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CLINICAL TRIALS

MEDIA PLANET

Jillian McNulty

Since her cystic fibrosis diagnosis, McNulty has become an exemplary clinical trials volunteer and advocate

Clinical trials depend on the participation of a large pool of volunteers

A diverse clinical trial population is necessary to understand how all people respond to new treatments

READ ON

Clinical trial patients hold the keys to unlocking some innovative new treatments. **Page 5**

Effective communication with patients in trials leads to better, more insightful findings. **Page 7**

Before participating in your first clinical trial, make sure you consult this handy, helpful infographic. **Page 8**

The Real Reason Clinical Trial Participants Are Called Medical Heroes

Progress in medical science would be delayed were it not for the people volunteering their time and energy to be part of clinical trials.

This supplement is part of an ongoing nationwide effort to both raise awareness of the importance of clinical research, and to increase public appreciation for the millions of people who participate in clinical trials every year in the service of advancing medical knowledge.

Today, there are more than 4,000 experimental treatments being used in active clinical trials. Every year the number of promising new medical therapies grows, as more is learned about detecting, diagnosing, and treating disease. Most importantly, the success of all 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 — would not be possible without clinical trial volunteers.

Regular people, medical heroes

We call these brave volunteers "medical heroes," and they can be found everywhere. We are all indebted to them. Medical heroes are your parents, family members, friends, colleagues, and people you've never met who have chosen to give the extraordinary gift of participating in clinical research. Their decision to volunteer is a selfless act for two reasons: One, because participation in a clinical trial always carries risk; and two, because it is likely that for many study volunteers it will bring no direct personal benefit.

Ken Getz
Founder and Chairman, Center for Information and Study on Clinical Research Participation; Associate Professor, Tufts University School of Medicine

Ultimately, future generations are the ones who benefit most. Through the process of participating in the trials, medical heroes profoundly contribute to society's collective knowledge about the nature and progression of diseases, and how to treat them.

An unexpected opportunity

For the vast majority of people, the idea of clinical trials is a new and unfamiliar one. Most people first learn of them at the same time they are diagnosed with a serious illness for which no medication is available or adequate. Typically, patients, their families, friends, and healthcare providers must gather information quickly to make decisions about whether to participate. This rush to navigate the unfamiliar terrain of clinical trials is often overwhelming and confusing.

Seventeen years ago, the Center for Information and Study on Clinical Research Participation was founded to provide outreach and education to these individuals and their support network considering participating 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, educating patients and the public, and enhancing study volunteer experiences before, during, and after clinical trial participation.

We hope that this educational supplement will be a valuable reference resource offering an introduction and insight into the clinical research process and the decision to participate. We hope that you find it to be both informative and inspiring. ■

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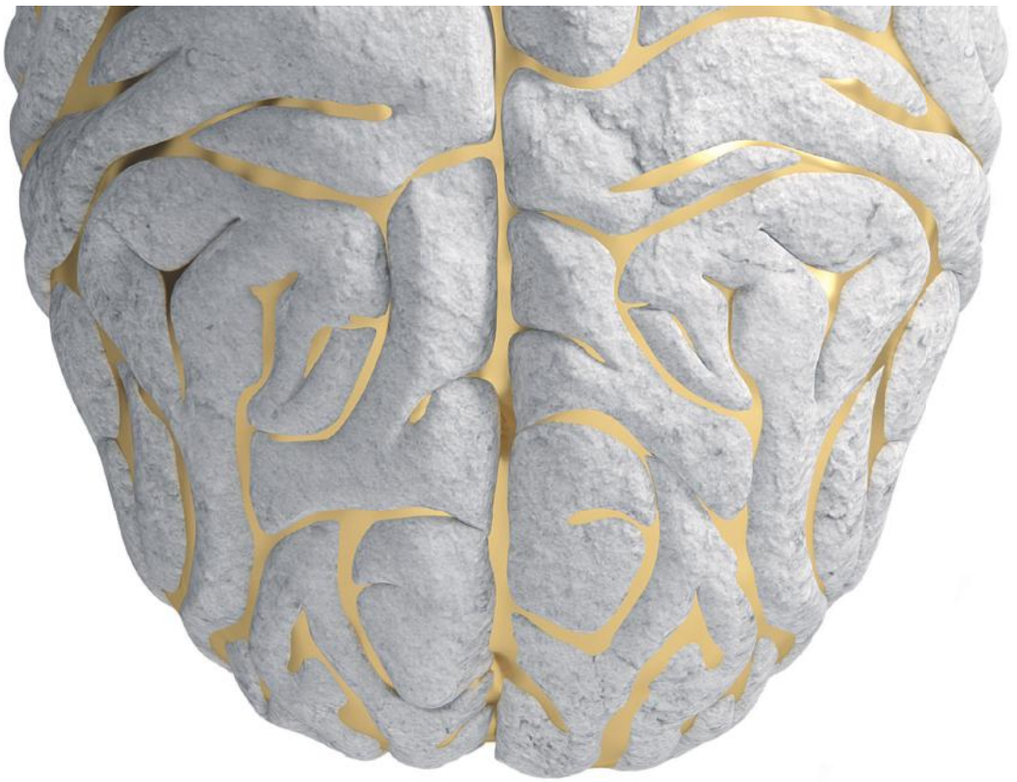

