Abstract from HAEi Global Leadership Workshop, Frankfurt 6–9 Oct 2022

Focussing on children – treating C1-inhibitor deficiency in childhood

Noémi Andrási[1,2,3], Hanga Horváth[1,2], Zsuzsanna Balla[1,2], Beáta Visy[4], Lilian Varga[1], Henriette Farkas[1].

1) Hungarian Angioedema Center of Reference and Excellence, Department of Internal Medicine and Haematology, Semmelweis University, Budapest, Hungary; 2) School of PhD Studies, Semmelweis University, Budapest, Hungary; 3) 2nd Department of Pediatrics, Semmelweis University, Budapest, Hungary; 4) Heim Pál Children's Hospital, Budapest, Hungary.

Rationale: Hereditary angioedema with C1-inhibitor deficiency is a rare, life-threatening disease. Recurrent oedematous (HAE) attacks may occur at any age. Early diagnosis is critical since it enables the provision of targeted treatment for the patients.

Methods: We analyzed the clinical data (diagnosis, symptom onset, number of HAE attacks, used treatment) of 25 pediatric patients (mean age: 9.53 years) treated and followed-up at the Hungarian Angioedema Center of Reference and Excellence in 2021.

Results: Out of the 25 pediatric patients 13 experienced HAE attacks, out of whom 4/13 experienced it prior to the diagnosis. The average age of symptom onset was 5,87 years, while average age at diagnosis was 4,11 years. The first HAE attacks in the 13 symptomatic patients affected: the genitalia (1), the face (4), the neck (1), limbs (6), and the abdomen (1). In 6 cases, plasma-derived C1-inhibitor (pdC1-INH) was applied, in 1 case, although pdC1-INH treatment was available, calcium was taken, in 1 case, tranexamic acid was applied, while in 5 cases, no treatment was administered. In 2020 a total of 70 HAE attacks occurred, which affected 9 children. 42/70 attacks affected the limbs, 9/70 the gastrointestinal tract, 3/70 the genitalia, 1/70 the back, while 13/70 were multilocal. 70% (49/70) of the attacks were experienced by 2 patients. Long-term prophylaxis (lanadelumab) was applied in the case of 1 patient. Out of the 70 HAE attacks, acute treatment was introduced in 39 cases: in 28 cases, pdC1-INH, in 3 cases, recombinant C1-INH, in 4 cases, icatibant, and in 4 cases, icatibant and pdC1-INH were applied. In the case of 31 HAE attacks, no medical treatment was administered.

Conclusions: In childhood, acute treatment is usually sufficient to support adequate quality of life, although in some cases, long- or short-term prophylaxis may be necessary. Regular follow-up is essential since the course of the disease may change from year to year.