

Beth Sufian FDA Written Statement  
Open Public Hearing Session  
Meeting of the Anti-Infective Drugs Advisory Committee  
New Drug Application 050-814  
Inhaled Aztreonam  
December 10, 2009

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## **I. Background**

Thank you for allowing me to be here today to present my testimony to the members of the FDA Anti-Infective Drugs Advisory Committee Meeting.

My name is Beth Sufian, I am 44 years old and have Cystic Fibrosis. I live in Houston, Texas where I have practiced law for nearly 20 years. I am a partner in the law firm of Sufian & Passamano. I am the author of the books Representing ADA Plaintiffs and A Clinician's Guide to the Legal Rights of People with Cystic Fibrosis. I have published 9 articles in CHEST, The American Journal of Respiratory and Critical Care Medicine and Pediatric Pulmonology. I have presented over 70 speeches to over 10,000 people at hospital and medical conferences on access to care and insurance coverage issues for people with Cystic Fibrosis and other serious medical conditions.

I am also the director of the CF Legal Information Hotline ("CF Hotline"), which has been in operation since 1998 and has received over 14,000 calls from all 50 states. The Hotline receives grant funding from the CF Foundation. Hotline callers are CF patients, family members and healthcare providers who need information related to their legal rights in the areas of health insurance, government benefits, education and employment. My testimony today reflects not only my own experience living with CF, but also the experiences of the 14,000 others who have contacted the CF Hotline and related their experiences to me.

People with CF can easily cross-infect each other with a dangerous bacteria known as b. cepacia. Therefore, the CF Foundation Infection Control Consensus Conference Committee advises people with CF to avoid being indoors with each other if at all possible. In addition December is a very dangerous month for people with CF to travel as there are many healthy people who are sick and people with CF are highly susceptible to catching germs from others as well. There are thousands of people with CF who would have liked to have attended the hearing today but for safety reasons I must be their voice today.

## **II. Personal Experience**

### **A. Unmet Medical Need**

I first learned about inhaled Aztreonam from a CF Hotline caller. She told me of the dramatic improvement in her health she had experienced after participating in a research study for Aztreonam. At the time, I had been experiencing an increase in CF related health issues and had become resistant to the inhaled antibiotic TOBI. This left me without an FDA approved drug to treat *Pseudomonas aeruginosa* that was colonized in my lungs. I would wake up in the morning and be suffocating from the thick mucus that had developed in my lungs while I had been sleeping. I had to perform 4 hours of medical treatments to try and clear some of the mucus from my lungs. The CF patient has mucous that is the consistency of silly puddy and it is very difficult to clear it from the lungs. My weight would fluctuate between 90-102 pounds depending on if I was experiencing a pulmonary exacerbation. Low body weight is dangerous for a person with CF.

I was afraid to use an off label drug that was not designed or intended to be used as an inhaled antibiotic. I had heard from others with CF who had called the CF Hotline who had lost their hearing while using inhaled Colystin off-label.

I experienced 4 pulmonary exacerbations in 2005. I needed IV antibiotics that were administered every 6 hours. Every medication took 2 hours to infuse so I only had 3 hour periods when I was not infusing IV antibiotics. I was unable to participate in activities with my husband of 21 years and my daughter who was 5 years old at the time. My CF physician was suggesting I stop work. I love my work and was devastated at the idea of having to retire.

### **B. Beginning the Study**

I enrolled in the inhaled Aztreonam 005 study at the CF Care Center in Houston, Texas at the end of November 2005. I started taking medication at the beginning of January of 2006. The study was a double blind study, but I knew I was receiving the drug. Children and adults who live with CF are very aware of their symptoms and can easily detect either an improvement or a decline in their health. Given the complexities of the disease we become very adept at reporting symptoms to our physicians and family members. We know how important it is to monitor changes in our health. I immediately experienced a dramatic increase in my health in the first month of participating in the study:

- I was able to easily clear a large amount of mucus from my airways;
- The color of my mucus changed from dark green (always an indication that I had a lung infection) to clear;

- I went from coughing every 10 minutes to coughing only a few times a day or not at all.
- My pulmonary function went from an FEV1 of 50% to an FEV1 of 80%.
- My weight started to increase from a low of 90 pounds to 118 pounds.
- To state it simply: I had never felt so good.

My experience with Aztreonam has continued to be extremely positive. Before I began using the drug, when I did a pulmonary function test at the CF Care Center my FEV1 was between 50%-60%. After use of Aztreonam my FEV1 has been stable at 80%. My body weight before Aztreonam was between 90-102 pounds, which for someone with CF is not a good thing. Being nutritionally compromised makes someone with CF more susceptible to infection and makes it harder to fight off infection. My weight has increased to 118 pounds. While most people would not want to gain weight I know that my ability to keep weight on indicates that my health has improved and that the weight helps me fight off infections.