ALZHEIMER'S IS A TOUGH OPPONENT. BUT SO ARE WE.

Developing new medications is hard. In fact, only around 14% of drugs that enter clinical research ever reach patients. Still, that doesn't make the decision to halt a clinical trial any easier.

Recently, we announced the early termination of trials investigating aducanumab for Alzheimer's disease – a difficult decision made after learning that the drug was not slowing the disease's progression.

We understand and share the sadness and disappointment of the Alzheimer's community. However, every clinical trial, including those that don't achieve the outcome we hope for, expands our knowledge and moves us closer toward the goal of finding future treatments for those in need.

So we'd like to take this opportunity to express our deepest gratitude to all involved in the aducanumab clinical trials: participants, caregivers, family and medical professionals alike.

Thank you for the time you devoted to these trials. Thank you for helping us understand more about Alzheimer's and how the disease responds to different clinical approaches. And above all, thank you for your courage, endurance and commitment in the face of this disease.

Medical breakthroughs are not easily won. But despite the frustration and heartache you may be experiencing today, please know that you have contributed toward a more hopeful future for people with Alzheimer's.

We won't give up.

Biogen

Debunking the Myths Surrounding Cancer Trials and Research

Getting a cancer diagnosis can be frightening, and picking a course of treatment may feel overwhelming. But new innovations in research are changing the way cancer is being treated, and clinical trials are providing cutting-edge new treatments that may improve future diagnoses. The goal of clinical trials is to find treatments that are better than what's currently available. Virtually every therapy approved to treat cancer has been made possible by those who agree to participate in clinical trials.

There are currently over 22,000 active cancer clinical trials in the United States that are enrolling patients or preparing to recruit participants. However, very few adult cancer patients join a trial, and 1 in 5 cancer clinical trials aren't completed due to a lack of participants. Without enough patient data, a promising treatment might never get approved.

Debunking myths

In addition to the lack of awareness of the necessity of clinical trials, there are many misunderstandings and myths that need to be addressed. For example, clinical trials are not just a last hope for patients for whom the traditional standard of care has failed; many cancer trials enroll patients who have not received prior treatment. There is also a misunderstanding concerning the use of placebos, which are rarely used. Most clinical trials provide either the experimental treatment or the treatment that is the most current "standard of care."

Another issue to consider is that members of minority communities are too often underrepresented in clinical trials. According to the FDA's Drug Trials Snapshots, in 2017, nearly 75 percent of the participants in cancer clinical trials leading to drug approval were Caucasian, whereas only 4 percent were African American, 4 percent were Hispanic, and 12 percent were Asian.

Public awareness

Seeking to increase the public's awareness of clinical trials, Stand Up To Cancer has launched an extensive Public Service Awareness campaign encouraging patients to ask their doctors which clinical trial may be right for them. The campaign features Stand Up To Cancer ambassador Sonequa Martin-Green, as well as diverse cancer patients, and the online resources available in both English and Spanish aim to include people from all ethnicities and backgrounds.

Visitors to StandUpToCancer.org/ClinicalTrials (and StandUpToCancer.org/es/ensayosclinicos) will find content that is easy to understand, including short videos explaining clinical trials and different types of treatment, the terms one may hear when discussing trials, the benefits of participating, and what to expect if you or a loved one participates in a clinical trial. The website also provides a list of questions that can be printed for reference during discussions with your healthcare provider. Additionally, Stand Up To Cancer provides a free and confidential Clinical Trial Finder service through EmergingMed that allows patients or caregivers interested in finding an appropriate clinical trial to submit an online form or call a toll-free number to begin the process.

