Profile Feature as seen in Nature 20th October 2016
Founded in 1949, The Leukemia & Lymphoma Society (LLS) has invested more than $1 billion in research to advance treatments for blood cancers, such as acute myeloid leukemia (AML). Unfortunately, the standard treatment for the disease hasn’t changed much in the past four decades. Now, thanks to advances in gene sequencing, LLS is launching a master trial designed to test simultaneously a number of drug candidates developed by different companies in the hope of improving patient survival.

Amy Burd, a doctor of pharmacology, has been LLS’s vice president of research and strategy since 2009. She is leading the society’s new Beat AML initiative.

Q: What is the Beat AML initiative?

The Beat AML initiative involves a significant research effort called a master trial, involving multiple clinical sites. The master trial is designed to target newly diagnosed patients with AML, 60 years and older, for whom the standard of care has not changed in 40 years.

Q: How will this trial be conducted?

Most trials of AML drugs are in patients who have relapsed. However, the aim of this trial is to treat newly diagnosed patients. The patients we identify will provide a bone marrow sample, which will be sequenced using a next generation sequencing platform at Foundation Medicine, a company that provides genomic testing. Based on the patients’ genomic information, we’ll determine the appropriate treatment for their mutations. Patients who do not have a mutation that qualifies for one of the drugs in the master trial, will get a broad-acting agent – an epigenetic inhibitor or immunotherapy or another combination therapy. Thus, all patients in the study will receive a tailored treatment option.

Q: How long will the study take and how many patients will participate?

We expect the trial to last three to five years. Based on the mechanism of each of the drugs and the trial endpoints, some drugs will have longer-term efficacy than others. That means that with some drugs the endpoint may be progression-free survival at two years, meaning the disease has not become worse, while for others the endpoint will be complete response, meaning no evidence of the cancer, and the duration of that complete response. Our goal is to enroll between 250 and 500 patients. Each study arm will have around 25 patients, and based on the success of those 25 we will extend to between 60 and 100.

Q: Why is the LLS running this study?

In our view, LLS is the only cancer organization equipped to lead this study. We can bring together the Food & Drug Administration, academic researchers and pharmaceutical companies to make this trial successful. No one pharmaceutical company would hold an Investigational New Drug (IND) that would allow other drugs to be tested. LLS is holding the IND for this study which is unique for a non-profit organization.

Q: Did you have any trouble selling this idea to the research community?

All key stakeholders are very enthusiastic about the premise of this trial. There has been a recent increase in research in AML, which resulted in a litany of new targeted investigational therapies. So we brought together Drs. Brian Druker of Oregon Health and Science University, Ross Levine of Memorial Sloan Kettering Cancer Center and John Byrd of The Ohio State University to create this study. Our discussions with thought leaders in the AML field validated our thesis that the time was right to have a trial of this design. Moreover, our feedback from more than 25 pharmaceutical companies further reassured us about this concept. We are very heartened to see the level of collaboration this AML trial is engendering.

Q: You’ll also have to explain it to physicians and patients, won’t you?

Because this protocol is so unusual we’re going to do significant education outreach. We’re creating a lot of information on our website www.lls.org/beat-aml and people can contact LLS Information Resource Center at 1-800-955-4572.

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We think this trial represents a true paradigm shift for AML as well as for clinical trials across all types of cancer. It’s creating an infrastructure where we can test drugs quickly, determine if they’re working, and if they’re not we move on to something else. It’s a way of creating an efficient clinical trial network.