IBM Watson Health™

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: August 11, 2021

PACKET: 2123

DRUG: Acalabrutinib

USE: Waldenström macroglobulinemia

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, E, 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)]

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EVIDENCE CONSIDERED:

*to meet requirements 2 and 4

CITATION	STUDY-SPECIFIC COMMENTS	LITERATURE CODE
Castillo, JJ, Advani, RH, Branagan, AR, et al: Consensus treatment recommendations from the tenth International Workshop for Waldenstrom Macroglobulinaemia. Lancet Haematol Nov 2020; Vol 7, Issue 11; pp. e827-e837.		S
Owen, RG, McCarthy, H, Rule, S, et al: Acalabrutinib monotherapy in patients with Waldenstrom macroglobulinemia: a single-arm, multicentre, phase 2 study. Lancet Haematol Feb 2020; Vol 7, Issue 2; pp. e112-e121.	This was a prospective single-arm phase 2 clinical trial that investigated acalabrutinib in patients with Waldenström macroglobulinemia. The risk of bias due to confounding, selection, classification of and deviation from intervention, and missing data were deemed low risk. The risk of bias associated with measurement and selection of outcome were deemed high risk due to the primary outcome being investigator-assessed and overall response. A major caveat of the study is the lack of a control group.	S
None Listed: Correction to Lancet Haematol 2020; 7: e112-21. Lancet Hematol Apr 2021; Vol 8, Issue 4; p. e249.	Erratum for above	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	EXPERT REVIEW	DISCLOSURES
Megan Smith	None		
Stacy LaClaire, PharmD	None		
Catherine Sabatos, PharmD	None		
		John Roberts	None
		Todd Gersten	None
		Richard LoCicero	Incyte Corporation:

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			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	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases		B
Todd Gersten	Effective	Class I: Recommended	Acalabrutinib demonstrates high response rates and durability of disease control in treatment naive and relapsed refractory WM patients.	
Richard LoCicero	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	A single phase II trial demonstrated the efficacy of acalabrutinib for the treatment of Waldenstrom macroglobulinemia. In the 122 patient trial, Overall response rate was 93% without unexpected toxicity. Many other treatment options are available for this disease.	
John Roberts	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	In a single arm trial acalabrutinib showed promising activity in terms of response rates in both previously treated and treatment naive patients living with Waldenström macroglobulinemia.Toxicity was moderate. Several classes of agents have shown similar activity, and there is limited prospective comparative treatment data upon which to base clinical decision-making. Acalabrutinib is one option.	

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