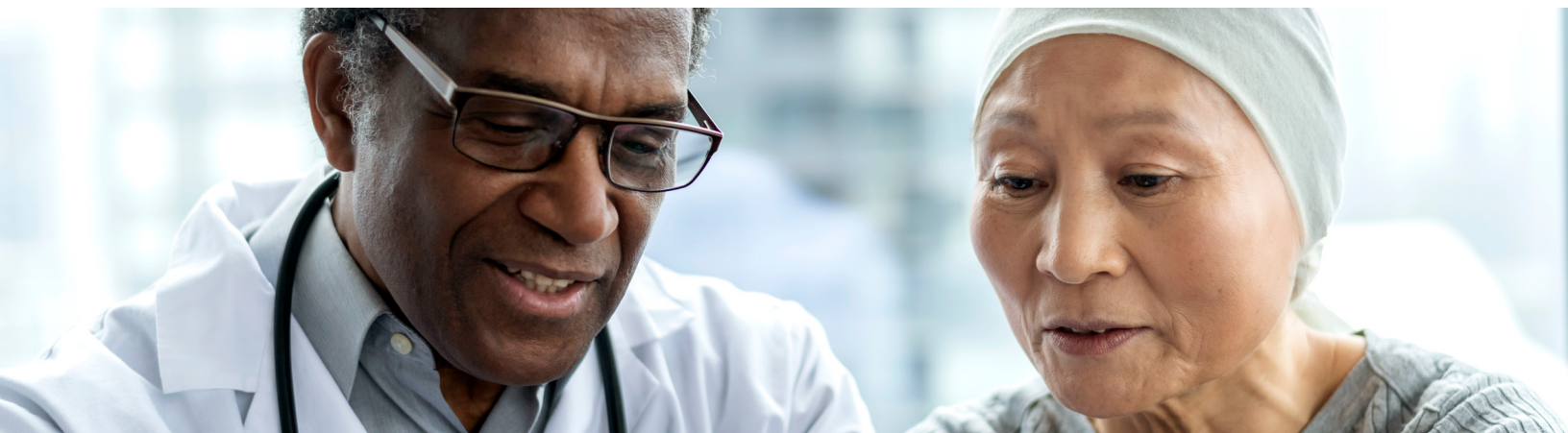


Summary of Clinical Trial Results

For Laypersons



A study to learn how the body handles venetoclax plus pomalidomide and dexamethasone in adult patients with relapsed or refractory multiple myeloma

Overall Summary

- Multiple myeloma (MM) is a cancer of the blood's plasma (a type of white blood cell in the bone marrow).
- Researchers are looking for a better way to treat adult patients with MM that has relapsed (come back) or was refractory (did not get better after earlier treatment).
- In this study, doctors wanted to know whether a drug called venetoclax when used in combination with the standard treatment of pomalidomide and dexamethasone (PomDex) could help treat MM.
- This study took place from December 2018 to June 2020 in Spain, the United Kingdom and the United States.
- The main goals of the study were to find out how the body handles venetoclax, to find out the highest and safest dose of venetoclax when given with PomDex in MM patients, if patients experienced medical events after treatment with venetoclax, and if treatment with venetoclax lowers the amount of cancer in the body.
- Due to results of another venetoclax study in patients with MM that showed an increase in deaths, all venetoclax MM studies were suspended. Some venetoclax MM studies resumed but researchers chose to end this study early to focus efforts on other studies.
- 100% of patients (8 patients) had side effects. The most common side effects were abnormally low levels of a type of white blood cell called neutrophils (neutropenia), low levels of red blood cells (anemia), and high blood sugar (hyperglycemia).
- At the time the study ended, 5 patients (62.5% of patients) had less cancer in the body.
- The results of this specific study will not be used by researchers to further develop venetoclax in combination with PomDex because it ended early, but other studies of venetoclax in MM are ongoing.
- If you participated in this study and have questions about your individual care, contact the doctor or staff at your study site.

1. General information about the study

1.1. What was the main objective of this study?



Multiple myeloma (MM) is a cancer of the plasma (a type of white blood cell in the bone marrow). Although many patients' cancer improves with their first treatment, the cancer can come back again (relapsed) or not get better with treatment (refractory).

Researchers are looking for a better way to treat MM and wanted to know whether a new drug called venetoclax could help treat relapsed or refractory MM. Venetoclax helps to stop cancer cells from repairing themselves by blocking a type of abnormal protein and causing cancer cells to die and is approved to treat other cancers. Venetoclax is also being tested in patients with other types of cancer.

