Background

- HEReditary angioedema (HAE) is characterized by recurrent and unpredictable episodes of subcutaneous or submucosal swelling which can affect the abdomen, extremities, genitals, face, and larynx.
- All currently approved HAE on-demand treatments must be administered parenterally, which results in significant treatment burden.

Objective

- The objectives of this qualitative study were to understand patients’ likes and dislikes related to their current on-demand treatment, their recent experiences and route of administration (ROA) preferences for on-demand treatment.

Methods

- The US Hereditary Angioedema Association (HAEA) recruited people living with type 1 or type 2 HAE to be interviewed.
- Participants were not informed of the identity of the study sponsor.
- Study population included both adults (18 to 69yrs) and adolescents (12 to 17yrs); had at least one HAE attack within the past six months; currently taking on-demand treatment (C1-INH replacement or Bradykinin receptor 2 antagonist or kalikrein inhibitor).
- The sampling plan aimed to obtain half of each age group currently taking both on-demand treatment and long-term prophylaxis (LTP) and half taking only on-demand treatment.
- Open-ended questions were asked to participants to understand their likes and dislikes associated with their current on-demand treatment.
- Open-ended questions were then asked to understand the trade-offs patients are willing to make when choosing a preferred ROA. Hypothetical self-administered injection and oral on-demand treatments were initially presented with similar efficacy and tolerability/mild side-effect risk profiles (Figure 1), which were then better/worse dependent upon participants’ initial treatment choice.
- Profiles were based on on-demand injection treatments’ US package inserts and clinical trial data for oral on-demand treatment in development.

Figure 1. Hypothetical Trade-Off Scenario

Table 1. Resultant Characteristics

Table 2. Reported “Likes” and “Dislikes” of Most Recent Acute Attack Treatment by Mode of Administration

Table 3. Responses to Trade-Off Scenarios

References


Disclosures

This study was sponsored by KalVista Pharmaceuticals, Inc. All authors met the ICMJE authorship criteria and had full access to relevant data. The authors had full editorial control of the data presented and provided final approval of all content. Neither honoraria nor payments were made for authorship.

Don Bukstein — Represents a speaker for Regeneron.
Vibha Desai — Employee of KalVista Pharmaceuticals, Inc.
Leda Goga — Employee of KalVista Pharmaceuticals, Inc.
Shawn Czado — Employee of KalVista Pharmaceuticals, Inc.
Michelle Brown — Employee of RTI Health Solutions.
Kelley Myers — Employee of RTI Health Solutions.
Paul Audhya — Employee of KalVista Pharmaceuticals, Inc.
Laurence Bouillet — Reports grants and personal fees from Takeda, Biocryst, Behring, Blueprint, GSK.

The PBL Institute, Madison, Wisconsin, USA; Global Medical Affairs/Outcomes Research, KalVista Pharmaceuticals, Inc., Cambridge, Massachusetts, USA; Global Market Access, KalVista Pharmaceuticals, Inc., Cambridge, Massachusetts, USA; RTI International, Inc., North Carolina, United States; Internal Medicine, Grenoble Alps University, National Reference Center for Angioedema, Grenoble, France.

Route of administration preferences of people living with hereditary angioedema for on-demand treatment: A US-based qualitative study

Don Bukstein, Vibha Desai, Leda Goga, Shawn Czado, Michelle Brown, Kelley Myers, Paul Audhya, Laurence Bouillet.