NOVARTIS

Clinical Trial Results Summary

A clinical trial to learn more about the safety of PDR001, MBG453, or both given with or without other drugs in people with certain blood cancers

Thank you!

Thank you to the participants who took part in the clinical trial for blood cancers including certain types of **acute myeloid leukemia (AML)**, **myelodysplastic syndrome (MDS)**, and **chronic myelomonocytic leukemia (CMML)**. Every participant helped the researchers learn more about the trial drugs **PDR001**, also called **spartalizumab**, and **MBG453**, also called **sabatolimab**, given with or without other drugs.

Novartis sponsored this trial and believes it is important to share what was learned from the results of this trial with the participants and the public. We hope this helps the participants understand their important role in medical research.

Trial information

Trial number: CPDR001X2105

Novartis drug studied: **PDR001**, also called spartalizumab, and **MBG453**, also called sabatolimab

Sponsor: Novartis

If you were a participant and have any questions about the results, please talk to the doctor or staff at the trial site.

This summary only shows the results of a single clinical trial. Other clinical trials may have different results.

What was the main purpose of this trial?

The purpose of this trial was to learn about the safety of **MBG453** and **PDR001** given together and with other drugs in people with certain blood cancers. The purpose was also to learn about the safety of **MBG453** given alone. Researchers also wanted to find the highest doses of **PDR001**, **MBG453**, or both that were safe for participants to receive with or without other drugs.

The **blood cancers** in this trial were:

- **Acute myeloid leukemia (AML)** is a fast growing cancer that starts in the cells that turn into blood cells in the bone marrow, most often in cells that turn into white blood cells. Bone marrow is the tissue inside of bones that helps make blood cells. The cancer cells build up and slow down the making of normal blood cells.
- **Myelodysplastic syndrome (MDS)**, which are a group of blood cancers that are caused by problems during the process that turns cells into blood cells. As a result, blood cells do not fully develop or work as they should. People with certain types of MDS are more likely to get AML.
- **Chronic myelomonocytic leukemia (CMML)** is a type of cancer that starts in the cells that turn into blood cells, resulting in too many abnormal white blood cells called monocytes

PDR001, also called spartalizumab (pronounced as spar-ta-liz-ue-mab), is a trial drug designed to help the immune system fight cancer by blocking a protein called PD-1. PD-1 can prevent the immune system from killing cancer cells.

MBG453, also called sabatolimab (pronounced as sa-buh-tol-ue-mab), is a trial drug designed to help the immune system fight cancer by blocking a protein called TIM-3. TIM-3 can lower the immune system's activity, which prevents it from killing cancer cells.

Decitabine (pronounced as dee-sai-tuh-been) is a drug that is approved to use alone to treat AML, MDS, and CMML in certain countries, including the United States and in Europe. In this trial, it was given with **PDR001**, **MBG453**, or both.

Azacitidine (pronounced as a-zuh-si-tuh-deen) is a drug that is approved to use alone to treat AML and MDS in certain countries, including the United States and in Europe. In this trial, it was given with **MBG453**.

The trial's purpose was to answer these main questions:

- What were the highest doses of PDR001, MBG453, or both that were safe for participants to receive with or without other drugs?
- What medical problems, also called adverse events, happened during this trial?

  ↳ An **adverse event** is any sign or symptom that participants have during a trial. Adverse events **may** or **may not** be caused by treatments in the trial.

How long was this trial?

The trial began in July 2017 and ended in September 2023. The participants could be in this trial for up to 5 years.

Who was in this trial?

241 participants with AML, MDS, or CMML were in this trial – 146 men and 95 women. Participants' average age was 69 years.
The number of participants by race is shown below.

