Single Technology Appraisal

Ruxolitinib for treating non-segmental vitiligo in people 12 years and over [ID3998]

Patient Organisation Submission

Thank you for agreeing to give us your organisation’s views on this technology and its possible use in the NHS.

You can provide a unique perspective on conditions and their treatment that is not typically available from other sources.

To help you give your views, please use this questionnaire with our guide for patient submissions.

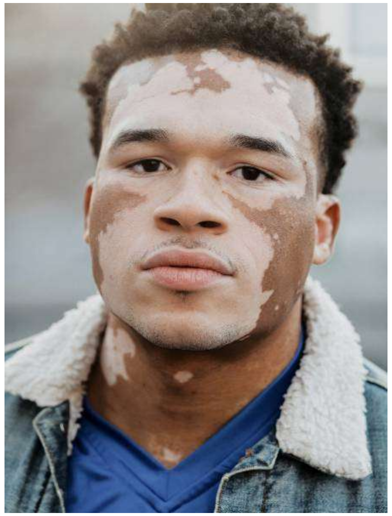
You do not have to answer every question – they are prompts to guide you. The text boxes will expand as you type. [Please note that declarations of interests relevant to this topic are compulsory].

Information on completing this submission

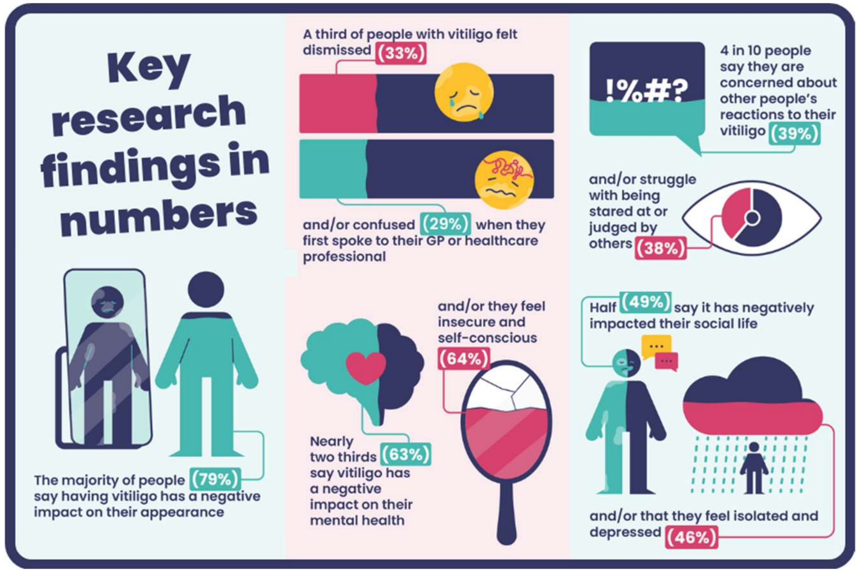
- Please do not embed documents (such as a PDF) in a submission because this may lead to the information being mislaid or make the submission unreadable
- We are committed to meeting the requirements of copyright legislation. If you intend to include **journal articles** in your submission you must have copyright clearance for these articles. We can accept journal articles in NICE Docs.
- Your response should not be longer than 10 pages.

About you

1.Your name	**Abigail Hurrell**
2. Name of organisation	The Vitiligo Society
3. Job title or position	Charity Director
4a. Brief description of the organisation (including who funds it). How many members does it have?	The Vitiligo Society was **established in 1985**, and we are the only **national charity supporting people living with vitiligo in the UK**. Our Mission is to beat vitiligo by eradicating the psychological, social and physical effects that vitiligo has on people's lives, and by finding effective treatments and a cure. To achieve our mission, we focus on five priority areas of work: education, research, support, advocating for patients and campaigning for better awareness of the condition.  The charity has over **800 donating members** and **3000 newsletter subscribers**. Our closed support group has almost **7000 members** and we have a wider online following of **28,000 individuals** with **10,000 unique visitors** to our website **every month**. The charity is primarily **funded by donations** made by members and the wider public. We also receive ad hoc restricted grants from lottery funders, trusts, and pharmaceutical companies to undertake specific project work.
4b. Has the organisation received any funding from the company bringing the treatment to NICE for evaluation or any of the comparator treatment companies in the last 12 months? [Relevant companies are	In the interest of transparency, the charity has received the following funding from pharmaceutical companies in the **past 18 months**.  November 2023: £25,000 from Incyte Uk and £23,700 from Pfizer Uk to run a public awareness campaign. Funding has been used in line with the ABPI Code of Practice to engage external organisations to undertake campaign work on our behalf, under our guidance and instruction. Funding has been declared in line with transparency rules set by the ABPI. Neither

listed in the appraisal stakeholder list.] **If so, please state the name of the company, amount, and purpose of funding.**	pharmaceutical company has had editorial oversight or input into the content produced or the way it was shared. We have shared this as, although it was paid more than 12 months ago, the activity funded is still being delivered.
4c. Do you have any direct or indirect links with, or funding from, the tobacco industry?	No
5. How did you gather information about the experiences of patients and carers to include in your submission?	The Vitiligo Society issued a callout to the vitiligo community inviting individuals who specifically wanted to share their experiences and contribute to this submission. **Over 160 individuals** stepped forward and were provided with questions about their experiences and views, which has helped to guide our response. Some **anonymised quotations** from these people have been included in our submission.  A broader range of experiences and views from across the community have been included through various surveys in recent years: ● The Vitiligo Society instructed Social Change UK to conduct research on our behalf to help us gain more understanding of the physical, psychological and social implications of vitiligo in the UK. **The research involved 828 individuals**, 712 were people living with vitiligo and 116 were a relative or carer of a vitiligo patient. This research was published by The Vitiligo Society in November 2023 ● The Vitiligo Society **surveyed 304 individuals** between October 2010 and May 2011 to understand the effectiveness of treatments currently available to patients. ● We also drew on external, published research which is referenced in the appendix at the end of the submission A final draft of this submission was circulated to **The Vitiligo Society's Board of Trustees** to review and provide additional comments. Our Board of Trustees is entirely formed of people who either have **personal lived experience of vitiligo**, or close family members living with the disease.

