

Clinical Trials

Rachel Petties
continues to fight and
raise awareness for
her young daughter's
rare genetic disorder

DISCOVER

why study volunteers for clinical trials
are essential for the advancement of
life-saving treatments

LEARN

more about how clinical trials are
perceived by participants and the
general public



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IN THIS ISSUE



Discover why the future of cancer research depends on federal funding for clinical trials.
Page 3



Learn what study volunteers need to ask in order to receive the results of their clinical trials.
Page 7



Browse more stories online, including why clinical trials are falling short of their enrollment goals.
Online

Recognizing the Medical Heroes Who Participate in Clinical Trials



Ken Getz
Founder and Chairman,
CISCRP

Behind every medicine and available treatment are the people who gave the extraordinary gift of participation in clinical trials.

Around the world, people are living longer, healthier and happier lives because of the millions of volunteers who participate in clinical trials each year. We call these brave individuals “medical heroes.” Medical heroes are parents, siblings, children, friends and community members who have chosen to participate in clinical trials.

Hope for future generations

The decision to participate is a courageous and selfless act because it always carries risk and is unlikely to bring any direct personal benefit. Participation in a clinical trial often gives hope to patients and their loved ones. However, the direct recipients of the gift of participation are ultimately those who will be managing their disease in the years to come.

Future generations benefit from the new knowledge about the nature of their disease, its progression and how to treat it.

For the vast majority of people, the idea of participating in a clinical trial is new. Most 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 and their families, friends and health-care providers must gather information quickly to make decisions about whether they should participate in a trial. This rush to navigate the unfamiliar terrain of clinical trials invariably feels overwhelming and confusing.

Providing education

In 2004, the Center for Information and Study on Clinical

Research Participation was founded to provide outreach and education to those individuals considering participation in clinical trials. Based in the Boston area but with a global reach, this nonprofit organization focuses its energy and resources on raising general awareness, on educating patients and the public and on enhancing volunteer experiences during and after clinical trial participation.

Today nearly 4,000 experimental drugs and therapies are in active clinical trials. That number continues to grow as improvements are made in detecting disease, in discovering new medical innovations and in understanding and addressing the root cause of acute and chronic illnesses.

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

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PLEASE RECYCLE AFTER READING

The Future of Cancer Research Depends on Federal Funding

For many cancer patients, clinical trials offer new hope. Therefore, government funding is a necessity in bettering the chance to cure cancer once and for all.



When I began my oncology career nearly 30 years ago, the possibility of conquering cancer would have seemed an implausible pipe dream. Now, with the tremendous progress being made in understanding and treating cancer, I believe that dream is firmly within our grasp.

Nearly every American is affected in some way by cancer, and many of us know firsthand how a cancer diagnosis can disrupt the lives of patients and their families. As a surgical oncologist, cancer researcher and the President of the American Society of Clinical

Oncology (ASCO), I'm honored to have a front row seat in today's cancer revolution and to directly contribute — in a role that is amplified by the strength of ASCO's oncologists and the patients that we serve — to advances in cancer prevention, diagnosis and treatment.

Providing hope

During my career, I have seen how cancer research can help patients live longer with better quality of life and how many patients can be cured. The National Cancer Institute (NCI) has an essential role in this progress, with federal research unlocking answers

to questions about preventing cancer or identifying treatments that cause the fewest side effects. Research supported by the NCI has spurred innovation. Most recently this includes the first immunotherapy treatments for cancer. This new treatment approach has produced dramatic improvement in outcomes for some patients with previously difficult-to-treat cancers. We are driven by this success to further unlock the secrets that will make this result achievable for all patients with cancer.

The need for funding

With all this good news, it's hard

to see 88 percent of qualified research go unfunded. I'm thankful that Congress has marshalled bipartisan support for the NCI, providing funding increases that aim to reverse nearly a decade of flat funding. However, even with these recent investments, the decline in purchasing power means that the NCI can fund only 12 percent of qualified research applications. That is less than half of the 27 percent of research proposals funded in 2003. And support for our publicly funded National Clinical Trial Network continues to decline in real dollars.

For some of my patients, a clinical trial is the best option. We need to make it easier for patients to participate in important research that not only affords them state-of-the-art cancer treatment, but also helps others who face a cancer diagnosis. I believe in the promise of tomorrow and the discoveries that will expand our understanding of how best to prevent, treat and conquer cancer. That is why I know we need a renewed national commitment to federally funded cancer research. ■

Monica Bertagnolli, M.D., Fasco,
President, The American Society
of Clinical Oncology

Oklahoma Mom on a Mission Reflects on Daughter's Rare Diagnosis

Rachel Petties, a mother of five, refuses to let her daughter's rare genetic disorder define the lives of their family.



PHOTO: ANNA MARIE PHOTOGRAPHY

As the ultimate recipients of new therapies, and the primary advocates in clinical trial consideration and search, patients' preferences regarding clinical trial information online should be of prime importance.

