



Medical Hero in the Spotlight

Athlete embraces clinical trials with a competitor's spirit and hope

Randy Ulrey, a 39-year-old athlete and healthcare company vice president, is used to mastering tough challenges. Today, he is bringing the self-discipline, determination and healthy habits of a lifelong athlete to bear as a clinical trial participant battling cancer.

Fourteen years ago Randy discovered a mole on the upper left side of his back and was diagnosed with Stage I malignant melanoma, a cancer found in the outer layer of the skin that has not spread beyond the original cancer site. Doctors removed the mole and Randy went about his life. He stayed active in sports and tried to be careful about exposure to the sun.

In late 2009, Randy started noticing critical symptoms, including blood in his urine. His doctor ordered a CT scan, which revealed a tumor in his bladder and three metastases. Surgeons removed the tumor in the bladder, but could not remove those outside the bladder.

“Two other tumors are still present outside my right kidney,” says Randy. “We are fighting those with clinical trials medication.”

Randy's father asked the Center for Information & Study on Clinical Research Participation (CISCRP) to help locate a clinical trial that might benefit his son. CISCRP staff members ran a custom search and identified a number of studies dealing with melanoma.

“For me, the bigger decision was which clinical trial

to enroll in, not if I should enroll,” says Randy, who sought input from family, physicians, and friends in making his decision. “I have at least 25 valued people who I discuss this with and who give me opinions.”

Randy is currently enrolled in a clinical trial in Santa Monica. His experimental medication utilizes an existing drug mixed with a new medication. Randy has been going to a clinic weekly since autumn of 2009.

Randy's side effects from the medication include a bit of nausea and fatigue. For him, however, the big downside of taking part in a clinical trial is not the physical effect of the drugs, but the waiting time at the clinical trial site.

“I'm there for 4 to 5 hours every week,” he says. While the research team is knowledgeable and caring, Randy says it's so busy on Tuesdays; and with all the rules and regulations from the drug company, there is a patient bottleneck.

Despite being a cancer patient, Randy has the optimistic attitude of a top athlete. “My attitude is as good as it can be,” he says. “I'm as positive as I ever have been that I will beat this.”

Randy's current aim is to promote more efforts to help people learn about clinical trials; find the information they need; and help them make decisions about these potentially-lifesaving treatments.

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Making the right choice: Understanding the differences between standard treatment and clinical trials

7he decision to participate in a clinical trial is a deeply personal one. What's right for one person may not be right for another. Before making this important decision, it's crucial to understand the difference between standard medical treatment and care during a clinical trial.

When you receive treatment as a clinical trial volunteer, it's not the same as receiving standard medical treatment as a patient. In standard medical treatment, your doctor's only goal is to help you get better. To try to make that happen, she'll assess your condition, discuss treatment options with you, and recommend the treatment she thinks best meets your needs. In some cases, there may be a variety of treatment options for your condition, including medications and surgical procedures. Your doctor will discuss the pros and cons of those choices with you. During the course of your treatment, your doctor might alter your treatment to try and achieve better results or to alleviate any side effects you might be experiencing. For example, she might adjust the dosage of your medication or try a different prescription.

In a clinical trial, the researcher's goal is to find out how a drug or device works in your body. The trial is about collecting research data while making sure all volunteers are treated safely and fairly. The range of treatment options is limited by the trial's research design or "protocol." That means you only have access to what's allowed by the protocol.

There are medical benefits to participating in a clinical trial. Volunteers have access to new treatments, which may be more effective than standard therapies and may not be available elsewhere. Participants receive regular and careful medical attention from a research team that includes doctors and other health professionals, and they may be the first to benefit from the treatment being studied.

But participating in a clinical trial involves medical risks as well. You may experience unpleasant or even dangerous side effects. The drug or procedure being studied may not work as well as standard treatments, or you may be given a placebo or "dummy drug" instead of an active medication. This is important to consider if you are planning to stop your regular medical therapy in order to participate in a trial, since the study treatment may or may not be as effective.

Before consenting to participate in a trial, ask the researcher to compare the risks and benefits of the standard treatment to the potential risks and benefits of the trial and talk to your doctor about standard care so you can make an informed choice.

If you choose to participate in a trial, chances are you'll have questions along the way. Continue to ask questions of the trial staff and keep your own physician in the loop. Remember that when you sign up for a trial you do so with the understanding that you will be well treated. It's part of your responsibility as a volunteer to make sure that happens.



Serving on an IRB

Every day, medical research helps save and improve the quality of life for countless people. But the heroes behind medical research aren't just the scientists working to treat and prevent healthcare problems. They're the everyday people who step up to say, "I can help."

