Ask the HAE experts:
Treating special clinical cases

1st December, 2021

18:30–20:00 CET

HAExpert
HAE eXchange Programme for ExpeRTs

In collaboration with ACARE
Angioedema Centers of Reference and Excellence
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CSL Behring
Biotherapies for Life™

EUR-BRN-0329

Ask the HAE experts: Treating special clinical cases

Chair	
Marcus Maurer	Angioedema Center of Reference and Excellence (ACARE) Department of Dermatology and Allergy Charité - Universitätsmedizin Berlin Berlin, Germany

Speakers	
Danny Cohn	Angioedema Center of Reference and Excellence (ACARE) Department of Vascular Medicine Amsterdam UMC, The University of Amsterdam Amsterdam, Netherlands
Maria Pedrosa	Department of Allergy La Paz University Hospital Madrid, Spain
Petra Staubach-Renz	Angioedema Center of Reference and Excellence (ACARE) Department of Dermatology University Medical Center Mainz Mainz, Germany
Roman Hakl	Department of Clinical Immunology and Allergology St Anne's University Hospital in Brno Faculty of Medicine, Masaryk University Brno, Czech Republic

Poll: Which country are you joining from today?

More information about our esteemed faculty can be found here

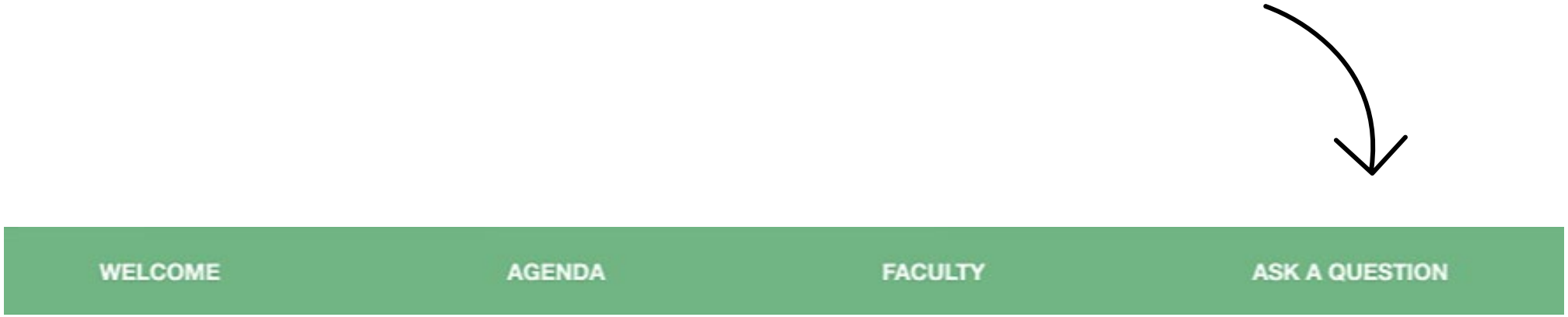
Webinar Agenda

Time (CET)	Session	Speaker
18:30–18:35	**Welcome and introduction**	Marcus Maurer (Chair)
18:35–18:50	**Treating HAE during pregnancy** • Presentation of case (10 min) • Discussion (5 min)	Danny Cohn
18:50–19:05	**Managing the transitioning HAE adolescent** • Presentation of case (10 min) • Discussion (5 min)	Maria Pedrosa
19:05–19:20	**Patients with HAE and comorbidities** • Presentation of case (10 min) • Discussion (5 min)	Petra Staubach-Renz
19:20–19:35	**Managing HAE in a severely affected patient** • Presentation of case (10 min) • Discussion (5 min)	Roman Hakl
19:35–19:55	**Q&A**	All
19:55–20:00	**Summary and close**	Marcus Maurer (Chair)

HAE, hereditary angioedema; Q&A, question and answer

Join the discussion!

We encourage you to submit questions during the webinar via this tab



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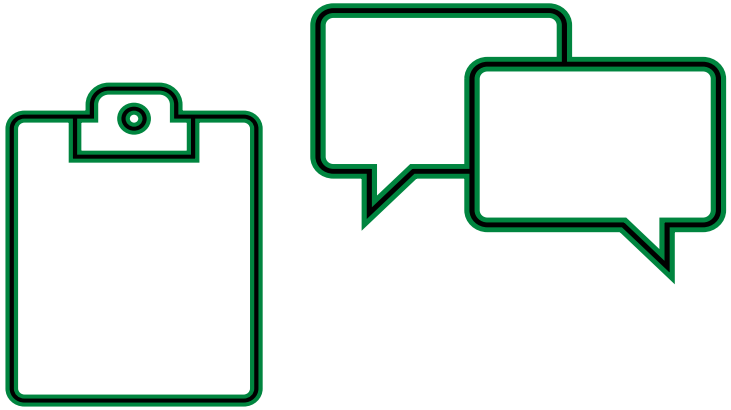
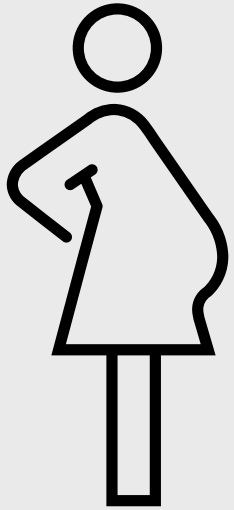


Treating HAE during pregnancy



Dr. Danny Cohn

ACARE Center Amsterdam UMC

University of Amsterdam

Netherlands

Disclosures

- Speaking and/or consultancy fees from Biocryst, CSL Behring, Ionis Pharmaceuticals, KalVistaTakeda, Pharming, Pharvaris and Sanofi/Genzyme

