

## COMPENDIA TRANSPARENCY TRACKING FORM FOR ONCOLOGY OFF-LABEL USES DERIVED FROM GUIDELINES

**DATE:** April 1, 2025

**DRUG NAME:** Vismodegib

**OFF-LABEL USE:** Gorlin syndrome

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, \*to meet requirement 1(C, L, R)

CODE	EVALUATION/PRIORITIZATION CRITERIA
A	Treatment represents an established standard of care or significant <b>advance</b> over current therapies
C	<b>Cancer</b> or cancer-related condition
E	Quantity and robustness of <b>evidence</b> for use support consideration
L	<b>Limited</b> alternative therapies exist for condition of interest
P	<b>Pediatric</b> condition
R	<b>Rare</b> disease
S	<b>Serious</b> , 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
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Verkouteren BJA, Cosgun B, Reinders MGHC, Kessler PAWK, Vermeulen RJ, Klaassens M, Lambrechts S, van Rheeën JR, van Geel M, Vreeburg M, Mosterd K. A guideline for the clinical management of basal cell naevus syndrome (Gorlin-Goltz syndrome). Br J Dermatol. 2022 Feb;186(2):215-226. doi: 10.1111/bjd.20700. Epub 2021 Nov 8. PMID: 34375441; PMCID: PMC9298899.	S
Calik J, Oslizlo M, Słocka-Romaniuk B, Elsaftawy A, Sauer N. Case report: Sequential treatment strategy for advanced basal cell carcinoma in Gorlin-Goltz syndrome: integration of vismodegib, radiotherapy, surgery, and high-intensity focused ultrasound. Front Oncol. 2024 Jul 18;14:1428702. doi: 10.3389/fonc.2024.1428702. PMID: 39091908; PMCID: PMC11291366.	S
Palmeiro AG, Carvalho M, Gonçalves Castro C, Pimentel B, Catorze G. Vismodegib in Gorlin-Goltz syndrome: A systematic review. Australas J Dermatol. 2024 Sep;65(6):e123-e133. doi: 10.1111/ajd.14326. Epub 2024 Jun 12. PMID: 38867459.	S
Kesireddy M, Mendiola VL, Jana B, Patel S. Long-term Response to Vismodegib in a Patient with Gorlin-Goltz Syndrome: A Case Report and Review of Pathological Mechanisms Involved. Cureus. 2019 Aug 13;11(8):e5383. doi: 10.7759/cureus.5383. PMID: 31616614; PMCID: PMC6786843.	S

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
Stacy LaClaire, PharmD	None
Sarah Breeding, Pharm D	None

### ASSIGNMENT OF RATINGS:

\*to meet requirement 4

	EFFICACY	STRENGTH OF RECOMMENDATION	STRENGTH OF EVIDENCE
MERATIVE MICROMEDEX	Evidence Favors Efficacy	Class IIB	B

