HAE Australasia

hope • advocacy • education

Australasian Patient Organisation for Hereditary Angioedema

Patient and Carer Webinar Series 2024

HAE In Children and Young People

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Acknowledgement of Country

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We would like to begin by acknowledging the Traditional Owners of the land on which we meet today. We would also like to pay respects to Elders past and present.

HAE IN CHILDREN AND YOUNG PEOPLE

- When is it appropriate to test children for HAE
- When do children generally develop HAE symptoms
- What symptoms do children show
- How do we treat children with HAE
- Future considerations

WHEN TO TEST CHILDREN FOR HAE

- Focusing on HAE 1 and 2. HAE with normal complement is very rare in children
- Consideration testing in asymptomatic child or if expected birth in family
- 50% chance of inheritance with positive family history (AD)

    - Test at birth (blood/cord blood)
    - Test in the first 6 months
    - Test 6-12 months
    - 1-2 years
    - Only when symptomatic

WHEN TO TEST CHILDREN FOR HAE

- No right or wrong answer for each family/child
- Recommend to test before child is symptomatic
    - Usually recommend testing when at least 12m age
    - Cord blood or testing soon after birth can give false results
    - Functional testing C4 and C1 Est Inhibitor is very good test
    - Some places offer genetics (SERPING) but usually not required
    - Genetic useful in confirming the status of young children who manifest normal or near-normal test with concerning Hx & FamHx

WHAT AGE DO HAE ATTACKS START

- Some children present early with frequent attacks, this can be associated with more severe disease
- Severe first episodes are rare in childhood
- Rare to have attacks in children before 2 years of age
- Some countries have evidence of early onset among their population (US, Germany) while other countries report later onset for most patients (UK). We tend to have an onset experience similar to the UK.
- Majority develop symptoms 4-10 years age. 90% sxs <20y age
- The number of attacks increase around teenage years
- Some pts are asymptomatic despite mutation (Risk severe attack later on)

WHAT SYMPTOMS DO CHILDREN HAVE

- Similar symptoms to adults. Slow onset over days 2-5d
- Severe attacks are rare, particularly <10y
- Tend to have abdominal attacks. Difficult to work out (~50%)
- Plenty of peripheral swellings as well (~50%)
- Laryngeal attacks are rare. Unfortunately assoc with undiagnosed HAE
- Fatal attacks very rare
- Worsening & more frequency with pubertal changes
- Triggered by illness, stress, trauma, hormonal changes (40-50%)

HOW DO WE TREAT CHILDREN

- Most of treatments are injections & can exacerbate distress in children
- Aim for least invasive treatment & upskill families for self-admin
- Discussion with families early regarding attacks/burden
- Discussion around On demand treatment, short term prophylaxis and longer term prophylaxis
- Review HAE action plan & regularly by following up with your Health Care Professional/Local health team

ON DEMAND TREATMENT

BERINERT. C1 Esterase inhibitor. Plasma derived C1 esterase inhibitor (Human). Indicated for acute treatment of attacks in adults and children. Delivered IV. 20U/kg. Approved for self-administration after education. Funded by NBA

Adults and children

Self-administered

Intravenous

ICATIBANT. Bradykinin B2 receptor antagonist. Indicated for treatment of acute attacks of HAE in adults, adolescent and children >2y. Delivered SC. Approved for self-administration after education. Funded by PBS

Adults and children

Self-administered

Subcutaneous

SHORT TERM PROPHYLAXIS

BERINERT. C1 Esterase inhibitor. Plasma derived C1 esterase inhibitor (Human). Indicated for acute treatment of attacks in adults and children. Delivered IV. 20u/kg. Usually given 1-6h before surgery/dental. Not for self-administration Funded by NBA

Adults and children

Self-administered

Intravenous

LONG TERM PROPHYLAXIS

BERINERT. C1 Esterase inhibitor. Plasma derived C1 esterase inhibitor (Human). Indicated for prophylaxis in adults and children. Delivered IV/SC 40-60U/kg. twice weekly. Approved for self-administration after education. Funded by NBA

Adults and children

Self-administered

Intravenous

LANADELUMAB. mAb inhibits plasma kallikrein. Indicated for long term prophylaxis in adolescents and adults HAE. Delivered SC. 300mg Q2-4/52.Approved for self-administration after education. Funded by PBS

Adults and children

Self-administered

Subcutaneous

OTHER POINTS ON TREATMENT

- Tranexamic acid can be considered for long term prophylaxis although limited efficacy. < 10% pts respond
- Androgens are no longer used
- Icatibant has helped change landscape in childhood HAE. Simple to administer. Not a lot to remember/forget compared to other prescribed medications. Very useful early on. Dose based on weight
- Self- administration has been key in improving Quality of Life

CONSIDERATIONS AND FUTUERE

- More prescriptions options & self-admin available. Better Quality of Life for Patients
- Newer oral kallikrein inhibitors in horizon (Some in use in USA/CANADA/EU)
- Importance of knowing diagnosis & family history. Diagnosis and test after 1y age
- Ensure action plans are up to date and discuss with your Health Care Practitioner early
- Seek support from consumer organisations (HAE Australasia)
- Have clear plan for on demand treatment and discuss prophylaxis treatment (Short team and long term)
- Manage burden of disease. Minimise impact, pain, absence from school & reduce hospitalisations
- On demand treatment at home should be discussed each time. Early intervention is better

Thank you for joining us