### Identifying Treatment

Investigational treatments may be appropriate if your patient has no available treatment, has exhausted all FDA-approved options, and you believe he or she might benefit from an investigational treatment. There are several ways to research investigational treatments.

### How to Research Possible Treatments

There are hundreds of investigational treatments currently in the pipeline for FDA review. Even if you follow drug development, it can be difficult to keep track of all the investigational treatments that might help your patient. If you do not know what investigational treatments exist for your patient’s condition, there are a few ways to start your research.

Primary literature, such as medical journals, and professional societies, including the American Society of Clinical Oncology (ASCO), are resources for the latest information on investigational treatments.

Patient advocacy groups are also a resource for patients and physicians beginning to explore treatments. Many of these groups provide information on investigational treatments and some offer clinical trial finder services.

The most comprehensive resource that you can use to find investigational treatments under development for your patient’s condition is ClinicalTrials.gov. ClinicalTrials.gov is a web-based resource, maintained by the National Library of Medicine at the National Institutes of Health, which serves as a registry of clinical trials and studies for a wide range of diseases and conditions. Information about clinical studies and expanded access is provided to ClinicalTrials.gov by the sponsor or investigator of the study.

For guidance on how to navigate the ClinicalTrials.gov website, refer to our ClinicalTrials.gov User Guide.

### Exploring Clinical Trials

Clinical trials can provide some patients with access to an investigational therapy for

### How Clinical Trials Provide Access to Investigational Therapies

Clinical trials help determine if a new investigational therapy works as well or better than therapies currently in use for a given medical condition. These trials weigh risks, which all
research purposes. You should consider clinical trials before pursuing single-patient expanded access.

therapies have, with benefits. Clinical trials are essential for the approval of a new therapy and a critical step toward bringing a therapy to market.

Often, it is a challenge to recruit enough patients for a clinical trial because patients are not aware of the option, or because they do not meet the eligibility requirements. Eligibility requirements may include age, gender, medical condition, treatment history, and/or use of other medications, so that the results are not confounded. These requirements ensure valid clinical results and also prevent enrollment of patients for whom the therapy may be harmful.

THINGS TO CONSIDER AND DISCUSS WITH YOUR PATIENT

Clinical trials may be an option for a patient who has exhausted all other approved therapies. Consider the following as you discuss possible clinical trial enrollment with your patient:

- Investigational therapies involve risk. The earlier in the drug development and review process, the less is known about the therapy. In fact, most investigational therapies do not ultimately receive FDA approval due to safety and/or efficacy concerns discovered during the clinical trial process.
- The clinical trial location may be a long distance from the patient’s home, requiring travel. The cost of transportation, childcare, meals, places to stay, and time away from work can be a financial burden for the patient.
- There may be other costs associated with participation in a clinical trial, and the amount covered by the trial sponsor will vary by trial. It is important for patients to speak with their insurer and the clinical trial sponsor to determine what costs will be covered and what costs they may be responsible for paying.
- A clinical trial follows a research protocol, which means an enrolled patient may or may not receive the investigational therapy and instead might receive the current standard of care, a placebo or no intervention at all. Additionally, the patient may be removed from a
If a patient decides to enroll in a clinical trial, he or she may be treated by a physician at the clinical trial site for medical care associated with the trial. The patient's current treating physician often is not involved in treatments related to the trial.

**NEXT STEPS FOR PURSUING A CLINICAL TRIAL**

If you and your patient decide that a clinical trial may be appropriate, you can obtain information about ongoing and enrolling trials on ClinicalTrials.gov. To determine your patient’s eligibility for a clinical trial, find the appropriate trial using ClinicalTrials.gov and review the enrollment and contact information within the record for more information on qualification.

If your patient is unable to participate in a clinical trial, keep reading for more information on expanded access programs (EAPs).