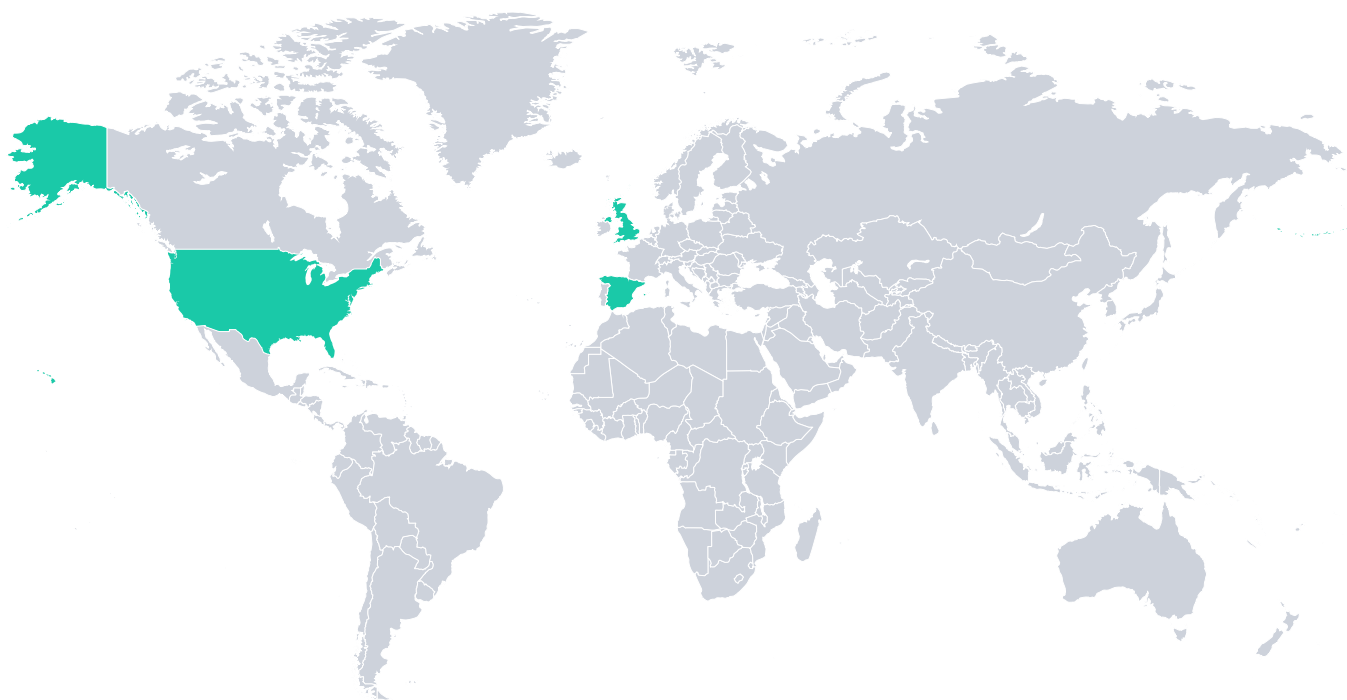
The main goal of the study was to find out how the body handles venetoclax, to find the highest and safest dose of venetoclax that patients could take with standard MM treatment with pomalidomide and dexamethasone (PomDex), see if patients had any medical events when receiving treatment with venetoclax, and to see if treatment with venetoclax lowers the amount of cancer in the body.

Medical events may or may not have been related to taking the study drug. Side effects are unwanted medical events that were considered by the study doctor to be at least possibly related to taking the study drug. Both medical events and side effects can range from very mild to severe.

Researchers planned this study as a Phase 2, open-label study. Phase 2 studies test potential new treatments in a small number of patients with a condition or disease. The study was "open-label" which means that both patients and study doctors knew which dose of venetoclax was given to patients.

1.2. When and where was the study done?

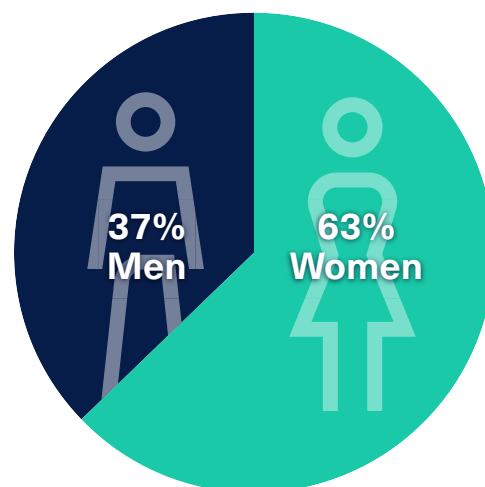
This study took place from December 2018 to June 2020 in the following countries: Spain, the United Kingdom, and the United States.



2. What patients were included in this study?

This study included 2 parts: Part 1 (Dose Escalation) and Part 2 (Safety Expansion). Eight adult patients with MM that had come back or gotten worse during or after their last treatment joined Part 1. The study ended early before Part 1 finished so no patients joined Part 2.

At the time the study ended, there were more women (63%) than men (37%) enrolled.



