Understanding Clinical Trials
A GUIDE FOR PATIENTS AND THEIR FAMILIES

WHERE INFORMATION EQUALS HOPE

CONTENT REVIEWED BY A DISTINGUISHED MEDICAL ADVISORY BOARD
NOT JUST FOR YOU, FOR THEM.

Men and women aged 18 years or older who have advanced breast cancer due to a BRCA1 or BRCA2 gene mutation are invited to see if they may qualify for the Brocade Study. The purpose of this medical research study is to determine the safety and effectiveness of the investigational medication Veliparib (a PARP-inhibitor) in combination with chemotherapy in patients with metastatic or locally recurrent breast cancer. Each individual will be evaluated to determine his or her eligibility. Those who qualify will receive chemotherapy in combination with either the investigational medication Veliparib or an inactive placebo, BRCA-related testing, study-related medical exams, and lab tests at no charge. Compensation for time and travel may also be available. To see if you may qualify, call 1.855.5ONCOLOGY (1.855.566.2656) or visit BrocadeStudy.com.
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Nearly all of the cancer-fighting drugs and devices currently available exist only because they were thoroughly tested beforehand. These tests, known as clinical trials, are research studies designed to evaluate the safety and effectiveness of new drugs or other types of therapies. If you’ve been diagnosed with cancer, a clinical trial may be one of many treatment options available to you.

The primary benefit of a clinical trial is access to the highest quality of cancer treatment, with the possibility of receiving a new treatment before it is widely available. People who have participated in a clinical trial have identified several perceived advantages to participation, including having access to the best available care, receiving newer/better treatment, receiving increased medical attention, playing an active role in their own health care, and making a valuable contribution to cancer research.

To make an informed decision about volunteering for a clinical trial, educate yourself about clinical trials and weigh the advantages and disadvantages of a trial recommended by your doctor.

### MAKING THE DECISION / to volunteer in a clinical trial

#### TABLE 1 | WEIGHING ADVANTAGES AND DISADVANTAGES OF CLINICAL TRIALS

<table>
<thead>
<tr>
<th>Potential advantages</th>
<th>Potential disadvantages</th>
</tr>
</thead>
<tbody>
<tr>
<td>Access to the best possible care through treatment with either the current standard of care or a treatment thought to offer more benefit than the standard of care</td>
<td>Lack of clearly defined side effects or risks associated with experimental treatment</td>
</tr>
<tr>
<td>Potential for being treated with a new drug before it is widely available</td>
<td>Chance that experimental treatment may not be better than the standard of care</td>
</tr>
<tr>
<td>Close monitoring during treatment and follow-up (often more so than in routine practice)</td>
<td>Potential that not all participants will benefit from experimental treatment</td>
</tr>
<tr>
<td>Chance to play active role in health care</td>
<td>Need for frequent tests and clinic visits</td>
</tr>
<tr>
<td>Opportunity to help future generations of individuals with cancer</td>
<td>Possible need for travel to clinical trial site</td>
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#### QUESTIONS TO ASK BEFORE VOLUNTEERING FOR A CLINICAL TRIAL

- Why is this trial being done?
- Why is it believed that the treatment being studied may be better than the standard treatment?
- What are my other options (standard treatments, other studies)?
- What were the results of any previous studies of this new treatment?
- What are the possible side effects or risks of the new treatment?
- What are the possible benefits?
- How will the doctor measure if the treatment is working?
- Can I choose to continue to get this treatment after the trial ends?
- How long will the trial last?
- What kinds of procedures or tests are involved?
- What impact will the trial have on my daily life?
- Will I have to travel to receive treatment? Will I be compensated for expenses?
- How often will I need to travel to receive treatment?
- Will I have to be hospitalized as part of the trial?
- What type of long-term follow-up care will be done?
- Will I still be under the care of my doctor, or will I see someone different?
- What costs (if any) will be my responsibility to pay?

