Preliminary Results - April 2013 FDA Workshop Survey

Demographics:

85% of our subjects were female and almost all subjects were Caucasian. The average age was 51 and the average duration of illness was 18 years. For 50% of our respondents, this survey is the only way they are participating in this workshop.

Symptoms:

The 5 most significant symptoms were fatigue, post-exertional malaise, pain, sleep, and cognitive problems. Symptoms that haven't been studied as much such as multiple chemical sensitivities, gastrointestinal symptoms, and orthostatic intolerance were also deemed to be significant by over 50% of our subjects..

99% felt that their illness was not improving over time, citing worsening of existing symptoms and appearance of new symptoms.

Out of five objective tests we asked about, 66% had at least one abnormal result. For natural killer cell activity, for example, 70% noted an abnormal result. Tests like these could be used as study inclusion criteria, as outcome measures, and as a way to select subgroups for analysis. We believe that studying subgroups may help yield more effective treatments given the heterogenous nature of CFS.

Impact on Daily Life:

Using a standardized measure of physical functioning, we found our respondents to be more disabled than 95% of the general US population. Only 13% were employed, with almost all citing ME and or CFS as the reason for why they could not work. For even basic personal care, 89% needed assistance or had to change their pre-illness routine. On their worse days, 60% were bedridden. On their best days, 75% could only do some light housework or less.

Perspectives on Current Treatment:

75% of people felt current treatments were not helpful or only slightly helpful but not enough to improve their day-to-day function. Patients repeatedly wrote about the need

for disease-modifying treatments and not only for treatments that helped control symptoms.

We asked about how well currently recommended treatments worked the illness overall and for three common symptoms: sleep, pain, and cognitive problems.

These treatments were cited as helpful by more than 50% of subjects:

- for the overall illness balancing rest with activity, restricting or modifying physical/ mental activities
- for sleep CPAP, over-the-counter medicines, zolpidem, eszoplicone, TCAs, trazodone, benzodiazepines
- for pain short/ long-acting opiods, flexeril, ibuprofen

Some alternative/ complementary/ non-drug treatments also made the cut. Helpful prescription drugs cited in patient comments included gamma-hyroxybutyric acid for sleep, lisdexamfetamine for thinking, tizanadine/ baclofen for pain, and midodrine/ florinef/ beta-blockers for orthostatic intolerance. In contrast, exercise programs, administered by a professional and part of the current standard of care, were noted by 62% as worsening their health.

The main reasons cited for stopping a medicine were side effects followed by decrease in effectiveness over time, especially with sleep medications. For effective non-drug treatments, cost and insurance coverage were major reasons for discontinuation. Respondents were also concerned being more sensitive to drugs and the dependency potential of some medications.