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PRESS RELEASE

Novartis ianalumab Phase III trial meets primary endpoint in ITP, demonstrating statistically significant improvement in time to treatment failure

- *Ianalumab prolonged the duration of safe platelet levels during and after treatment in patients with primary immune thrombocytopenia (ITP) previously treated with corticosteroids*[1,2]
- *Patients treated with ianalumab also experienced a significantly higher rate of sustained improvements in platelet count, the key secondary endpoint of the study*[1]
- *Ianalumab, administered as four once-monthly doses in the ITP setting, could offer long-term disease control through a short course of treatment and potentially allow patients extended time off treatment, if approved*
- *Data expected to be presented at an upcoming medical meeting and included in future regulatory submissions in 2027 along with results from the ongoing first-line ITP trial, VAYHIT1*

Basel, August 12, 2025 – Novartis today announced positive top-line results from VAYHIT2, a Phase III trial evaluating ianalumab plus eltrombopag in patients with primary immune thrombocytopenia (ITP) previously treated with corticosteroids[1,2]. Ianalumab plus eltrombopag, compared to placebo plus eltrombopag, significantly prolonged the time to treatment failure (TTF), the primary endpoint that assesses how long patients maintain safe platelet levels during and after the treatment period[1,2]. Ianalumab is being investigated in other B cell-driven autoimmune diseases, including ongoing Phase III trials in first-line ITP and in second and later lines of warm autoimmune hemolytic anemia, with readouts expected in 2026[3,4].

In VAYHIT2, patients treated with ianalumab plus eltrombopag experienced a significantly higher rate of sustained improvements in platelet count at six months, the key secondary endpoint of the study[1]. The safety profile of ianalumab was consistent with what was previously observed in clinical studies, with no new safety signals[1].

“While current treatments for ITP are generally effective in raising platelet counts, many patients require life-long treatment to maintain safe levels, which can create a lasting treatment burden,” said Adam Cuker, M.D., Professor of Medicine and Chief, Section of Hematology, University of Pennsylvania. “The results from VAYHIT2 are encouraging, as they

suggest that ianalumab may support longer periods of disease control and reduce the need for continuous treatment."

ITP is a rare autoimmune disorder characterized by low platelet counts leading to an increased risk of bleeding, bruising and chronic fatigue[5-7]. Many people living with ITP cycle through multiple therapies, unable to achieve long-term disease control[7]. There is a need for other treatment options with novel mechanisms of action that offer durable responses while reducing the burden of long-term treatment[8].

"For many people living with ITP, chronic treatment can disrupt their daily life due to the burden of regular dosing, dose adjustments and side effects," said Shreeram Aradhye, M.D., President, Development and Chief Medical Officer, Novartis. "These positive top-line results from the Phase III study highlight the potential of ianalumab, if approved, to deliver long-term disease control with four once-monthly doses and enable extended time off treatment."

Data is expected to be presented at an upcoming medical meeting and included in future regulatory submissions in 2027 along with results from the ongoing first-line ITP trial, VAYHIT1. Ianalumab has been granted Orphan Drug Designation by the US Food and Drug Administration and the European Medicines Agency[9,10]. Recently, Novartis announced positive top-line results for ianalumab in adults with active Sjögren's disease.

About ianalumab
Ianalumab (VAY736) is a novel fully human monoclonal antibody being investigated for its potential to treat various B cell-driven autoimmune diseases, including Sjögren's disease, immune thrombocytopenia (ITP), systemic lupus erythematosus (SLE), lupus nephritis (LN), warm autoimmune hemolytic anemia (wAIHA) and diffuse cutaneous systemic sclerosis (dcSSc)[2,4,11-16]. Its mechanism of action targets B cells in two ways, namely combining B cell depletion via antibody-dependent cellular toxicity (ADCC) and interruption of BAFF-R mediated signals of B cell function and survival[11]. In clinical trials, ianalumab showed promising efficacy and a favorable safety profile in Sjögren's disease, systemic lupus erythematosus, and immune thrombocytopenia[17-19]. Ianalumab originates from an early collaboration with MorphoSys AG, a company which Novartis later acquired in 2024[20].

About primary immune thrombocytopenia
Primary immune thrombocytopenia (ITP) is a rare, autoimmune disorder in which the immune system mistakenly targets and destroys platelets, the cells essential for blood clotting[5]. This can lead to symptoms such as prolonged bleeding, easy bruising and chronic fatigue, which can significantly impact daily life[5,6].

Despite available treatments, many people living with ITP cycle through multiple therapies, unable to achieve long-term disease control[7]. Current options often focus on maintaining safe platelet levels and preventing bleeding complications and may require ongoing use[7,21]. The burden of chronic treatment and unpredictability of relapses can significantly impact quality of life[6,22]. There is a need for therapies that offer durable response while reducing the burden of long-term treatment[8].

About VAYHIT2
VAYHIT2 (NCT05653219) is a Phase III, multi-center, randomized, double-blind study evaluating the efficacy and safety of two different doses of ianalumab versus placebo, in addition to eltrombopag, in adults with primary immune thrombocytopenia (ITP) (platelet count <30 G/L) who failed previous first-line treatment with corticosteroids[2]. Alongside eltrombopag, patients were randomized 1:1:1 to receive four once-monthly intravenous infusions of ianalumab at 3 mg/kg, ianalumab at 9 mg/kg or placebo[2]. The primary endpoint was time to treatment failure, which is defined as the time from randomization until either: a platelet count of less than 30 G/L later than 8 weeks from randomization; the need for rescue therapy later than 8 weeks from randomization; initiation of a new ITP treatment at any time; ineligibility or inability to taper/discontinue eltrombopag; or death[2]. The key secondary endpoint is the

percentage of patients with a stable platelet count response at Month 6[2]. Other secondary endpoints include measures of depth and duration of platelet response as well as patient-reported outcomes that measure quality of life and fatigue, among other endpoints[2].

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About Novartis

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