### Get the facts

Learn as much as you can about clinical trials because many myths have circulated among the general public. As a first step, know the difference between the myths and the real facts (see sidebar). You should also learn as much as you can about any specific trials for your type of cancer. Some people may think that a clinical trial is not an option for them because their doctor didn’t recommend it. However, if your doctor does not ask you about clinical trials, you should raise the discussion yourself. Ask your doctor and medical team about trials that may be appropriate for you.

Even if your doctor recommends a clinical trial, the decision is a personal one and is yours alone to make. Many individuals with cancer have found it helpful to talk about the decision with family members or friends. Ask your doctor if you can talk with someone who has participated in a previous trial. Such a conversation can be beneficial, as studies have shown that the overwhelming majority of clinical trial participants had a positive experience. Also ask your doctor or a member of your medical team about clinical trial resources available online or in your local community.

When educating yourself, think about how you learn best. Educational resources are available in a variety of formats, such as print (hard copy and online), Web-based interactive tutorials and video. Print materials may also be available in several languages and may be designed for people with varying levels of education. Talk to a member of your research team about choosing resources that best fit your needs and learning style.
Weigh the advantages and disadvantages of volunteering for a clinical trial. These have been detailed by cancer experts as well as by clinical trial participants themselves (Table 1). As you think about these advantages and disadvantages, focus on what is most important specifically to you. Make sure you understand the details of the particular trial you’re considering; asking several questions can help you in this decision-making process.

Your doctor can tell you about specific details that may be associated with the particular trial that he or she recommends. The research team will also explain the details of the study, including the benefits and potential risks, and if you agree to volunteer for a trial, you will sign an informed consent document to confirm that you understand what is involved in the study. You can take this document home and talk to your family and friends about the advantages and disadvantages of a clinical trial. The informed consent document is not a contract and does not obligate you to remain in the trial. You may withdraw from the trial at any time and you would be able to receive the standard treatment for your cancer outside of the trial.

Find your match
Find a clinical trial that’s right for you by first asking the members of your medical team if they can recommend a clinical trial that might benefit you. In addition, a number of government and private organizations provide listings of clinical trials and information about the trials on their websites (see sidebar).

**DISPELLING THE MYTHS OF CANCER CLINICAL TRIALS**

**MYTH** Some participants in a clinical trial will get a placebo (sugar pill) instead of treatment.

**FACT** Participants in cancer clinical trials will receive the standard of care as a foundation and then the experimental treatment or a placebo will be added to it. They will never receive a placebo instead of a cancer treatment.

**MYTH** Clinical trials are not safe.

**FACT** Clinical trials have many built-in safeguards to ensure that participants’ rights and safety are protected. These safeguards include an institutional review board, data and safety monitoring board, and an ongoing informed consent process.

**MYTH** Clinical trials only take place at large hospitals or cancer centers.

**FACT** Many clinical trials are now done at local hospitals, cancer centers and doctors’ offices.

**MYTH** The cost of care in a clinical trial is not covered by health insurance.

**FACT** Under the new health care laws, patient care costs (such as going to the doctor, any stays in the hospital or certain testing procedures) are covered by insurance. Research costs are those directly related to the study. It’s common for the trial sponsor to cover these costs but not guaranteed, and insurance does not typically cover research costs.

**MYTH** Clinical trials involve treatments with unknown safety and efficacy.

**FACT** Phase III clinical trials involve treatments that have been shown to be safe and effective in earlier-phase trials. Phase III trials also include treatment with the standard of care, which has well-established evidence of safety and efficacy. Phase I and II trials are based on the results of preclinical trials showing the potential for efficacy and are carefully designed to ensure the safety of participants.

**MYTH** Participants in clinical trials are treated like “guinea pigs.”

**FACT** The overwhelming majority of clinical trial participants (97 percent) say they were treated with dignity and respect; 93 percent report having a positive experience in the trial.

