

Unmet needs and systemic challenges of HAE pediatric patient families in Europe

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INTRODUCTION

The recent therapeutic innovations and improving disease awareness levels in Europe have led to a better quality of life of nowadays hereditary angioedema (HAE) patients when compared to earlier generations. In spite of this progress, practical experience indicates that the youngest HAE patient community still faces a high level of quality of life issues, systemic challenges and unmet informational needs.

OBJECTIVE

The objective of this study is to map out the “unprompted” status of quality of life issues, systemic challenges and unmet informational needs faced by HAE pediatric patients and their caregivers in Europe as the basis for future educational/ support activities.

MATERIALS AND METHODS

Between October 2024 and February 2025 the authors conducted an AI-powered multilingual natural language processing analysis of 537 anonymised patient queries/ stories posted since 2019. Written contributions from a total of 105 patients/ caregivers from 11 European countries* were analysed. The study was based on real patient stories gathered from relevant social networks, forums and other sources of user generated content**. Semantic Hub proprietary technologies were used for text processing: crawling/ pre-processing, information extraction from texts (NLU), generating results, visualization and report preparation.

RESULTS

The research highlights persisting critical gaps in diagnosis, treatment, and support for pediatric HAE patients and their families.

- **Quality of life** issues were mentioned by 78% of authors, **systemic challenges in diagnostics and treatment** were identified in 74% of cases, while **unmet informational needs** were highlighted by 35% (see fig.1).
- Pediatric HAE patients and their families experience quality of life challenges that may lead to long-term health and economic consequences (see fig. 2). The most frequently mentioned QoL issue is related to **family burden (84%)**, as multiple family members are often affected. Other QoL issues relate to the **mental health struggle (38%)**, **severity of attacks (37%)** and **financial burden (17%)**.
- As per figure 3, the main systemic challenges include **diagnosis challenges (60%)**, **inadequate specialist support (54%)** and **difficulties in accessing treatment (32%)**. The diagnosis challenges relate to long waiting, different opinions on optimal testing age, misleading symptoms. The caregiver/ patient-perceived inadequate specialist support is related to limited availability of pediatric HAE experts, changes in medical teams, etc. Among those who discussed systemic challenges, 32% specifically addressed issues related to treatment access: both financial/insurance challenges and medication shortages are mentioned.
- The most common **unmet informational challenge** relates to **treatment experience, its effectiveness, and adjustment (dosage for children)**, as 35% questions addressed this topic (see fig. 4). Caregivers seek support within their communities to understand the treatment experience of other children and how treatment effectiveness can change as children grow.

When considered from a geographical perspective, the research results indicate:

- **Western European challenges** (Germany, the UK, France, Belgium) revolve around cost, insurance, and continuity of care rather than basic access to treatment. Caregivers of pediatric patients struggle with being reassigned to new HAE physicians/ centers or facing bureaucratic hurdles in the healthcare system.
- **Central European challenges** (Poland, Czech Republic) consist of insufficient HAE awareness in pediatric emergency care, inadequate employability support of caregivers, and more restrictive pediatric reimbursement rules or discontinued reimbursement of select HAE treatment options.
- In **Southern Europe** (Greece, Croatia, Cyprus, Serbia, North Macedonia) the primary concerns are treatment accessibility and geographical barriers, indicating that regional disparities (e.g., islands, limited number of HAE specialists) make treatment access difficult.

CONCLUSIONS

The study findings show that the experience of European pediatric HAE patients and their families implies significant health, emotional and financial burdens. Improving specialist availability, treatment access and convenience, upgrading emergency response and providing holistic family care are essential to further enhancing the quality of life of families with children and adolescents living with HAE in Europe.

