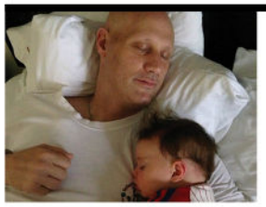
Clinical Trials

Melanoma survivor **T.J. Sharpe** talks about the power of clinical trials and how others can educate themselves on these life-saving studies.

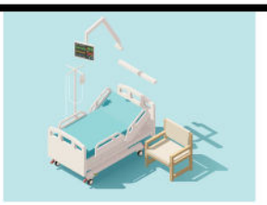
DISCOVER why clinical trial volunteers are the real heroes of medical research

LEARN how one researcher's work ended up saving her grandchildren's lives



Filling the Gaps
Discover why clinical trials often fall short of enrollment goals and how we can help raise awareness. Page 6

Getting Informed
Signing up for clinical trials can be an overwhelming process. Here's what every patient needs to know. Page 10



Tailor-Made Trials
Learn how patients have become more involved in their own care, helping doctors design clinical trials. Page 10

Why Patients in Clinical Trials Are True Medical Heroes

In medicine, we tend to think of doctors as the heroes. But they couldn't make research breakthroughs without the selfless participation of patient volunteers.

Today, nearly 4,000 experimental drugs and interventions are in active clinical trials. Powered by a productive medical innovation engine that is fueled by improvements in detecting disease and by new understanding about the root cause of acute and chronic illnesses and how to treat them, the number of promising new drugs and therapies continues to grow.

But the success of these innovations — ultimately measured by improvements in the quality of patients' lives and by the availability of new treatments and cures for unmet medical needs — is also fueled by the millions of people who participate in clinical trials each year. We call these brave individuals medical heroes and they can be found everywhere.

The brave volunteers

Medical heroes are mothers and fathers, siblings, children, friends, colleagues and ordinary people who have chosen to give the extraordinary gift of participation in clinical research. Their decision to participate is a selfless act, an altruistic gift. For while this act always carries risk it is likely that it will bring no direct personal benefit.

It is true that participation may bring hope to patients and their loved ones. But ultimately future generations are the direct recipients of the gift of participation. Medical heroes — through their participation and partnership with the clinical research enterprise — profoundly contribute to society's collective knowledge about the nature of disease, its progression and how and how not to treat it.

Ken Getz
Founder and Chairman, Center for Information & Study on Clinical Research Participation

Their decision to participate is a selfless act, an altruistic gift.

What are clinical trials?

For the vast majority of people, the idea of clinical trials is an unfamiliar concept. Most people stumble upon clinical trials when faced with the sudden and often unexpected prospect of a serious and debilitating illness for which no medication is available or adequate.

Typically patients, their families, friends and their health care providers must gather information quickly to make decisions about whether to participate. This rush to navigate the unfamiliar terrain of clinical trials invariably feels like an overwhelming and confusing undertaking.

Spreading the word

Organizations have been founded to provide outreach and education to those individuals and their support network considering participation in clinical trials. Nonprofits are focusing energy and resources on raising general awareness, on educating patients and the public and on enhancing study volunteer experiences during and after clinical trial participation.

This special supplement is part of an ongoing effort to raise public awareness about the importance of clinical research and to increase public recognition of the millions of study volunteers and clinical research professionals who, together, help advance medical knowledge.

At the very heart of a robust and exciting period of promising, life-saving and life-altering research activity are medical heroes to whom we owe our deepest appreciation for the profound gift of their participation. ■

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We Need to Talk About Clinical Trials

While clinical trials can lead to life saving medical breakthroughs, they often fall short of their enrollment goals. Here's how we can start raising awareness.

Ever discussed the possibility of a clinical trial with your physician? If your answer is "no," you're far from alone.

Yet that conversation could be crucial to raising awareness and ending the trend of under-enrolled clinical trials. About 37 percent of clinical trials sites can't meet their enrollment goals, according to the Tufts Center for the Study of Drug Development. That lack of participation stymies the development of cures and treatments that so many patients need.