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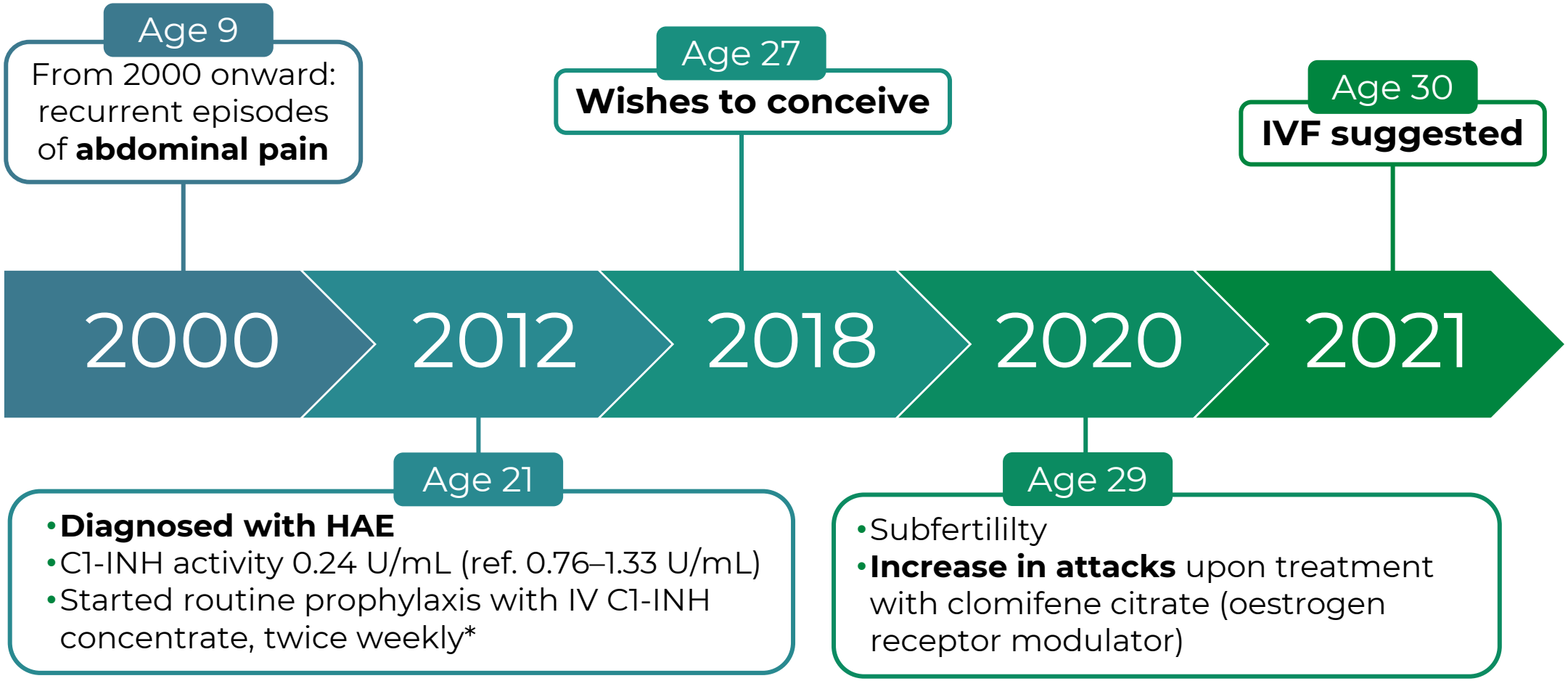
Patient case

- 30-year-old female with HAE
- Frequent **abdominal attacks** despite routine prophylaxis with IV C1-INH concentrate*
- Wish to conceive
- **Emotional distress**

**The use of intravenous C1 inhibitor for long-term prophylaxis is dependent on manufacturer's Summary of Product Characteristics*

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IV, intravenous

Case history



**The use of intravenous C1 inhibitor for long-term prophylaxis is dependent on manufacturer's Summary of Product Characteristics*

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IV, intravenous; IVF, in-vitro fertilisation

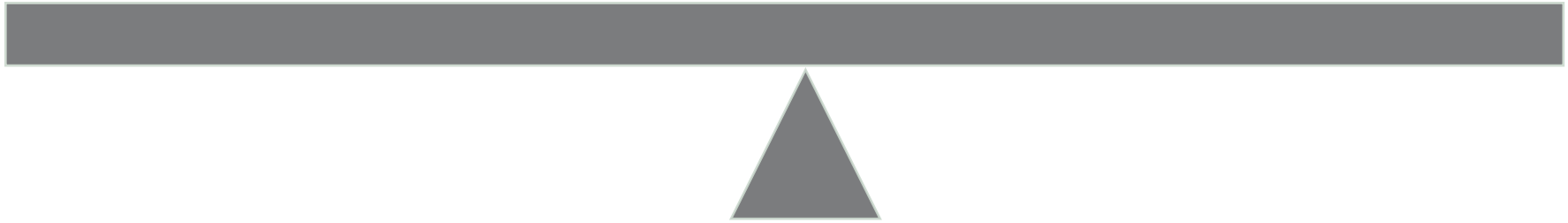
Conflicting treatment goals in pregnancy

IVF/pregnancy

- Stimulation of ovulation may induce angioedema attacks
- Emotional distress may even further evoke angioedema attacks
- Treatment options limited to pdC1-INH concentrates

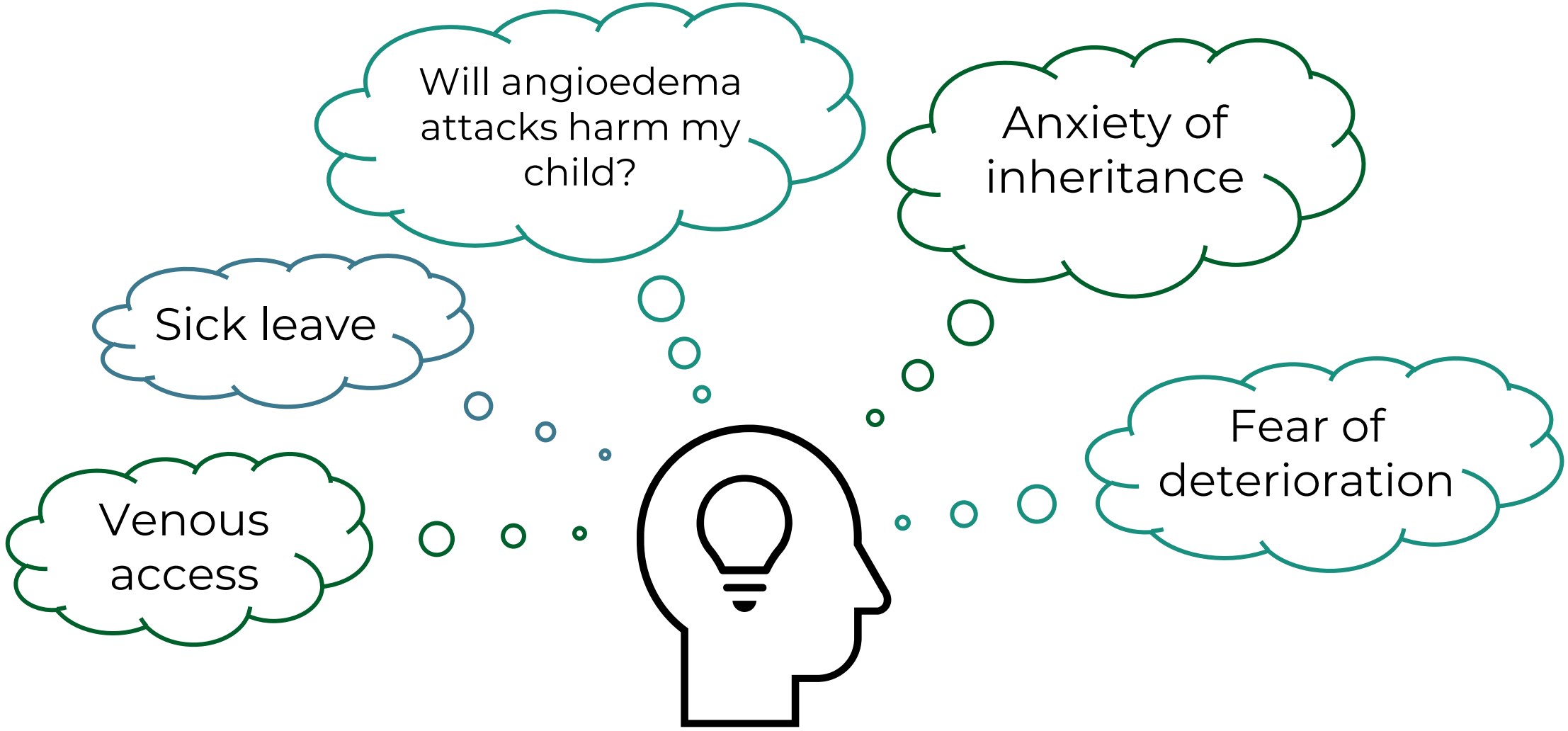
Improved HAE control

- More treatment options available for both prophylaxis and acute treatment
- Aimed at reduced oestrogen levels



