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Summary of Clinical Trial Results

For Laypersons

An extension study to learn how safe and effective a medicine containing tilavonemab is to treat Progressive Supranuclear Palsy (PSP)

Overall Summary

- Researchers are looking for a better way to treat Progressive Supranuclear Palsy (PSP).
- Researchers in this study wanted to know whether tilavonemab, an investigational drug not yet approved by regulatory authorities, could help treat PSP.
- This study took place from January 2018 to December 2019 in 4 countries.
- This study is a continuation of the first study (M15-562) conducted in humans that gave different doses of tilavonemab to patients with PSP. A total of 142 adult patients from the parent study, M15-562, took study drug and all patients left the study. This was due to the study sponsor ending the study early because tilavonemab showed a lack of effectiveness.
- The study was divided into 2 parts: Treatment Period and Post-Treatment Follow-Up Period.
- Patients who received tilavonemab in the M15-562 study continued to get the same dose in the extension study. While patients from the M15-562 placebo (no medicine) group were randomly (by chance) assigned to tilavonemab (lower or higher doses).
- This study found that treatment with tilavonemab did not significantly improve symptoms of PSP. As a result, this study was ended early.
- Side effects in this patient population were similar to side effects seen in patients with PSP.
- Both dose levels of tilavonemab appeared to be safe and well-tolerated.
- The results of the study may be used by researchers to further develop this medicine in other diseases or conditions.
- 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?

Researchers are looking for a better way to treat Progressive Supranuclear Palsy (PSP). PSP is a rare brain disease that occurs when cells in specific brain areas that control body movement, thinking, and behavior become damaged or die. This may be caused by a protein called "tau" that is naturally present in the brain but can become abnormal and build up in cells. Symptoms of PSP continue to worsen, preventing patients from completing their daily tasks and living independently.

Researchers in this study wanted to know whether tilavonemab, an investigational drug not yet approved by regulatory authorities, could help treat PSP. Tilavonemab is an antibody, which is a protein produced by the body's immune (defense) system to fight off harmful substances. Tilavonemab is an anti-tau antibody previously studied in animals to lower the amount of tau protein in the brain. This study is a continuation of the first study (M15-562) conducted in humans that gave different doses of tilavonemab to patients with PSP. The researchers in this extension study wanted to test whether or not tilavonemab is effective and safe in patients with PSP.

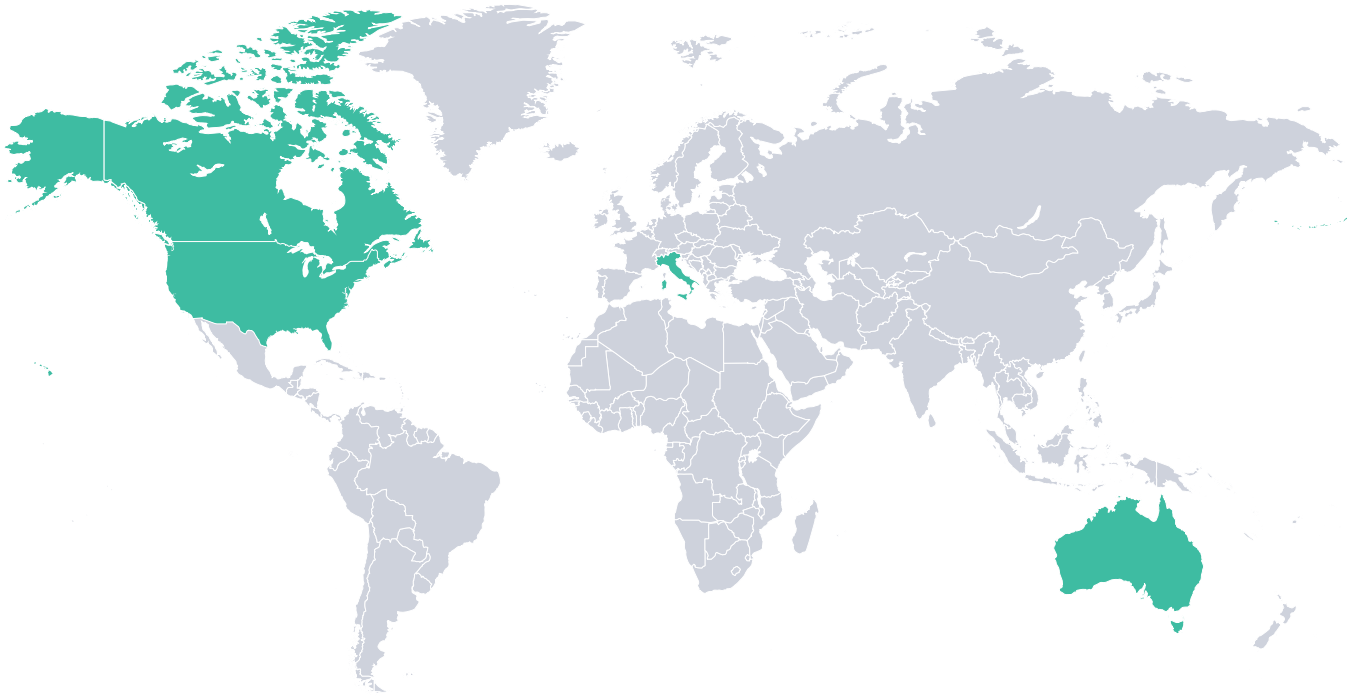
Researchers planned this study as a Phase 2, double-blind, multiple-dose randomized study.

