IBM Watson Health

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: 10/21/2019

PACKET: 1912

DRUG: Pomalidomide

USE: AL amyloidosis; Relapsed or refractory, combination therapy with dexamethasone

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	STUDY-SPECIFIC COMMENTS	LITERATURE CODE
Dispenzieri, A, Buadi, F, Laumann, K, et al: Activity of pomalidomide in patients with immunoglobulin light-chain amyloidosis. Blood Jun 07, 2012; Vol 119, Issue 23; pp. 5397-5404.	This was an open-label, single-arm phase II clinical trial that investigated pomalidomide plus dexamethasone treatment in patients with previously treated light-chain amyloidosis. There was low risk of bias associated with selection of cohorts, assessment of outcomes, and follow-up. Data was gathered prospectively for objective outcomes based on international consensus, and analyses were performed on the intent-to-treat population. Median follow-up was 28.1 months (range, 14.1 to 37.8). One caveat is that the study lacked a control group.	S
Palladini, G, Milani, P, Foli, A, et al: A phase 2 trial of pomalidomide and dexamethasone rescue treatment in patients with AL amyloidosis. Blood Apr 13, 2017; Vol 129, Issue 15; pp. 2120-2123.	This was an open-label, single-arm phase II clinical trial that investigated pomalidomide plus dexamethasone treatment in patients with previously treated light-chain amyloidosis. There was low risk of bias associated with selection of cohorts, assessment of outcomes, and follow-up. Data was gathered prospectively for objective outcomes based on international consensus, and analyses were performed on the intent-to-treat population. Median follow-up was 44 months. One caveat is that the study lacked a control group.	S
Sanchorawala, V, Shelton, AC, Lo, S, et al: Pomalidomide and dexamethasone in the treatment of AL amyloidosis: results of a phase 1 and 2 trial. Blood Aug 25, 2016; Vol 128, Issue 8; pp. 1059-1062.		3
Sharpley, FA, Manwani, R, Mahmood, S, et al: Real world outcomes of pomalidomide for treatment of relapsed light chain amyloidosis. Br J Haematol Nov 2018; Vol 183, Issue 4; pp. 557-563.		3
Wechalekar,A.D., Gillmore,J.D., Bird,J., et al: Guidelines on the management of AL amyloidosis. British Journal of Haematology Jan 01, 2015; Vol 168, Issue 2; pp. 186-206.		S

Vaxman, I and Gertz, M: Recent advances in the diagnosis, risk stratification, and management of systemic light-chain amyloidosis. Acta Haematol 2019; Vol 141, Issue 2; pp. 93-106.		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
Megan Smith	None		
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
IBM MICROMEDEX	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases		B
Richard LoCicero	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	Limited phase II trials have established efficacy of pomalidomide combined with dexamethaone in relapsed/refractory AL amyloidosis. No unexpected toxicity was observed. Treatment for AL amyloidosis follows treatment used in the management of myeloma. Pomalidomide is an established therapy in myeloma.	
Jeffrey Klein	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	The use of Pomalidomide with dexamethasone shows a good degree of efficacy. The results were seen in a few months and were sustained. The incidence of infections and neutropenia might limit the use of Pomalidomide.o	
John Roberts	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	Two small single arm studies show promising response rates for pomalidomide + dexamethasone in relapsed or refractory AL amyloidosis. Whether these responses reflect improvement in survival or improvement in quality of life is unknown. There are many treatment options for relapsed or refractory AL amyloidosis, but there are very few comparative trials to guide treatment selection. Treatments should be selected on the basis of prior therapy and consideration of the risk profiles of the various regimens as compared with disease and other co-morbidities in the individual patient.	