National Institute for Health and Care Excellence

Health Technology Evaluation

Ruxolitinib for treating polycythaemia vera (review of TA356) [ID5106]
Response to stakeholder organisation comments on the draft remit and draft scope

Please note: Comments received in the course of consultations carried out by NICE are published in the interests of openness and transparency, and to promote understanding of how recommendations are developed. The comments are published as a record of the submissions that NICE has received, and are not endorsed by NICE, its officers or advisory committees.

Comment 1: the draft remit and proposed process

Section	Stakeholder	Comments [sic]	Action
Appropriateness of an evaluation and proposed evaluation route	Novartis	Novartis agree with the evaluation and proposed evaluation route.	Comment noted. No action required.
Wording	Novartis	Novartis agree with the wording of the draft scope.	Comment noted. No action required.
Additional comments on the draft remit	Novartis	Novartis find the timing of the draft scope to be appropriate.	Comment noted. No action required.

Comment 2: the draft scope

Section	Consultee/ Commentator	Comments [sic]	Action
Background information	Novartis	*"No epidemiological data are available about the proportion of people receiving treatment for polycythaemia vera whose disease transforms into acute myeloid leukaemia."* There are multiple studies providing epidemiological data on the proportion of patients who develop acute myeloid leukaemia (AML) following polycythaemia vera (PV): • Cerquozzi et al (2015) reported a cumulative risk of leukaemic transformation in patients with PV of 2.3–14.4% at 10 years, 5.5–18.7% at 15 years and 7.9-17% at 20 years. This should be reflected in the background information. References: Cerquozzi S, Tefferi A.Blood Cancer J. Blast transformation and fibrotic progression in polycythemia vera and essential thrombocythemia: a literature review of incidence and risk factors. 2015 Nov 13;5(11):e366. doi: 10.1038/bcj.2015.95.	Comment noted. The scope provides a very brief description of the disease. The company will be able to include this information in their submission. No change required.
The technology/ intervention	Novartis	"Ruxolitinib has a marketing authorisation in the UK for the treatment of patients with polycythaemia vera, who are resistant to or intolerant of hydroxycarbamide, with or without splenomegaly". The presence/absence of splenomegaly is not mentioned in the licence. Therefore, for consistency with the licence wording, please amend as below:	Comment noted. The technology section has been amended accordingly.

Section	Consultee/ Commentator	Comments [sic]	Action
		"Ruxolitinib has a marketing authorisation in the UK for the treatment of patients with polycythaemia vera, who are resistant to or intolerant of hydroxycarbamide".	
Population	Novartis	The population is appropriately defined.	Comment noted. No action required.
Subgroups	Novartis	The subgroups considered are relevant.	Comment noted. No action required.
Comparators	Novartis	No comment.	Comment noted. No action required.
Outcomes	Novartis	Time to treatment discontinuation should be included as an outcome as this is a clinically relevant outcome.	Comment noted. The outcome section has been amended accordingly.
Equality	Novartis	No comment.	Comment noted. No action required.
Other considerations	Novartis	None	Comment noted. No action required.
Innovation	Novartis	There are currently no treatments recommended by NICE for the treatment of patients with PV who are resistant to or intolerant of hydroxycarbamide (HC), also referred as hydroxyurea (HU). Only a small proportion of patients who are resistant to or intolerant to HC/HU will subsequently achieve a response when treated with currently available cytoreductive therapies. Ruxolitinib can be considered a step-change in the management of PV as it is a treatment	Comment noted. The scope is a brief document. The company will be able to present its case for

Section	Consultee/ Commentator	Comments [sic]	Action
		option for patients resistant to or intolerant to HC/HU and provides greater response compared to current available therapy.	innovation in their submission.
Questions for consultation	Novartis	1) Where do you consider ruxolitinib will fit into the existing care pathway for polycythaemia vera? Second-line after HC/HU treatment.	Comment noted. No action required.
	Novartis	2) Would ruxolitinib be a candidate for managed access? No, as no further pivotal studies are planned, and existing pivotal trials (RESPONSE & RESPONSE-2) are complete & been fully reported	Comment noted. No action required.
Additional comments on the draft scope	Novartis	None	Comment noted. No action required.

The following stakeholders indicated that they had no comments on the draft remit and/or the draft scope

None