

**Testimony of Diane Dorman
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**Before the
FDA Blood Products Advisory Committee
Regarding Plasma-Derived C1 Esterase Inhibitor (Cinryze)
for Hereditary Angioedema (HAE)**

May 2, 2008

Thank you for the opportunity to speak to you today about the importance of an FDA approved treatment for hereditary angioedema (HAE), also known as C1 inhibitor deficiency. I am Diane Dorman, Vice President for Public Policy, for the National Organization for Rare Disorders (NORD).

NORD is the consumer organization that advocated for enactment of the Orphan Drug Act of 1983. We continue to monitor implementation of the law, especially encouraging research and development of new treatments for rare “orphan” diseases. Our most important mission is to assure that research on rare diseases is widespread and productive, leading to medicines, biologics, devices and diagnostics that will help patients with rare diseases. We support the incentives in law that stimulates companies to do orphan drug research. We want safe and effective products to get to market as quickly as possible.

Hereditary angioedema (HAE) is a very rare genetic disease that is commonly undiagnosed or misdiagnosed in the United States. It results from a defect in the gene controlling the synthesis of C1 inhibitor. Patients experience repeated and unpredictable attacks of inflammation affecting the larynx, abdomen, face, urogenital tract, and extremities. Attacks are painful, frightening, debilitating and they can be life-threatening.

Most patients have a family history of the disease, although many don't know it until their own diagnosis. However, a significant minority of those with the disease appear to have gotten it from spontaneous mutations. On both counts, this adds yet more unpredictability to the disease, increases misdiagnosis, and makes disease onset and manifestations more frightening.

Treatment for HAE has been available in Europe for more than 35 years. But there is no approved treatment yet for HAE in the United States. Lev Pharmaceuticals has developed Cinryze for the U.S. market and has applied for FDA approval. Cinryze is nearly identical to the C1 inhibitor that has been available in Europe for decades, where it is known to be safe and effective.

Patients can import plasma-derived product from Europe, but only if they can afford it, since health insurance does not reimburse for drugs that are not approved for marketing by the FDA. In the absence of an FDA-approved treatment, however, doctors have few other treatment options. Patients are ordinarily prescribed corticosteroids, antihistamines, danazol, and epinephrine, none of which have been proven effective in stopping HAE attacks.

For those who are undiagnosed or have an attack so debilitating that they cannot advocate for themselves, there is likely to be an emergency room that will give them medications or even surgery that will not help them. Experience tells us that the approval of an orphan product in the US results in greater physician and patient awareness, speeding diagnosis and access to available treatments and reducing inappropriate care.

We ask you to keep in mind that HAE is a life-threatening disease and after 35 years, patients cannot risk waiting any longer. Several times in past years, hopes were destroyed when investigational biologics for HAE did not prove to be effective and development was stopped. We ask you now to review the data for Cinryze, keeping in mind that there are no other treatment options available on the American market. HAE patients are desperate for a safe and effective treatment.

Thank you.