**INTRODUCTION**

- Colony stimulating factor 1 receptor (CSF1R) is a receptor tyrosine kinase that is implicated in the pathogenesis of a broad spectrum of tumors associated with inflammation (e.g., melanoma, glioblastoma, breast cancer).
- CSF1R has emerged as a potential target for the treatment of malignancies with high macrophage activity.
- DCC-3014 is an orally administered, potent, and selective CSF1R inhibitor.
- Cohort 1 was initiated at a dose of 10 mg once daily.
- Cohort 2 was initiated at a dose of 20 mg once daily.
- Cohort 3 was initiated at a dose of 30 mg once daily.

**METHODS**

- This is a phase 1b, multicenter, open-label, dose escalation trial.
- Patients were randomized to receive the recommended phase 2 dose (RP2D) and the maximum tolerated dose (MTD) from Cohort 3.
- Cohort 4 was a single-arm study in patients with recurrent/malignant tumors.
- Cohort 5 was a single-arm study in patients with type TGCT.
- Cohort 6 was a single-arm study in patients with type TGCT.

**RESULTS**

- **Safety**
  - Among treatment-emergent adverse events (TEAEs) occurring in 71% (502/700) of patients (95% CI of patients at risk of 66%–76%) in phase 1 (Cohorts 1–3).
  - No grade 5 TEAEs were reported in 71% of all patients.
  - No grade 5 adverse events (AEs) were reported in 71% of patients.
  - No grade 5 pharmacodynamics (PD) study events were reported in 71% of patients.

- **Pharmacodynamics**
  - Week 5 (12.8): one patient had a confirmed partial response by cycle 3; sustained for 9 months and ongoing as of last investigator report.

- **Pharmacokinetics**
  - Week 5 (12.8): one patient had a confirmed partial response by cycle 3; sustained for 9 months and ongoing as of last investigator report.

- **Case studies**
  - **Patient 1**
    - 24-year-old female patient diagnosed with diffuse-type TGCT right posterior knee in June 2018.
    - Recurrence/regrowth on MRI by Dec 2018.
  - **Patient 2**
    - Symptom improvement/tumor assessment on the study.
  - **Patient 3**
    - Current activity in Cycle 5.

**CONCLUSION**

- DCC-3014 is an orally administered, potent, and selective CSF1R inhibitor.
- DCC-3014 treatment caused a dose-related increase in plasma CSF1 and IL-34 and a reduction of CD16+ monocytes in diffuse-type TGCT patients.
- Patients with type TGCT were reported to have PK/PD and pharmacokinetics (PK) profiles in diffuse solid tumors, including diffuse-type TGCT.
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**References**

- New Orleans, LA.
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