**FIND A CLINICAL TRIAL**

If your doctor does not mention a clinical trial as an option for you, ask about this possibility. An increasing number of websites are available where both physicians and patients can find out about clinical trials:

- Center for Information and Study on Clinical Research Participation: www.searchclinicaltrials.org
- Coalition of Cancer Cooperative Groups: www.cancertrialshelp.com
- My Clinical Trial Locator: http://myclinicaltriallocator.com
- National Cancer Institute: www.cancer.gov/clinicaltrials
- National Institutes of Health: www.clinicaltrials.gov
- TrialCheck: www.trialcheck.org

**FACT** Clinical trials are only for people who have no other options for treatment (a “last resort”).

**FACT** Clinical trials of cancer treatment are designed for individuals with cancer of all types and stages.

**MYTH** Signing a consent form to participate “locks” you into staying in a trial.

**FACT** Once you have signed a consent form, you are free to change your mind and not participate. You can also decide to drop out of a trial at any time for any reason.

**MYTH** A clinical trial must be recommended by a doctor in order for a person to participate.

**FACT** If your doctor does not talk to you about clinical trials, raise the topic yourself. You can also search for clinical trials online; contact information for trials is given.

**FACT** Many clinical trials are now done at local hospitals, cancer centers and doctors’ offices.

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Clinical trials may seem complex, but understanding the various types, phases and aspects of the process will help you make an informed decision about whether you want to participate.

Types of clinical trials
Five types of cancer clinical trials exist. Treatment trials are specifically for individuals who have cancer. Treatment trials evaluate whether a new treatment (e.g., drug, surgery, radiation therapy) or new combination of treatments is better than the treatment currently considered to be the standard of care. Quality-of-life trials, which are clinical trials for supportive and palliative care, study ways to improve the quality of life for cancer patients and survivors. These studies focus on people experiencing symptoms associated with cancer and cancer treatment. These trials may also study the effects of nutrition, group therapy, counseling and other therapies that may help cancer patients and survivors.

The other three types – prevention, screening and diagnostic trials – evaluate and study ways to reduce your chances of getting cancer. Many participants do not have cancer but some are people who have had cancer and are at risk of recurrence (the cancer returning) or a second cancer (development of a cancer other than the original diagnosis).

Phases of clinical trials
Most cancer treatment clinical trials that are part of a new-drug development take place after the preclinical trials (including laboratory and animal testing) have proven that the drug is reasonably safe for human testing and can be expected to perform as intended. Other trials may involve a drug that has already been approved by the FDA for wide use in people with a different type of cancer.

Clinical trials are designed in phases that build on one another. This systematic approach allows researchers to ask and answer questions in a way that produces the most reliable information in the safest way. Typically a clinical trial will consist of Phases I, II and III.

■ Phase I trials are the first studies in which a drug or new drug combination is evaluated in humans. They involve a small number of volunteers and are designed primarily to determine the best dose, delivery method (e.g., oral, intravenous) and schedule (how often it is given) for the drug. Side effects and safety are evaluated.

■ Phase II trials involve a larger group of participants and usually focus on the effectiveness of the drug against a specific type of a cancer. Side effects and safety are further evaluated.

■ Phase III clinical trials further determine the risk versus benefits and the overall safety and efficacy of a drug or new drug combination. For cancer treatment clinical trials, this phase compares the drug against the current standard of care for a particular type of cancer.

When the results of a phase III trial indicate that the new drug leads to better outcomes for patients and is reasonably safe (the benefits outweigh the risks), the FDA will review the results and determine whether it’s approved for market.

Once a drug is approved and becomes available to treat patients, there may be a phase IV clinical trial to further evaluate long-term safety and side effects over a longer period of time, and/or in a greater number of people.

Safety of clinical trials
All clinical trials are developed according to strict scientific and ethical principles and are conducted under the supervision of physicians and expert research professionals. Every clinical trial follows a set of rules called a protocol. The protocol defines the eligibility criteria, specifies the tests to be done and the procedures to be used, describes the medications and their doses, and establishes the duration of the study. Several safeguards are in place and regulated to ensure the safety of all participants.

