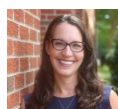


# JADPRO Best Practices Summit

## Sharing AP Perspectives & Practice in Treatment and Management of Patients With Desmoid Tumors

Desmoid tumors (also known as aggressive fibromatoses) are rare, locally aggressive, nonmetastatic mesenchymal growths known for their slow progression, high rates of recurrence, and unpredictable clinical course. The U.S. Food and Drug Administration (FDA) approval of the gamma secretase inhibitor nirogacestat (Osgiveo) in 2023 marked a new era in the treatment of these noncancerous masses but prompted questions regarding its optimal use and side effect management. To better understand the current practice patterns, a survey was distributed to 15 qualified advanced practitioners (APs) in both academic and community practices around the United States.

Survey data was collated and then discussed during the JADPRO Best Practices Summit: Sharing AP Perspectives & Practice in Treatment and Management of Patients With Desmoid Tumors, a live meeting moderated by Tammy Triglianios, DNP, ANP-BC, AOCNP®, of UNC Lineberger Comprehensive Cancer Center, Chapel Hill. The attendees discussed the survey data and their real-world experiences with addressing barriers, providing insight into the ideal state of care.



“[Per NCCN Guidelines,] all patients should be evaluated by a multidisciplinary team.”

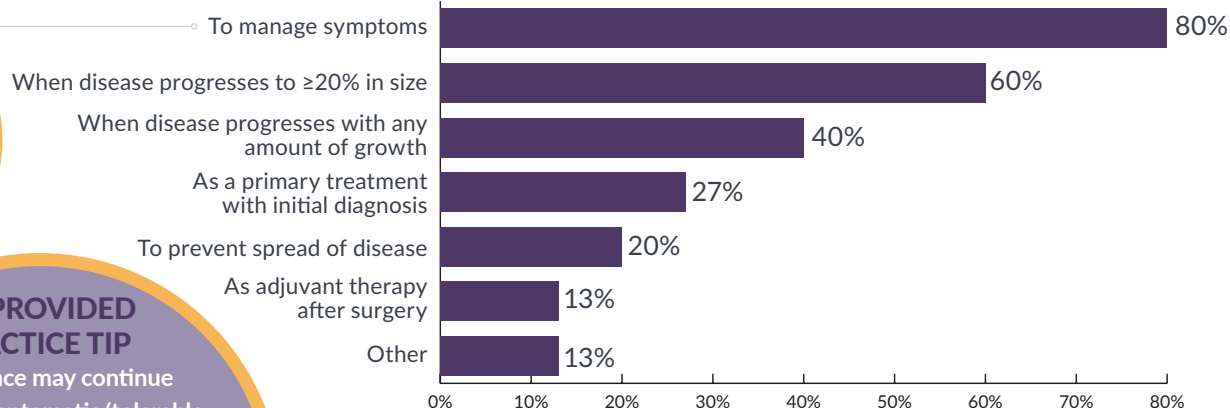
– TAMMY TRIGLIANIOS, DNP, ANP-BC, AOCNP®, MODERATOR

## When to Recommend Treatment: The AP Perspective

Pain was the most frequently managed symptom (93%) among the responding APs.

### AP-PROVIDED PRACTICE TIP

Surveillance may continue despite asymptomatic/tolerable disease progression, provided the tumor does not increase in size by more than 20%. In patients with limited disease progression, nonsteroidal anti-inflammatory drugs (e.g., sulindac) may be considered to alleviate symptoms.



“We are learning that the rate of spontaneous regression is more than what we have historically thought, so we need to take that into consideration.”

– TAMMY TRIGLIANIOS

### NCCN-RECOMMENDED SYSTEMIC THERAPIES: DATA SUPPORTING CATEGORY 1 EVIDENCE

- **Nirogacestat:** The **phase III DeFi trial** showed significant benefits in progression-free survival, objective response, pain, symptom burden, physical functioning, role functioning, and health-related quality of life in patients with progressing desmoid tumors who were treated with nirogacestat vs. placebo.<sup>1</sup> Nirogacestat-related adverse events were common but mostly low grade.

