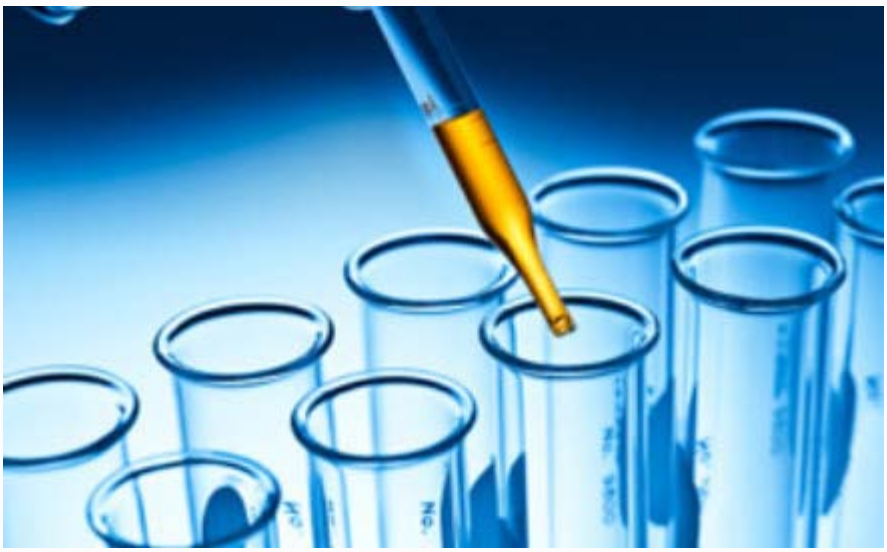
Call Us Today 202-742-1500

PATRICK MALONE & ASSOCIATES

Better Healthcare Newsletter from Patrick Malone

Give a lasting holiday gift of health: Volunteer for a medical research study



Dear Jessica,

That toy the kids whined about for weeks may be broken hours after emerging from the box. The gift card you gave will be frittered away, probably on junk. That adorable new pet adorned with a bow? It may be a pain in the parents' anatomy by the new year.

If you're like millions of Americans, you want this festive holiday season to also have higher meaning, especially when it comes to

IN THIS ISSUE

BY THE NUMBERS

10,000

Estimated number of diseases, for which experts say only 500 treatments or effective means of management currently exist.

231,169

Number of clinical studies now listed with U.S. National Institutes of Health in 50 states and 194 nations.

sharing things of value. So here's some “out-of-the-box” thinking. Consider giving a unique gift of health by participating in medical research.

This volunteerism can be done by young and old, the well and the sick. It may benefit you in many ways: You will know you helped others in a special way. You may assist in advancing new treatments. You also may get extra medical attention, even access to therapies unavailable to the public.

Just by joining a clinical study, you will gain insights into a critical component of contemporary medicine. And you will learn more about a vital element in your health care—your right to informed consent.

Participating in medical research has risks. It requires a commitment not made lightly. Don’t let your eyes go all aglow at the prospect. Keep them and your mind wide open as you unwrap a distinctive way to help others. And read on.

Clinical trials can benefit volunteers

The dramatic headlines almost daily detail medical-scientific progress, including: New treatments based on patients’ genes, cancer drugs that get the body’s own systems to attack disease, vaccines being developed for viral infections like Zika and HIV-AIDS, and improved ways to avert heart disease. Look closely at any of these, and they share something fundamental: They require human testing, medical research conducted in rigorous, tightly supervised, and ethical fashion in what broadly

37.4

Percentage of 106,000 individuals who report 'no conditions' (they are healthy) and who have volunteered to participate in medical research through a major nonprofit organization.

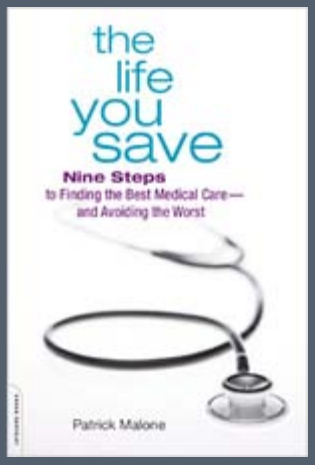
QUICK LINKS

Our firm's website

Read an excerpt from Patrick Malone's book:

The life you save

Nine Steps
to Finding the Best
Medical Care —
and Avoiding the Worst



LEARN MORE

Read our Patient Safety Blog, which has news

are termed clinical trials. These occur, as the figure above shows, relatively late in what can be a long scientific process. Only after experts have labored long in labs, in research and development (R&D) and especially with animal studies, do they progress to clinical, interventional, or observational studies with slowly increasing numbers of human subjects.