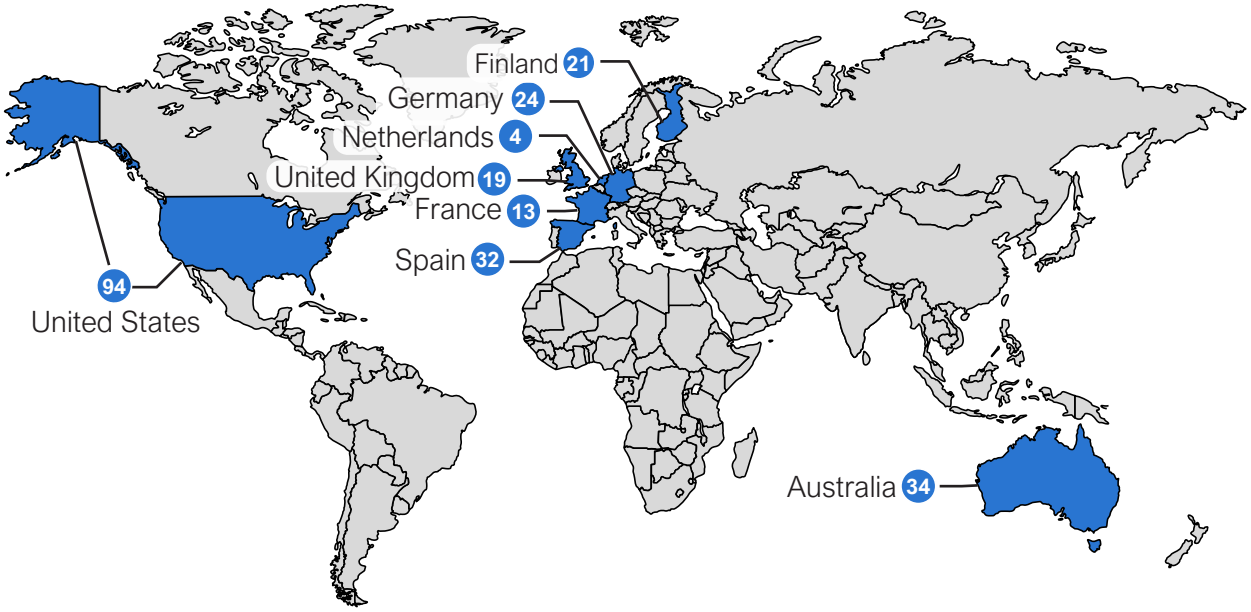
Race

8	Asian	4	Black	203	White (Caucasian)
23	Not collected	3	Other		

The participants could take part in this trial if they:

- Had **AML** that was:
  - **Relapsed** or **refractory**, which means cancer came back after treatment or approved treatments didn't work to shrink or stop cancer growth
  - **Newly diagnosed**, which means the participant had not yet been treated
- Had **MDS** that was **intermediate, high risk, or very high risk,** which means MDS grows more quickly or will become AML more quickly if not treated
- Had **CMML**
- Did not have cancer in their brain or spinal cord
- Did not have another type of blood cancer

241 participants from 8 countries were in this trial. The map below shows the number of participants who took part in each country.



What treatments did the participants receive?

The treatments in this trial were given in cycles. A **cycle** is a treatment period that is repeated. In this trial, a cycle was 4 weeks. The treatments in this trial were:

- **PDR001**, 400 milligrams (mg), given through a needle in a vein as an intravenous (IV) infusion one time during each cycle (every 4 weeks).
- **MBG453**, 80, 160, 240, 400, 800, or 1,200 mg, given as an IV infusion 1 or 2 times during each cycle (every 2 or 4 weeks).
- **Decitabine**, 20 milligrams per square meter (mg/m²) of **body surface area (BSA)** based on the label instructions. Each participant's dose was calculated from their BSA and given as an IV infusion 5 times during each cycle.
- **Azacitidine**, 75 mg/m² of BSA based on the label instructions. Each participant's dose was calculated from their BSA and given as an IV infusion or an injection under the skin 7 times during each cycle.

What is body surface area?

Body surface area (BSA) is a measure of the amount of skin that covers a person's body, based on their height and weight. Doctors use BSA to make sure a person gets the correct dose of treatment for their body size.

The trial doctors could lower a participant's dose of any of the trial treatments, if needed.

Each participant received trial treatment until their cancer got worse, they could not tolerate the treatment, or they or the trial doctor decided to stop treatment.

In this trial, the participants and clinical trial team knew what treatment each participant received.

What happened during this trial?

Before treatment About 1 month

Trial staff checked to make sure the participants could be in this trial.

During treatment Up to about 4 years

241 participants were in this trial, but 9 participants only received **decitabine** or **azacitidine.**

232 participants with AML, MDS, or CMML received treatment in one of these groups during 4-week cycles:

	AML 124 participants	MDS 93 participants	CMML 15 participants
Group 1 400 mg **PDR001** and **decitabine**	13 participants	3 participants	0 participants
Group 2 240, 400, or 800 mg **MBG453** and **decitabine**	51 participants	26 participants	5 participants
Group 3 160, 240, or 400 mg **MBG453**, 400 mg **PDR001**, and **decitabine**	12 participants	6 participants	0 participants
Group 4 400 or 1,200 mg **MBG453**	16 participants	10 participants	0 participants
Group 5 80 or 240 mg **MBG453** and 400 mg **PDR001**	6 participants	5 participants	0 participants
Group 6 240, 400, or 800 mg **MBG453** and **azacitidine**	26 participants	43 participants	10 participants

In Groups 2 to 6, the first participants received the lowest dose of **MBG453**. If there were no safety concerns during their first 8 weeks of treatment, the next few participants had a higher dose. This continued until researchers found the highest dose of **MBG453** that was safe for participants to receive alone or with other drugs.

