



Memorial Sloan Kettering  
Cancer Center

# Demystifying the Clinical Trial

Date October 27 2017

Presenters:

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# Phases of a Clinical Trial

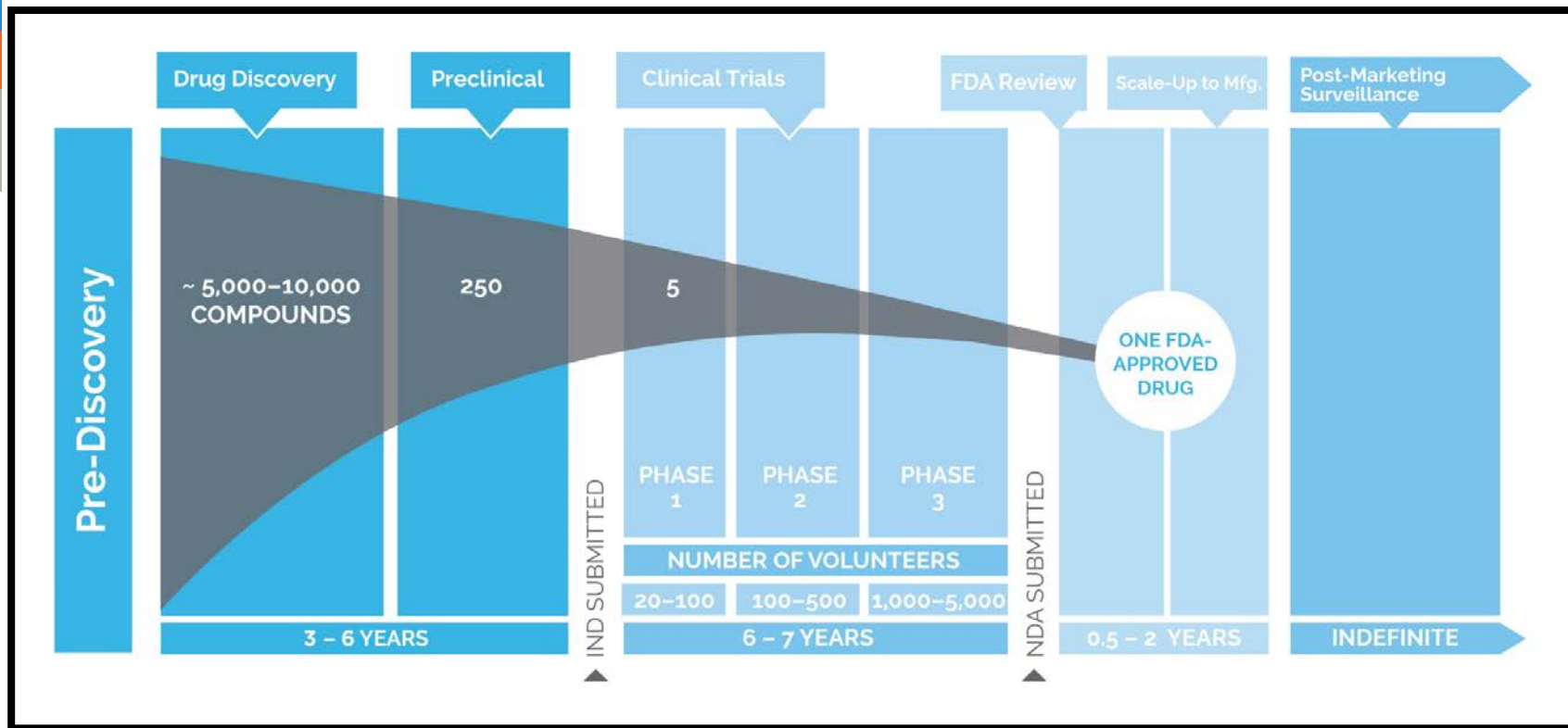
Phase I : Is it safe?

Phase II : Does it work?

Phase III : Does it work better?

Phase IV: Are there long term side effects?





Applied [clinicaltrials.com](https://www.clinicaltrials.com)



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# MYTHS

- 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

We updated the design of this site on September 25th. [Learn more.](#)

We will be updating this site in phases. This allows us to move faster and to deliver better services.

Show less ▲

 U.S. National Library of Medicine

**ClinicalTrials.gov**

[Find Studies](#) ▼ [About Studies](#) ▼ [Submit Studies](#) ▼ [Resources](#) ▼ [About Site](#) ▼ Saved Studies (0)

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

Explore 255,735 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:**

**Other Terms:**

**Country:**

[Find a study to participate in](#) [Search all studies](#)

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**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.



