AMYLOIDOSIS SUPPORT GROUPS INC.

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June 20, 2012

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Dear Concerned and Involved Doctors and Directors,

"I am shocked. I can't believe it was rejected. On top of all this with John - this is my worst day. It happens to be my birthday as well. I am heart broken. Really heart broken."**

*** From an email from Kevin Mui, patient, sent June 18, 2012.*

"…do you know if there are patients in Europe already who've had liver transplants and taking tafamidis and how they're doing?
My vision is worse today and seems slowly declining and depresses me. It's a major source of anxiety and fear and despair. This prevents me on being productive...its like why bother?"***

**** From an email from the late John Lee, Kevin's brother sent to Muriel Finkel on May 21, 2012.*

After hearing of the FDA rejection of tafamidis today, Kevin, one of the patients who testified at the Advisory Committee meeting on May 24, sent the above email to the Amyloidosis Support Groups. His brother, John, died a June 18, 2012, in his 40's, from familial amyloidosis (FAP-TTR). John had a liver transplant in 2005, but his peripheral neuropathy rendered him housebound. His diarrhea caused him much embarrassment and, combined with the neuropathy, gave him a sad quality of life. His heart eventually gave out. Kevin's mother also died from the disease, as did his uncle. Kevin is in his

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NORD
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National Organization Member

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thirties and desperately fighting for his own life as the amyloid continues to build up in his body. He was praying to have tafamidis in his arsenal.

Most of the patients who testified at the meeting traveled to Silver Spring despite serious physical problems and overcame many travel difficulties, not unlike Kevin's late brother John who was unable to come. Some of those patients who were not able to come to the meeting wrote compelling letters for the committee's consideration. We need to know if the patients' testimony had any influence or impact on the FDA's decision. We were under the impression that there was to be a new emphasis on patient input, and on facilitating treatments for those with rare diseases. But we now question the value of our patients making such sacrifices only to doubt that their presence was at all meaningful.

In its briefing document for the Advisory Committee, the FDA took an adversarial position and no evidence presented at the meeting seemed to influence their thinking. Despite the FDA public commitment to guidelines that look at drugs for rare diseases in a different way, the status quo mind-set seemed to prevail.

At the meeting the FDA was focused on numbers. Some patients achieved a reduction in the symptoms of neuropathy. The efficacy of the drug was proven, just not to the standard statistics of the FDA. Familial Amyloidosis is a disease that always gets worse. Any improvement is evidence of efficacy. Slowing down the progression of amyloid infiltration to any degree is a life changing effect.

Why did the FDA ignore the recommendation of its advisory panel that the drug could be accepted based on efficacy of a surrogate endpoint? Surrogate endpoints were met. TTR *WAS* stabilized by preventing the misfolding of the protein. This is the essential point and the real goal of any therapy. It cannot logically be dismissed. In addition, BMI improved in some patients. Due to constant diarrhea because of the buildup of amyloid in the digestive system, most patients become emaciated. Stability or progression in BMI is a very important endpoint.

We feel that our patients need a safe drug treatment, and at this time, tafamidis is the only one available. It has been accepted and is effective in Europe. Patients here have the same disease and the same related challenges. Most, if not all, are ready to go to their doctor for a prescription.

We are aware that Pfizer received a Complete Response Letter from the FDA, requesting a second efficacy study. This will take many months or years. But we have little time. Daily the amyloid is misfiling and log-jamming our patients' organs and causing serious peripheral neuropathy. We urge you to reconsider your decision and expedite bringing tafamidis to our patients in need.

Sincerely,

Muriel Finkel

Muriel Finkel
Founder and President