HAE, hereditary angioedema; IVF, in-vitro fertilisation; pdC1-INH, plasma-derived C1-esterase inhibitor

Patient’s concerns regarding IVF and pregnancy



IVF, in-vitro fertilisation

Poll: Which strategy would you recommend for this patient?

A. Increase dose of IV C1-INH concentrate

B. Reduce dosing interval of IV C1-INH concentrate

C. Consider other route of administration of C1-INH concentrate

D. Refer to psychologist with HAE expertise

E. Wait and see

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IV, intravenous

Course 2021

- IVF postponed
- Continued angioedema attacks despite frequent IV C1-INH concentrate use
- Poor disease control (AECT 7)

AECT, angioedema control test; C1-INH, C1-esterase inhibitor; IV, intravenous; IVF, in-vitro fertilisation

Course 2021

- IVF postponed
- Continued angioedema attacks despite frequent IV C1-INH concentrate use
- Poor disease control (AECT 7)

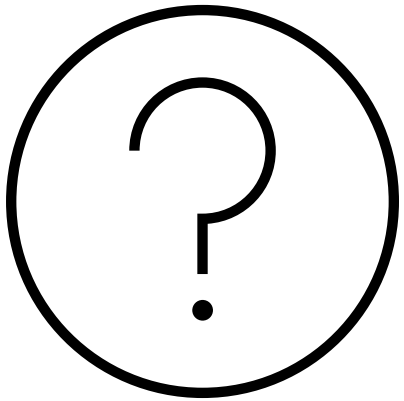
- Compassionate use of SC C1-INH granted
- 2 minor attacks in three months, AECT improved (AECT 14)
- Increased confidence in HAE course during a future pregnancy

**According to the Summary of Product Characteristics of SC C1-INH (Berinert® 2000/3000) or IV C1-INH (Berinert® 500/1500), there are limited data that suggests no increased risk from the use of C1-INH products in pregnant women*

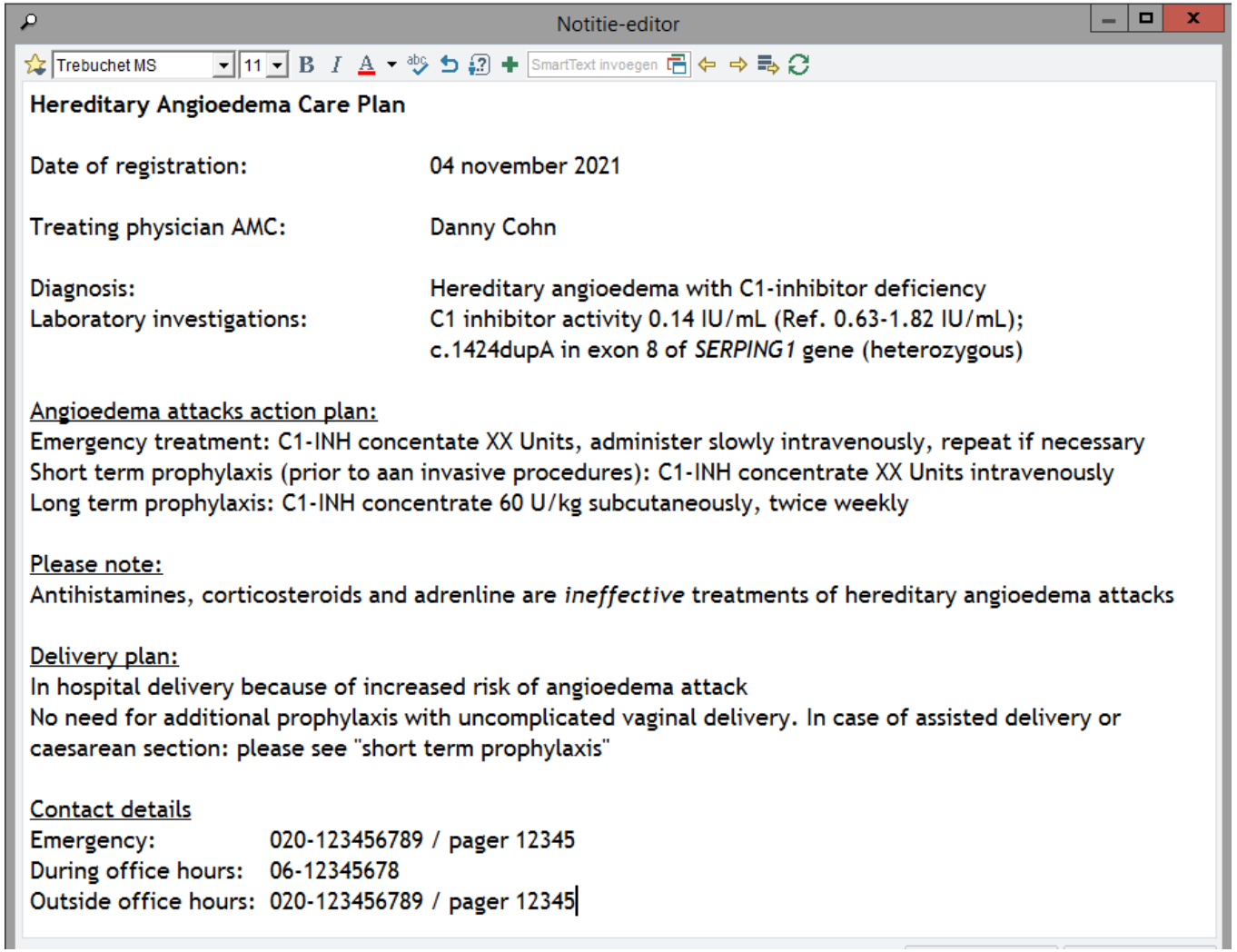
AECT, angioedema control test; C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IV, intravenous; IVF, in-vitro fertilisation; SC, subcutaneous

Question to the panel

What special recommendations do you have for an individualised care plan regarding pregnancy and delivery?



