Public Comment

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The recent (April 25/26 2013) FDA Drug Development for ME and CFS Public Workshop is much appreciated. The engagement and collaboration with which this meeting was arranged and conducted can serve as helpful models for all agencies. The interactive format of the panels and patient participation helped produce clarifying questions which seem to have resulted in a deeper understanding of the impact of ME on our lives.
We look forward to continued collaboration with FDA on the next steps forward such as Guidance for Industry.

The FDA meeting made it clear that there are building blocks that must be set in place before we can expect successful drug development for ME.

We need consensus on definition – this can be right away done by adopting the Canadian Consensus Criteria (http://www.mefmaction.com/images/stories/Medical/ME-CFS-Consensus-Document.pdf) to define ME.

Consensus on definition will provide framework for development of clinical trial structure. Using appropriate definitions and cohorts in appropriately funded studies will aid in identifying outcome measures and biomarkers.

We also need adequate funding for ME research, identification of, and agreement on outcome measures and validation of biomarkers.

Making the change from a diagnosis of CFS to ME will require (among other things), changing research, insurance and disability guidelines as well as changing medical education. Managing these changes must be done in full partnership with ME stakeholders – including patients, caregivers, advocates and ME experts.

In order to implement the necessary building blocks, I strongly urge that the CFSAC 2013 List of High Priority Recommendations include the following recommendations as originally worded by the committee.

Regarding research:

1. **NIH should fund ME/CFS research commensurate with the magnitude of the problem, and issue an RFA specifically for ME/CFS.** Recommendation made May 2011 - ME/CFS is an illness with enormous economic and human costs. The April 2011 NIH State of Knowledge Workshop identified a number of gaps in what is known about the illness. To address these gaps warrants an interagency effort comprising, but not limited to, NIH,CDC, and AHRQ. Further, the focus should be on interdisciplinary discovery and translational research involving interacting networks of clinical and basic

science researchers. Areas to be examined would include the following: identification of patient subsets for detailed phenotyping and targeted therapeutic interventions, biomarker discovery, systems biology approaches and disability assessment. To facilitate the above goal, CFSAC recommends that ME/CFS research receive funding commensurate with the magnitude of the problem and that the NIH (and/or other appropriate agencies) issue an RFA specifically for ME/CFS.

2. **Pool resources to create Centers of Excellence, using physical or virtual locations.** Recommendation made November 2011 - CFSAC would like to encourage and support the creation of the DHHS Interagency Working Group on Chronic Fatigue Syndrome and ask this group to work together to pool resources that would put into place the "Centers of Excellence" concept that has been recommended repeatedly by this advisory committee. Specifically, CFSAC encourages utilizing HHS agency programs and demonstration projects, available through the various agencies, to develop and coordinate an effort supporting innovative platforms that facilitate evaluation and treatment, research, and public and provider education. These could take the form of appropriately staffed physical locations, or be virtual networks comprising groups of qualified individuals who interact through a variety of electronic media. Outreach and availability to underserved populations, including people who do not have access to expert care, should be a priority in this effort.

3. **NIH should issue a $7-10 million RFA for outcomes measures, and biomarker discovery and validation.** Recommendation made October 2012 - CFSAC recommends that you instruct the NIH to issue an RFA (funded at the $7-10 million range) for projects to establish outcomes measures for ME/CFS diagnosis, prognosis and treatment which would include but not be limited to biomarker discovery and validation in patients with ME/CFS.

And because it does not reflect appropriate treatment for patients, and also because it undermines the necessary building blocks for success:

4. **Remove the CDC Toolkit**. Recommendation made June 2012 - CFSAC asks that the Centers for Disease Control and Prevention (CDC) remove the CFS Toolkit (both English and Spanish versions) from the CDC website.

On a different note – please post the Guideline.gov link to the IACFMSE Primer http://guideline.gov/content.aspx?id=38316 on the front page of the CFSAC website. Easy access to the Primer via that link will be very helpful for all who come to the CFSAC website.