Living with the condition

6. What is it like to live with the condition? What do carers experience when caring for someone with the condition?	*“**Unpredictable and frightening**. I experience fear. How will my child’s vitiligo progress, how will she be accepted by peers and strangers…will she become **targeted, bullied, become isolated and suicidal** as others have experienced that we know personally… **It is heartbreaking to have a child with vitiligo** as you can’t fight their battles for them, you can’t stop other people’s reactions to differences, and even when I try so hard to build up her resilience and body image, is it enough?”*  *“**It has absolutely destroyed me.** I struggle every single day, to the point **I no longer want to be here**. I cry nearly every day and wish it would just go away.”* Vitiligo is a **chronic autoimmune disease** and is widely considered to have one of the **most psychologically devastating effects on the skin** as it challenges societal norms related to skin pigmentation across various cultures. The impact of vitiligo is: • **Physical** • **Psychological** • **Social** These themes are explored in detail below: **Physical Impact** One of the most recognisable symptoms of vitiligo is the **loss of pigmentation to the skin** – which can appear anywhere but often begins in areas of friction or where skin is damaged – such as knees and elbows, eyelids, around the mouth and lips, and around the genitals. In addition to the visible depigmentation of the skin, **some people living with vitiligo also experience some physical discomfort (32%)**[3]. Some patients report itchiness, dryness and painfulness, whilst others mention a heightened sensitivity in the affected areas. In some cases, these areas are more sensitive to sunlight, which results in burns, with some reporting that it can make the patches spread faster. Other physical health issues can be linked to vitiligo, even if these are not direct effects of the condition. For example, there is **a risk and potential for comorbidities, such as autoimmune diseases, diabetes, other skin conditions,**

and joint and connective tissue disorders like arthritis. Regular check-ups and talking to healthcare providers about these risks is a recommended part of treatment guidance but testing for these conditions is still not common practice for those diagnosed, and additional checkups are difficult to obtain – adding to the anxiety and frustration of the disease.

"[Vitiligo is] horrendous. ***Your self-confidence is shattered.*** *You never know where the next patch will come and how significant it may be. It's ruined my self-confidence…I have vitiligo on my eyes, breasts, bottom, hands and genital area.* ***I dread going on holiday*** *and it's become a* ***daily struggle****."*

"It's hard to explain to children, both mine have it*. It affects their* ***mental health greatly but also physical health.*** *Spots need extra care during summer months as the* ***skin can become red and irritable."***

Psychological impact

People living with vitiligo often experience a range of psychological effects. For example,**79% reported it having a negative impact on their appearance**, and **63% on their mental health**. This also included a **significant reduction in their quality of life**, feelings of **isolation, sadness, frustration, stress, worry, difficulty in relationships, depression, anxiety, body image issues (46%), and lack of confidence. 41%** of those surveyed **struggled with their self-esteem** and **64% reported feeling insecure** or self-conscious about how their skin looks [3]. One of the most prominent psychological effects is low self-esteem, which can lead to negative thoughts about their appearance and patches, notably their characteristics, changes and spread, all which impact self-confidence. The **fear of being judged** or rejected due to appearance, and changes in normal routine (e.g., having to avoid sunlight), can also contribute to mental health conditions like depression and anxiety. **Social stigma** is a significant factor that can lead to social isolation due to avoiding social interactions and fearing negative reactions and comments from other people.

"In summer my 16-year-old son's vitiligo is VERY visible, and he is very conscious of it… ***I've cried myself to sleep some nights when I see the effect that vitiligo has on my son."***

"Since my vitiligo has worsened, I have ***developed social anxiety.*** *I used to feel good about myself but now my self-esteem is very low,* ***I have stopped dating completely."***

*"My life has been totally turned upside down… **not a day has passed where I have not woken up thinking about vitiligo** and going to sleep without thinking about vitiligo. I literally **dread each day,** the future and the chance that one of **my children inherit this awful condition.**"*

Social impact

Vitiligo can have a significant **impact on an individual's social life (49%),** often leading to **isolation** and **difficulties with social interactions**. Most participants mentioned negative reactions and misconceptions about the condition, such as comments like **"did you put bleach on your hands?"** or **"is it contagious if I touch you?"**. Some Individuals may experience the loss of friends and missed job opportunities due to fears of being judged and withdrawing from social gatherings (**29% reported noticing a negative impact in relationships with family and friends**). Another important social impact to mention relates to personal and intimate experiences. **37% noticed an impact on their intimate and romantic relationships**, such as dating and sexual encounters, which can also bring concerns regarding possible judgements and important conversations with partners[3].

