Treatment Priorities and Unmet Need Among Adults and Adolescents With Nonsegmental Vitiligo in the United States

Iltefat Hamzavi,[1] Josh Coulter,[2] Arun Balaji,[3] Lia Franco,[4] Savanna Darnell,[2] Ernest Law,[2] Samantha Kurosky,[2] Roni Adiri,[5] Nada Elbuluk,[6] Brett Hauber[2]

[1]Hamzavi Dermatology, Canton, MI, USA; [2]Pfizer Inc., New York, NY, USA; [3]Pfizer Inc., Pearl River, NY, USA; [4]Pfizer Inc., Groton, CT, USA (current affiliation: Kellogg Business School, Northwestern University); [5]Pfizer Pharmaceutical Ltd, Herzliya Pituach, Israel; [6]Keck School of Medicine, University of Southern California, Los Angeles, CA, USA

BACKGROUND

- Nonsegmental vitiligo is an autoimmune disease resulting in skin depigmentation. The global prevalence of vitiligo is approximately 0.5% to 2% and can impact individuals of any sex, ethnicity, or age[1]
- As vitiligo has primarily visible symptoms, it can be stigmatizing and is associated with substantial psychosocial burden, including depression, low self-esteem, and anxiety. Patients also report impacts to health-related quality of life due to living with vitiligo[2]
- Traditional treatment options typically include topical and oral corticosteroids, topical calcineurin inhibitors, and phototherapy.[3,4] In 2022, ruxolitinib, a topical Janus kinase (JAK) 1/2 inhibitor, was approved in the US to treat up to 10% of the body surface area in patients with nonsegmental vitiligo. Several oral kinase inhibitors, ritlecitinib, povorcitinib, and upadacitinib, are currently being studied in phase 3 trials as systemic treatments for nonsegmental vitiligo
- With the emergence of potential new treatments for nonsegmental vitiligo, understanding patients' treatment priorities is critical to ensuring that treatment alternatives address patients' unmet medical needs

OBJECTIVE

- To quantify treatment priorities and unmet need among adults and adolescents with vitiligo in the United States

METHODS

Patients

- US participants aged ≥12 years with a self-reported physician diagnosis of nonsegmental vitiligo who were able to read and understand English to provide consent (adults age ≥18 years, older adolescents aged 15-17 years) or assent (younger adolescents aged 12-14 years with accompanying consent from parent or legal guardian) were recruited for the study
- Excluded were people with Vogt Koyanagi Harada disease, loss of skin color caused by chemicals or drugs, skin color disease related to pregnancy, cancer, psoriasis, leukoderma, acne, urticaria, atopic dermatitis/eczema, or scleroderma

Survey Instrument

- The preference-elicitation included 26 attributes related to treatment efficacy, safety, and mode of administration
- A 3-step adaptive self-explicated preference-elicitation method was used[5]
  - **Step 1**: Respondents rated the desirability of each level of an individual attribute on an 11-point scale from 0 (not at all desirable) to 10 (extremely desirable)
  - **Step 2**: Respondents sorted attributes into 3 groups based on perceived importance and then ranked the attributes within each group in order of importance
  - **Step 3**: Each respondent was then presented with a series of attribute pairs. For each pair, respondents were asked to allocate 100 points between the attributes to reflect the relative importance of the attributes. Pairs were generated based on an adaptive design matrix that maximizes the information provided by each question[6]
- For each respondent, satisfaction with each of their 10 most important attributes was elicited using a 5-point rating scale ranging from "completely dissatisfied" to "completely satisfied"

Statistical Analysis

- Latent class analysis (LCA) identified 3 preference classes in the full sample (pooled analysis of adults and adolescents)
- Relative importance (RI) of each attribute was estimated for each of the preference classes
- Unmet need was estimated using a modified outcome-driven innovation approach that defines unmet need as high importance combined with low satisfaction[7]
- Unmet need in this study was calculated as:

$$Unmet\ Need_i = RI_i + min(RI_i - Satisfaction_i, 0)^*$$

* Difference minimum = 0 to avoid negative scores.

