

Managing HAE in a severely affected patient



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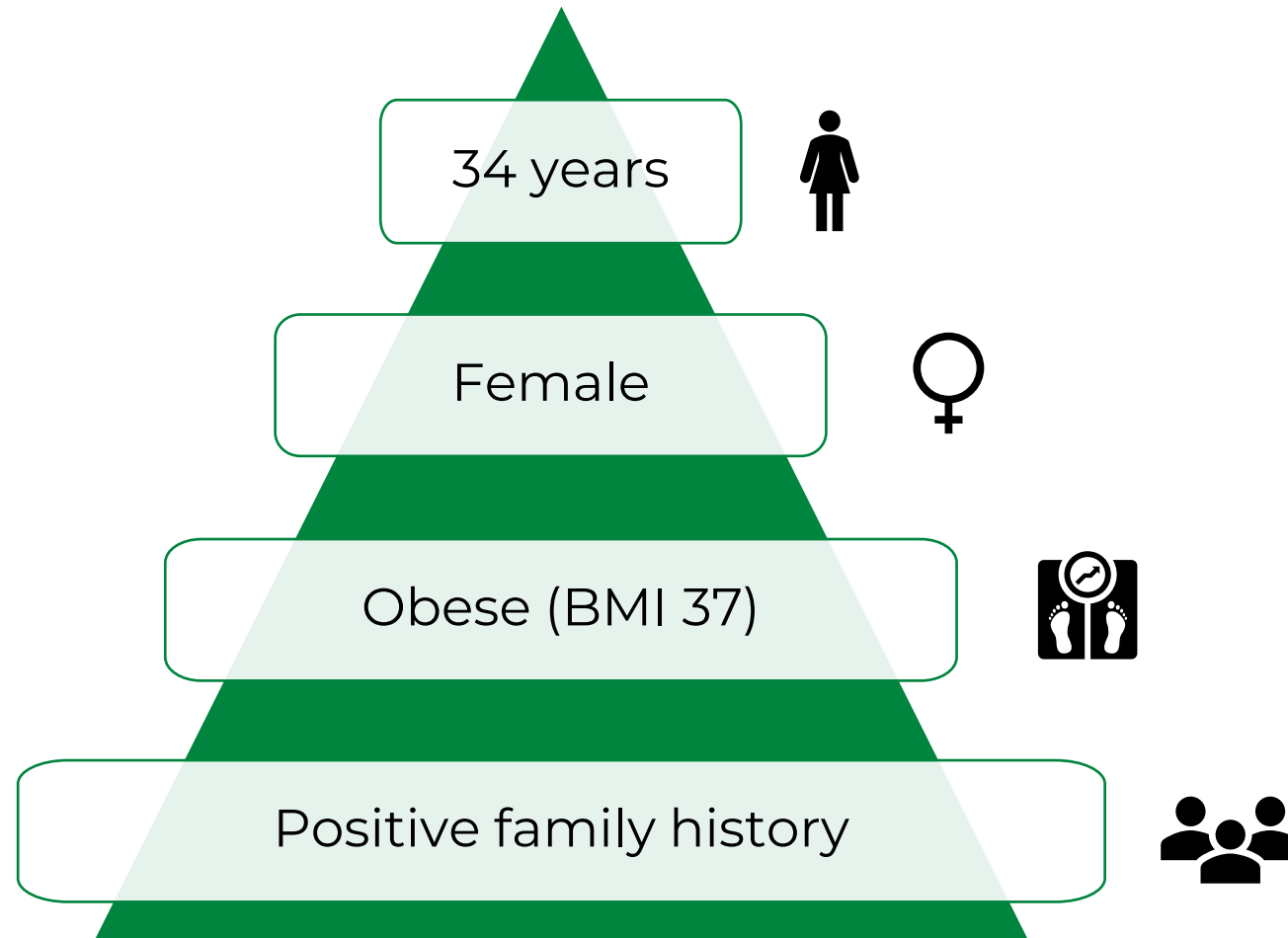
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- Speaker and/or consultancy fees received from CSL Behring, Shire and Takeda Pharmaceutical Co. Ltd.
- Served as a Principal Investigator for clinical trials sponsored by BioCryst Pharmaceuticals, Pharvaris Netherlands BV, Pharming Group NV, CSL Behring and KalVista Pharmaceuticals

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- The views, information or opinions expressed herein are those of the speaker; they do not necessarily reflect those of CSL Behring
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Patient description