Individual care plan

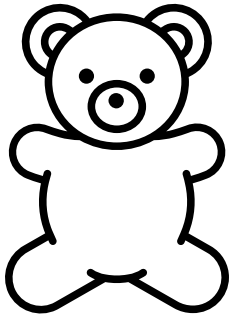


Delivery and HAE

In-hospital delivery recommended

No need for preprocedural prophylaxis if unassisted vaginal delivery

Acute treatment readily available



HAE, hereditary angioedema

Take-home messages

IVF and pregnancy may increase angioedema attacks for multiple reasons:
- Emotional distress
- Stimulation of ovulation
- Increased oestrogen levels

HAE treatment options are limited to pdC1-INH concentrate in pregnancy

Personalised treatment and delivery plan highly recommended

IVF, in-vitro fertilisation; pdC1-INH, plasma-derived C1-esterase inhibitor

Managing the transitioning HAE adolescent

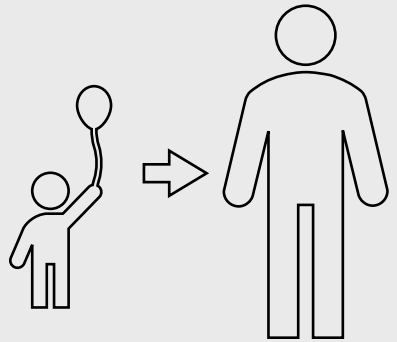
Adolescence: More than just hormones

Dr. María Pedrosa

Department of Allergy

La Paz University Hospital

Madrid, Spain



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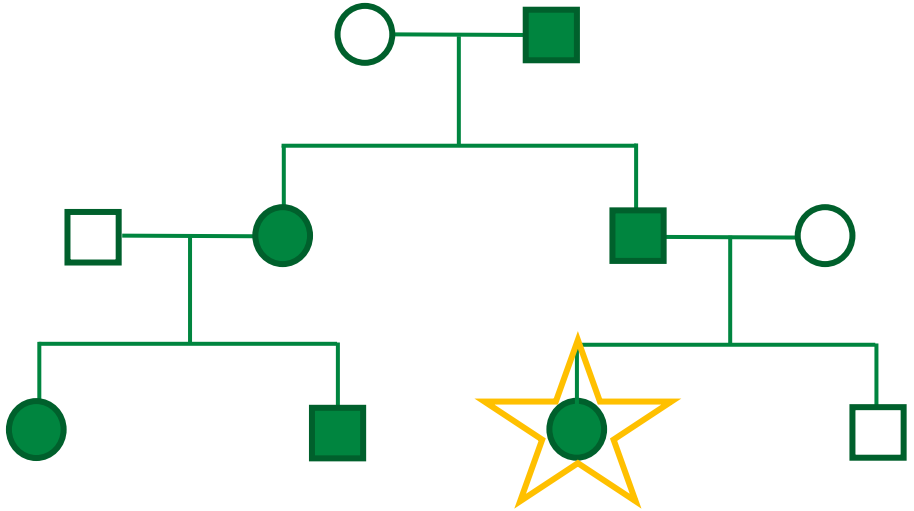
Patient description

- 15-year-old female (born in 2006)
- Family history of angioedema (father)
- Diagnosed at screening
  - C1-INH: 6.17 mg/dL (normal range 12.00–25.00 mg/dL)
  - Functional C1-INH: 26.84% (normal range ≥38%)

Action plan:

- On-demand treatment in case of acute attack with IV pdC1-INH 20 IU/kg
- Pre-procedural prophylaxis with IV pdC1-INH 20 IU/kg



C1-INH, C1-esterase inhibitor; IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor

Case history of attacks

2013

- Age 7
  - Angioedema of the right hand lasting 2 days, accompanied by abdominal pain ➡ Emergency room
  - Treated with IV pdC1-INH resulting in improvement within 1 hour

2014–2015

- Patient suffered 5–7 attacks per year

Action plan:

- On-demand in case of acute attack with IV pdC1-INH 20 IU/kg
- Pre-procedural prophylaxis with IV pdC1-INH 20 IU/kg

IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor

Case history of attacks

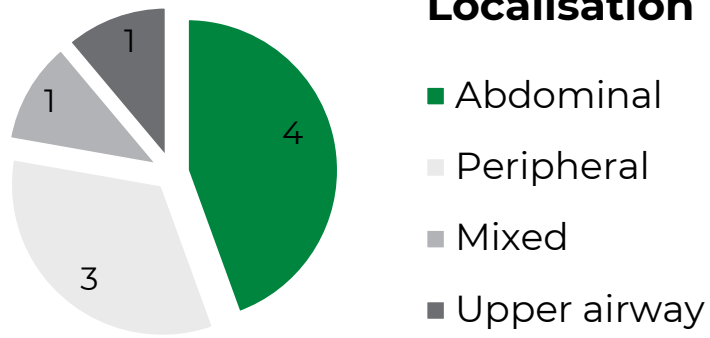
2016

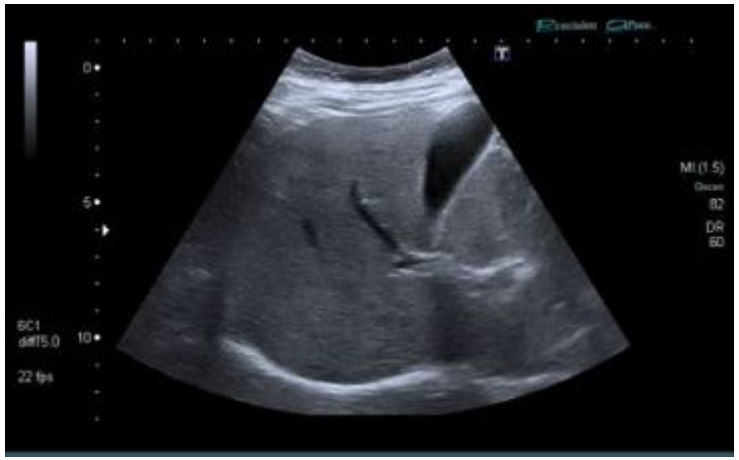
- Age 10
- Patient suffered 9 attacks within a 6-month period

Action plan:

- Long-term prophylactic treatment with tranexamic acid 40 mg/kg/day
- Pre-procedural prophylaxis with IV pdC1-INH 20 IU/kg

- *Helicobacter pylori* was ruled out
- Abdominal ultrasound showed a fatty liver





IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor

Treatment options for children and adolescents in Europe

On-demand	Administration
Plasma-derived C1-INH	IV*
Recombinant C1-INH (≥2 years)	IV
Icatibant (≥2 years)	SC
Pre-procedural prophylaxis	**Administration**
Plasma-derived C1-INH	IV*
Long-term prophylaxis	**Administration**
Plasma-derived C1-INH (≥6 years)	IV†
Plasma-derived C1-INH (≥12 years)	SC
Lanadelumab (≥12 years)	SC
Berotralstat (≥12 years)	Oral
Tranexamic acid	Oral

Due to adverse effects, attenuated androgens are no longer recommended for long-term prophylaxis in children or adolescents prior to Tanner Stage 5 with HAE[1]