What doctors can do

Physicians like me are eager to be part of the solution. But for some, fitting in a conversation about clinical trials hardly seems possible between diagnosing patients, ordering tests and interpreting results, prescribing and monitoring patients' responses to medications. And it's not just the time constraints.

Outside of academic research facilities, many physicians may not be abreast of the latest trial opportunities. And primary care physicians who deal with a range of conditions face the virtually impossible task of aligning a patient with the appropriate trial.

Yet, somehow, this conversation must happen. Maybe it means featuring clinical trials information on waiting room bulletin boards. Perhaps we use electronic patient portals to direct patients to trial finder apps. At the very least, we should convey that clinical trials exist, that participation is an option and that enrolling could be a gift to patients like themselves.

A social movement

Physicians alone cannot turn the tide. Clinical trials deserve a much larger, more coordinated and multi-prong approach, powered by federal funding. Consider the Donate Life effort of several decades ago as an apt model. A full-scale campaign that marries television, social media, celebrity endorsements, educational materials and consensus among policymakers — that could push clinical trials awareness to its tipping point.

But, for now, it might just begin with one physician and one patient, together in a clinic discussing how a clinical trial might change that patient's life — and the lives of many others. ■

By Wesley Mizutani, M.D., Rheumatologist and Member, Coalition for Clinical Trials Awareness

Creating a New Standard to Improve Competence in Clinical Research

Today there are a number of factors convening to mitigate both the impact of clinical trials and the appeal of such work to would-be research associates.

If workforce development and the standardization of competence and career paths both continue to be low on the priority list, clinical trials will remain at risk. And it will play out in continued variance in research conduct quality.

Changing the outlook

There is no standard path for becoming a clinical researcher. Most people gain knowledge on the job, and organizations utilize disparate onboarding and training programs. This creates a tremendous amount of variance in research conduct, processes and workforce competence, which culminates in a detriment to research quality.

Look no further than the persistence of common U.S. Food and Drug Administration (FDA) inspection findings for proof that the current approach to workforce development is not working. Our mission is to promote excellence in clinical research, so we are leading several initiatives to standardize workforce development and improve clinical trial quality.

Setting a standard for professionals

There is a major shortage of clinical research associates (CRAs) in the workforce. At the same time, it's clear from our own data and continued FDA inspection findings that there is an insufficient level of knowledge, skills and abilities in the CRA workforce.

The root cause of the CRA shortage is prioritization of a two-year experience requirement over validated competence. Competency based on tenure discourages a talented pool of potential CRAs who lack an industry-imposed, calendar-based requirement. The current system to train and mentor new CRAs is demonstrably inadequate to meet a growing demand for new skills and talents. ■

By Jim Kremidas, Executive Director, Association of Clinical Research Professionals (ACRP)

Why One Cancer Survivor Wants All Patients to Consider Clinical Trials

By Zoe Alexander

PHOTOS: COURTESY OF CISCRP

When melanoma unexpectedly returned after a successful surgery twelve years prior, T.J. Sharpe was both a husband and a father. Second, third and fourth opinions later, Sharpe was finally offered a life-changing option.

"I wanted a chance to see my children grow up and be the husband and father I could be," says Sharpe. With the first few doctors, Sharpe's predicted life expectancy was under two years, which he was determined to extend. "I wanted the best chance at a long-term response."

Seizing an opportunity

Sharpe was on his fourth oncologist when he was offered his first clinical trial. It was for a new treatment in the form of a pill, and Sharpe was the first patient in the trial. Because it was so new, he ran into bureaucratic barriers. "I was the first patient to try it, so there were a few stakeholder companies and pharmaceutical companies that I had to wait for over a month to get the contract approved."

After contacting the stakeholders himself to push the paperwork through, Sharpe started the trial. "When you have a family and you are facing mortality, I wasn't going to miss the chance to see these kids grow up because I was missing part of a signature."

An incredible recovery

After months on the pill treatment, Sharpe's tumors weren't responding. But with determination came plan B, and Sharpe started on his second trial. After twelve weeks, he saw a 46 percent reduction in his tumors. Four years later, Sharpe's only signs of cancer are small spots that have stabilized for over two years. Today, he remains in the trial to continue monitoring his response and overall system.