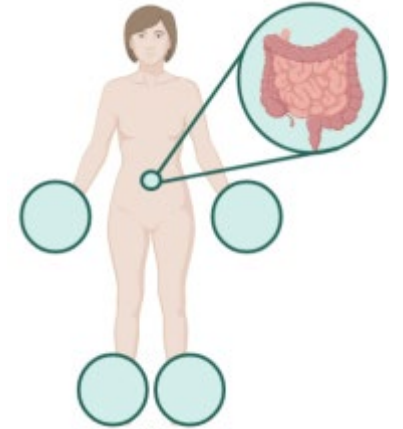


Case history

- Family history of HAE
 - Mother, sister and son



- One year old when first HAE attack occurred
 - First symptoms were irregular in frequency
 - Attack locations were specific to **GI** and **peripheral regions**
- Fluctuation in frequency of attacks beginning in 2000
 - Ranging from irregular attacks to over 80 per year



Diagnosis

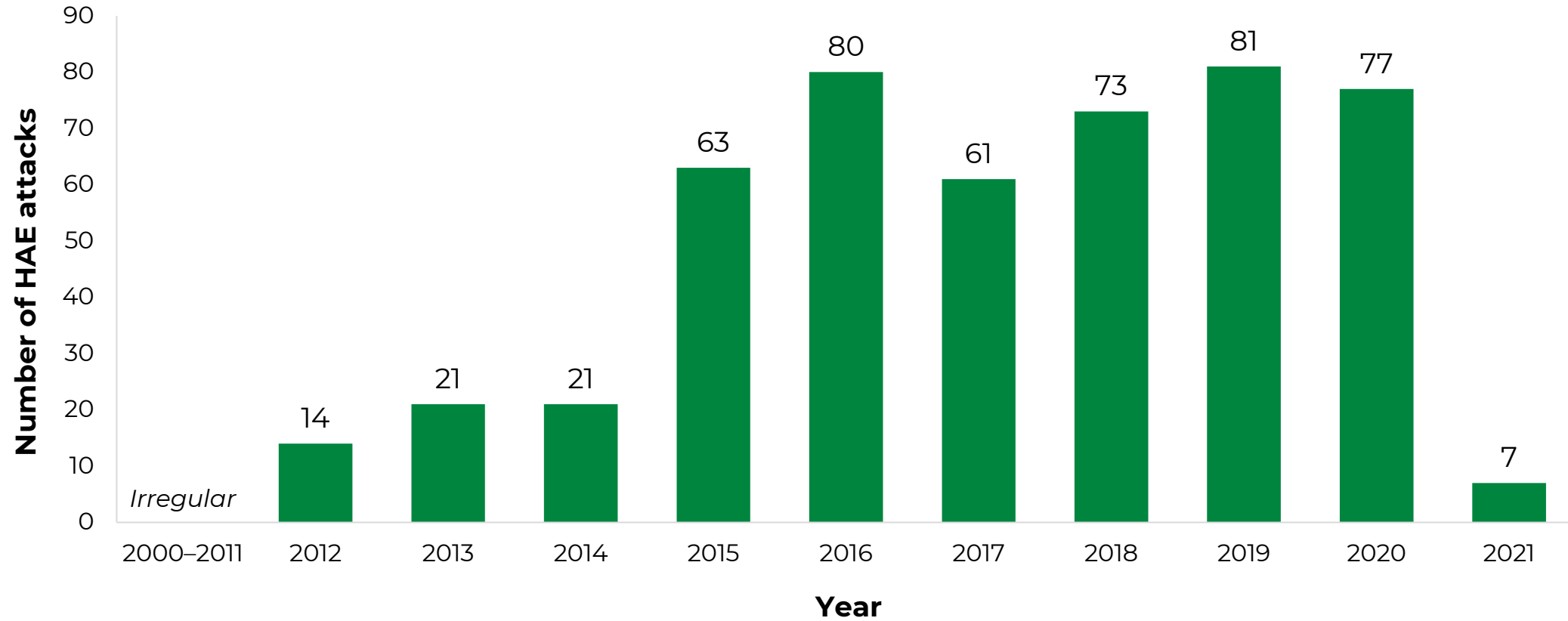
- Diagnostic delay of 12 years (diagnosis at age 13)
 - Specialty of physicians → Allergy
- Given the patient's family history, the following investigations were performed:
 - C1-INH concentration
 - C1-INH function
 - Genetic testing

