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Plain language summary of IMerge, a Phase 3 study of imetelstat versus placebo in people with lower-risk myelodysplastic syndromes and anemia

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Plain Language Summary of Publication

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Plain language summary of IMerge, a Phase 3 study of imetelstat versus placebo in people with lower-risk myelodysplastic syndromes and anemia

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Where can I find the original article on which this summary is based?

This summary is based on the original article titled, 'Imetelstat in patients with lower-risk myelodysplastic syndromes who have relapsed or are refractory to erythropoiesis-stimulating agents (IMerge): a multinational, randomised, double-blind, placebo-controlled, phase 3 trial,' which was published in *The Lancet* in 2024. You can read the full article for a fee at: https://www.thelancet.com/journals/lancet/article/PIIS0140-6736(23)01724-5/abstract.

Summary

How to say (download PDF and double-click sound icon to play sound)...

- **Anemia:** uh-NEE-mee-uh
- **Erythropoiesis:** eh-RITH-roh-poy-EE-sis
- **Hemoglobin:** hee-muh-glow-bn
- **Imetelstat:** ih-meh-TEL-stat
- **Myelodysplastic:** migh-uh-loh-diss-PLASS-tick
- **Neutropenia:** NOO-trow-PEE-nee-uh
- **Telomerase:** teh-LOH-meh-rays
- **Thrombocytopenia:** thraam-bow-sy-tow-PEE-nee-uh

What is this summary about?

Myelodysplastic syndromes, or MDS for short, are a group of blood cancers that usually cause anemia, a condition where the body does not make enough healthy red blood cells. People with anemia caused by MDS often feel tired, have physical weakness and shortness of breath, and may need to have red blood cell transfusions to feel better. They may also be eligible to take a medicine called an erythropoiesis-stimulating agent, or ESA, which may help the body to make more red blood cells. However, ESAs may not work or may stop working in many people and those people may need regular red blood cell transfusions to stay healthy, which is known as being transfusion dependent. A transfusion, which is a procedure where red blood cells from a healthy donor are put into a person's bloodstream through a vein, may help the person feel better for a short period of time, but it is not a cure. Unfortunately, getting transfusions too often can cause side effects or other health problems.

Imetelstat is a prescription medicine used to treat a group of MDS called low- to intermediate-1–risk MDS (or lower-risk MDS [LR-MDS]) in adults with anemia who need blood transfusions of 4 or more red blood cell units over 8 weeks and who have not responded to, have stopped responding to, or cannot be treated with ESAs.

Imetelstat is given as an intravenous infusion (using a needle into the vein) usually every 4 weeks.

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New medicines, like imetelstat, are tested in clinical trials to understand how they work in people. The effects of imetelstat were evaluated in a Phase 3 study called IMerge. The United States Food and Drug Administration and the European Medicines Agency approved imetelstat for use based on the positive results of IMerge. This summary highlights the results from the IMerge study.

What were the results?

- Among people given imetelstat, 40% went at least 8 weeks without needing red blood cell transfusions (this was the key measure of the study), compared with 15% of people given placebo.
- A total of 28% of people given imetelstat went at least 24 weeks without needing red blood cell transfusions, compared with 3% of people given placebo.
- The most common side effects experienced by people who received imetelstat were less neutrophils in the blood (neutrophils are a type of white blood cell that helps the body to fight infections) and less platelets in the blood (platelets are blood cells that help to stop bleeding).
- If a person had a lot fewer neutrophils or platelets, their imetelstat dose was delayed, reduced, or stopped. Some people received medicine or transfusions to help increase the number of neutrophils or platelets.

What were the main conclusions reported by the researchers?

- In the IMerge study, imetelstat helped some people with transfusion-dependent anemia caused by LR-MDS to go at least 8 weeks or more without red blood cell transfusions.

What is the purpose of this plain language summary?

The purpose of this plain language summary is to help you understand the findings from recent research. The results of this study may differ from those of other studies. Healthcare professionals should make treatment decisions based on all available evidence and not on the results of a single study.

Who should read this article?

This summary is for people who have LR-MDS with transfusion-dependent anemia and for their families and caregivers who want to understand the results of the IMerge Phase 3 study.

Who sponsored this study?