The results of the clinical trial have so far doubled his life expectancy, an accomplishment Sharpe does not take lightly. "Clinical trials should be considered as an option for care in every single case," he says. For Sharpe, the norm should be to hear your standard care options, but in conjunction with the clinical trial options.

At that point, let the patient and their doctor make the most informed decision. "When it comes down to it, we are all patients at some point, so we should know what all of our options are before making decisions." ■

When a Clinical Researcher's Work Hits Close to Home

PHOTO: SILVERSCAPE PHOTOGRAPHY, JERI BURR

Do researchers ever get to see the effects of their work? One nurse shares how her own research ended up making a critical difference in the lives of her children and grandchildren.

Ever since I was a child, I wanted to be a nurse. During the 17 years I worked at the bedside, I never imagined having a career in clinical research. However, for the past 20 years I have been able to publish about and participate in important cutting edge research for children.

A personal impact

I have four very sweet grandchildren whose lives have all been affected by what I do. My 5-year-old grandson has the rare disorder, eosinophilic esophagitis (EoE). There is no cure for this rare autoimmune disease characterized by eosinophils attacking the esophagus. As a board member on the university's IRB, I have participated in the review and approval of multiple studies that are looking for a cure or new therapies for EoE.

I am grateful I have the clinical research knowledge to participate in reviews of the latest research that could benefit my grandson in the future. My second two-year-old grandson was hospitalized at the children's hospital with RSV bronchiolitis. He was very ill, placed on high flow oxygen and treated based on the most recent RSV/bronchiolitis protocols. I felt a great deal of personal satisfaction when I observed the nurses caring for him using a bronchiolitis scoring tool I helped develop many years earlier.

Making a difference

My beautiful six-month old granddaughter is a miracle. She would not be here today if her dad, my son, had not survived a NICU admission at birth for aspiration pneumonia. Thanks to life-saving medications, approved in clinical trials, he was able to survive respiratory failure as an infant, grow up, and be a parent himself.

All of my children were required to get Tdap vaccines when expecting my grandchildren. The work on critical pertussis that I have contributed to is expected to be of benefit to U.S. children as well as the 500,000 children who still die annually of critical pertussis in the global community.

When I reflect on my work in clinical research, I am glad in some small way to have contributed to the health, welfare and treatment of my own family and future generations. ■

By Jeri Burr, RN-BC, CCRC, Executive Director, Trial Innovation Center at the University of Utah

A Clinical Trial Through a Patient's Eyes

For the unfamiliar, clinical trial research comes with many fears — but they're only about the unknown. One patient is shattering those misconceptions because knowledge is power.

By Adam Sass

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ACCELERATING THE DEVELOPMENT OF NEW MEDICINES

When Meisha Brown began her first clinical trial, it was her only option left. It was also her summer vacation after second grade. "I had lymphoma, my prognosis was grim and it had spread across my body," says Meisha. She acquired a septic infection and was put on life support in the ICU. They squeezed her hand, but she couldn't squeeze back.

"I felt helpless," Meisha says. "I could see and hear my parents, but I couldn't talk to them. I couldn't even blink."

Meisha recalls lying in her hospital bed and thinking that if she could survive this moment, she would commit to learning as much as she could about what was happening to her. When she made it through, Meisha and her family decided to turn to a clinical trial.

Knowing your adversary

While Meisha's initial care was geared toward her parents' understanding, her trial was tailored so she could understand what was happening to her and how they were trying to fight it.

"My oncologist explained [my cancer treatment] using teddy bear demonstrations and drawing characters on a white board," Meisha remembers. "They taught me that my particular lymphoma was affecting my B-cells. At the time, I connected to that on the alphabet level: 'Oh, well, A is better than B, so what do I need to do to get this to an A?'"

Meisha adds that being engaged with on her cancer in this way was empowering. "The more I understood, the more it helped me hold out hope," she says. "I understood my cancer better than my parents did."

