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PRESS RELEASE

Novartis ianalumab significantly extends disease control in patients with immune thrombocytopenia with only four once-monthly doses

- *Ianalumab (9 mg/kg) plus eltrombopag extended ITP disease control by 45% with patients maintaining disease control 2.8 times longer than with placebo plus eltrombopag*[1,2]
- *62% of patients treated with ianalumab plus eltrombopag achieved sustained platelet response at six months compared to 39% with placebo plus eltrombopag*[1,2]
- *Ianalumab, administered as four once-monthly intravenous doses in the ITP setting, has the potential to reduce the need for chronic treatment and deliver durable disease control in ITP*
- *Novartis plans to submit VAYHIT2 data from second-line ITP with results from ongoing first-line ITP trial, VAYHIT1, to health authorities in 2027*

Basel, December 9, 2025 – Novartis today announced positive results from VAYHIT2, a Phase III trial evaluating ianalumab plus eltrombopag in patients with primary immune thrombocytopenia (ITP) previously treated with corticosteroids[1-3]. Ianalumab (9 mg/kg) plus eltrombopag extended ITP disease control by 45%, based on the primary endpoint of time to treatment failure (TTF), which assesses how long patients maintain safe platelet levels during and after the treatment period[1,2]. The median time to treatment failure for patients receiving ianalumab plus eltrombopag was 2.8 times longer than those on placebo plus eltrombopag (13.0 months vs. 4.7 months)[1,2].

Detailed data will be presented during the Late-Breaking Abstract Session at the 67th American Society of Hematology Annual Meeting & Exposition (ASH) and simultaneously published in The New England Journal of Medicine[1,2].

“Treatments for ITP have historically focused on raising platelet counts, often requiring chronic therapy to control ITP. This means many patients remain on treatment long-term, facing persistent disease burden and symptoms like fatigue,” said Hanny Al-Samkari, M.D., Peggy S. Blitz Endowed Chair in Hematology/Oncology, Mass General Brigham, and Associate Professor of Medicine, Harvard Medical School. “The VAYHIT2 trial results are encouraging, as they demonstrated improved disease control even while patients spend time off treatment, pointing toward possible progress for people living with ITP.”

Patients receiving ianalumab (9 mg/kg) plus eltrombopag also achieved a significantly higher rate of sustained platelet count improvement at six months versus placebo plus eltrombopag (62% vs. 39%), meeting the key secondary endpoint[1,2]. Fatigue improvement, as measured by PROMIS Fatigue, showed a mean reduction of 7.7 points with ianalumab plus eltrombopag versus 3.6 points with placebo plus eltrombopag[1,2].

“B cells drive the autoimmune response that leads to platelet destruction and increased bleeding risk in ITP. The novel dual mechanism of action of ianalumab aims to deplete B cells while blocking their survival signals,” said Mark Rutstein, M.D., Global Head, Oncology Development, Novartis. “Guided by our decades-long experience advancing ITP care, the VAYHIT2 findings underscore the potential of ianalumab to deliver durable control with a short course of four once-monthly doses, offering patients the possibility of achieving disease stability without ongoing treatment.”

Two doses of ianalumab were assessed in VAYHIT2 with ianalumab 9 mg/kg demonstrating statistically significant improvements across both the primary and key secondary endpoints, and ianalumab 3 mg/kg demonstrating statistically significant improvements in the primary endpoint and numerical improvements in the key secondary endpoint[1-3].

	Ianalumab 9 mg/kg + eltrombopag (N=50)	**Ianalumab 3 mg/kg + eltrombopag (N=51)**	**Placebo + eltrombopag (N=51)**
Primary endpoint: Time to treatment failure (TTF)	13.0 months (HR 0.55; 95% CI: 0.32, 0.92; p=0.021[a])	Not estimable (HR 0.58; 95% CI: 0.34, 0.98; p=0.023[a])	4.7 months
Key secondary endpoint: Stable response at 6 months (SR6)	62.0% (p=0.023[a])	56.9% (p=0.035[a])	39.2%

a. Required p-value for statistical significance is one-sided <0.025

Ianalumab was well tolerated with no new safety signals, and the side effect profile was consistent with previous studies[1,2]. Adverse events were comparable between the ianalumab and placebo arms, with the most common AEs for ianalumab plus eltrombopag being headache (14% with 9 mg/kg, 10% with 3 mg/kg vs. 8% with placebo) and infusion-related reactions (14% with 9 mg/kg, 8% with 3 mg/kg vs. 8% with placebo)[1,2]. Neutropenia* occurred more frequently in the ianalumab groups (16% with 9 mg/kg, 12% with 3 mg/kg) compared to placebo (2%) with most cases resolving without requiring treatment or dose interruption[1,2]. No on-treatment adverse event led to permanent discontinuation of therapy[1,2].

VAYHIT2 marks the third positive Phase III trial with ianalumab, following two positive trials in adults with active Sjögren's disease[1,4]. Novartis plans to submit the data from VAYHIT2 along with results from the ongoing first-line ITP trial, VAYHIT1, in 2027. Ianalumab has been granted Orphan Drug Designation by the US Food and Drug Administration and the European Medicines Agency[5,6].

**An adverse event of special interest encompassing several terms related to low levels of neutrophils, neutrophil precursors and leukocytes*

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)[3,7-13]. 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[8]. In clinical trials, ianalumab showed promising efficacy and a favorable safety profile in Sjögren’s disease, systemic lupus erythematosus, and immune thrombocytopenia[4,14-16]. Ianalumab originates from an early collaboration with MorphoSys AG, a company which Novartis later acquired in 2024[17].

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[18]. This can lead to symptoms such as prolonged bleeding, easy bruising and chronic fatigue, which can significantly impact daily life[18,19].

Despite available treatments, many people living with ITP cycle through multiple therapies, unable to achieve long-term disease control[20]. Current options often focus on maintaining safe platelet levels and preventing bleeding complications and may require ongoing use[20,21]. The burden of chronic treatment and unpredictability of relapses can significantly impact quality of life[19,22]. There is a need for therapies that offer durable response while reducing the burden of long-term treatment[23].

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[3]. 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[3]. 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[3]. The key secondary endpoint is the percentage of patients with a stable platelet count response at Month 6[3]. 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[3].

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This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as “potential,” “can,” “will,” “plan,” “may,” “could,” “would,” “expect,” “anticipate,” “look forward,” “believe,” “committed,” “investigational,” “pipeline,” “launch,” or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for the investigational or approved products described in this press release, or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that the investigational or approved products described in this press release will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that such products will be commercially successful in the future. In particular, our expectations regarding such products could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG’s current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

About Novartis

Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach nearly 300 million people worldwide.

Reimagine medicine with us: Visit us at **https://www.novartis.com** and connect with us on **LinkedIn**, **Facebook**, **X/Twitter** and **Instagram**.

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