A Study of LB-100 in Patients With Low or Intermediate-1 Risk Myelodysplastic Syndromes (MDS)

CLINICALTRIALS.GOV IDENTIFIER	RECRUITMENT STATUS	FIRST POSTED	LAST UPDATE POSTED
NCT03886662	RECRUITING	MARCH 22, 2019	APRIL 8, 2019

STUDY DESCRIPTION

Brief Summary

The purpose of this study is to test the safety and efficacy (benefits) of an investigational drug LB-100, for treatment of myelodysplastic syndromes. LB-100 has previously been administered to patients with various solid tumors. In this study, LB-100 will be administered as an intravenous infusion over 120 minutes. This study will be conducted in 2 phases. In phase Ib, escalating doses of LB-100 will be administered to patients to study the safety and to determine a safe dose of LB-100. In phase 2, patients will be administered LB-100 at the dose that was found to be safe in phase Ib. The efficacy (benefits) and safety of LB-100 will be determined in this phase of the study.

Condition or Disease: Myelodysplastic Syndromes

Intervention/treatment: Drug: LB-100

Phase: Phase 1/Phase 2

DETAILED DESCRIPTION

N/A

STUDY DESIGN

Study Type:	Interventional
Estimated Enrollment :	47 participants
Intervention Model :	Single Group Assignment
Masking:	None (Open Label) ()
Primary Purpose:	Treatment
Official Title:	A Phase 1b/2 Study Evaluating the Safety and Efficacy of Intravenous LB-100 in Patients With Low or Intermediate-1 Risk Myelodysplastic Syndromes (MDS) Who Had Disease Progression or Are Intolerant to Prior Therapy
Estimated Study Start Date:	April 2019
Estimated Primary Completion Date:	May 2021
Estimated Study Completion Date:	July 2021

ARMS AND INTERVENTIONS

Arm	Intervention/treatment
Experimental: LB-100 for Intravenous administration Phase Ib: Escalating doses of LB-100 administered. Phase 2: Safe dose of LB-100 from phase Ib administered.	Drug: LB-100 Phase Ib: Two escalating doses of LB-100 in two separate cohorts will be administered intravenously on days 1, 3 and 5 of a 21-day cycle over 120 minutes. Phase 2: Safe dose of LB-100 as determined from phase Ib will be administered intravenously on days 1, 3 and 5 of a 21-day cycle over 120 minutes.

OUTCOME MEASURES

Primary Outcome Measures: 1. For Phase Ib - Number of patients with adverse events related to the study treatment as a measure of safety and tolerability of LB-100 study drug [Time Frame: From the first dose of the study drug to 30-days following last dose of the study drug]
Number of patients with treatment-related adverse events as assessed by Common Terminology Criteria for Adverse Events v5.0 (CTCAE v5.0)
2. For Phase 2 - Best overall response rate of patients to the study treatment as a measure of efficacy of LB-100 study drug [Time Frame: At screening and then at the end of Cycle 3 and Cycle 6. (Each cycle is 21 days)]
Best overall response rate of the patients to the study treatment as assessed by International Working Group (IWG) 2006 criteria

ELIGIBILITY CRITERIA

Ages Eligible for Study: 18 Years and older (Adult, Older Adult)

Sexes Eligible for Study: All

Accepts Healthy Volunteers: No

Criteria

Inclusion Criteria:
1. Patient has signed the Informed Consent Form (ICF) and is able to comply with protocol requirements.
2. Patient has adequate organ function as defined by the following laboratory values:
- Creatinine clearance (CrCl) ≥ 60ml/min
- Total serum bilirubin < 1.5 x Upper Limit of Normal (ULN) or total bilirubin ≤ 3.0 x ULN with direct bilirubin within normal range only in patients with well documented Gilbert's syndrome or hemolysis or who required regular blood transfusions - Alanine aminotransferase (AST) and aspartate aminotransferase (ALT) < 3.0 x ULN 3. Age ≥18 years at the time of signing the informed consent form. 4. Documented diagnosis of MDS or MDS/myeloproliferative neoplasm (MPN) by World Health Organization (WHO) criteria that require treatment due to cytopenias and meet the International Prognostic Scoring System (IPSS) criteria for low or int-1 risk. 5. For non-del(5q) patients, failed prior treatment with at least 2 cycles started of azacitidine or decitabine or lenalidomide defined as no response to treatment, loss of response at any time point while on treatment or within 6 months of treatment discontinuation, or progressive disease/intolerance to therapy. 6. For del(5q) patients, failed prior treatment with at least 2 cycles started of lenalidomide defined as no response to treatment, loss of response at any time point, or progressive disease/intolerance to therapy. 7. An Eastern Cooperative Oncology Group (ECOG) performance status score of 0, 1, or 2. 8. Women of child-bearing potential and men must agree to use adequate contraception (hormonal or barrier method of birth control; abstinence; tubal ligation, partner's vasectomy) prior to Cycle 1 Day 1 (C1D1) and for the duration of study participation. Should a woman become pregnant or suspect she is pregnant while participating in this study, she should inform her treating physician immediately. Exclusion Criteria: 1. Patient has a known history of HIV infection (testing not mandatory). 2. Patient has any of the following cardiac abnormalities: - symptomatic congestive heart failure - myocardial infarction ≤ 6 months prior to enrollment - unstable angina pectoris as designated by the treating physician - serious uncontrolled cardiac arrhythmia as designated by the treating physician - QTcF (Fridericia's correction formula) ≥ 450 msec 3. Concomitant malignancies or previous malignancies with less than a 1-year disease free interval at the time of enrollment. Patients with adequately resected basal or squamous cell carcinoma of the skin, or adequately resected carcinoma in situ (i.e. cervix) may enroll irrespective of the time of diagnosis. 4. Use of chemotherapeutic agents or experimental agents (agents that are not commercially available) for the treatment of MDS within 14 days of the first day of study drug treatment. 5. No concurrent use of erythroid stimulating agents, Granulocyte-colony stimulating factor (G-CSF), Granulocyte-macrophage colony-stimulating factor (GM-CSF) is allowed during study except in cases of febrile neutropenia where G-CSF can be used for short term. Growth factors must be stopped two weeks prior to study. 6. Pregnant women are excluded from this study because LB-100 has not been studied in pregnant subjects. Because there is an unknown but potential risk for adverse events in nursing infants secondary to treatment of the mother with LB-100, breastfeeding should be discontinued if the mother is treated with LB-100.

CONTACTS AND LOCATIONS

Contacts

Locations

United States, Florida	H. Lee Moffitt Cancer Center & Research Institute	Tampa

Sponsors and Collaborators

Lixte Biotechnology Holdings, Inc.

Investigator

Principal Investigator :	Rami Komrokji, MD	H. Lee Moffitt Cancer Center and Research Institute

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MORE INFORMATION

Responsible Party :	Lixte Biotechnology Holdings, Inc.
ClinicalTrials.gov Identifier :	NCT03886662
Other Study ID Numbers :	MCC-19635
First Posted :	March 22, 2019
Last Update Posted :	April 8, 2019
Last Verified :	April 2019
Studies a U.S. FDA-regulated Drug Product:	Yes
Studies a U.S. FDA-regulated Device Product:	No
Additional relevant MeSH terms :	*Preleukemia* *Syndrome* *Myelodysplastic Syndromes*