The Clinical Trial Registry of the Future (ROTF) concept could help bring about change to existing, government-owned registries across the globe.

ROTF 

We need your help to turn this concept into a reality

PATIENTS WANT TO KNOW

Potential Risks and Benefits of the study*

 **81%**

Contact Information of the medical center*

 **76%**

* Responses are from patients and care givers to a survey conducted by Center for Information and Study on Clinical Research Participation (CISCRP) in collaboration with TransCelerate BioPharma Inc., Clariness and CenterWatch in the Fall of 2016.

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Visit www.TransCelerateBioPharmainc.com/registryofthefuture to review our proposal and wireframe concept and **GIVE US YOUR FEEDBACK**

From the moment she first laid eyes on her daughter, Rachel Petties knew something was wrong.

"Alani was all muscle," she recalls. "She had no body fat whatsoever. Her head size seemed abnormal, and her belly was very large. She was stiff and cried all the time."

It was the beginning of a difficult journey.

"I ended up dragging my infant baby girl in and out of hospitals, doctor to doctor, specialist to specialist," Petties says. "You have no idea what loneliness is until you have to pack your child up, hop on a plane and trust someone to help you when you know no one else believes you."

Receiving the diagnosis

In 2015, Alani was clinically diagnosed with congenital generalized lipodystrophy.

"My world crashed, and I'm still recovering from the loss," recalls Petties, a single mother of five. "Receiving her diagnosis was a bittersweet moment — my suspicions were confirmed, but I was very sad because what was wrong with her was worse than I expected."

Alani, now 4 years old, was enrolled in a long-term clinical trial program at the National Institutes of Health to evaluate her treatment and find improved ways of treating the disease through pre-screening and other medical advances. The hope is to prevent future complications by identifying and addressing problems early on.

"She's a patient for life," says Petties. "I'm extremely encouraged. Alani has elite specialists studying her case. She's monitored

closely and watched by top professionals in the field.

"We have to travel once a year to Bethesda, Maryland for a host of tests that usually last 2-3 days. It's a very stressful time for us, but we know it's necessary."

“

I am rare... I am a mother of a child who holds a title of 1 in 10 million...

Finding the strength

Petties' days are long and exhausting. She must prepare special foods for Alani and is the only family member trained to administer crucial injections.

"It's very isolating, and I'm afraid of what will happen if I'm ever physically unable to give her this medicine," she says.

Restricted mostly to the indoors, Alani can be awake for 24 hours and sleep for 15 hours straight. Her home must be bleached daily to fight germs, and she must be monitored for excessive eating.

"Before gaining some control over this disorder through the clinical trial she's currently on, Alani would eat the paint off the walls, erasers, socks and soap," Petties says

Although she's cut back dramatically to focus on her kids, Petties is committed to getting the word out about lipodystrophy.

"I woke up every morning with the mentality to outrun this dis-

ease," she says. "I ran state to state, city to city, advocating with every ounce of life in me. We marched in Washington and visited senators' offices, petitioning for their help."

"Sometimes you'll want to fight, other times you won't care one bit. I had to teach myself that it's important to be okay with all those emotions and just get through the day, because every day Alani wakes up is another day this disease didn't win."

Alani, who enjoys dancing and performing karaoke, knows she's different and doesn't care, according to her devoted mom. And although weary, Petties herself finds beauty in the darkness.

"I am rare," she says. "I am a mother of a child who holds a title of 1 in 10 million. That by far is nothing to be sad about. She is incredible and unique, and she's all mine." ■

Cindy Riley

Why We Invent

AT MERCK, WE ARE INVENTING FOR LIFE.

We are taking on many of the world's most challenging diseases because the world still needs cures for cancer, Alzheimer's disease, HIV, and so many other causes of widespread suffering in people and animals.

We invent to help people go on, unburdened, to experience, create and live their best lives.

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DATA

CISCRP Perceptions & Insights Study 2017

Sample size: 10,843

Those reporting they had heard of the term “clinical trial” or “clinical research”

Sample size: 8,920

Those reporting they understood the term “clinical trial” or “clinical research” well

Sample size: 2,194

Former study participants

DID YOU KNOW?

- Most people have heard the term “clinical research study” or “clinical trial” and feel that they understand the term well (83%). **However, people only have a basic understanding...**
 - Six out of ten people (62%) cannot name an agency that oversees clinical research safety, and half (51%) do not know where clinical research is conducted.
- Half (52%) of people who have participated in a clinical trial considered the medical care they received during the trial to be better than the routine care they received from their regular doctor.
- The top two things people liked most about their clinical trial participation was helping to advance science and medical treatments and helping others who have the same disease or condition as themselves.
- Almost all people (94%) who have participated in a clinical trial report that they would be willing to participate again, and a similar percentage would recommend participation to others.

Jasmine Bengier, CISCRP Research Services Project Manager

Since 2013, CISCRP has conducted the Perceptions and Insights Study every two years among the public and patient communities. Trends and insights from this large global study help identify better ways for the public, patients and clinical research professionals to work together to develop and investigate new and much-needed medical treatments.