- **Phase 2 studies** test potential new treatments in a small number of patients with a condition or disease. In this Phase 2 study, the study doctors looked at the benefits of multiple doses of tilavonemab in adult patients with PSP.
- This study **randomized** patients, which means that both patients who received placebo and patients who received tilavonemab in study M15-562 were randomly (by chance) assigned to tilavonemab dosing groups in this study.
- This study was also **"double-blinded"**, which means that neither the patients nor the study doctors knew who was given higher or lower doses of tilavonemab.

The main goal of the study was to find out whether treatment with tilavonemab is safe and effective in adult patients with PSP long-term. The study doctors also looked for any side effects patients might have had after treatment with the study drug. This summary only includes the results of this study, which may be different from the results from other studies for PSP.

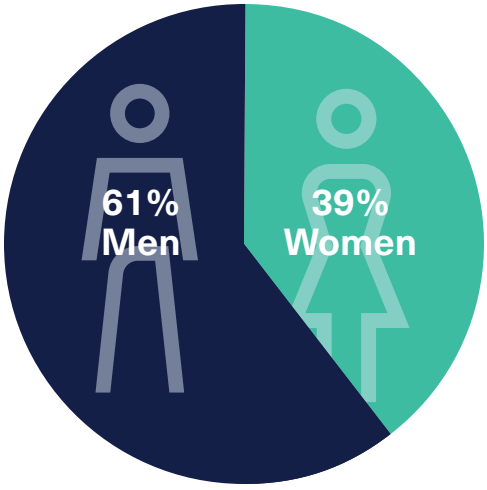
1.2. When and where was the study done?

This study took place from January 2018 to December 2019 in the following countries: United States, Canada, Italy, and Australia.



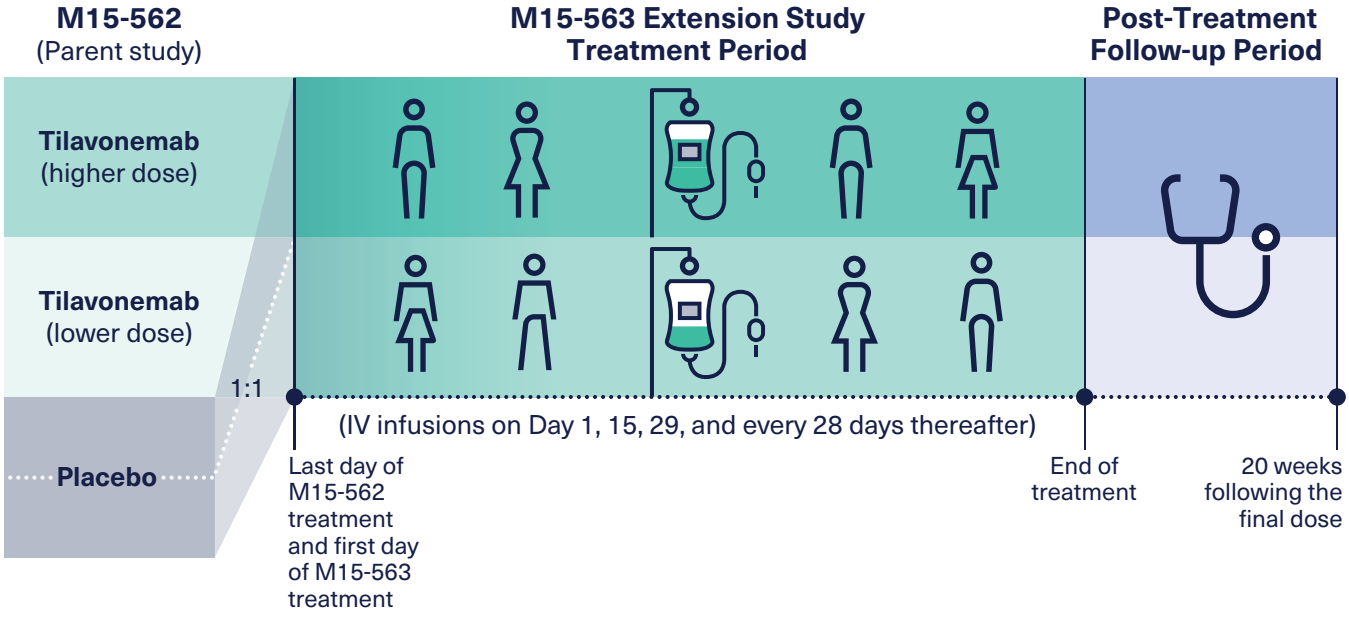
2. What patients were included in this study?

