

Calling all patients

Disease-orientated consumer online communities radically change the way in which individuals monitor their health, but they could also create new ways of testing treatments and speed patient recruitment into clinical trials.

Several online communities for patients now offer a wealth of anecdotal and factual information about health, and tools for networking with like-minded individuals. The web sites are public, collaborative and simple to use. They are also starting to offer patients content that goes beyond what is available through existing gatekeeper-controlled healthcare infrastructures. Some even offer to host personal medical data, empowering patients to understand and manage their individual care in a manner that is powerful and disruptive to current medical practice. If these 'user-generated healthcare' data can be harnessed with data from conventional biomedical and clinical research, the benefits could extend beyond patients to payors, providers and the drug industry itself.

According to a 2006 study by the Pew Research Center, in the United States alone 80% of Internet users (~113 million adults) obtain health information from the web—often before they even visit a medical professional. But the Internet also provides a means of exchanging ideas and information. And over the past few years, decentralized, user-generated patient community social networks, such as DailyStrength (<http://www.dailystrength.org/>) or Inspire (<http://www.inspire.com/>), have sprung up to give information about disorders and facilitate connections with other sufferers.

At first glance, PatientsLikeMe (<http://www.patientslikeme.com/>) looks similar to these patient chat rooms and support groups. The site, which has been operating since 2006, currently has >7,000 registrants organized into disease-based communities, such as amyotrophic later sclerosis (ALS), Parkinson's, multiple sclerosis, AIDS, depression and anxiety.

But there is a key difference. PatientsLikeMe asks patients to record data about themselves and share it in an open environment. Using standardized metrics provided on the site, patients can log their symptoms, severity and progression, and drug regimens and dosages, together with the efficacy and side effects. All the data is then neatly displayed in bar graphs and progress curves.

Patients can thus rapidly identify others with similar ailments in similar stages of disease. They can use the wisdom of the crowd to learn which treatments work and which don't. This is particularly useful for patients with rare conditions (and their physicians) who might not otherwise encounter comparable sufferers.

And for patients with limited life expectancy, the ability to participate in a very rough, low-level clinical study on a new treatment is far more appealing and timely than waiting for clinical data to be published in peer-reviewed literature. An example of how user-generated data could speed treatment uptake and evaluation is illustrated by a paper published in February (*Proc. Natl. Acad. Sci. USA* **105**, 2052–2057; 2008) that suggested that the mood-stabilizer drug lithium delays progression of ALS. PatientsLikeMe was able to rapidly recruit >100 patients (more than four times as many as in the original trial) and, with physician cooperation, put them on lithium and track their progress. A rich data set is thus being captured that is unlike anything else coming out of healthcare.

But the approach is clearly not without its problems. Systems like PatientsLikeMe do not provide ideal clinical data. User-generated data is highly variable and poorly controlled. Factors like the over-reporting of symptoms, 'placebo effect', data quality, selection bias and attrition bias will all affect user-reported outcomes. Users might self-medicate without consulting their physician. And, perhaps most importantly, confidentiality is clearly compromised, particularly as PatientsLikeMe plans to sell anonymized, aggregated patient data to drug companies. And then there is a question as to whether consumers who post their personal health information fully understand the potential for harm and misuse.

But such concerns are unlikely to weigh too heavily on people who are facing their own death armed with charts of the fates of others. And the user-generated medical data paradigm could also have profound implications for drug developers and clinical development.

First, PatientsLikeMe (and web sites like it) could be used to speed patient recruitment to trials—an inefficient process of protracted referrals to clinical centers that eats up company funds and stalls development programs. Indeed, Novartis is already collaborating with the website to recruit patients for a trial of a multiple sclerosis drug.

Second, patient-reported data could be used to complement the traditional adverse-event reporting system. Again, the data would not be as reliable as data from physicians or other professionals, but it could direct the formal vigilance of physicians, healthcare providers and companies.

Third, companies might also be able to use website data to gather information on treatment outcomes when patients use drugs in off-label indications. Again, user-generated data would not be as definitive as more traditional studies, but would be a rapid means of providing hints as to ways in which drugs could be expanded into new markets. In effect, user-generated data would also provide companies with a rapid, cost-effective means of assessing lack of efficacy or presence of toxicity, facilitating the decision not to go ahead with large-scale clinical trials.

Perhaps most intriguing, disease community sites like PatientsLikeMe could also expose snake-oil therapies. One of the biggest problems in healthcare at present is the suffering and financial ruin that patients experience when conventional caregivers run out of options and patients turn to treatments of last resort. At present, there is no way of gathering reliable information on, for example, the use of stem cell transplants in off-shore clinics. User-generated healthcare with reporting systems like PatientsLikeMe could dissuade patients from continuing to spend their life's savings on futile treatments.

Hands-on, patient-driven healthcare is not the answer to all of medicine's current travails. If used poorly, it might even encourage patients to reach for options that aggravate, rather than help, their conditions. But if adequate patient protections and safeguards to avoid 'gaming' of the system can be put in place, the potential for driving just a few of the snake-oil sellers out of business makes it worth a serious look.