

COMPENDIA TRANSPARENCY TRACKING FORM

DRUG: Thalidomide

INDICATION: Systemic mast cell disease

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
Gruson,B., Lortholary,O., Canioni,D., et al: Thalidomide in systemic mastocytosis: results from an open-label, multicentre, phase II study. Br J Haematol May 2013; Vol 161, Issue 3; pp. 434-442.	<u>Study methodology comments:</u> This was an open-label, single-arm phase II clinical trial. There was low risk of bias associated with selection of cohorts and assessment of outcomes. Data was gathered prospectively for objective outcomes. Four subjects were not included in the efficacy analyses. The results should be interpreted with caution since the study lacked a control group.	S
Gruson,B., Lortholary,O., Canioni,D., et al: Thalidomide in advanced mastocytosis. results from an open-label, multicentric, phase II study. Blood Nov 18, 2011; Vol 118, Issue 21.		3
Damaj,G., Bernit,E., Ghez,D., et al: Thalidomide in advanced mastocytosis. Br J Haematol Apr 2008; Vol 141, Issue 2; pp. 249-253.		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
Margi Schiefelbein, PA	None	Thomas McNeil Beck, MD	None
Stacy LaClaire, PharmD	None	James E. Liebmann, MD	None
Felicia Gelsey, MS	None	Jeffrey A. Bubis, DO	Other payments: Dendreon
		Keith A. Thompson, MD	None
		John M. Valgus, PharmD	None

ASSIGNMENT OF RATINGS:

*to meet requirement 4

	EFFICACY	STRENGTH OF RECOMMENDATION	COMMENTS	STRENGTH OF EVIDENCE
MICROMEDEX	---	---		B
Thomas McNeil Beck, MD	Evidence favors efficacy	Class IIb - Recommended, In Some Cases	A rare disease without abundant therapeutic options.	N/A
James E. Liebmann, MD	Evidence is inconclusive	Class IIb - Recommended, In Some Cases	There is no good standard therapy for systemic mastocytosis. This is an uncommon disease and it is unlikely there will ever be a randomized trial comparing treatment options. The Gruson et al study documents some improvement in rash and symptoms with thalidomide. Serious side effects were uncommon. Thalidomide may be useful for a time in an occasional patient with this disease.	N/A
Jeffrey A. Bubis, DO	Evidence favors efficacy	Class IIb - Recommended, In Some Cases	In patients that have failed other therapies, thalidomide is a reasonable option has roughly half of patients achieve at least a transient response with minimal toxicity.	N/A

Keith A. Thompson, MD	Evidence favors efficacy	Class IIb - Recommended, In Some Cases	None	N/A
John M. Valgus, PharmD	Evidence is inconclusive	Class IIb - Recommended, In Some Cases	This was a small non-comparative trial which demonstrated activity of thalidomide but with questionable clinical benefit. Although a high-rate of responses were seen, they were short-lived. Some symptoms were reduced but over all QOL was not impacted and side effects were burdensome.	N/A