Policy Number	**MED203.002**
Policy Effective Date	**07/15/2025**
Policy End Date	**12/31/2025**

Antineoplaston Cancer Therapy

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Related Policies (if applicable)
None

Disclaimer

Medical policies are a set of written guidelines that support current standards of practice. They are based on current peer-reviewed scientific literature. A requested therapy must be proven effective for the relevant diagnosis or procedure. For drug therapy, the proposed dose, frequency and duration of therapy must be consistent with recommendations in at least one authoritative source. This medical policy is supported by FDA-approved labeling and/or nationally recognized authoritative references to major drug compendia, peer reviewed scientific literature and acceptable standards of medical practice. These references include, but are not limited to: MCG care guidelines, DrugDex (IIa level of evidence or higher), NCCN Guidelines (IIb level of evidence or higher), NCCN Compendia (IIb level of evidence or higher), professional society guidelines, and CMS coverage policy.

Carefully check state regulations and/or the member contract.
Each benefit plan, summary plan description or contract defines which services are covered, which services are excluded, and which services are subject to dollar caps or other limitations, conditions or exclusions. Members and their providers have the responsibility for consulting the member's benefit plan, summary plan description or contract to determine if there are any exclusions or other benefit limitations applicable to this service or supply. **If there is a discrepancy between a Medical Policy and a member's benefit plan, summary plan description or contract, the benefit plan, summary plan description or contract will govern.**

Coverage

Antineoplaston therapy for the treatment of cancer **is considered experimental, investigational and/or unproven.** This includes, but is not limited to, the following malignancies:

- Brain,
- Head, neck and throat,
- Breast,
- Lung,
- Colon,
- Blood,

- Lymphoma,
- Melanoma,
- Myeloma, or
- Sarcoma.

Policy Guidelines

None.

Description

Antineoplaston (AN) therapy is an alternative form of cancer treatment that involves using a group of synthetic chemicals called ANs to protect the body from disease.

Background
ANs are hypothesized by proponents to have anti-tumor activity. ANs are made up mostly of peptides and amino acids originally taken from human blood and urine. The therapy can be given orally or by injection into a vein. Proponents claim AN cancer therapy has been successful in treating many forms of cancer. They claim people with cancer have a deficiency of naturally occurring ANs, and that this therapy replenishes the body's supply allowing the biochemical defense system of the body to convert cancer cells into normal cells.

AN cancer therapy is offered exclusively in the U.S. by the Burzynski Research Institute (BRI) in Houston, Texas, and has long been a controversial treatment for various types of malignancy.

Regulatory Status
The U.S. Food and Drug Administration (FDA) has not approved any form of AN cancer therapy. On September 23, 2013, the FDA notified the BRI Institutional Review Board (IRB) of objectionable conditions observed during an inspection. The purpose of the inspection was to determine whether the IRB's activities and procedures for the protection of human subjects comply with the FDA regulations published in Title 21 of the Code of Federal Regulations (21 CFR), parts 50 and 56. (1) These regulations govern clinical investigations of products regulated by the FDA and help ensure that human subjects are protected from undue hazard or risk during the course of clinical investigations. The following restrictions were in place:

- No new studies subject to the requirements of 21 CFR parts 50 and 56 are to be approved by BRI IRB (21 CFR 56.103[a] and 56.120[b]);
- No new subjects are to be added to ongoing studies subject to 21 CFR part 56 (21 CFR 56.120[b]).

The FDA suspended the IRB's use of the expedited review procedure to protect the rights or welfare of subjects (21 CFR 56.110[d]). The restrictions were removed April 11, 2014. (1)

Rationale

The policy was created in 1996 based on scientific literature reviewed from the PubMed database. The literature reviews do not identify any large, controlled studies of antineoplaston (AN) cancer therapy. The bulk of the literature consists of case reports, case series, phase I clinical trials, and non-randomized data from single institution phase II trials. (2-17).

One randomized non-blinded, phase II study from Japan was published in 2015 by Ogata et al. (18) This study, from 1998-2004, compared the efficacy of hepatic arterial infusion with 5-fluorouracil, with or without ANs as a postoperative therapy for colorectal metastases to the liver. Confirmed metastatic colon adenocarcinoma in the liver in 65 patients had been treated with hepatectomy and/or thermal ablation for liver metastases. The primary endpoint was cancer specific survival; secondary endpoints were relapse free survival, status and extent of recurrence, salvage surgery rate and toxicity. The overall survival was not statistically improved (p=0.105) in the AN arm. The relapse free survival was not significant (p=0.343). However, the cancer specific survival was significantly higher in the AN arm versus the control arm (N=33) with median survival of 67 months (95% confidence interval [CI]; 43 not calculated) versus 39 months (95% CI; 28-47) (p=0.037) and 5-year cancer specific survival rate 60% versus 32% respectively. The authors reported that cancer recurred more often in a single organ than in multiple organs in the AN arm versus the control arm. They concluded that the limited extent of recurrent tumors in the AN arm meant more patients remained eligible for salvage surgery; and that AN therapy might be useful as an adjunctive therapy.