- **Sorafenib:** Based on a **phase III trial**, sorafenib vs. placebo seemed to significantly prolong progression-free survival and induce durable responses in patients with progressive, refractory, or symptomatic desmoid tumors.<sup>2</sup>

1. Gounder M, et al. *N Engl J Med.* 2023;388(10):898-912.

2. Gounder MM, et al. *N Engl J Med.* 2018;379(25):2417-2428.

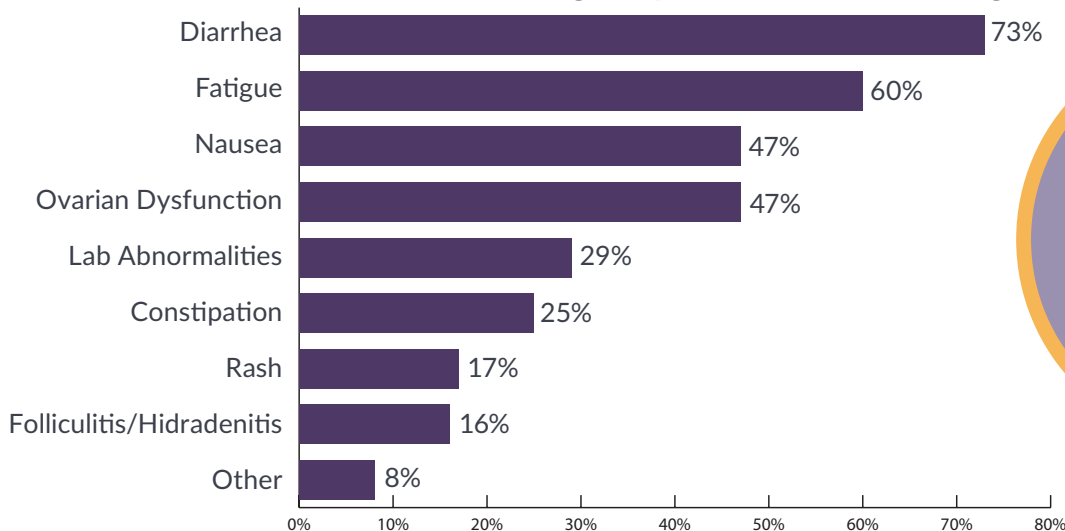
## Practical Guidance for APs When Using Nirogacestat

KEY AREAS	DETAILS
Response Evaluation	Improvements in symptoms (e.g., pain) may be seen before radiographic changes in tumor size. Until the 6-month scan, APs should make judgements based on clinical response and consider whether there is a decrease in the rate of growth.
Follow-Up Visits	Nearly half of the responding APs (47%) reported seeing patients every 4 weeks after starting treatment. APs should consider scheduling a 1-week follow-up visit (virtual or in-person) to ensure the patient has taken the drug. Periodic phone calls are also good practice within the first 2 months to confirm continued adherence.
Treatment Duration	So far, 3-year follow-up data are available <sup>1</sup> ; thus, APs recommend continuing treatment with nirogacestat until disease progression or unacceptable toxicity.
Fertility Concerns	Based on the DeFi trial, of the 27 women of childbearing potential who experienced nirogacestat-related ovarian dysfunction, 20 had resolution during or after therapy. <sup>2</sup> However, given a lack of long-term follow-up data, some APs recommend implementing shared decision-making and considering an ovarian preservation strategy beforehand.

1. Ratan R, et al. Nirogacestat treatment in adult patients with desmoid tumors: Long term efficacy and safety from the phase 3 DeFi trial. Connective Tissue Oncology Society (CTOS) annual meeting, 2024; 2. Gounder M, et al. *N Engl J Med.* 2023;388(10):898-912.

## Real-World Observations: Nirogacestat Side Effects

Side effects APs manage for patients treated with nirogacestat



### AP-PROVIDED PRACTICE TIP

Consider optimizing supportive care (e.g., patient education, medications for symptom management, nutritional support) prior to making dose adjustments.

- Rashes are common early in treatment but are manageable; they may resolve after a couple of months or with triamcinolone.
- Dose reductions and/or treatment discontinuation are sometimes required for diarrhea.
- Attendees observed successful outcomes with tamoxifen after the discontinuation of nirogacestat, despite limited data.
- Attendees reported that it is uncommon but potentially feasible to start with a lower dose of nirogacestat and titrate up to minimize side effects.

### What to Share With Patients

- It is understandable to want your desmoid tumor removed, but surgery is typically not a first-line option, and it may increase tumor growth.
- Observation alone may be an appropriate treatment option for you.
- Use sunscreen to minimize the risk of developing a nirogacestat-related rash, especially if you reside in an area with intense sun exposure.
- If you are of childbearing age or potential, discuss with your care team whether nirogacestat is right for you.

### What to Share With the Broader AP Community

- Desmoid tumors should be managed by a multidisciplinary team.
- Explain when and how to treat patients with desmoid tumors (e.g., assess the need for treatment based on symptoms and tumor growth, discuss treatment options, avoid surgery).
- Explain the importance of seeking out reliable educational resources (e.g., regular discussion, educational events, treatment guidelines) to stay up to date and hear the experiences of others.
- Patients benefit from individualized decision-making and emotional reassurance.