*Indications for each product are according to European Prescribing Information. *Indicated for all ages, depending on manufacturer. †Depending on manufacturer.*

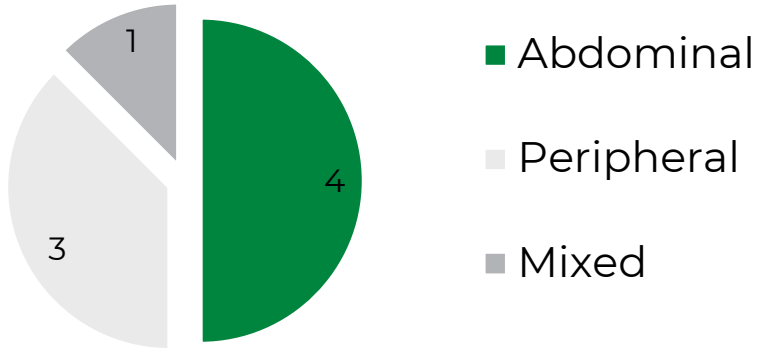
C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IV, intravenous; SC, subcutaneous

1. Maurer M. *et al. World Allergy Organ J*. 2018; 73(8):1575–1596.

Treatment with tranexamic acid

Mid 2016–mid 2017

- Age 10
- 8 attacks
- Tranexamic acid was suspended
  - Not effective
- Progestins were evaluated but finally discarded as per consensus with endocrinology



Action plan:

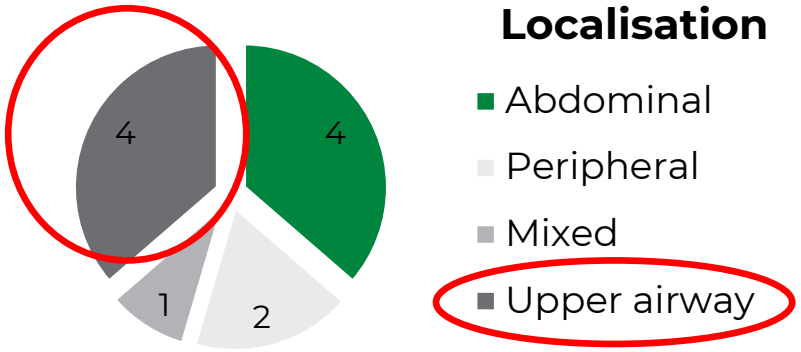
- On-demand in case of acute attack with IV pdC1-INH 20 IU/kg or icatibant acetate
- Pre-procedural prophylaxis with IV pdC1-INH 20 IU/kg

IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor

On-demand treatment

Mid 2017–mid 2018

- Age 11
- Patient suffered 11 attacks
- Self-administration with IV pdC1-INH
- SC icatibant acetate
  - Patient prefers not to use, influenced by her father's experience
- Does not treat peripheral attacks
  - Does not want to administer at school
  - Does not want to go home for treatment and miss out at school



IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor; SC, subcutaneous

Considerations for school children

- It is important that teachers and school nurses are aware of symptoms and the treatment of HAE
- However, there are issues surrounding teachers' willingness to treat
  - Liability
  - Do not want to take the risk of administering treatment in case the outcome is not desirable

Poll: Which factor do you think is the most important when selecting a treatment/developing a treatment plan for adolescents?

A. Current frequency and severity of attacks

B. Quality of life

C. Patient preference

D. Patient lifestyle and activity

Triggers

- Age 12
- Exam period increased attack frequency:
  - Missed some exams
  - Poor academic results
- Psychological stress
  - Felt different from friends
  - Self-excluded from activities
  - Social isolation

Action plan:

- Short-term prophylactic treatment with IV pdC1-INH 20 IU/kg twice weekly during the exam period*

**Berinert® 500/1500 (C1-INH IV) is approved for the treatment and pre-procedure prevention of acute episodes of HAE. CSL Behring does not suggest or recommend the use of C1-INH (IV) in any way other than as described in the Summary of Product Characteristics.*

C1-INH, C1-esterase inhibitor; IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor

Impact on quality of life

- Quality of life impairment:
  - Anxiety
  - Fear of attacks (unpredictability)
  - Avoided after-school activities
  - Avoided travelling without parents' supervision

Action plan:

- Short-term prophylactic treatment with IV pdC1-INH 20 IU/kg before school trips or visiting grandparents on her own*

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C1-INH, C1-esterase inhibitor; IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor.

Long-term prophylaxis

- Despite STP, the patient suffered 17 attacks in 6 months

- Therefore, at age 13 when SC LTP became available, the patient switched to SC pdC1-INH 40 IU/kg twice weekly*
  - Asymptomatic for 9 months
  - Improvement in quality of life

- Patient decided to suspend LTP
  - Fewer triggers during lockdown (2020) – no school etc. ?
  - Uses on-demand therapy to treat attacks (1 attack every 2–3 months)
  - Patient is now more efficient at treating on demand

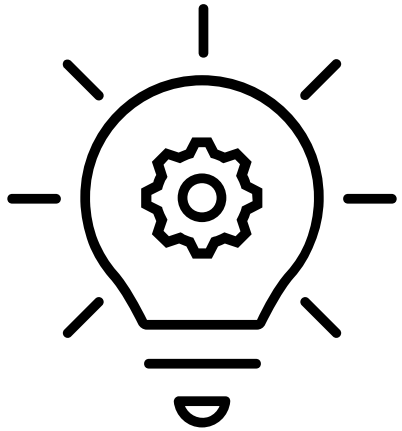
**The approved dosing of C1-INH (SC) is 60 IU/kg twice weekly. CSL Behring does not suggest or recommend the use of C1-INH (SC) in any way other than as described in the Summary of Product Characteristics.*

C1-INH, C1-esterase inhibitor; IU, international units; IV, intravenous; pdC1-INH, plasma-derived C1-esterase inhibitor; SC, subcutaneous; STP, short-term prophylaxis; LTP, long-term prophylaxis

Take-home messages

Older children may hide their symptoms due to anxiety or fear of social isolation

HAE is dynamic during adolescence and the psychological impact of the disease should be proactively assessed



Impact of HAE attacks on school attendance/performance may prevent future career or education opportunities

Treatment plans should be continually re-assessed

Self-administration in young adolescence is feasible

As the disease is constantly changing in adolescence, the indication of LTP should be carefully evaluated

HAE, hereditary angioedema; LTP, long-term prophylaxis

Patients with HAE and comorbidities

Prof. Dr. Petra Staubach-Renz

Angioedema Center of Reference and Excellence (ACARE)

Department of Dermatology

University Medical Center Mainz

Mainz, Germany

Disclosures

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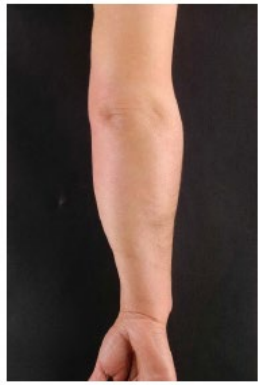
Patient description

