The ITP Patient Advocate

A Resource for Your Journey With Chronic ITP

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Medical News About Nplate®

This issue looks at a recent 1-year Nplate® medical study that supports the results of two earlier, 6-month studies of Nplate® in adult patients with chronic ITP.[1-3]

Nplate® is a man-made protein medicine used to treat low blood platelet counts in adults with chronic immune thrombocytopenia (ITP), when certain other medicines, or surgery to remove your spleen, have not worked well enough.[1]

Nplate® is not for use in people with a precancerous condition called myelodysplastic syndrome (MDS) or low platelet count caused by any condition other than chronic (lasting a long time) immune thrombocytopenia (ITP). Nplate® is only used if your low platelet count and medical condition increase your risk of bleeding. Nplate® is used to try to keep your platelet count about 50,000 per microliter in order to lower the risk for bleeding. Nplate® is not used to make your platelet count normal. It is not known if Nplate® works or is safe in people under the age of 18.[1]

Nplate® raised and sustained platelet counts in the 6-month studies[1,2]

- The primary goal was achievement of a durable platelet response in patients who received either Nplate® or placebo for six months.
  - Durable response was defined as a weekly platelet count of 50,000 per microliter or greater for at least 6 of the last 8 weeks of the study, and patients could not have received rescue therapy* at any point in the study.
- **Results:**
  - Patients with spleens: 61% (25/41) of patients on Nplate® achieved durable platelet responses vs 5% (1/21) of those taking placebo.
  - Patients whose spleens had been removed: 38% (16/42) of patients on Nplate® achieved durable platelet responses vs 0% (0/21) of those taking placebo.
  - Headache was the most common side effect.

*A rescue therapy was defined as a treatment given to increase platelet counts to help prevent life-threatening bleeding. Examples include intravenous immunoglobulin (IVIG), corticosteroids, anti-D, or platelet transfusion.[1,2]

A 1-year study of Nplate® supports the outcome of 6-month studies[3]

This study looked at patients taking either Nplate® or other commonly used ITP treatments. The results of this study support the safety and efficacy profile established in the 6-month studies. See pages 2 and 3 for more details.

Individual results will vary.

Important Safety Information

- Nplate® can cause serious side effects: worsening of a precancerous blood condition to a blood cancer (leukemia) in patients with MDS, higher risk for blood clots, and loss of response.

Please see additional Important Safety Information on page 4.

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A closer look at the 1-year study[3]

- A study of 234 adult patients with ITP, who still had their spleens.
- Almost two thirds (157) of patients were given Nplate®.*
- Nearly one third (77) were given other commonly used ITP treatments chosen by their doctors, including corticosteroids, immunoglobulins, rituximab†, azathioprine†, platelet transfusions, and other medicines for ITP.

It is important to know that this study looked at the other commonly used ITP treatments as a group. It was not meant to compare Nplate® to any other single treatment.

*Other ITP treatments used by patients in the Nplate® group at any time during the 1-year study included corticosteroids (37% of patients), immunoglobulins (7%), rituximab (1%)†, azathioprine (1%)†, danazol (2%)†, other medications (6%), and platelet transfusions (6%).
†Not approved by the FDA for treatment of ITP.

Primary goals of the study

1. To look at the number of patients who had treatment failure over one year

How treatment failure was defined

- A platelet count of 20,000 per microliter or less for four consecutive weeks at the highest recommended dose of study treatment

OR

- A change in treatment, including spleen removal surgery (called a splenectomy), due to side effects or bleeding symptoms

