Extension to the Study of Efficacy of CDZ173 in Patients With APDS/PASLI

ClinicalTrials.gov Identifier: NCT02859727

Brief Summary:This study is designed to provide long-term CDZ173 treatment, a selective PI3Kδ inhibitor, to the patients with genetically activated PI3Kδ, i.e., patients with APDS/PASLI who participated in the CCDZ173X2201 study or who were treated previously with PI3Kδ inhibitors other than CDZ173.

The study is open-label designed to establish the long-term safety, tolerability, efficacy and pharmacokinetics of CDZ173 in the target population.

Outcome Measures:

- To evaluate the long term safety and tolerability of CDZ173 in patients with APDS/PASLI [Time Frame: 3 years 3 months]All safety parameters (including AEs, physical exam, vital signs, ECG, safety laboratory (hematology, blood chemistry, urinalysis))

Secondary Outcome Measures

- To evaluate the long term efficacy of CDZ173 to modify health-related quality of life in patients with APDS/PASLI [Time Frame: 3 years]
  - SF-36 (Short Form 36) Survey and WPAI-CIQ (Work Productivity Activity Impairment plus Classroom Impairment Questionnaire), Visual analogue scales for Physician's Global Assessment (PGA) and Patient's Global Assessment (PtGA), patient narratives by Investigator
- To evaluate the long term efficacy of CDZ173 by means of biomarkers reflecting the efficacy of CDZ173 to reduce systemic inflammatory components of the disease in patients with APDS/PASLI [Time Frame: 3 years 3 months]
  - High sensitivity C-reactive protein (CRP), lactate dehydrogenase (LDH), frequencies of infections and other disease complication
- To characterize the pharmacokinetics (trough concentration) of CDZ173 in patients with APDS/PASLI [Time Frame: 9 months]Steady-state trough concentration of CDZ173
- To evaluate the pharmacokinetics and relative bioavailability of CDZ173 film-coated tablets compared to CDZ173 hard-gelatin capsules [Time Frame: up to 6 months]
  - PK parameters (including but not limited to AUC0-12,ss and Cmax,ss)

lInformation from the National Library of Medicine
To learn more about this study, you or your doctor may contact the study research staff using the contact iinformation provided by the sponsor.

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