*"**My daughter** is young and partly oblivious to other people; however, I notice **people looking and staring** and commenting on it. It has a huge **potential to damage her confidence and** subsequently her **mental health** whilst she develops."*

*"At times **I hate myself…** [I] can't wear shorts or dresses, … **I dread the summer,** especially when people are wearing summer clothing, and I always look the odd one out… It has a **dreadful effect on my everyday life.**"*

*"It impacts my mental health as I see **other people say comments about my skin**…I have had people be hesitant & **reluctant to shake my hand.**"*

*"I even think about it **when having my photo taken,** like **not putting my arms around my children** because it will clearly show my white patches on the pictures."*

Current treatment of the condition in the NHS

7. What do patients or carers think of current treatments and care available on the NHS?	*"I've tried them, and **they didn't work.** There are **no more options** other than to 'live with it' which is something I can't bear the thought of."* There are **three key themes** that emerge from feedback from patients regarding available NHS treatment and care: - **Treatments are either not offered or are difficult to access** - **Light therapy is too disruptive and can make vitiligo worse** - **Available treatments are not effective** These themes are explored in detail below:  **Treatments are either not offered or are difficult to access** The treatment journey for individuals living with vitiligo is a diverse and constantly developing process. It often starts with the diagnosis, during which healthcare professionals may provide and introduce information about potential treatments. However, research has shown that this process may not always offer the level of reassurance and guidance people need. This is why people described their **treatment journey as a 'trial-and-error' process**, while exploring different treatment options. Almost all the participants (75%) expressed a shared desire for a cure for vitiligo, and 15% expressed being resigned from finding a cure[3]. Data from our research into experience of treatments shows that of the 290 participants **137 (45%) had not undertaken any treatment**. Patients informed us that this was because either they are **told no treatments are available**, or they are **warned that the available treatments are not effective**[6]. **48%** of patients said they still had **concerns about their vitiligo spreading** or progressing in the future[3]. *"Our care has been **totally inadequate.** Six years ago, when she [our daughter] was diagnosed we had one appointment with the GP who prescribed steroid drugs and said there was **nothing else he could do.** We have been **waiting for a dermatology assessment for over 18 months."*** **"There isn't anything that seems to work.** I have been given creams, but they are **awful to use** and **don't seem to do anything**. They also can't be applied to my face, which is the main place that I am concerned about."

Light therapy is too disruptive and can make vitiligo worse

Treatments that combine creams with light therapy are generally reported by patients as being the most effective option in terms of repigmentation, however the **results are nearly always short-term**. The **disruption** of having to visit a clinical setting for light therapy means that most patients who are able to access the treatment feel that the **limited results did not justify the disruption to their lives**. These treatments also **present barriers to access** for many patients who are already at a disadvantage due to geographical location, lack of mobility, work, education, childcare commitments, or financial constraints.

*“**Current availability of treatments is lacking.** I haven’t had any treatment in years now, just because the hospitals haven’t been able to see me, and the waits are so long… you can only do the lamps at the hospital, that combined with creams and ointments are the only option”*

*“I have had **several rounds of NB UVB treatment** and even though the treatment is **gruelling** it does work, but **after a while the patches just come back.**”*

*“Light therapy is **difficult to get** and also if you have a full-time job **it is not sustainable.**”*

*“**Light treatment escalated my white patches**. [I] wish I’d never agreed to it but I was **desperate** and still am.”*

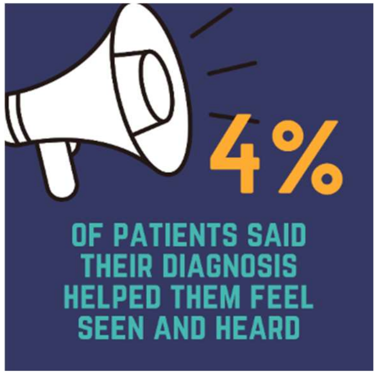
Available treatments are not effective

Insight from our online support groups have shown that most people who reach out to us feel **there are not any effective NHS treatments available for vitiligo.** Those that do experience repigmentation via the available treatment methods, report that this is **rarely permanent or wholly effective**. Within our community there is a **sense of desperation** for more effective treatments to become available. It’s not uncommon for people to be so desperate that **they purchase unapproved ‘treatments’** from the internet, and our support groups are often **targeted by individuals trying to sell ‘miracle cures’** to patients. This presents **a real danger** where patients are trying products that are **not regulated and potentially harmful**.

Only **71 individuals (24%)** surveyed **reported the occurrence of repigmentation that persisted after treatment**. A key figure is that of those surveyed **just 3 individuals (1%) marked that they considered their treatment to have been ‘very successful’** [6].

*“Last time I saw my dermatologist **he said I need to live with it.** It makes me feel like no one cares what I'm going through.”*

	“They did their best but unfortunately ***nothing works. I tried everything.”*** ***“I do not feel the NHS are trying to actively look for new solutions…*** *after Protopic stops working* ***you are discharged and left to your own dark thoughts feeling like your life is over,”***
8. Is there an unmet need for patients with this condition?	***“They can’t explain why we’ve got it…*** *There is no cure, I do understand that. But* ***they don’t give you any hope at all.*** *They’re like ‘we can try this cream, but* ***it’s probably not going to work.”*** *“The* ***wait times for diagnosis are extremely long*** *and which does not help with trying to manage spread earlier.* ***Mental health support is difficult to receive.”*** **96%** **OF THOSE SURVEYED FELT THERE WERE UNMET NEEDS FOR PATIENTS WITH VITILIGO** Our research highlighted **5 areas of unmet need** for patients with vitiligo. Currently most patients must navigate this disease without: - **A clear, informative diagnosis** - **A timely referral to specialist services** - **A clear treatment pathway** - **Access to effective, licenced treatments** - **Testing for other associated autoimmune diseases** These themes are explored in detail below: **A clear, informative diagnosis** While most people first learn about vitiligo through a GP or healthcare professional, their overall **experience with the diagnosis is often not reassuring or positive**. Patients describe feeling **uninformed, unsupported, unheard, distrustful, and/or pessimistic** when receiving the diagnosis. Similarly, other feelings such as **neglect, confusion, dismissal, and fear** were experienced to a lesser extent. It’s very common for individuals to turn to a GP as their initial source of help and information, however patients report that the information received during this stage is **often perceived as not useful**, and the possible **psychological impact of this disease is not mentioned** or addressed by medical professionals. One prevalent issue is that **people often feel dismissed** and are being told that it is only a ‘cosmetic condition’, which can be invalidating and frustrating. Additionally, many vitiligo patients are **not referred to a dermatologist or a specialist** that could provide more information in relation to all aspects of the condition, leaving individuals feel dismissed & hopeless. Often being diagnosed only helps vitiligo patients understand what vitiligo is because they do a lot of research about it after being



diagnosed. Patients report that GP appointments in relation to vitiligo often lack certainty, reassurance and information about potential risks around related health conditions. In fact, **only 4% of surveyed participants said that their diagnosis helped them to feel seen and heard** by healthcare professionals[3].