Figure 1. Share of anonymized authors who mentioned the given issue type

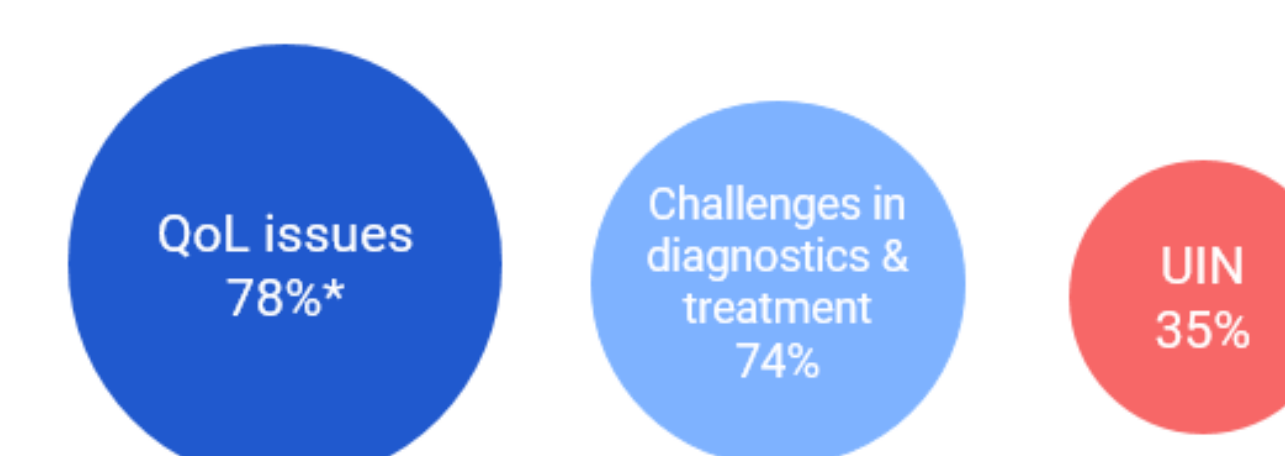


Figure 2. Quality of life issues

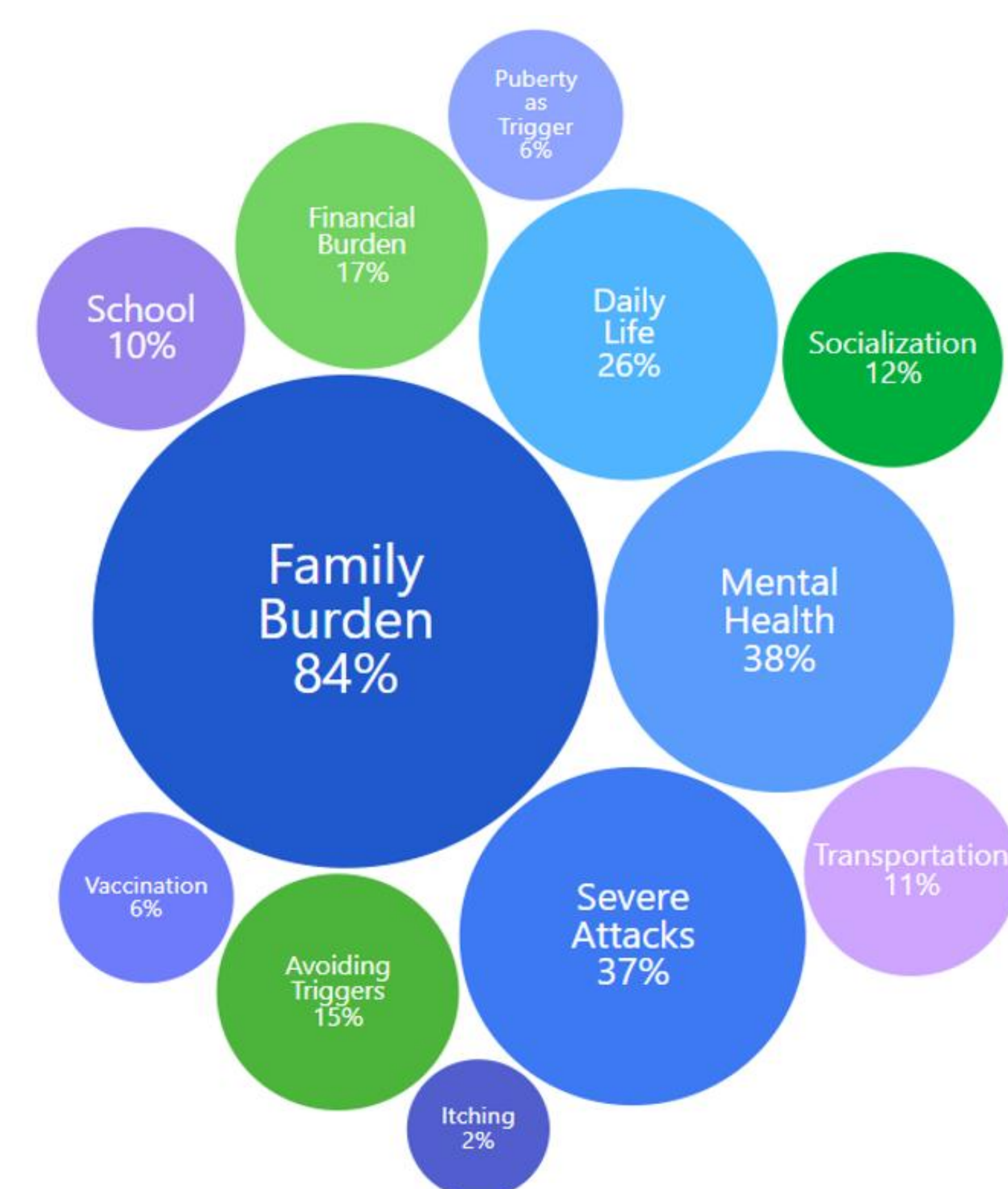


