May 25, 2011

Transmitted via EMAIL
Dr. Margaret Hamburg, M.D., Commissioner
United States Food and Drug Administration
10903 New Hampshire Ave.
Silver Spring, MD 20093-0002

Dear Commissioner Hamburg,

We are "People with Bleeding Disorders and HCV." On behalf of patients and supporters of people with bleeding disorders affected by hepatitis C (HCV)[1], we seek your additional assistance in meeting our urgent need for development and access to new, better, HCV therapies.

In previous communications with FDA we have explained our situation; we have requested changes in FDA guidance; and we have provided suggestions on draft FDA actions. The excellent and detailed FDA response to our Citizen Petition[2] indicated substantial understanding of our need which has carried into other agency policy[3] [4].

However, FDA policy statements have yet to translate into actual treatment progress for our community. HCV patients on average will likely see some therapeutic improvements with the approval of first generation direct-acting antivirals (DAAs) against HCV. However, our community may not see much benefit from these first generation DAAs because our infirmity is often 'difficult to treat[5]' requiring long term treatment with mediocre success rates, and because anti-hemostasis side effects from those drugs may be particularly challenging for us[6].

As a result, we remain endangered and medically underserved.

New therapies are on the horizon. Recent small studies appear to prove that HCV can be cured by combination therapy with DAAs[7]. But approval of those combination therapies is years away. The FDA response to our Citizen Petition recommended and encouraged individual, or small group, treatment-oriented clinical trials as pathways to allow expedited access to potentially curative therapy for endangered and underserved groups. However, we have yet to find a method to make those pathways work for us.
We therefore request that FDA specify an agency contact /resource / advocate to assist patient groups in defining practical pathways for expedited access to promising new HCV drugs.

Sincerely,

/s/, /s/
Paul Brayshaw and Mark Antell,
Co-chairs "People with Bleeding Disorders and HCV"
accesshcvtherapy@gmail.com

cc.
HFA: Kim Haugstad, Susan Swindle
NHF: Dr. Craig Kessler, Neil Frick, Mark Skinner (WFH), Val Bias
COTT: Corey Dubin, Dave Cavenaugh

attachment

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[1] Our campaign receives encouragement from the Hemophilia Federation of America (HFA), the National Hemophilia Foundation (NHF) and the Committee of Ten Thousand (COTT)
We attach a relevant resolution adopted this week (5/22/2011) by the board of the National Hemophilia Foundation.
In a followup communication to us, Dr. Craig Kessler, M.D., chair of the Medical and Scientific Advisory Committee of the NHF, states that the NHF resolution provides, "expert medical corroboration for your position."

[2] Our Citizen Petition of September 2009; the FDA response (Docket FDA-2009-P-0471, dated April 23, 2010); our comments on HCV drug testing; our comments on combination drug testing; and our comments on approval for Telaprevir and Boceprevir are available on our website under Public Positions:
http://sites.google.com/site/accesshcvtherapy/start

[3] FDA "Guidance for Industry, Chronic Hepatitis C Virus Infection: Developing Direct-Acting Antiviral Agents for Treatment."

[4] FDA "Guidance for Industry, Co-development of Two or More Unmarketed Investigational Drugs for Use in Combination."

[5] HCV is the leading cause of death for people with bleeding disorders. Nearly our entire cohort over the age of 30 was exposed to HCV via human-blood-derived Factor concentrates used therapeutically to control bleeding events. As a result most of us now have long-term, often advanced, HCV disease.
Ragni MV, Moore CG, Soadwa K, et al; THE HHH STUDY GROUP. Impact of HIV on liver fibrosis in men with hepatitis C infection and haemophilia. Haemophilia. 2011 Jan;17(1):103
Further we have high levels of HIV coinfection due to the same contaminated medicine. HIV is associated with faster HCV progression, increased side effects from HCV drug therapy and decreased likelihood of HCV drug therapy success.

[6] For a broad survey of the utility and weaknesses of new HCV therapy see the Treatment Action Group, "HCV Therapy Pipeline Report," available at the following website:
http://www.treatmentactiongroup.org/uploadedFiles/About/Publications/TAG_Publications/2011/HCV%20pipeline%202011%20final.pdf

[7] Link from our website to story: **Proof of Concept for Direct Acting Antivirals to Cure HCV (March 27, 2011)**.