Since launching this campaign in February, Stand Up To Cancer has engaged dozens of advocacy organizations to increase the reach of these assets. As we collectively increase awareness, and more diverse cancer patients participate in clinical trials, researchers will learn about new treatments that may be more effective in fighting cancer. ■

Mediaplanet Staff

Designing Clinical Trials: Listening to, and Learning From, Patients

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Medical breakthroughs wouldn't be possible without the people who participate in clinical trial research every year. Traditionally, scientists and researchers design the studies to test investigational medicines and therapies for people who are living with a disease. But now, patients have a bigger role in the process: helping guide the design.

"Some ideas can only be uncovered by learning from, and listening to, people who live with their conditions every day," says Ricardo Rojo, global clinical lead at Pfizer, a biopharmaceutical company that is one of the world's largest sponsors of clinical trials.

Pfizer works closely with patients and patient advocacy groups so that they are a part of drafting clinical trial designs. Researchers know that it's important to try to make the process as comfortable and convenient as possible for the study participants.

"We try to think from the vantage point of our participants," says Judy Sewards, vice president, head of clinical trial experience for Pfizer. "We're grateful to them for participating. They make significant contributions, and their voice in clinical trials is central and influential for us as we develop new medicines."

Patient-focused

Lisa Butler, vice president of strategic partnerships at the National Eczema Association (NEA), says this engaged approach to research is inspiring hope within the eczema community, which has an urgent need for more effective treatments. Through the NEA, people living with eczema have done small focus groups with Pfizer, and the feedback has been positive.

"Patients were able to be involved, share their story, and see that they actually made a difference, not just for themselves, but for potential patients in the future," says Butler.

For example, for one study, the initial protocol design required that participants not treat their eczema for seven days, a standard "washout period" before introducing an investigational therapy. However, patients said this was too long a time for them to be off their medicine because the symptoms of their skin disease could worsen. As a result of this feedback, the team shortened the "washout period." They also addressed other needs raised, such as adding more information about the study and streamlining patient paperwork.

"Hearing firsthand from patients not only motivates us by reminding us why our work is so important, it also helps us study many of the outcomes that matter most to them," says Rojo. ■

Kristen Castillo

TO LEARN MORE ABOUT CLINICAL RESEARCH AT PFIZER OR FIND A CLINICAL TRIAL THAT MAY BE RIGHT FOR YOU, VISIT HTTPS://WWW.PFIZER.COM/SCIENCE/CLINICAL-TRIALS.

Meet Jillian McNulty: Cystic Fibrosis Patient and Clinical Trials Advocate

Born with cystic fibrosis, Jillian McNulty has spent her entire life fighting to stay healthy.

It was only during a clinical trial, when she tried a new medicine called Orkambi, that she met with success. Since then, she's been fighting for other cystic fibrosis (CF) patients to have access to the drug too.

One of four children, McNulty's oldest brother had cystic fibrosis and died when he was five-and-a-half. Her prognosis wasn't good either. When she was born, doctors told her parents she wouldn't live past her fifth birthday. She's now 43 and jokes that she has geriatric CF, and is grateful to be alive.

Last hope

Over the years, McNulty has suffered from recurring pneumonia, and sometimes spent 8-9 months out of the year in the hospital.

"I didn't have a great quality of life," she recalls, noting her condition was at its worst in 2012 after her other brother, who had special needs, died. She was able to run a marathon before her lung function dropped from the high 50s (percent) to the 30s. She became dependent on IVs and antibiotics.

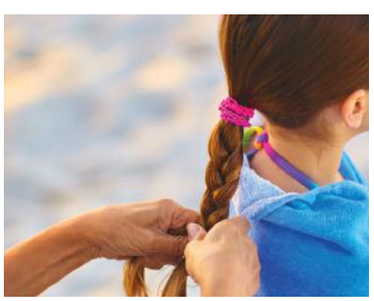
Fortunately, McNulty qualified for the Orkambi clinical trial, which required her to have lung functioning in the 40s and to not be hospitalized for four or more weeks.

She got emotional when she got the news that she qualified. "I can remember I cried my eyes out because to me this was my last chance, my last hope. Things started to turn after that."