  - for which RI_i is the mean relative importance score from the LCA for attribute i for the sample, scaled such that *max(RI)* = 10 and $Satisfaction_i$ is the proportion of the sample in each preference class who rated their current satisfaction with attribute *i* as "satisfied" or "very satisfied," rescaled such that *max(Satisfaction)* = 10

RESULTS

Sample

- The sample comprised adults (N=321) and adolescents (N=201) who received vitiligo care from 83 sites across the US
- Adults and adolescents had a mean (SD) age of 26 (9.1) and 14 (1.6) years, respectively
- More than 50% of participants self-identified as non-White
- 50% of participants were female
- Fitzpatrick skin types were 23.9% type I and II (pale white, fair), 43.6% III and IV (darker white, light brown), and 32.4% V and VI (brown, black)
- Participant characteristics by preference segment are presented in **Table 1**

Table 1. Participant characteristics by preference segment

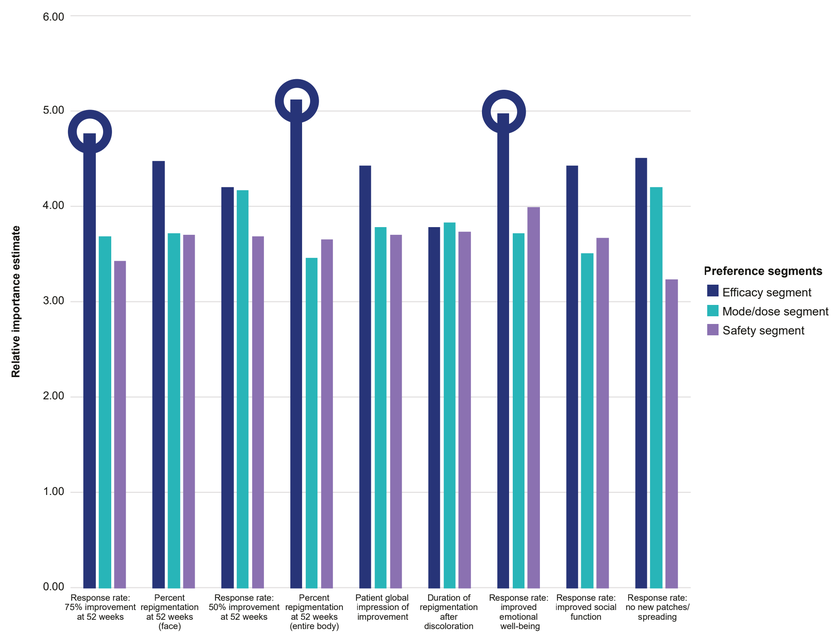
Characteristic	Segment 1 Efficacy (N=182)		Segment 2 MOA and Dosing (N=159)		Segment 3 Safety (N=181)		Total (N=522)		Pearson X^2
	N	%	N	%	N	%	N	%	
Race/ethnicity									
White	95	52.2	72	45.3	79	43.6	246	47.1	
Black/African American	25	13.7	18	11.3	14	7.7	57	10.9	
Latino/Hispanic	20	11.0	26	16.4	31	17.1	77	14.8	0.472
Middle Eastern	24	13.2	22	13.8	34	18.8	80	15.3	
Other	18	9.0	21	13.2	23	12.7	62	11.7	
Annual household income									
<$25,000	26	14.7	21	13.9	26	15.2	73	14.6	0.944
$25,000-$74,999	90	50.8	66	43.7	79	46.2	235	47.1	0.305
$75,000-250,000	61	34.5	64	42.4	66	38.6	191	38.3	0.436
Prefer not to answer	5	-	8	-	10	-	23	-	0.392
Highest level of education									
≤ High school graduate	36	33.6	34	33	29	29.3	99	32.0	0.427
≥ Some college	71	66.4	69	67	70	70.7	210	68.0	0.620
Prefer not to answer	2	-	3	-	7		12	-	0.195
Sex assigned at birth									
Male	98	53.8	78	49.1	85	47.0	261	50.0	
Female	81	44.5	75	47.2	88	48.6	244	46.7	0.468
Prefer not to answer	3	1.6	6	3.8	8	4.4	17	3.3	
Age, years									
Mean (SD)	26.8 (12.5)	-	28.7 (12.9)	-	27.5 (13.3)	-	27.5 (13.3)	-	0.868
Median	25	-	29	-	25	-	25	-	
Body surface area covered by vitiligo									
≥5%	85	46.7	77	48.4	61	33.7	223	42.7	0.010*
<5%	97	53.3	82	51.6	120	66.3	299	57.3	0.010*
Fitzpatrick skin type									
Type I-II	54	29.7	42	26.4	29	16.0	125	23.9	0.007*
Type III-IV	73	40.1	67	42.1	88	48.6	228	43.7	0.236
Type V-VI	55	30.2	50	31.4	64	35.4	169	32.4	0.553

SD, standard deviation. Pearson χ2 indicates difference between segments, <0.05 considered statistically significant.