A total of 142 adult patients who completed the parent study (M15-562) took part in this study. All patients left the study, mostly due to the study sponsor ending the study early. More men (61%) than women (39%) participated in the study. Patients ranged from 55 to 87 years of age, with an average age of 70 years. Adult patients 55 years of age or older participated in this study, as this is a degenerative disease that affects mostly older people. To participate, patients must have completed the 52-week treatment period of the parent study (M15-562).



3. Which medicines were studied?

The medicine in this study was tilavonemab. The diagram below shows how the study was organized.



The study was divided into 2 parts: Treatment Period and Post-Treatment Follow-Up Period.

Study doctors checked if patients from the parent study, M15-562, met the entry criteria to join this extension study. The last visit of the parent study (M15-562) was the first day of the extension study (M15-563). Patients who received tilavonemab in the M15-562 study continued to get the same dose in this extension study, while patients who received a placebo (no medicine) in M15-562, were randomly (by chance) assigned to receive either the low dose or the high dose of tilavonemab. Neither the patients nor study doctors knew which dose patients were assigned to.

In the Treatment Period, patients received tilavonemab as an IV infusion into the vein for up to 76 weeks.

In the Post-Treatment Follow-Up Period, patients were followed for 20 weeks after the final dose of the Treatment Period.

This study was stopped early by the study sponsor as tilavonemab was not found effective to treat PSP in the parent study.

4. What were the side effects?

Side effects are unwanted medical events that happen during a study. They may or may not be caused by the treatment in the study, and they may or may not be related to the disease.

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.

Related side effects are side effects that were considered by the study doctor to be at least possibly related to the study treatment.

- About 21.1% of patients (30 patients) had serious side effects during the study. The total number of patients that had serious side effects considered possibly related to the study treatment was 1.4% of patients (2 patients).
- About 1.4% of patients (2 patients) stopped treatment because of side effects during the study. None were considered related to study treatment.
- A total of 13 patients (9.2% of patients) died during the study. None were considered related to study treatment.

The table below shows information about the related serious side effects patients had during the study, as well as related side effects patients had that led to the patient stopping the study drug, and related side effects leading to death.

Overall Study	
	All Patients (N= 142 patients)
Number of patients with related serious side effects	2 (1.4% of patients)
Related serious side effects	
• Upper respiratory tract infection (infection of the throat and upper airways)	1 (0.7% of patients)
• Partial seizures	1 (0.7% of patients)
Number of patients who stopped taking study drug because of related side effects	0 (0.0% of patients)
Number of related side effects leading to death	0 (0.0% of patients)

About 67.6% of patients (96 patients) had side effects during the study. The total number of patients that had side effects considered possibly related to the study drug was 24 patients (16.9% of patients).

The table below shows information about the common related side effects (in at least 2 or more patients overall) in this study. The most common related side effect was asthenia (weakness).

Overall Study	
	All Patients (N= 142 patients)
Number of patients with at least 1 related side effect	24 (16.9% of patients)
Related side effects in 2 or more patients overall	
• Asthenia (weakness)	3 (2.1% of patients)
• Pyrexia (fever)	2 (1.4% of patients)
• Fall	2 (1.4% of patients)
• Skin laceration (cut or wound on the skin)	2 (1.4% of patients)

5. What were the overall results of the study?

The parent study (M15-562) found that treatment with tilavonemab did not significantly improve symptoms of PSP. As a result, this study was ended early. The effect of the study drug on symptoms was measured using a specific rating scale known as the Progressive Supranuclear Palsy Rating Scale (PSPRS), which measured changes in PSP symptoms from before study treatment through Week 52. This scale measures the impact of treatment on criteria such as daily activities, motor skills, and balance. No improvement in symptoms of PSP was shown in patients who received tilavonemab (lower or higher dose).

Side effects in this patient population were similar to side effects seen in patients with PSP. Both dose levels of tilavonemab appeared to be safe and well tolerated.

6. How has the study helped patients and researchers?

The parent study (M15-562) and this study (M15-563) found that patients treated with tilavonemab did not have improved symptoms of PSP at either of the 2 different dose levels. This study also found that tilavonemab is generally well tolerated. Results from this study may be used in other studies to learn whether patients with other diseases are helped by tilavonemab.

This summary only shows the results of this study, which may be different from the results of other similar studies. Patients should consult their physicians and/or study doctors with further questions about their individual care and should not make changes in their treatment based on the results of a single study.

7. Are there any plans for future studies?

No future studies of tilavonemab for the treatment of PSP are planned.

8. Who sponsored this study?

This study was sponsored by AbbVie Inc. 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	An Extension Study of ABBV-8E12 in Progressive Supranuclear Palsy (PSP)
Protocol Number	M15-563
Clinicaltrials.gov	NCT03391765 https://clinicaltrials.gov/ct2/show/NCT03391765?term=M15-563&draw=2&rank=1
EudraCT	2017-001590-16 https://www.clinicaltrialsregister.eu/ctr-search/search?query=2017-001590-16
Study Sponsor	AbbVie Inc. Phone: (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!