In these studies, investigators may seek to understand how a disease runs its course, how to prevent or test for it, as well as how best to diagnose and treat it. The research may determine patients' quality of life with drugs, devices, or treatments. Health professionals (doctors, nurses, and Ph.Ds.) conduct these studies, backed by research institutions (including medical schools and universities), the federal government, and businesses (drug or device makers). It often occurs at universities and academic medical centers, because this kind of study can be costly, time- and resource-intensive. It adds to institutional prestige—and potentially profits. The Washington, D.C.-area is rich with clinical trial sites, including top hospitals, elite academic medical centers, and the various institutes of the National Institutes of Health (NIH) in Bethesda. That could make your role in medical research easier and more convenient.

Types of clinical trials

In interventional studies, Uncle Sam notes, patients may be treated with: "Medical products, such as drugs or devices; procedures; or changes to participants' behavior, such as diet. [These] trials may compare a new medical approach to a standard one that is already available, to a placebo that contains no active ingredients, or to no intervention. Some … compare interventions that are already available to each other. When a new product or approach is being studied, it is not usually known whether it will be helpful, harmful, or no different than available alternatives (including no intervention). The investigators try to determine the safety and efficacy of the intervention by measuring certain outcomes in the participants."

In contrast, in observational studies, experts “assess health outcomes in groups of participants according to a research plan or protocol. Participants may receive interventions (which can include medical products such as drugs or devices) or procedures as part of their routine medical care, but participants are not assigned to specific interventions by the investigator (as in a clinical trial).” Observational research can involve following and recording data on large groups of subjects over extensive time. This is how the decades-long Framingham heart study led experts to emphasize exercise, diet, and other factors like stress-reduction in preventing coronary disease.

Participants in clinical studies must meet stringent requirements to qualify. Researchers value highly specialized subjects, including those with certain illnesses, past or present. It’s misunderstood that clinical studies only involve the sick or those with close relatives with specific diseases or conditions. Studies are planned in phases (see figure above). Early on, in Phase One and even Phase Two, researchers may want younger, healthier subjects to get baseline, control data. Children, veterans, and seniors also are welcome participants, enrolling under strict protections.

Benefits of participation

Study volunteers can benefit. They can get the satisfaction of knowing their participation helps others and may advance medicine. They often receive some compensation for their time and trouble. Besides their regular care—and anyone considering clinical trials should consult their personal physician—they may receive added medical attention. They may undergo intensive exams to assess their qualification for or status during trials. If seasonal allergies, say, seem to tee you up for a test of prescription drops to help ease watery, hay fever-inflamed eyes, you may also undergo at no charge a fuller battery of expensive allergy tests. If you’re about to be a control subject for a new blood pressure medication, you might get a complete, no-cost physical.

More important, in later stage trials, patients with chronic or serious illness may receive novel or innovative drugs or treatments not yet available to the public. The costs of this therapy may be partially or fully covered by trial sponsors and participating research institutions. Some gravely ill cancer patients have enrolled in trials and received drugs costing more than $100,000 a year—and that made huge differences in their health. But the effectiveness of experimental drugs may not last or they may require them for longer than is covered in a clinical trial. All research outcomes aren't optimal and cheery. Volunteers must understand this from the start.

Risks? Informed consent is critical

You're upset after learning that a loved one is afflicted with a disease with few favorable treatments. You're healthy and decide you want to do something to prevent this from happening to anyone else. Or maybe you discover that your family has a predisposition (like the BRCA gene mutation for cancer, for instance), and, though it hasn't hit you yet, you want to assist investigators in attacking it before it does. Or you may have cancer, heart disease, or other conditions, and you have decided, at some stage in your illness, that you want to help others. No matter the motivation, when you choose to enroll in a clinical trial you will be a volunteer. And among the few.

Although opinion polls show they're admired for doing so, only a small percentage of Americans participate in clinical trials. Fewer than 5 percent of cancer patients participate in them. Many studies get canceled due to lack of subjects. Uncle Sam is making a big push to get more, now under-represented women and people of color into studies. If you decide you want to be included, you quickly will learn that investigators, mindful that the mere mention of human experimentation sends shivers down the spines of some, will provide you with abundant information about your prospective role.

Informed consent

This isn't a mere formality. Laws require investigators to detail for you answers to dozens of questions about a clinical trial, including who will run it, who will benefit from it, as well as its goals, methods, duration, demands (time and travel), potential costs to you, and, most critically, your possible health benefits and harms. Who oversees patient safety? What kind of protections will you have during and after the trial? Will drugs or treatments have side effects, and will these be lasting? The voluminous flow of information starts at the beginning of the process—it gets prominent display in online resources about medical studies and signing up to be considered for them (see below). If you're healthy and raring to go, this may be patience testing; if you're sick and possibly failing, it may seem excruciating.