Facing uncertainty

Even though her trial had a positive outcome, Meisha has had several chronic complications from her cancer — ones she's been happy to seek treatment for in other trials over the years.

A major reason for her coming back to trials is that abundance of firsthand communication between patient and physician. It can feel more like collaboration, and that goes a long way toward fighting fear.

Paulo Moreira, vice president of Global Clinical Operations-External Innovations at EMD Serono, a TransCelerate member, has experience with the fearful misconceptions people have participating in their first trial. "I hear that fear in the beginning," he says. "I'm always surprised to hear people use the term 'guinea pig.' Clinical research is a highly-regulated activity performed by passionate, trained individuals."

"Before patients begin their trial, a review board of scientists, non-scientists and regulatory agencies have given it its blessing," says Dr. Dalvir Gill, CEO of TransCelerate, a nonprofit organization that brings together pharmaceutical companies to collaborate with a goal of enhancing clinical research. A priority for TransCelerate is improving the patient experience by decreasing patient burden and improving clinical research awareness and engagement.

Recognizing patient involvement

Craig Lipset, head of innovation at Pfizer, a TransCelerate member, notes that helping patients become aware of clinical research as an option is a universal challenge. "We can't do this in isolation, we need neutral stakeholders like CISCRP (the Center for Information and Study on Clinical Research Participation) and TransCelerate who can bring the research community together with patients to ensure patients are well-informed, recognized as partners and treated with respect and gratitude."

"We have information that if a patient's experience is positive, they are likely to be open to clinical research in the future," says Dalvir, who also adds that the increased engagement during a trial helps the physician as well as the patient. "We can better pinpoint potential risks and burdens for future trials."

Meisha's experience attests to that: Now 27 and in her second year of a Ph.D., she's kept the commitment she made as a little girl to learn as much as she could. She credits everything she's learned over the years as a clinical trial patient to becoming a better researcher and health practitioner — as well as giving to a cause greater than herself. ■

A Clinical Trials Guide for Patients

Considering clinical trials as part of your treatment plan? The decision can be overwhelming. Here are the basic facts every patient needs.

The decision to participate in a clinical trial is very important — one that must be taken very seriously and that requires you to do your homework, and gather as much information as you can. A good motto is "education before participation."

A variety of outcomes

A good place to start the learning process is to address the common misconception that receiving treatment in clinical trials is the same as receiving medical treatment as a patient. When you're a study volunteer, you typically feel like a patient in a doctor's office. You're examined by a doctor, undergo lab tests and other common procedures, and receive a medication.

The difference is that a doctor's primary goal is to help you feel better, whereas in a clinical trial, the principal investigator's primary goal is to see how you will react to a new drug, and to determine whether that drug will be safe and medically useful.

This truth doesn't change the fact that many people feel better while on an investigational drug. Some investigational products prove to be far superior to the older drugs they will one day replace. Some drugs administered during clinical trials have not only improved, but also have saved, thousands of lives. But the opposite is also true: some drugs administered during clinical trials have worsened people's conditions and have caused death.

Each volunteer's outcome in a clinical trial depends not only on the specific study in which they participate, but also into which part of the study they are randomized. Subjects who get randomized into the control group will, at best, get a standard treatment that is already available at pharmacies and drugstores.

The common benefits

People who participate in clinical trials often learn a great deal more about their illness and about other conditions (including underlying heart disease and diabetes) they may not have known about. Study-related x-rays, lab tests and physical exams have picked up unsuspected cases of many types of cancers early enough to be successfully treated by specialists. For some participants, the best end result of a clinical trial is that they start taking better care of their own health.

An important benefit for all clinical volunteers is the opportunity to meet research professionals who can help introduce them to other patients suffering from similar illnesses. Volunteers also may meet scientists and professionals who can help them better understand their illness and can tell them about new treatment options under development. The many people whom you meet in a clinical trial can greatly enrich your knowledge.