OR

- A major bleeding event, such as an event that would require seeing a doctor

2. To see how many patients had a splenectomy

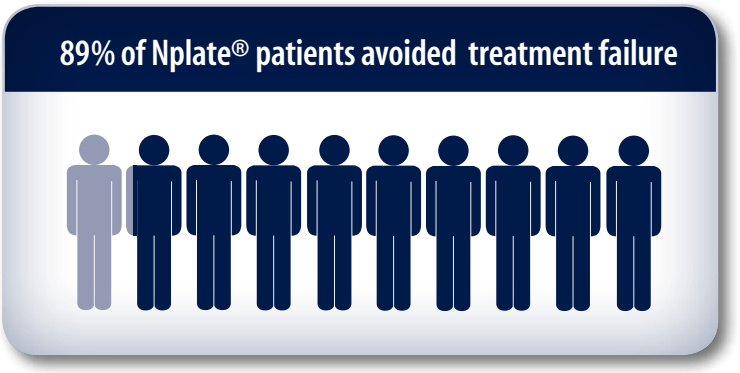
Note: For the purposes of the study, leaving the study before treatment failure occurred was recorded as "treatment failure." Leaving the study before splenectomy was recorded as "splenectomy."

Download My Platelet Tracker at **www.platelettracker.com** to help you keep track of your platelet counts

Results of the study

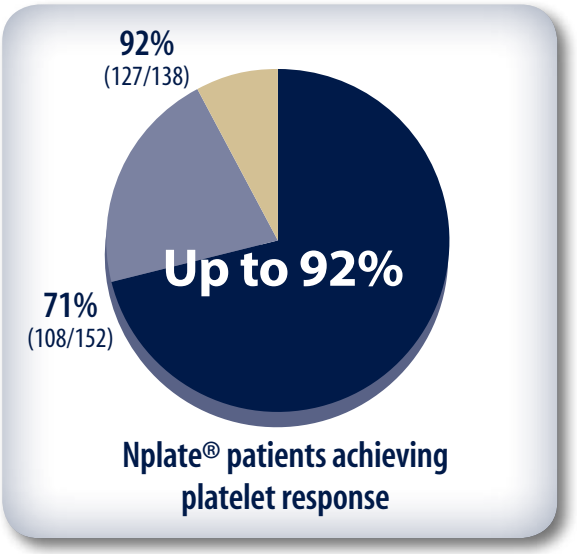
Nearly 9 out of 10 Nplate® patients avoided treatment failure[3]

- Most Nplate® patients in the 1-year study successfully increased and sustained their platelet counts—with no major bleeding or side effects serious enough to cause them to stop treatment.
- Results were consistent with those seen in the 6-month study of patients who still had their spleens.[1,2]



Majority of Nplate® patients reached platelet count goals

- Platelet response was measured at scheduled visits from week 2 through the end of the 1-year study. At any given visit, 71% to 92% of patients had a platelet count greater than 50,000 per microliter.



Individual results will vary.

The most common side effects seen in this study were headache and fatigue.
—Safety results were consistent with those in the 6-month studies and a long-term follow-up study[1-4]

Ask your doctor if you have questions about Nplate®

Important Safety Information

- Nplate® can cause serious side effects: worsening of a precancerous blood condition to a blood cancer (leukemia) in patients with MDS, higher risk for blood clots, and loss of response.

Please see additional Important Safety Information on page 4.

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Important Safety Information

What is the most important information I should know about Nplate®?

Nplate® can cause serious side effects:

