MedImmune VIELABIO

CLINICAL TRIAL
RESULTS

A study of inebilizumab infusions in people with neuromyelitis optica and neuromyelitis optica spectrum disorders.

STUDY IDENTIFICATION INFORMATION

TREATMENT STUDIED: MEDI-551 (inebilizumab)

FULL TITLE: A double-masked, placebo-controlled study with open-label period to evaluate the efficacy and safety of MEDI-551 in adult subjects with neuromyelitis optica and neuromyelitis optica spectrum disorders.

STUDY NUMBERS: United States, NCT02200770 | Europe, 2014-000253-36 | Protocol, CD-IA-MEDI-551-1155

ADDITIONAL INFORMATION: This was a Phase 2/3 study. Phase 2/3 studies can take several years to complete and look at how safe and effective a potential new treatment is. An independent committee was used in this study to assess its safety and the overall conduct. Based on their recommendations, the study was stopped early because the benefit of inebilizumab infusions compared with placebo had been demonstrated and it was no longer ethical to treat participants with placebo infusions.

CONTENTS

WHY WAS THE STUDY DONE?

In this study, researchers wanted to test if an intravenous drug called inebilizumab, which is administered into a vein, would help to treat people with neuromyelitis optica spectrum disorders, also known as NMO or NMOSD. Additionally, they wanted to find out what side effects people had when they were given inebilizumab infusions during the study.

NMOSD is a rare condition which affects the nerves of the eyes, called the optic nerves. Patients with NMOSD experience attacks of the condition such as eye pain, loss of vision, and in-patient hospitalizations.

The main questions the researchers wanted to answer in this study were:

01 Did the treatments reduce the risk of NMOSD attacks?

02 Did either treatment reduce the participant's risk of worseningdisability?

03 Were any differences seen in the participant's vision during the study?

04 Were any changes seen on MRI scans during the study?

05 Did the treatments reduce the risk of NMOSD hospitalizations?

OVERALL RESULTS

The results of this study showed that participants treated with inebilizumab infusions had a significant reduction in the risk of having an NMOSD-related attack. Inebilizumab infusions were found to be safe and generally well-tolerated.

Please be aware that this summary only shows the results from this one study. Other studies of similar treatments may find different results.

HOW WAS THE STUDY DONE?

Inebilizumab infusions were compared with placebo infusions. The placebo looked the same as inebilizumab but did not have any "active ingredient" in it.

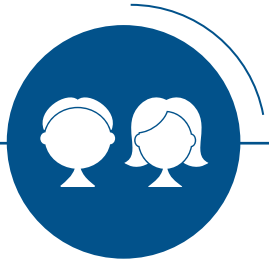
A computer randomly selected who was given inebilizumab infusions and who was given placebo infusions. This process is called randomization.

Neither the participants, treating physician nor study team, were told which treatment was administered until the end of the study. This is called a double-blind study.

For every 4 people who took part, 3 people were given inebilizumab infusions and 1 person was given placebo infusions. Participants therefore had a 75% chance of being treated with inebilizumab.

The study started in January 2015 and finished in October 2018.