*"This condition causes **mental health issues…recognition is needed** to stop this condition and mental health in its tracks."*

*"I've **had to explain to doctors what vitiligo is** and then they say, 'oh that's fine you know **it doesn't affect anything'.**"*

A clear treatment pathway

Even after diagnosis, many people living with vitiligo do not feel that they possess an understanding of the condition. The **difficulty of finding information** related to vitiligo is one of the factors that contributes to this issue, and even those who are able to find information easily report **uncertainty surrounding the information's accuracy**. Many **patients are not aware that there are treatment options**. For those that can find information the **process of treatment can vary significantly** depending on the type of vitiligo, location and colour of the patches, the extent of their vitiligo, as well as individual preferences, goals and needs. For instance, some individuals opt for treatments focused on repigmentation, aiming to restore the colour of the depigmented areas, and may explore options like topical corticosteroids, phototherapy, or advanced therapies such as an excimer laser or microskin.

Patients that are not properly informed by their healthcare professional can find **navigating treatment pathways to be stressful and difficult**. Treatment outcomes may take time to become evident and involve a **lengthy process of finding the right treatment**, trusting it, and using it constantly. This is why many people will choose to stop treating their vitiligo - **they give up hope.**

"An effective treatment and even a dermatologist appointment is not available for this condition"

*"I have **never been offered any treatment."***

Effective, licensed and accessible treatments

A key unmet need in the community is access to licensed treatments. With the US and Europe approving Ruxolitinib for use and making it available to healthcare patients, **UK patients are feeling frustrated and abandoned**. Instead of one licensed treatment, patients are **left to navigate a selection of unlicenced options** which can be **emotionally challenging, time consuming, expensive, and often difficult to access**. It is generally accepted amongst the vitiligo patient community that NHS treatments are not effective, and that NHS care is not helpful. As a result, many patients look to other solutions such as private practitioners, the internet, psychological support and skin camouflage services.

*“**How can there be treatment available in other countries which have proven positive effects and yet we cannot have it in the UK?** When I learnt about Ruxolitinib I was so unbelievably excited. And **when I saw it hadn’t been approved, I cried and cried and cried…**I don’t think that people with normal skin can ever understand how it feels.”*

*“Treatment is available, but **we are deprived of something that can give us a meaningful life again….** I believe this treatment can be made available through the NHS.”*

*“We need to be able to try Ruxolitinib. **It feels very unfair that a treatment exists, and that we are not able to access it.”***

A timely referral to specialist services

A direct consequence of the lack of effective, licenced treatment options is **an unmet need for timely referral to counselling services, and suitable camouflage services**. Patients are having to **wait months and often years** to see their dermatologist and then when an initial treatment plan proves ineffective, they must wait even longer to obtain the psychological support they require. Patients also report to us that they are having to wait months to access camouflage services and are often faced with geographical and financial barriers when trying to access appointments. Patients with darker skin specifically report finding **camouflage services ineffective** as the options for darker skin tones are limited and ‘unnatural looking’.

*“[there is a] **lack of a therapy** that can really address vitiligo and lack of understanding that vitiligo has a serious impact on the mental well-being of people who experience it. **You won't be able to understand it unless you have it”***

*“[There is] **little consideration for the emotional and mental impact** of living with this…I **have had to research everything myself.** Nothing I have come across or used has been recommended/shared or advised to me by the NHS.”*

	 **Testing for other associated autoimmune conditions** Vitiligo is often viewed as a 'skin condition', rather than being recognised as a chronic autoimmune disease. For this reason, patients are rarely tested for associated autoimmune diseases, despite treatment guidelines stating that this should form a part of the diagnosis process. This means that the discovery that there is **a risk and potential for comorbidities, such as other autoimmune diseases** is often made via online groups and chat rooms – causing panic, fear and further mistrust in NHS care. *"The NHS prescribes a cream and that's it…**they do not even test for possible other symptoms.** Autoimmune diseases can affect people in many ways."*

Advantages of the technology

9. What do patients or carers think are the advantages of the technology?	*"**Feeling completely helpless for a child is the worst thing**; it feels like hell…**Having these new medications coming** through and being developed **gives hope** but only to be crushed when it is then refused."*  Feedback from the patient community highlighted **5 themes** where **patients felt this technology had specific advantages**, especially when compared to other treatment options: • **Better real-world results** • **Giving hope to patients** • **A simple, accessible, licenced solution** • **Alleviate the financial burden on individuals desperate of a solution** These themes are explored in detail below: **Better real-world results** There is much to be learnt from real-world experiences of different treatments. We have relationships with patient support groups in the US and Europe where Ruxolitinib is currently available. We are told that members of these support groups have been **experiencing positive real-world results** in terms of facial repigmentation whilst using this treatment, especially by

comparison to other treatments currently available. Our **UK patient community is reading about that success** and is left **feeling increasing frustrated** knowing that there is something that is working for patients around the globe, but they are not able to access it.

