What is it: As part of its commitment under the recently approved Prescription Drug User Fee Act (PDUFA V), the Food and Drug Administration (FDA) will be conducting an initiative, called the patient-focused drug development initiative, to provide for a more systematic approach to obtain the patient's perspective on the disease severity and the currently available treatments. The intent is to ensure a thorough understanding of the severity of the treated condition and the adequacy of the existing treatment options.

This initiative will be conducted for each of 20 different disease areas over a period of 5 years. The FDA has nominated an initial list of 39 diseases, including ME/CFS, using the following criteria:

- Disease areas that are chronic, symptomatic, or affect functioning and activities of daily living;
- Disease areas that reflect a range of severity;
- Disease areas for which aspects of the disease are not formally captured in clinical trials;
- Disease areas that have a severe impact on identifiable subpopulations (such as children or the elderly);
- Disease areas that represent a broad range in terms of size of the affected population
- Disease areas for which there are currently no therapies or very few therapies, or the available therapies do not directly affect how a patient feels, functions, or survives.

The next step in the process is for the FDA to gather public input between now and November 1st on which of disease areas should be selected for inclusion in this initiative. Additional disease areas may also be nominated during this time.

Note that initiative is in addition to the ME/CFS FDA stakeholder meeting that Dr. Woodcock, Director of the Center for Drug Evaluation and Research at the FDA, has already committed to.

How can you help: For ME/CFS, this is an excellent opportunity to help the FDA better understand how ME/CFS affects the patients. Your support is essential to ensure that ME/CFS is one of the 20 selected diseases.

Please send your comments in by November 1 to ensure that the FDA understands why ME/CFS should be selected as one of the 20 diseases.
A sample letter has been provided in case you want to pull from it to develop your own. As you write your own letter, make points that reflect how ME/CFS meets the criteria above.

Comments should be submitted electronically at: http://www.regulations.gov/#!docketDetail;D=FDA-2012-N-0967 . Select 'Individual Consumer' for the 'Category' and 'None' for 'Organization' if no other choice is appropriate . Written comments can be submitted to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, Rm. 1061, Rockville, MD 20852.

Further information on the patient focused drug development initiative and the list of 39 diseases initially nominated can be found here: https://www.federalregister.gov/articles/2012/09/24/2012-23454/prescription-drug-user-fee-act-patient-focused-drug-development-public-meeting-and-request-for

Sample Letter

Sample letter must be limited to 2000 characters

To: FDA Patient Focused Drug Development Initiative
From:

I am writing to request that chronic fatigue syndrome (also called myalgic encephalomyelitis or ME/CFS) be included as one of the 20 diseases in the patient focused drug development initiative.

ME/CFS is a complex, neuroimmune disease that affects one million Americans. It affects people of all ages, races and income levels. Patients can be sick for decades, with 25% house, bed or wheelchair bound, struggling to take care of themselves, let alone take care of their families or work. According to the CDC, ME/CFS can be as debilitating as Multiple Sclerosis (MS), end-stage renal disease, chronic obstructive pulmonary disease (COPD) and similar chronic conditions. One study suggests patients can die prematurely from cancer, heart disease and suicide.

What is tragic is that almost thirty years after the outbreaks that brought ME/CFS to national attention, there have been almost no clinical trials for drugs to treat ME/CFS and there are still NO approved treatments and NO biomarkers or outcome measures have been agreed upon.

(include your personal story here)

By ensuring a thorough understanding of the severity of ME/CFS from a patient perspective, especially given the lack of any viable treatments to change the course of the disease, the patient focused drug development initiative could make a tremendous difference in the lives of one million Americans.