The Pharmacokinetics of Voxelotor Following Single Doses in Pediatric Patients With Sickle Cell Disease

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BACKGROUND

Voxelotor (GBT440) is a first-in-class, oral, once-daily therapy that is designed to modulate the oxygen affinity of Hb for oxygen (O2). By keeping HbS in the oxygenated state, voxelotor inhibits polymerization and disease; T1/2, half-life; V, apparent volume of distribution.

METHODS

PK PARAMETERS

Tables 1 and 2. Whole Blood and Plasma PK Parameters Following a Single Dose of Voxelotor

GTB440-007 PART A STUDY OBJECTIVES

PRIMARY OBJECTIVE

To evaluate the safety and tolerability of voxelotor following a single dose of voxelotor 600 mg.

SECONDARY OBJECTIVE

To evaluate the pharmacokinetics (PK) of voxelotor in whole blood and plasma following a single dose of voxelotor 600 mg.

FOCUS ON PHARMACOLOGY

Voxelotor (GBT440) is a first-in-class, oral, once-daily therapy that is designed to modulate the oxygen affinity of Hb for oxygen (O2). By keeping HbS in the oxygenated state, voxelotor inhibits polymerization and disease.

RESULTS

Whole Blood Voxelotor

Table 2. Whole Blood and Plasma PK Parameters Following a Single Dose of Voxelotor

Safety

Table 3. Model PPK Parameter Estimates for Voxelotor in Children, Adolescent, and Adult

CONCLUSIONS

The safety and tolerability of voxelotor following a single dose (600 mg) in children aged 6 to 11 years and in adolescents (aged 12 to 17 years) with sickle cell disease is generally consistent with that reported in prior studies (GTB440-007 and GTB440-007 PART B) in adult patients with sickle cell disease.

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References