For individuals diagnosed with a severe and possibly life-threatening illness, the greatest benefit of clinical trials is that they offer hope. At its best, a clinical trial is an enlargement of — not a substitution for — a patient's regular medical-care team and support circle. ■

By Ken Getz, Founder and Chairman, CISCRP

How Are Patients Helping Drive Drug Development?

More than ever before, patients are getting more involved in their health care. So, what does this industry-wide shift mean for how drugs are developed?

By Adam Sass

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Patients are engaging as active, vocal research partners with a newly critical role to play in the development of pharmaceuticals. This sea change of involvement has catalyzed companies to break from the traditional mode of doing things "for" patients to doing things "with" patients — at every stage of the development process.

The patient perspective is being incorporated into everything from strategy, research and development, and the highly important clinical trial experience.

Knowledge is power

"It's not so much that the role [of the patient] is changing, it's that our perspective is changing on why patient input should be prioritized," said Margaret Anderson of FasterCures, a center of the Milken Institute. "There's been a rapid transformation of the environment in which we all live in as patients. Patients have easier access to medical records now that they're electronic — technology has been very democratizing."

Margaret stresses that as patients become more educated and empowered, they're helping create what she calls "the science of patient input." "We're [encouraging patient input] not only out of some gesture of goodwill," she says. "It's actually going to provide better research."

Tailor-made trials

"When insights are gathered from patients, we can more effectively shape clinical trials," says Katie Mazuk of Janssen Pharmaceuticals. "Early and direct feedback — not from proxies — allow us to understand the potential burdens a trial could have on patients or their caregivers."

Based on this feedback, Katie says they could better modify elements of the patient experience, such as length of study visits, transportation needs and even special support needed during study visits.

"Patients have information that no one else does," says Katherine Capperella of Janssen. "It's important to speak directly to the patient. By using their input to make clinical trials better meet their needs, we've reduced dropouts. Through this, we may reduce the time it takes to make medicines available to people who need them." ■

Improving Clinical Trials Through Industry Collaboration

While clinical trials can lead to many medical breakthroughs, they're often weighed down by inefficiency and red tape. To fix the system, we need to reach across fields.

Many have called for major improvements in clinical trials that can accelerate the delivery of life-changing treatments to patients, yet randomized clinical trials continue to increase in cost and complexity, and questions of quality remain. Why is it so difficult to right the ship? Improving any single process among the hundreds involved in designing and conducting a clinical trial is unlikely to transform the overall system, nor can changes by just one stakeholder move the needle.

A systematic, evidence-based approach to addressing issues of efficiency and quality in clinical trials — one that includes participants from across the clinical trials enterprise as equal partners — is the method needed to achieve widespread, measurable improvements in clinical trials.

A collaborative approach

An approach that engages everyone with a stake in the success of clinical research is critical to any attempt to transform the system as a whole. This is because the lens through which a pharmaceutical company employee sees a problem is, not surprisingly, different from that of a patient representative, a physician or a government agency representative.

> This collaborative dialogue is essential to the foundation of real and lasting change.

Yet all these points of view matter and are equally needed to create solutions that work for everyone. When we bring together diverse perspectives to tackle a challenging issue, stakeholders can understand the issue with new context, and eureka moments abound. Breaking down silos allows us to find solutions that empower all stakeholders to make a positive difference. This collaborative dialogue is essential to the foundation of real and lasting change.

Data-powered change

Additionally, to transform clinical trials, we need workable solutions based on evidence, not on anecdote. We must employ rigorous methods to identify areas of greatest need, gather evidence to better understand the issues and use data as the foundation for recommendations and tools that help to improve clinical trial quality and efficiency.

The effort of those who use our findings and recommendations are improving today's clinical trials. The impact of this work can be seen in policy decisions and practice changes, and we will continue to work toward a better clinical trials system — one that speeds new therapies to the patients and families who need them most. ■

By Annemarie Forrest, Associate Director of Projects, Clinical Trials Transformation Initiative

Here's to all those who make medicine better.

We'd like to recognize all the men and women who take part in clinical research.
You not only advance knowledge about disease, you also improve medical treatments for generations to come.
For more information about clinical research, please visit CISCRP.org.

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