This is where possibility may become reality.
 This is where discovery meets humanity.
 This is where new treatments can be tested faster.
This is where you come in.



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 at LillyTrialGuide.com



What to Expect When Receiving Your Trial Results

For the majority of clinical trials conducted each year, study volunteers don't receive their results or don't know how to access them.

Nearly all study volunteers want to receive the results of their clinical trials. Regardless of the outcome, participants want any information available that could help them make critical health decisions, and they want to know that their involvement helped others living with disease and illness. In fact, receiving study results is one of the top reasons patients choose to participate in a clinical trial, ranked even above the ability to access free medical care.

Pharmaceutical companies and government agencies sponsoring clinical trials are obligated to share these results. However, for the majority of clinical trials conducted each year, study volunteers don't receive their results. Most patients first learn about the outcome of their participation only after a general announcement has been published in a newspaper or broadcasted in the news. For many study volunteers, the end-of-study experience often leaves them feeling dissatisfied and unappreciated.

Ways to access results

Some clinical trial results are posted on the websites of phar-

maceutical companies. Also, federal law requires that clinical trial results are routinely posted on a government website within a year of study completion even if the drug or medical device being tested hasn't been approved. These results, however, are not usually shared with study participants.

There is good news, however. A growing number of pharmaceutical and biotechnology companies sponsoring clinical trials want to deliver results in non-technical, plain-language summaries to their study volunteers. At the moment, research sponsors are mainly focused on providing general clinical trial results, but some companies are piloting initiatives to share more detailed,

personalized findings with their study volunteers. In the future, study volunteers could potentially attach their own data from clinical trials to their electronic health and medical records, allowing them to share the results with whomever is providing treatment.

What to ask your doctor

If you, a family member or friend is considering participating in a clinical trial, be sure to ask if the clinical trial results will be shared. Carefully think about whether you want to participate in a clinical trial if there is no guarantee that the results will be provided to you. If a plain-language summary of the results will be provided, you

should ask what options you have for receiving that information.

If you have recently participated in a clinical trial, ask your doctor or nurse — or the study staff — to help navigate the clinical trials website or the pharmaceutical company's website to locate information about your clinical trial. Your nurse and doctor also can be helpful in translating medical jargon into easy-to-understand language.

Study volunteers want to know the outcome of their participation in clinical trials and that their participation mattered. As partners in the clinical trials process, study participants should expect to receive — and those sponsoring or conducting the research are obligated to provide — plain-language result summaries. ■

Ken Getz, Founder and Chairman, CISC RP
He is the author of the book, *The Gift of Participation*, now in its 2nd edition (available at www.ciscrp.org).

How Clinical Trials Are Focusing More on the Needs of Patients

Patients have always played an important role as participants in clinical trials, helping advance disease research and treatments. Increasingly, clinical trial sponsors are focusing on improving participants' clinical research experiences.

clinical research education and information.

At CISC RP, which at times has received funding from Pfizer, Getz has a big-picture view of what's happening in the industry. He credits the company with leading the way in patient-centric approaches.

"They've been very open-minded about a wide variety of educational and clinical trial convenience enhancing initiatives," says Getz, who calls Pfizer "pioneers," explaining they're among the first clinical trial sponsors to think about how to give patients personal results from clinical trial participation, as well as looking into integrating clinical trial results into a patient's electronic medical record. As a result, he says participants feel more respected and more engaged in the process.

"Patients are not only the recipients, but they're also members of the team in health care and research," says Dr. Schneider. ■

Kristen Castillo

SPONSORED



and outcomes," says Roslyn F. Schneider, M.D., Pfizer's global patient affairs lead.

"We're evolving for it to become a better, more personalized experience," she says, with examples that patient insights can inform the clinical trial protocol to include what's important to the patient community.

Some considerations are practical, such as convenience of appointments. Others are more involved, such as how long patients living with a particular illness might be willing to

stay off their usual medications if required for participation in a clinical trial.

Pfizer is making changes to meet the needs of participants. They created Pfizerlink.com, an alumni community for people who have participated in Pfizer clinical trials, as a platform to return research results and share other information of interest to patients. They also revised their informed consent documents based on patient feedback to include more graphics and better flow.

Engagement

Nationally, nearly 30,000 people participated in clinical trials in 2015. While participation numbers vary each year, it's important to keep patients interested in clinical research.

"It's about great science, but at the same time, how do we make sure we've heard the patient's voice in the design and development of the new therapy?" says Ken Getz, founder of The Center for Information and Study on Clinical Research Participation (CISC RP), a nonprofit providing

Pfizer is prioritizing patient involvement in the design, planning and execution of clinical trials.

"There's more of a push across health care to have shared decision-making to improve quality



Hero to her today.

Medical hero to millions tomorrow.

To all clinical research volunteers, thank you.

A sincere thank you to all of the men and women who take part in clinical research studies each year. By volunteering today, you become a medical hero forever. For more information about clinical research, please visit CISCRP.org.