Though she struggled for the first four months of the trial, at six months her hospitalizations started to decrease. But things took a turn in 2016, when she contracted swine flu and influenza A at the same time. Her lung function was down to 11 percent. Things were grim, but McNulty pushed on.

Changing lives

"Orkambi brought me back from the brink," McNulty says, explaining it took three months to recover.



She says the drug, which is manufactured by Vertex, works on the underlying cause of CF, a life-threatening disease that affects approximately 75,000 people in North America, Europe, and Australia.

"It's not a cure but it tweaks the channels so our bodies respond better," says McNulty, whose lung function is now at 42 percent.

These days, she spends only six weeks a year hospitalized. But now she's struggling with end-stage kidney disease, another chronic condition she's had for years, and needs a kidney transplant. But before that, she needs a lung transplant.

Still she's optimistic about her future and the future of other CF patients.

"It's all changing. People with CF are going to live so much longer," says McNulty. "That's incredible."

The CF advocate

For the past 11 years, McNulty has campaigned for CF patients in her native Ireland, including lobbying the Irish government to make sure Orkambi and other medications were made available for CF patients. Her campaign was successful and she obtained "pipeline" approval for 10 years of promised accessibility to the drug.

PHOTO: SHELLEY CORCORAN PHOTOGRAPHY

"My friends were dying and I couldn't stand by and say nothing," says McNulty, who's won three awards for her advocacy. "I needed people to see what Orkambi had done for me."

She's also been instrumental in campaigns related to the hospitalization of CF patients, helping ensure they get single-unit hospital rooms to prevent them from being exposed to other sick patients while their immune systems were so compromised.

Her advocacy continues on, and she urges patients with CF and other conditions to participate in clinical trials: "It's worth the chance, it's worth the risk. It has the potential to transform your life in ways you can't imagine." ■

Kristen Castillo

Demystifying the Clinical Trial — It All Starts With a Conversation

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Clinical trials depend on their most vital resource: the patients. A new program is working to improve their experience.

Clinical trials benefit humanity as a whole and may have a positive impact on the the lives of the people who participate in them. This is especially true when they can establish strong relationships with the investigators and staff — studies demonstrate that better communication between patients and medical professionals results in better care and less stress for both.

A lack of effective communication remains a challenge, however. "I think clinical trials are a mystery to many people," says Suzann Johnson, associate director of patient experience at Janssen Pharmaceuticals. "We had conversations with our investigators, and I think the investigators are recognizing there may be a need to improve communication with patients."

Improving communication

Susan Kesler, a nurse with 35 years of experience working as a site coordinator with Dayton Gastroenterology, Inc., agrees, "It's really all about the patient," she says. "We want to help you with your conditions. I think more open communication with the patient from the physician is very important."

The key, Kesler says, is treating each patient as unique. "It's all about, how can we help you?" she says. "The patient needs to feel safe and secure. We always give them all the information and encourage them to talk to their family. I like to tell my patients, you have to be comfortable with what you're doing."

Janssen sees a future where every clinical trial is patient-focused. "Communication is difficult as it is," says Johnson. "When you add in the variable of someone who's dealing with a disease, they don't know what to expect in clinical research. That was the impetus behind developing the HealthCaring Conversations program."

A patient-focused roadmap

HealthCaring Conversations is focused on ensuring participants in clinical trials feel heard and empowered. "HealthCaring Conversations provides a blueprint to a conversation that really keeps the patient at the center," says Johnson, "while also acting as a roadmap that speaks to what the patient is interested in. It really personalizes the conversation."

The program is a 20-minute interactive module clinicians take in their own time, modeling conversations and allowing the clinician to choose responses. "Within that span of 20 minutes," says Johnson, "we're bringing forward this model with three main tenets: understand, connect, and empower."

That empowerment is crucial, she says, because the future depends on the people who volunteer for clinical trials. "The reality is that new treatments and new medicines don't come onto the market without clinical trial volunteers."

Kesler agrees. "We want patients to understand they're contributing to the larger picture. Down the road, this could help your grandchildren." ■

Jeff Somers

What to Expect From Your First Clinical Trial

Clinical trials have led to important medical breakthroughs, including vaccines and treatments for heart disease, diabetes, cancer, and other diseases. Here's what to consider before enrolling in a clinical trial:

1 Participation is voluntary

Participants can leave the trial for any reason, at any time.

