IBM Watson Health

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: October 7, 2021

PACKET: 2147

DRUG: Vemurafenib

USE: Hairy cell leukemia (clinical); Relapsed/Refractory, monotherapy

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	STUDY-SPECIFIC COMMENTS	LITERATURE CODE
Parry-Jones, N, Joshi, A, Forconi, F, et al: Guideline for diagnosis and management of hairy cell leukaemia (HCL) and hairy cell variant (HCL-V). Br J Haematol Dec 2020; Vol 191, Issue 5; pp. 730-737.		S
Andrasiak, I, Rybka, J, and Wrobel, T: Response to the therapy in hairy cell leukemia: systematic review and meta-analysis. Clin Lymphoma Myeloma Leuk Jun 2018; Vol 18, Issue 6; pp. 392-399.e3.		2
Tiacci, E, Park, JH, De Carolis, L, et al: Targeting mutant BRAF in relapsed or refractory hairy-cell leukemia. N Engl J Med Oct 29, 2015; Vol 373, Issue 18; pp. 1733-1747.	This was a report of 2 prospective, single-arm phase 2 clinical trials (one in Italy, one in the US) that assessed vemurafenib monotherapy in patients with relapsed/refractory hairy-cell leukemia. The risk of bias associated with confounding, selection of participants, classification of and deviation from interventions, measurement of outcome, and selective reporting were all deemed low risk.	S
Tiacci, E, De Carolis, L, Simonetti, E, et al: Vemurafenib plus rituximab in refractory or relapsed hairy-cell leukemia. N Engl J Med May 13, 2021; Vol 384, Issue 19; pp. 1810-1823.		1
Dietrich, S, Pircher, A, Endris, V, et al: BRAF inhibition in hairy cell leukemia with low-dose vemurafenib. Blood Jun 09, 2016; Vol 127, Issue 23; pp. 2847-2855.		2
Robak, T, Janus, A, Jamroziak, K, et al: Vemurafenib and rituximab in patients with hairy cell leukemia previously treated with moxetumomab pasudotox. J Clin Med Jun 25, 2021; Vol 10, Issue 13; p. 2800.		1

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
Megan Smith	None		
Stacy LaClaire, PharmD	None		
Catherine Sabatos, PharmD	None		
		John Roberts	None
		Todd Gersten	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
IBM MICROMEDEX	Effective	Class IIb: Recommended, in Some Cases		B
Todd Gersten	Effective	Class I: Recommended	There is no standard of care treatment for relapsed/refractory HCL. Now two small studies reveal high response rates in BRAF mutated disease making vemurafenib the standard of care for this patient population.	
Richard LoCicero	Effective	Class IIb: Recommended, in Some Cases	Two phase 2 trials have established the efficacy of vemurafenib for the treatment of BRAF mutated, relapsed or refractory hairy cell leukemia. No unexpected toxicity was observed. A stronger recommendation is limited by the nature of a phase 2 trial and the absence of a comparison arm.	
John Roberts	Evidence is Inconclusive	Class IIb: Recommended, in Some Cases	Two small, single arm studies of vemurafinib in mutated BRAF hairy cell leukemia show high response rates, moderate toxicity, and disappointing response durations. It is a reasonable 2nd line choice, only in mutated BRAF disease.	