Alliance Public Study Result Summary
Study Number A091401

What this study is about

A cancer study that looked at giving nivolumab alone or nivolumab and ipilimumab to treat patients with metastatic sarcoma.

The full title of this study is: Randomized Phase II Study of Nivolumab with or without Ipilimumab in Patients with Metastatic or Unresectable Sarcoma

Why the study was done

This study was done to test how well treating patients with either nivolumab alone or with the combination of nivolumab and ipilimumab together worked in patients with sarcoma.

Sarcomas are rare cancerous tumors which start growing in the connective tissues of the body. There are more than 100 different types of sarcoma.

Nivolumab and ipilimumab are immunotherapy drugs. Immunotherapy works by activating (turning on) the body's defense (immune) system against the cancer using specific drugs.

Study results

These results are for people with sarcoma who participated in this study.

The study found the following:

- In the patients taking only nivolumab, 5 out of every 100 patients (5%) had their cancer tumor(s) shrink.
- The most common serious side effects in more than 7 out of every 100 patients (7%) included:
    - 10 out of every 100 patients (10%) experienced anemia (low red blood cell count, you may feel tired)
    - 7 out of every 100 patients (7%) had decreased in lymphocyte counts (low white blood cell count)

- In the patients taking nivolumab plus ipilimumab (dual agent group), 16 out of every 100 patients (16%) had their cancer tumor(s) shrink.
- The most common serious side effects in more than 7 out of every 100 patients (7%) include:
    - 19 out of every 100 patients (19%) experienced anemia (low red blood cell count, you may feel tired)
    - 10 out of every 100 patients (10%) had hypotension (low blood pressure)
    - 9 out of every 100 patients (9%) had hyponatremia (low sodium in the blood)
    - 7 out of every 100 patients (7%) had increased alanine aminotransferase (worsened liver function)
    - 7 out of every 100 patients (7%) had pain
    - 7 out of every 100 patients (7%) had urinary tract infection

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What the results mean

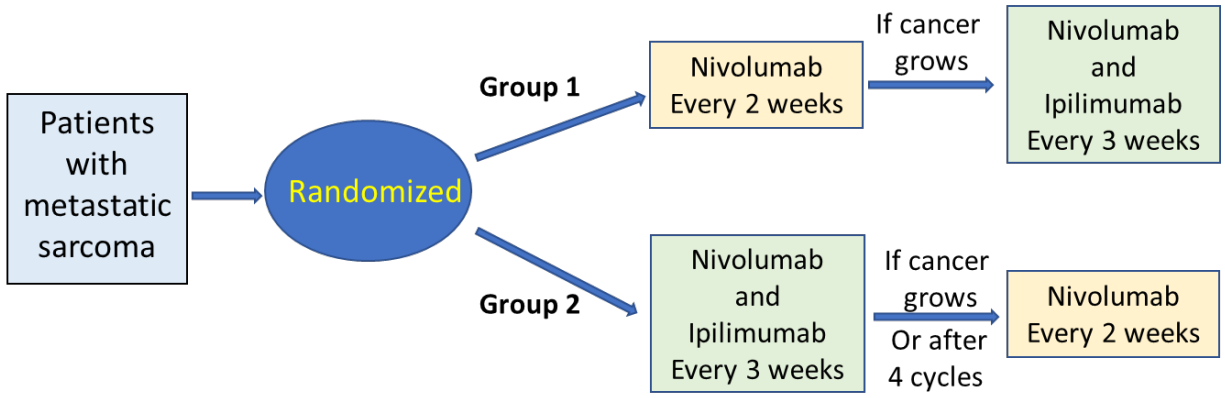
The results of this study showed that nivolumab alone did not work very well in treating sarcoma patients. The combination of nivolumab and ipilimumab worked better than nivolumab alone in treating sarcoma especially in patients with UPS (undifferentiated pleomorphic sarcoma), LMS (leiomyosarcoma), myxofibrosarcoma and angiosarcoma. While this combination had worse side effects they were considered manageable.

How the study worked

Patients were randomly assigned to one of two groups.

- Group 1 received nivolumab alone every two weeks until their cancer grew (progressed). Patients were then given the choice to cross over to Group 2 if it was considered safe for them.
- Group 2 received both nivolumab and ipilimumab every 3 weeks for 4 doses followed by nivolumab alone every 2 weeks until their cancer grew (progressed).

Here's a picture that explains how patients were placed into this study:



Randomization is the process by which patients are assigned by chance to separate groups

When did the study start and end? The study started in August, 2015. All patients were enrolled by March, 2016. Treatment was continued for two years unless a patient's tumor started growing or the side effects became unmanageable/intolerable. Patients were followed for 3 years.

How many patients joined? 96 patients agreed to be in this study but only 85 were able to continue on to the study for treatment because they met all the eligibility criteria.

Talk to your doctor if you want more information about this study.

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Scientific publications about this study

Details about the study can be found in this article:

- D'Angelo SP, Mahoney MR, Van Tine BA, Atkins J, Milhem MM, Jahagirdar BN, Antonescu CR, Horvath E, Tap WD, Schwartz GK, Streicher H. Nivolumab with or without ipilimumab treatment for metastatic sarcoma (Alliance A091401): two open-label, non-comparative, randomised, phase 2 trials. Lancet Oncol. 2018 Mar;19(3):416-426. doi: 10.1016/S1470-2045(18)30006-8. Epub 2018 Jan 19. PMID: 29370992; PMCID: PMC6126546.

To learn about this trial, visit the ClinicalTrials.gov website at:
https://clinicaltrials.gov/ct2/show/NCT02500797

This study was sponsored by the Alliance for Clinical Trials in Oncology – a national clinical trial network group that runs large cancer clinical trials. The Alliance is supported by the National Cancer Institute (NCI) and brings researchers together to develop better treatments for cancers. More information about the Alliance is at www.AllianceNCTN.org.

This summary lists what is known about this research study as of December, 2020.

We thank the people who joined this study and made it possible.
We do research to try to learn the best ways to help patients.
The people who joined this study helped us to do that.

Thank you for your interest in learning more about cancer research advances.