



Case Report

Successful Repigmentation of Extensive Pediatric Vitiligo with Oral Upadacitinib: A Case Report

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ABSTRACT

Generalized vitiligo in children is challenging to treat and often demonstrates an incomplete response to conventional therapies. In this case report, a 12-year-old boy with generalized vitiligo involving 75% of the body surface area had failed nearly two years of treatment with home narrowband UVB phototherapy, topical tacrolimus, topical corticosteroids, and pulsed oral dexamethasone. Oral upadacitinib at a dose of 30 mg daily was initiated in August 2025. Significant repigmentation was observed within three months, with complete clearance of facial lesions and marked improvement across the trunk and extremities. Treatment with oral upadacitinib as monotherapy allowed for the complete discontinuation of all topical agents and phototherapy. This case highlights the potential role of oral Janus kinase inhibitors as an effective therapeutic option in selected pediatric patients with refractory generalized vitiligo.

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1. Introduction

Vitiligo is an acquired autoimmune depigmentation disorder caused by destruction of melanocytes. It affects approximately 0.5–2% of the global population, with at least 25% of cases beginning before age 10 (1). In children with extensive disease (>50% body surface area [BSA]), vitiligo confers a substantial psychosocial burden, including stigma, bullying, anxiety, and impaired quality of life (2).

The pathogenesis is driven by interferon-gamma signaling through the Janus kinase (JAK)-signal transducer and activator of transcription (JAK-STAT) pathway, making JAK inhibitors (JAKi) a rational therapeutic target (3). First-line treatments including topical corticosteroids, tacrolimus, pulsed oral corticosteroids, and narrowband ultraviolet B (nbUVB) phototherapy, often yield only partial or transient repigmentation in refractory generalized disease and impose a significant treatment burden (4).

Topical ruxolitinib cream (Opzelura) received U.S. FDA approval in July 2022 for nonsegmental vitiligo in patients 12 years of age and older, based on two

phase 3 randomized trials demonstrating significant repigmentation (5). Despite this advance in topical JAK inhibition, oral JAKi remains an emerging therapeutic option, with only sparse data available in pediatric populations.

Oral JAKi have shown promising efficacy mainly in adults. Phase 2 and phase 3 trials have demonstrated significant repigmentation with once-daily upadacitinib in adults (6). In contrast, pediatric data remain scarce. A case series of 13 children (ages 6–16 years) treated with oral tofacitinib, which exhibits JAK1, JAK3 and some JAK2 inhibition, showed significant repigmentation in 8 patients (7). Experience with upadacitinib in pediatric vitiligo is limited to a small case series of four children (ages 6–7 years) achieving mean 60.2% Vitiligo Area Scoring Index improvement at 24 weeks (8).

Herein, we report a 12-year-old boy with longstanding generalized vitiligo refractory to nearly two years of conventional therapies who was treated with oral upadacitinib.

2. Case Report

A 10-year-old boy presented to our pediatric dermatology clinic on September 26, 2023, for a second opinion regarding widespread vitiligo that had been present for slightly over one year. The lesions were first noticed on the hands and chest as small white patches that quickly spread to the arms, back, legs, feet, hands, and face. The lesions were not itchy, painful, or cosmetically bothersome to the patient. His past medical history included mild intermittent asthma and reading difficulty. Family history was negative for hypothyroi-

dism, rheumatoid arthritis, or other autoimmune disorders. Laboratory evaluation by his primary care provider, including thyroid studies, vitamin D level, and hemoglobin A1c, was within normal limits. Physical examination (Figure 1) revealed well-circumscribed chalk-white depigmented macules and patches with moth-eaten borders involving the face, neck, chest, back, groin, buttocks, arms, hands, legs, and feet, affecting approximately 75% BSA. No leukotrichia was noted on examination.



Figure 1. Baseline appearance of generalized vitiligo in September 2023, prior to start of conventional therapy. Extensive well-circumscribed chalk-white depigmented macules and patches with moth-eaten borders involved the face, neck, trunk, extremities, groin, and acral sites, affecting approximately 75% body surface area (BSA).

The patient had previously been evaluated by an outside dermatology practice and treated with topical corticosteroids and topical tacrolimus without improvement. At our initial visit in September 2023, a shared decision-making approach led to initiation of topical tacrolimus 0.03% ointment twice daily to the face, neck, axillae, and groin; triamcinolone 0.1% ointment twice daily, three days per week, to the arms, legs, and trunk; pulsed oral dexamethasone 4 mg each Saturday and Sunday for 12 weeks; and home nbUVB phototherapy three times weekly. At the three-month follow-up in January 2024, the family reported significant improvement in pigmentation and BSA involvement had decreased to 50%. The regimen was continued, with ta-

crolicimus increased to 0.1% when possible.

By July 2025, when the patient was 12 years old, the disease had progressed again despite ongoing treatment. The family reported that his condition was worse than previous visits, with suspected new areas of depigmentation and recurrent sunburns (Figure 2). Adherence to topical therapies had become inconsistent because they were no longer perceived as providing meaningful improvement. Topical ruxolitinib was not selected, as FDA approval is limited to a maximum treatment area of 10% BSA. Instead, the option of an oral JAKi was discussed in detail with the patient and family, including common and serious risks. The patient had no family history of clotting disorders.