2 There may be some risks

The investigational treatment you receive may not help you. Additionally, it could cause medical problems.

3 It's a commitment

Participants may have to visit the research clinic several times and have several tests done.

4 Some questions to keep in mind

- How long is the trial going to last and what will I be asked to do as a participant?
- How will the treatment be given?
- Does the trial involve a placebo or a treatment already on the market?
- Do I have to pay for any part of the trial? Does insurance cover these costs?
- Will I be reimbursed for travel costs or childcare?
- Who is conducting the clinical trial?
- Does the trial doctor have financial or special interests in this trial? What are the trial doctor's credentials and research experience?

5 There are different types of clinical trials

- **Treatment trials:** test new therapies such as new drug combinations or new surgical approaches.
- **Prevention trials:** research ways to prevent disease, including medicines, vitamins, vaccines, or lifestyle changes.
- **Diagnostic trials:** research tests or procedures for diagnosing specific diseases or conditions.
- **Screening trials:** identify ways to detect certain diseases or health conditions.
- **Quality of Life trials:** also called Supportive Care trials, look at ways to improve comfort and the quality of life for people with chronic illness.

6 Clinical trials are conducted in phases

- **Phase I:** researchers test a new drug/treatment in a small group of 20-80 people for the first time, looking at safety, dosing, and side effects.
- **Phase II:** the study drug/treatment is given to 100-300 people to see if it's safe and effective.
- **Phase III:** the study drug/treatment is given to 1,000-3,000 people to confirm its effectiveness, monitor side effects, and compare it to commonly used treatments.
- **Phase IV:** once approved, the study drug/treatment is given to thousands of people to do additional research on its risks, benefits, and optimal use.

7 They're overseen by an IRB

All clinical trials in the United States must be reviewed and approved by an Institutional Review Board (IRB). This independent committee of professionals and members of the community ensures that risks are low and that the trial is worth the potential benefits.

Learn more about clinical trials by visiting CISCRP.org.

Personalized Medicines Mean Faster Treatments for Cancer Patients

In April of 2003, the U.S. National Human Genome Research Institute (NHGRI) announced that it had mapped 99 percent of a human genome, ushering in the possibility of a new age of medicine.

Reflecting on the significance of the event, Francis Collins, M.D., Ph.D., then the director of NHGRI, predicted that it would someday "give healthcare providers immense new powers to treat, prevent, and cure disease" by helping them confirm whether a treatment is likely to work for a given patient before the treatment is actually prescribed. This kind of "personalized" medicine can help patients get on effective treatment regimens faster, and avoid the expenses associated with treatments that do not end up helping them.

Personalized medicines

Today, patients have more opportunities than ever to participate in clinical trials for personalized medicines.

According to the Tufts Center for the Study of Drug Development, 42 percent of all medicines being developed are associated with personalized medicine strategies.

When it comes to cancer medicines, the number is even higher with potential personalized medicines representing 73 percent of the drugs being tested in that area. Because these treatments zero in on genes expressed only by cancer cells, they are often safer and more effective than chemotherapy, which kills both cancerous and healthy cells.

A new era

The Tufts study, which was conducted in 2015, also showed that researchers working to develop new drugs believed the number of personalized medicines in development would increase further by 69 percent by the year 2020.

By participating in clinical trials for personalized medicines, patients can help bring about a new era in medicine where physicians more commonly use molecular tests to determine which medical treatments will work best for each patient. By combining the data from those tests with an individual's medical history, circumstances, and values, healthcare providers will be able to develop targeted treatment and prevention plans and thereby move away from the one-size-fits all or trial-and-error medicine usually practiced today. ■

Edward Abrahams, President, Personalized Medicine Coalition

Considering Alternative Options When You Don't Qualify for a Clinical Trial

Finding and getting access to clinical trials can be challenging for patients. But for those who don't qualify for a study, but still want to gain access to the medicine, there are a few newer options: compassionate use and right to try.

"This is a very exciting time for patients to be considering clinical trials," says Ken Getz, the founder of The Center for Information and Study on Clinical Research Participation (CISCRP), a nonprofit dedicated to educating the public and patients about the clinical research process. "Clinical research professionals are looking for new ways to engage patients as partners in clinical research."

