CLINICAL TRIAL FACT SHEET

ClinicalTrials.gov ID: NCT05651932

Study Protocol K36-MMSET-001: A phase I clinical trial of KTX-1001, an oral, first-in-class, selective MMSET catalytic inhibitor that suppresses H3k36me2 in patients with relapsed and refractory multiple myeloma

Description:

A clinical trial is a medical research study with people who volunteer to test scientific approaches to a new treatment or a new combination therapy. Each clinical trial is designed to find better ways to prevent, detect, diagnose, or treat cancer and to answer scientific questions. The K36-MMSET-001 study is designed to determine the maximum-tolerated dose (MTD) and safety profile of KTX-1001, an oral investigational drug for patients with myeloma who have limited treatment options.

Objectives:

- **The primary objective** of this study is to determine how well the study drug works, the dose and the schedule, and/or the recommended phase II dose (RP2D) and schedule of KTX-1001.
- **The secondary objective** of this study is to evaluate how KTX-1001 affects the body of the patient (called pharmacokinetics or PK).
- **The exploratory objective** of this study is to evaluate the relationships between biological markers and PK, safety, and activity of KTX-1001.

Design:

The K36-MMSET-001 study has 2 two parts. Patients will be enrolled into 1 of the parts.

1. **Dose escalation (Part 1)** – Approximately 30–40 patients will receive KTX-1001 at dose levels based on safety information collected during the study. The dose of KTX-1001 will increase as more is learned about how KTX-1001 affects the body.
2. **Dose expansion (Part 2)** – Approximately 20 patients will receive KTX-1001 at the dose determined to be the most tolerable during the dose escalation part of the study.

Inclusion criteria:

- 18 years of age or older.
- Relapsed or refractory multiple myeloma (RRMM) per diagnostic criteria of the International Myeloma Working Group (IMWG).
- At least 3 prior lines of therapy (combination or single-agent treatment regimens) including a proteasome inhibitor, immunomodulatory agent, or anti-CD38 monoclonal antibody.
- Measurable disease, including at least 1 of the following:
  a. Monoclonal protein (myeloma protein or M-protein) ≥ 0.50 g/dL in the serum (liquid part of blood in which the blood cells are suspended).
  b. M-protein ≥ 200 mg (by 24-hour collection) in the urine.

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International Myeloma Foundation
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c. Serum free light chain (sFLC) involved light chain ≥ 10 mg/dL or 100 mg/L if sFLC ratio is abnormal.

d. ***For dose escalation (Part 1) only:*** At least 1 extramedullary lesion (tumor of monoclonal plasma cells in the soft tissue outside of the bone marrow and separate from bone), including at least 1 lesion that is 1 cm in size or larger.

e. ***For dose escalation (Part 1) only:*** Bone marrow plasma cells 10% or more.

- ***For dose expansion (Part 2) only:*** Must have t(4;14) genetic abnormality in which in which parts of chromosomes 4 and 14 are translocated (rearranged), written with a lowercase "t" followed by the numbers of the chromosomes with translocated genetic material.

Study schedule:

- Screening period of up to 28 days.
- Treatment cycles will be 28 days. Participants will take KTX-1001 orally, twice daily at home.
- Pharmacokinetics (PK) and pharmacodynamics (PD, the body's biological response) samples will be collected several times during Cycle 1, then at one time during Cycle 2 and beyond.
- Dose escalation (Part 1) patients will be evaluated for dose-limiting toxicity (DLT), side effects severe enough to prevent giving more of the treatment during Cycle 1.
- Dose expansion patients will receive a dose level already evaluated during the dose escalation phase to further define the safety and tolerability of KTX-1001 and provide preliminary efficacy information.
- End-of-treatment visit.
- Post-treatment visit 30 days after the last dose of KTX-1001.
- Follow-up visits by phone call every 3 months for us to 2 years.

Clinical trial participants will go to the study site weekly during Cycle 1, then every other week for Cycle 2, followed by visits on Day 1 of each subsequent cycle until the end-of-treatment visit.

Informed consent:

A doctor must give a patient the information about a clinical trial so that the patient can make an informed decision about whether to participate in the study. In addition, the doctor must explain all procedures, and address the issues of risks and benefits, and treatment alternatives.

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Locations enrolling patients:

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