Introduction Therapeutic options for children with severe asthma are limited. Clinical studies support the use of the anti-IgE antibody, omalizumab, in children with severe atopic asthma. However, children included in these studies had less severe disease than those in whom omalizumab is currently recommended. Little is known about the clinical efficacy of omalizumab in children with severe therapy resistant asthma (STRA).

Objectives To determine the short-term (16 weeks) and long-term (beyond 16 weeks) efficacy of omalizumab, and predictors of a successful therapeutic response in children with STRA in a clinical setting.

Methods This was an observational, prospective study of children with STRA who were commenced on omalizumab. Spirometry, bronchodilator reversibility (BDR), exhaled nitric oxide (FENO), asthma control test (ACT), mini asthma-related quality of life questionnaire (AQLQ), severe exacerbations (requiring a course of oral corticosteroids (OCS) for ≥3 days) and number of unscheduled healthcare visits (UHCV) and hospital admissions were recorded before and every 4 weeks after commencing treatment. Every 16 weeks, patients underwent a more thorough assessment to determine if the treatment should be continued.

Results 33 children (22 male) aged 5–16 years were commenced on omalizumab. At 16 weeks there were significant improvements in mini-AQLQ, ACT; FE_{NO}, maintenance OCS dose; severe exacerbations and UHCVs.

20/33 (60.6%) children continued omalizumab beyond the initial 16 weeks (up to 192 weeks). Compared to those who discontinued, at baseline these children had higher mini-AQLQ (4.28 vs. 3.05) and ACT (11 vs. 8), were younger (11 vs. 13 years) and were more likely to have been admitted to hospital (57.9% vs. 0%) and have had a severe exacerbation (95% vs. 50%) in the 16 weeks before starting omalizumab. Maximal reduction in number of exacerbations and hospital admissions was evident at 32 weeks; this was maintained for up to 144 weeks (Figure 1).

Conclusion This is the first longitudinal study demonstrating long-term clinical efficacy of omalizumab as add-on therapy in children with STRA. Omalizumab was most effective in those with an exacerbation-prone phenotype at baseline, highlighting the importance of thorough patient characterisation when considering this treatment option.