Getz, who's also an associate professor at the Center for the Study of Drug Development at Tufts University School of Medicine, writes extensively about clinical trials.

The third edition of his book, "The Gift of Participation," will soon be released. The book updates patients and participants about trends in, and changes to, the clinical trial industry. The book also discusses ways that people can gain access to investigational drugs when they don't qualify for a clinical trial.

Right to try

In 2018, a federal legislative act, The Right to Try Act, granted terminally ill patients in every state the "right to try" experimental treatments if they've exhausted all other options.

The patient and the physician petition the pharmaceutical company to provide the investigational treatment to the patient. Still, it can be a bit more complicated, since individual states and pharmaceutical companies often have additional rules. Patients need to accept risks and, in some cases, they may need to pay the cost of the investigational drugs.

"They may lose their insurance coverage, they have to provide their complete consent that they understand what they're getting into and then the pharma company sends them the study drug," says Getz, noting that only relatively small numbers of patients have already tried "right to try" medicines.

Compassionate use

Another option is compassionate use, also called expanded access, which is for people who are facing a life-threatening, but not necessarily terminally ill, condition. In that case, the doctor has to reach out to the drug company, then fill out a special form asking the FDA to grant permission for the patient to receive the investigational drug.

While a doctor's request is typically granted, the patient will also need to get approval from the Institutional Review Board (IRB), the ethics oversight committee for clinical trial participants.

Patient voice

While there's promise for patients to be able to try new therapies, there are a few downsides too.

"When you're dealing with a really debilitating and challenging illness, having to jump through some of these hoops takes some time," says Getz.

His book also addresses precision medicine and the growing role of biomarkers and genetic data in clinical trials. By sharing their personal genetic information, a patient's privacy is vulnerable.

"What are the issues you need to know about when you agree to make your biomarker and genetic data available, not only for your own trial, but that might be accessed in the future to support other clinical research?" he says. "How is that data archived and stored and how is your privacy protected?"

Overall, he's encouraged that so many pharmaceutical companies and doctors are focused on engaging patients in clinical trials. ■

FOR MORE INFORMATON, VISIT CISCRP.ORG.

Kristen Castillo

Improving the Clinical Trial With Increased Diversity

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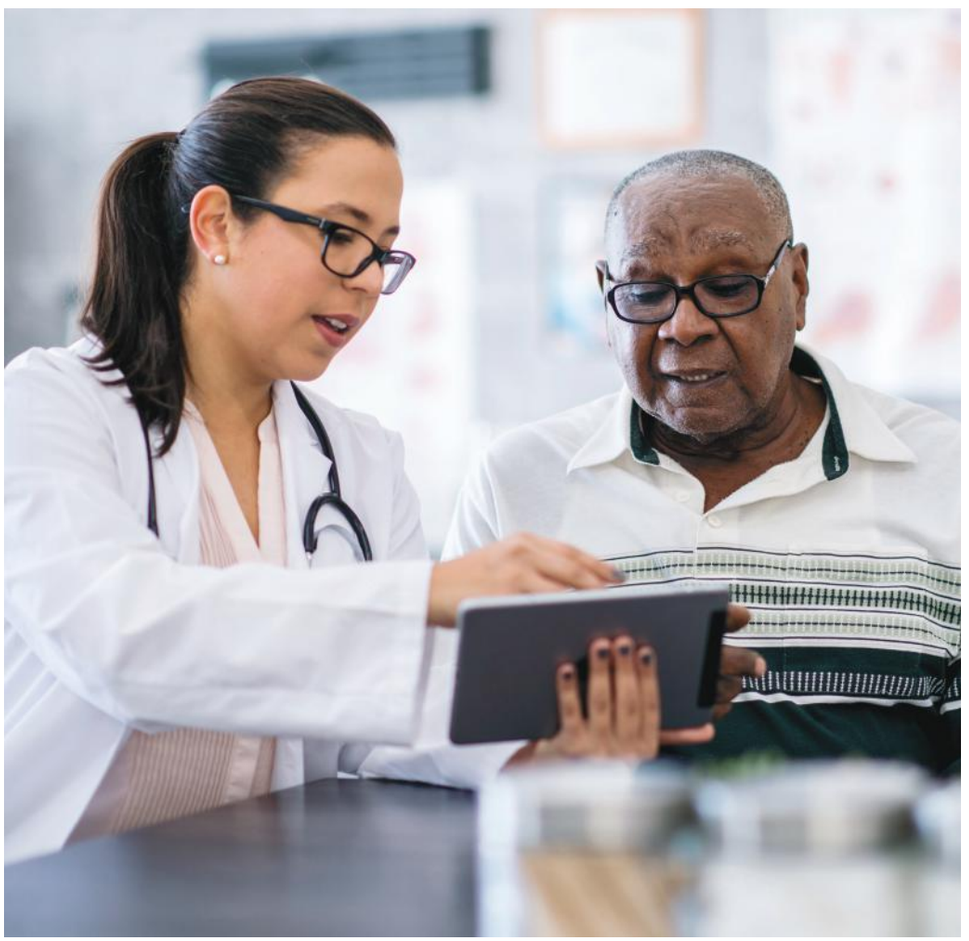
Bristol-Myers Squibb

