TRUVEN HEALTH ANALYTICS™

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: **November 2015**

PACKET: **1189**

DRUG: **Romiplostim**

USE: **Thrombocytopenic disorder and Myelodysplastic syndrome**

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, 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)]

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EVIDENCE CONSIDERED:

*to meet requirements 2 and 4

CITATION	STUDY-SPECIFIC COMMENTS	LITERATURE CODE
Prica,A., Sholzberg,M., and Buckstein,R.: Safety and efficacy of thrombopoietin-receptor agonists in myelodysplastic syndromes: a systematic review and meta-analysis of randomized controlled trials. Br J Haematol. Dec 2014; Vol 167, Issue 5; pp. 626-638.	Comments: This was a high quality systematic review that included five placebo-controlled, randomized trials of 384 patients. The risk of bias tool was used to assess the quality of the included trials. There was no serious risk of bias across studies for the outcomes. Additionally, the overall quality of evidence for each outcome was deemed moderate due to serious imprecision across studies for each outcome.	S
Giagounidis,A., et al: Results of a randomized, double-blind study of romiplostim versus placebo in patients with low/intermediate-1-risk myelodysplastic syndrome and thrombocytopenia. Cancer Jun 15, 2014; Vol 120, Issue 12; pp. 1838-1846.		2
Wang,E.S., et al: A randomized, double-blind, placebo-controlled phase 2 study evaluating the efficacy and safety of romiplostim treatment of patients with low or intermediate-1 risk myelodysplastic syndrome receiving lenalidomide. J Hematol.Oncol 2012; Vol 5, p. 71.		2
Greenberg,P.L., et al: A randomized controlled trial of romiplostim in patients with low- or intermediate-risk myelodysplastic syndrome receiving decitabine. Leukemia & lymphoma Feb 2013; Vol 54, Issue 2; pp. 321-328.		2

Sekeres,M.A., Kantarjian,H., Fenaux,P., et al: Subcutaneous or intravenous administration of romiplostim in thrombocytopenic patients with lower risk myelodysplastic syndromes. Cancer Mar 01, 2011; Vol 117, Issue 5; pp. 992-1000.		3
Killick,S.B., Carter,C., Culligan,D., et al: Guidelines for the diagnosis and management of adult myelodysplastic syndromes. British Journal of Haematology Feb 2014; Vol 164, Issue 4; pp. 503-525. Pubmed		4
Brierley,C.K. and Steensma,D.P.: Thrombopoiesis-stimulating agents and myelodysplastic syndromes. Br J Haematol. May 2015; Vol 169, Issue 3; pp. 309-323.		4
Lotfi,R., Moeller,P., Schmid,M., et al: Increased requirement for platelet transfusions concurrent with enhanced bleeding during romiplostim treatment in a patient with thrombocytopenia due to bone marrow failure. Annals of Hematology Nov 2011; Vol 90, Issue 11; pp. 1357-1359.		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
Felicia Gelsey, MS	None	Edward Balaban, DO	None
Stacy LaClaire, PharmD	None	Keith Thompson, MD	None
Catherine Sabatos, PharmD	None	James E. Liebmann, MD	None

ASSIGNMENT OF RATINGS:

*to meet requirement 4

	EFFICACY	STRENGTH OF RECOMMENDATION	COMMENTS	STRENGTH OF EVIDENCE
MICROMEDEX	---	---		B
Edward Balaban, DO	Ineffective	Class lll: Not Recommended	Data including Meta-Analyses demonstrates little efficacy and added risk.	N/A
Keith Thompson, MD	Ineffective	Class lll: Not Recommended	None	N/A

James E. Liebmann, MD	Evidence is Inconclusive	Class lll: Not Recommended	The review presented identified four small trials of romiplostim in patients with myelodysplasia (MDS). The meta-analysis showed minimal reduction in bleeding with the use of romiplostim and a modest reduction in platelet transfusions with the drug. However, there is a suggestion of an increased risk of progression to acute myeloid leukemia (AML) in patients treated with romiplostim, particularly patients with high risk MDS. Overall, the results are intriguing and merit further evaluation of romiplostim in larger trials in patients with MDS. However, the meager available data do no support routine use of the drug in patients with MDS outside of a clinical trial.	N/A