Demystifying the Clinical Trial

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Phases of a Clinical Trial

Phase I: Is it safe?

Phase II: Does it work?

Phase III: Does it work better?

Phase IV: Are there long term side effects?
- The Guinea Pig
- The Placebo
- Cost of Care
- The Last Ditch Treatment
Who Conducts a Clinical trial?

- Primary Investigator (usually a MD)
- The research team: Doctors, Nurses and other health care clinicians, Research Study Assistants
- Clinical studies can be sponsored, or funded by various individuals/companies or organizations.
Finding a Clinical Trial

ClinicalTrials.gov is a database of privately and publicly funded clinical studies conducted around the world.

Explore 255,733 research studies in all 50 states and in 200 countries.

ClinicalTrials.gov is a resource provided by the U.S. National Library of Medicine.

IMPORTANT: Listing a study does not mean it has been evaluated by the U.S. Federal Government. Read our disclaimer for details.

Before participating in a study, talk to your health care provider and learn about the risks and potential benefits.

Search (all fields optional)

- Condition / Disease: e.g. breast cancer
- Other Terms: e.g., NCT number, drug name, investigator name
- Country: [Dropdown]

Find a study to participate in | Search all studies

Advanced Search

Help | Studies by Topic | Studies on Map | Glossary

Patients and Families
Search for actively recruiting studies that you may be able to participate in or learn about new treatments that

Researchers
Search the database to stay up to date on developments in your field, find collaborators, and identify unmet needs

Study Record Managers
Learn about registering studies and about submitting their results after study completion

Memorial Sloan Kettering Cancer Center
<table>
<thead>
<tr>
<th>#</th>
<th>Recruiting</th>
<th>Study Title</th>
<th>Conditions</th>
<th>Interventions</th>
<th>Locations</th>
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<tbody>
<tr>
<td>1</td>
<td></td>
<td>Long-term Outcome After Venetoclax / BRAF Inhibitors Interruption in</td>
<td>Erdheim-Chester Disease</td>
<td></td>
<td>Memorial Sloan Kettering Cancer Center, New York, New York United States</td>
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ECD Global Alliance website

Studies & Trials

5th Annual Patient & Family Gathering

Registration is now open! The 2017 Patient & Family Gathering will be located in New York City on October 27, 2017. Join knowledgeable physicians and other ECD affected families from around the world for this annual gathering.

LEARN MORE

ECD Trials/Studies

There are ongoing ECD trials/studies that are currently accepting ECD patients.

Even if you or your loved one is not located near a trial/study center, you or the treating physician can talk to one of the researchers involved in a trial/study, prior to treatment. This will allow you to be updated on the latest clinical trials evaluating ECD treatments.

There are currently clinical trials open for ECD treatment. Some of these are for ECD patients with BRAF V600 mutation. According to the early publications, the B-Raf enzyme inhibitor vemurafenib is showing promising results.

The advantages to a patient for entering a trial include:

1. The drug is provided free of charge to the patient. (In some cases, travel may be provided as part of the trial.
2. Follow-up appointments are typically provided. (Depending on the trial, patients may be followed on an outpatient basis or admitted to the hospital)
3. Sometimes if a patient is treatment-related and outside of a trial, the patient will most likely be eligible to enter a trial for that treatment at a later date.

Advantages to the ECD community as a whole when a patient is entered into a trial include:

1. Trials can lead to FDA approval for the treatment of ECD. With FDA approval, patients will be more likely to continue treatment for the ECD.
An Open Label Phase 2 Multicohort Trial of Nivolumab in Advanced or Metastatic Malignancies

Study Objective: The purpose of this study is to determine whether Nivolumab is an effective treatment for advanced or metastatic cancer. The study will evaluate the clinical benefit rate of Nivolumab in subjects at 1.5 weeks from enrollment. Various advanced or metastatic tumor types are eligible for enrollment. Subjects must have received prior standard of care treatment for their cancer before enrollment.