What to expect in a clinical trial
In most clinical trials, study participants are randomly assigned to groups, and each group may receive something different (e.g., new drug, combination, placebo). In cancer treatment clinical trials, groups will typically receive either the current standard treatment or the current standard treatment plus the new drug. Studies may be blinded, meaning neither the doctors nor the study participants know what group they are in. This helps protect the results from any influence or expectations of the researchers and participants.

The research team will provide specific instructions for participating in the trial. Participants will be evaluated at the beginning of the trial and monitored carefully during the trial and should stay in touch after the trial ends. Participants have the greatest likelihood for benefit from the trial if they carefully follow the instructions and remain in contact with the research staff. Clinical trials are usually conducted in a hospital, doctor’s office or community clinic.

When a trial is being described to you, ask questions about anything you do not understand. You have the right to hear and read the information about a trial in the language you understand best.

WHAT'S THE COST?
Costs associated with a clinical trial fall into two categories:

■ Patient care costs – costs related to your treatment outside of the study, such as going to the doctor, any stays in the hospital or certain testing procedures (lab tests, imaging tests).

■ Research costs – costs directly related to the study, such as the study drug, testing performed for research purposes, or additional doctor visits.

Patient care costs are covered by insurance under the new health care laws. Research costs are typically covered by the trial sponsor, but it’s not guaranteed and insurance does not typically cover research costs. Talk to your insurance provider before taking part in a clinical trial.
The pharmaceutical industry holds the responsibility of first understanding a disease and then making a new, safer and more effective medicine to treat it. Through an extensive process, new medicines are being developed every day that provide doctors and patients with treatment options that are safer and more effective. And continued medical advancements are helping patients live longer, healthier, more productive lives.

About the industry

The global pharmaceuticals market is a $300 billion industry annually, with six of the 10 largest drug companies based in the U.S. With the cost of new drug development reaching nearly $1.2 billion per drug (or even higher using some estimates) – and taking an average of 12 years to develop – new drugs are a timely and expensive process. A drug being explored needs to show a good probability of a return on such a significant investment in order for companies to fund the research and development necessary to see it through. The pharmaceutical industry remains largely funded by the private sector.

Despite costs, the pharmaceutical research companies in the U.S. remain committed to the discovery and development of new medications, treatments and cures. And with more than 3,500 new compounds currently in various stages of research in the United States – more than anywhere else in the world – advances and breakthroughs are happening every day.

Economic impact

The pharmaceutical industry contributes one of the nation’s largest research and development sectors, directly employing more than 810,000 people, impacting more than 3 million additional jobs and contributing billions in spending. The medical advancements also contribute to the entire economy. If more people stay healthier, the workforce stays healthier. This leads to lower health care costs and improved accessibility for more Americans.

These companies also partner directly with research institutions and organizations around the country, including universities, medical schools, clinical research companies and local hospitals.

The role of the industry in cancer research and development

More than 1.6 million new cases of cancer were reported in the U.S. in 2013, but new drugs and treatments are being developed to fight cancer and increase survivorship, including 981 cancer drugs. That total includes 121 for lung cancer, 117 for lymphoma and 111 for breast cancer.

The number of cancer survivors in the U.S. has increased from 3 million in 1971 to 14 million today, and life expectancy for cancer patients continues to increase. Greater survivorship is attributed to earlier diagnosis and detection, better treatments and improved follow-up care. Advancements are being made every single day in the fight against cancer, and the new innovations around personalized medicine for cancer care are promising.

