Clinical trials for Blood Cancers:

How can participating in a clinical trial help me?

In This Program You Will Learn

- The role of clinical trials for blood cancers
- When a clinical trial is a viable option
- The benefits of clinical trials
- The role of a patient and caregiver before, during, and after the process: Keith and Tricia’s story
- How LLS can help locate clinical trials that may be right for you
The role of clinical trials for blood cancers

Clinical Trials

- Cancer clinical trials are
  - Carefully controlled research studies
  - Conducted by doctors to improve the care and treatment of cancer patients

- The aim of a clinical trial is to
  - Study a new therapy or a new use for an already approved therapy
  - Compare a new treatment with a standard treatment to find out which one works better and/or has fewer side effects
  - Improve quality of life
  - Increase the length of survival or length of disease free survival;
Clinical Trials

- Ask about therapies being studied in a clinical trial
  - Ask if a clinical trial might be right for you
  - Ask about benefits and risks of both standard treatment and treatment in the clinical trial and how they differ
  - Ask where the trials are located

- There are risks and benefits in standard treatment and in clinical trials

- Ask about side effects of each treatment option and how these will be managed

Having more information will help you make decisions and manage challenges

Clinical Trials

A key step in drug development

Initial development of new drug in lab → Drug studied in lab

Food and Drug Administration approves new drug application

The drug can now be studied in people in carefully controlled clinical trials
Clinical Trials

How do clinical trials work?

**Phase I** investigates for safety and side effects, dosage and best way to give treatment—includes 20 or more people.

**Phase II** determines effectiveness and safety—typically includes fewer than 100 (but may include up to 300) people.

**Phase III** looks at effectiveness, side effects and safety in comparison with other treatments—including 100s to 1000s of people.

**Phase IV** gathers more information after FDA approval & drug is on market.

Clinical Trials

Talk to your doctor:

- About all of your treatment options
  - Standard treatment
  - Clinical trials

Ask as many questions as you have, until the answers are clear to you.
Clinical trial study design or protocol

- Each cancer clinical trial has a written detailed study design called a protocol that includes:
  - Why the clinical trial is needed
  - Purpose of the clinical trial
  - What drug or drug(s) are being tested, with a treatment and follow-up schedule
  - Safety measures throughout the clinical trial program
  - How outcomes will be measured
  - Who is eligible for the clinical trial
  - How the clinical trial will be organized, one site or multiple sites
    - If the clinical trial is a multi-site trial, all participating physicians must follow the same protocol

Clinical Trial Protocols

Clinical trial protocols ensure that patients are closely monitored

- Patients get a lot of attention and support
- Patients are watched closely by their doctor, as well as other members of their medical team, to ensure their safety
Safety in clinical trials

- Sponsor asks outside experts to review merit of study
- Institutional Review Board (committee of experts)
  - Looks at trial’s scientific, legal and ethical merit
  - Reviews whether risks are minimized and reasonable vs. anticipated benefits
  - Examines whether informed consent process is in place and documented (no coercion or “undue” influence to participate)
  - Investigates whether data monitoring includes patient safety data
  - Determines whether there is a process to protect privacy of patients

Process of informed consent

- Your doctor must give you an informed consent document before you enroll in a clinical trial
  - Must be in a language you understand
  - Ask for a language interpreter if needed
- Bring an advocate
- Ask your doctor to explain anything you don’t understand

Take your time in reading and signing the informed consent form. You may take back your consent to participate at any time.
Healthcare coverage and clinical trials

- It is important to find out what will be covered
  - Some costs may be covered by the sponsor of the study
  - Other costs may be covered by your health insurance plan
- Costs usually covered by the sponsor at no cost to the patient
  - Research doctor and nurse time
  - Administration of drug
  - Cost of drug being studied
  - Monitoring and testing specifically related to trial
- Costs that may not be covered by your private or public health insurance (costs you may incur with standard treatment or in a clinical trial)
  - Hospitalizations
  - Doctor visits
  - Drugs not part of the study design
  - Transportation, lodging and meals
- It is important to find out what will be covered
  - Contact an LLS Information Specialist for financial assistance resources at 800-955-4572 (e.g. LLS Co-pay Assistance Program)

When a clinical trial is a viable option
Clinical trials are for people at every stage of disease

Clinical trials provide patients either the best treatment currently available, or a new and possibly more effective therapy.

