Results from Ongoing Phase 1/2 Clinical Trial of Tagraxofusp (SL-401) in Patients with Intermediate or High Risk Relapsed/Refractory Myelofibrosis

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Introduction and Highlights

Tagraxofusp

- Novel targeted therapy directed to CD123
- FDA-approved for the treatment of adult and pediatric patients, 2 years and older, with blastic plasmacytoid dendritic cell neoplasm (BPDCN), and monoblastic leukemia
- Marketing Authorization Application (MAA) for BPDCN granted accelerated assessment, and under review by the FDA

BPDCN

- Explored by multiple investigators, including several myeloproliferative neoplasms (MPN) such as chronic myelomonocytic leukemia (CMML), and myelodysplastic syndromes
- CD123+ extracts are elevated in BPDCN

Tagraxofusp and MF

- Tagraxofusp is a recombinant human IL-12/23 receptor antagonist monoclonal antibody, demonstrated clinical activity, with a predictable and manageable safety profile, in patients with myelofibrosis/MF
- Including post-MPL patients with monocytes, areas of cellular necrosis

Patient enrollment

- Based on these encouraging results, next steps for the programs are being evaluated including single agent, combination, and registration-directed trials in myelofibrosis/MF

Targeting MF via a CD123-directed therapy may offer a novel approach for treatment of these patients.

Background: Myelofibrosis (MF)

- MF is a BCR-ABL negative myeloproliferative neoplasm characterized by clonal hematopoiesis, including a myelofibrosis component, and increased risk of leukemia

Safety and Tolerance

- Predictable and manageable safety profile
- No apparent clinical activity change within the bone marrow, over multiple cycles

Clinical Activity Overview

- 6 patients with anemia, 12/24+ months
- 4 patients with thrombocytosis, 12/24+ months
- 4 patients with monocytosis, 12/24+ months
- 6 patients with dysplasia, 12/24+ months
- 5 patients with secondary ANemia

Spleen Responses in Patients with MF, including...