3. Which medicines were studied?

The medicines in this study were venetoclax in combination with the standard treatment of PomDex. All patients took tablets of venetoclax and PomDex by mouth. The diagram below shows how the study was organized.



The study was divided into 2 parts: Part 1 (Dose Escalation) and Part 2 (Safety Expansion).

In Part 1, patients were to be given increasing doses of venetoclax in combination with PomDex after monitoring side effects and tolerability in the lower doses. This part of the study planned to evaluate patients to determine the highest dose of venetoclax that patients could tolerate in order to find a dose that should be given to a new set of patients in Part 2.

The study ended before patients finished Part 1 and no patients joined Part 2.

4. What were the side effects?

Side effects are unwanted medical events that were considered by the study doctor to be at least possibly related to study drug.

A side effect is serious if it leads to death, is life-threatening, puts a patient in the hospital, keeps a patient in the hospital for a long time, or causes a disability that lasts a long time.

50.0% of patients (4 patients) had serious side effects during the study.

No patient stopped taking the study drug because of side effects during the study.

No patient died during the study due to side effects.

The table below shows information about the serious side effects patients had in the study.

	Venetoclax (8 Patients)
Number of patients with serious side effects	4 (50.0% of patients)
Serious Side Effects	
• Increased lactate dehydrogenase (LDH) in the blood (may be sign of tissue damage)	1 (12.5%)
• Lower respiratory tract infection (infection in lower lung) and stroke	1 (12.5%)
• Pneumococcus sinusitis (inflammation of sinus tissue due to bacteria)	1 (12.5%)
• Pneumonia (infection causing the lungs to fill with fluid or pus)	1 (12.5%)

100% of patients (8 patients) had side effects during the study. The table below shows information about the common side effects (in at least 3 or more patients) in this study. The most common side effects were abnormally low levels of a type of white blood cell called neutrophils (neutropenia), low level of red blood cells (anemia), and high blood sugar (hyperglycemia).

	Venetoclax (8 Patients)
Number of patients with at least one side effect	8 (100.0% of patients)
Abnormally low level of a type of white blood cell called neutrophils (neutropenia)	6 (75.0%)
Low level of red blood cells (anemia)	4 (50.0%)
High blood sugar (hyperglycemia)	3 (37.5%)
Low level of white blood cell count (leukopenia)	2 (25.0%)
White blood cells missing from the blood (lymphopenia)	2 (25.0%)
Decrease in number of platelets in the blood (thrombocytopenia)	2 (25.0%)
Tiredness (fatigue)	2 (25.0%)
Decreased white blood cell count	2 (25.0%)

5. What were the overall results of the study?

The study ended early because of results of another study of venetoclax. The study sponsor decided not to continue with this study. Because of the low number of patients included in the study before it ended early, study doctors may not know answers to some of the questions being studied.

This study looked at the number of medical events in patients after starting treatment with venetoclax as well as whether treatment with venetoclax lowered the amount of cancer in the body (overall response rate). Medical events may or may not have been related to treatment.

- 100% of patients (8 patients) had medical events during the study with the most common medical events being abnormally low levels of a type of white blood cell called neutrophils (neutropenia), low levels of red blood cells (anemia), tiredness, low levels of potassium in the blood (hypokalemia).
- Treatment with venetoclax in combination with the standard MM treatment of PomDex showed an overall response rate (ORR) of 62.5% which is an improvement in ORR (approximately 32%) seen in patients treated with PomDex alone in other studies.

6. How has the study helped patients and researchers?

The study was limited as only a small number of patients took part and the study ended early. Therefore, generalizing the results from this study for the larger MM patient population may not be appropriate. Additional studies are needed to learn more about the use of venetoclax in MM patients.

This summary only shows the results from this study, which may be different from the results of other studies.

7. Are there any plans for future studies?

Multiple studies of venetoclax are ongoing and additional studies may be considered.

8. Who sponsored this study?

This study was sponsored by AbbVie. This summary was reviewed for readability by a patient advocacy group.



9. Where can I find out more information about this study?

Title of Study	A Phase 2, Open-Label, Multicenter, Dose-Escalation and Expansion Study of Venetoclax in Combination With Pomalidomide and Dexamethasone in Subjects With Relapsed or Refractory Multiple Myeloma
Protocol Number	M16-085
Clinicaltrials.gov	NCT03567616 https://clinicaltrials.gov/ct2/show/NCT03567616?term=M16-085&draw=2&rank=1
EudraCT	2017-004232-11 https://www.clinicaltrialsregister.eu/ctr-search/search?query=2017-004232-11
Study Sponsor	AbbVie, Inc. Phone: +1 800-633-9110 Email: abbvieclinicaltrials@abbvie.com

Thank You

AbbVie wants to thank all the participants for their time and effort that went into making this study possible.

Clinical study participants help advance science!

