IBM Watson Health™

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: September 23, 2021

PACKET: 2129

DRUG: Vemurafenib

USE: Hairy cell leukemia (clinical); Relapsed/Refractory, in combination with rituximab

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.		4
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.		1
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.	This was a prospective, single-arm phase 2 clinical trial that assessed vemurafenib and rituximab 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
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.		1
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.		3

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 IIa: Recommended, in Most Cases		B
Todd Gersten	Effective	Class I: Recommended	There remains no standard of care for relapsed HCL. However, the combination of vemurafenib and Rituximab offers high response rates with over half of patients achieving negative minimal residual disease.	
Richard LoCicero	Effective	Class IIb: Recommended, in Some Cases	The combination of vemurafenib and rituximab was associated with high efficacy (87% complete response) in a small (30 patient) phase II trial. Unexpected toxicity was not observed.	
John Roberts	Effective	Class IIa: Recommended, in Most Cases	Treatment of hairy cell leukemia with single agent vemurafenib is associated with high response rates but short response durations. In a small, single arm study of vemurafenib in combination with rituximab for the treatment of BRAF V600E mutated, relapsed or refractory hairy cell leukemia, the complete response rate was high and response durations were long-lived. The combination is a reasonable, perhaps preferred, treatment for relapsed or refractory hairy cell leukemia that carries the BRAF V600E mutation.	