

COMPENDIA TRANSPARENCY TRACKING FORM

DRUG: Rituximab

INDICATION: Immune thrombocytopenia, previously treated [pediatric]

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, P, 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
<p>Liang,Y., Zhang,L., Gao,J., et al: Rituximab for children with immune thrombocytopenia: a systematic review. PLoS ONE [Electronic Resource] 2012; Vol 7, Issue 5; p. e36698.</p>	<p><u>Study methodology comments:</u> This was a meta-analysis that included 18 nonrandomized studies (352 patients) for efficacy assessment in children with primary ITP. A total of 30 nonrandomized trials were included. The authors evaluated the quality of the included studies with an assessment tool developed for case series by NICE. The analyses showed a moderate to high degree of heterogeneity (I^2 ranged from 56.7% to 67.5%) but the confidence intervals overlapped by a substantial amount. All of the criteria of the SR/MA worksheet were fulfilled.</p> <p><u>Clinical comments:</u> Comments from TR writer/reviewer</p> <p><u>Reviewer comments:</u> Comments from external reviewers</p>	<p>S</p>
<p>Akbayram,S., Dogan,M., Ustyol,L., et al: The clinical outcome of 260 pediatric ITP patients in one center. Clinical and Applied Thrombosis/Hemostasis 2011; Vol 17, Issue 6; pp. E30-E35.</p>		<p>2</p>
<p>Stiakaki,E., Perdikogianni,C., Thomou,C., et al: Idiopathic thrombocytopenic purpura in childhood: twenty years of experience in a single center. Pediatr Int Aug 2012; Vol 54, Issue 4; pp. 524-527.</p>		<p>2</p>
<p>Patel,V.L., Mahevas,M., Lee,S.Y., et al: Outcomes 5 years after response to rituximab therapy in children and adults with immune thrombocytopenia. Blood Jun 21, 2012; Vol 119, Issue 25; pp. 5989-5995.</p>		<p>2</p>

Mueller,B.U., Bennett,C.M., Feldman,H.A., et al: One year follow-up of children and adolescents with chronic Immune Thrombocytopenic Purpura (ITP) treated with rituximab. Pediatric Blood and Cancer Feb 2009; Vol 52, Issue 2; pp. 259-262.		2
Matsubara,K., Takahashi,Y., Hayakawa,A., et al: Long-term follow-up of children with refractory immune thrombocytopenia treated with rituximab. Int J Hematol Apr 2014; Vol 99, Issue 4; pp. 429-436.	<u>2</u>	2
Parodi,E., Nobili,B., Perrotta,S., et al: Rituximab (anti-CD20 monoclonal antibody) in children with chronic refractory symptomatic immune thrombocytopenic purpura: Efficacy and safety of treatment. International Journal of Hematology 2006; Vol 84, Issue 1; pp. 48-53.		2
Bader-Meunier,B., Aladjidi,N., Bellmann,F., et al: Rituximab therapy for childhood Evans syndrome. Haematologica Dec 2007; Vol 92, Issue 12; pp. 1691-1694.		2
Gokcebay,D.G., Tavit,B., Fettah,A., et al: Evaluation of children with chronic immune thrombocytopenic purpura and evans syndrome treated with rituximab. Clinical and Applied Thrombosis/Hemostasis 2013; Vol 19, Issue 6; pp. 663-667.		2
Del Vecchio,G.C., De,Santis A., Accettura,L., et al: Chronic immune thrombocytopenia in childhood. Blood Coagul.Fibrinolysis Jun 2014; Vol 25, Issue 4; pp. 297-299.		4

<p>Cooper,N.: A review of the management of childhood immune thrombocytopenia: how can we provide an evidence-based approach?. Br J Haematol Apr 25, 2014; Vol E Pub, p. 1.</p>		<p>4</p>
<p>Schweizer,C., Reu,F.J., Ho,A.D., et al: Low rate of long-lasting remissions after successful treatment of immune thrombocytopenic purpura with rituximab. Annals of Hematology Oct 2007; Vol 86, Issue 10; pp. 711-717.</p>		<p>1</p>
<p>Penalver,F.J., Jimenez-Yuste,V., Almagro,M., et al: Rituximab in the management of chronic immune thrombocytopenic purpura: An effective and safe therapeutic alternative in refractory patients. Annals of Hematology Jun 2006; Vol 85, Issue 6; pp. 400-406.</p>		<p>1</p>

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	Edward P. Balaban, DO	None
Stacy LaClaire, PharmD	None	Jeffrey Patton, MD	None
Felicia Gelsey, MS	None	Jeffrey A. Bubis, DO	None
		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				Insert SOE
Edward P. Balaban, DO	Effective	Class I: Recommended	There is enough literature with both adult and pediatric immune thrombocytopenia to support the use of Rituximab in this setting	N/A
Jeffrey Patton, MD	Evidence Favors Efficacy	Class IIa: Recommended, In Most Cases	Good response rates and duration for ITP	N/A
Jeffrey A. Bubis, DO	Evidence Favors Efficacy	Class IIb: Recommended, In Some Cases	No randomized trial data, but non-randomized data is compelling. Long term safety data is also lacking. In patients that fail standard therapy, this should be considered.	N/A
Keith A. Thompson, MD	Evidence Favors Efficacy	Class IIb: Recommended, In Some Cases	None	N/A

John M. Valgus, PharmD	Evidence Favors Efficacy	Class IIb: Recommended, In Some Cases	The existing evidence suggests a high response rate with rituximab therapy in this population and that this response is durable in many patients. The strength of the data suffers from a lack of comparative, controlled trials, however, this type of study is unlikely due to the relatively uncommon nature of this disease.	N/A
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