Sponsor: Bristol-Myers Squibb

Trial Contact Information: Fadi Braitel, 1-702-952-3400, fadi.braitel@usoncology.com

Requested Patient Involvement: ECD patients 18 Years and older

Known Centers Accepting ECD patients in the trial: Comprehensive Cancer Centers of Nevada ClinicalTrials.gov identifier: NCT02832167

A Safety, Tolerability and PK Study of DCC-2618 in Patients with Advanced Malignancies

Study Objective: This is a Phase 1 trial to investigate the safety and efficacy of the investigational drug, DCC-2618, administered orally (PO), in adult patients with advanced malignancies.

Sponsor: Deciphera Pharmaceuticals LLC

Trial Contact Information: Divya Sakamuri; 1-713-745-3296; dsakamuri@mdanderson.org or Filip Janku; 713-563-1930; FJanku@mdanderson.org

Requested Patient Involvement: ECD patients 18 years or older.

Known Centers Accepting ECD patients in the trial: MD Anderson, Houston, TX

ClinicalTrials.gov identifier: NCT02871036

A Study of DCC-2701 in Participants with Advanced Solid Tumors

Study Objective: The main purpose of this study is to investigate the safety and efficacy of the investigational drug DCC-2701 to help people who have advanced solid tumors or cancer that has spread to other parts of the body.

Sponsor: Deciphera Pharmaceuticals LLC

Trial Contact Information: Deeksha Vakhvaniltra, 713-563-1193, dvishwa@mdanderson.org or Filip Janku; 713-563-1930; FJanku@mdanderson.org

Requested Patient Involvement: ECD patients 18 years or older (preferentially with NTRK alterations) can participate.

Known Centers Accepting ECD patients in the trial: MD Anderson, Houston, TX

ClinicalTrials.gov identifier: NCT02228911

Anakinra or Denosumab and Everolimus in Advanced Cancer

Study Objective: The goal of this Phase I clinical research study is to determine the tolerable dose of the combination of Anakinra (everolimus) either with Lenirax (inaparin) or Xgeva (denosumab) for patients with
Steps of a Clinical Trial

• Discussion about Clinical Trial
• Eligibility and Screening
• Informed Consent
• Registration
• Testing
• Follow up
Steps of a clinical trial

- Discussion about trial as option
- Confirm eligibility
  - Trials may have specific pathologic criteria (i.e. BRAF mutation)
  - Lab values need to be within specific parameters
  - Past treatments may affect eligibility
  - Certain medications may exclude
  - Performance status “KPS”
- Informed consent
  - Informed Consent is a process
The Informed Consent Process

Discussion

Informed Consent Form & Research Authorization

Ongoing Information
Steps of a Clinical trial- Testing and Follow-Up

• Patient has the right to stop participation at any time and NOT affect ongoing care

• Study may include keeping side effect logs or pill diaries, extra blood tests “PK”s, imaging, or other safety measures (EKGs, eye exams)

• May include long term follow-up after completing treatment
Who pays?

Before enrolling talk to your insurance company and sponsor of the trial.

Sponsor coverage
- Investigational agent: the drug or equipment
- Research tests required by protocol
- Additional testing required by protocol

Patient responsibility /Insurance
- Doctor visits
- Lab tests
- Established treatment for ECD
- X-rays, MRIs and other imaging tests

For more information about cost:  www.cancer.gov
Is a Clinical Trial Right for Me??

Advantages
• Access to best care possible
• Receiving treatments before widely available
• Close monitoring
• Chance to play an active role in healthcare and research
• Helping future generations

Disadvantages
• Unknown risks and side effects
• Unknown benefit
• Frequent tests and visits
Summary:
Don’t be Afraid to Ask questions!
Many of the Answers will be found in the Consent

- How does the drug work?
- How often do I have to come to the hospital/clinic?
- How long will the study last?
- What expenses will I incur?