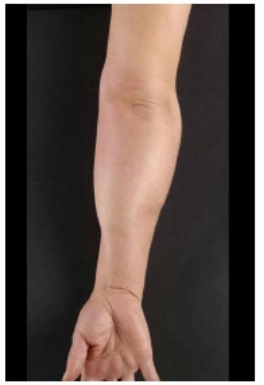
- Elderly, female patient aged 62
- Diagnosed with HAE type I
- Experiences severe HAE, with a high frequency of attacks in the last 40+ years
- Suffers from comorbidities such as **depression** and **hypertension**
- Profession: Cleaner (6 hours per day)

Case history

1978 (first symptoms aged 19)

- 3 months after pregnancy
- Swelling of the skin and abdomen biweekly





Treatment before diagnosis:

- Celestamine/antihistamines on-demand therapy (approx. 30 doses) – unsuccessful
- Celestamine oral tablets (1 per day for over 1 year)
- Celestan depot IV administrations (approx. 10 injections during treatment)

HAE, hereditary angioedema; IV, intravenous

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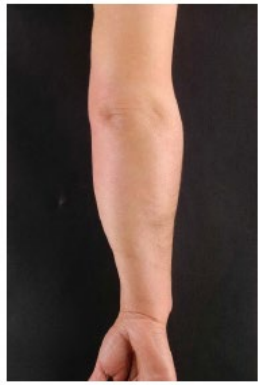

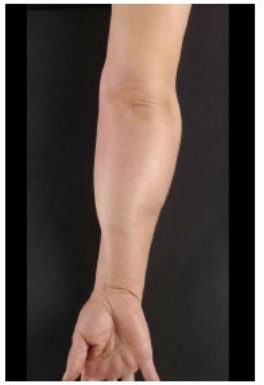

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1989 (diagnosis aged 30)

- HAE type I diagnosis
- Family history
  - Lives with husband, and 2 adult children live nearby
  - Has 5 siblings
  - No other family members with HAE or any who have displayed similar symptoms

HAE, hereditary angioedema; IV, intravenous

Treatment history

1989–1994

Danazol

(100 mg daily, 60 kg)

- Weight gain (>10 kg)
- Headaches
- Weakness
- Adynamia
- Increasing depressive episodes
- >30 attacks per year (medium–severe)

**The indications for use and dosing of intravenous C1 inhibitor are dependent on manufacturer's Summary of Product Characteristics. Berinert® 500/1500 is licensed for the treatment and pre-procedure prevention of acute episodes of HAE. The approved dose of Berinert® 500/1500 for the treatment of acute attacks is 20 IU/kg. CSL Behring does not suggest or recommend the use of C1-INH (IV) in any way other than as described in the Summary of Product Characteristics*

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IU, international unit; IV, intravenous; SC, subcutaneous

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1998–1999

C1-INH IV

(500 IU/kg, twice weekly in hospital*)

- Controlled disease and side-effects disappeared (except depression)

↓

- Treatment switched due to other issues

Tranexamic acid

(3 g per day – ineffective at controlling attacks)

Additional treatment used for breakthrough attacks:

- Danazol (100–200 mg per day)
- C1-INH IV on-demand
- Icatibant

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2000–2007

Danazol

(100–200 mg per day)

Persistent side-effects leading to reduced working:

- Increasing depressive episodes
- Weakness
- Nausea

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⬇

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- C1-INH IV on-demand
- Icatibant

2000–2007

Danazol

(100–200 mg per day)

Persistent side-effects leading to reduced working:

- Increasing depressive episodes
- Weakness
- Nausea

2010–2021

C1-INH IV

(500–1000 IU/kg, administered at home by husband as soon as attack appears*)

- Husband completed training programme for home administration
- HAE symptoms controlled after the second week

**The indications for use and dosing of intravenous C1 inhibitor are dependent on manufacturer's Summary of Product Characteristics. Berinert® 500/1500 is licensed for the treatment and pre-procedure prevention of acute episodes of HAE. The approved dose of Berinert® 500/1500 for the treatment of acute attacks is 20 IU/kg. CSL Behring does not suggest or recommend the use of C1-INH (IV) in any way other than as described in the Summary of Product Characteristics*

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IU, international unit; IV, intravenous; SC, subcutaneous

Treatment history

1989–1994

Danazol

(100 mg daily, 60 kg)

- Weight gain (>10 kg)
- Headaches
- Weakness
- Adynamia
- Increasing depressive episodes
- >30 attacks per year (medium–severe)

1998–1999

C1-INH IV

(500 IU/kg, twice weekly in hospital*)

- Controlled disease and side-effects disappeared (except depression)

⬇

- Treatment switched due to other issues

Tranexamic acid

(3 g per day – ineffective at controlling attacks)

Additional treatment used for breakthrough attacks:

- Danazol (100–200 mg per day)
- C1-INH IV on-demand
- Icatibant

2000–2007

Danazol

(100–200 mg per day)

Persistent side-effects leading to reduced working:

- Increasing depressive episodes
- Weakness
- Nausea

2010–2021

C1-INH IV

(500–1000 IU/kg, administered at home by husband as soon as attack appears*)

- Husband completed training programme for home administration
- HAE symptoms controlled after the second week

2020

C1-INH SC long-term prophylaxis

(2000/3000 administered for two weeks)

- Patient unable to adhere to LTP regimen

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C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema; IU, international unit; IV, intravenous; SC, subcutaneous

Current treatment plan

On-demand therapy

- Patient has only been interested in on-demand treatment for acute attacks
- She has a long history (approx. 23 years) of treatment with IV C1-INH (500–1000 IU/kg)
- Patient still declines long-term prophylaxis therapy – options discussed at every visit

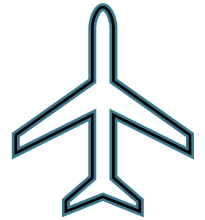
- HAE attacks are managed with IV C1-INH treatment
  - Experiences moderate attacks approx. twice per month (abdomen and/or skin)
  - Laryngeal attacks occur 3/4 times per year (managed in hospital)
  - >1000 IV C1-INH injections – treatment is still effective at managing acute attacks at the same dose

The indications for use and dosing of intravenous C1 inhibitor are dependent on manufacturer's Summary of Product Characteristics. The approved dose of Berinert® 500/1500 for the treatment of acute attacks is 20 IU/kg. CSL Behring does not suggest or recommend the use of C1-INH (IV) in any way other than as described in the Summary of Product Characteristics

C1-INH, C1-esterase inhibitor; IV, intravenous; IU, international unit

Issues encountered during treatment

- Language barrier
- Travel to homeland, where C1-INH was not available at that time
- Not able to drive to the hospital
- GP will not administer C1-INH IV on demand or prophylaxis
- Husband works and cannot help administer
- Injection site reactions with SC injection



C1-INH, C1-esterase inhibitor; GP, general practitioner; IV, intravenous; SC, subcutaneous

Treatment and patient reported outcomes

Treatment outcome

- **No loss of efficacy** present after 20+ years of receiving treatment with C1-INH
- No interaction with additional medication

Patient reported outcomes

- Angioedema control test (AECT) was used to assess disease control (score: 14)
- Angioedema quality of life questionnaire (AEQoL) was not feasible due to language barriers

C1-INH, C1-esterase inhibitor

Treating patients with comorbidities

- The presence of comorbidities may affect the clinical course of HAE in patients ≥65 years old[1]
  - Annual mean attack frequency has shown to be greater in patients with concomitant diseases, particularly in women
- Anxiety and depression are common comorbidities that affect 35% and 21% of patients with HAE[2]

HAE, hereditary angioedema

1. Bernstein JA. *et al., Ann Allergy Asthma Immunol.* 2020; 125(3):334–340.e1. 2. Banerji A. *et al., Ann Allergy Asthma Immunol.* 2020; 124(6):600–607.

