**Category**

Best Pharmaceutical Product

**General Information**

**Company Name ***

Incyte Corporation

**Product/Solution Name ***

Opzelura® (ruxolitinib) cream 1.5%

**Compound/Tech Name***

Ruxolitinib cream

**Trade Name ***

Opzelura®

**Corporate Name ***

Opzelura®

**Date of Approval ***

2021-09-21

**Indications ***

Opzelura® (ruxolitinib) cream 1.5% is indicated for the topical short-term and non-continuous chronic treatment of mild to moderate atopic dermatitis in non-immunocompromised adult and pediatric patients 12 years of age and older whose disease is not adequately controlled with topical prescription therapies or when those therapies are not advisable. It is also indicated for the topical treatment of nonsegmental vitiligo in adult and pediatric patients 12 years of age and older.1

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**Therapeutic Areas ***

Dermatology - atopic dermatitis and vitiligo

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Attached Files:

- Opzelura_Prix Galien_Nomination_7July2025_FINAL.pdf

**Background information and need for drug / device**
**(please be as specific as possible in your description; limit 500 words)**

Opzelura® (ruxolitinib) cream 1.5% is a steroid-free topical formulation of Incyte's JAK1/JAK2 inhibitor, ruxolitinib. In September 2021, Opzelura became the first FDA-approved topical JAK inhibitor for the treatment of mild to moderate atopic dermatitis (AD). AD, the most common type of eczema, is a chronic, immune-mediated condition that is difficult to manage.

AD affects more than 21 million people aged 12 years and older in the U.S. and is characterized by inflammation and itch.2 Signs and symptoms include irritated and itchy skin that can cause red lesions that may ooze and crust. People with AD are also more susceptible to bacterial, viral and fungal infections.3

AD can profoundly impact a person's daily life. Patients and caregivers have expressed dissatisfaction and shortcomings with treatments, including fears associated with injections, and stinging or burning topical treatments.4

The FDA approved Opzelura for mild to moderate AD in patients 12 years and older.

In July 2022, Opzelura became the first FDA-approved pharmacologic treatment for repigmentation in adult and pediatric patients 12 years of age and older with nonsegmental vitiligo.1

Vitiligo is a chronic autoimmune condition characterized by white patches of depigmented skin on affected areas of the face and body, resulting from the loss of pigment-producing cells called melanocytes. The condition occurs with similar frequency across all races and backgrounds, and it can occur at any age, although initial symptoms usually appear before age 30.5

In the United States, vitiligo affects approximately 2-3 million adults, with most patients (approximately 85%) living with nonsegmental vitiligo.6 Until Opzelura, there were no FDA-approved pharmacologic therapies for repigmentation.7

Some people with vitiligo anecdotally explain that losing pigment can feel like losing their identity and call the visible condition "life-altering."8 There are many misconceptions about vitiligo, such as the condition being contagious or just a cosmetic issue, which can cause those living with it to feel misunderstood and can hinder proper understanding and effective disease management.9

In the absence of FDA-approved therapies for repigmentation, some people with vitiligo say they accepted the condition as "something they have to live with."8 Now, Opzelura offers the first pharmacologic treatment option for people with vitiligo who choose to pursue repigmentation, in partnership with a dermatologist.

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**History of the development of the solution/product ***
**(please be as specific as possible in your description; 500 words)**

Ruxolitinib, the active ingredient of Opzelura, is a JAK1/JAK2 inhibitor developed by Incyte through years of pioneering research into the therapeutic potential of this mechanism of action.10 Ruxolitinib inhibits the JAK-STAT pathway, which is responsible for the downstream signaling and production of key pro-inflammatory cytokines implicated in many immune-mediated dermatologic conditions.