Patient was diagnosed with HAE type I

↓ C1-INH ↓ function C1-INH



HAE attack history



Treatment plan

- On-demand therapy

Since 2000 - **Plasma-derived C1-INH (pdC1-INH)** 1500–2000 IU IV

Since 2011 - **Icatibant** 30 mg SC

Since 2014 - **Recombinant human C1-INH (rC1-INH)** 4200 IU IV

- LTP

2001–2014 - **Tranexamic acid**

2006 - **Attenuated androgens**

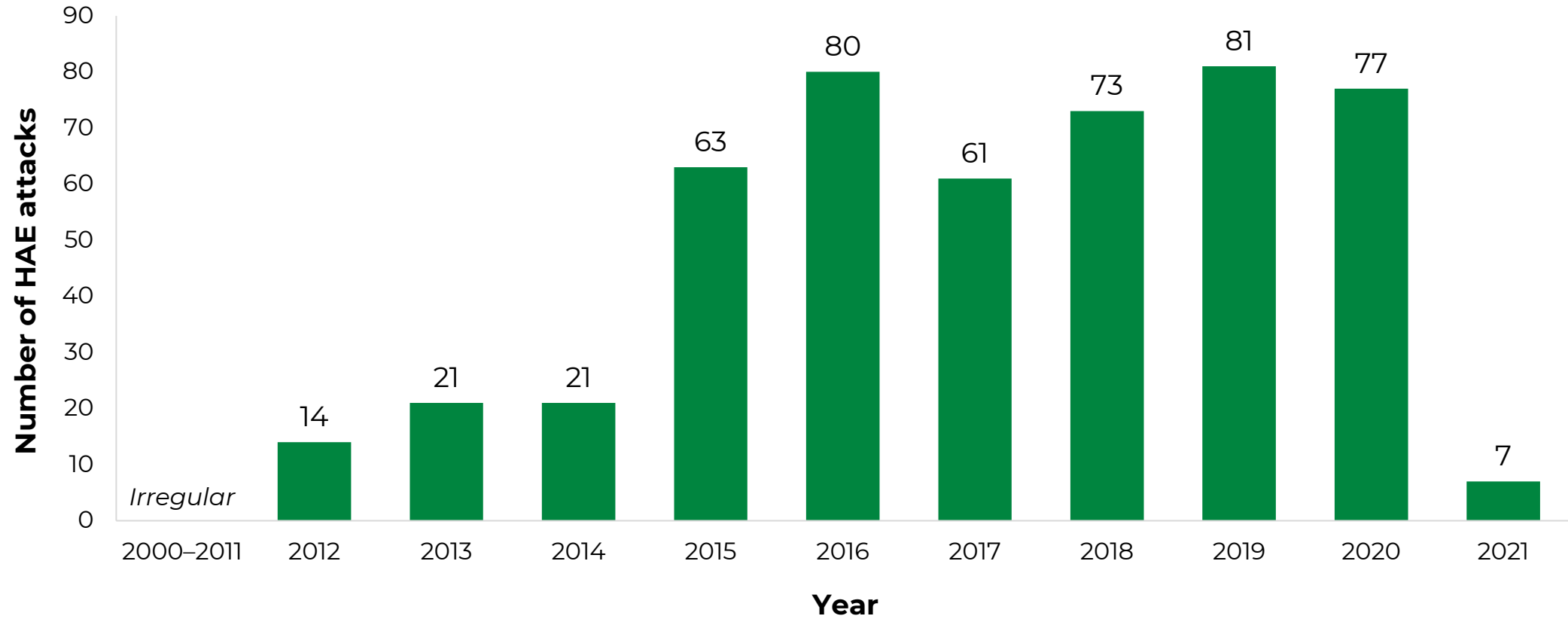
2015 - **rC1-INH** – clinical trial

2020 - **pdC1-INH** SC 3000 60 IU/kg twice weekly

2021 - **pdC1-INH** SC 3000 40 IU/kg twice weekly*

**The approved dosing of C1-INH (SC) is 60 IU/kg twice weekly. CSL Behring does not suggest or recommend the use of C1-INH (SC) in any way other than as described in the Summary of Product Characteristics.*

HAE attack history



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Outcome of treatment plan

HCPs' perspective



Enjoys a normal life and plays sport as a result of fewer attacks



Weight reduction observed in patient



Patient to continue LTP with pdC1-INH SC 3000, 40 IU/kg twice weekly*

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Poll: What is the typical age category for first symptoms in your patients?

- A. 1–10
- B. 11–20
- C. 21–30
- D. 31–40

Poll: In your experience, is there an association between age of patients at symptom onset and severity of the disease?

A. Yes

B. No

C. Unsure

Take-home messages

HAE is an unpredictable disease

Symptoms vary widely in frequency, location, and severity, even within a family

Treatment strategy must be adjusted individually for each patient

Thank you for your attention

