Trial for Relapsed or Refractory Hairy Cell Leukemia (HCL) – information for patients

'Why did my HCL come back?'

HCL is a chronic disease, which often completely responds to purine analogs, like cladribine and pentostatin, but often minimal residual disease (MRD) remains, even after complete remission, and many believe that these residual HCL cells grow, usually over many years, and cause relapse.

'When should my HCL be treated again?'

It is generally agreed that MRD by itself does not need to be treated since many patients can live many years without symptoms. However, when the normal blood counts go down because of HCL growing in the bone marrow, it is often necessary to treat to prevent complications from infections and bleeding.

'What is being tested in this clinical Trial?'

The National Cancer Institute (NCI) is sponsoring a phase III trial of Moxetumomab Pasudotox. It is not and works differently chemotherapy. It selectively targets a protein called CD22, which is in high amounts on hairy cells. The drug contains a bacterial enzyme which kills the HCL cell after it binds and gets in.

'Will this drug be effective for me?'

In the recently published Phase I results in 49 patients (http://www.ncbi.nlm.nih.gov/pubmed/22355053 and https://ash.confex.com/ash/2013/webprogram/Paper64736.html), 57% achieved complete remissions, and none had severe toxicities which might be seen with chemotherapy. 88% of patients responded with improved blood counts. These results appear superior to other reported non-chemotherapy approaches for relapsed HCL. Our data also suggest that hairy cells carrying the BRAF V600E mutation are effectively treated with moxetumomab pasudotox. So, while you can't be sure the drug will be effective or safe for you, the effectiveness and safety profile of the drug has supported its testing in patients like you.

'Could I end up getting a placebo?'

No. Although this is a phase III trial, no-one gets a placebo. It is non-randomized and all will get treated.

'How can I tell if I'm eligible?''

The key eligibility criteria are:

- Hairy cell leukemia with at least two prior treatments
- Requiring treatment due to at least one factor:
  - neutrophils below 1000/mm^3
  - platelets below 100,000/mm^3
  - hemoglobin below 10 g/dL)
  - Pain from enlarged spleen

'Where do I need to go and is it expensive to participate?'

Three sites so far include the National Institutes of Health in Bethesda, MD, MD Anderson in Houston, TX, and The Ohio State University in Columbus Ohio. You will not need to pay for the moxetumomab pasudotox and many of the tests are covered as well. At the Bethesda location, patients on protocol have both travel from US locations and medical care covered as well.

'How can I enroll or get more information?'

Contact Dr. Robert J. Kreitman (rk21n@nih.gov) at 301-648-7375 or one of our protocol nurse coordinators, Laura Wisch, R.N. (Laura.Wisch@nih.gov) at 301-594-1827, Elizabeth Maestri, R.N. (maestrie@mail.nih.gov) at 301-402-5633, or Sandra Brinkman-Denny, R.N.(sandra.brinkman-denney@nih.gov) at 301-496-9458. Trial info is at: http://clinicaltrials.gov/show/NCT01829711
Dr. Kreitman's Website: http://ccr.cancer.gov/staff/staff.asp?profileid=5789
Trials: https://bethesdatrials.cancer.gov/clinical-trials-search-physician?field_investigator_name_value=kreitman&=Apply