A Proof-of Concept PK/PD Study of BXQ-350 in Cancer Patients

Exposed to Oxaliplatin or Taxane-Based Chemotherapy

 A proof-of-Concept Study Investigating BXQ-350’s CIPN Mitigation Properties in Cancer Patients with Established Chemotherapy Induced Peripheral Neuropathy

Background:

Quality of life (QoL) for cancer patients is negatively impacted in many ways. One of the leading syndromes that afflicts QoL is chemotherapy-induced peripheral neuropathy (CIPN). Many chemotherapeutic agents induce CIPN, including oxaliplatin and taxanes. There is currently no treatment for CIPN which damages the peripheral nerves and is a well described clinical problem that may impact clinical outcome.

BXQ-350 was investigated in a Phase 1 dose-escalation study in cancer patients (NCT02859857); results showed that it was well-tolerated with signs of single agent activity. Additionally, patients with established CIPN at the time of enrollment reported improvements of their symptoms shortly after receiving BXQ-350. The properties of BXQ-350 towards mitigating CIPN effects were investigated preclinically *in vitro* and *in vivo*; results showed that BXQ-350 stimulated neurite growth, protected neuronal cells from the cytotoxic effects of multiple chemotherapeutics, and reduced thermal and mechanical allodynia in a preclinical oxaliplatin-induced CIPN model.

Method:

A randomized, placebo controlled, double blind proof of concept study entitled “A Pilot Proof of Concept Pharmacokinetic/Pharmacodynamic Study of BXQ-350 in Cancer Patients Exposed to Oxaliplatin and/or Taxane Based Chemotherapy”has been initiated (NCT05291286). The primary objectives of this study in cancer patients with existing CIPN due to previous treatments with oxaliplatin or taxanes are: i) to assess PK/PD relationships with response to study treatment by investigating PK plasma concentrations and biomarkers associated with CIPN including but not limited to ceramides, circulating cytokines, and sphingosine-1-phosphate; ii) to determine if BXQ-350 mitigates CIPN symptoms thereby improving QoL using the European Organization for Research and Treatment of Cancer Quality of Life Questionnaire (EORTC QLQ-C30)and total sensory neuropathy scores deduced from the EORTC QLQ-CIPN20 questionnaire. Additional safety parameters will also be collected to further ascertain the tolerability of BXQ-350.

The planned sample size is20 participants (10 with CIPN from oxaliplatin, 10 from taxane-based chemotherapy). This trial is currently enrolling at one US site; as of November 2022, two participants have been enrolled.