FSGS 1343

Ongoin: active inclusion

S-nummer: S66448 Phase IIa study
Principal Investigator: prof. dr. Björn Meijers

Title: A multicenter, randomized, double-blind, parallel group, placebo-controlled study to assess the efficacy, safety, tolerability, pharmacokinetics and pharmacodynamics profile of BI 764198 administered orally once daily for 12 weeks in patients with focal segmental glomerulosclerosis.

Purpose and rationale: The aim of the study is to explore the efficacy of 3 doses (20 mg, 40 mg, and 80 mg) of BI 764198 with respect to their benefit to lower proteinuria. Additionally, the safety, tolerability, and pharmacokinetic and pharmacodynamic profiles of BI 764198 vs placebo will also be evaluated.

Primary endpoint: Patients achieving at least 25% reduction in 24- hour urine protein-creatinine ratio (UPCR) relative to baseline at week 12.

Medication/treatment: BI 764198 (TRPC6 inhibitor). 20 mg, 40 mg, 80 mg or matching placebo taken orally once daily. (Randomization 1:1:1:1)

Duration of study: Approximately 5 months, consisting of 11 visits of which 4 via a telephone call.

Key inclusion criteria:
- Male and female patients between the age of 18 and 75
- Diagnosed with biopsy proven primary FSGS or documented TRPC6 gene mutation causing FSGS
- UPCR ≥ 1000 mg/g
- Corticosteroids, ACE inhibitors, ARBs, finerenone, aldosterone inhibitors, or SGLT2 inhibitors must be on a stable dose for at least 4 weeks prior to screening

Key exclusion criteria:
- Known monogenic (with the exception of TRPC6 gene mutations) or clinical or histologic evidence of secondary FSGS
- Alport syndrome, Nail Patella syndrome, diabetic nephropathy, IgA-nephropathy, lupus nephritis, or monoclonal gammopathy
- History of organ transplantation or planned transplantation
- Uncontrolled hypertension (SBP >160 mmHg)
- eGFR < 30 mL/min/1.73 m2; ALT/AST >3X ULN
- QTc intervals > 450ms in males or > 470ms in females