- **Worsening of a precancerous blood condition to a blood cancer (leukemia):** Nplate® is not for use in people with a precancerous condition called myelodysplastic syndromes (MDS) or for any condition other than chronic (lasting a long time) immune thrombocytopenia (ITP). If you have MDS and receive Nplate®, your MDS condition may worsen and become an acute leukemia. If MDS worsens to become acute leukemia you may die sooner from the acute leukemia.
- **Higher risk for blood clots:**
  - —You may have a higher risk of getting a blood clot if your platelet count becomes high during treatment with Nplate®. You may have severe complications or die from some forms of blood clots, such as clots that spread to the lungs or that cause heart attacks or strokes. Your healthcare provider will check your blood platelet counts and change your dose or stop Nplate® if your platelet counts get high.
  - —If you have a chronic liver disease, you may get blood clots in the veins of your liver. This may affect your liver function.
- **Loss of response:** If you do not experience results from Nplate®, your immune system may have created a response that is counteractive to Nplate®. Your healthcare provider will monitor your platelet counts and test your blood regularly to determine if this is an issue.
- **Blood test monitoring:** Your healthcare provider will check your platelet count every week and change your dose of Nplate® as needed. This will continue until your healthcare provider decides that your dose of Nplate® can stay the same. After that, you will need to have blood tests every month. When you stop receiving Nplate®, you will need blood tests for at least 2 weeks to check if your platelet count drops too low.
- **What are the possible side effects of Nplate®?**
  - —Nplate® may cause serious side effects. See "What is the most important information I should know about Nplate®?"
  - —The most common side effects of Nplate® are:
    - Headache
    - Joint pain
    - Dizziness
    - Trouble sleeping
    - Muscle tenderness or weakness
    - Pain in arms and legs
    - Abdominal pain
    - Shoulder pain
    - Indigestion
    - Tingling or numbness in hands and feet
  - —People who take Nplate® may have an increased risk of developing new or worsening changes in the bone marrow called "increased reticulin." These changes may improve if you stop taking Nplate®. Your healthcare provider may need to check your bone marrow for this problem during treatment with Nplate®.
  - —These are not all the possible side effects of Nplate®. Tell your healthcare provider if you have any side effect that bothers you or that does not go away. For more information, ask your healthcare provider or pharmacist.
  - —If you have any questions about this information, be sure to discuss them with your doctor. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch, or call 1-800-FDA-1088.

Please see Prescribing Information and Medication Guide for more information about Nplate®.

Co-pay assistance may help you manage out-of-pocket expenses*

*To find out if you are eligible for co-pay assistance with the Nplate FIRST STEP™ Program, please contact My Nplate® Center at 1-855-MYCENTER or visit nplate.com/mnc for more information and a complete description of program benefits, eligibility requirements, and restrictions.

Visit **nplate.com/mnc**

Or call toll-free

1-855-MYCENTER
(1-855-692-3683)

Monday-Friday, 9 AM-8 PM Eastern Time

Open to see study results and Important Safety Information

Important Safety Information

- Nplate® can cause serious side effects: worsening of a precancerous blood condition to a blood cancer (leukemia) in patients with MDS, higher risk for blood clots, and loss of response.
- In medical studies with Nplate®, headache was the most common side effect.

Please see additional Important Safety Information on page 4.

References: 1. Nplate® (romiplostim) prescribing information, Amgen. **2.** Kuter DJ, Bussel JB, Lyons RM, et al. Efficacy of romiplostim in patients with chronic immune thrombocytopenic purpura: a double-blind randomised controlled trial. *Lancet.* 2008;371:395-403. **3.** Kuter DJ, Rummel M, Boccia R, et al. Romiplostim or standard of care in patients with immune thrombocytopenia. *N Engl J Med.* 2010;363:1889-1899. **4.** Kuter DJ, Bussel JB, Newland A, et al. Long-term treatment with romiplostim in patients with chronic immune thrombocytopenia: safety and efficacy. *Br J Haematol.* 2013;161:411-423.

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The Effectiveness and Safety of Nplate® Have Been Evaluated in Multiple Medical Studies

Study results: Nplate® raised and sustained platelet counts[1-4]

- Nplate® helped most patients reach a treatment goal of 50,000 platelets per microliter.
- The most common side effects seen in the pivotal studies for Nplate® were headache, joint pain, and dizziness. These side effects were mild to moderate. Side effects in the other Nplate® medical studies were similar and did not increase over time.

Nplate® medical studies included:

- Two 6-month key medical studies[1,2]
  - — One study with patients who had spleens, the other with patients whose spleens had been removed
- A 1-year medical study that looked at how patients did on Nplate® or on other ITP therapies[3]
- A medical study looking at the long-term safety of Nplate® in patients from previous Nplate® studies[4]

Individual results will vary.

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- Nplate® can cause serious side effects: worsening of a precancerous blood condition to a blood cancer (leukemia) in patients with MDS, higher risk for blood clots, and loss of response.

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