Clinical trials struggle to include diverse patient groups that enrich and refine their results, but new tools are turning the tide.



Clinical trials are critical to the development of medicines that can improve and extend lives. But the medical research world is waking up to a problem: A lack of diversity in the patient populations. In 2016, for example, out of 31,468 participants in clinical trials, 76 percent were white.

This isn't just an issue of representation. "Differences between people can often lead to variable or different responses to the same medication," explains Kate Owen, senior vice president of global clinical operations at Bristol-Myers Squibb. "The gender, age, race, and ethnic origin of a patient may play a role in how treatment may work or even how safe it may be."

A crisis

Thomas Farrington, a prostate cancer survivor, author, and founder of the Prostate Health Education Network (PHEN), knows from personal experience the importance of diversity in clinical trials. "Black men are 60 percent more likely to be diagnosed with prostate cancer and suffer a 130-150 percent higher mortality rate," he says. "We have an ongoing clinical trial initiative to educate about participation in clinical trials and the benefits of clinical trials because diversity in clinical trials is absolutely an ongoing challenge."

This matters because researchers need data. "The participation rate for African American men for prostate cancer is at levels that are not statistically significant. [This] presents a problem when you want to look at some population analysis — for instance, how African American men may fare with a certain treatment relative to the population as a whole, because the participation is so low, it's hard to rely upon that data. So there is a need for a big push to increase diversity in clinical trials."

New tools

Online tools are seen as a good way to make that push. "We've created a prostate cancer-specific search tool on our website we call Find Your Trial (FYT)," says Farrington. "With one click you can identify trials that may be appropriate for you."

Bristol-Myers Squibb is also using the Internet to drive up trial participation in diverse groups across many different conditions and diseases. "We have over 150 studies being conducted across the globe," says Owen, "and we've developed a website called Study Connect (www.bmsstudyconnect.com) as a starting point. Patients have the opportunity to complete a brief pre-screening questionnaire which can help match them to an active BMS clinical trial. We are also proud that Study Connect will not only match patients to BMS trials, but non-BMS available trials as well."

Trial education

Another way to increase diversity is through educating people on the benefits of participating in clinical trials. "Through our clinical trials, we are working to match the right clinical trial with the right patient at the right time," says Owen. "We are exploring a variety of innovative tactics that bring those trials to the communities where the patients are found, including focusing on the geographic locations where the patients with the diseases we are studying are living. We also work with physicians, disease-specific organizations, and community groups to bring awareness, accessibility, and the voice of the patient to clinical trials."

And clinical trials aren't reserved for the terminally ill. "A lot of patients don't know that clinical trials are available along a journey," notes Farrington, "for example, a prostate cancer journey. There are clinical trials available for the newly diagnosed and for the last stage of treatment — through the whole continuum of the disease journey."

The push for diversity is receiving whole-hearted support from large pharmaceutical companies that run clinical trials like Bristol-Myers Squibb. "BMS is committed to ensuring that our clinical trials reflect the patients most affected by the conditions we are researching," Owen says proudly.

For Owen, the bottom line is simple. "Broader representation leads to better science," she says, "and better science leads to safer and more appropriate medical practice and care for all of us." ■

Jeff Somers

TO THE MILLIONS OF PEOPLE WHO VOLUNTEER FOR CLINICAL TRIALS EACH YEAR.

Clinical research participants make our lives better. They help advance knowledge about disease and improve medicine for generations to come. To learn more about clinical research and to show your appreciation, visit CISCRP.org.

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