After treatment Until the trial ended

Each participant returned to the trial site or had a phone call with trial staff to check their health either:

- 30 days after their last dose of **azacitidine** or **decitabine**
- 150 days after their last dose of **PDR001** or **MBG453**

If a participant's cancer did not get worse during trial treatment, they had follow-up visits or phone calls with trial staff to check on their cancer every 2 months until their cancer got worse or the trial ended.

Trial doctors checked the participants' general health throughout the trial.

What were the main results of this trial?

What were the highest doses of PDR001, MBG453, or both that were safe for participants to receive with or without other drugs?

The researchers concluded that all of the doses of **MBG453** given with or without **PDR001** and other drugs were safe for participants in this trial. The researchers also concluded that the recommended dose of **MBG453** to use in any future trials is 400 mg every 2 weeks or 800 mg every 4 weeks with other drugs.

To learn this, researchers kept track of how many participants with each type of blood cancer had:

- **Dose limiting toxicities (DLTs)**, which are medical problems with risk of serious harm if the dose went up. DLTs are not related to cancer and the researchers think they could possibly be related to the trial treatment. Trial doctors kept track of DLTs that happened:
  - During the first treatment cycle for participants in Group 4 (**MBG453** alone)
  - During the first 2 treatment cycles for participants Groups 1, 2, 3, 5, and 6

  If a participant did not complete the number of treatment cycles above, researchers did not include them in the DLT results in the tables on the next pages.
- **To pause a trial treatment**, which means stopping a treatment for a period of time before receiving it again. This is called a dose interruption.
- **To lower the dose of a trial treatment,** which means receiving a smaller amount or receiving it less often. This is called a dose reduction.

How many participants had a DLT, paused treatment, or had to lower the dose?

Group 1: 400 mg PDR001 every 4 weeks and decitabine

Participants with AML or MDS

	400 mg PDR001 + decitabine newly diagnosed AML 1 participant	400 mg PDR001 + decitabine relapsed/refractory AML 12 participants	400 mg PDR001 + decitabine high/very high risk MDS 3 participants
All DLTs	**0 of 1** 0%	**1 of 10** 10%	**0 of 3** 0%
Lower the dose of PDR001	**0 of 1** 0%	**0 of 12** 0%	**0 of 3** 0%
Pause treatment with PDR001	**0 of 1** 0%	**4 of 12** 33%	**2 of 3** 67%
Lower the dose of decitabine	**0 of 1** 0%	**1 of 12** 8%	**0 of 3** 0%
Pause treatment with decitabine	**0 of 1** 0%	**5 of 12** 42%	**0 of 3** 0%

1 participant with AML in Group 1 had a DLT of **severe inflammation (swelling) in the spinal cord** (myelitis)

Group 2: 240 or 400 mg MBG453 every 2 weeks, or 800 mg MBG453 every 4 weeks and decitabine

Participants with AML

	240 mg MBG453 every 2 weeks + decitabine newly diagnosed AML 3 participants	240 mg MBG453 every 2 weeks + decitabine relapsed/refractory AML 9 participants	400 mg MBG453 every 2 weeks + decitabine newly diagnosed AML 12 participants	400 mg MBG453 every 2 weeks + decitabine relapsed/refractory AML 11 participants	800 mg MBG453 every 4 weeks + decitabine newly diagnosed AML 7 participants	800 mg MBG453 every 4 weeks + decitabine relapsed/refractory AML 9 participants
All DLTs	**1 of 3** 33%	**0 of 9** 0%	**0 of 8** 0%	**0 of 8** 0%	**0 of 6** 0%	**0 of 7** 0%
Lower the dose of MBG453	**1 of 3** 33%	**0 of 9** 0%	**0 of 12** 0%	**0 of 11** 0%	**0 of 7** 0%	**0 of 9** 0%
Pause treatment with MBG453	**2 of 3** 67%	**2 of 9** 22%	**3 of 12** 25%	**2 of 11** 18%	**3 of 7** 43%	**0 of 9** 0%
Lower the dose of decitabine	**0 of 3** 0%	**1 of 9** 11%	**0 of 12** 0%	**0 of 11** 0%	**1 of 7** 14%	**0 of 9** 0%
Pause treatment with decitabine	**2 of 3** 67%	**2 of 9** 22%	**3 of 12** 25%	**1 of 11** 9%	**5 of 7** 71%	**1 of 9** 11%

