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Synopsis of a Planned Phase 1/2 Study of Tegavivant (BC2059) in desmoid tumors

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TBL1 is a recently discovered protein that binds to free cytoplasmic beta catenin and escorts it to the nucleus where it protects beta catenin from degradation. The small molecule BC2059 (Tegavivant) inhibits the binding of beta catenin to TBL1 and thus decreases the amount of nuclear beta catenin. As nuclear beta catenin is a hallmark of desmoid tumors, Beta Cat Pharmaceuticals plans a phase 1 trial in that disease beginning in the first quarter of 2018. The trial will be an open-label, non-randomized study to evaluate the safety of BC2059 administered intravenously to subjects with proven primary or recurrent desmoid tumor that is unresectable and symptomatic or progressive. Single patient cohorts will be utilized for the first two dose levels followed by a conventional 3+3 dose escalation phase to achieve the maximally tolerated dose (MTD) or recommended phase 2 dose level (RP2D). Once MTD or RP2D is determined, dose expansion will begin with recruitment of a total of 14 subjects enrolled to assess additional safety PK/PD data and preliminary efficacy. If at least 1 patient has clinical benefit, 11 patients will be recruited (25 total in dose expansion). Early cohorts will be allowed to dose escalate later so that all enrolled patients can be included at the RP2D.