*“**The results are promising for this new drug.** I think patients in the UK should be given the chance of using it like others in Europe.”*

*“**Ruxolitinib could address a range of issues such as anxiety, low self-esteem and confidence** and would also help to support people financially **by removing dependency from purchasing make-up** and **reducing the stress and energy** that goes into applying make-up on a daily basis.”*

Giving hope to patients

For many people in our community, it is the loss of pigmentation on their face that is the key cause of anxiety - especially when leaving the house or meeting people for the first time. This treatment **represents hope for many people** in our community. Whilst it may not be a cure and we recognise it won’t alleviate all the social, psychological and physical impacts of vitiligo, it does have the potential to lessen these impacts and enable patients to be seen, heard, and to live with more confidence.

*“It would be **a life saver for a lot of new teenage sufferers** who have just found out they have it **and for long term sufferers** who have lived with it for a while.”*

*“**It would give us hope** and belief that we have some control over this debilitating autoimmune condition.”*

“Please give us… hope. People will give up on life, trust me they will. Please, please give us this cream.”

A simple, accessible, licenced solution

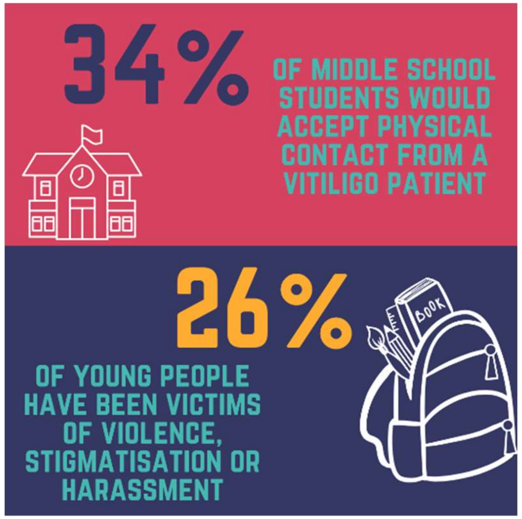
We know that the **treatment pathway is complex and confusing** for vitiligo patients and healthcare professionals to navigate, we believe that the introduction of a licenced treatment will help provide patients with both the hope of a new treatment but also **reinstate some confidence within the healthcare system** as there will be a treatment specifically licensed for their condition. In addition, it would **help tackle some of the inequalities created by the current comparator** to this technology (light therapy) which is not easily accessible for many groups of individuals.

	"It's effective. It's evidenced based treatment. It offers long term results. It is easy to take this treatment" ***"A genuine and accessible treatment option would finally be available." "A simple, effective home treatment"*** **Alleviate the financial burden on individuals desperate of a solution** The reality is that vitiligo patients are so **desperate for access to new treatments** that individuals are getting into **serious financial difficulties** in order to purchase this new treatment privately or turning to the internet a trusting to so-called 'miracle cures' that are advertised. This can be extremely harmful but also indicates the level of desperation that patients are feeling.  *"It would be **life changing** to my current way of life, not only for me but also for my partner. **I have to pay for my own private medication** to manage vitiligo, which is a **strain on our finances and** also our **relationship."*** *"There have been **many positive and encouraging stories from other countries** in this treatment and it being available via the NHS would **allow not just a select few to benefit** from this **very costly private treatment."***

Disadvantages of the technology

10. What do patients or carers think are the disadvantages of the technology?	As a patient support organisation, **we believe in choice**. Currently patients feel they have no choice, so regardless of the disadvantages listed below we feel it is important that this treatment be made **available to all those who might benefit** from it. The **3 key disadvantages** of this treatment that we have identified are: ● **Restriction to facial involvement:** We understand that the indication for this treatment is **restricted to non-segmental vitiligo**, and where **vitiligo is present on the face**. We are concerned by this restriction as we know that very **few patients are properly checked using a Wood Lamp to confirm the various locations where vitiligo is present**. This means that **small vitiligo legions on face may be missed**, and as a result **this treatment may not be considered** until the vitiligo on the face has become more visible. This delay in treatment could **negatively impact the effectiveness** of the treatment and result in **further psychological damage** to the patient.  ● **Restriction by age and type of vitiligo:** The **restriction to ages 12 and over** and **restriction for use in only non-segmental vitiligo** has caused concern amongst patients. Young people would especially benefit from access to the treatment given the increased burden the condition has on these patients. It should be noted that patients are equally impacted regardless of the type of vitiligo – so if any effectiveness has been shown for use with segmental vitiligo, then we believe those patients should also have the opportunity to access this treatment if they wish to try it. ● **Length of time to access a second line treatment:** We understand that Ruxolitinib is being considered as a second-line treatment option, meaning that it would only be available following a referral to a specialist clinics or hospital. Our concern is the **difficulty patients experience in securing a referral to a specialist**, and the **subsequent waiting time** many patients face. This means that in many cases patients will have been diagnosed for well over a year prior to being able to access this treatment, by which time their vitiligo may have progressed significantly causing **further emotional distress**.