1 participant with AML in Group 2 had a DLT of **severe inflammation (swelling) in the liver** (hepatitis)

Participants with MDS

	240 mg MBG453 every 2 weeks + decitabine high/very high risk MDS 9 participants	400 mg MBG453 every 2 weeks + decitabine high/very high risk MDS 4 participants	400 mg MBG453 every 2 weeks + decitabine intermediate risk MDS 5 participants	800 mg MBG453 every 4 weeks + decitabine high/very high risk MDS 6 participants	800 mg MBG453 every 4 weeks + decitabine intermediate risk MDS 2 participants
All DLTs	0 of 8 0%	0 of 3 0%	0 of 5 0%	0 of 4 0%	0 of 1 0%
Lower the dose of MBG453	0 of 9 0%	0 of 4 0%	0 of 5 0%	1 of 6 17%	0 of 2 0%
Pause treatment with MBG453	4 of 9 44%	4 of 4 100%	1 of 5 20%	3 of 6 50%	0 of 2 0%
Lower the dose of decitabine	1 of 9 11%	0 of 4 0%	0 of 5 0%	1 of 6 17%	0 of 2 0%
Pause treatment with decitabine	3 of 9 33%	3 of 4 75%	3 of 5 60%	3 of 6 50%	0 of 2 0%

Participants with CMML

	240 mg MBG453 every 2 weeks + decitabine CMML 1 participant	400 mg MBG453 every 2 weeks + decitabine CMML 3 participants	800 mg MBG453 every 4 weeks + decitabine CMML 1 participant
All DLTs	0 of 1 0%	0 of 2 0%	0 of 1 0%
Lower the dose of MBG453	0 of 1 0%	0 of 3 0%	0 of 1 0%
Pause treatment with MBG453	1 of 1 100%	2 of 3 67%	0 of 1 0%
Lower the dose of decitabine	0 of 1 0%	1 of 3 33%	0 of 1 0%
Pause treatment with decitabine	1 of 1 100%	3 of 3 100%	1 of 1 100%

Group 3: 160, 240, or 400 mg MBG453 every 2 weeks and 400 mg PDR001 and decitabine

Participants with AML

	160 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 3 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine newly diagnosed AML 2 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine newly diagnosed AML 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 3 participants
All DLTs	1 of 2 50%	0 of 1 0%	0 of 1 0%	0 of 2 0%	0 of 2 0%
Lower the dose of MBG453	0 of 3 0%	0 of 2 0%	0 of 2 0%	0 of 2 0%	0 of 3 0%
Pause treatment with MBG453	0 of 3 0%	0 of 2 0%	0 of 2 0%	2 of 2 100%	2 of 3 67%
Lower the dose of PDR001	0 of 3 0%	0 of 2 0%	0 of 2 0%	0 of 2 0%	0 of 3 0%
Pause treatment with PDR001	1 of 3 33%	0 of 2 0%	0 of 2 0%	1 of 2 50%	2 of 3 67%
Lower the dose of decitabine	0 of 3 0%	0 of 2 0%	0 of 2 0%	0 of 2 0%	0 of 3 0%
Pause treatment with decitabine	1 of 3 33%	0 of 2 0%	1 of 2 50%	2 of 2 100%	2 of 3 67%

1 participant with AML in Group 3 had a DLT of **severe inflammation (swelling) around the kidneys** (tubulointerstitial nephritis)

Participants with MDS

	160 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 3 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 1 participant
All DLTs	0 of 1 0%	0 of 2 0%	1 of 1 100%
Lower the dose of MBG453	0 of 3 0%	0 of 2 0%	0 of 1 0%
Pause treatment with MBG453	1 of 3 33%	1 of 2 50%	1 of 1 100%
Lower the dose of PDR001	0 of 3 0%	0 of 2 0%	0 of 1 0%
Pause treatment with PDR001	1 of 3 33%	1 of 2 50%	0 of 1 0%
Lower the dose of decitabine	0 of 3 0%	0 of 2 0%	1 of 1 100%
Pause treatment with decitabine	1 of 3 33%	1 of 2 50%	1 of 1 100%

1 participant with MDS in Group 3 had a DLT of **severe inflammation (swelling) in the eye** (uveitis)