Poll: Which quality of life tools are more appropriate for elderly patients?

A. Angioedema control test (AECT)

B. Angioedema quality of life questionnaire (AEQoL)

C. Angioedema activity score (AAS)

D. HAE activity score (HAE-AS)

Personalised medicine

Burden of disease = disease activity + quality of life

Quality of life may differ in presentation for patients across all ages

Elderly patients may be unable to complete long questionnaires to assess quality of life and disease control

According to a recent Delphi consensus, achieving total control of HAE and normalisation of patients' lives should be the goal of treatment in HAE[1]

HAE, hereditary angioedema

1. Maurer M. *et al., J Allergy Clin Immunol.* 2021; S0091–6749(21)00821–6

Take-home messages

- Treatment should be individualised to suit a patient's needs and preference
  - Despite having the option of long-term prophylaxis, on-demand therapy may be preferred, as seen in this case

- Comorbidities can affect the course of the disease and may influence the patient's treatment preference
  - These should be taken into account when developing a treatment plan

- Discussions around other treatment options at least once per year
  - Important to make patient aware of other/newer treatment options that are available

Managing HAE in a severely affected patient

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EUR-BRN-0325

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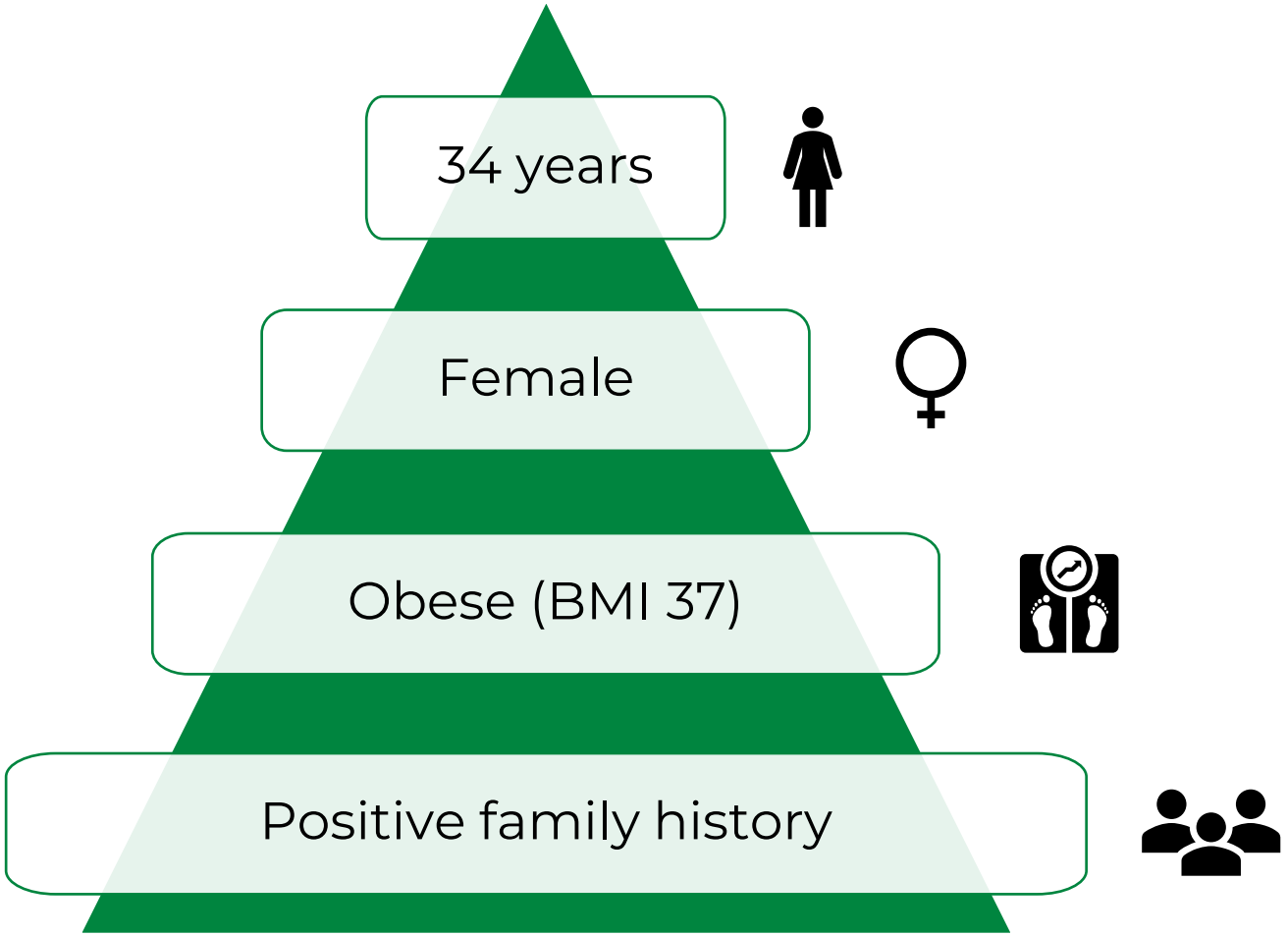
Disclaimer

- The views, information or opinions expressed herein are those of the speaker; they do not necessarily reflect those of CSL Behring
- Slides may contain off-label content

Patient description

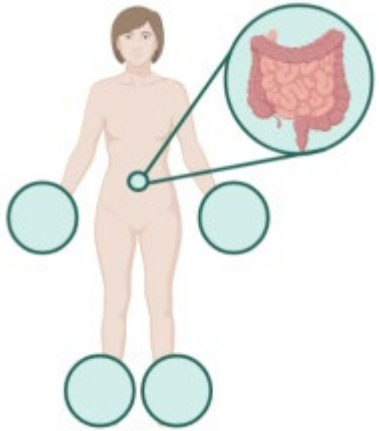


BMI, body mass index

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

GI, gastrointestinal; HAE, hereditary angioedema

Diagnosis

- Diagnostic delay of 12 years (diagnosis at age 13)
  - Specialty of physicians → Allergy

