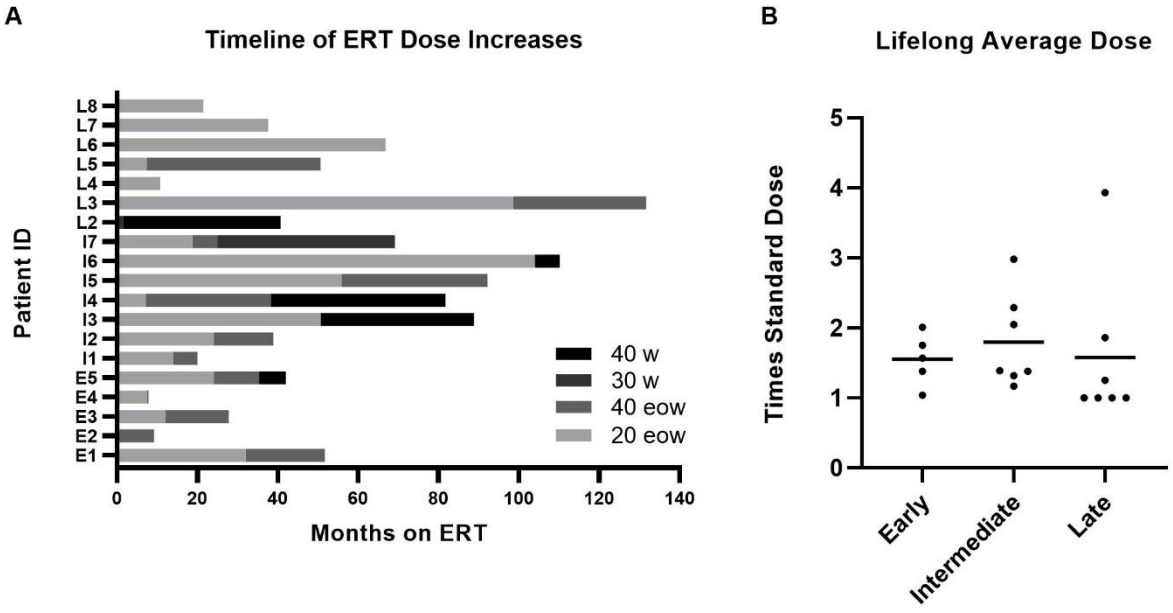
SUPPLEMENTARY FIGURES

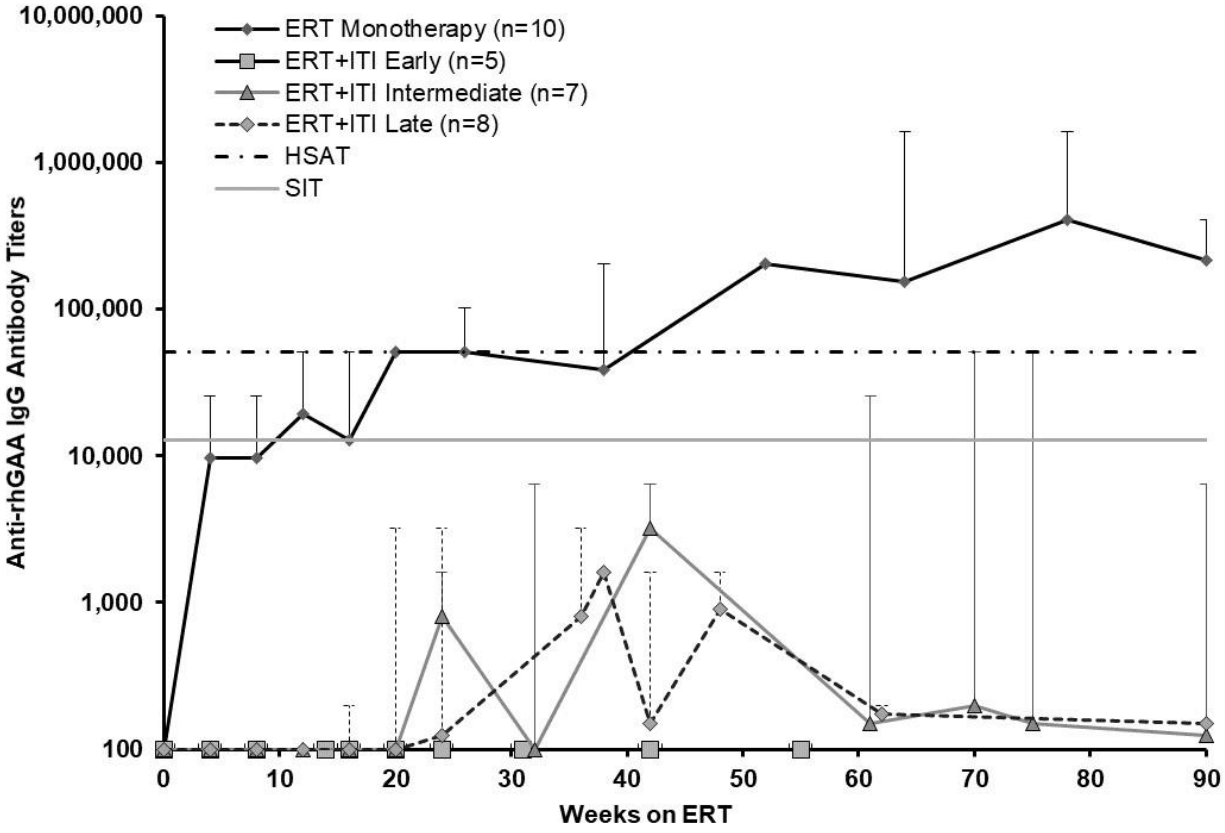
Figure S1. Dose of ERT administered in CRIM-negative IPD patients treated with ERT+ITI



A. Dose of ERT received over the course of study. For consistency, ERT doses of 20 mg/kg weekly were considered to be the same as 40 mg/kg every other week. By the time of final assessment, all patients in the ETG (patients E1-E5) and the ITG (patients I1-I7) were receiving a higher dose than the standard 20 mg/kg EOW. Of the seven patients with available dosing data in the late treatment group (L2-L8), only three were receiving an increased ERT dose by final assessment. The median age at dose escalation was 12.6 months (0-32.1 months, n=5), 25.5 months (8.9-107 months, n=7), and 11.8 months (4.1-102.4 months, n=3) in the ETG, ITG, and LTG, respectively. The median time on ERT at dose escalation was 12.1 months (0-32.0 months, n=5), 24.1 months (7.1-104 months, n=7), and 7.4 months (0.5-98.6 months, n=3) in the ETG, ITG, and LTG respectively. There was no significant difference in either median age or median time on ERT at dose escalation between groups.

B. Lifetime average dose. Lifetime average dose was calculated based on a previously published method, using the product between the proportion of time the patient spent receiving each dose and a multiplier relative to the standard labeled dose of 20 mg/kg EOW. For example, if a patient spent 1/3 of the time receiving 20 mg/kg EOW, and 2/3 of the time receiving 40 mg/kg EOW, the lifetime average dose would be 1 x 1/3 + 2 x 2/3 = 1.67. Both 40 mg/kg EOW and 20 mg/kg W were considered twice the standard dose and assigned a multiplier of 2.

Figure S2. Anti-rhGAA IgG antibodies in patients treated with ERT+ITI and ERT monotherapy



Median peak titer was significantly lower in patients treated with ERT+ITI at 0 (n=20, range 0-51,200) than those treated with ERT monotherapy, who had median peak titer of 204,800 (n=10, range 25,600 – 1,638,400). All patients in the early treatment group remained seronegative.