Group 4: 400 or 1,200 mg MBG453 every 2 weeks

Participants with AML and MDS

	400 mg MBG453 every 2 weeks relapsed/refractory AML 10 participants	1,200 mg MBG453 every 2 weeks relapsed/refractory AML 6 participants	400 mg MBG453 every 2 weeks high/very high risk MDS 3 participants	1,200 mg MBG453 every 2 weeks high/very high risk MDS 5 participants	1,200 mg MBG453 every 2 weeks intermediate risk MDS 2 participants
All DLTs	0 of 7 0%	0 of 6 0%	0 of 3 0%	0 of 5 0%	0 of 2 0%
Lower the dose of MBG453	0 of 10 0%	0 of 6 0%	0 of 3 0%	0 of 5 0%	0 of 2 0%
Pause treatment with MBG453	2 of 10 20%	0 of 6 0%	0 of 3 0%	0 of 5 0%	0 of 2 0%

Group 5: 80 or 240 mg MBG453 every 2 weeks and 400 mg PDR001

Participants with AML and MDS

	80 mg MBG453 every 2 weeks + 400 mg PDR001 relapsed/refractory AML 1 participant	240 mg MBG453 every 2 weeks + 400 mg PDR001 relapsed/refractory AML 5 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 high/very high risk MDS 5 participants
All DLTs	0 of 0	1 of 3 33%	0 of 5 0%
Lower the dose of MBG453	0 of 1 0%	0 of 5 0%	0 of 5 0%
Pause treatment with MBG453	0 of 1 0%	1 of 5 20%	1 of 5 20%
Lower the dose of PDR001	0 of 1 0%	0 of 5 0%	0 of 5 0%
Pause treatment with PDR001	0 of 1 0%	1 of 5 20%	1 of 5 20%

1 participant with AML in Group 5 had a DLT of **severe inflammation (swelling) in the brain** (encephalitis)

Group 6: 240 or 400 mg every 2 weeks, or 800 mg MBG453 every 4 weeks and azacitidine

Participants with AML

	240 mg MBG453 every 2 weeks + azacitidine newly diagnosed AML 6 participants	400 mg MBG453 every 2 weeks + azacitidine newly diagnosed AML 14 participants	800 mg MBG453 every 4 weeks + azacitidine newly diagnosed AML 6 participants
All DLTs	0 of 6 0%	0 of 10 0%	0 of 4 0%
Lower the dose of MBG453	1 of 6 17%	0 of 14 0%	0 of 6 0%
Pause treatment with MBG453	4 of 6 67%	5 of 14 36%	1 of 6 17%
Lower the dose of azacitidine	0 of 6 0%	1 of 14 7%	1 of 6 17%
Pause treatment with azacitidine	3 of 6 50%	7 of 14 50%	4 of 6 67%

Participants with MDS

	240 mg MBG453 every 2 weeks + azacitidine high/very high risk MDS 3 participants	240 mg MBG453 every 2 weeks + azacitidine intermediate risk MDS 2 participants	400 mg MBG453 every 2 weeks + azacitidine high/very high risk MDS 14 participants	400 mg MBG453 every 2 weeks + azacitidine intermediate risk MDS 5 participants	800 mg MBG453 every 4 weeks + azacitidine high/very high risk MDS 17 participants	800 mg MBG453 every 4 weeks + azacitidine intermediate risk MDS 2 participants
All DLTs	0 of 2 0%	0 of 2 0%	0 of 14 0%	0 of 4 0%	0 of 15 0%	1 of 2 50%
Lower the dose of MBG453	0 of 3 0%	0 of 2 0%	0 of 14 0%	0 of 5 0%	0 of 17 0%	0 of 2 0%
Pause treatment with MBG453	0 of 3 0%	0 of 2 0%	4 of 14 29%	0 of 5 0%	6 of 17 35%	0 of 2 0%
Lower the dose of azacitidine	0 of 3 0%	0 of 2 0%	0 of 14 0%	0 of 5 0%	3 of 17 18%	0 of 2 0%
Pause treatment with azacitidine	0 of 3 0%	0 of 2 0%	4 of 14 29%	0 of 5 0%	7 of 17 41%	0 of 2 0%

1 participant with MDS in Group 6 had a DLT of **sweet syndrome** (acute febrile neutrophilic dermatosis)

Participants with CMML

	400 mg MBG453 every 2 weeks + azacitidine CMML 5 participants	800 mg MBG453 every 4 weeks + azacitidine CMML 5 participants
All DLTs	0 of 5 0%	0 of 4 0%
Lower the dose of MBG453	0 of 5 0%	0 of 5 0%
Pause treatment with MBG453	0 of 5 0%	2 of 5 40%
Lower the dose of azacitidine	0 of 5 0%	0 of 5 0%
Pause treatment with azacitidine	0 of 5 0%	2 of 5 40%

What medical problems, also called adverse events, happened during this trial?

Trial doctors keep track of all medical problems called **adverse events** that happen in trials, even if they think the adverse events are not related to the trial treatments.