Patient population

11. Are there any groups of patients who might benefit more or less from the technology than others? If so, please describe them and explain why.	*“Having* ***darker skin makes it more noticeable*** *and knowing that there is possible treatment for it but the NHS will not provide it is not only disappointing, but I feel it is* ***discriminatory to people of colour.”*** ***“I worry a huge amount about my son's mental health****. He is* ***only 8****, but I can already* ***see the impact having a visible difference has on his confidence*** *and how he reacts when* ***people ask about his skin*** *and why it looks different****.”***  Any patient, regardless of their demographics or extent of the vitiligo has the potential to suffer the extreme, negative socio and psychological effects associated with the condition, and would therefore benefit considerably from the addition of a new treatment option. There is a real lack of formal, published research relating to this area, however research we have conducted and our experience of supporting patients has shown that **those most severely affected by vitiligo are statistically more likely to have at least one the following four characteristics**: ● **Have medium to dark skin colour** ● **Belong to culturally diverse communities** ● **Young people** ● **People from low socio-economic groups** These themes are explored in detail below: **Those who have medium to dark skin colour** Data from our research and discussions from our support groups have identified that those who have medium to dark skin tone are **more likely to be negatively affected by their vitiligo** and have **a lower quality of life** as a result of the condition[3]. The **reasons behind this are complex**, but from our experience key factors include: ○ Skin depigmentation is **more noticeable** in darker skin tones. The appearance of vitiligo can have significant consequences on the quality of life for individuals with darker skin, attributed to the noticeable contrast[2]. ○ Camouflage service users with darker skin are more likely to find **the colour selection inadequate to conceal their patches**. ○ Individuals with darker skin also have a **higher likelihood of living in areas of higher indexes of multiple deprivation**; have **longer waits to access health care** and so treatments are less likely to be effective due to the time it takes to receive them. Research shows that early intervention results in greater treatment success with vitiligo patients (especially younger ones).

- This demographic is more likely to be **working in public facing jobs**, **facing social reactions and stigma on a daily basis**, which in turn affects their career and ability to excel in work environments.
- This demographic of individuals is **less likely to be able to easily travel to appointments**; less likely to **participate in clinical trials**; and are **more likely to have work patterns which prevent them from accessing treatments** based in clinical settings such as phototherapy.

"I believe by not providing it to people of colour is ***discriminatory and*** *potentially* ***falls foul of equality laws."***

Those who belong to culturally diverse communities (CDCs)

Again, understanding how CDCs are more severely affected by vitiligo **is not straight forward**. Key factors we are aware include:

- Individuals from CDCs are **more likely to have darker skin tones** (see above)
- Our experience is that CDCs are often **more tight-knit and closed to external influences** – this means they are more distrustful of national services such as healthcare, support services, and medical and scientific researchers. They are **less likely to engage in support groups** or to participate in clinical trials. **Family pressure and heightened social stigma** often results in individuals feeling more compelled to hide their vitiligo.
- We are aware of individuals from CDCs being **forced into arranged marriages** and **sent overseas** as they are **required to be matched with another individual with vitiligo**, again due to heightened social stigma surrounding the condition.

The psychosocial impact of vitiligo is underscored by the intricate interplay between visible differences and social stigma, which significantly affects an individual's self-esteem. **Thompson et al. (2010),** conducted a study of British South Asian women, delving into the experiences of living with vitiligo. The study emphasized that societal perceptions, cultural norms, and beauty ideals are pivotal in shaping the emotional landscape of individuals with vitiligo. The study found that **within specific cultural contexts**, the influence of unique cultural norms and societal beauty ideals becomes more pronounced, **intensifying the pressure on individuals to adhere to established appearance standards**. This increased emphasis on **conformity to cultural expectations** can further compound the marginalization experienced by those affected by vitiligo. Therefore, there is a growing urgency to establish support systems that are culturally sensitive and tailored to address these unique challenges effectively[1]**.**

Another example of unique cultural challenges can be understood when exploring our relationship with the Indian vitiligo support group, the Shweta Association. Based in India, a key service the association provides is their Marriage Bureau:

*"**Marriage Bureau** & Matrimonial Meets is **one of our most sought-after activities.** Up to the year 2015, more than 1500 boys and girls have registered and 950 marriages have been arranged successfully. We register **candidates having vitiligo** or candidates whose parents or other family members have vitiligo. However, we also welcome people who do not have Vitiligo but out of a **sense of social responsibility are willing to marry** our members."*

The Shweta Associations work is not limited to supporting the marriages of those in India, but to **match those of Indian heritage who have vitiligo from around the globe**. Such is the stigma of the condition in this culture, that **families in the UK will send young people abroad to secure a marriage** match with another person diagnosed with the condition. This is often out of fear that without such action their child will never be able to get married or lead a normal life. For many patients in the UK with Indian heritage, **a diagnosis can mean the end of their life in this country**.

*"Communities like mine are just very closed and **we keep matters to ourselves.** We won't go looking for support from outside organisations."*

Young People

Vitiligo has **especially strong effects on the mental health of 12-25 year olds**. Again, the reasons for this are not straight forward, but we have listed some key factors below:

- Research[4] shows that **middle school students are the least informed and tolerant of people living with vitiligo**, yet puberty is a pivotal age in the acceptance of the disease by patients and those around them. Vitiligo remains unknown among young people (since only 30% of young people "know about vitiligo", while 51% said "they have heard of it"). This lack of awareness causes a stigmatisation of vitiligo, which appears particularly strong in middle school: **only 34% of middle school students would accept physical contact with a person suffering from the disease**, and **only 38% would agree to interact with them**.
- Furthermore, while **26% of individuals aged 12 to 25** who have suffered from a visible skin problem say they have been **victims of violence, stigmatisation or**

harassment, and 20% of young people who know about vitiligo have witnessed a situation of stigmatisation.[4]

  - This stage of development often involves heightened insecurity. A staggering **76% of young people who participated in the study believe that the way society views people with vitiligo harms their mental health.**[4]
  - In addition, young people often struggle to adjust to the current treatment available with many reporting that **current treatments irritate their skin** or prove **too disruptive to their education** to be viable options for them.

In addition to improved quality of life, this technology stands to benefit young people further as **research shows that the earlier the treatment intervention, the more effective and long lasting repigmentation is likely to be**[5]. Therefore, young people may have both the greatest burden to alleviate and stand the greatest chance of experiencing positive results, given that a significant portion of vitiligo patients are diagnosed at an early age.

