

# Analysis of Prophylactic Treatment of Patients with Hereditary Angioedema in the Slovak Republic in 2022: Results of the Descriptive Cross-Sectional Database Study

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## INTRODUCTION

### Hereditary angioedema (HAE):

- A specific form of life-threatening inborn errors of immunity
- Characterized by sudden, severe, life-threatening attacks of skin and submucous tissue swelling caused by increased vascular permeability due to overproduction of bradykinin<sup>1</sup>
- Attacks triggered by emotional stress, dental treatment, infection, surgery, injury, medication, menstruation or alcohol<sup>2,3</sup>
- The trigger is often unknown<sup>2,3</sup>
- In Slovakia, the estimated prevalence and incidence is 1:41 280 and 1:1 360 000, respectively<sup>4</sup>

## OBJECTIVE

Current guidelines recommend regular assessment of the disease burden, disease control and quality of life.

In Slovakia there is long-term unavailability of hard data on healthcare management and treatment of HAE, which can be used for preparation of cost-effectiveness analysis and budget impact analysis.

A descriptive cross-sectional database study analyzed the prophylactic treatment of patients with HAE<sup>5</sup>.

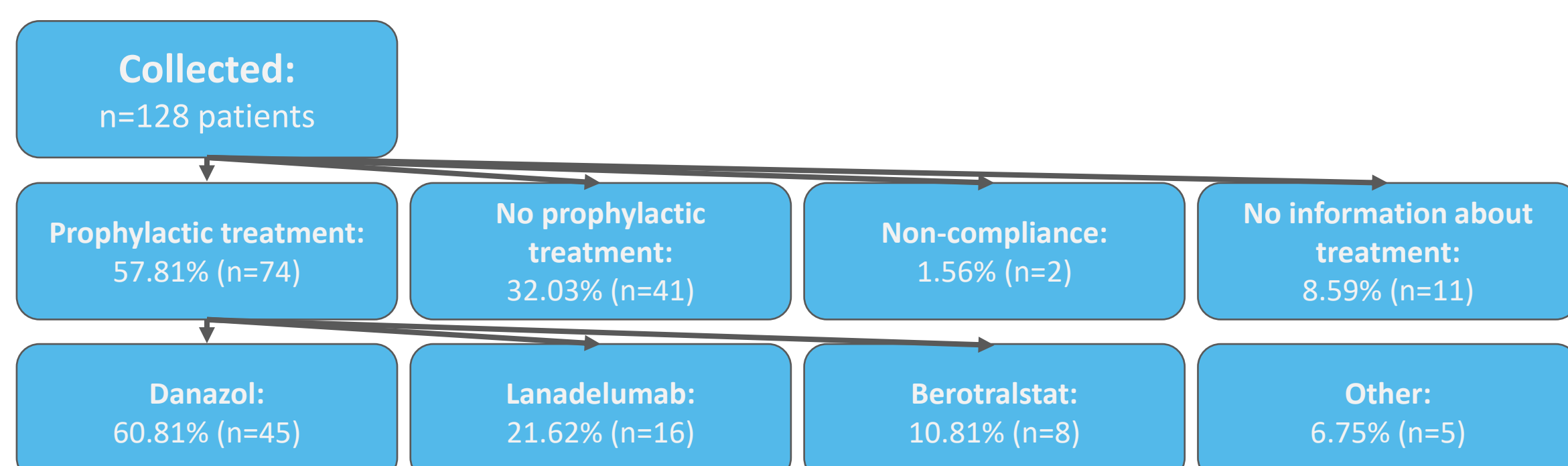
A sub-analyses of the database study was conducted to summarize individual types of prophylactic treatment and describe the effect of treatment on the course of HAE.

## METHOD

- Individual unstructured and unique anonymous data on patients were summarized in an Excel form (valid to date 15.10.2022).
- **Inclusion criteria:**
  - Definitively diagnosed HAE at any time
  - Signed consent for the processing of personal data for the purpose of participation in the presented study
  - Citizenship of the Slovak Republic
- **Exclusion criteria:**
  - Death of the patient
  - Diagnosis of another serious disease in the terminal stage

## RESULTS

128 patients – a representative cohort considering the prevalence of the disease in Slovakia.



57.81% were treated with **prophylactic treatment**, 93.24% of them with danazol, lanadelumab or berotralstat. The rest of patients were treated with tranexamic acid (4.05%), pdC1-INH (1.35%) and rhC1-INH (1.35%).

Treatment tolerance	Danazol (n=45)	Lanadelumab (n=16)	Berotralstat (n=8)
Good	46.67%	100.00%	50.00 %
Adverse events reported	11.11%	0.00 %	25.00 %
Not specified (unknown)	42.22%	0.00 %	25.00 %

**Prophylactic treatment with danazol was well tolerated** in 46.67% of patients, any adverse events were reported in 11.11%. Prophylactic treatment with lanadelumab was well tolerated in all treated patients, no adverse events were reported. Prophylactic treatment with berotralstat was well tolerated in 50.00% of patients, adverse events were reported in 25.00%.

Treatment effect	Danazol (n=45)	Lanadelumab (n=16)	Berotralstat (n=8)
Excellent	82.22%	100.00%	87.50 %
Moderate	17.78%	0.00%	12.50 %

The prophylactic treatment was rated as **excellent** in 82.22% of patients treated with danazol, in 100% of patients treated with lanadelumab and in 87.60% of patients treated with berotralstat.

Reduction of seizure frequency	Danazol (n=45)	Lanadelumab (n=16)	Berotralstat (n=8)
By less than 90%	80.00%	6.67%	50.00%
By 90% and more	20.00%	93.33%	50.00%

A **reduction in seizure frequency** by more than 90% was predominantly observed in patients treated with lanadelumab (93.33%) compared to patients treated with berotralstat (50.00%) and danazol (20.00%).

**Rescue treatment** was indicated in 81.25% of all patients. The most frequently used rescue treatments were icatibant (37.50%), pdC1-INH (27.88%) and rhC1-INH (19.23%).

	Prophylactic treatment (n=74)	No prophylactic treatment (n=41)	Unknown/Non-compliance (n=13)
Rescue treatment	91.89%	82.93%	15.38%
No rescue treatment	5.41%	12.20%	0.00%
Unknown/Non-compliance	2.70%	4.88%	84.62%

Of all patients on prophylaxis, 91.89% needed rescue treatment. This can be explained by the fact that prophylactic treatment is mainly given to patients with moderate to severe form of HAE.

## CONCLUSIONS

In the cross-sectional database study, selected characteristics of patients with HAE and the impact of prophylactic treatment on the frequency of seizures were investigated in the real clinical practice in Slovakia.

In this study, lanadelumab compared to danazol and berotralstat, dominated in the reduction of seizures frequency and was well tolerated.

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