CBCI
COLORADO BLOOD CANCER INSTITUTE
AT PRESBYTERIAN/ST. LUKE'S

Clinical Trials

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Objectives

- Define Clinical Trials/Research
- Why do we participate in clinical trials?
- Who can participate in a clinical trial?
- Ethical considerations of clinical trials
- Define the phases of clinical research
- How do we monitor safety/efficacy?
- What are the pros/cons?
- Where can one obtain trial info for WM?

What is a clinical trial?

- A clinical trial involves research using human volunteers and is intended to add to medical knowledge/advance treatment
  - Interventional
  - Observational

Why do we participate in clinical trials?

- To evaluate an intervention, such as a medication or medical device, for treating a certain disease
- To obtain information about the way diseases behave and why (genetics)
- To assess safety and toxicity in a group of patients
- To determine new drug combinations

Who can participate in a trial?

- There are standards outlining who can participate
  - Inclusion Criteria
    - Must be older than 18 years
    - Must have a diagnosis of Waldenstrom's Macroglobulemia
    - Must be symptomatic from the disease (ex: anemia, fatigue)
  - Exclusion Criteria
    - Cannot be pregnant
    - May not have GFR < 30 (kidney function)
    - May not have LFT > 2.5x ULN (liver function)
- Participant must provide informed consent

Ethical Considerations

- Informed Consent
  - Intended to protect participants and give him/her enough information to understand:
    - What is the purpose of the study?
    - Risks
    - Potential benefits
    - Alternatives to participating in the study
- Institutional Review Board (IRB)
  - 3rd party responsible for making sure the trial is ethical and safety is maximized
  - Advocates for the safety of the study participant
- Study participation is 100% voluntary
  - You can come off the study **at any time for any reason**

Phases of Clinical Trials

- Phase I:
  - Treatment is tested in a small group of people for the first time
    - To evaluate for safety and to determine a safe dose range
    - Evaluate/determine side effects
- Phase II:
  - Treatment is given to a larger group of people to look for effectiveness and further evaluate safety

Phases of Clinical Trials (cont.)

- Phase III:
  - The treatment is give to large groups to:
    - Confirm it's effectiveness
    - Monitor side effects
    - Compare to commonly used treatments
    - Collect information so the treatment can be used safely
- Phase IV:
  - Studies are done after the drug has been marketed/FDA approved to gather data in various populations and to determine safety in long term use

How do we monitor safety?

- Frequent assessment of side effects
  - Laboratory/radiology information
  - Patient reporting of symptoms
  - We follow the CTCAE (Common Terminology Criteria for Adverse Events) v4.0 guidelines.

Gastrointestinal disorders					
	Grade				
Adverse Event	1	2	3	4	5
Diarrhea	Increase of <4 stools per day over baseline; mild increase in ostomy output compared to baseline	Increase of 4 - 6 stools per day over baseline; moderate increase in ostomy output compared to baseline	Increase of >=7 stools per day over baseline; incontinence; hospitalization indicated; severe increase in ostomy output compared to baseline; limiting self care ADL	Life-threatening consequences; urgent intervention indicated	Death
Definition: A disorder characterized by frequent and watery bowel movements.					

How do we monitor efficacy?

- Endpoints are determined before the study starts
  - What research questions are being asked?
    - Example #1: Will this treatment improve progression free survival? (amount of time before the disease recurs)
    - Example #2: Will this treatment prolong overall survival? (does the treatment prolong life)
  - Data is gathered to assess disease response:
    - CT scans to measure the tumor size
    - Labs to look for change in IgM

What are the pros/cons?

Pros

- Access to a new treatment not currently FDA approved
- Contribute to science/gathering of new medical information
- Advance science/treatment becomes FDA approved
- More oversight from 3rd party (IRB, data monitoring committees)
- Closer evaluation by providers (more frequent visits/imaging)
- Medication may be provided free of charge

Cons

- May have to follow a more rigorous schedule
- More time intensive
- Less flexibility with schedule
- Treatment may have unknown/undiscovered side effects

Where can I find info re: clinical trials for WM?

- Your healthcare provider may have info, especially if they participate in research
- If he/she doesn't:
  - Consider obtaining a second opinion at a site that participates in clinical research
  - Look online at www.clinicaltrials.gov.
    - Search "Waldenstrom's Macroglobulemia"

ClinicalTrials.gov

A service of the U.S. National Institutes of Health

ClinicalTrials.gov is a registry and results database of publicly and privately supported clinical studies of human participants conducted around the world. Learn more about clinical studies and about this site, including relevant history, policies, and laws.

Find Studies | About Clinical Studies | Submit Studies | Resources | About This Site

ClinicalTrials.gov currently lists 207,627 studies with locations in all 50 States and in 191 countries.

Text Size

Search for Studies

Example: "Heart attack" AND "Los Angeles"

waldenstrom's macroglobulinemia | Search

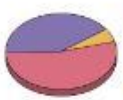
Advanced Search | See Studies by Topic

See Studies on Map

Search Help

- How to search
- How to find results of studies
- How to read a study record

Locations of Recruiting Studies



- Non-U.S. only (54%)
- U.S. only (41%)
- Both U.S. and non-U.S. (6%)

Total N = 37,340 studies
(Data as of January 31, 2016)

- See more trends, charts, and maps

For Patients and Families

- How to find studies
- See studies by topic
- Learn about clinical studies
- Learn more

For Researchers

- How to submit studies
- Download content for analysis
- About the results database
- Learn more

For Study Record Managers

- Why register?
- How to register your study
- FDAAA 801 requirements
- Learn more

Learn More

- Tutorials for using ClinicalTrials.gov
- Glossary of common site terms
- For the press
- Using our RSS feeds

HOME | RSS FEEDS | SITE MAP | TERMS AND CONDITIONS | DISCLAIMER | CONTACT NLM HELP DESK

76 studies found for: waldenstrom's macroglobulinemia | Open Studies

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List | By Topic | On Map | Search Details

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☑ Include only open studies ☐ Exclude studies with Unknown status

Rank	Status	Study
1	Not yet recruiting	**Expression of Ku70/XRCC6 in Waldenström's Macroglobulinemia** **Condition:** Waldenström Macroglobulinemia **Intervention:** Biological: Blood or bone marrow samples
2	Recruiting	**Phase 1/2 Dose Escalation Study in Patients With Relapsed or Refractory Waldenstrom's Macroglobulinemia** **Condition:** Waldenstrom's Macroglobulinemia **Intervention:** Drug: IMO-8400
3	Recruiting	**Study of Phosphatidylinositol-3-kinase (PI3K) Inhibitor Idelalisib (GS-1101) in Waldenström Macroglobulinemia** **Condition:** Waldenstrom's Macroglobulinemia **Intervention:** Drug: GS-1101
4	Recruiting	**Trial of Ixazomib, Dexamethasone and Rituximab in Patients With Untreated Waldenstrom's Macroglobulinemia** **Condition:** Waldenstrom's Macroglobulinemia **Interventions:** Drug: Ixazomib; Drug: Dexamethasone; Drug: Rituximab
5	Recruiting	**Study of Ibrutinib in Patients With Symptomatic, Previously Untreated Waldenstrom's Macroglobulinemia, and Impact on Tumor Genomic Evolution Using Whole Genome Sequencing**

- Q & A
- Thank you!