Modern medical science, though, is haunted still by the horrors of cruel exploitation of human subjects. It wasn't that long ago that sadists from the Axis powers conducted heinous human experiments. It was only in 1997 that President Clinton formally apologized to victims of federally subsidized experiments in Tuskegee, Ala., on African American syphilis patients in the 1930s (in which doctors deliberately withheld curative drugs from patients to observe the effects of late-stage syphilis). It was only in 2010, after Rebecca Skloot wrote her nonfiction best-seller, that the nation learned the sad saga of Henrietta Lacks. She was a poor African American mother of five who died in 1951 at

Johns Hopkins in Baltimore of an aggressive cancer—but not before her cells were harvested. Neither Lacks nor her family knew about or benefited from years of researchers' cultivation of the "immortal" *HeLa* (cryptically named for her) experimental strain of cells, which paved the way for advances in chemotherapy, the polio vaccine, and *in vitro* fertilization.

Patient involvement and transparency have become a major aspect of medical research. And this includes a critical affirmation of one of your basic, vital patient rights: *informed consent*. This isn't something that gets handled with a lot of signatures and initials on piles of paperwork. It is the duty of anyone who provides you any kind of medical treatment. Informed consent expresses a concept at the core of any free society: Each person has a right to decide what to do with his or her own body, as long as you don't hurt someone else. That includes medical care. But this is a right that many scared people are quick to give up. They want to surrender decisions to doctors, not realizing two critical facts: Doctors may have very different values. They may not, for example, put as much emphasis on whether a surgery disfigures the body if it offers a slightly higher chance of a patient's living some uncertain additional amount of time. Doctors also may have conflicts of interest that make it hard to give objective advice. For example, they may make a lot of money if you opt for their surgery and no money at all if you make a different choice.

Because MDs are just as flawed and imperfect as everyone else but know a lot more medical facts than most lay people, the law and medical ethics place a simple duty on every doctor: Give patients the important facts so they can make intelligent decisions about what treatment to have and where to get it. That's why *informed* comes before *consent.* Information comes first. Consent is meaningless without the facts. Informed consent is really about building a bond between doctor and patient through a candid dialog that doesn't leave out anything important. Patients, medical ethicists say, want to make a leap of trust with professional caregivers. But trust is built on honest information. It's not a leap of blind faith. This is especially true in clinical trials,

where participation is voluntary.

IRBs, single- and double-blind studies, and placebos

At research sites, special panels known as Institutional Review Boards (IRBs) oversee clinical studies to ensure they are ethical and to protect participants' rights and welfare. These doctors, researchers, and community members legally must ensure that research risks are minimized and are reasonable in relation to any potential benefits. IRBs can prod investigators to clarify—especially if you or your loved one is in the late-stage of an illness and hoping to join research under way—whether a study is single- or double-blind and involves a placebo. In double-blind studies, neither patients nor caregivers know who is or isn't receiving a drug or treatment under research. In single-blind trials, a pharmacist might know who gets an inert alternative or the test treatment, but patients must go to a special site to get drugs from her. In many trials for drugs for late-stage diseases, patients needn't fear they will get just a placebo. Many studies, instead, compare existing drugs that are treatment standards against new medications. There also may be compassion exemptions so that more patients than not receive a medication under scrutiny, particularly if it shows promise; trials also may be suspended or halted if it becomes clear that hoped-for outcomes aren't occurring or patients are suffering bad side effects, even dying.

Investigators legally must report what are known as *adverse events* or *serious adverse events* that occur to subjects in trials. Because many volunteers may get drugs or treatments, they can suffer the same harms as occur in medical practice: Some powerful drugs may be toxic and have side-effects that make them ill. They may have reactions to medications, even inert or placebo drugs. They may be injured when receiving injections or IVs. They may develop other illnesses or complications during and even after a study, and these must be reported, even if they are only suspicious and not directly tied to a trial. Some studies, particularly of late-stage drugs or treatments, are halted when

patients die. International attention focused on such a recent incident in France, where six subjects in a drug trial were hospitalized, with one pronounced brain dead. It also should be underscored that patients can withdraw at any time from a clinical trial, and they should be told how.