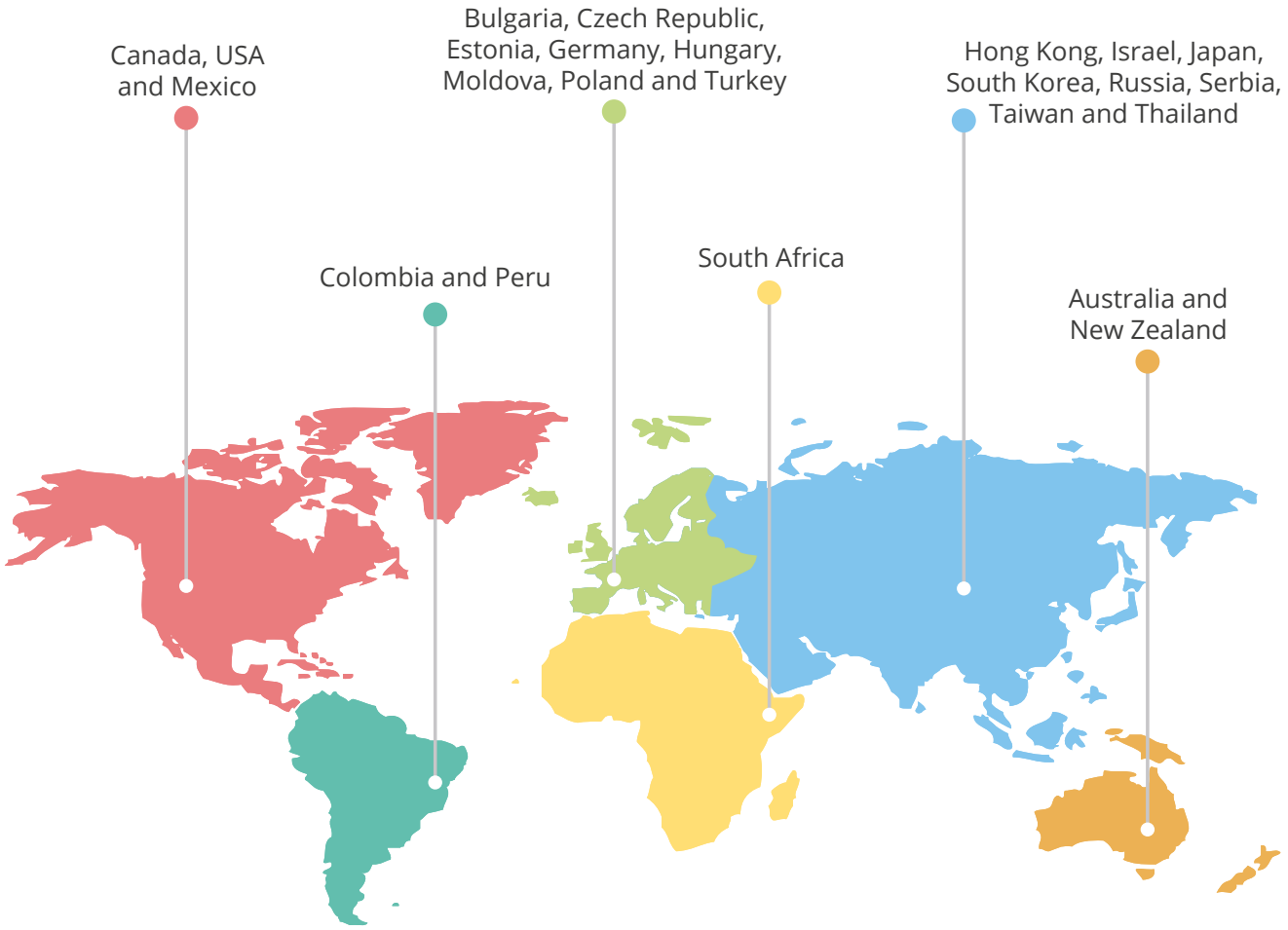
WHO COULD TAKE PART?



Aged 18 years or older

At least 1 NMOSD attack in the last year (or 2 or more attacks in the past 2 years) which needed treatment

WHO TOOK PART?



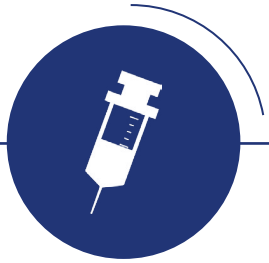
230
participants

21
men (9%)

209
women (91%)

Participants were aged between 18 and 74 years. The average age of participants was 43 years. The average used in this study was the mean ("average of all the data").

WHAT TREATMENTS WERE USED?



INEBILIZUMAB

Inebilizumab (300 mg) given via an intravenous pump for 90 minutes on 2 separate occasions over 28 weeks.

174 participants were treated with inebilizumab infusions.

PLACEBO

A water-based solution given via an intravenous pump for 90 minutes on 2 separate occasions over 28 weeks.

