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Shoreview, MN 55126

October 8, 2012

Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Dear Sir or Madam:

Subject: Docket No. FDA-2012-N-0967

I am writing in response to the request for public comments relating to the FDA's patient-focused drug development initiative, which is being conducted under the terms of the fifth authorization of the Prescription Drug User Fee Act (PDUFA V).

In section II of the document identified by the docket number above, the FDA lists 39 disease areas that are "potential candidates for the focus of one of the 20 future public meetings." This preliminary list ranges from very serious diseases such as HIV, heart failure and lung disease to generally non-life-threatening conditions such as alopecia areata, irritable bowel syndrome and chronic fatigue syndrome. Nocturia (the need to get up in the night to urinate), "fracture healing" and female sexual dysfunction are among 39 conditions the FDA identifies as being under consideration for this program.

Amyotrophic lateral sclerosis (ALS) or "Lou Gehrig's disease" does not make the list.

Identified criteria for making recommendations for additional disease areas to consider include those:

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

I have ALS and can attest to the fact that this disease meets all of the above criteria. It is incurable, always fatal, and the last criterion listed above applies to ALS as to perhaps no other disease. ALS patients have a life expectancy of 2 to 5 years from diagnosis. The one drug approved by the FDA to treat it (riluzole) is thought to provide an average of an additional three months survival. I just completed a phase III trial of a new drug (dexpramipexole) that it is hoped will realize an average reduction in disease progression of 30%. So perhaps, if it is successful, it will provide patients an average life extension of one half-year to 18 months.

We have nothing else. Several drugs, however, are in the early stages of development. But–and this relates to the third criterion on the list–it has proven to be very difficult to develop and conduct effective drug trials for ALS. Currently, most trials accept only patients who can begin therapy within

the first two years after the onset of their symptoms. The average length of time from earliest symptoms to diagnosis is one year, so ALS patients typically have a very short window in which they are eligible to join trials. Since only about 5,600 people are diagnosed in the U. S. each year and many of these will be ineligible for trials for a variety of reasons other than the duration of their symptoms, the pool of possible test subjects is not large. Unfortunately, because of the great variability in individual presentations of the disease, each study requires a sizeable number of participants in order to produce statistically significant results.

Within the community of people with ALS (PALS), there is widespread dissatisfaction with and distrust of pharmaceutical companies and, especially, the FDA. Reasons for this include the perception that the FDA is insensitive to the needs of a group of people who are faced with an unimaginably cruel and incurable disease, and that the agency's regulations serve to stifle and slow drug development. Further, that the FDA stands in the way of the humanitarian use of drugs that while not yet proven to be of benefit, have passed basic safety tests and do have at least the potential to be of help. Some PALS have taken to designing and conducting their own experiments, and are using the Internet to publish their results and to recruit others who are unwilling to wait on slowly-conducted FDA-approved trials. Their stories are being told in the media (see for example “Frustrated ALS Patients Concoct Their Own Drug” in the Wall Street Journal, April 15, 2012; the story has also been covered by the New York Times, ABC News and others). Another group, the registered nonprofit ALS Treat Us Now (www.treatalsnow.org) uses social media to push the FDA to allow expanded access programs for PALS. Nearly 20,000 people have joined this effort.

There is a very real potential that frustration within the ALS community will result in fewer willing volunteers for FDA-approved drug trials. This could have a devastating effect on hopes for a cure in a drug development climate that is under significant stress (see the September 2012 report by The President's Council of Advisers on Science and Technology). Meanwhile there are indications that the incidences of ALS are rising. It is more important now than ever to establish and maintain a relationship of trust between the ALS community, the FDA, and drug companies. Leaving ALS off of any list of 20 diseases targeted for special attention and increased communication will be a grave mistake and a serious injustice.

Please consider adding amyotrophic lateral sclerosis to the list of disease areas given priority in the FDA's patient-focused drug development initiative under PDUFA V.

Sincerely yours,

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