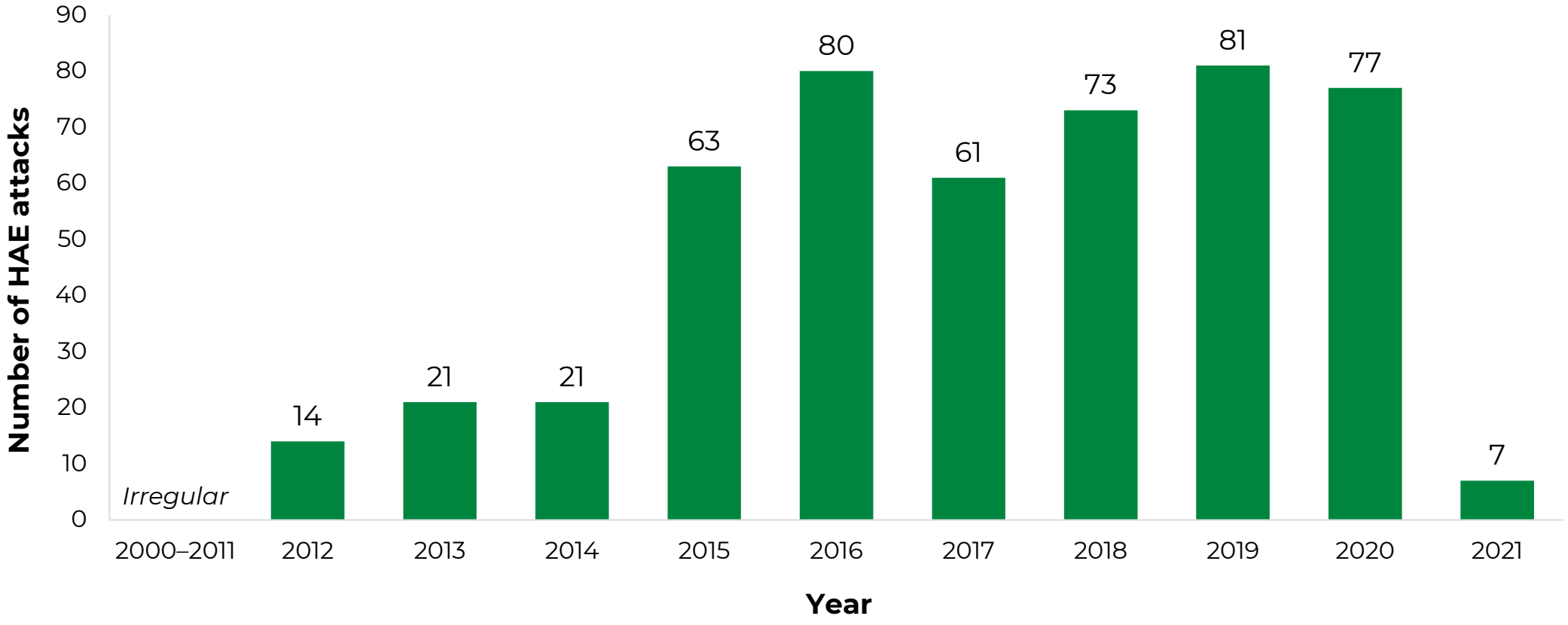
- Given the patient's family history, the following investigations were performed:
  - C1-INH concentration
  - C1-INH function
  - Genetic testing

Patient was diagnosed with HAE type I

↓ C1-INH ↓ function C1-INH

C1-INH, C1-esterase inhibitor; HAE, hereditary angioedema

HAE attack history



HAE, hereditary angioedema

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*

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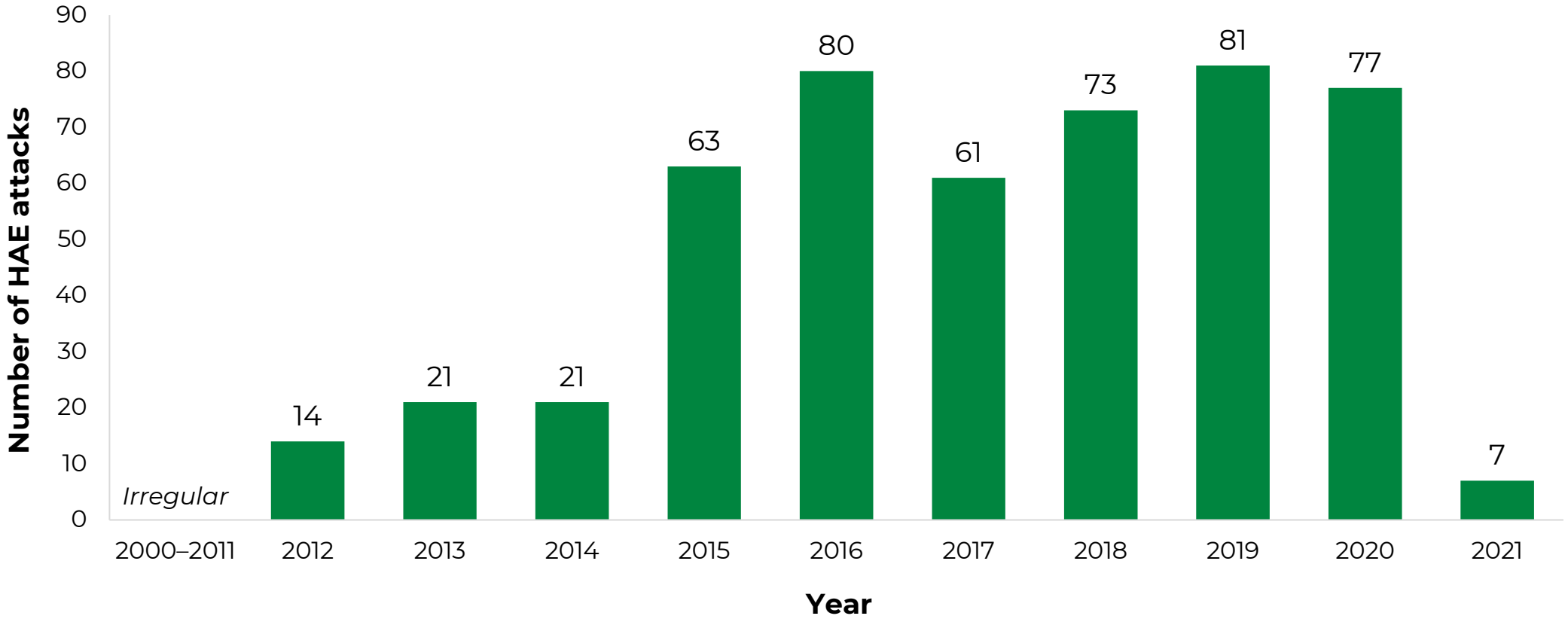
HAE attack history



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HAE, hereditary angioedema

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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HCP, healthcare professional; IU, international units; LTP, long-term prophylaxis; pdC1-INH, plasma-derived C1-esterase inhibitor; SC, subcutaneous

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

HAE, hereditary angioedema

Thank you for your attention

Q&A

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