Many trials are needed to know if a drug or treatment causes an adverse event.

This section is a summary of the adverse events that happened from the start of treatment up to:

- 30 days after their last dose of **azacitidine** or **decitabine**
- 150 days after their last dose of **PDR001** or **MBG453**

An **adverse event** is:

- Any **sign or symptom** that the participants have during a trial
- Considered **serious** when it is life-threatening, causes lasting problems, the participant needs hospital care, or results in death

Adverse events **may** or **may not** be caused by treatments in the trial.

Almost all the 241 participants had adverse events. 170 participants had adverse events that were considered serious. 104 participants died. 11 participants left the trial due to an adverse event. The researchers concluded there were no unexpected safety concerns for **PDR001** and **MBG453** given with or without other drugs in this trial.

How many participants had adverse events?

Group 1: 400 mg PDR001 and decitabine

Participants with AML and MDS

	400 mg PDR001 + decitabine newly diagnosed AML 1 participant	400 mg PDR001 + decitabine relapsed/refractory AML 12 participants	400 mg PDR001 + decitabine high/very high risk MDS 3 participants
Had at least 1 serious adverse event	1 of 1 100%	11 of 12 92%	3 of 3 100%
Had at least 1 adverse event	1 of 1 100%	11 of 12 92%	3 of 3 100%
Left the trial due to an adverse event	1 of 1 100%	2 of 12 17%	0 of 3 0%
Died	1 of 1 100%	6 of 12 50%	1 of 3 33%

Group 2: 240 or 400 mg every 2 weeks, or 800 mg MBG453 every 4 weeks and decitabine

Participants with AML

	240 mg MBG453 every 2 weeks + decitabine newly diagnosed AML 3 participants	240 mg MBG453 every 2 weeks + decitabine relapsed/refractory AML 9 participants	400 mg MBG453 every 2 weeks + decitabine newly diagnosed AML 12 participants	400 mg MBG453 every 2 weeks + decitabine relapsed/refractory AML 11 participants	800 mg MBG453 every 4 weeks + decitabine newly diagnosed AML 7 participants	800 mg MBG453 every 4 weeks + decitabine relapsed/refractory AML 9 participants
Had at least 1 serious adverse event	3 of 3 100%	8 of 9 89%	10 of 12 83%	8 of 11 73%	5 of 7 71%	7 of 9 78%
Had at least 1 adverse event	3 of 3 100%	9 of 9 100%	12 of 12 100%	11 of 11 100%	7 of 7 100%	9 of 9 100%
Left the trial due to an adverse event	0 of 3 0%	0 of 9 0%	0 of 12 0%	0 of 11 0%	2 of 7 29%	0 of 9 0%
Died	1 of 3 33%	7 of 9 78%	5 of 12 42%	9 of 11 82%	4 of 7 57%	6 of 9 67%

Participants with MDS

	240 mg MBG453 every 2 weeks + decitabine high/very high risk MDS 9 participants	400 mg MBG453 every 2 weeks + decitabine high/very high risk MDS 4 participants	400 mg MBG453 every 2 weeks + decitabine intermediate risk MDS 5 participants	800 mg MBG453 every 4 weeks + decitabine high/very high risk MDS 6 participants	800 mg MBG453 every 4 weeks + decitabine intermediate risk MDS 2 participants
Had at least 1 serious adverse event	7 of 9 78%	3 of 4 75%	4 of 5 80%	3 of 6 50%	2 of 2 100%
Had at least 1 adverse event	9 of 9 100%	4 of 4 100%	5 of 5 100%	6 of 6 100%	2 of 2 100%
Left the trial due to an adverse event	0 of 9 0%	0 of 4 0%	1 of 5 20%	0 of 6 0%	0 of 2 0%
Died	1 of 9 11%	0 of 4 0%	1 of 5 20%	1 of 6 17%	0 of 2 0%

Participants with CMML

	240 mg MBG453 every 2 weeks + decitabine CMML 1 participant	400 mg MBG453 every 2 weeks + decitabine CMML 3 participants	800 mg MBG453 every 4 weeks + decitabine CMML 1 participant
Had at least 1 serious adverse event	0 of 1 0%	3 of 3 100%	1 of 1 100%
Had at least 1 adverse event	1 of 1 100%	3 of 3 100%	1 of 1 100%
Left the trial due to an adverse event	0 of 1 0%	0 of 3 0%	0 of 1 0%
Died	1 of 1 100%	1 of 3 33%	1 of 1 100%

Group 3: 160, 240, or 400 mg MBG453 every 2 weeks and 400 mg PDR001 and decitabine