Scientific advances also may force researchers and patients alike to confront tough issues about who benefits from medical research, especially clinical trials. Many innovative therapies now target diseases' genetic or molecular structures. To conduct robust investigations in these areas, researchers must collect bigger amounts of patient tissues and data. These both are going into huge banks and databases. But who owns the material or medical byproducts? If you have a gene sequence that offers protections from a disease or condition, is it yours, or does it "belong" to researchers who found it and a way to turn it into a therapy? And for how long? If your blood or tissue was routinely collected as part of a trial, is it anonymized to protect your privacy, and if it is later found to have value, must researchers let you know?

Idealism and profit potential

To be sure, the idealistic wish to heal the sick and cure diseases drives and sustains many fine doctors, nurses, and Ph.Ds, as well as universities, academic medical centers, and hospitals where they labor. But health care is a big business with huge financial stakes. Institutions gain a giant reputation boost and competitive advantage by being leaders in medical-scientific research. Drug development, via clinical trials, can lead to major profits. Although many medical studies seek to and eventually produce modest advances, some research can be likened to oil wildcatting—and many institutions of note not only are out there digging, they have intellectual property teams ready to reap riches from the rare gusher. In 2014, just 15 major American universities produced 70 percent of all revenues among their peers from patent-license royalties. New York University got $650 million in such payments for the rheumatoid arthritis drug Remicade. Emory University received $525 million in royalties for

Emtriva, an HIV drug. The University of California at Los Angeles has signed a deal for $520 million for Xtandi, a prostate cancer treatment. And Northwestern University says that as much as 18 percent of its $18 billion endowment, the eighth largest in the country, can be traced to pioneering work in campus labs that resulted in Lyrica, a hot-selling, non-opioid prescription drug to treat nerve pain and seizures.

Here’s hoping that you're blessed with great holidays, and if you choose to give your time to enroll in a clinical trial, that it helps you, your loved ones, and all of us with some significant health benefits!

How do I sign up?

Intrigued? If you want to learn more about enrolling in a clinical trial, talk to your own doctor first. She may be able to offer counsel on suitable choices. If you're volunteering because you want to help a friend or loved one, you may want to talk with them and their health professionals to see if they have insights about appropriate research opportunities for you. Advocacy or support groups for specific illnesses or conditions also may offer productive ideas.

Uncle Sam has important resources you can tap, starting with the web site clinicaltrials.gov, which can be accessed here. This is one of the major registries of trials, and officials have sought to provide abundant information about research studies, participating in them, and other pertinent resources available through

Improving a key component of modern health care

Although volunteers play a crucial role in medical research, investigators struggle to recruit them. Few Americans participate in clinical trials. Doctors may need to step up their role in letting patients know about them. Ditto for advocacy groups that aim to help individuals with specific diseases.

Uncle Sam, too, has come under fire for not doing more to assist. In response, the U.S. National Institutes of Health has tried to improve its major web site resource on clinical trials, boosting the information it provides, as well as aiming to simplify and clarify how users search for appropriate research projects.

this site. The search engine allows volunteers to search a database of more than 230,000 trials around the world. Type in "healthy volunteer," if that fits you, and many options will pop up. Alas, the trial abstracts are replete with medical and scientific jargon and may not be the easiest for lay people to figure out. Researchers are still grappling with this issue. It is invaluable, though, for prospective trial participants to study thoroughly the available information, including from the federal Food and Drug Administration, about risks and benefits of trial enrollment.

To assist, nonprofits also have stepped in. Prospective volunteers may, for example, want to click here to access the website of researchmatch.org, an organization that says it has more 100,000 volunteers who hope to assist more than 100 institutions in clinical trials. It also may be worth clicking here to check out the web site of the Center for Information and Study on Clinical Research Participation. It's a nonprofit that says it even will conduct hand-searches to help volunteers find appropriate trials.

The Washington, D.C., area is rich in medical research sites. The NIH web site on clinical trials can be accessed here, while the region's many former service personnel may wish to access the U.S. Department of Veterans Affairs site for research information here. Meantime, Johns Hopkins and Georgetown are among the many teaching and academic medical centers and major hospitals in the region where many research initiatives also are under way. If you or your MD deal often

Hospitals, academic medical centers, and other research institutions have stepped up their communications about their trials.

Full disclosure: Medical research is a field rife with its own controversies. Concerns are growing that trials may produce skewed results and fuel health disparities because the willing subjects lack sufficient gender, racial, and ethnic diversity. Critics, to their credit, point out that investigators have been too pokey under federal law about disclosing results, particularly when they're bad. Volunteers are pressing researchers more—and good for them for doing so—to share not only their studies' final results but also more of the health information they gather on patients during trials.

