TRUVEN HEALTH ANALYTICS
an IBM Company

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: 9/6/2018

PACKET: 1699

DRUG: Pegfilgrastim

USE: Harvesting of peripheral blood stem cells, prior to autologous stem-cell transplantation

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, P *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)]

TRUVEN HEALTH ANALYTICS
an IBM Company

EVIDENCE CONSIDERED:

*to meet requirements 2 and 4

CITATION	STUDY-SPECIFIC COMMENTS	LITERATURE CODE
Cesaro et al. A Phase II study on the safety and efficacy of a single dose of pegfilgrastim for mobilization and transplantation of autologous hematopoietic stem cells in pediatric oncohematology patients. TRANSFUSION 2011;51:2480-2487.	Comments: This was a multicenter, phase II study that evaluated the safety and efficacy of a single dose of 100 mg/kg pegfilgrastim in mobilizing PBSCs in pediatric patients who were candidates for autologous PBSC transplantation. The study included matched historical controls. There was low risk of bias associated with selection of cohorts and assessment of outcomes. Data was gathered prospectively for objective outcomes. Data was gathered from medical records for the matched historical controls. Study met power requirements.	S
Merlin,E., Zohar,S., Jerome,C., et al: Hematopoietic progenitor cell mobilization and harvesting in children with malignancies: do the advantages of pegfilgrastim really translate into clinical benefit?. Bone Marrow Transplant Jun 2009; Vol 43, Issue 12; pp. 919-925.	Comments: This was a phase IIA open-label, single-center, prospective study. The study was stopped after the inclusion of 26 patients due to analyses showing that the stopping criterion was met. Sixteen of twenty-six patients met the success criterion. After 26 inclusions, the Bayesian analysis gave a mean estimated success rate of 60.7% (95% credibility interval: 42.0–78.0%). The stopping criterion associated with inefficacy was not met, the probability that the success rate was lower than 30% after 26 inclusions being estimated at 0.0005. The predictive probability of observing two or more successes in the next five patients was 90%. All clinical and biological data were recorded by the same investigator, with particular attention being paid to previously reported adverse events related to filgrastim or pegfilgrastim administration. There was low risk of bias associated with selection of cohorts and assessment of outcomes. Authors assessed potential confounding factors.	S
Kuan,J.W., Su,A.T., Wong,S.P., et al: A randomized double blind control trial comparing filgrastim and pegfilgrastim in cyclophosphamide peripheral blood hematopoietic stem cell mobilization. Transfus.Apher Sci. Oct 2015; Vol 53, Issue 2; pp. 196-204.	Comments: This was a triple blinded (investigator, subjects, and statistician), randomized controlled, single center trial that compared the effectiveness of filgrastim and pegfilgrastim in PBSC mobilization. Key bias criteria evaluated were (1) random sequence generation of randomization; (2) lack of allocation concealment, (3) lack of blinding, (4) incomplete accounting of patients and outcome events, and (5) selective outcome reporting bias. The study was at low risk of bias for these key criteria, and no additional biases were identified.	1

Cesaro,S., Tintori,V., Nesi,F., et al: A prospective study on the efficacy of mobilization of autologous peripheral stem cells in pediatric oncohematology patients. Transfusion Jul 2013; Vol 53, Issue 7; pp. 1501-1509.		3
Fritsch,P., Schwinger,W., Schwantzer,G., et al: Peripheral blood stem cell mobilization with pegfilgrastim compared to filgrastim in children and young adults with malignancies. Pediatr Blood Cancer Jan 2010; Vol 54, Issue 1; pp. 134-137. Pubmed ID		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
Felicia Gelsey, MS	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
MICROMEDEX	Evidence Favors Efficacy	Class IIa: Recommended, In Most		B
John D Roberts	Effective	Class I: Recommended	One case control study and one prospective study show that in combination with chemotherapy pegfilgastrim is at least as effective as filgastrim for mobilization of peripheral blood stem cells for autologous stem cell transplantation following high dose chemotherapy. Both studies assessed frequency of successful harvest, and the case control study assessed long term events (engraftment, complications of cytopenias).	N/A
Jeffrey Klein	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	The use of Pegfilgrastim in children to assist in harvesting stem cells prior to transplant appears to be effective and demonstrates a clinical benefit over filgrastim. In addition pegfilgrastim had minimal adverse effects.	N/A
Richard LoCicero	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	At least two phase II studies have demonstrated the efficacy of pegfilgrastim to mobilize peripheral blood stem cells prior to autologous stem cell transplant. Pegfilgrastim is a PEG-conjugated fomr of G-CSF (filgrastim) that is currently used. The PEG-conjugated form allows for a single injection instead of multiple injections. No unexpected toxicity was observed with the use of pegfilgrastim.	N/A