This study was **sponsored** by Janssen Research & Development before April 18, 2019, and Geron Corporation thereafter.

Sponsor: A company or organization that oversees and pays for a clinical research study. The sponsor also collects and analyzes the information from the study.

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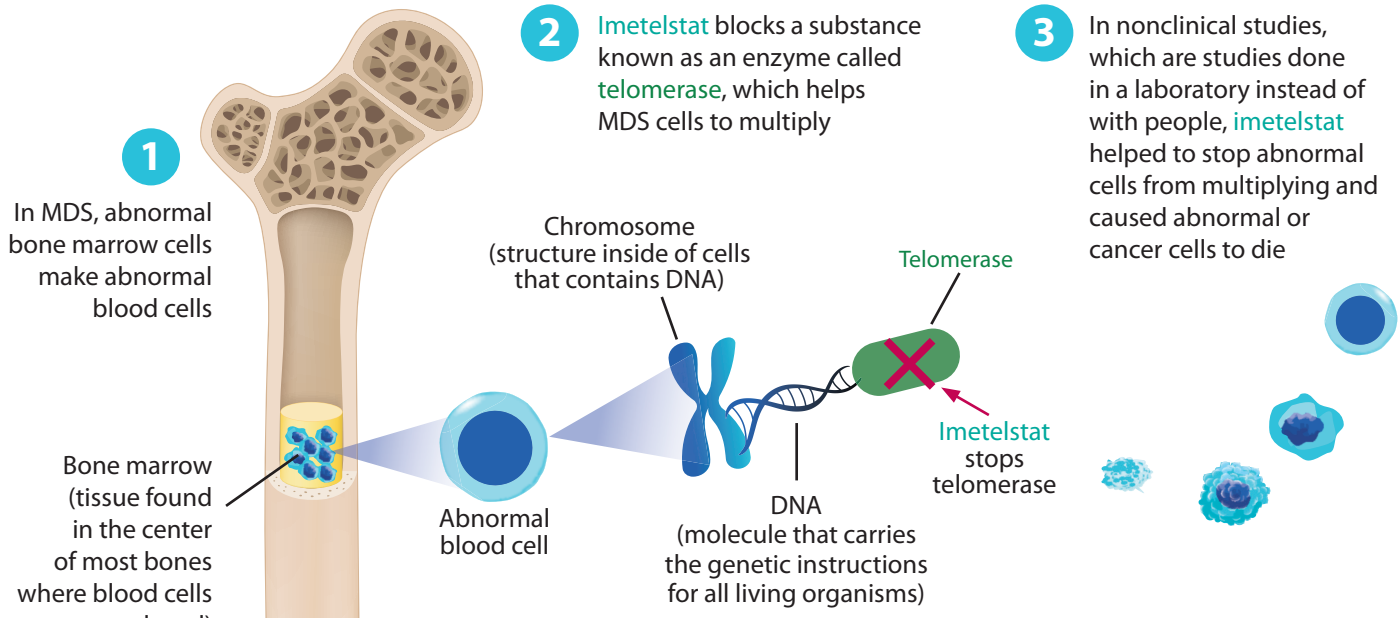
What is imetelstat and how does it work?

Imetelstat is a new medicine that treats people with LR-MDS and transfusion-dependent anemia. It is the first medicine of its kind.



Who participated in the study?

178 Adults

- 18 years or older
- Had anemia related to LR-MDS and had not been given medicines such as lenalidomide or hypomethylating agents (medicines that can turn off genes)
- Were not eligible to receive ESAs (medicines that may help the body to make more red blood cells), were not responding to ESAs, or had lost response to ESAs
- Were dependent on transfusions and required at least 4 units of red blood cells over an 8-week period

Where did the study take place?

People who took part in the study were treated at hospitals, cancer centers, and outpatient clinics in 17 different countries, including United States, Canada, Belgium, Czech Republic, France, Germany, Israel, Italy, Netherlands, Poland, Spain, United Kingdom, Republic of Korea, Russia, Switzerland, Turkey, and Ukraine.

What medicines did people receive and how did they take them?

For every 2 people who received imetelstat, 1 person received placebo (a substance that looks the same as, and is given the same way as, the medicine being tested but has no effects when taken).