Among those heroes are the so-called "public members" who serve on the Institutional Review Boards (IRBs) that oversee all clinical trials. IRBs are independent committees whose job is to review, approve and monitor trials.

All IRBs share the same mission: to protect research volunteers and make sure a clinical trial adheres to high ethical and scientific standards. IRBs only approve trials if the potential benefits outweigh the foreseen risks, and if the rights of research volunteers – particularly vulnerable participants, such as prisoners, pregnant women, mentally disabled persons – are protected.

An IRB is responsible for reviewing everything from a study's protocols and informed consent form to its advertising with the express purpose of protecting volunteers. For example, an IRB will decide issues such as whether the use of a placebo in a trial is ethical or whether would-be volunteers might be unduly influenced by a certain level of monetary compensation. The IRB must also review and approve the consent form to make sure it is clear and comprehensive. The IRB serves as the overseeing body and the volunteers' advocate throughout the course of the study.

The federal government requires every clinical trial to have an IRB that consists of at least five members. Members must include at least one scientist, a non-scientist, such as a lawyer, religious leader or academic professor, and a "public member" who is not connected with any institution or sponsor related to the trial.



The public member's role is to bring a lay person's perspective to the board and serve as the voice of the community. As lay people, they aren't tied to the research institutions conducting the trials and aren't interested in solving scientific riddles. Rather, their job is to ask the questions and raise the concerns that members of the community might raise. If a lay member of the IRB can't understand a scientific concept in a protocol or finds the description of possible side effects confusing, chances are a research volunteer would have the same problem. By voicing those concerns, public members represent and safeguard research participants.

Serving as a member of an IRB is a big time commitment. The typical IRB at a university or hospital meets every other week and meetings can last for two or three hours. Some IRBs pay their community members to attend meetings, while others allow community members to participate via conference call. In addition to meetings, IRB members must spend a lot of time reviewing study protocols and consent forms.

Despite the demands, individuals who serve as public members of IRBs say it's gratifying to know their efforts keep study participants safe, while helping facilitate medical breakthroughs.

If you'd like to find an IRB near you, visit the Office for Human Research Protection's website and search their database:

<http://ohrp.cit.nih.gov/search/search.aspx>.

Hollywood celebrates a medical hero's story



Hollywood loves an underdog. It loves passion. It loves a hero.

It found them all in John Crowley. Crowley, who will serve as the keynote speaker at CISC RP's annual meeting June 14, is the real-life hero behind the just-released film, *Extraordinary Measures*.

Extraordinary Measures, which features Harrison Ford, Brendan Fraser and Keri Russell, tells the true story of a successful young business man whose two youngest children, Megan and Patrick, are diagnosed with a fatal disease. The movie details how Crowley, in a desperate race against time, harnessed his business skills and teamed up with an unconventional scientist to form a bio-tech company focused on developing a life-saving drug – a “special medicine” – to save his children. It brings to life a father's passionate devotion to his children and offers a window into the life-changing power of medical research and the courage of research participants.

While the movie takes liberties with some of the story – the film's Dr. Stonehill is a composite figure representing many scientists Crowley worked with during his journey to find a cure – it is true to the central tenet of the story: John Crowley would not give up.

While Hollywood knows how to make great movies, it's John's story that makes the film so compelling, particularly for anyone familiar with the clinical trial process. John's children were only infants when they were diagnosed with Pompe Disease, a rare and nearly always fatal neuromuscular disorder. As it progresses, Pompe Disease causes muscle weakness throughout the body, particularly in the heart, skeletal muscles, liver and nervous system. If untreated, patients are eventually unable to eat, breathe, speak or walk. Ultimately, many suffer from heart and respiratory failure.

Based on Geeta Anand's book *The Cure*, *Extraordi-*

nary Measures highlights the desperation patients and their loved ones feel as they search for answers. While everyone involved in clinical trials is looking for a solution – a cure, a treatment, a prevention – sick patients and their loved ones feel a unique sense of urgency. Their hope lies with researchers who must ensure the quality of their science by rigorously adhering to protocols and high scientific standards. John's story helps viewers understand what its like to listen to the clock tick. Viewers can feel his billowing frustration as he



encounters repeated delays, setbacks and roadblocks. They can empathize with his profound parental need to “do something” and appreciate the incredible strain the disease puts on his family.

In the end, John's story is also a beautiful testament to hope and the life-changing power of research. Megan and Patrick Crowley are alive today because their father believed in the power of research and invested all his energy in tapping it.