c.1548G>A	c.525delT	Nonsense	Frameshift
7	c.2560C>T	c.2560C>T	Nonsense	Nonsense
8	c.525_526delTG	c.525_526delTG	Frameshift	Frameshift
9	c.2560C>T	c.2560C>T	Nonsense	Nonsense
12	c.2560C>T	c.2560C>T	Nonsense	Nonsense

Supplementary Table 2. Demographics and *GAA* variant data of CRIM-negative IPD patients treated with ERT monotherapy

Pt.	Gender	Race/Ethnicity	Age at initiation of ERT (months)	<i>GAA</i> allele 1	<i>GAA</i> allele 2	Allele 1 type	Allele 2 type
1	M	Caucasian	4.2	c.722_723delTT	c.1687C>T	Frameshift	Nonsense
2	M	African American	2.8	c.2560C>T	c.2560C>T	Nonsense	Nonsense
3	M	Asian	4.8	c.148_859_11del	c.685_686insCGGC	Frameshift	Frameshift
4	F	Arab	2.7	c.2560C>T	c.2560C>T	Nonsense	Nonsense
5	F	Caucasian	7.0	c.1075G>T	c.1075G>T	Nonsense	Nonsense
6	F	African American	2.3	c.2560C>T	c.2560C>T	Nonsense	Nonsense
7	M	African American	6.2	c.2560C>T	c.2560C>T	Nonsense	Nonsense
8	F	Caucasian	2.1	c.722_723delTT	c.1754 +1G>A	Frameshift	Splice site
9	F	Arabic	3.7	c.1209delC	c.1209delC	Frameshift	Frameshift
10	M	Hispanic	0.3	c.1496G>A	c.1496G>A	Nonsense	Nonsense