In 2019 the National Cancer Institute's Physician Data Query (PDQ) for cancer information was updated. The summary regarding antineoplastons states "The evidence for use of antineoplaston therapy as a treatment for cancer is inconclusive. Controlled clinical trials are necessary to assess the value of this therapy." (2)

Ongoing and Unpublished Clinical Trials

Some currently ongoing and unpublished trials that might influence this policy are listed in Table 1. (19)

Table 1. Summary of Key Trials (19)

NCT Number	Trial Name	Planned Enrollment	Completion Date
Ongoing			
NCT02864888	A Controlled, Study of the Effect of Combination Antineoplaston Therapy [Atengenal (A10) and Astugenal (AS2-1)] on the QT/QTc Interval In Subjects With Newly Diagnosed Diffuse, Intrinsic, Brainstem Glioma	30	Dec 2025 (Not yet recruiting)
NCT02887040	A Randomized Phase 3 Study of Combination Antineoplaston Therapy [Antineoplastons A10 (Atengenal) and AS2-1 (Astugenal)] Plus	92	Jun 2026 (Not yet recruiting)

	Radiation Therapy vs. Radiation Therapy Only in Subjects With Newly Diagnosed Diffuse, Intrinsic, Brainstem Glioma		
NCT02742883	A Phase 2 Study of Atengenal (A-10) and Astugenal (AS2-1) in Diffuse, Intrinsic Pontine Glioma (DIPG)	1	Mar 2028 (Suspended, clinical hold)

NCT: national clinical trial.

Practice Guidelines and Position Statements

No practice guidelines or position statements on antineoplaston cancer therapy were identified.

Summary of Evidence

Currently, there remains inadequate published scientific literature, including practice guidelines or position statements that would change the coverage position of this medical policy. Antineoplaston cancer therapy is considered experimental, investigational and/or unproven for the treatment of any type of malignancy.

Coding

Procedure codes on Medical Policy documents are included **only** as a general reference tool for each policy. **They may not be all-inclusive.**

The presence or absence of procedure, service, supply, or device codes in a Medical Policy document has no relevance for determination of benefit coverage for members or reimbursement for providers. **Only the written coverage position in a Medical Policy should be used for such determinations.**

Benefit coverage determinations based on written Medical Policy coverage positions must include review of the member's benefit contract or Summary Plan Description (SPD) for defined coverage vs. non-coverage, benefit exclusions, and benefit limitations such as dollar or duration caps.

CPT Codes	96549
HCPCS Codes	J9999

*Current Procedural Terminology (CPT®) ©2024 American Medical Association: Chicago, IL.

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Centers for Medicare and Medicaid Services (CMS)

The information contained in this section is for informational purposes only. HCSC makes no representation as to the accuracy of this information. It is not to be used for claims adjudication for HCSC Plans.

The Centers for Medicare and Medicaid Services (CMS) does not have a national Medicare coverage position. Coverage may be subject to local carrier discretion.

A national coverage position for Medicare may have been developed since this medical policy document was written. See Medicare's National Coverage at <https://www.cms.hhs.gov>.

Policy History/Revision

Date	Description of Change
07/15/2025	Reviewed. No changes.
06/15/2024	Document updated with literature review. Coverage unchanged. No new references added, some references removed.
03/15/2023	Reviewed. No changes.
05/15/2022	Document updated with literature review. Coverage unchanged. No new references added.
02/15/2021	Reviewed. No changes.
10/01/2020	Document updated with literature review. Coverage unchanged. References 22-23 added, and others revised.
01/15/2019	Reviewed. No changes.
01/15/2018	Document updated with literature review. Coverage unchanged.
01/15/2017	Reviewed. No changes.
04/15/2016	Document updated with literature review. Coverage unchanged.
02/01/2015	Reviewed. No changes.
11/01/2013	Literature reviewed. No changes.
05/15/2008	Policy reviewed without literature review; new review date only. This policy is no longer scheduled for routine literature review and update.
02/01/2007	Revised/updated entire document
10/24/2003	Revised/updated entire document
03/01/1996	New medical document