New Solve ME/CFS Initiative Patient Registry – Lily Chu, MD, MSHS

Recruiting participants for medical studies is a major, if not the top, barrier to accelerating progress for many medical conditions. Studies are delayed by months to years if not abandoned entirely due to not being able to recruit enough subjects. For a condition like ME/CFS where an estimated 84%-91% of people affected are not yet diagnosed, an easily accessible and quick way for researchers to identify potential subjects is crucial for progress.

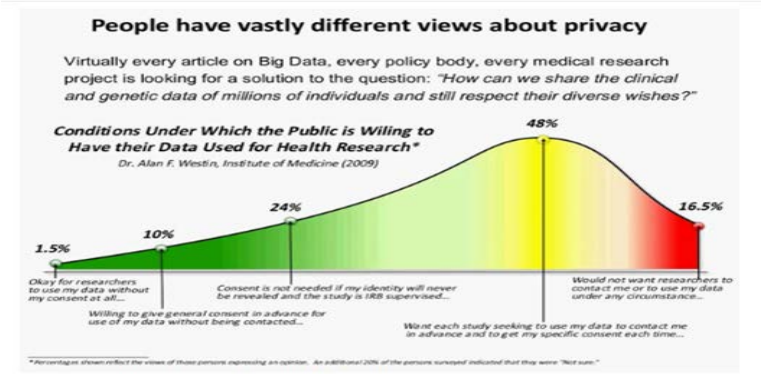
Consequently, it piqued Dr. Lily Chu's interest earlier this year when she heard that the Solve ME/CFS Initiative (SMCI) had been awarded a grant by the Robert Wood Johnson Foundation White Label PEER (Platform for Engaging Everyone Responsibly) program to take advantage of a proprietary online registry platform that has some unique features. Recently, she had a chance to talk with SMCI's Dr. Zaher Nahle about what this means for patients, clinicians, and researchers. Note that SMCI has operated a REDCap (Research Electronic Data Capture application) patient registry and biobank since 2008 but this registry is a sophisticated platform independent of those projects.

These are early days yet so some things mentioned might be different in the final version of the registry and other aspects are not yet fully developed.

From a patient perspective:

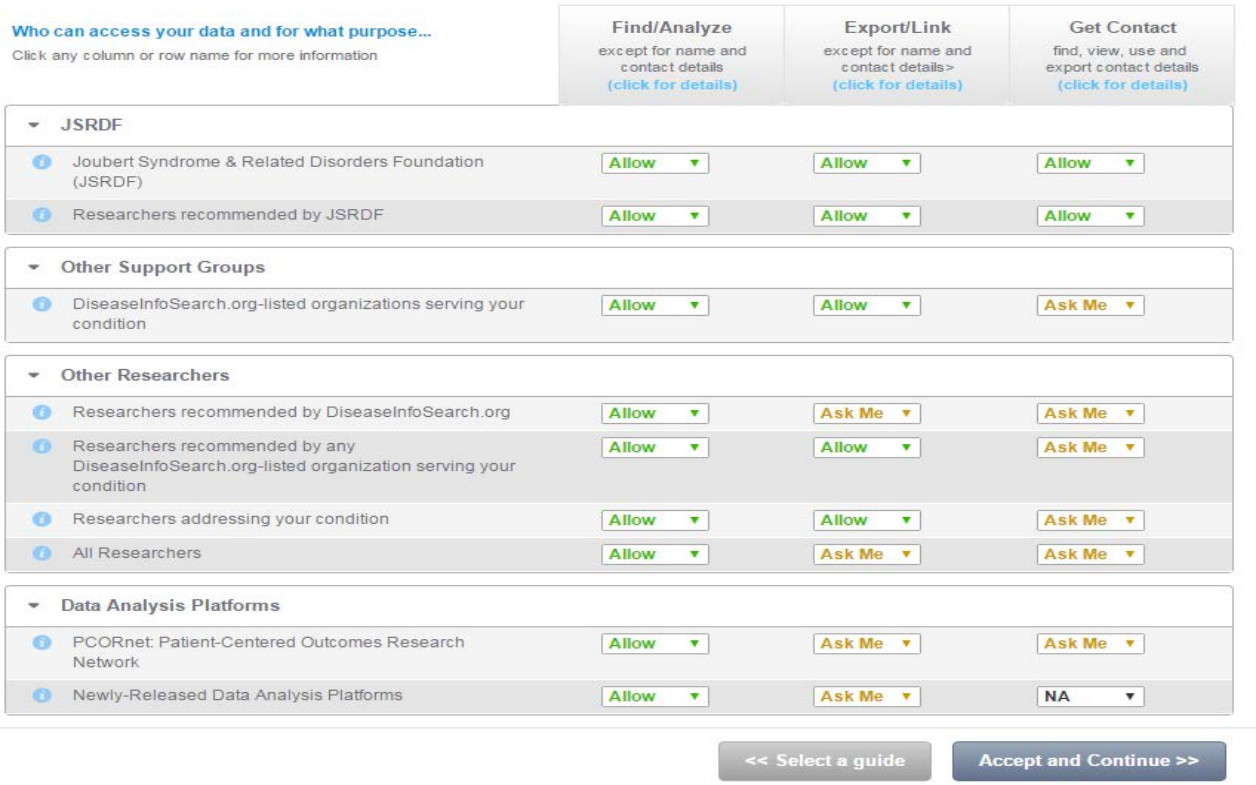
SMCI will inform patients, their supporters, and clinicians about the program through various channels like their newsletter, website, Facebook page, and webinars. Patients can self-enroll directly or via certain clinicians; in some cases, clinicians may be upload patient data onto the registry itself. The enrollment process consists of answering basic demographic items and a series of questionnaires. This information is used for contacting patients in the future, can be analyzed for studies, or used by researchers to recruit particular subjects.