Clinical Trial Myths

- I can only join a clinical trial if I have exhausted all other options
  Clinical trials are available throughout the disease process

- Clinical trials are not safe and I will not benefit from them
  Process that starts in the lab and is regulated by FDA in the US

- I might get a placebo or a sugar pill instead of a real drug if I join a clinical trial
  Regulations require patients to know if placebo
  Placebos are rarely used in serious or life-threatening diseases

- Clinical trials are free
  The drug that is being studied is free
  However, the patient is responsible for standard of care therapy, admission to hospitals, physicians and other associated costs.
### Barriers To Enrollment

<table>
<thead>
<tr>
<th>Patient Barriers</th>
<th>Physician Barriers</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fear of being a “guinea pig”</td>
<td>Lack of awareness of opportunities</td>
</tr>
<tr>
<td>Misunderstanding the role of placebos in oncology</td>
<td>Insufficient time to research trials for all diseases</td>
</tr>
<tr>
<td>Lack of awareness of opportunities</td>
<td>“Losing” patient to another center</td>
</tr>
<tr>
<td>Complexity of access and stringency of trial participation</td>
<td>Lack of understanding about clinical trials</td>
</tr>
</tbody>
</table>

### The benefits of clinical trials
### Why Are Clinical Trials Important?

<table>
<thead>
<tr>
<th>Benefits</th>
<th>Risks</th>
</tr>
</thead>
<tbody>
<tr>
<td>Contribution to present and the future</td>
<td>Possibility treatment will be ineffective</td>
</tr>
<tr>
<td>Financial access to new treatments</td>
<td>Unknown side effects</td>
</tr>
<tr>
<td>Early access to new therapies</td>
<td></td>
</tr>
<tr>
<td>Delay standard therapy</td>
<td></td>
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<tr>
<td>Receive, at minimum, the best treatment available</td>
<td></td>
</tr>
<tr>
<td>Be among the first to benefit from a new treatment</td>
<td></td>
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<tr>
<td>Receive a lot of attention and support, including close monitoring to ensure safety</td>
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<tr>
<td>Have access to doctors with extensive experience in the type of cancer you have</td>
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</tbody>
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### Why Do Patients Enroll Into Clinical Trials

**Top participation reasons**

<table>
<thead>
<tr>
<th>Reason</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Help advance science, treatment of disease/condition</td>
<td>49%</td>
</tr>
<tr>
<td>Obtain better treatment</td>
<td>44%</td>
</tr>
<tr>
<td>Help others with same disease/condition</td>
<td>39%</td>
</tr>
<tr>
<td>Monetary compensation</td>
<td>29%</td>
</tr>
<tr>
<td>Study information read/seen/heard influenced</td>
<td>27%</td>
</tr>
<tr>
<td>Obtain edu. about treatment/improving health</td>
<td>22%</td>
</tr>
<tr>
<td>Obtain free medication, treatment</td>
<td>20%</td>
</tr>
<tr>
<td>PCP recommended study</td>
<td>18%</td>
</tr>
</tbody>
</table>

*Sample Size = 2,194, Base: Clinical trial participants. Red shaded cells indicate statistical significance within row at the 95% CI.*

*From CISCRP 2017 Participation and Insight Study*
Changing the Conversation About Clinical Trials

The Majority of Volunteers Would Participate in a Clinical Trial Again

![Survey Results](image)

<table>
<thead>
<tr>
<th>Year</th>
<th>Number of Patients Enrolled</th>
<th>Participation Rate</th>
<th>RACE</th>
<th>Somewhat Willing</th>
<th>Very Willing</th>
</tr>
</thead>
<tbody>
<tr>
<td>2015</td>
<td>1,152</td>
<td>34%</td>
<td>White</td>
<td>28%</td>
<td>56%</td>
</tr>
<tr>
<td>2017</td>
<td>1,914</td>
<td>54%</td>
<td>Black/African American</td>
<td>21%</td>
<td>78%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Asian</td>
<td>46%</td>
<td>38%</td>
</tr>
</tbody>
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Advancements in Clinical Trials

Gleevec ® (imatinib mesylate)

- 2013: FDA Approval (1,230 total patients)
- 2001: Phase II Trials Initiated (1,100 Patients Enrolled)
- 1999: Gleevec First Synthesized
- 1993: Gleevec First Synthesized

**Timeline:**
- 2014: 36,685 Living with CML
Advancements in Clinical Trials

Rituxan ® (rituximab)

- **First anti-CD20 Antibodies Produced**: 1991
- **Phase I Trial Initiated**: 1993
  - 20 Patients Enrolled
- **Phase II Trial Initiated**: 1993
  - 37 Patients Enrolled
- **Phase III Multicenter Study Initiated**: 1996
  - 166 Patients Enrolled

