

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: August 22, 2023

OFF-LABEL ID #: 2571

DRUG NAME: Temozolomide

OFF-LABEL USE: (Adult) Medulloblastoma; Relapsed or recurrent

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, S *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
Franceschi, E, Hofer, S, Brandes, AA, et al: EANO-EURACAN clinical practice guideline for diagnosis, treatment, and follow-up of post-pubertal and adult patients with medulloblastoma. <i>Lancet Oncol</i> Dec 2019; Vol 20, Issue 12; pp. e715-e728.	S
Luque, R, Benavdes, M, Del Barco, S, et al: SEOM clinical guideline for management of adult medulloblastoma (2020). <i>Clin Transl Oncol</i> May 2021; Vol 23, Issue 5; pp. 940-947.	S
Frappaz, D, Barritault, M, Montane, L, et al: MEVITEM-a phase I/II trial of vismodegib + temozolomide vs temozolomide in patients with recurrent/refractory medulloblastoma with Sonic Hedgehog pathway activation. <i>Neuro Oncol</i> Nov 02, 2021; Vol 23, Issue 11; pp. 1949-1960.	3
Bonney, PA, Santucci, JA, Maurer, AJ, et al: Dramatic response to temozolomide, irinotecan, and bevacizumab for recurrent medulloblastoma with widespread osseous metastases. <i>J Clin Neurosci</i> Apr 2016; Vol 26, pp. 161-163.	4
Durando, X, Thivat, E, Gilliot, O, et al: Temozolomide treatment of an adult with a relapsing medulloblastoma. <i>Cancer Invest</i> Sep 2007; Vol 25, Issue 6; pp. 470-475.	4
Arbabi-Kalati, F, Dadras, A, and Nami, M: An efficient and novel treatment regimen including temozolomide for medulloblastoma: A case study. <i>J Radiother Pract</i> 2023; Vol 22, p. e4.	4
Poelen, J, Bernsen, HJ, and Prick, MJ: Metastatic medulloblastoma in an adult; treatment with temozolomide. <i>Acta Neurol Belg</i> Jun 2007; Vol 107, Issue 2; pp. 51-54.	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
Richard LoCicero	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	Medulloblastoma is a rare malignancy in adults. Limited clinical trial data is available. A phase I/II trial has established a 20% response rate of temozolomide in relapsed/recurrent disease. The European Association of Neuro-Oncology and European Rare Cancer guidelines support the use of temozolomide in relapsed/recurrent disease. The Spanish Society of Medical Oncology clinical guidelines for management of adult medulloblastoma also support the use of temozolomide in relapsed/recurrent disease.	

Jeffrey Klein	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	The use of Temozolamide for adult medulloblastoma patients who have relapsed appears to be quite effective. It is not very clear if temozolamide can be used as monotherapy. The adverse effect profile is mild. The studies for this evaluation are small, and the most effective dose needs to be determined.	
Todd Gersten	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	In a multicenter single arm study, TMZ demonstrated a solid response rate and durability of disease control in a disease with limited treatment options. The lack of a comparator arm (i.e. placebo or other chemo) limits the efficacy and strength rating.	