NOVARTIS

Clinical Trial Results Summary

A clinical trial on participant satisfaction with a trial designed to provide close-to-home care

Thank you!

Thank you to the participants who took part in the clinical trial. The purpose was to help researchers learn more about participant satisfaction with trials designed to provide close-to-home care.

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: CBYL719A03201

Drug studied: Alpelisib

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.

What was the main purpose of this trial?

This trial was done upon request from the Swedish health authority, also named as Medical Product Agency (MPA), to test a type of clinical trial that is designed to provide close-to-home care, known as a hybrid decentralized trial design (DCT), to reach participants living in remote areas.

Hybrid DCTs are trials that permit participants to do a mix of different things, including:

- Going to the trial site
- Visiting a local doctor close to their home
- Using electronic devices to record their health status and receiving home visits from a local doctor or nurse
- Getting the trial medication delivered directly to their home

This approach can help people living in remote areas, or those unable to travel to trial sites, with more opportunities to participate in a clinical trial.

Advanced breast cancer- cancer that has spread from the breast, and areas around it, or to other parts of the body.

The trial purpose was to measure how satisfied the participants with advanced breast cancer were with participating in a hybrid DCT design.

How long was this trial?

The trial began in March 2022 and ended in September 2022.

It was planned for 20 participants to be in this trial for about 1 year. In September 2022, the researchers made the decision to stop this trial early after 2 participants had joined. The decision to stop was due to delays and difficulties in enrolling participants into this hybrid DCT design. The limited participation was due to the lack of awareness among hospital staff and researchers regarding the benefits of this hybrid DCT. This was because the technology used was not common in clinical studies.

Who was in this trial?

2 participants from Sweden took part in this trial. Their average age was 54 years. Their gender and race information was not collected according to Swedish data privacy laws.

The participants could take part in this trial if they:

- Were 18 years of age or older and had advanced breast cancer that met all the 3 conditions
- Were willing to use electronic devices, such as phones, for recording their vital signs and their experience of participating in the trial
- Had not previously received medicines that block the activities of proteins called PI3K, mTOR, and AKT

HR-Positive
Responsive to female hormone therapy

HER2-Negative
Non-responsive to treatment targeted at a protein called human epidermal growth factor receptor 2 (HER2)

PIK3CA mutation
Changes (mutation) in the PIK3CA gene that causes cancer cells to grow rapidly

What treatments did the participants receive?

The treatments in this hybrid DCT were:

Alpelisib, also known as BYL719, as tablets taken by mouth.

Fulvestrant as an injection into a muscle.

Goserelin as an injection into a muscle given only to women of childbearing age

Alpelisib in combination with fulvestrant is an approved treatment for advanced breast cancer in post-menopausal women as well as in men. These medicines are also being tested for breast cancer in women of childbearing age. Goserelin is an approved treatment for reducing the levels of female hormones in the body, which is required in certain conditions including breast cancer.

All the participants, trial doctors, and trial staff knew what treatment each participant received.

What happened during this trial?

Before treatment (On site) 28 days

Trial doctors checked participants' overall health at the trial site to ensure they could be in this clinical trial.
Participants were trained on using the electronic devices they would use from home.

During treatment Up to 12 months

2 participants took the below treatments in cycles of 28 days each, as long as they benefitted from it.

- Alpelisib: 300 milligram (mg) as tablets taken by mouth once a day for 12 cycles
- Fulvestrant: 500 mg as an injection into a muscle on Day 1 and Day 15 of Cycle 1 and then on Day 1 of each cycle from Cycle 2 until Cycle 12
- Goserelin: 3.6 mg as an injection into a muscle given only to women of childbearing age on Day 1 of each cycle

On-site

Participants were given the first dose of the trial treatment during their first visit to the trial site.

Remote

Participants continued to receive treatment at home and were cared for by local doctors and nurses. Participants recorded their satisfaction with the clinical trial approach and answered questions about their health using electronic devices.

After treatment (On site) 1 day

Participants were asked to return to the trial site after completing 12 cycles of treatment for a routine health check up.
As the trial was stopped early, none of the participants returned to the trial site after treatment.