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<thead>
<tr>
<th>CONSIDERING EA PROGRAMS</th>
<th>HOW EA PROGRAMS PROVIDE ACCESS TO INVESTIGATIONAL TREATMENTS</th>
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<td>Expanded access programs (EAPs) may be an option if your patient is not eligible for (or unable to participate in) a clinical trial, or if you can’t find a trial for the investigational treatment you have identified.</td>
<td>Expanded access programs (EAPs) are coordinated by pharmaceutical companies and granted permission to proceed by the FDA. EAPs – often labeled as “intermediate” or “large size” – follow protocols with specific eligibility criteria. In some ways, EAPs are similar to clinical trials, but minimal data are collected and these programs are designed to accommodate patients who could not participate in a clinical trial. Sometimes, EAPs are opened to bridge the gap in time between the end of a clinical trial and the availability of a treatment in the marketplace, pending FDA approval. EAPs are not always offered for all investigational treatments.</td>
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### THINGS TO CONSIDER AND DISCUSS WITH YOUR PATIENT

Many of the considerations for assessing a clinical trial option also apply to intermediate or large size EAPs, including safety, efficacy, travel, and potential costs. An established EAP is fundamentally different from a clinical trial because there is no control group, therefore all patients receive the investigational treatment versus a placebo or the existing standard of care. Refer to the clinical trials section above to review these considerations and discuss them with your patient before pursuing an EAP.

### NEXT STEPS FOR PURSuing AN EAP

You can use ClinicalTrials.gov to identify relevant established EAPs and review eligibility requirements. Use our ClinicalTrials.gov User Guide to learn how to find EAPs on the website. You and your patient can both use contact information provided in the EAP record on ClinicalTrials.gov to get more information about the program.

If your patient is unable to participate in an intermediate or large size EAP, keep reading for more information on single-patient expanded access.

### CONSIDERING SINGLE-PATIENT EA

Single-patient expanded access may be an option if you and your patient have determined that neither clinical trials nor EAPs are appropriate or available for the investigational treatment you are seeking.

### HOW SINGLE-PATIENT EA PROVIDES ACCESS TO INVESTIGATIONAL TREATMENTS

Single-patient expanded access is a course of action to consider for your seriously ill patient if you have exhausted approved treatment options and ruled out clinical trials and EAPs as possibilities.

Under these circumstances, single-patient expanded access can be requested by you, the treating physician, on behalf of the patient. Pharmaceutical companies developing new treatments, as well as the FDA, require that such a request come from a licensed physician who will be overseeing treatment.
Single-patient EA requests can be made to the FDA for emergency and non-emergency situations. In an extremely time sensitive emergency situation where review from the FDA is needed urgently, such as in a matter of hours, contact FDA’s Division of Drug Information at 855-543-3784 or druginfo@fda.hhs.gov.

For non-emergency situations, continue using the Navigator for information on completing the single-patient expanded access application process.

**THINGS TO CONSIDER AS A PHYSICIAN SEEKING SINGLE-PATIENT EA**

If you are the licensed, treating physician of a patient considering single-patient EA, there are several considerations you will want to review before moving forward. The single-patient EA request process can be time consuming and you may not be reimbursed for time spent pursuing this option. For this reason, many physicians seeking single-patient EA enlist administrative support.

**THINGS TO CONSIDER AND DISCUSS WITH YOUR PATIENT**

It is critical that you have an open and honest discussion with your patient about the uncertainties and risks associated with investigational treatments. Investigational treatments are early in the development and review process, and therefore little is known about possible risks and benefits.

Additionally, because each single-patient EA request is unique to the individual, the time required to evaluate the request will vary. This evaluation and approval process involves the pharmaceutical company, the FDA, and applicable institutional review boards. There is no guarantee that the request will be approved or that access to the treatment will be granted.

Keep scrolling for more detailed information on the expanded access application process.
### REQUESTING EA

To request single-patient EA for your patient, you need to contact an appropriate representative at the pharmaceutical company that produces the identified treatment. Be prepared to answer a few questions and have patient information on hand.

### HOW TO REQUEST EA FROM A PHARMACEUTICAL COMPANY

You should request expanded access from a pharmaceutical company only after you have identified the desired investigational treatment and the company that offers it.

To make a request for single-patient expanded access, the treating physician must follow the instructions found on the appropriate company’s website or in the EA Navigator Company Directory. The request must come from the treating physician.

As the treating physician, you will be required to provide information, including but not limited to: patient diagnosis, medical and treatment history, and rationale to support a request for the investigational treatment. Sometimes, additional information will be requested by a representative at the company. Responding to these inquiries promptly will help the company to review your request efficiently and provide you with a decision as soon as possible.