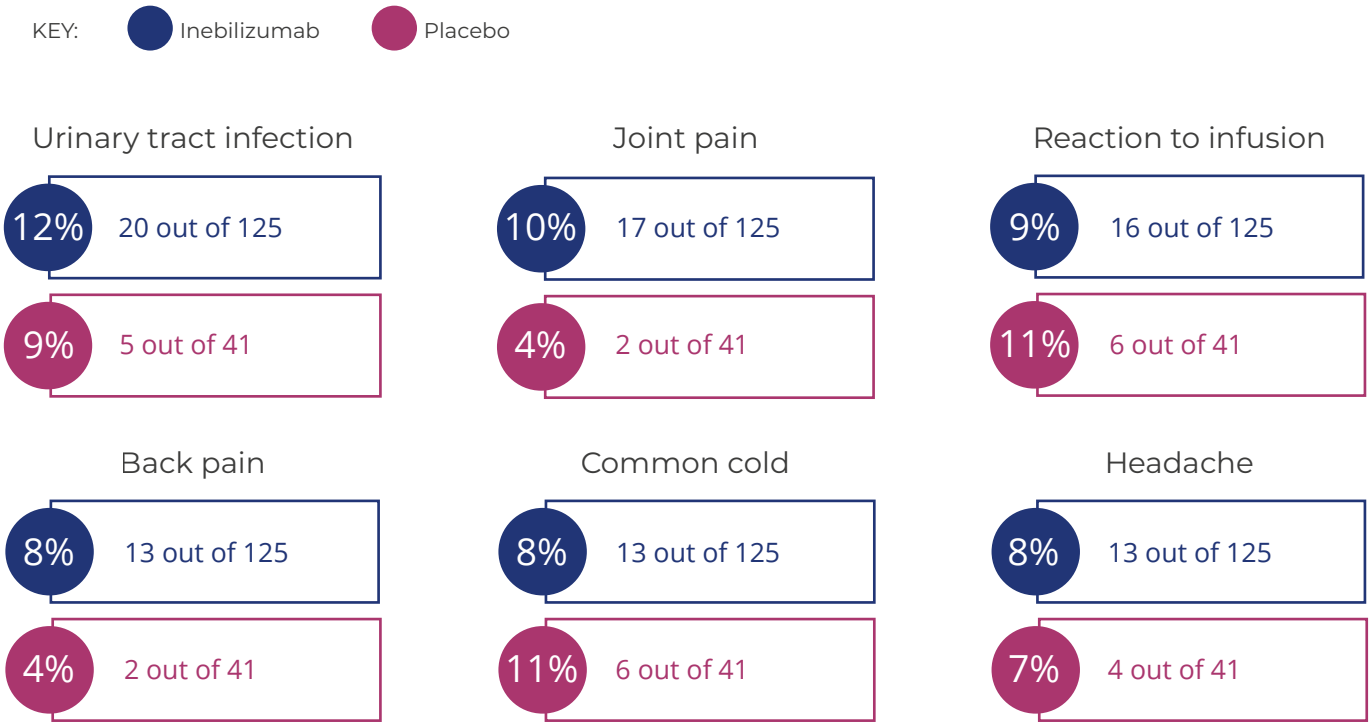
56 participants were treated with placebo infusions.

WHAT WERE THE SIDE EFFECTS?

During clinical studies, participants are asked to tell the study team about any side effect or symptom they have while taking part. Side effects can vary from person to person and a lot of research is needed to understand which side effects are caused by the study treatments and which side effects happen by chance. If the study doctor thinks a side effect may be related to the treatment the participant is taking, it is called a treatment-related side effect.

Of the 230 participants who took part in the study, 125 out of 174 people (72%) treated with inebilizumab infusions and 41 out of 56 people (73%) treated with placebo infusions had side effects which may or may not have been caused by the infusions they were given.

The side effects shown on the next page were experienced by more than 5% of the participants.



How many side effects were related to the study treatments?

41 out of 174 people (24%) treated with inebilizumab infusions and 14 out of 56 people (25%) treated with placebo infusions had a side effect that the study doctor thought was related to the study treatments.

Any side effect that is thought to be an important medical event, requires a person to go to the hospital, or could be life-threatening is called a “serious side effect”. A serious side effect may or may not be related to the treatment the participant was being given.

In this study, 13 out of 230 participants (6%) had a serious side effect. 8 out of 174 participants (5%) were treated with inebilizumab infusions and 5 out of 56 participants (9%) were treated with placebo infusions. There were no commonly reported serious side effects.

2 out of 230 participants (1%) stopped taking part in the study because of the side effects they had. Both of the participants were treated with inebilizumab infusions.

WHAT WERE THE RESULTS?

Did the treatments reduce the risk of NMOSD attacks?

87%

INEBILIZUMAB

At the end of the study, participants treated with inebilizumab infusions had an 87% chance of not having an attack.

60%

PLACEBO

At the end of the study, participants treated with placebo infusions had a 60% chance of not having an attack.

Did either treatment reduce the participant's risk of worsening disability?

