
Hereditary angioedema: a current state-of-the-art review, IX: the US Hereditary Angioedema Association: a message from the president

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Hereditary angioedema (HAE) is a catastrophic unmet medical need in the United States because there is no therapy available to treat an attack once it begins. 17- α -Alkylated anabolic corticosteroids are useful for HAE prophylaxis in certain adults, but data from an unpublished survey conducted by the US Hereditary Angioedema Association (HAEA) revealed that many patients continue to experience periodic acute attacks notwithstanding ongoing therapy. In addition, more than two-thirds of the patients queried said that it is important to reduce or eliminate corticosteroid use. The utility of these agents is further limited because they are not well tolerated by women, and their use is generally contraindicated for children, some of whom, tragically, are severely affected and experience frequent attacks. Is it not ironic that 300-lb NFL linemen are suspended and counseled on the extreme danger posed by relatively short courses of these drugs while our patient community is relegated to long-term use of these potentially toxic and highly undesirable agents?

The HAEA represents a new breed of rare disease patient organizations that have been enabled by the Internet's uncanny capacity for creating communications channels that result in online communities. What started out in 1999 as a technologically savvy patient's personal Internet page that included a few paragraphs about a family's struggle to cope with HAE soon grew into a group that shared experiences through a Yahoo! e-mail service. Prompted by continuous growth that was abetted by the increasing sophistication of Internet search engines, a short year later the group evolved into a duly chartered nonprofit organization. The HAEA's membership continues to grow, and today it tops 1,500 patients. In addition, the organization has already identified and works to keep in touch with more than 600 physicians who treat patients with HAE.

Our remarkable and ongoing growth has been enabled and sustained by the relationships built among key stakeholders: motivated patients, who have a catastrophic unmet medical need; compassionate physician-researchers, who are staunch advocates for better treatment options; and pharmaceutical companies, which invest in HAE clinical programs and provide the HAEA with generous financial support. Indeed,

these are exciting times for patients with HAE, who are now facing the pleasant prospect of having 1 or more new HAE therapies available in the next 2 years. The HAEA is in the process of working with HAE physician-researchers to foster a dialog that we believe will result in a uniquely American HAE treatment algorithm that features choices and an approach that tailors therapy to meet each individual patient's needs.

As might be expected from a "digital age" organization that was conceived in cyberspace, the HAEA's Web page (<http://www.haea.org>) serves as the principal focal point for information dissemination and interaction among our patient community. The Web page contains authoritative information for patients and physicians on HAE symptoms, diagnosis, and treatment. It also serves as our principal tool for recruiting members and clinical trial participants. To date, the HAEA has referred more than 800 patients to the 5 pharmaceutical companies that have been conducting HAE clinical trials in the United States during the past several years. The HAEA also keeps in touch with patients and physicians who treat HAE by publishing a quarterly newsletter and conducting regional and national patient conferences. The HAEA's nationwide volunteer network provides us with a presence in every region of the United States, and individual members have taken the initiative to speak about HAE at local events and to sponsor fundraising activities.

Since May 2003, the HAEA has provided technical assistance for patients who want to purchase C1 inhibitor concentrate, the current gold standard HAE acute attack therapy. The concentrate is licensed in Europe and other parts of the world but not yet in the United States, although 2 companies are involved in C1 inhibitor clinical programs and plan to file for Food and Drug Administration approval. We help patients comply with the Food and Drug Administration's personal importation guideline and work to secure the product from reliable overseas sources. C1 inhibitor is an expensive medicine, and no insurance company will provide reimbursement for an unlicensed therapy. Most patients who truly need access to this therapy do not have the financial wherewithal to participate, and even those who can purchase the concentrate soon realize that there are limits on the number of times they can remortgage their homes or rely on the generosity of relatives and friends. Fortunately, few patients now need to purchase the concentrate because the most severely affected members of our community are now participating in one of

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the clinical trials that offer open-label therapy after initial randomized placebo-controlled exposure.

Our board of directors and officers believe that a relatively small organization such as the HAEA can dramatically increase the quality of life for patients HAE by leveraging the success and lessons learned by others. Under the aegis of our “continuous improvement” program, the HAEA staff studies and strategically adopts the operating and business “best practices” found in other rare disease organizations. Based on recent evaluations, the HAEA is working with medical advisors to develop an HAE registry that will provide scientists with a pool of patients willing to participate in research designed to further elucidate the pathogenesis of HAE and identify better therapeutic options. We are also planning to adopt a version of the Alpha One Foundation’s innovative approach to health care management that features “patients helping patients.” The program we are designing features specially trained patients with HAE who will serve as “coordinators” for helping patients manage their HAE, fulfill their physician’s instructions, and make regular entries into the HAE registry that would enable longitudinal studies of disease natural history, progression, and treatment outcomes.

Born in the digital age and nurtured by leveraging the efficient and cost-effective communications enabled by the Internet, the HAEA represents a new breed of patient organizations. Our Web page serves as a portal to authoritative information and interaction with patients with this debilitating and catastrophic unmet medical need. Going forward, we look to accelerating our growth and leading the effort to adopt a treatment algorithm that features choice and meets individual needs. We will also continue providing a range of patient services and working with all stakeholders to enhance our ability to positively affect the quality of life for all patients with this debilitating disease.

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