Every other month I take Aztreonam 3 times a day. I rarely miss a dose because I see such a dramatic improvement in my health when I use the drug 3 times a day. Each treatment takes 2-3 minutes to administer via a small handheld nebulizer. The positive health effects of Aztreonam usually last until the end of the 2<sup>nd</sup> week I have been off the drug. Then the final 2 weeks without the drug the mucus in my lungs increases and turns green. After 4 weeks off of the drug I begin taking Aztreonam and my mucus turns to clear in about a week, is reduced to almost nothing and it becomes extremely easy to breathe again.

As most CF patients age, they have increased frequency of pulmonary exacerbations and declining FEV1. Between 1995 and 2005 my FEV1 had been declining by 4-5 points each year. If I had not been on Aztreonam I know my FEV1 would have continued to decline. I expect I would not have been able to travel here today and would have had an FEV1 of 30% which would mean I would have been considering lung transplantation. There is a strong likelihood I would not have lived to be transplanted and would have died after complications caused by a *Pseudomonas aeruginosa* infection. Aztreonam has saved my life.

### **C. Enrollment in Early Access Program**

When the 005 study ended I was able to enroll in the 006 Open Label Follow-up Study, which ran from April 2006 to August 2007. After that, I enrolled in the Early Access Program which was available to those who participated in the 005 study

regardless of pulmonary function. There is not a CF Care Center in the Houston area that has an Early Access Program so I must travel to Denver every other month to obtain Aztreonam. I know my health would deteriorate if I did not have access to Aztreonam. I am lucky that my health is good enough to travel and that I have the funds to travel to Denver. Most of the CF community is not as lucky.

### **III. The Early Access Program is not an Adequate Substitute for FDA Approval**

The Early Access Program (“EAP”) is not an adequate substitute for the FDA’s approval of the drug and general availability of Aztreonam to the CF community that will follow approval. Most CF patients who are eligible for participation in the EAP but do not have an EAP in their city are either too sick to travel to a CF Center with an EAP or do not have the funds to do so. Only a small number of patients participated in the 005 study and if you did not participate in that trial the only way to participate in the EAP is to have an FEV1 of 50% or below.

Many patients with an FEV1 above 50% need an FDA approved inhaled drug for the months that they are not on TOBI. TOBI can only be used for 28 days and then the patient must stop taking the drug for 28 days. Those individuals are not able to obtain Aztreonam from the Early Access Program. Those who have an FEV1 above 50% but are resistant to TOBI are left with no other choice but the use of drugs that were not intended or designed for inhaled use. Such off label use has proven dangerous and deadly.

### **IV. Use of Off Label Drugs not Intended for Inhalation**

Off label use of certain IV drugs like Colystin, as an inhalation drug are dangerous and not a substitute for the use of an FDA approved drug. These drugs were never intended or designed to be administered via inhalation. Patients use these drugs off label because they are desperate for a drug to treat their pulmonary infection. Physicians resort to use of these drugs off label because they have no other FDA drug to use for these patients.

The main drug used off label as an inhaled drug is a drug known as Colystin. A limited number of patients use Colystin off label but most patients are too afraid to use Colystin. My experience with the Hotline leads me to believe that most patients are unwilling to accept the substantial health risks, including toxicity, renal damage, neurotoxicity and broncho-spasm associated with inhaled Colystin. Also there has been one death, reported to the FDA in the past 2 years, from the use of inhaled Colystin which has made many physicians and patients afraid to use the drug.

It is unacceptable for children and adults with CF to be left with no other alternative to treatment than use of an off label drug that has been shown to be dangerous when Aztreonam has been extensively studied and has no safety concerns. People with CF need access to Aztreonam now.

## **V. Conclusion**

Thirty other countries, including Canada and all countries in the European Union have approved Aztreonam. The CF community is wondering why patients with CF in the United States do not have the same access to Aztreonam as the rest of the industrialized world. Access to Aztreonam will give children and adults with CF the opportunity to live a better life. You have the power to change the lives of people with CF who struggle every day just to breathe by advising the FDA that Aztreonam should be approved for use by individuals with CF.

It is difficult to count the number of patients and parents of children with CF who call the Hotline and express their deep frustration and fear about the future. That frustration has increased since the FDA denied approval for Aztreonam in September of 2008. Patients and parents know that those who participated in the Aztreonam studies had a dramatic improvement in health and they cannot understand why they or their child does not have access to the drug. They fear that they – or their child – will continue to suffer needlessly or die prematurely while waiting for the FDA to approve Aztreonam. I know you will all do your best to make sure that children and adults in the CF community get the best holiday gift they could hope for- access to inhaled Aztreonam.

Beth Sufian, J.D.