When Disruption is Nothing More than Good Common Sense

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We need to stop saying sponsors and investigators *may* work with patients and start staying they *must* work with patients.

It's no secret that U.S. clinical trials are in crisis with powerfully depressing statistics documenting rampant recruitment and retention failures, glacial trial completion times, and burgeoning costs[1]. Billions of public and private dollars are being wasted annually due to issues with clinical trial quality and efficiency. The current underperforming research enterprise is an unsustainable dinosaur that fails patients at a time when there is increasing need for rigorous and timely scientific evidence to evaluate new devices, drugs, and biologics, to determine best medical practice, and to compare effectiveness of diagnostic and therapeutic alternatives. Time equals lives, and too many patients are losing theirs while waiting for this broken and band-aided system to produce answers and cures.

Cooperative design, a practice that places designers and users on equal footing, emerged in the Scandinavian IT space in the 1970s[2]. A similar concept known as participatory design has been in the English-speaking construct of North America since at least 1990[3], a time when HIV/AIDS and breast cancer advocates were on the front lines fighting to put patient needs at the center of research. Dr. Anthony Fauci of NIH and leaders of the U.S. FDA have said time and time again that these advocates had great impact. They forced the research and regulatory systems to be accountable and produce timely results for patients. Yet, some 25 years later, we are still trying to retrofit a sponsor- and investigator-centric system by *allowing* patient voice rather than *requiring* it.

The transformational change needed is nothing more than good common sense: stop saying sponsors and investigators *may* work with patients and start staying they *must* work with patients. If patients are to be subjected to a protocol and accept the risk and burden of participation, that protocol must be developed in partnership with patients or caregivers representative of the study population. Period. End of story. Additionally, to prevent recruitment and retention failures, no study or marketing application should move forward until a trial has been assessed by patients for feasibility and undergone a simulation exercise. The days of "our best guess" recruitment planning by people who've never organized and engaged a particular patient community must also come to an end. Further, benefit-risk assessments are imperative. Attempting to predict patients' values, preferences, and comfort level with uncertainty as an intellectual or observer-reported exercise is preposterous. Patients and caregivers with lived experience must be the ones to speak for their own communities.

Politely suggesting engagement with patients and caregivers in research is not enough. There is now qualitative and quantitative evidence on best practices for effective engagement with patient groups around clinical trials[4].

PDUFA negotiations are in process, and NIH is asking for an increase in biomedical research funding. It's time to educate sponsors of research about best practices for effective engagement with patients and then mandate it in NIH-funded and registration trials if we are to stop wasting our finite resources and truly put patients at the center.

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1. Tufts Center for the Study of Drug Development. Impact Report, 2013.
2. Joan Greenbaum and Morten Kyng (Eds), Design At Work - Cooperative Design of Computer Systems (Lawrence Erlbaum Associates, 1991).
3. Douglas Schuler and Aki Namioka (Eds), Participatory Design: Principles and Practices (Lawrence Erlbaum Associates, 1993); Marting Helander, Thomas Landauer, and Prasad Prabhu (Eds), Handbook of Human-Computer Interaction (Elsevier Science Inc., 1997), ch. 11.
4. Clinical Trials Transformation Initiative, "Patient Group and Clinical Trials Project," January 2015.