ForPatients
by Roche

Beta-Thalassemia

A Study of Bitopertin (RO4917838) in Adults With Non-Transfusion-Dependent (NTD) Beta-Thalassemia

Trial Status	Trial Runs In	Trial Identifier
Completed	3 Countries	NCT03271541 2016-004799-23 BP39642

The information is taken directly from public registry websites such as ClinicalTrials.gov, EuClinicalTrials.eu, ISRCTN.com, etc., and has not been edited.

Official Title:

A Phase II, Single Arm, Multicenter, Proof-of-Mechanism Study to Investigate the Safety, Tolerability, Efficacy, Pharmacokinetics, and Pharmacodynamics of Bitopertin (RO4917838) in Adults With Non-Transfusion-Dependent #eta-Thalassemia

Trial Summary:

This proof-of-mechanism study is being performed to investigate the safety, tolerability, efficacy, pharmacokinetics, and pharmacodynamics of multiple oral doses of bitopertin in adults with NTD beta-thalassemia. This study consists of two parts: Part 1 - The main study - 16 weeks in total: Participants will undergo a 6-week dose-escalation period followed by 10 weeks of treatment at the attained target dose. Part 2 - Open Label Extension (OLE) - up to an additional 12 months. Participants will be given the option to enroll into the OLE once the 16-week treatment of Part 1 has been completed. Participants who decide not to enroll in the OLE, at the end of Part 1 will enter a 6-week follow-up period.

Hoffmann-La Roche
Sponsor

Phase 2
Phase

NCT03271541 2016-004799-23 BP39642
Trial Identifiers

Eligibility Criteria:

Gender	Age	Healthy Volunteers
All	# 18 Years & # 55 Years	No

Inclusion Criteria:

- Confirmed diagnosis of beta-thalassemia
- Clinically defined non-transfusion-dependent anemia (Part 1 only), defined as Hb concentrations >7.5 grams per deciliter (g/dL) and <9.5 g/dL, less than or equal to 4 transfusions of red blood cell units within 1 year prior to study enrollment, and no transfusion within 12 weeks prior to study enrollment
- Completion of 16 weeks of treatment with bitopertin in Part 1 of this study with more than 80% compliance from expected use of study medication (based on patient diary and study drug accountability; Part 2 only)
- A favorable benefit-risk ratio from treatment with bitopertin as assessed by the Investigator (Part 2 only)

Exclusion Criteria:

- Any history of gene therapy
- History of hemolytic anemia except for beta-thalassemia
- Severe symptomatic splenomegaly and/or hepatomegaly with hypersplenism (Part 1 only)
- Any use of an erythropoiesis-stimulating agent within 24 weeks prior to enrollment.
- Initiation of iron chelation therapy or hydroxyurea within 24 weeks prior to enrollment (Part 1 only)
- Depression, treatment with anti-depressants, or other psychiatric illnesses and/or drug abuse
- Clinically significant/uncontrolled comorbid disease
- Pregnant or breastfeeding females
- Use of cytochrome P450 (CYP) 3A4 inhibitors within 2 weeks or CYP3A4 inducers within 4 weeks prior to study drug
- Active hepatitis B or C or known positive human immunodeficiency virus (HIV) test result
- Diagnosis of cancer within previous 5 years unless treatment has resulted in complete freedom from disease for at least 2 years
- Any major illness within 1 month or febrile illness within 1 week prior to study drug
- Pulmonary hypertension requiring oxygen therapy (Part 1 only)