COMPENDIA TRANSPARENCY TRACKING FORM

DATE: June 5, 2024

OFF-LABEL ID #: 2675

DRUG NAME: Bortezomib

OFF-LABEL USE: Thrombotic thrombocytopenic purpura Relapsed or refractory

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	LITERATURE CODE
Lee, NCJ, Yates, S, Rambally, S, et al: Bortezomib in relapsed/refractory immune thrombotic thrombocytopenic purpura: a single-centre retrospective cohort and systematic literature review. Br J Haematol Feb 2024; Vol 204, Issue 2; pp. 638-643. Pubmed ID: 37571963 Publication Types: Systematic Review CC CMSID: CMS3734392	S
Yin, J, Tian, H, Kong, D, et al: Bortezomib, a promising alternative for patients with refractory or relapsed thrombotic thrombocytopenic purpura after rituximab treatment. Br J Haematol Nov 2022; Vol 199, Issue 4; pp. 619-622. Pubmed ID: 36076349 Publication Types: Letter; Research Support, Non-US. Gov't CC CMSID: CMS3734628	2

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
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
MERATIVE MICROMEDEX	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases		B
Jeffrey Klein	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	The use of bortezomib for TTP patients who failed rituximab, PLEX. and corticosteroids, appears to be very effective and well tolerated in this small limited study. Most patients were female in the study.	
Todd Gersten	Effective	Class I: Recommended	Bortezomib demonstrates a high degree of efficacy, with often durable complete remissions in a population/disease with very limited, if any, treatment options.	

Warren Brenner	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	I think this study shows that bortezomib appears to have good efficacy and well tolerated in a difficult patient population. As the study is retrospective I thought the evidence favors efficacy but ranked less than effective due to predominant retroactive data. I recommended in most cases as the complete response rate is high in a difficult to treat population in a highly complex disease.	