Note: A cycle of cancer treatment is a period of time when participants receive the cancer drugs followed by a rest period to allow the body to recover.

What was the main result of this trial?

How satisfied were the participants who had advanced breast cancer with the hybrid DCT?

Since the trial ended early after only 2 participants joined, the researchers could not collect enough information to confirm participant satisfaction.

What adverse events did the participants have?

Trial doctors keep track of all 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 during this trial.

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.

Both the participants had adverse events. No participants had adverse events that were considered serious. No participants died during the trial. 1 participant left the trial due to an adverse event, and not due to the hybrid DCT design. The researchers concluded that there were no new safety concerns for this trial.

NOTE: The other participant left the trial as it ended early.

How many participants had adverse events?

Participants who:	Alpelisib + fulvestrant 2 participants
Had at least 1 adverse event	2 of 2 100%
Had at least 1 serious adverse event	0
Stopped treatment due to an adverse event	1 of 2 50%
Died during the trial	0

What serious adverse events did the participants have?

None of the participants had serious adverse events and no participant died during the trial.

What other adverse events did the participants have?

The table below shows the other adverse events that happened during the trial.

	Alpelisib + fulvestrant 2 participants
Diarrhea	1 of 2 50%
Dry mouth	1 of 2 50%
Feeling tired Fatigue	2 of 2 100%
Feeling sick Nausea	2 of 2 100%
Hair loss Alopecia	1 of 2 50%
High blood glucose levels Hyperglycaemia	2 of 2 100%
Reduced hunger Decreased appetite	1 of 2 50%
Stomach pain Abdominal pain	1 of 2 50%
Swelling of the mouth Stomatitis	1 of 2 50%
Swelling of the stomach lining Gastritis	1 of 2 50%
Weight loss Weight decreased	1 of 2 50%

How many participants stopped treatment due to adverse events?

During the trial, 1 participant stopped treatment due to high blood glucose levels.

What was learned from this trial?

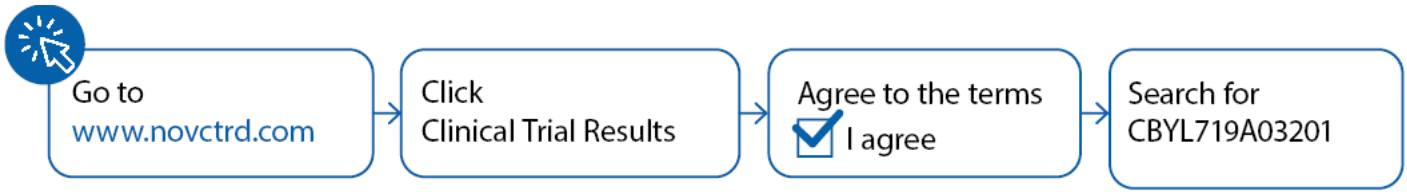
The trial aimed to learn about how satisfied the participants with advanced breast cancer were with the hybrid DCT approach. Since the trial ended early after only 2 participants joined the trial, researchers could not confirm participant satisfaction. The decision to end the trial early was due to delays and difficulties in enrolling participants into this hybrid DCT design. The limited participation was due of the lack of awareness among hospital staff and researchers regarding the benefits of this hybrid DCT. This was because the technology used was not common in clinical studies.

Novartis has a number of ongoing trials that utilize a similar hybrid DCT approach and has other trials in which remote elements are planned. No specific satisfaction surveys of hybrid DCTs are planned at this time.

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 any of these websites:

- clinicaltrials.gov – search using the number NCT04862143
- clinicaltrialsregister.eu/ctr-search/search – search using the number 2020-005882-15

Another trial CBYL719X2402 explored the same treatment combination in both menopausal women and women of childbearing age.

If more trials are planned, they will appear on the public websites above. When there, search for BYL719, alpelisib, or decentralized trial.

Full clinical trial title: Open-label, multicenter, pilot-trial evaluating the safety and utility of a hybrid decentralized clinical trial (DCT) approach using a TELEmedicine platform in patients with HR positive/HER2-negative advanced breast cancer with a PIK3CA mutation treated with alpelisib fulvestrant TELEPIK Trial

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

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