Opzelura received its first approval in 2021 for mild to moderate AD in patients 12 years and older based on 2, identically-designed, 8-week, Phase 3 studies. In these studies, over half of treated patients achieved clear or almost clear skin and experienced clinically meaningful itch relief, with some noting itch reduction within days.1,11,12 With this approval, Opzelura became the first topical JAK inhibitor for AD.10

Less than a year later, Opzelura became the first pharmacologic therapy FDA-approved for repigmentation of nonsegmental vitiligo in patients 12 years of age and older.1,13 In clinical studies, one-third of patients using Opzelura saw at least 75% improvement to vitiligo areas on the face at Week 24, meeting the primary endpoint, and approximately 25% reached at least 50% improvement to vitiligo areas across the entire body.13 Continued use of Opzelura resulted in further improvement in facial and total body repigmentation at Week 52. At this point, approximately 50% of patients using Opzelura saw at least 75% improvement in facial vitiligo, and an even greater proportion of patients achieved at least 50% improvement to vitiligo areas across the entire body.13

Long-term extension data from the Phase 3 studies in vitiligo showed over half of patients with limited to no facial repigmentation at Week 24 achieved ≥75% improvement by Week 104. Notably, 97.1% of facial and 93.3% of body non-responders at Week 24 saw significant repigmentation improvements by Week 104.14,15

These findings support Opzelura as a treatment option for immune-mediated dermatologic conditions.

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**Why this drug or device is innovative, the broad implications for future research, and/or how it will improve the human condition ***

Opzelura represents a major advancement in dermatologic treatment. As the first FDA-approved topical JAK inhibitor, it offers a non-steroidal solution for AD and vitiligo patients whose lives are impacted by their respective chronic skin conditions.

The approval of Opzelura in AD represented an important treatment advancement for patients and their healthcare providers. For those with AD, chronic and recurrent itch is frequently reported among patients as the most burdensome symptom, and many patients did not respond well to existing treatment options, resulting in uncontrolled disease.1 Phase 3 clinical studies of Opzelura assessed clinically meaningful itch improvement as a key secondary endpoint, which was reported by >50% of patients.1 Additionally, in extension data from the Phase 3 studies in AD, patients continued with twice-daily applications of Opzelura and achieved sustained disease control, as 74.1% to 77.8% of patients reached an Investigator's Global Assessment (IGA) score of 0/1. The extension study

concluded that Opzelura maintained effective control with minimal systemic absorption and infrequent application site reactions.17 Opzelura provides a non-steroidal, topical treatment that is not only easy to apply and manage during flares but also alleviates itch, setting it apart from traditional therapies.

For those with vitiligo, the approval of Opzelura represented a significant milestone for an underserved patient population with high unmet medical need and significant disease burden.18 Phase 3 data supporting the approval showed that treatment with Opzelura resulted in improvements in facial and total body repigmentation, compared to the vehicle control cream.13

Prior to the FDA approval of Opzelura, no other pharmacologic treatments for repigmentation existed for patients with nonsegmental vitiligo. Now, people with nonsegmental vitiligo who choose to pursue repigmentation have a pharmacologic treatment option to include in their management plans.

Building on its current approved indications, Incyte aims to provide younger children with AD and their families with another, much-needed, steroid-free topical treatment option and has submitted a supplemental New Drug Application (sNDA) to the FDA for potential approval of ruxolitinib cream (Opzelura) use in children ages 2-11 with AD, supported by data from the Phase 3 TRuE-AD3 trial.19 This population is investigational and is still under FDA review. No conclusions of efficacy or safety can be made based on this trial.

Beyond its approved use in AD and vitiligo, multiple clinical studies of ruxolitinib cream are underway in pediatric vitiligo and other immune-mediated dermatologic conditions such as prurigo nodularis and hidradenitis suppurativa, a devastating disease with very limited options. These clinical studies will explore its potential to bring solutions to patients in need beyond the current uses.20 Leveraging its knowledge and understanding of cellular pathways and immune system function, Incyte continues to advance research in inflammation and autoimmunity to address areas of critical healthcare importance.

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**Please provide appropriate references (PubMed, Abstract, Website) ***

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