

Testimony

Joan Grobstein, M.D.

It's hard to know where to start with this testimony. There is so much that needs to be done. It is possible that the leaders of the NIH think that they have done enough for now, having arranged a State of the Knowledge Workshop. However, a single Workshop does not address all of the barriers that patients with ME/CFS face on a daily basis: difficulty obtaining disability benefits, difficulty finding a knowledgeable doctor, difficulty paying for expensive treatments if they can get them at all, difficulty establishing that they have a credible disease so that their family, friends, neighbors and health care providers do not look on them with scorn, difficulty with their daily activities.

How can the CFSAC help with all of this? First and foremost, **it can make a strong recommendation to the Secretary that no further taxpayer-funded research be done on patient populations defined by the Reeves (2005) criteria.** This research is misleading and a waste of money. It leads away from the truth about this disease, not toward it. The Committee has already made a recommendation, in 2009, condemning the use of this definition. Secretary Sebelius, or her deputy, needs to respond to this recommendation. It must be implemented.

The CFSAC also needs to endorse a better definition of this disease, the Canadian Consensus Definition, for all future research. The NIH and CDC should provide leadership by insisting that all future research on ME/CFS include details about which of the criteria for diagnosis subjects do and do not meet, so that data can be easily analyzed to begin to establish subgroups. Some patients are concerned that they do not meet the Canadian Consensus Definition, although they have been diagnosed with CFS by the Fukuda criteria. Until we have more information, clinicians should understand that there is undoubtedly a spectrum of this illness and also that symptom prominence waxes and wanes. However, in order to define a consistent data set, research criteria must be strict.

The NIH should convene a workshop to discuss appropriate study designs for research about ME/CFS. There are several issues: research studies must be adequately sized to allow for analysis of subgroups. In the past, small negative studies with numbers as small as twenty to forty patients have been used to justify abandoning promising avenues of research. Since there has been so little funding for any research at all, researchers are reluctant to revisit a negative study. Many

of the studies in the past have looked at patients who probably didn't meet the Canadian Consensus Criteria. Some of the patients may well have had Idiopathic Chronic Fatigue, a different diagnosis. The studies will have to be repeated using appropriate patient groups and study designs.

It was clear at the NIH State of the Knowledge conference that at least some potential biomarkers are not measurable until the subjects are challenged, either by a mental task or by repeated exercise challenge over at least two days. This may be true of all biomarkers, including blood tests for XMRV and other pathogens. Challenge conditions should be a part of all study designs until we know what biomarkers are reliable without challenge conditions.

Dr. Rowe's concerns about study designs for ME/CFS should be taken very seriously. If he is willing, he should lead the study design workshop. There should be serious consideration of n of one study designs, as well as other innovative designs that can lead to faster conclusions, as well as take into account the heterogeneity of this disease.

Another focus of the design workshop or, if necessary, a separate workshop should be Dr. Giorgioni's excellent suggestion that a web-based password-protected database be established for ME/CFS. Consistency of data collection is key, and there will need to be considerable discussion of what data is collected and how. This will facilitate collaboration among researchers, allow for larger sample sizes and result in faster synthesis of results that can benefit patients.

A clinical database should be established at the NIH. Doctors who are seeing large numbers of ME/CFS patients should be encouraged to submit data on their well-characterized patients. The database should include baseline data, and longitudinal data. Doctors should be compensated adequately for participating in the database. A workshop should be convened of expert ME/CFS doctors to determine how to implement this database.

The NIH must devote considerably more funds to ME/CFS research. This should be accomplished in whatever way works best. From the outside it looks like this will only be accomplished by moving ME/CFS research into one of the Institutes, where it will have a Director who can ensure the research is high quality and cost-effective.

The CDC must stop using its current data set for research. The Reeves (2005)-defined data set has been discredited. It is a waste of time to continue to use it.

The CDC should be required to do epidemiological studies of all cluster and family outbreaks of ME/CFS. This should have been done long ago.

The CDC needs to revise its website. This website is one of the first places newly diagnosed patients and their families and physicians go for information. It needs to accurately reflect what we know about the illness, not what the CDC imagines we know. It should include the recommendation that patients be tested for viral illnesses, orthostatic intolerance, autonomic dysfunction, low blood volume, natural killer function and other immunological markers as they are established, and sleep abnormalities. Treatments for each of these should be recommended.

Patients cannot wait two or three years for the results of the Lipkin study and the Blood Group study to find out if they need to be treated for XMRV. They also need information about transmissibility sooner rather than later. A true replication study of the original Lombardi study needs to be done as soon as possible, using an equivalent patient population and all the techniques used in that study.

The very sickest patients need study and treatment. It is not currently clear how this can be accomplished, since most are ignored by the medical system. Perhaps the CDC and NIH could work together to do develop a plan to help these very seriously ill patients. Perhaps a multi-disciplinary study could be implemented at one of the clinical research centers at the NIH or at several university-based clinical research centers. At the very least, a plan needs to be developed to serve these patients.

A clinical trials network is needed.

There is much more that needs to be done, but if these few things could be done in the next six months it would be the beginning of much-needed change. Many are revenue-neutral, or, in the words of Christine Williams of AHRQ “decimal dust”. The increase in funding for research is, of course, costly. But the cost of doing nothing is higher.