Hemophilia Association of the Capital Area

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Food and Drug Administration
5360 Fishers Lane Room 1061
Rockville MD 20852

RE: Docket Number: 2006N-0062
RIN No: 0910-AF14

To Whom It May Concern:

On behalf of the Hemophilia Association of America (HACA), I am writing in support of the proposed rules that will increase access to investigational new drugs for members of the bleeding disorders community. HACA is a not-for-profit organization that seeks to improve the quality of life for persons and families affected by bleeding disorders in Northern Virginia, Washington, D.C., and parts of Maryland.

Hemophilia is a lifelong, genetic bleeding disorder which usually affects males. Everyday activities may become the cause of sudden, painful, and lingering injuries. Today, people with hemophilia can use recombinant clotting factor to stop bleeding, reduce recurring injuries, and limit the crippling effects of bleeds. This, however, is a relatively recent development.

Prior to the introduction of recombinant medications, anti-hemophilic clotting factor was plasma-based. As recently as twenty years ago, that clotting factor – unbeknownst to the individuals who used it – was contaminated by HIV, hepatitis, and (potentially) other infectious agents. As a result, more than half of the bleeding disorders community contracted HIV. Over the same period, nearly 80 percent contracted Hepatitis C through these products, a majority of those are infected with genotype 1.

Access to investigational new drugs is vitally important for this segment of the bleeding disorders community. A 2005 prospective randomized study by Jacobson published in *Hepatology* reported response rates of 29-34 percent with FDA-approved hepatitis medicines. This means a majority of the affected individuals have no effective (or no alternative) treatment options to treat—or to halt the progression of—their disease.

During the early 1980s, many of our members were infected with HIV. Many of those members have now spent 15 or more years taking protease inhibitors. An increasing number are experiencing decreasing success with their therapies as their bodies are building resistance to more and more drugs. Access to the two new classes of drugs that have been found to block virus replication, the integrase inhibitors and CCR5 inhibitors is also vitally important for the HIV infected segment of the bleeding disorders population.

The alternative to the potential life saving effects of investigational new drugs is death. Investigational drugs should be available to individuals who have no alternatives, and who understand the balance of potential risks versus an improved prospect of living. While safety is important, it is nothing without life.

Please consider the situation of the bleeding disorders population as the final regulations are determined.

If you have any questions, please contact me directly.

Sincerely,

Sandi Qualley

Sandi Qualley
Executive Director