COMPENDIA TRANSPARENCY TRACKING FORM

DATE: October 8, 2024

OFF-LABEL ID #: 2713

DRUG NAME: Sorafenib Tosylate

OFF-LABEL USE: Aggressive fibromatosis Advanced

COMPENDIA TRANSPARENCY REQUIREMENTS	
1	Provide criteria used to evaluate/prioritize the request (therapy)
2	Disclose evidentiary materials reviewed or considered
3	Provide names of individuals who have substantively participated in the review or disposition of the request and disclose their potential direct or indirect conflicts of interest
4	Provide meeting minutes and records of votes for disposition of the request (therapy)

EVALUATION/PRIORITIZATION CRITERIA: C, L, R *to meet requirement 1

CODE	EVALUATION/PRIORITIZATION CRITERIA
A	Treatment represents an established standard of care or significant **advance** over current therapies
C	**Cancer** or cancer-related condition
E	Quantity and robustness of **evidence** for use support consideration
L	**Limited** alternative therapies exist for condition of interest
P	**Pediatric** condition
R	**Rare** disease
S	**Serious**, life-threatening condition

Note: a combination of codes may be applied to fully reflect points of consideration [eg, therapy may represent an advance in the treatment of a life-threatening condition with limited treatment alternatives (ASL)]

EVIDENCE CONSIDERED:

*to meet requirements 2 and 4

CITATION	LITERATURE CODE
Gounder, MM, Mahoney, MR, Van TIne, BA, et al: Sorafenib for advanced and refractory desmoid tumors. N Engl J Med Dec 20, 2018; Vol 379, Issue 25; pp. 2417-2428. Pubmed ID: 30575484	S
Paksoy, N, Ferhatoglu, F, Dogan, I, et al: Efficacy of sorafenib in symptomatic patients with pretreated progressive desmoid tumors. Eurasian J Med Invest 2022; Vol 6, Issue 3; pp. 326-331.	3
Garg, V, Gangadharaiah, BB, Rastogi, S, et al: Efficacy and tolerability of sorafenib in desmoid-type fibromatosis: a need to review dose. Eur J Cancer Jun 2023; Vol 186, pp. 142-150. Pubmed ID: 37062211	3
Gronchi, A, Miah, AB, Dei Tos, AP, et al: Soft tissue and visceral sarcomas: ESMO-EURACAN-GENTURIS Clinical Practice Guidelines for diagnosis, treatment and follow-up. Ann Oncol Nov 2021; Vol 32, Issue 11; pp. 1348-1365. Pubmed ID: 34303806	S
Benech, N, Bonvalot, S, Dufresne, A, et al: Desmoid tumors located in the abdomen or associated with adenomatous polyposis: French intergroup clinical practice guidelines for diagnosis, treatment, and follow-up (SNFGE, FFCD, GERCOR, UNICANCER, SFCD, SFED, SFRO, ACHBT, SFR). Dig Liver Dis Jun 2022; Vol 54, Issue 6; pp. 737-746. Pubmed ID: 35508462	S
Tsukamoto, S, Takahama, T, Mavrogenis, AF, et al: Clinical outcomes of medical treatments for progressive desmoid tumors following active surveillance: a systematic review. Musculoskelet Surg Mar 2023; Vol 107, Issue 1; pp. 7-18. Pubmed ID: 35150408	4
Mangla, A, Agarwal, N, and Schwartz, G: Desmoid tumors: current perspective and treatment. Curr Treat Options Oncol Feb 2024; Vol 25, Issue 2; pp. 161-175. Pubmed ID: 38270798	4

Literature evaluation codes: S = Literature selected; 1 = Literature rejected = Topic not suitable for scope of content; 2 = Literature rejected = Does not add clinically significant new information; 3 = Literature rejected = Methodology flawed/Methodology limited and unacceptable; 4 = Other (review article, letter, commentary, or editorial)

CONTRIBUTORS:

*to meet requirement 3

PACKET PREPARATION	DISCLOSURES	EXPERT REVIEW	DISCLOSURES
Stacy LaClaire, PharmD	None		
Catherine Sabatos, PharmD	None		
		John D Roberts	None
		Jeffrey Klein	None
		Richard LoCicero	Incyte Corporation Local PI for REVEAL. Study is a multicenter, non-interventional, non-randomized, prospective, observational study in an adult population for patients who have been diagnosed with clinically overt PV and are being followed in either community or academic medical centers in the US who will be enrolled over a 12-month period and observed for 36 months.

ASSIGNMENT OF RATINGS:

*to meet requirement 4

	EFFICACY	STRENGTH OF RECOMMENDATION	COMMENTS	STRENGTH OF EVIDENCE
MERATIVE MICROMEDEX	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases		B
Jeffrey Klein	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	The use of Sorafenib to treat advanced aggressive fibtomatosis (desmoid tumors) demonstrated a very favorable progression free survival over placebo. The response time with Sorafenib was also faster over placebo as well. The degree of grade 3 and 4 adverse events was higher in the Sorafenib group.	
Todd Gersten	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	Sorafenib has been shown to induce a higher percentage of response and extend time to disease progression beyond that of placebo.	

Warren Brenner	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	This was a well conducted study in a vary rare neoplasm and used a blinded placebo controlled design with a 2;1 randomization. Due to the rarity of the tumor it is a small study for a phase III trial with <100 patients randomized although this would be expected for an ultra rare tumor. The primary end point was met with improved PFS which seems meaningful. Expected side effects of TKI therapy was noted. I gave it a efficacy rating of favoring efficacy as there were responses in the placebo group and there is no data as far as improvement in QOL scores or OS. I gave it a class 118 recommendation due to the lack of this data, the unusual natural course of these tumors with some patients having spontaneous regressions and its side effect profile and the different rx options including surgery, radiation and other systemic options.	