- Treatment groups were chosen randomly (by chance), and neither researchers nor participants knew who was taking either treatment.
- People received imetelstat or placebo intravenously (using a needle into the vein) over 2 hours, every 4 weeks.
- The dose of imetelstat was determined by the person's body weight.

Who was in the study?

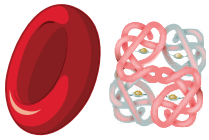
6.2 of 10 people in the study were male

9 of 10 people in the study had been treated with ESAs before; 1 out of 10 were not eligible to receive ESA treatment

Median (middle) number of red blood cell transfusions needed:

6 UNITS
over 8 weeks

4.7 of 10 people needed more than 6 units over 8 weeks

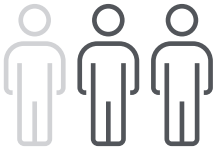


Median (middle) hemoglobin:
8 g/dL

Median (middle) level in the blood of erythropoietin, a hormone that stimulates the production of red blood cells:
399 mU/mL

Group of MDS

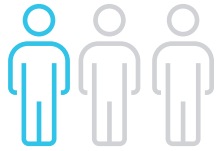
Low risk
(small likelihood of progression to more serious disease)



2 of 3

Intermediate-1 risk
(medium likelihood of progression to more serious disease)

1 of 3



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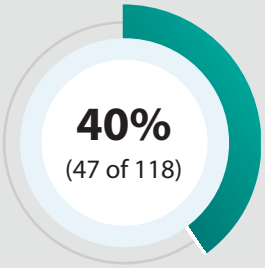
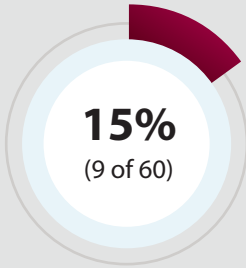
What were the primary results of the study?



40% (47 of 118)

of people given imetelstat did not need red blood cell transfusions
for 8 weeks or longer

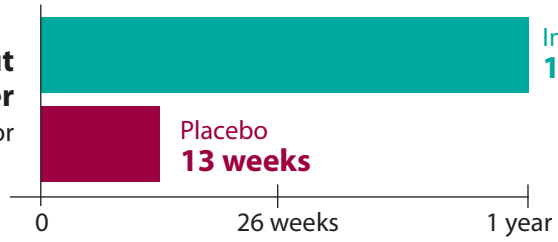


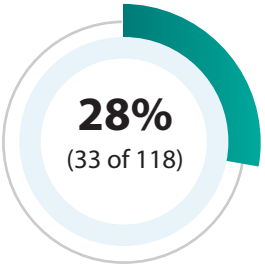
15% (9 of 60)

of people given placebo did not need red blood cell transfusions
for 8 weeks or longer

What other results did researchers observe?

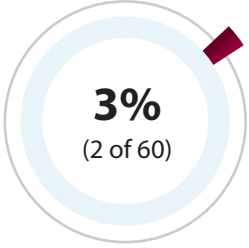
The median (middle) length of time without red blood cell transfusions was 4 times longer
for people who did not need red blood cell transfusions for at least 8 weeks, with imetelstat compared with placebo





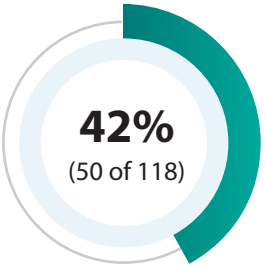
28% (33 of 118)

of people given imetelstat did not need red blood cell transfusions
for 24 weeks or longer



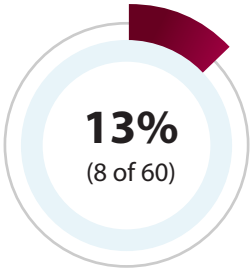
3% (2 of 60)

of people given placebo did not need red blood cell transfusions
for 24 weeks or longer



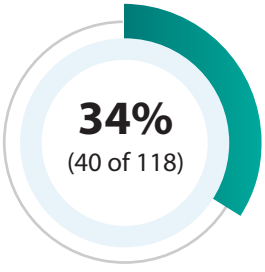
42% (50 of 118)

of people given imetelstat achieved hematologic improvement-erythroid, or HI-E, which is a positive sign that their body is making healthy red blood cells



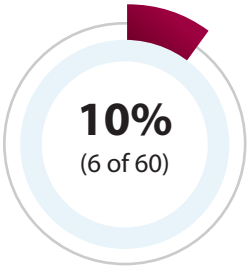
13% (8 of 60)

of people given placebo achieved HI-E



34% (40 of 118)

of people given imetelstat had their hemoglobin levels go up
by 1.5 g/dL or more for 8 weeks or longer



10% (6 of 60)

of people given placebo had their hemoglobin levels go up
by 1.5 g/dL or more for 8 weeks or longer

Due to the way the study was designed, researchers were not able to confidently determine if the differences between imetelstat and placebo groups for HI-E and hemoglobin levels were meaningful.

