IBM Watson Health

COMPENDIA TRANSPARENCY TRACKING FORM

DATE: 5/15/2019

PACKET: 1899

DRUG: Alemtuzumab

USE: (PEDIATRIC) Graft versus host disease, in patients receiving allogeneic stem cell transplant for hematologic malignancies, steroid-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: A, C, 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
Khandelwal,P., Lawrence,J., Filipovich,A.H., et al: The successful use of alemtuzumab for treatment of steroid-refractory acute graft-versus-host disease in pediatric patients. Pediatric Transplantation Feb 2014; Vol 18, Issue 1; pp. 94-102.	This was a single-center retrospective study. There was a low risk of bias associated with selection of cohorts and assessment of outcomes. Data was gathered from medical records. All subjects were included in the analyses. The study did not include a control group.	S
Khandelwal,P., Emoto,C., Fukuda,T., et al: A prospective study of alemtuzumab as a second-line agent for steroid-refractory acute graft-versus-host disease in pediatric and young adult allogeneic hematopoietic stem cell transplantation. Biology of Blood and Marrow Transplantation 2016; Vol 22, Issue 12; pp. 2220-2225.	This was a single-center observational study. There was a low risk of bias associated with selection of cohorts and assessment of outcomes. Data was gathered prospectively for objective outcomes. An interim analysis was performed after every 5 enrolled patients to adjust dosage. All subjects were included in the analyses. The study did not include a control group.	S
Meunier,M., Bulabois,C.E., Thiebaut-Bertrand,A., et al: Alemtuzumab for severe steroid-refractory gastrointestinal acute graft-versus-host disease. Biology of Blood and Marrow Transplantation 2014; Vol 20, Issue 9; pp. 1451-1454.		1

Schnitzler,M., Hasskarl,J., Egger,M., et al: Successful treatment of severe acute intestinal graft-versus-host resistant to systemic and topical steroids with alemtuzumab. Biology of Blood and Marrow Transplantation 2009; Vol 15, Issue 8; pp. 910-918.		1
Gomez-Almaguer,D., Ruiz-Arguelles,G.J., del Carmen Tarin-Arzaga,L, et al: Alemtuzumab for the treatment of steroid-refractory acute graft-versus-host disease. Biol.Blood Marrow.Transplant Jan 2008; Vol 14, Issue 1; pp. 10-15.		1
Martinez,C., Solano,C., Ferra,C., et al: Alemtuzumab as treatment of steroid-refractory acute graft-versus-host disease: results of a phase II study. Biol.Blood Marrow.Transplant May 2009; Vol 15, Issue 5; pp. 639-642.		1

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		
Margi Schiefelbein, PA	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
Jeffrey Klein	Evidence Favors Efficacy	Class IIa: Recommended, in Most Cases	The use of Alemtuzumab to treat GVHD pediatric patients (2nd line-steroid refractory) showed a very good response rate in this small study. Most patients were even able to discontine their steroids. This treatment did have a high rate of infections (despite prophylaxis agents being given), that were treated with antimicrobials. The optimal dose is not fully established as well.	
Richard LoCicero	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	Retrospective and observational studies have shown efficacy of Alemtuzumab in the treatment of pediatric steroid-refractory GVHD (after allogeneic stem transplant for hematologic malignancies). Infectious complications were common. The absence of control groups limits other conclusions.	
John D Roberts	Evidence Favors Efficacy	Class IIb: Recommended, in Some Cases	Alemtuzumab showed clinically meaningful responses in pediatric acute graft versus host disease following allogeneic stem cell transplant in one retrospective and one prospective single center studies, which had no comparator arms. There are other therapeutic options with similar evidence of efficacy.	