The pharmaceutical industry and the FDA

Drug safety is a top priority in the pharmaceutical industry, which is why it works closely with the Food and Drug Administration to ensure the safety and integrity of new medications. The FDA regulates several of the steps in the drug development process, requiring extensive research and applications prior to and after clinical testing. More than 300 new medicines have been approved by the FDA in the past decade and approximately 97 percent of them are still on the market and considered safe. Learn more about the FDA drug approval process on page 7.
Several previously deadly diseases have become treatable conditions, and many individuals suffering from chronic conditions are now living healthier, longer lives, due in part to medications developed by pharmaceutical companies. Developing a new medication is a long, difficult process, however, requiring several steps from pre-discovery to manufacturing. In fact, the journey can take more than a decade (see Figure 1).

Pre-discovery
Understanding a disease or condition as completely as possible is the first step in developing a new medication. Scientists work to discover underlying causes, how a disease can be treated and ways to target causes and symptoms. In the U.S., this research is a collaborative effort with government, industry and academic contributions.

Drug discovery
Once there’s a general understanding of the disease, the research is used to develop an idea from which researchers will work to understand the biological “targets” of the disease, such as a specific protein, molecule or gene. A researcher will then focus on a target and conduct studies to determine if that target can be influenced by a medication. Then, they look for any molecule that has the potential to influence the target. These selected molecules, which are potential medications, are called compounds.

Preclinical testing
The drug discovery phase reduces the thousands of considered compounds to only hundreds that show the most potential. These compounds then undergo preclinical testing (laboratory and animal) to determine whether the new drug is reasonably safe for human testing and if it can be expected to work as intended. Preclinical testing can take several years to complete – three to six years including drug discovery – and only a handful of compounds typically move on to the clinical trial phase. At this point, the pharmaceutical research company must file an Investigational New Drug Application (IND) with the Food and Drug Administration (FDA). The IND must include results from the animal studies (including toxicity) as well as the drug ingredients, manufacturing and processing information, and protocols for all proposed clinical trials.

Clinical trials
During clinical trials, the new drug is tested in humans. These trials are federally regulated to ensure participants aren’t subjected to unreasonable risk and that all potential volunteers are made fully aware of the risks associated with the trial. The safety and integrity of the trial are extremely important factors in the clinical trial process. Before a drug can be submitted to the FDA for review, it must complete all of the primary phases of clinical testing (see page 4), which on average takes six to seven years.

Review and approval
If the drug has been proven safe and effective in all three phases of clinical trials, the pharmaceutical company can then submit a New Drug Application (NDA) to the FDA. The application must include results from the animal studies, data from all of the clinical trials, ingredients in the drug, and drug manufacturing, processing and packaging information.

The FDA reviewer must then conclude that the drug is safe and effective for its proposed use, that the benefits outweigh the risks, that the labeling has accurate and appropriate content, and that the methods of manufacturing and quality control adequately preserve the identity, purity, quality and strength of the drug.

Manufacturing
Once a new drug is FDA-approved, it can be manufactured for use. It’s important to ensure that a drug can be consistently and efficiently produced for as long as it’s needed, so manufacturing facilities must be considered carefully. Drug manufacturing companies are regulated by the FDA and held to the highest standards to ensure safety and quality in every step in the production of human pharmaceuticals.

Continued research
The FDA requires continued monitoring and safety reporting of any drug as long it stays on the market; any adverse events must be reported. Continued research may also be conducted to review additional uses for the drug, improved dosage forms and delivery systems, and potential use in combination treatments.

BY THE NUMBERS
- The FDA has approved more than 300 new medications in the past decade.
- Developing a new drug usually takes 10 to 15 years.
- Average cost, including failures, is estimated to be $1.2 billion per drug.
- For every 5,000 to 10,000 proposed new medications, only one makes it to approval.
- Only two out of 10 approved drugs are considered successful, with revenue that either matches or exceeds the cost of research and development.

