

This abstract was submitted to DTRF for the September 2022 Int'l Research Workshop.

Initial Results of Phase 2/3 Trial of AL102 for Treatment of Desmoid Tumors (DT)

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Background: AL102 is a potent, orally available, selective gamma secretase inhibitor (GSI) under investigation as an antineoplastic agent. GSIs have antitumor activity against DT, including AL101 (2 confirmed PRs >3.5 years and >1 year; 1 stable disease for 1 year). There remains an unmet need for effective systemic therapy in DT, associated with durable tolerability, tumor regression, and symptom improvement.

Methods: RINGSIDE is a Phase 2/3 randomized study in patients with progressing DT. Part A (Phase 2) is an open-label part with safety as the primary endpoint. Patients are randomized to three dosing regimens: 1.2 mg QD, 2 mg intermittent BIW (2 days on 5 days off), or 4 mg intermittent BIW. Part B is a Phase 3 double-blind, placebo-controlled study with PFS as primary endpoint. The dose for part B will be determined based on the totality of data collected in part A.

Results and Conclusions: Safety, efficacy, PRO, PK, and biomarker results from Part A of the study will be presented at the meeting.