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6 Studies found for:  
Erdheim-Chester Disease | United States

List By Topic On Map Search Details

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Studies

Studies:

- ☐ Not yet recruiting
- ☐ Recruiting
- ☐ Enrolling by invitation
- ☐ Active, not recruiting
- ☐ Suspended
- ☐ Terminated
- ☐ Completed
- ☐ Withdrawn
- ☐ Unknown status†

Expanded Access:

Eligibility Criteria

Age:

years OR

Group:

- ☐ Child (birth–17)
- ☐ Adult (18–65)
- ☐ Senior (66+)

Sex:

- ☒ All
- ☐ Female
- ☐ Male

☐ Accepts Healthy Volunteers

Study Type

Study Results

Study Phase


Row	Saved	Status	Study Title	Conditions	Interventions	Locations
1	<input type="checkbox"/>	Recruiting	<a href="#">Long-term Outcome After Vemurafenib / BRAF Inhibitors Interruption in Erdheim-Chester Disease</a>	• Erdheim-Chester Disease		<ul style="list-style-type: none"> <li>Memorial Sloan Kettering Cancer Center New York, New York <b>United States</b></li> <li>AP-HP, Groupe Hospitalier Pitié-Salpêtrière Paris France</li> </ul>
2	<input type="checkbox"/>	Suspended	<a href="#">Dabrafenib and Trametinib in People With BRAF V600E Mutation Positive Lesions in Erdheim Chester Disease</a>	• BRAF V600E Mutation	<ul style="list-style-type: none"> <li>Drug: Dabrafenib Mesylate</li> <li>Drug: Trametinib Dimethyl Sulfoxide</li> </ul>	<ul style="list-style-type: none"> <li>National Institutes of Health Clinical Center, 9000 Rockville Pike Bethesda, Maryland <b>United States</b></li> </ul>
3	<input type="checkbox"/>	Recruiting	<a href="#">Clinical and Basic Investigations Into Erdheim Chester Disease</a>	<ul style="list-style-type: none"> <li>Myelofibrosis</li> <li>Gaucher Disease</li> <li>Pulmonary Fibrosis</li> <li>(and 2 more...)</li> </ul>		<ul style="list-style-type: none"> <li>National Institutes of Health Clinical Center, 9000 Rockville Pike Bethesda, Maryland <b>United States</b></li> </ul>
4	<input type="checkbox"/>	Recruiting	<a href="#">A Study of Lenalidomide for Adult Histiocyte Disorders</a>	<ul style="list-style-type: none"> <li>Langerhans Cell Histiocytosis (LCH)</li> <li>Histiocytoses Erdheim-Chester Disease</li> <li>Histiocytic Sarcoma (HS)</li> </ul>	• Drug: Lenalidomide	<ul style="list-style-type: none"> <li>Dana-Farber Cancer Institute Boston, Massachusetts <b>United States</b></li> </ul>
5	<input type="checkbox"/>	Recruiting	<a href="#">Uptake and Biodistribution of 18F-fluorocholine in Histiocytic Disorders by PET Imaging and Biopsy Measurement</a>	<ul style="list-style-type: none"> <li>Erdheim-Chester Disease</li> <li>Langerhans Cell Histiocytosis</li> <li>Histiocytic Disorders</li> </ul>	<ul style="list-style-type: none"> <li>Radiation: F-choline PET Scan</li> <li>Device: FDG-PET</li> <li>Procedure: biopsy</li> </ul>	<ul style="list-style-type: none"> <li>Memorial Sloan Kettering Cancer Center New York, New York <b>United States</b></li> </ul>
6	<input type="checkbox"/>	Recruiting	<a href="#">A Study of Memory, Thinking, and Brain Imaging in Adults With Histiocytosis</a>	• Histiocytosis	<ul style="list-style-type: none"> <li>Behavioral: Trail Making Test, Parts A &amp; B</li> <li>Behavioral: Brief Test of Attention</li> <li>Behavioral: Symbol Span</li> <li>(and 7 more...)</li> </ul>	<ul style="list-style-type: none"> <li>Memorial Sloan Kettering Basking Ridge Basking Ridge, New Jersey <b>United States</b></li> <li>Memorial Sloan Kettering Monmouth Middletown, New Jersey <b>United States</b></li> <li>Memorial Sloan Kettering Cancer Center @ Suffolk Commack, New York <b>United States</b></li> </ul>



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# ECD Global Alliance website

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## Studies & Trials

Home

### 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 are not located near a trial/study center, you or the treat talk to one of the researchers involved in a trial/study, prior to treatment. This will allow leading research in the fast changing world of ECD treatments.

There are currently clinical trials open for ECD treatment. Some of these are for ECD pc BRAF V600 mutation. According to the early publications, the B-Raf enzyme inhibitors a helping ECD patients.

The advantages to a patient for entering a trial include:

1. The drug is provided free of charge to the patient. (In some cases, testing and travel may also be provided as part of the trial. Patients should always ask what costs are included by the trial.)
2. Follow-up appointments are very thorough and side effects are closely monitored and treated. (Where there are known side effects of a treatment, the trial protocols are set up to take immediate action should these be encountered.)
3. Often times if a treatment is used off-label, outside of a trial, a patient will most likely be ineligible to enter 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, payers will be more likely to approve payment for the treatment.