People from Low Socio-Economic Groups (LSEGs)

From our support groups we have seen a trend that **those in more public facing jobs** such as hospitality, retail, teaching and care etc, **will often experience a great social impact from their vitiligo**. These roles are often linked to lower salaries and lower socioeconomic status and **face greater barriers in accessing treatment**, especially treatments that take place within a clinical setting. In addition, they are **less likely to be able to access private support** such as counselling, pay for private treatment, or afford effective camouflage products. Due to the difficulty in accessing healthcare, more and more vitiligo patients who can afford it, choose to access private support services and products. This means that **individuals from deprived areas are more likely to benefit from new treatments**, and they are most likely to be without access to psychological support and effective camouflage products.

*"There are always equality issues in the world, but I feel it's so unfair that **this treatment isn't available unless you are rich.**"*

Equality

12. Are there any potential equality issues that should be taken into account when considering this condition and the technology?	In the previous section we highlighted that certain demographics of the vitiligo patient population are more likely to benefit from the treatment. We identified that those most severely affected by vitiligo are more likely to have the following characteristics: • **Have medium to dark skin colour** • **Belong to culturally diverse communities** • **Young people** • **People from low socio-economic groups** We also acknowledged that anyone can be severely physically, psychologically, socially affected by vitiligo. The above groups are statistically more likely to be adversely affected when compared to the general population. **We believe the that above section (question 11.) should be taken into consideration as these characteristics present significant equality issues when considering the technology.**

Other issues

13. Are there any other issues that you would like the committee to consider?	There are **four additional key issues** that we would like to highlight to the committed: • **Do trial populations reflect real world patients?** • **Current studies related to the mutation burden of narrowband UVB** • **The need to fully explore commercial negotiations fully.** • **Voices from our patient community** These issues are explored in detail below: **80%** **OF PATIENTS SURVEYED WANTED ASSURANCES THAT NICE AND NHS ENGLAND WERE CONSIDERING ALL COMMERCIAL OPTIONS FOR THE APPROVAL OF RUXOLITINIB AS PART OF THIS PROCESS** **Do trial populations and data reflect real world patients?** We would like NICE to consider what we have presented as the real experience of current health care and the potential life changing benefits this treatment could have. We appreciate that NICE bases much of its decision on cost effectiveness analysis and scientific quality of life scales, but we ask that they **consider the real voices and experiences** that we have shared and **give appropriate weight to what we, as the community of patients, are saying.** We question from our knowledge of those communities who are most affected by vitiligo (and who are most likely to benefit from the treatment) was this population (CDCs, LSEGs, Young People and those with darker skin) adequately represented in clinical trial data? We know these groups would face the greatest barriers to

participating in such research. We also know **these groups have not been represented adequately in this process** thus far, so we urge NICE to read the quotes and take note of the data included here.

Current studies related to the mutation burden of narrowband UVB

We note that the NICE commented in their draft guidance that **"it is uncertain how well ruxolitinib works compared with phototherapy because the company provided no evidence to support this comparison".** It may be worth noting that the **University of Southampton has recently conducted a study investigating the mutation burden of narrowband UVB (NB-UVB) in patients** with psoriasis and have found that, when the NB-UVB mutation burden is considered in the context of natural sun exposure**, NB-UVB looks more carcinogenic than previously estimated** (the manuscript is currently under review). Admittedly, this study has not tested for mutations in vitiliginous skin, and it is the risk of skin cancer in vitiligo is still unclear, but despite the JAMA Dermatol 2020 article by Bae et al not seeing a higher risk of skin cancer, it did report that **"the risk of actinic keratosis increased significantly for those who had undergone 200 or more NBUVB phototherapy sessions".** We understand from our Medical and Scientific Advisors that actinic keratoses have more mutations than the majority of internal cancers and melanoma, and we know from recent genetic work that some actinic keratoses can develop into squamous cell cancers, so researchers have suggested that **it may be just a matter of time before skin cancers are seen in vitiliginous patients who receive sufficient amounts of phototherapy**.

The need to fully explore commercial negotiations fully.

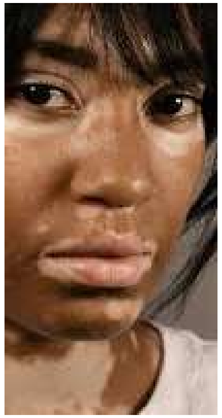
We are calling on NICE, NHSE, the Government and drug manufacturers to come together to **explore all options to enable timely and equitable patient access** to this new treatment. We remain concerned that without further commercial negotiation and flexibility we again risk a scenario where UK patients will not be able to access the first vitiligo treatment breakthrough, when it has already been deemed cost-effective in countries across Europe.

It is **not sufficient that the Appraisal Committee take a judgement on cost effectiveness without fully exploring all funding avenues and options** as part of this ongoing processes.

We call on the Appraisal Committee to use this opportunity to work with the company to find a solution to make this treatment a via option for NHS use. Our community is not resilient enough to withstand another negative outcome from this NICE process.

The voices from our patient community:

We want to give the final words of this response over to some of the 160 vitiligo patients who participated in our survey:



"I've been waiting for a treatment opportunity like this ***all my life*** *and* ***it's devastating*** *that it hasn't been made available."*

"If your own child had vitiligo, would you even consider whether to offer this treatment or not? *Probably not. You must* ***put the ethical and moral reasons*** *for approving this treatment* ***above all else."***

"This cream is the only hope I have. *I know other treatments are under trials, but this will take years. By then it will be too late for some people.* ***This glimmer of hope keeps us going and you are taking it away from us."***

"This condition ruined my life. I don't understand why anyone would prevent people accessing treatment that could change their lives."