Participants with AML

	160 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 3 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine newly diagnosed AML 2 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine newly diagnosed AML 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine relapsed/refractory AML 3 participants
Had at least 1 serious adverse event	2 of 3 67%	2 of 2 100%	2 of 2 100%	2 of 2 100%	2 of 3 67%
Had at least 1 adverse event	3 of 3 100%	2 of 2 100%	2 of 2 100%	2 of 2 100%	3 of 3 100%
Left the trial due to an adverse event	0 of 3 0%	0 of 2 0%	0 of 2 0%	0 of 2 0%	0 of 3 0%
Died	2 of 3 67%	2 of 2 100%	0 of 2 0%	1 of 2 50%	2 of 3 67%

Participants with MDS

	160 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 3 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 2 participants	400 mg MBG453 every 2 weeks + 400 mg PDR001 + decitabine high/very high risk MDS 1 participant
Had at least 1 serious adverse event	3 of 3 100%	2 of 2 100%	1 of 1 100%
Had at least 1 adverse event	3 of 3 100%	2 of 2 100%	1 of 1 100%
Left the trial due to an adverse event	0 of 3 0%	0 of 2 0%	0 of 1 0%
Died	1 of 3 33%	0 of 2 0%	0 of 1 0%

Group 4: 400 or 1,200 mg MBG453 every 2 weeks

Participants with AML and MDS

	400 mg MBG453 every 2 weeks relapsed/refractory AML 10 participants	1,200 mg MBG453 every 2 weeks relapsed/refractory AML 6 participants	400 mg MBG453 every 2 weeks high/very high risk MDS 3 participants	1,200 mg MBG453 every 2 weeks high/very high risk MDS 5 participants	1,200 mg MBG453 every 2 weeks intermediate risk MDS 2 participants
Had at least 1 serious adverse event	9 of 10 90%	4 of 6 67%	1 of 3 33%	2 of 5 40%	1 of 2 50%
Had at least 1 adverse event	10 of 10 100%	6 of 6 100%	3 of 3 100%	5 of 5 100%	2 of 2 100%
Left the trial due to an adverse event	1 of 10 10%	1 of 6 17%	0 of 3 0%	0 of 5 0%	0 of 2 0%
Died	10 of 10 100%	3 of 6 50%	0 of 3 0%	2 of 5 40%	0 of 2 0%

Group 5: 80 or 240 mg MBG453 every 2 weeks and 400 mg PDR001

Participants with AML and MDS

	80 mg MBG453 every 2 weeks + 400 mg PDR001 relapsed/refractory AML 1 participant	240 mg MBG453 every 2 weeks + 400 mg PDR001 relapsed/refractory AML 5 participants	240 mg MBG453 every 2 weeks + 400 mg PDR001 high/very high risk MDS 5 participants
Had at least 1 serious adverse event	0 of 1 0%	5 of 5 100%	3 of 5 60%
Had at least 1 adverse event	1 of 1 100%	5 of 5 100%	5 of 5 100%
Left the trial due to an adverse event	0 of 1 0%	0 of 5 0%	1 of 5 20%
Died	1 of 1 100%	5 of 5 100%	2 of 5 40%

Group 6: 240 or 400 mg every 2 weeks, or 800 mg MBG453 every 4 weeks and azacitidine

Participants with AML

	240 mg MBG453 every 2 weeks + azacitidine newly diagnosed AML 6 participants	400 mg MBG453 every 2 weeks + azacitidine newly diagnosed AML 14 participants	800 mg MBG453 every 2 weeks + azacitidine newly diagnosed AML 6 participants
Had at least 1 serious adverse event	3 of 6 50%	10 of 14 71%	4 of 6 67%
Had at least 1 adverse event	6 of 6 100%	14 of 14 100%	6 of 6 100%
Left the trial due to an adverse event	0 of 6 0%	0 of 14 0%	1 of 6 17%
Died	2 of 6 33%	8 of 14 57%	4 of 6 67%

Participants with MDS

	240 mg MBG453 every 2 weeks + azacitidine high/very high risk MDS 3 participants	240 mg MBG453 every 2 weeks + azacitidine intermediate risk MDS 2 participants	400 mg MBG453 every 2 weeks + azacitidine high/very high risk MDS 14 participants	400 mg MBG453 every 2 weeks + azacitidine intermediate risk MDS 5 participants	800 mg MBG453 every 4 weeks + azacitidine high/very high risk MDS 17 participants	800 mg MBG453 every 4 weeks + azacitidine intermediate risk MDS 2 participants
Had at least 1 serious adverse event	1 of 3 33%	0 of 2 0%	5 of 14 36%	2 of 5 40%	12 of 17 71%	1 of 2 50%
Had at least 1 adverse event	3 of 3 100%	2 of 2 100%	14 of 14 100%	5 of 5 100%	17 of 17 100%	2 of 2 100%
Left the trial due to an adverse event	0 of 3 0%	0 of 2 0%	0 of 14 0%	0 of 5 0%	0 of 17 0%	1 of 2 50%
Died	1 of 3 33%	0 of 2 0%	4 of 14 29%	1 of 5 20%	4 of 17 24%	0 of 2 0%

