

## National Institute for Health and Care Excellence

## Health Technology Evaluation

**Sirolimus gel for treating facial angiofibroma from tuberous sclerosis complex in people 6 years and older  
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	Tuberous Sclerosis Association	Tuberous Sclerosis Association agrees with NICE that the treatment should be evaluated through its Single Technology Appraisal process	Thank you for your comment. No action needed.
Wording	Tuberous Sclerosis Association	Tuberous Sclerosis Association supports the wording of the remit as set out on the draft scope.	Thank you for your comment. No action needed.
Timing issues	Tuberous Sclerosis Association	Timing issues: Tuberous Sclerosis Complex (TSC) associated facial angiofibromas have been reported in up to 74.5% of paediatric TSC patients, rising to up to 88% in adults >30 years old. The facial appearance resulting from facial angiofibroma lesions is associated with high psychological and physical morbidity (for example, recurrent bleeding or nasal obstruction), as such it may be considered alongside acne vulgaris, port wine stains, atopic dermatitis, congenital melanocytic nevi and other conditions as a	Thank you for your comment. No action needed.

Section	Stakeholder	Comments [sic]	Action
		psychodermatological condition with both physical and psychosocial impacts. The onset of facial dermatological conditions during school-age and adolescence has been found to cause a particularly negative psychosocial impact at a time when peer relationships gain importance and self-concept matures. Therefore, Tuberous Sclerosis Association considers this an urgent topic that should be appraised as soon as possible.	
Additional comments on the draft remit	Tuberous Sclerosis Association	No comment.	No action needed.

**Comment 2: the draft scope**

Section	Consultee/ Commentator	Comments [sic]	Action
Background information	Tuberous Sclerosis Association	No comment.	No action needed.
Population	Tuberous Sclerosis Association	Tuberous Sclerosis Association considers the population to be defined correctly.	Thank you for your comment. No action needed.
Subgroups	Tuberous Sclerosis Association	Tuberous Sclerosis Association believes that subgroups by age should be considered. Young children and adolescents with TSC will benefit most from the licenced sirolimus treatment. It is well documented that paediatric skin disorders can affect children's self-esteem, relationships with caregivers and peers, and performance in school and activities [Vivar and Kruse, 2017. Int J Womens Dermatology 12;4(1):27-31]. The onset of dermatological conditions	Thank you for your comments. The population included in the scope for consideration by the committee is in line with

Section	Consultee/ Commentator	Comments [sic]	Action
		<p>like facial dermatological conditions during school-age and adolescence has been found to cause a particularly negative psychosocial impact at a time when peer relationships gain importance and self-concept matures. It often leads to bullying and mental health issues.</p> <p>In 2020, Egawa et al published a report highlighting improved health-related quality of life in patients treated with sirolimus gel for TSC-associated facial angiofibromas. In their study, the three scale scores associated with mental health were significantly improved compared to before the treatment. They also noted that the health-related quality of life in patients receiving sirolimus gel treatment was more strongly affected by the treatment efficacy than adverse events [Egawa et al, 2020. Orphanet J Rare Dis; 5: 133]</p>	<p>the marketing authorisation for sirolimus gel. That is, people aged 6 years and over. Current treatment options for people with TSC-associated facial angiofibroma are understood to be the same across all age groups. So, age has not been included as a subgroup in the scope. The committee may consider subgroups for whom sirolimus gel might be particularly clinically effective or provide value for money in its decision making if the evidence allows. The committee's recommendations will also consider whether its recommendations could have a different impact on people protected by the</p>

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			equality legislation than on the wider population.
Comparators	Tuberous Sclerosis Association	Tuberous Sclerosis Association believes all relevant comparators have been included in the draft scope.	No action needed.
Outcomes	Tuberous Sclerosis Association	Tuberous Sclerosis Association suggest that the following relevant outcome measure is added to the scope to differentiate it from the health-related quality of life of the patient:  <ul style="list-style-type: none"> <li>Caregiver health-related quality of life</li> </ul>	Thank you for your comment. The scope includes the outcome “health-related quality of life”, and this would include carers. Therefore, no changes to the scope have been made.
Equality	Tuberous Sclerosis Association	No comment.	No action.
Other considerations	Tuberous Sclerosis Association	No comment.	No action.
Questions for consultation	Tuberous Sclerosis Association	No comment.	No action.

Section	Consultee/ Commentator	Comments [sic]	Action
Additional comments on the draft scope	Tuberous Sclerosis Association	No comment.	No action.

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

- Plusultra
- Genetic alliance
- Neonatal and Paediatrics Pharmacists Group (NPPG)