"This condition is ***depressing, embarrassing and ridiculously difficult to hide****. Every few days* ***I scrub my skin raw*** *so I can put on fresh fake tan to cover it up. Even then****, I have to put on 2 layers of fake tan*** *to match up my normal skin… It's exhausting. My children often ask me what the first thing I'd buy if I won the lottery, and I always say I'd get my skin sorted out. If I had the money, I would buy it privately. If I didn't have to work or take care of my children, I would put myself forward for clinical trials - however they're too far away… I write this with tears streaming down my face because it's difficult to admit all of this. I just hope that ..that the decision to allow this treatment on the NHS is overturned so that me and all the others suffering from this, can be treated and be normal again."*

"Do not underestimate the impact vitiligo has on an already crippled mental health service. *I work with children and young people with mental health needs as a therapist and am fully aware of how hard it is to access any support in both CAMHS and adult mental health services.* ***The cost of treatment for mental health support must be considered*** *when considering the cost of benefits of patients accessing ruxolitinib as well as the massive impact it would have on every person's quality of life who has vitiligo and wants to use the cream."*

	 ***“Vitiligo sufferers have been virtually ignored in terms of available treatments, despite the fact that this condition can have serious ramifications in terms of mental health, this needs to change in order to improve our quality of life.”*** ***“You'll change people's lives.*** *I'm sure all members of the committee in one way or another remember what it was like to be a child and experience bullying in one form or another. Imagine that but not being able to do anything about your condition at all. Imagine* ***a fear so powerful you can't move*** *when you're asked to go swimming or out with your friends in the summer, to wear shorts or a t-shirt, the anxiety to go out, the looks, the questions, the remarks or sniggering behind your back.* ***You, the committee, have the power to completely eradicate this.*** *Please do this right thing and agree to provide this. At the very least for the young children who suffer from this.”* ***“People with vitiligo must have the right to an effective treatment on the NHS. We have waited too long.”***

Key messages

14. In up to 5 bullet points, please summarise the key messages of your submission.	• Vitiligo (and its associated symptoms) often has a significant impact on the lives of people with the disease and their close family. The **devastating social and psychological impacts** can also represent a risk of **decades, if not lifelong, mental health struggles**. In recent years vitiligo patients have campaigned for improved mental health referrals and camouflage options to be available on the NHS. **Demand for social and psychological support** from our charity **has increased tenfold**, suggesting severe limitations in the effectiveness, or access to, current treatments and wider healthcare & support services. • We are calling on NICE, NHSE, the Government and drug manufacturers to come together to **explore all options to enable timely and equitable patient access** to this new treatment. We remain concerned that without further commercial negotiation and flexibility we again risk a scenario where UK patients will not be able to access this first vitiligo treatment breakthrough, when it has already been deemed cost-effective in countries across Europe. It is **not sufficient that the Appraisal Committee take a judgement on cost effectiveness without fully exploring all funding avenues and options** as part of this ongoing processes. **Our community is not resilient enough to withstand another negative outcome from this NICE process.** • Ruxolitinib offers the only licenced treatment option for vitiligo patients, **representing hope** for those with the disease. • There exist **complex equality issues that should be taken into account** and explored fully when considering this condition and appraising this technology. • As a therapy that can be undertaken at home, **Ruxolitinib presents the best treatment option for patients when considering effectiveness and accessibility**. Many patients are currently at a considerable disadvantage in terms of ability to access preferred current treatments & care due to geographical location, lack of mobility, work, education, or childcare commitments, or financial constraints.

Thank you for your time.

Please log in to your NICE Docs account to upload your completed submission.

Your privacy

The information that you provide on this form will be used to contact you about the topic above.

Please select YES if you would like to receive information about other NICE topics - NO

For more information about how we process your personal data please see our privacy notice.

Appendix	1. Vitiligo linked to stigmatization in British South Asian women: a qualitative study of the experiences of living with vitiligo. Thompson AR, Clarke SA, Newell RJ, Gawkrodger DJ. Br J Dermatol. 2010;163:481–486. doi: 10.1111/j.1365-2133.2010.09828.x. [DOI] [PubMed] [Google Scholar]] 2. [Factors affecting responses on Dermatology Life Quality Index items among vitiligo sufferers. Kent G, al-Abadie M. Clin Exp Dermatol. 1996;21:330–333. [PubMed] [Google Scholar]] 3. Exploring perceptions and experiences with vitiligo. Social Change UK, prepared for The Vitiligo Society. November 2023. [Access HERE] 4. Vitiligo in young people: the results of an enlightening study. IFOP, prepared for the French Vitiligo Society and Baltasar Firm.2024. [Access summary webpage HERE] 5. Vitiligo: Current Therapies and Future Treatments Seneschal J, Boniface K. 2023 Dec 1;13 [PubMed] 6. Online Survey Treatment Results. Conducted by The Vitiligo 2011. Data is no longer available publicly but can be provided on request. 7. Ezzedine K, Eleftheriadou V, Jones H, et al. Psychosocial Effects of Vitiligo: A Systematic Literature Review. Am J Clin Dermatol.2021;22(6):757-774. [PubMed] 8. Thompson AR, Eleftheriadou V, Nesnas J. The mental health associations of vitiligo: UK population-based cohort study. BJPsych Open.2022 Oct 21;8(6):e190. [PubMed] 9. Eleftheriadou V, Delattre C, Khan I et al. Burden of Disease and Treatment Patterns Amongst Patients With Vitiligo: Findings From a National, Longitudinal Retrospective Study in the United Kingdom. B J Derm 2024 [PubMed]