PDS0101-HNC-201
CSR Lay Summary

02Apr2026

1 Study Name

Researchers look at the results of many studies to understand which drugs work and how they work. This summary only shows the results from one Phase 2 study. Other studies may find different results.

1.1 Study Title

A Phase 2, Open-Label, Multi-Center Study of PDS0101 and Pembrolizumab Combination Immunotherapy in Participants with Recurrent and/or Metastatic Head and Neck Cancer and High-Risk HPV16 Infection.

1.2 Protocol Number

PDS0101-HNC-201

1.3 EU Trial Number

2021-004046-38

1.4 Other Identifiers

NCT04260126

ISRCTN15021247

1.5 Abstract

Purpose of the study: To test a new treatment for head and neck cancer from the human papillomavirus-16 (HPV16). The cancer cannot be removed with surgery (unresectable) and has come back (recurrent) or has spread to areas outside of the head and neck (metastatic). Current treatments for this type of cancer often don't work well and can have serious side effects, so new options are needed.

The study goals were to test if this treatment is safe and can help shrink head and neck cancer tumors.

What was tested: PDS0101 and pembrolizumab combination

People taking part in this study: Patients with HPV16 infection and head and neck cancer that had come back or spread

Results: Group 1 had 62 patients who had not been treated with a similar type of cancer drug before. Group 2 had 25 patients who had been treated with a similar type of cancer drug before. The study treatment helped shrink the tumors in Group 1.

Safety: Researchers found that PDS0101 and pembrolizumab can be used safely. Side effects were like those seen with pembrolizumab alone.

2 Who sponsored this study?

PDS Biotechnology Corporation

3 General Information about the Clinical Trial

3.1 Where was the study done?

United States, United Kingdom, and Ireland

3.2 When was this study done?

March 2021 to May 2025

3.3 What was the main objective of this study?

To find out if the treatment was safe and if it helped shrink tumors in patients with head and neck cancer

4 What patients/people were included in the study?

4.1 Age Group and Gender Breakdown

87 patients: average age 64 years; 81 males; 6 females

4.2 Inclusion and Exclusion Criteria

Give written informed consent, 18 years of age or older, had squamous cell cancer of the head and neck that had come back or spread, had good general health and able to do light work, recovered from the side effects of major surgery/radiation therapy and if they could become pregnant, agreed to use birth control.

Group 1 had a confirmed HPV16 infection, PDL1 testing, and no prior immunotherapy treatment.

Group 2 had confirmed HPV16 infection, PDL1 testing, prior similar cancer treatment, and scan proof that cancer had progressed or come back.

Patients could not participate in the study if they were pregnant or on treatments that could interfere with the study treatment or its safety.

5 Which medicines were studied?

PDS0101: new vaccine that trains the body's immune system to recognize and attack cells infected with HPV16

Pembrolizumab: approved drug shown to help the body's own immune system find and attack cancer cells by blocking a "shield" called PD-1 that some cancers use to hide from immune cells

6 What were the side effects?

Side effects (unwanted medical events, like a headache) in this study were reported by the study doctor:

- 5 out of 87 patients (5.7%) had a serious side effect related to PDS0101
- 75 out of 87 patients (86.2%) had a side effect related to PDS0101. Most common side effects were injection site pain (55.2%), fatigue (33.3%), and injection site swelling (31.0%).
- Some patients stopped treatment because of side effects: 9 out of 62 patients (14.5%) in Group 1 and 2 out of 25 patients (8.0%) in Group 2

- Some patients died during the study. No deaths were caused by the treatment. Deaths were expected due to the type of cancer the patients had.

7 What were the overall results of the study?

One goal of the study was to measure the size of the tumor to see if it had shrunk, stayed the same, or grew. A total of 18 of 53 patients (34.0%) had tumors shrink or disappear in Group 1, while none of the patients in Group 2 had tumors shrink or disappear.

The study also looked at the length of time patients lived without their cancer getting worse. Patients lived without worsening about 5.3 months in Group 1 and about 2.0 months in Group 2.

Patients lived from the start of study treatment about 39.3 months in Group 1 and about 14.8 months in Group 2.

Overall, the study combination can be used safely without serious side effects in this patient population. The side effects were like taking pembrolizumab by itself. No new safety problems were found.

8 How has this study helped patients and researchers? Are there plans for further studies?

Findings from this study were used to plan a Phase 3 study to confirm the results in more patients.

9 Where can I find more information about this study?

NCT04260126 at ClinicalTrials.gov

ISRCTN15021247 at www.isrctn.com