Pilot Study of Pemtrexed for the Treatment of Chordoma

Official Title: Pilot Study of Pemtrexed for the Treatment of Chordoma

This is an open-label, single-arm, pilot study of pemtrexed for the treatment of patients with chordoma. The study is designed to assess the safety profile and response rate of high-dose pemtrexed and to collect biological specimens before, during, and after treatment to identify potential biomarkers of response and safety.

Pemtrexed is a folate analog metabolic inhibitor that disrupts folate-dependent metabolic processes essential for cell replication. Pemtrexed inhibits three enzymes used in the biosynthesis of thymidine and purine nucleotides—thymidylate synthase (TS), dihydrofolate reductase, and glycinamide ribonucleotide formyltransferase (GARFT).

Chordomas are rare brain and bone tumors that can occur anywhere along the spine. Surgery and radian therapy are currently used as treatment, as there are no drugs at this time that are approved for treating this cancer type. The current research is being done to determine whether pemtrexed can be used safely and to learn more about how pemtrexed might affect the growth of your chordoma.

Key Inclusion Criteria:
- Diagnosis of chordoma
- Age ≥18
- KPS score of ≥50

Key Exclusion Criteria:
- Active bacterial infection requiring intravenous antibiotics
- Fungal infection or detectable viral infection