- **2014**: 739,928 NHL or CLL Survivors *Living with or in remission*

The role of a patient and caregiver before, during and after the process:

**Keith and Tricia’s story**
Keith and Tricia Rohleder’s Story

Blastic plasmacytoid dendritic cell neoplasm, (BPDCN)

- Blastic plasmacytoid dendritic cell neoplasm, or BPDCN, is a rare, aggressive blood cancer, with features of both lymphoma and leukemia.
- There are little data about BPDCN and there is no established treatment.
- Blastic plasmacytoid dendritic cell neoplasm (BPDCN) is very often misdiagnosed and under-reported. Diagnosing a patient with BPDCN is difficult.
- The skin is the most frequently involved site of disease (80 percent of cases). However, BPDCN usually progresses with bone marrow involvement and a decrease in red blood cell, white blood cell and platelet counts.
- Because the current treatments do not result in long term remission, patients need to consider clinical trials for new therapies. One example of a therapy that is currently in clinical trials is SL-401. SL-401 is a novel biologic targeted therapy and has shown significant antitumor effects.
How LLS can help locate clinical trials that may be right for you

CTSC
- Highly trained nurses specialized in hematological malignancies
- Provide education to patient
- Provide patient with a professional, detailed, individualized search to discuss with their HCP
- Provide guidance and advocates throughout the clinical trial process
- Personal connection

LLS Commitment: To Providing Resources To Help Patients Access Clinical Trials
Clinical Trial Support Center (CTSC)

- Get to know the patient
  - Reasons seeking treatment under a trial; Barriers to enrollment (financial, support);
    General level of understanding of diagnosis, treatment, and clinical trials

- Support and education
  - Help patients understand the clinical trial process including rights and obligations as
    a participant
  - Clinical trial phases, criteria, demands, trade-offs

- Clinical intake
  - Prior treatments/response, current physical condition and past medical history that
    may impact eligibility for certain trials

- Conduct the search through clinicaltrials.gov

Clinical Trial Support Center (CTSC)

- Provide list of trials that patient can discuss with their healthcare team
  - Healthcare team often contacts site and proceeds with next steps in enrollment

- If the treatment team cannot contact site, CTSC will guide the patient in their efforts
  to enroll in a trial including connecting the patient with trial sites

- Help address obstacles to enrollment such as travel and lodging expense

- Give patients the tools needed to make informed decisions

- Be available for support throughout your experience in the trial
Clinical Trial Support Center (CTSC)

To access the CTSC, call 1-800-955-4572

Discussion

If you are considering treatment in a clinical trial, what questions do you have about participating?
Where to Learn More About Clinical Trials

Speak one-on-one with an Information Specialist who can assist you through cancer treatment, financial and social challenges and give accurate, up-to-date disease, treatment and support information, as well as information about the Clinical Trial Support Center.

- Contact an LLS Information Specialist for details about our Clinical Trial Support Center (CTSC)

**How to contact us**
- **Call:** (800) 955-4572
  Monday to Friday, 9 a.m. to 9 p.m. ET
- **Email:** Infocenter@lls.org

Check out these Free LLS resources

- Clinical Trial Support Center (CTSC) - [www.LLS.org/ctsc](http://www.LLS.org/ctsc)
- Publications - [www.LLS.org/booklets](http://www.LLS.org/booklets)
- Podcasts - [www.LLS.org/podcasts](http://www.LLS.org/podcasts)
- Telephone/web education programs - [www.LLS.org/programs](http://www.LLS.org/programs)
- Videos - [www.LLS.org/educationvideos](http://www.LLS.org/educationvideos)
- Information Specialists - [www.LLS.org/informationspecialists](http://www.LLS.org/informationspecialists)
- Family Support Groups - [www.LLS.org/supportgroups](http://www.LLS.org/supportgroups)
- Moderated online chats - [www.LLS.org/chat](http://www.LLS.org/chat)
- Peer-to-peer support - [www.LLS.org/firstconnection](http://www.LLS.org/firstconnection)
- Patient Community - [www.LLS.org/community](http://www.LLS.org/community)
- Financial assistance - [www.LLS.org/finances](http://www.LLS.org/finances)
- Nutrition Consultation - [www.LLS.org/nutrition](http://www.LLS.org/nutrition)
- Your local LLS chapter - [www.LLS.org/chapterfind](http://www.LLS.org/chapterfind)

For information and to order materials, contact an LLS Information Specialist at (800) 955-4572 or visit [www.LLS.org](http://www.LLS.org)