Participants were asked at the beginning and at the end of the study to answer questions about their level of disability using two common questionnaires; the Expanded Disability Status Scale and the modified Rankin Scale. The questionnaires were designed to help researchers see if a person's disability got worse over time. When the researchers compared the scores from the beginning and the end of the study, the results showed:

Expanded Disability Status Scale

Participants who were treated with inebilizumab infusions were 63% less likely to have worsening of their disability than those participants treated with placebo infusions.

Modified Rankin Scale

Participants who were treated with inebilizumab infusions were 66% less likely to have disability than those participants treated with placebo infusions.

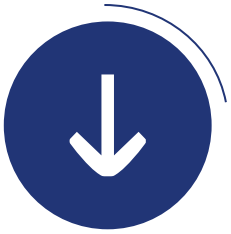
Were any differences seen in the participant's vision during the study?

In this study, the researchers wanted to see if inebilizumab infusions could prevent deterioration in vision. All participants were asked at the beginning and at the end of the study to take a vision test. The test involved looking at a chart and letting the tester know the smallest characters that could be seen.

The participant's visual loss was similar between the inebilizumab treatment and the placebo treatment.

Were any changes seen on MRI scans during the study?

All participants had an MRI scan of their central nervous system (an internal image of the optic nerve, spinal cord and brain) at the beginning of the study and again at the end of the study. The researchers wanted to see if either of the study treatments reduced the number of new abnormal growths (also called lesions) seen on the MRI scans over time.



Among participants who had new lesions, those treated with inebilizumab infusions had more than 1 new lesion on their MRI scan (on average 1.6 new lesions). In comparision, participants treated with placebo infusions had more than 2 new lesions on their MRI scan (on average 2.3 new lesions).

Treatment with inebilizumab infusions therefore reduced the number of new MRI lesions compared to treatment with placebo infusions.

Did the treatments reduce the risk of NMOSD hospitalizations?

INEBILIZUMAB

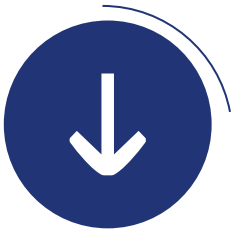
10 out of 174 participants (6%) treated with inebilizumab infusions were hospitalized during the study.

These participants experienced 1 hospitalization each.

PLACEBO

8 out of 56 participants (14%) treated with placebo infusions were hospitalized during the study.

Hospitalizations ranged between 1 and 3 each.



Inebilizumab infusions therefore reduced the number of NMOSD-related hospitalizations compared with placebo infusions.

The results of this study showed that people who were treated with inebilizumab infusions had a significant reduction in the risk of having an NMOSD-related attack, were less likely to have worsening of their disability, and had a reduced number of new MRI lesions and NMOSD-related hospitalizations compared with placebo treatment. There was no difference between inebilizumab and placebo in improving the participant's vision, or their overall quality of life. Inebilizumab infusions were found to be safe and generally well-tolerated.

HOW HAS THIS STUDY HELPED PARTICIPANTS AND RESEARCHERS?

This study has provided evidence of the clinical benefit of inebilizumab infusions for the treatment of NMOSD. It is important to note that this summary shows the overall results from one clinical study. It does not show the results for individual people. Other studies of treatments for NMOSD may produce different results.

Before a treatment can be approved for patients to take, researchers look at the results of many studies to decide which treatments work best and can be used safely.

You should not change your treatment based on the results of this study without talking to a doctor first.

FURTHER STUDIES

At the end of the study, participants were invited to join a second part, which is known as an 'open-label' phase. Open-label means all participants receive inebilizumab infusions and no one is treated with placebo. The aim of the open-label phase is to look at the effects of inebilizumab infusions over a longer period.

At the time of writing this summary, the open-label phase was still ongoing. It is intended that the results will be made available within 12 months of the study finishing.

WHERE CAN I FIND OUT MORE?

For more information about NMOSD and available treatments, please speak to a healthcare professional.

This document provides a summary of the main results of the study. It includes information about the side effects that happened in the study and the results of the main questions the researchers wanted to answer.

A full report of this study may be available to read at one of the following clinical trial registers:

www.clinicaltrials.gov

Use the study number

NCT02200770

to search for more information on this website.

www.clinicaltrialsregister.eu

Use the study number

2014-000253-36

to search for more information on this website.

For information about clinical trials that are currently looking for participants to take part, please visit the clinical trial registers.

Contact Information

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THANK YOU!

We thank the participants and their families for their important role in medical research and helping us learn more about treatments for neuromyelitis optica spectrum disorders.