

## November 15, 2018 Des Moines, IA

Dear Chair and members of the Pharmaceutical and Therapeutics Committee.

I am a patient with severe HAE and am privileged to serve our patient community as Research Director for the US Hereditary Angioedema Association (HAEA)—a 5600 member non-profit advocacy and research organization, founded and staffed by HAE patients and care givers.

HAE is a disabling and potentially fatal disorder that causes attacks of massive swelling in various body parts that can last from 3 to 5 days. I can tell you from personal experience that swelling that occurs in hands, feet, face, and abdomen is extremely painful and disabling. But the biggest fear is laryngeal swelling, because it can cause death by suffocation. Peer reviewed burden of illness studies and other research reveal that inadequately treated HAE causes severe disability, and the historical *mortality rate* for untreated patients is over 40 percent.

Several years ago, the HAEA's Medical Advisory Board - a distinguished group of expert physician/scientists - published "Recommendations on the Management of HAE" in a prestigious medical journal.

I am here to ask that this review of Medicaid Preferred Drugs for HAE include careful consideration of the Medical Advisory Board 's HAE Management Recommendations. This document offers a set of principles and practices to **ensure efficient and cost-effective use** of the FDA-approved HAE therapies available to treat this disabling and potentially life-threatening disease.

I would like to highlight a few key points from the Recommendations:

- 1) Because not all patients respond the same to all medications, an expert physician must work with each patient to determine the optimal treatment.
  - For that reason, we urge Iowa Medicaid to continue allowing access to all medicines FDA-approved to treat HAE.
- 2) For some patients, on-demand treatment alone is sufficient; for other patients, prophylactic treatment is indicated as first-line treatment together with on-demand treatment for breakthrough attacks.
- 3) Since life threatening laryngeal edema can occur at any time, patients should have access to at least 2 standard doses of an FDA-approved medicine for on-demand treatment of acute HAE attacks.
- And lastly, because of the disfiguring and painful effects of swelling, all attacks should be considered for treatment as soon as the symptoms are clearly recognized.

In closing, I would like to stress again that **first** key point made in the Recommendations, that not all patients respond the same to all HAE medications, so an expert physician must work with each patient to determine the optimal therapy for them. Therefore, we again kindly ask that Iowa Medicaid allow access to **all** FDA-approved therapies (including on formulary) to treat HAE...the lives of all HAE patients in Iowa depend on it.

Thank you