Background: Long-term treatment options of atopic dermatitis (AD) in children are limited. We report efficacy and safety data from the ongoing, long-term LIBERTY AD PED-OLE trial of dupilumab (NCT02612454).
Methods: Patients aged ≥ 6 months to < 18 years with moderate-to-severe AD who had participated in a previous dupilumab study were enrolled in this OLE study. Data reported here include patients aged ≥ 6 years to < 12 years (n = 362 at OLE baseline, n = 309 at Wk 4, n = 34 at Wk 52; data cutoff: July 22, 2019).
Patients received 300 mg every 4 weeks (q4w), which could be up-titrated in case of inadequate clinical response at Week (Wk) 16 as: patients < 60 kg—200 mg q2w; patients ≥ 60 kg—300 mg q2w.
Results: 18% of patients had an Investigator's Global Assessment score of 0/1 at OLE baseline, 24.6% at Wk 4, 37.8% at Wk 28, and 44.1% at Wk52. Mean percent change (standard deviation) from parent study baseline (PSBL) to OLE baseline in Eczema Area and Severity Index (EASI) was − 59.4(36.4), with incremental improvement at Wk 4 (− 71.1 [26.2]), Wk 28 (− 81.8 [17.9]), and Wk 52 (− 85.7 [17.5]). At OLE baseline, 41.2% of patients achieved ≥ 75% reduction in EASI relative to PSBL, increasing to 54.4% at Wk 4, 72.4% at Wk 28 and 79.4% at Wk 52. Treatment-emergent adverse events (TEAEs) were reported in 58.8% of patients; 2.5% of patients had a serious TEAE. Most common TEAEs were AD exacerbation (15.5%) and nasopharyngitis (13.0%).
Conclusions: Long-term treatment with dupilumab showed sustained improvement in signs of AD in the cohort of patients who completed up to 52 weeks. Safety data were consistent with the known dupilumab safety profile.

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Evaluation of rotavirus vaccine adherence for the 22q11.2DS patient population

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Background: 22q11.2 Deletion Syndrome (22q11.2DS) can result in congenital abnormalities including immune dysfunction. International guidelines recommend immune evaluation of 22q11.2DS patients prior to administration of live vaccines [1]. A rotavirus immunization program for infants aged 2 and 4 months was implemented in British Columbia (BC) in January 2012 [2]. Prior to the Rotavirus vaccine, the first live vaccine was administered at 12 months. Adherence to immune workup recommendations prior to 2 months of age in patients with 22q11.2DS and adverse events following immunization is not known.
Methods: A retrospective chart review of children diagnosed with 22q11.2DS in BC from January 1, 2012 to January 1, 2019 was conducted. Demographic, clinical, laboratory, and immunization data and adverse reactions to vaccines were obtained from hospital records. International pediatric consensus guidelines were used as a Reference to determine adherence to guidelines for immunologic workup [1]. Institutional research ethics board approval was obtained.
Results: Records of forty-two children with 22q11.2DS were reviewed and 39 children had immunization records available. Twenty-two out of 39 (56.4%) received at least one dose of a live attenuated rotavirus vaccine. No adverse events following immunization were noted. Ten (25.6%) infants received an immunological assessment prior to rotavirus vaccine administration, and six out of the ten (15.3%) had a CD4+ lymphocyte count higher than the cut-off of 500 × 10^6cells/L to qualify for safe administration of a live attenuated vaccination yet did not receive the Rotavirus vaccine [3].
Conclusions: In this cohort of patients with 22q11 DS, the majority did not receive immunological workup consistent with international guidelines for immunization, though no adverse immunization events were identified. Further assessment is warranted to determine what immunological workup is needed prior to Rotavirus vaccine. Greater dissemination of 22q11.2DS guidelines and improved infant screening for 22q11.2DS in BC is recommended.

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Hereditary angioedema in Canada: Changes in medication use and untreated attacks between the 2017 and 2020 surveys

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Background: HAE is a genetic disease leading to intermittent attacks of angioedema of the face, the extremities, genitalia and the abdomen. Access to treatment may be impacted by the mode of delivery. Many approved medications are delivered intravenously. Newer approved medications are delivered subcutaneously. The oral medications used to treat HAE (androgen and tranexamic acid) are older, not approved for HAE (androgen), have unwanted side effects and are less effective. Newer, effective oral medications are desired by patients with HAE.
Methods: Using data from the HAE Canada patient surveys performed in 2017 and 2020, we analyzed and compared responses to questions on the type of medications used to treat HAE and number of attacks not treated because of access to medication.
Results: IV medication use was similar in 2017 (on-demand: 58.8%, prophylaxis 41.3%) and 2020 (on-demand: 41.3%, short-term (44.0%), long-term (32.5%) prophylaxis). Similarly, there was little change in oral medication use. In 2017: 3.8% for on-demand and prophylaxis; in 2020: 2.5%, on demand, 9.2% short-term and 3.4% long-term prophylaxis. By contrast, subcutaneous medication use has increased from in 2017: 10.0% on-demand, 1.25% prophylaxis to, in 2020, 60.4% on-demand, 28.1% short-term and 38.4% long-term prophylaxis. Percentages may exceed 100% because of multiple categories for use. In 2017, the number of attacks not treated because of medication access was zero for 4.35% of patients while in 2020 it was zero for 59.7%.
Conclusions: The use of newer, self-administered HAE treatments has significantly increased between 2017 and 2020. In parallel, the percent of patients with attacks not treated because of lack of access to medication has decreased significantly. Medications taken at home which are non-invasive and accessible (subcutaneous or oral) are desired by HAE patients and will likely have a significant impact on their quality of life.