5 of 10 people given imetelstat
reported experiencing some improvement in fatigue that lasted for 8 weeks or longer

4 of 10 people given placebo
reported experiencing some improvement in fatigue that lasted for 8 weeks or longer

Due to the way the study was designed, researchers were not able to confidently determine if the difference between imetelstat and placebo groups was meaningful in this case.

What were the most common side effects?

Researchers evaluated side effects based on how severe they were

GRADE 1	GRADE 2	GRADE 3	GRADE 4	GRADE 5
Mild or no symptoms	Moderate symptoms	Severe but not immediately life-threatening symptoms	Immediately life-threatening symptoms	Death

The most common side effects of any grade reported in at least 15% of people given imetelstat during the study were lowered platelet counts, lowered red blood cell counts, lowered neutrophil counts, tiredness, and COVID-19 infections.

The most common grade 3 or grade 4 side effects reported in people given imetelstat during the study were neutropenia (lowered neutrophil counts) and thrombocytopenia (lowered platelet counts) measured in their blood.

	Thrombocytopenia (low platelets, a type of blood cell that stops bleeding)		**Neutropenia** (low neutrophils, a type of white blood cell that helps the body to fight infections)	
	Imetelstat	**Placebo**	**Imetelstat**	**Placebo**
Any grade	**75%** (89 of 118) of people	**10%** (6 of 5) of people	**74%** (87 of 118) of people	**7%** (4 of 59) of people
Grade 3 or 4	**62%** (73 of 118) of people	**8%** (5 of 59) of people	**68%** (80 of 118) of people	**3%** (2 of 59) of people
	Most cases of grade 3 or 4 thrombocytopenia and neutropenia typically lasted for a short time and happened early on during treatment (within the first 3 treatment cycles). Serious clinical effects because of thrombocytopenia or neutropenia, including grade 3 or 4 bleeding events or infections, were similar for imetelstat and placebo.			
Median (middle) duration of grade 3 or 4 events	**1.4 weeks**	**2.0 weeks**	**1.9 weeks**	**2.2 weeks**

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How did doctors manage side effects?

IN MOST CASES, the side effects of imetelstat got better by **delaying the next dose** (longer time before receiving the medicine again) or **lowering the dose**. **IN SOME CASES,** people had to **stop treatment.**

Of 118 people given imetelstat...

DELAY DOSE

55 people (47%) had to delay their next dose of imetelstat due to thrombocytopenia

60 people (51%) had to delay their next dose of imetelstat due to neutropenia

5 people (4%) had to delay their next dose of imetelstat due to infusion-related side effects

LOWER DOSE

27 people (23%) had to receive a lower dose of imetelstat due to thrombocytopenia

39 people (33%) had to receive a lower dose of imetelstat due to neutropenia

STOP TREATMENT

19 people (16%) given imetelstat had to stop the treatment because of side effects (no one given placebo stopped treatment because of side effects)

What do the results of this study mean?

- This is the first study to show that the new medicine, imetelstat, significantly reduced the number of red blood cell transfusions needed in some people with LR-MDS and anemia requiring regular red blood cell transfusions compared with placebo.
- Due to the nature of the study design, researchers were unable to calculate whether some differences between imetelstat and placebo groups were meaningful.
- Based on these results, imetelstat was approved in the United States and European Union.
- Imetelstat is a prescription medicine used to treat a condition called low- to intermediate-1–risk MDS in adults:
  - » With anemia (low red blood cell count) who need blood transfusions of 4 or more red blood cell units over 8 weeks, and,
  - » Who have not responded to, have stopped responding to, or cannot be treated with ESAs.

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Disclosure statement

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