The request submission process and subsequent reviews by the FDA and IRB can be time consuming.

### HOW PHARMACEUTICAL COMPANIES CONSIDER EA REQUESTS

There are several questions a pharmaceutical company may consider before providing access to an investigational treatment, including:

- Is the patient suffering from a serious or life-threatening illness?
- Is the patient eligible to enroll and able to participate in a clinical trial or EAP?
- Has the patient exhausted approved therapeutic options?
- Is the patient’s medical diagnosis/status appropriate for the investigational treatment?
- Is the requested investigational treatment approved for any indication in the country concerned (U.S.)?
• Are clinical data available to suggest that the potential benefits for this patient outweigh the risks of the investigational treatment?
• Is the physician appropriately licensed and qualified?
• Is the company able to supply the investigational treatment?
• Will providing access on a single-patient basis negatively affect the company’s ability to complete its clinical trials?

Upon receipt of the required information, the company will evaluate your request and contact you regarding its decision.

If your request is approved, the company will provide you with next steps including, but not limited to, treatment supply and physician requirements for treatment and reporting of adverse events.

WHAT THE PHARMACEUTICAL COMPANY PROVIDES

If the company grants your request, you will receive required documents such as a Letter of Authorization (LOA). You will submit a copy of this required documentation to the FDA and the Institutional Review Board (IRB) later in the application process in order to verify the company has granted a supply of the investigational treatment for use.

SUBMITTING TO THE FDA

If your request for single-patient EA is granted by the pharmaceutical company, you will work closely with the FDA to complete additional necessary paperwork. It is important to fully review this paperwork and to understand the responsibilities you will

HOW TO SUBMIT FORM FDA 3926

If a pharmaceutical company agrees to supply the investigational treatment for your patient, you must next request permission to proceed from the FDA. You will need to use Internet Explorer to open and electronically fill out Form FDA 3926, also known as the Individual Patient Expanded Access Investigational New Drug Application, OR you can see a sample of the form on the Navigator’s resources section here.

Form FDA 3926 is a two-page form that requests:

• A brief clinical history of the patient
have as the treating physician for single-patient EA.

| • The name of the treatment and the company that will supply the treatment |
| • The treatment plan, including dose, route of administration, duration of therapy, and any planned monitoring procedures |
| • Statement that you will obtain informed consent from the patient |
| • Commitment that you will receive approval from an Institutional Review Board (IRB) |
| • Information concerning your training, experience, and licensure, which can be fulfilled by attaching your curriculum vitae (CV) |
| • Signature to indicate you will fulfill all applicable regulatory requirements |

As the treating physician, you will submit Form FDA 3926 to the FDA along with required documents provided by the pharmaceutical company. Instructions for filling out Form FDA 3926 are available here. Contact information and procedures for the submission of Form FDA 3926 can be found here.

**HOW THE FDA REVIEWS YOUR APPLICATION**

The FDA has up to 30 days to review an Individual Patient Expanded Access Investigational New Drug Application. The treating physician may proceed with use of the investigational treatment after receiving FDA notification or, if no notification occurs, 30 days after the completed Form FDA 3926 is received.

In order to permit EA use to proceed, the FDA must determine that:

| • The patient has a serious or immediately life-threatening disease or condition |
| • There is no comparable or satisfactory alternative treatment available |
| • The potential benefit justifies the potential risks to the patient |
| • The potential risks are not unreasonable in the context of the disease or condition to be treated |
Providing the investigational treatment for the requested use will not interfere with the clinical investigations that could support marketing approval of the EA use

Authorizing the EA request does not otherwise compromise the potential development of the EA use

Some reasons why the FDA may not allow an EA treatment to proceed include:

- Nonclinical information deemed necessary for the study to safely proceed is missing
- Patients are or would be exposed to an unreasonable and significant risk of illness or injury
- Sufficient information is not provided for the FDA to assess the risk to the patient

If permitted to proceed, the physician will receive an IND number. The IND number may be requested by the IRB and is often requested by the company to permit shipping of the investigational treatment.

Continue scrolling for more information on securing IRB approval and the reporting requirements for single-patient expanded access.

### SEEKING IRB APPROVAL

After completing required steps with the FDA, the treating physician must work with an Institutional Review Board (IRB) to secure approval for the treatment protocol and informed consent form.