A unique feature of PEER system is that patients or their guardians, and not SMCI, PEER, researchers, or other entities, determine which pieces of information they want to share, who they want to share it with, and the level of sharing. On PEER's website is a picture (see below) showing US residents perspectives about sharing their personal data for research. A minority of subjects, 1.5% and 16.5%, respectively, either are willing to share all data without giving consent or not share any data at all but most people, 48%, opt for a moderate stance, open to sharing their personal information if they are informed of the study beforehand and give consent.



Sharon Terry of Genetic Alliance discusses participant engagment.

During the enrollment process, patients are shown a chart (see below with the disease condition Joubert Syndrome as an example) with the rows corresponding to different groups (e.g. the sponsoring organization (SMCI), their affiliated researchers, all researchers asking for information) and the columns corresponding to different privileges those groups have (e.g. Can the group received the individual's contact information? Can the group use an individual's data if identifying information is removed?, etc.) For each category corresponding to a specific group and a specific privilege, patient can select one of three options from a drop-down menu: "Allow", "Do Not Allow", and "Ask Me." The latter option means the patient is open to being further contacted with details by the specific group and may make their decision based on those details. Patients may also choose to see video vignettes of patients, supporters, clinicians, and researchers with their advice on how to go about choosing options. No option chosen is permanent and patients may go back to change their answers at a later time.



While somewhat cumbersome, in my opinion, this does allow patients more exacting control of who gets their information and how it may be used. Other than concerns about unintentional data leaks and just each individual's personal preference for privacy, some private companies, like PatientsLikeMe, and even national health systems, like England's National Health Service, have been in the news for sellin individuals' data to groups like insurance companies and the pharmaceutical industry. While this might be acceptable with individual consent, some groups have not told patients about this aspect of their business model while others bury the information in small, fine print and legalese. For this registry, SMCI assures me they have no intention of selling enrollee information without clear consent.

As mentioned earlier, researchers will use information directly from the registry or conversely contact enrollees who give permission to be contacted when there is a study that fits them. Whether study participants received individual results or not after a study is completed will be decided on a study-by-study basis by the researchers involved. SMCI will convey aggregate results of studies to participants via the communication outlets they have already.

From a researcher perspective:

Over the years, SMCI has had contact with multiple researchers and institutions and these individuals/ groups will be informed of the registry and invited to participate. Information about the registry will also be disseminated to the general medical and scientific community. The registry is still in development as particular attention is being paid to the effective design of common data elements; at some point, a data dictionary with details about what types of subjects

and what information or surveys make up the registry will be made available. Researchers can inquire about becoming a member without a specific project in mind OR can submit a research proposal either using the registry data available or asking to contact subjects so that information beyond that in the registry can be collected. For now, SMCI anticipates that no fees will be asked of researchers to obtain participant-level data. However, researchers and their proposals will be vetted and approved beforehand by SMCI's Research Advisory Council (RAC) , which also will include some lay reviewers.

There are no specific requirements currently in terms of publication, e.g. how soon after study completion results need to be submitted for publication, what types of journals the articles need to be submitted to, whether results need to be presented at conferences, etc., but it is expected that all and any use of this registry will be shared with the community with a particular emphasis on rigor and quality.

Conclusion:

Overall, a patient registry like SMCI's holds great promise in helping to advance ME/CFS research. Individuals patients will have to assess for themselves how to balance their individual privacy needs with their desires to help advance the research: the PEER system allows them a unique way to express their views on this issue to researchers. For researchers, the registry may make study recruitment much easier and faster. Some questions remain which I hope can be addressed in the future including how the registry will be sustained financially and logistically over time, how underrepresented patient groups in ME/CFS research (e.g. children, ethnic minorities, men, etc.) can be incentivized to join, and how SMCI will comply with growing societal demands (e.g. from journal editors and readers) for access to raw data. I also brought up the idea of having SMCI ask researchers who benefit from the registry to "re-deposit" any new data they collect into the registry so that future researchers can use it and that was welcomed. In this context, Nahle explained that they have a major outcome research initiative on this coming up soon that will interface with the new platform and national resources. If you have any comments or questions about the registry or registries in general, feel free to write us here at Newslettereditor@iacfsme.org.