A Study of Efficacy and Safety of Ianalumab in Previously Treated Patients With Warm Autoimmune Hemolytic Anemia

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A Phase 3, Randomized, Double-blind, Study to Assess Efficacy and Safety of Ianalumab (VAY736) Versus Placebo in Warm Autoimmune Hemolytic Anemia (wAIHA) Patients Who Failed at Least One Line of Treatment
ClinicalTrials.gov Identifier:
NCT05648968
Novartis Reference Number:CVAY736O12301
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All compounds are either investigational or being studied for (a) new use(s). Efficacy and safety have not been established. There is no guarantee that they will become commercially available for the use(s) under investigation.

Study Description

The purpose of this study is to evaluate efficacy and safety of ianalumab compared to placebo in patients with warm autoimmune hemolytic anemia, who failed at least one line of treatment. The primary objective is to demonstrate that either dose of ianalumab induces a durable hemoglobin response compared to placebo in patients with wAIHA.

The key secondary objective is to demonstrate that either dose of ianalumab maintains a durable hemoglobin response that is sustained beyond end of the treatment period, compared to placebo.

Participants are randomized to two different doses of ianalumab or placebo. Participants who were assigned to placebo arm and not responding to treatment may be treated with open label ianalumab using the higher dose.

The investigational treatment will be supplied in a double-blinded manner. For the open label period, ianalumab will be provided in an open label manner.

In addition to the randomized treatment (ianalumab or placebo), specific supportive care medication as defined in the protocol is allowed. If clinically indicated (e.g., to ensure patient safety), the treating physician may also administer rescue medication.

The study consists of the treatment period, efficacy and safety follow-up periods. The visit frequency will be every other week during the treatment and primary endpoint follow up period; for safety monitoring monthly during the first 20 weeks after last dose and afterwards quarterly up to 2 years from the last dose. For participants in durable response, additional visits for efficacy will occur monthly during the first 2 years after the last dose, and afterwards quarterly until loss of response or end of study, latest until up to 39 months post randomization of the last participant.

Condition
Warm Autoimmune Hemolytic Anemia (wAIHA)

Phase
Phase3
Overall Status
Recruiting
Number of Participants
90
Start Date
Dec 30, 2022
Completion Date
May 02, 2029
Gender
All
Age(s)
18 Years - 100 Years (Adult, Older Adult)

Interventions

Biological

Ianalumab

i.v. infusion, prepared from concentrate solution
Drug

Placebo

i.v. infusion, prepared from matching placebo

Eligibility Criteria

Key Inclusion Criteria:

* 18 years and older at time of signing consent
* Patients with primary or secondary wAIHA documented by positive direct antiglobulin test specific for anti-IgG or anti-IgA, who had an insufficient response to, or relapsed after at least one line of treatment, including patients with steroid resistance, dependence or intolerance
* Hemoglobin concentration at screening and at Week 1 \>=5 g/dL and \<10 g/dL, associated with presence of symptoms related to anemia
* The dose of supportive care must be stable for at least 4 weeks prior to randomization into the study

Key Exclusion Criteria:

* wAIHA secondary to hematologic disease involving bone marrow (e.g., CLL) or another immunologic disease requiring prohibited medication as per protocol. Patients with autoimmune diseases after wash-out from the treatments are allowed.
* Presence of other forms of AIHA (cold or intermediate forms), Evans Syndrome or other cytopenias
* Prior use of B-cell depleting therapy (e.g., rituximab) within 12 weeks prior to randomization, or without hematological response to the last course of B-cell depleting therapy
* Neutrophils: \<1000/mm3

* Serum creatinine \>1.5 × upper limit of normal (ULN)
* Immunoglobulin G (IgG) \<5g/L
* Active viral, bacterial or other infections (including tuberculosis and SARS-CoV-2) requiring systemic treatment at time of screening, or history of recurrent clinically significant infection
* Positivity for hepatitis C virus, hepatitis B surface antigen (HBsAg), or hepatitis B core antibody (HBcAb). HBcAb positive patients can be enrolled if HBsAg negative, HBV DNA negative, no pre-existing liver fibrosis is present and antiviral prophylaxis is given.
* Known history of primary or secondary immunodeficiency, or a positive human immune deficiency virus (HIV) test result
* Live or live-attenuated vaccination within 4 weeks before randomization
* History of splenectomy

Other protocol-defined Inclusion/Exclusion may apply.

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