NEW MEDICATIONS BY THE NUMBERS

ADDITIONAL RESOURCES
Innovation.org: www.innovation.org
Drug Discovery and Development
Pharmaceutical Research and Manufacturers of America: www.phrma.org
Clinical Trials: The Phases of Drug Testing and Approval
U.S. Food and Drug Administration: www.fda.gov
Investigational New Drug (IND) Application
New Drug Application (NDA)
New drugs are constantly being developed. Sometimes they’re created to treat a specific disease, while at other times an important use of a drug is discovered by accident. Regardless of how or why drugs are developed or discovered, they all must pass a series of tests and undergo a rigorous evaluation process by the Food and Drug Administration’s Center for Drug Evaluation and Research (CDER) before they’re made available to the public. This is because the CDER wants to make sure all new medications in the United States are safe and effective for human use.

Research and development

It takes an average of 12 years for a medication to make its way from the laboratory to the pharmacy. During the research and development process, all new drugs must pass through each of the following steps:

- Development by a drug sponsor
- Preclinical laboratory testing in animals
- Submission of an investigational new drug (IND) application from the drug sponsor to the FDA to begin testing the drug in humans
- Approval from the FDA to enter clinical trials
- Three phases of clinical trials (see page 4)
- Submission of a new drug application (NDA) from the drug sponsor to the FDA for final drug approval
- Drug label drafting and approval
- Inspection of the drug manufacturing facility
- Final approval from the FDA

Safety checks

At various stages during the approval process, a team of CDER doctors, statisticians, chemists, pharmacologists and other scientists is enlisted to scrutinize the medication. When a drug’s health benefits are found to outweigh its known risks, approval to move forward is granted. However, when issues arise, the process is delayed or even stopped. Some of the most common problems that prevent or delay a drug’s approval include:

- Unexpected safety issues
- Failure to demonstrate the drug’s effectiveness
- Failure to follow good manufacturing practices
- An inability to accurately mass-produce the drug (quality-control issues)

If one or more of these problems is identified, the CDER will send a letter to the drug sponsor to explain the issues. Upon receiving the letter, the drug sponsor can choose to meet with a CDER official for further discussion, ask for a hearing, correct the problem(s) and submit new information, or withdraw the application altogether.

Post-market monitoring

While the research and development process is extensive, it’s impossible to learn everything about a drug’s effects during that time period. Therefore, it’s critical to continue to monitor the drug’s safety and effectiveness after it’s approved and available for prescription.

The FDA’s post-marketing safety system is tasked with identifying any of these unexpected and/or adverse effects, which are usually detected in one of two ways:

- Through the periodic safety updates that the drug sponsor is required to submit to the FDA
- Through voluntary reports of adverse effects by doctors and/or patients to the FDA’s MedWatch system

When new effects are discovered, they’re usually added to the drug label, and the public is notified through letters, public health advisories or some other form of communication. In severe instances, the drug’s use is significantly limited, and in rare circumstances, the drug is pulled from the market.

ORPHAN DRUG STATUS

Because developing and marketing new drugs is an expensive undertaking, drug sponsors are sometimes hesitant to work on medications that will treat rare diseases (diseases that affect fewer than 200,000 people in the U.S.). This is because there’s little chance that the drug’s sales will cover the development and marketing costs, not to mention produce a profit.

In an attempt to reduce this profit risk, the FDA’s Office of Orphan Products Development (OOPD) can assign “orphan drug status” to medications that target rare diseases. This status makes it easier for the drug to gain approval, and other financial incentives for the drug sponsor may be available as well. Since implementing this program in 1983, the OOPD has facilitated the release of more than 400 medications for rare diseases. In contrast, fewer than 10 medications for rare diseases reached the market between 1978 and 1983.
Prior to their approval, new drugs being tested for their safety and effectiveness are called either “investigational” or “experimental” medications. In some cases, patients and their doctors may want to gain access to one or more of these types of medications before they’re officially approved by the Food and Drug Administration (FDA).

For example, a patient with a life-threatening disease who cannot be successfully treated with an existing FDA-approved drug may seek treatment with an experimental drug. Or, a patient experiencing severe side effects from an FDA-approved medication may be interested in switching to an investigational drug.

In circumstances such as these, patients can seek access to investigational drugs through one of two routes: clinical trials or the FDA’s expanded access program.