Contrary to persistent, misleading media reports on health research, medical science advances slowly. You know now why care needs to be exercised in discussing trials in early phases, or in jumping to conclusions about research that differs because it is interventional versus observational (where associations are not causes).

But the sometimes glacial pace at which health investigations proceed is an issue—both for the desperately ill (for example, those in past days with then-deadly HIV-AIDS) and for commercial interests like device makers and Big Pharma. The federal Food and Drug Administration, as the main regulator in this area, has taken the most heat and has tried to respond to criticisms, for example, with expedited drug reviews. Congress, even as this newsletter is written, is in the midst of a

with a major hospital that you both like and respect, check out the institutions' web sites to see what clinical trials may be under way there that you might volunteer for.

year's work on a sprawling health measure with potentially significant effects on medical research. Stay tuned.

Recent Health Care Blog Posts

Here are some recent posts on our patient safety blog that might interest you:

- Three Washington, D.C.-area teaching hospitals have ranked in the lowest-scoring group nationally on preventing infections when their patients are hooked up to central lines, intravenous tubes that supply fluids, medications, and nutrients to those in dire need. Two institutions in the region rated highly. Consumer Reports deserves credit for its continuing reporting on hospital-acquired infections (HAIs), a scourge that in 2011 afflicted 650,000 already ailing Americans and which contributed to 75,000 deaths. The advocacy group says 27,000 patients were felled with central line infections in 2015, with a quarter of these especially sick and frail individuals dying of them. Treating patients for central line infections cost on average $46,000—more than for any other HAI. The area teaching hospitals that the magazine ranked poorly, based on an analysis of federal data from 2011 to 2015, were: George Washington University Hospital, Holy Cross Hospital in Silver Spring, and Howard University Hospital. The two high-ranking institutions were: MedStar Franklin Square Medical Center in Baltimore and Sentara Norfolk (Va.) General Hospital.

- Your kid takes a tumble and breaks an arm at a sleep-over. Your spouse, on a business trip, suffers sudden chest pain and shortness of breath. You're in beach slippers and step by accident on a shard of glass stuck in the sand. Now, you've got oodles of time to check your insurance policy to find the nearest emergency room that's covered by your insurer, right? And you'll be asking every physician who treats you if they're part of your network, right? Well, no, nobody does that. So brace yourself: a new study says that 1 in 5 Americans gets whacked after their ER visit with added charges not covered by insurers for out-of-network care. The surprise medical bills averaged $900 but ran as much as $19,000."To put it in very, very blunt terms: This is the health equivalent of a carjacking," Zack Cooper, an assistant professor of health policy and economics at Yale University, commented to the New York Times. He is a co-author of the paper on surprise medical bills, published in the peer-reviewed New England Journal of Medicine.

- American adults' cigarette smoking keeps declining, but this "persistent and preventable

health threat" takes a terrible toll on those who still light up, with 40 percent of cancer diagnoses in the country linked to tobacco use, public health experts say. The federal Centers for Disease Control and Prevention reports that between 2005 and 2015, "smoking among adults declined from 20.9 percent, or 45.1 million people, to 15.1 percent, or 36.5 million. The overall rate fell 1.7 percentage points last year alone, resulting in the lowest prevalence since the CDC began collecting data in 1965," the Washington Post notes.That might sound like good news. But CDC Director Tom Frieden also told reporters, "Of the 36 million current smokers, nearly half could die prematurely from tobacco-related illnesses, including 6 million from cancer, unless we implement the programs that will help smokers quit." The CDC noted that each year between 2009 and 2013, 660,000 people were diagnosed annually with tobacco-related cancers, and 340,000 Americans died of those cancers.

- They keep expanding, even though evidence-based research indicates they're not working. They're unfair to employees. They intrude on workers' privacy. And so AARP, the largest advocacy group for older Americans, is suing a federal agency to try to curb workplace wellness programs. AARP says in its suit that the Equal Employment Opportunity Commission, which oversees many workplace practices, must recognize that employer wellness programs are discriminatory, and they violate workers' rights to keep private their personal health information. Believing the programs help reduce ever-rising medical costs by encouraging healthier behaviors, employers are offering increasing incentives to get their employees into wellness programs. Some pay as much as 30 percent of workers' annual health insurance premiums. The New York Times, citing Kaiser Family Foundation data, said those yearly premiums for an individual average run $6,435. That means a worker could lose as much as $2,000 by declining to join a company wellness program. But AARP says the EEOC should immediately bar these kinds of incentives, because they are biased against those who are ill and do not want to disclose that to their employers.

HERE'S TO A HEALTHY 2016!

Sincerely,

Patrick Malone
Patrick Malone & Associates