CLDK378X2103: A Phase I, Open-Label, Dose Escalation Study of LDK378 in Pediatric Patients with Malignancies that have a Genetic Alteration in Anaplastic Lymphoma Kinase (ALK).

PURPOSE: The purpose of this study is to estimate the maximum tolerated dose of LDK378 as a single agent, assess safety, tolerability and anti-tumor activity and characterize single and multiple-dose pharmacokinetics when administered orally to pediatric patients with ALK-activated tumors.

Study Type: Interventional
Masking: Open Label
Primary Purpose: Treatment

Detailed Description:
LDK378 is a novel inhibitor of ALK that is active in a broad range of ALK-activated tumor models, including models driven by mutated versions of ALK known to be resistant to crizotinib, and by ALK gene amplification.

The primary purpose of this study is to determine the maximum tolerated dose and/or recommended dose for expansion in pediatric patients, and to delineate a clinical dose to be used in any future pediatric studies. This study will also assess the safety, tolerability, PK and preliminary evidence of antitumor activity of LDK378 in pediatric patients with neuroblastoma, and other ALK-activated tumors.

AGES ELIGIBLE FOR STUDY: 12 Months to 17 Years

Criteria
Inclusion Criteria:

- Diagnosed with a locally advanced or metastatic malignancy that has progressed despite standard therapy, or for which no effective standard therapy exists
- Age ≥ 12 months and ≤ 17 years
- The tumor must carry a genetic alteration of ALK
- Patients must have evaluable or measurable disease

Exclusion criteria:

- Symptomatic central nervous system (CNS) metastases who are neurologically unstable or require increasing doses of steroids or local CNS directed therapy (such as radiotherapy, surgery or intrathecal chemotherapy) to control their CNS disease
- Clinically significant, uncontrolled heart disease
- Inadequate end organ function as defined by specified laboratory values
- Use of medications that are known to be strong inhibitors or inducers of CYP3A4/5 that cannot be discontinued at least 1 week prior to start of treatment with LDK378 and for the duration of the study
- Use of medications that are mainly metabolized by CYP3A4/5 or CYP2C9 that cannot be discontinued at least 1 week prior to start of treatment with LDK378 and for the duration of the study.

For more information contact:

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