

# NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

## Health Technology Evaluation

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

#### Final scope

#### 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.<sup>1,2</sup> 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).<sup>3</sup>

Vitiligo may cause psychological distress and is associated with other auto-immune conditions such as Grave's disease.

Vitiligo affects males and females, and all ethnicities equally. It may be more noticeable in people with darker skin tones and its noticeability may also vary by the proportion and location of the skin affected. 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.<sup>1,2</sup> Based on 2020 figures, it is estimated that approximately 570,000 people in England and Wales have non-segmental vitiligo.<sup>4</sup>

There are currently no licensed treatments for vitiligo. People with vitiligo are given advice on sun protection and vitamin D supplements and may use skin camouflage. Current off-label pharmacological treatments include corticosteroids and calcineurin inhibitors such as topical pimecrolimus or topical tacrolimus. Other treatments include phototherapy (as monotherapy or with oral corticosteroids), but access to phototherapy for vitiligo is reported to be limited.

### 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.

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| <b>Intervention</b>         | Ruxolitinib cream                                                                                                                                                                                                                                                                                                                                                                                                                                                                    |
| <b>Population</b>           | People aged 12 years and older with non-segmental vitiligo with facial involvement.                                                                                                                                                                                                                                                                                                                                                                                                  |
| <b>Comparators</b>          | Established clinical management without ruxolitinib                                                                                                                                                                                                                                                                                                                                                                                                                                  |
| <b>Outcomes</b>             | <p>The outcome measures to be considered include:</p> <ul style="list-style-type: none"> <li>• re-pigmentation</li> <li>• maintenance of response</li> <li>• cessation of spread or stabilisation of vitiligo</li> <li>• global assessment of vitiligo</li> <li>• cosmetic acceptability</li> <li>• adverse effects of treatment</li> <li>• health related quality of life.</li> </ul>                                                                                               |
| <b>Economic analysis</b>    | <p>The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year.</p> <p>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.</p> <p>Costs will be considered from an NHS and Personal Social Services perspective.</p> |
| <b>Other considerations</b> | <p>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.</p>                                                                                                                                                         |

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| <b>Related NICE recommendations</b> | None.                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                         |
| <b>Related National Policy</b>      | <p>The NHS Long Term Plan, 2019. <a href="#">NHS Long Term Plan</a></p> <p>Department of Health and Social Care, <a href="#">NHS Outcomes Framework 2016-2017</a>: Domains 1 to 5.</p> <p>NHS England (2013) <a href="#">2013/14 NHS Standard Contract For Specialised Dermatology Services (All Ages)</a></p> <p>Department of Health and Social Care (2016) <a href="#">NHS outcomes framework 2016 to 2017</a></p> <p>NHS Digital (2022) <a href="#">NHS Outcomes Framework England, March 2022 Annual Publication</a></p> |

### 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.