Participants with CMML

	400 mg MBG453 every 2 weeks + azacitidine CMML 5 participants	800 mg MBG453 every 4 weeks + azacitidine CMML 5 participants
Had at least 1 serious adverse event	3 of 5 60%	1 of 5 20%
Had at least 1 adverse event	5 of 5 100%	5 of 5 100%
Left the trial due to an adverse event	0 of 5 0%	0 of 5 0%
Died	1 of 5 20%	0 of 5 0%

Other participants: Decitabine or azacitidine alone

	Decitabine only Any blood cancer 5 participants	Azacitidine only Any blood cancer 4 participants
Had at least 1 serious adverse event	4 of 5 80%	4 of 4 100%
Had at least 1 adverse event	5 of 5 100%	4 of 4 100%
Left the trial due to an adverse event	0 of 5 0%	0 of 4 0%
Died	2 of 5 40%	0 of 4 0%

What serious adverse events did the participants have?

104 of 241 participants died during this trial. The most common cause of death was cancer.

170 participants had serious adverse events. The most common serious adverse event that happened in **50 or more** participants was:

- **Fever with low levels of white blood cells** (febrile neutropenia)

Additional serious adverse events happened in fewer participants.

What other adverse events did the participants have?

237 participants had other adverse events.

The most common other adverse events that happened in **80 or more** participants were:

- **Feeling sick to the stomach** (nausea)
- **Unable to have regular bowel movements** (constipation)
- **Low levels of red blood cells** (anemia)
- **Low levels of platelets** (thrombocytopenia)
- **Low levels of a type of white blood cell** (neutropenia)

Additional adverse events happened in fewer participants.

What was learned from this trial?

Researchers learned about the safety of **MBG453** and **PDR001** given together and with other drugs in people with certain blood cancers. They also to learned about the safety of **MBG453** given alone.

The researchers concluded that:

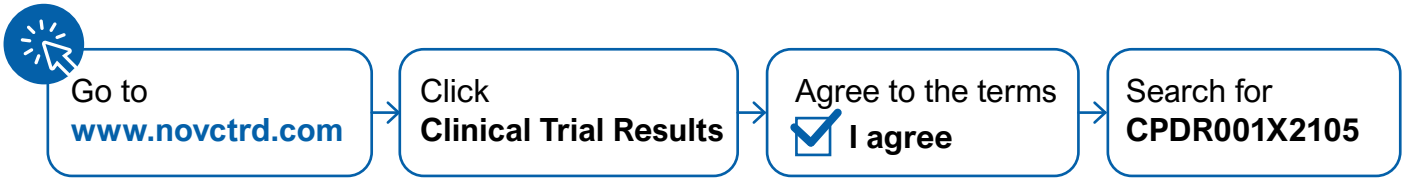
- With other drugs, the highest safe dose of **MBG453** was 400 mg every 2 weeks or 800 mg every 4 weeks
- There were no unexpected safety concerns for **PDR001** and **MBG453** given with or without other drugs in this trial

When this summary was written, the sponsor had no plans for future trials of **PDR001** and **MBG453** given with or without other drugs in people with certain types of AML, MDS, or CMML.

Where can I learn more about this trial?

More information about the results and adverse events in this trial can be found in the scientific summary of the results available on the Novartis Clinical Trial Results website **www.novctrd.com**

Follow these steps to find the scientific summary:



For more information about this trial, go to this website:

- clinicaltrials.gov – search using the number **NCT03066648**

Other trials of **PDR001** and **MBG453** may appear on the public websites above. When there, search for **PDR001**, **spartalizumab, MBG453**, or **sabatolimab.**

Full clinical trial title: Phase 1b, multi-arm, open-label study of PDR001 and/or MBG453 in combination with decitabine in patients with acute myeloid leukemia or high risk myelodysplastic syndrome

NOVARTIS

Novartis is a global healthcare company based in Switzerland that provides solutions to address the evolving needs of patients worldwide.

1-888-669-6682 (US) | +41-61-324 1111 (EU)

www.novartis.com/clinicaltrials