

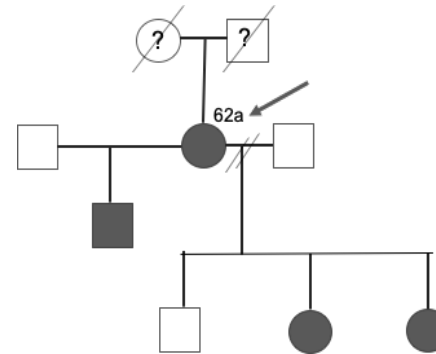
What are the goals of prophylaxis in HAE?

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Why is this topic important?



62 years



*with permission

18 years

Onset of symptoms

52 years

HAE-C1-INH diagnosis

Jan 2010–Oct 2015:

- Stanozolol (1 attack every 3–4 months)

March 2016:

- Danazol

60 years

February 2019:

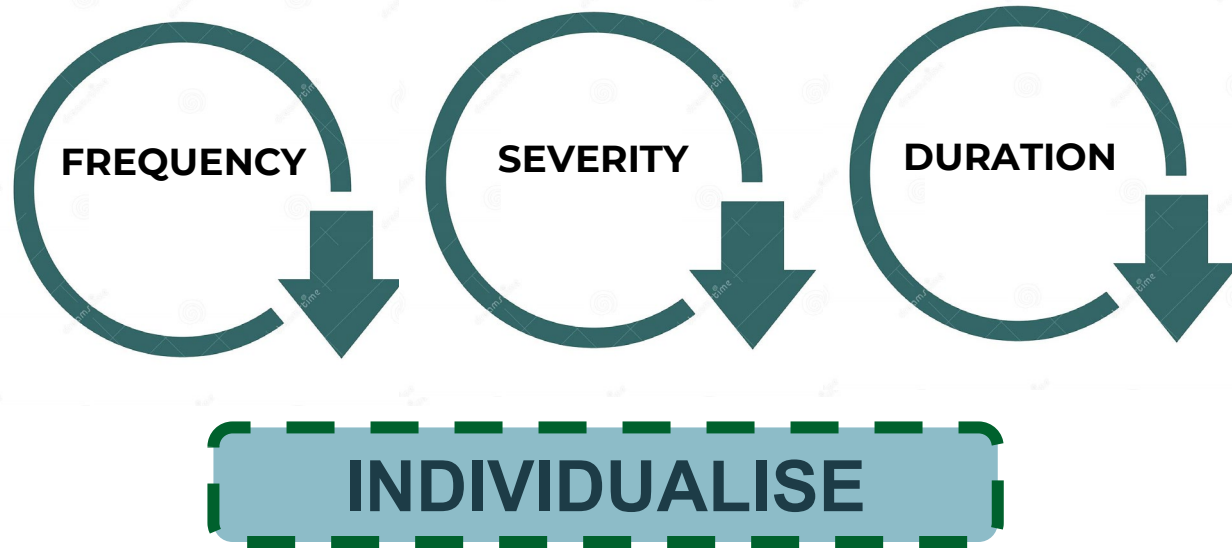
- Dyslipidaemia
- Reduced danazol dose
- 1 attack/month (managed with icatibant)
- Refused LTP with pdC1-INH IV

61 years

June 2020:

- 3–6 attacks/month (abdominal, peripheral, 2 laryngeal)
- AE-QoL 38

What does the science say?



2019 International/Canadian guideline¹

“The decision to start or stop LTP depends on multiple factors and should be made by the patient and an HAE specialist”:

Level of Evidence: Consensus (97.06% agrees)

Strength of recommendation: Strong (97.06% agrees)

- ✓ Reduce the burden of disease
- ✓ Improve quality of life
- ✓ Reduce activity of the disease
- ✓ Achieve control of disease



HAE, hereditary angioedema; LTP, long-term prophylaxis.

1. Betschel et al. *Allergy, Asthma Clin Immunol.* 2019;15:72; 2. Craig et al. *Ann Allergy Asthma Immunol.* 2018;121(6):673–679; 3. Maurer et al. *World Allergy Organ J.* 2018;11:5.

What does the science say?

From now on, treatment goals should be:

- ✓ **Achieve total control of disease – no attacks**
- ✓ **Normalise the patient's life**

The following should be considered when determining whether the patient's life is normalised:

Number of hours of activity impairment

Number of days of sick leave

Mean length of attack-free period

Number of ER visits

Hospitalisations

My experiences

How was our patient treated with this goal in mind?

- Review the potential triggers/associated comorbidities
- Need for effective LTP was clear (*severe and frequent attacks, high impact on QoL, able to self-administer IV*)
- Stopped androgens
- Educational programme for self-administration of pdC1NH SC
- pdC1-INH SC twice/week → 4000 IU twice/week → no attacks
- **AE-QoL: 18**
- AECT 13



62 years



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ULTIMATE GOAL: ACHIEVE COMPLETE CONTROL AND NORMALISE THE PATIENT'S LIFE