NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Ruxolitinib for treating non-segmental vitiligo in people 12 years and over

Draft scope

Draft remit/appraisal objective

To appraise the clinical and cost effectiveness of ruxolitinib within its marketing authorisation for treating non-segmental vitiligo in people 12 years and over.

Background

Vitiligo is a chronic auto-immune condition in which areas of the skin lose its normal colour (pigment) and become very pale, white or light pink. Depigmentation (loss of colour) occurs when melanocytes, the cells that make the pigment, melanin become inactive. The causes are unclear but may be related to disorders of the immune system, family history or trigger events such as skin trauma, severe sunburn or stress. The extent and speed that vitiligo affects the skin are unpredictable, and can range from small single patches to a total loss of colour. Vitiligo commonly affects the face, neck, hands and skin creases. It can be classified as segmental (unilateral or localised) in which only one area of the body is affected or non-segmental where symmetrical patches can appear on both sides of the body.[1,2] Non-segmental vitiligo may be categorised as:

- focal (small, isolated, depigmented patch without an obvious segmental distribution pattern)
- acrofacial (depigmented patches on the hands, feet and or face, especially around orifices)
- mucosal (depigmented patches on the mouth and/or genital mucosa)
- follicular (depigmented body hairs)
- generalised (usually symmetrical, depigmented patches randomly occurring over the entire body)
- universal (complete or nearly complete skin depigmentation)
- mixed (both segmental and non-segmental forms co-exist), or
- unclassified (isolated mucosal involvement and persistent focal vitiligo).[3]

Vitiligo affects males and females, and all ethnicities equally but it is more noticeable in people with darker skin tones. It can start at any age, but in more than half of the people with vitiligo, it appears before 20 years of age. In the UK, it is estimated that 1 in 100 people have vitiligo, of which 85% to 90% have the non-segmental type.[1,2] Based on 2020 figures, it is estimated that at least 507,620 people in England and Wales have non-segmental vitiligo.[4]

There are currently no licensed treatments for vitiligo. Current off-label pharmacological treatments include corticosteroids and calcineurin inhibitors such as topical pimecrolimus or topical tacrolimus. Other treatments include phototherapy, excimer laser treatment, skin camouflage, depigmentation (removal of the remaining

pigment) and surgery. Advice may be given on sun protection, vitamin D supplements and psychological treatments.[1]

The technology

Ruxolitinib (Opzelura, Incyte) is a topical formulation.

Ruxolitinib does not currently have a marketing authorisation in the UK for non-segmental vitiligo. It is being studied in clinical trials comparing ruxolitinib with vehicle cream in people aged 12 years and older with non-segmental vitiligo.

Intervention	Ruxolitinib cream
Population	People aged 12 years and older with non-segmental vitiligo
Comparators	Established clinical management without ruxolitinib
Outcomes	The outcome measures to be considered include: • re-pigmentation • maintenance of response • cessation of spread or stabilisation of vitiligo • global assessment of vitiligo • cosmetic acceptability • adverse effects of treatment • health related quality of life.
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.
Related NICE recommendations	None.
Related National Policy	The NHS Long Term Plan, 2019. NHS Long Term Plan Department of Health and Social Care, NHS Outcomes Framework 2016-2017: Domains 1 to 5.

	NHS England (2013) 2013/14 NHS Standard Contract For Specialised Dermatology Services (All Ages) Department of Health and Social Care (2016) NHS outcomes framework 2016 to 2017 NHS Digital (2022) NHS Outcomes Framework England, March 2022 Annual Publication

Questions for consultation

Have all relevant comparators for ruxolitinib been included in the scope? Which treatments are considered to be established clinical practice in the NHS for segmental and non-segmental vitiligo?

Are the outcomes listed appropriate?

Are there any subgroups of people in whom ruxolitinib is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Where do you consider ruxolitinib will fit into the existing care pathway for non-segmental vitiligo?

Do you consider ruxolitinib to be innovative in its potential to make a significant and substantial impact on health-related benefits and how it might improve the way that current need is met (is this a 'step-change' in the management of the condition)?

Do you consider that the use of ruxolitinib can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which ruxolitinib will be licensed;
- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;
- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. We welcome comments on the appropriateness of appraising this topic

through this process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. British Association of Dermatologists 2021 Vitiligo. Accessed March 2022.

2. National Health Service 2019 Vitiligo – Overview. Accessed March 2022.

3. National Institute for Health and Care Excellence 2020 Clinical Knowledge Summaries – Vitiligo. Accessed March 2022.

4. Office of National Statistics 2021 Population estimates for the UK, England and Wales, Scotland and Northern Ireland: mid-2020. Accessed March 2022.