Figure 3. Systemic challenges in diagnostics and treatment

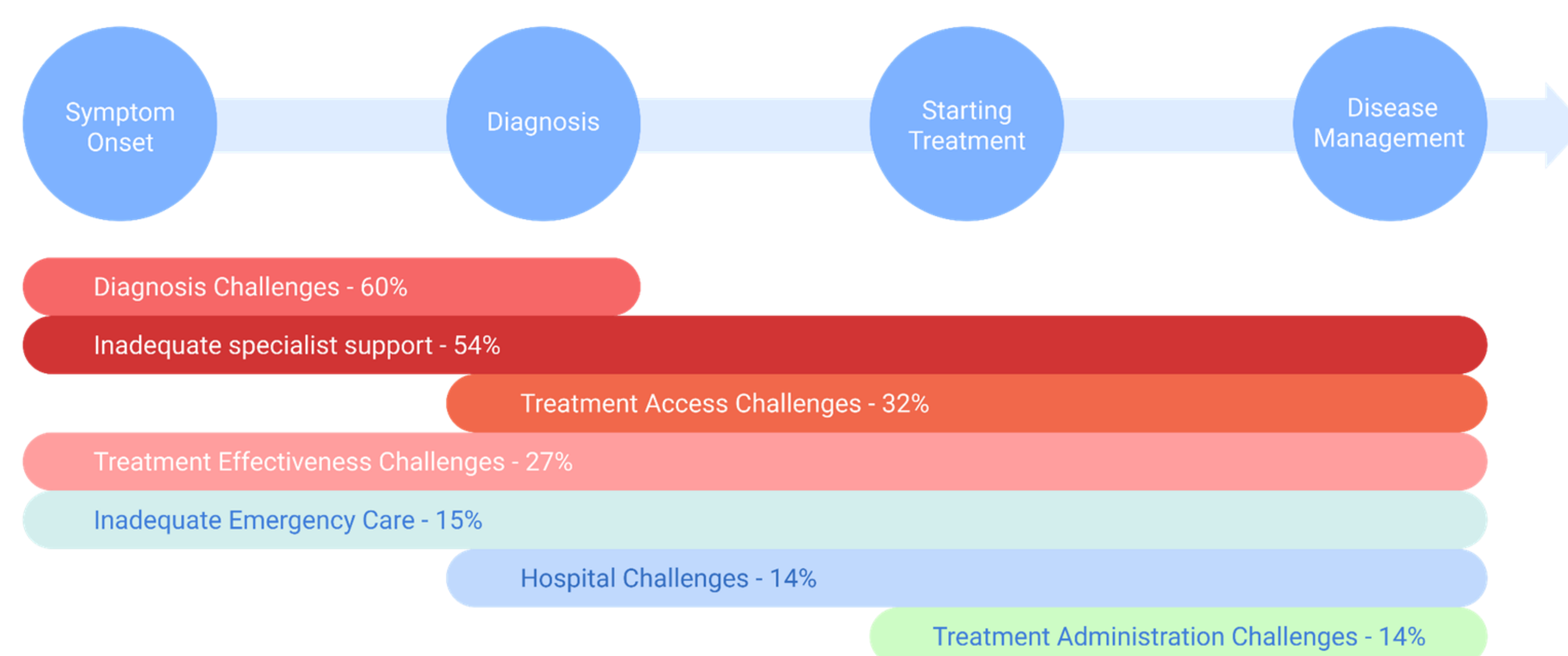
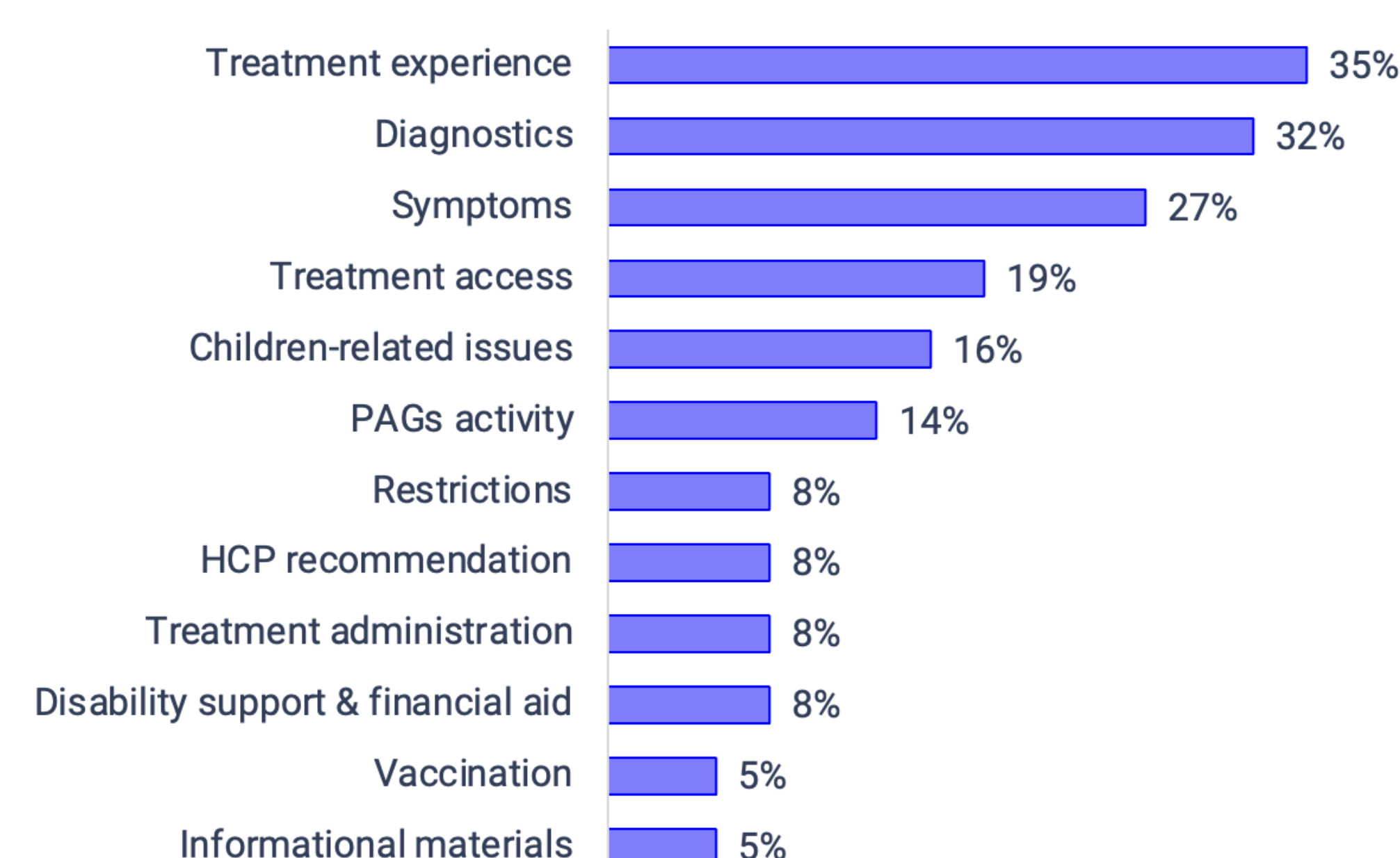


Figure 4. Unmet informational needs



*Germany, Poland, the UK, Czech Republic, Greece, Croatia, Belgium, Cyprus, France, Serbia, North Macedonia

**All stories and messages have been fully anonymised according to GDPR