Clinical trials
Clinical trials are controlled studies of investigational drugs, and they’re a necessary part of every drug’s approval process (see page 7).

The main goal of clinical trials is to validate a drug’s safety and effectiveness, but they also provide information about a variety of other factors, including the drug’s associated side effects, correct dosages, contraindications (conditions or other drugs with which the investigational drug should not be mixed), and more. The results of clinical trials help the FDA decide whether to approve drugs and release them for public use.

Without patient participants, clinical trials would not be possible. And patients who participate are granted a number of benefits, perhaps most importantly, early access to potentially revolutionary new medications. By enrolling in a clinical trial, a patient gains access to a new medication not yet available to the public. He or she also plays a big role in advancing medical research and enjoys the very best standard of care.

Expanded access program
Because clinical trials often have very specific criteria that patients must meet in order to enroll (such as age and a lack of other health problems), not everyone is eligible to participate. To gain access to medications that haven’t yet been approved for public use, these patients may be eligible for the FDA’s expanded access program, which is sometimes called the “compassionate use” program.

Before a patient can gain access to an investigational medication through the expanded access program, all of the following factors must be established:
• The patient’s disease or disorder may benefit from treatment with the drug.
• The drug will not expose the patient to any unreasonable risks.
• The patient has no other satisfactory treatment options (e.g., an FDA-approved drug or an existing clinical trial).
• The manufacturer is willing to make the drug available for expanded access use.

If you’re interested in looking into clinical trials or the expanded access program, start by talking to your doctor or another member of your health care team to see if either might be a reasonable option for you.

QUICK ACCESS PROGRAMS

The FDA has a variety of programs that help speed the development, approval and availability of drugs that improve the current standard of care, treat serious or life-threatening diseases, and/or address an unmet medical need.

- **Priority Review Program:** If a drug shows the potential to significantly improve the safety or effectiveness of the current standard of care, it may qualify for the FDA’s Priority Review Program. The FDA is required to review applications on drugs in the priority review program within six months, compared to the 10 months it has to review applications under the standard review program. Priority review does not affect the length of the clinical trial period, but it can shorten the amount of time it takes a drug to reach the market.

- **Accelerated Approval Program:** While the standard review program requires absolute knowledge of a drug’s clinical benefit, the Accelerated Approval Program only requires early evidence that points toward the likelihood of the drug’s clinical benefit (called a surrogate or intermediate endpoint). For example, rather than waiting to see if a new cancer drug actually extends survival (clinical benefit), the FDA can approve it for that use based on evidence that the drug shrinks tumors (intermediate endpoint), since that effect is considered reasonably likely to lengthen survival. Using surrogate and intermediate endpoints can save a significant amount of time in the approval process for a qualifying drug.

- **Fast Track Program:** The goal of this program is to get important new drugs to patients more quickly than the standard, priority and accelerated review programs allow. To be designated as “fast track,” a drug must show promise in treating a serious or life-threatening condition and have the potential to address an unmet medical need. Under the fast track program, drug companies can submit their approval applications in pieces, rather than waiting for all of the information to become available (called a rolling review). The FDA receives approximately 100 to 130 Fast Track applications per year and approves approximately 80 percent of them.

- **Breakthrough Therapy Program:** This program is the newest of the quick access programs, and it targets drugs that not only treat a serious or life-threatening medical condition but also that, in early clinical trials, show a substantial improvement and/or clear advantage over currently available treatments. If a drug qualifies for the Breakthrough Therapy Program, the FDA will help guide the drug manufacturer through the most efficient drug development process, thereby helping the drug reach the market faster.

### ADDITIONAL RESOURCES

- **U.S. Food and Drug Administration:**
  - [www.fda.gov](http://www.fda.gov)
  - Access to Investigational Drugs
  - Speeding Access to Important New Therapies
- **U.S. National Library of Medicine:**
  - What is “Expanded Access”?

8  PatientResource.com
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