### HOW TO IDENTIFY AN INSTITUTIONAL REVIEW BOARD

Once the pharmaceutical company has agreed to provide the investigational treatment and you have submitted Form FDA 3926 to the FDA, you must submit an expanded access treatment protocol and informed consent document to an Institutional Review Board (IRB). IRBs monitor all research involving human subjects, including clinical trials. Treatment via single-patient EA also falls under their purview because of the investigational status of the treatment.

Many academic medical centers have their own IRBs, which can be used by the physicians who practice there. Alternatively, treating physicians can find an IRB through the HHS Registry of IRBs. There are several independent IRBs that are familiar with reviewing EA requests, including: Western
IRB, Quorum Review IRB, Chesapeake IRB, and Sterling IRB. For more information, questions or assistance with IRB procedures, visit the WCG Foundation.

THE ROLE OF AN IRB IN REVIEWING AN EA TREATMENT REQUEST

The role of an IRB is to protect and monitor patients receiving investigational treatments to ensure ethical treatment of patients. The IRB reviews informed consent documents to ensure that the patient is provided all the information about a protocol, including possible outcomes and risks. An explanation of procedures, potential benefits, possible risks, alternative treatments (if they exist), confidentiality of records, and what the patient’s rights are form the basis of a well-written informed consent.

Most IRB’s will request to also review Form FDA 3926 and the pharmaceutical company’s required documentation. At the onset of the IRB review process, you may want to ask the IRB about possible review fees and the anticipated timeline for review.

WHAT THE IRB WILL CONSIDER DURING THE REVIEW

Approval from the IRB must be attained before the EA treatment can begin. During the review process an IRB will look for:

- Assurance that informed consent and appropriate permissions from the patient will be obtained and documented
- Assurance that the physician’s treatment plan makes adequate provisions for ensuring the safety of the patient, including monitoring and appropriate plans for collecting and reporting data
- Confirmation that HIPAA requirements will be followed to ensure confidentiality of the medical record
- Confirmation that the treating physician will follow standard medical practice to protect the privacy interests of the patient
• Confirmation that additional safeguards are included in the treatment plan where the therapy may require special monitoring or other safeguards

After reviewing the request, the IRB will notify the physician as soon as possible in writing if the request is approved, requires more information, or is rejected.

The IRB approval letter also will stipulate requirements for reporting serious adverse events, any changes in the treatment plan, and a summary when the treatment ends.

### TREATING AND REPORTING

With all approvals and proper documentation in place, EA treatment can begin. First, the pharmaceutical company will provide the investigational treatment. Then you, the treating physician, will administer the investigational treatment. There are several reporting requirements to follow as you treat your patient.

### WHAT HAPPENS AFTER THE EA TREATMENT IS APPROVED

After receiving the necessary permissions from the FDA and approval from an IRB, the pharmaceutical company will ship the investigational treatment to you, the treating physician. The company may request confirmation of investigational treatment receipt and may also provide you with instructions on how to request resupply of the treatment, if applicable.

It is important to note that the length of time from initial EA request to treatment shipment varies and can take up to 2 or more months.

At this stage you, the treating physician, will be responsible for ensuring the investigational product is fully accounted for and that any remaining product is returned or destroyed at the end of the treatment use.

### REPORTING REQUIREMENTS DURING EA TREATMENT

As your patient undergoes treatment, you must follow the agreed upon treatment protocol. If there are any changes to the treatment plan you must notify the FDA, the pharmaceutical company, and the IRB.

You will also need to report serious and unexpected adverse events to the FDA in an IND safety report. Guidance on reporting is available to help you determine what is considered an adverse event, when to report these events, and to whom
they should be reported. To see a sample of an IND safety report click [here](#).

Once treatment has concluded, you must provide the FDA with a written summary of the results of the investigational treatment, including patient response, all adverse events, and treatment product disposition. If the treatment continues longer than a year, an annual report is required for each year the patient receives the investigational treatment.

If you have additional questions about reporting, you may contact FDA’s [Division of Drug Information](#) at 855-543-3784 or druginfo@fda.hhs.gov, or the FDA [Office of Health and Constituent Affairs](#) at 301-796-8460, or PatientNetwork@fda.hhs.gov.