Attribute Relative Importance

- The Efficacy-Focused preference segment (35% of the sample) placed the greatest importance on repigmentation and improving emotional well-being (**Figure 1**)
- The Safety-Focused preference segment (35% of the sample) placed the greatest importance on avoiding serious risks, including cardiovascular events, malignancies, and serious infections (**Figure 2**)
- The Mode of Administration and Dosing Focused preference segment (30% of the sample) placed the greatest importance on having an oral systemic treatment (**Figure 3**)

Figure 1. Relative importance of efficacy attributes, by preference segment

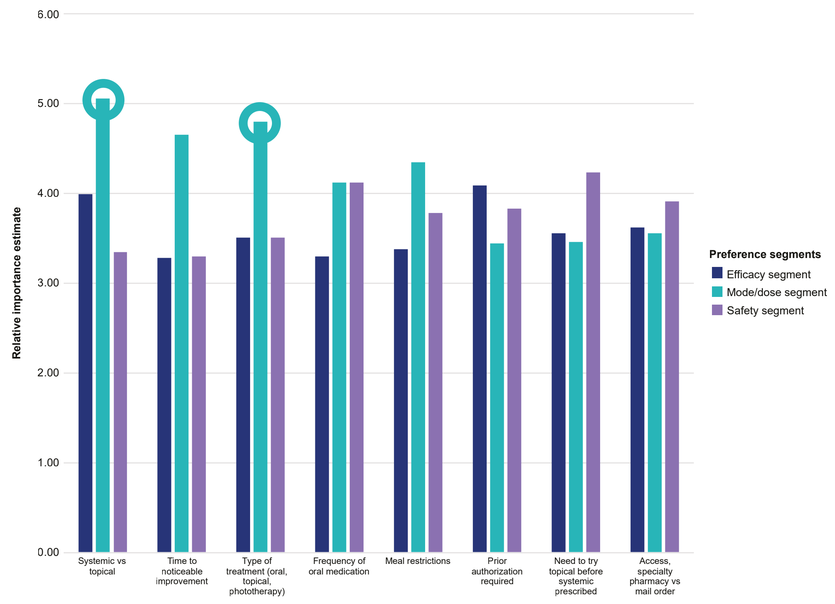


Note: The height of each bar indicates the relative importance of the attribute. The circles denote the highest relative importance scores within the attribute category.

Figure 2. Relative importance of safety attributes, by preference segment

Note: The height of each bar indicates the relative importance of the attribute. The circles denote the highest relative importance scores within the attribute category.

Figure 3. Relative importance of mode of administration and dosing attributes, by preference segment



Note: The height of each bar indicates the relative importance of the attribute. The circles denote the highest relative importance scores within the attribute category.

Unmet Need

- Among the full sample, the greatest areas of unmet need were reducing the emotional burden of vitiligo and having access to an oral systemic (rather than topical) treatment (**Table 2**)
- Within each preference segment, unmet need was correlated with the attributes most important to patients in that segment

Table 2. Highest unmet need by preference segment

Attribute	Unmet Need Scores			
	Segment 1 Efficacy (N=182)	Segment 2 MOA & Dosing (N=159)	Segment 3 Safety (N=181)	Total (N=522)
Amount of improvement (**repigmentation**) in vitiligo on the ***entire body*** after 1 year	11.74	6.87	8.05	10.95
Proportion of patients who an increase in **emotional well-being** because of an improvement (repigmentation) on the ***entire body*** after 1 year	11.64	7.40	9.34	11.91
Risk of blood clot in the lung or leg while taking treatment	6.04	7.14	12.50	10.26
Risk of shingles (a painful rash that develops on 1 side of the face or body) within 3 years of starting treatment	7.91	6.95	12.04	10.93
Part of the body targeted by the medication (**systemic over targeted**)	9.05	12.12	7.37	11.80
The way the treatment is given (**oral over topical**)	6.79	10.60	7.71	9.86

MOA, mode of administration.
Note: Green highlighting indicates highest unmet need by preference segment and for the full sample.

CONCLUSIONS

- Treatment preferences among people with vitiligo are heterogeneous
- In addition to repigmentation, reducing the emotional burden of vitiligo is a key treatment goal for patients
- The safety events that are most concerning to patients are cardiovascular events, serious infections, and malignancy, which are considered to be associated with JAK inhibition
- An effective oral systemic treatment could help address unmet need in this patient population

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