### Our Organization

- Mission
- What we do
- Accomplishments
- Member Locations
- Policy
- Board/Staff
- Contact Us

### Community Connections

- Chat
- Chat Sessions
- Blogs
- Social Connections









#### [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 16 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 Braiteh, 1-702-952-3400, [fadi.braiteh@usoncology.com](mailto:fadi.braiteh@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](mailto:dsakamuri@mdanderson.org) or Filip Janku; 713-563-1930; [FJanku@mdanderson.org](mailto: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:** NCT02571036

#### [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 Vishwamitra, 713-563-1193, [dtvishwa@mdanderson.org](mailto:dtvishwa@mdanderson.org) or Filip Janku; 713-563-1930; [FJanku@mdanderson.org](mailto:FJanku@mdanderson.org)

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

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

**ClinicalTrials.gov Identifier:** NCT02228811

#### [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 Afinitor (everolimus) either with Kineret (anakinra) or Xgeva (denosumab) for patients with





# Steps of a Clinical Trial

- Discussion about Clinical Trial
- Eligibility and Screening
- Informed Consent
- Registration
- Testing
- Follow up

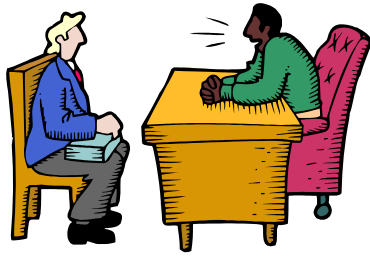


# Steps of a clinical trial

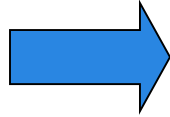
- Discussion about trial as option
- Confirm eligibility
  - Trials may have specific pathologic criteria (i.e.- BRAF mutation)
  - Lab values need to be within specific parameters
  - Past treatments may affect eligibility
  - Certain medications may exclude
  - Performance status “KPS”
- Informed consent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



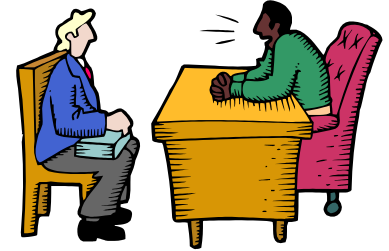
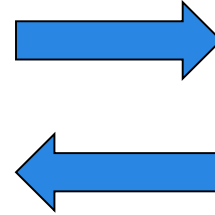
# The Informed Consent Process



Discussion



Informed  
Consent Form  
&  
Research  
Authorization



Ongoing  
Information



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# 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?

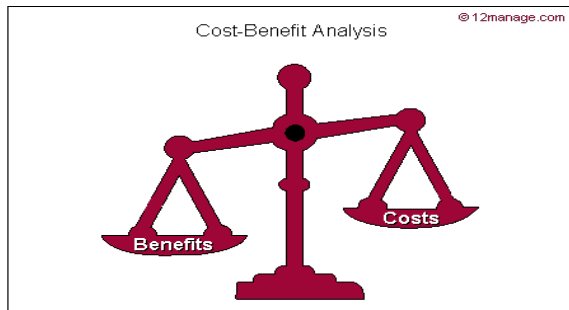
Before enrolling talk to your insurance company and sponsor of the trial.

## 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](http://www.cancer.gov)



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# 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



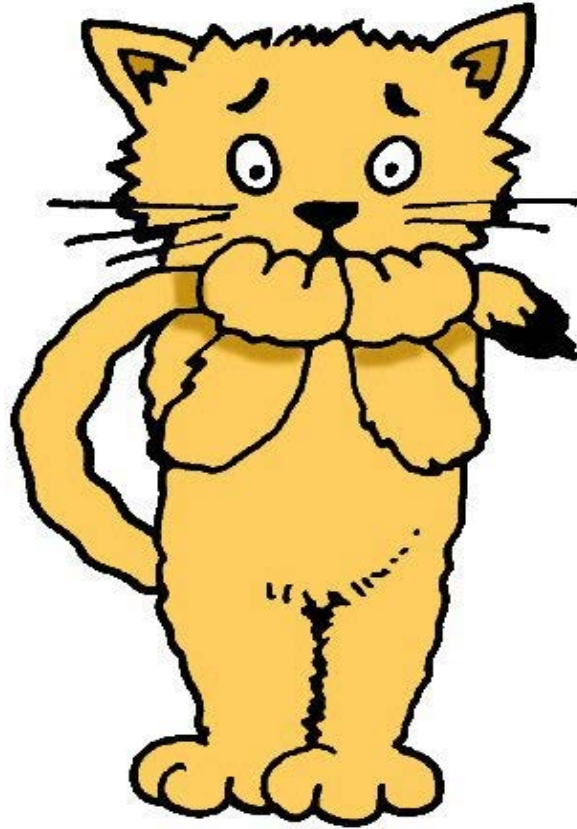


**SIGN ME  
UP!**



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## 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?