Figure 2. Appearance of vitiligo in July 2025, after nearly 2 years of conservative therapy with home narrowband UVB phototherapy, topical tacrolimus, topical corticosteroids, and pulsed oral dexamethasone. The disease remained largely refractory with persistent involvement of approximately 50% BSA prior to initiation of oral upadacitinib.

Treatment was initiated with oral upadacitinib 30 mg daily in August 2025 after screening laboratories returned normal and prior authorization was approved. All topical medications and phototherapy were discontinued. Baseline laboratory evaluation prior to initiating upadacitinib included complete blood count, comprehensive metabolic panel, lipid panel, hepatitis serologies, and tuberculosis testing, all of which were within normal limits except for mildly elevated triglycerides. Repeat laboratory monitoring in October 2025 showed no significant changes. Given the absence of major risk factors and stable laboratory findings, repeat laboratory monitoring was planned for every 6-12 months unless clinically indicated. The patient and family were counseled on medication administration, periodic laboratory monitoring, and precautions regarding live vaccines.

At the three-month follow-up in November 2025 the patient and his father reported significant improvement.

Facial involvement had completely resolved and repigmentation was noted on the chest and back (Figure 3). He remained on upadacitinib 30 mg daily. Mild morning fatigue was noted but did not interfere with daily activities. At the six-month follow-up, steady improvement was observed across the face, chest, back, arms, and legs, with many previously depigmented areas now showing light tan macules coalescing into tan patches. Clinical assessment demonstrated substantial repigmentation, with complete clearance of facial involvement and marked improvement on the trunk and extremities, corresponding to a reduction in affected body surface area from approximately 75% at baseline prior to upadacitinib to <50%. The patient had experienced four mild self-limited upper respiratory infections over the winter. Due to mild fatigue and the increased frequency of upper respiratory infections, the dose was reduced to upadacitinib 15 mg daily.



Figure 3. Significant repigmentation in February 2026, six months after initiation of oral upadacitinib. Multiple previously depigmented areas on the face, chest, back, arms, and legs demonstrate light tan macules coalescing into tan patches.

3. Discussion

This case demonstrates a marked response to oral upadacitinib in a pediatric patient with refractory generalized vitiligo. After nearly two years of conventional therapies including home nbUVB phototherapy, topical tacrolimus, topical triamcinolone, and pulsed oral dexamethasone, the patient achieved complete facial repigmentation and substantial improvement across the trunk and extremities within six months of initiating upadacitinib 30 mg daily. This response allowed

complete discontinuation of all topical agents and phototherapy and represents successful use of oral JAKi in a child with extensive disease that had failed standard first-line options.

In adult patients with extensive non-segmental vitiligo, oral upadacitinib has shown strong efficacy. The phase 2 randomized trial by Passeron et al reported significant facial and total body repigmentation with once-daily dosing (6). However, pediatric data remain

scarce. While small case series using a different JAKi, oral tofacitinib, have reported repigmentation rates of 70–80% (7), responses appear to be slower and less complete than those seen in adult trials. Experience with upadacitinib in children is even more limited.

This pediatric case describes the successful treatment of extensive generalized vitiligo, initially involving 75% of the BSA, which had remained refractory despite nearly two years of conventional therapies (Figures 1–3). This report also provides practical, real-world insight into the management of common adverse effects. Mild morning fatigue and an increased frequency of upper respiratory infections prompted a dose reduction from upadacitinib 30 mg to 15 mg daily at the six-month follow-up visit. These effects are recognized, dose-dependent adverse events associated with JAK1 inhibition. At the time of dose reduction, the patient had already achieved significant and sustained repigmentation, and the dose adjustment was undertaken to optimize tolerability while maintaining clinical response.

Importantly, oral upadacitinib used as monotherapy proved sufficient for significant and sustained repigmentation, allowing complete discontinuation of all topical agents and phototherapy. This simplified treat-

ment regimen may improve adherence and reduce treatment burden and suggests that oral JAKi may emerge as a promising monotherapy for pediatric patients with refractory generalized vitiligo.

The safety profile observed in this pediatric patient aligns with the known profile of upadacitinib. No serious infections, laboratory abnormalities, cardiovascular events, or thromboembolic complications were observed. Mild morning fatigue and recurrent upper respiratory infections were managed with dose reduction, consistent with longer-term data from atopic dermatitis trials in adolescents, in which similar mild adverse events were managed without discontinuation (9).

Limitations of this report include its single-patient nature and relatively short follow-up of six months. Larger, longer-term studies are needed to confirm durability of response, optimal dosing strategies, and long-term safety in the pediatric population. Despite these constraints, the degree of repigmentation achieved with oral monotherapy following failure of conventional therapies is noteworthy.

4. Conclusions

This case demonstrates that oral upadacitinib can induce significant and sustained repigmentation in a pediatric patient with extensive generalized vitiligo refractory to nearly two years of conventional therapies.

The successful use of oral JAK inhibition as monotherapy highlights its potential as a valuable treatment option for pediatric vitiligo when standard therapies fail.

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