Incyte Announces Additional FDA Approval of Opzelura® (Ruxolitinib) Cream in Children Ages 2-11 with Atopic Dermatitis

September 18, 2025

- *Opzelura is the first topical JAK inhibitor approved in the U.S. for pediatric atopic dermatitis (AD)*
- *Phase 3 data supporting the approval show that treatment with Opzelura resulted in significant efficacy, with no new safety concerns identified*
- *This milestone marks the third U.S. approval for Opzelura, which is now indicated to treat mild to moderate AD in non-immunocompromised patients 2+ years of age and nonsegmental vitiligo in patients 12+ years of age*

WILMINGTON, Del.--(BUSINESS WIRE)--Sep. 18, 2025-- Incyte (Nasdaq: INCY) today announced that the U.S. Food and Drug Administration (FDA) has approved Opzelura® (ruxolitinib) cream 1.5%, a topical Janus kinase (JAK) inhibitor, for the short-term and non-continuous chronic treatment of mild to moderate atopic dermatitis (AD) in non-immunocompromised children two years of age and older whose disease is not well controlled with topical prescription therapies, or when those therapies are not recommended.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20250918551027/en/

Opzelura® (ruxolitinib) cream 1.5% logo

"With this approval, we are now able to offer younger children with atopic dermatitis and their families a much-needed, steroid-free topical treatment option with the potential to significantly improve the burdensome symptoms they experience every day," said Bill Meury, Chief Executive Officer, Incyte. "At Incyte, we are committed to delivering innovative solutions that address every stage of a patient's journey; this approval is another step toward addressing the real-world challenges faced by patients suffering from chronic skin conditions, including people living with atopic dermatitis."

The FDA approval of the supplemental New Drug Application (sNDA) for Opzelura was based on data from the pivotal Phase 3 TRuE-AD3 trial, which evaluated the safety and efficacy of ruxolitinib cream in children (age ≥2 to <12 years) with AD.[1] The TRuE-AD3 study met its primary endpoint with significantly more patients treated with Opzelura achieving Investigator's Global Assessment-treatment success (IGA-TS), a measure of treatment efficacy, than patients treated with vehicle control (non-medicated cream). In addition, a secondary endpoint of patients demonstrating at least a 75% improvement in the Eczema Area and Severity Index (EASI75) at Week 8 was also achieved.

The overall safety profile of Opzelura in the TRuE-AD3 trial was consistent with previous data, and no new safety signals were observed. No serious infections, major adverse cardiovascular events (MACE), malignancies or thromboses were reported during the 8-week vehicle-controlled period. The most common adverse reaction was upper respiratory tract infection.

"Navigating a complex condition like atopic dermatitis can be very challenging for children, who currently have limited treatment options to meet their specific needs," said Dr. Peter Lio, M.D., Clinical Assistant Professor of Dermatology & Pediatrics at Northwestern University Feinberg School of Medicine. "With this approval, we now have a new, non-steroidal topical option that expands how we care for kids with this chronic disease. This is a meaningful step forward and marks a significant advancement in our ability to better support our pediatric patients."

AD, the most common type of eczema, is a chronic immune-mediated skin disease, which in the U.S. affects an estimated 2-3 million patients ages 2-11 and more than 21 million people 12 years of age and older.[2,3] Signs and symptoms include irritated skin that can cause red lesions that may ooze and crust. People with AD are also more susceptible to bacterial, viral and fungal infections.[4]

"As a parent of a child with atopic dermatitis, it has been challenging to see my daughter struggle growing up with her condition. There were many times her mother and I felt frustrated with the lack of treatment options that worked for our family, which was very difficult to manage as a parent," said Adam Flores, father of Piper (age 10), who lives with AD. "We spent years searching for the right treatment for Piper and, after joining the Opzelura clinical trial, we finally saw real relief for her eczema symptoms. This approval brings hope for a new treatment to families like ours who have spent years searching for answers."

"While every child's journey with AD is unique, for many, the skin redness and irritation can profoundly impact their well being," said Korey Capozza, Founder of Global Parents for Eczema Research (GPER). "When you're managing a condition that can affect daily life, access to safe, effective, and age-appropriate options is critical. With limited, safe treatment options currently available, especially for younger children, the addition of new therapies that control symptoms is so important to meet the needs and goals for children with AD and their families."

In September 2021, Opzelura was approved by the FDA for the topical short-term and non-continuous chronic treatment of mild to moderate AD in non-immunocompromised patients 12 years of age and older whose disease is not adequately controlled with topical prescription therapies, or when those therapies are not advisable. In July 2022, Opzelura was approved by the FDA for the treatment of nonsegmental vitiligo in adult and pediatric patients 12 years of age and older.

Incyte is committed to supporting patients and removing barriers to access medicines. Eligible patients in the U.S. who are prescribed Opzelura have access to Opzelura On Trac™, a patient support program providing tools and resources for patients along their treatment journey with Opzelura, including how to obtain, use and afford their prescription and refills. For more information, please visit https://www.opzeluraontrac.com/.

About TRuE-AD3

TRuE-AD3 (NCT04921969) is a randomized, double-blind, vehicle-controlled Phase 3 study evaluating the safety and efficacy of ruxolitinib cream compared to vehicle (non-medicated cream) in children with atopic dermatitis (AD). The study enrolled over 300 patients (age ≥2 to <12 years) diagnosed with AD for at least three months and who were candidates for topical therapy.

Patients with an Investigator's Global Assessment (IGA) score of 2 to 3 (a measure of disease severity), and with AD on 3% to 20% of their Body Surface Area (BSA; excluding scalp) were randomized 2:2:1 to receive ruxolitinib cream 0.75% administered twice daily (BID); ruxolitinib cream 1.5% BID; or vehicle (non-medicated cream) BID. Patients who successfully completed an efficacy assessment at Week 8 were offered participation in the 44-week long-term safety treatment extension period with their same treatment group (ruxolitinib cream 0.75% or 1.5% BID). Patients initially randomized to vehicle cream were re-randomized (1:1) in a blinded manner to one of the active treatment groups.

The primary endpoint of TRuE-AD3 is the proportion of patients achieving an Investigator's Global Assessment Treatment Success (IGA-TS), defined as an IGA score of 0 (clear) or 1 (almost clear) with at least a two-point improvement from baseline at Week 8. Secondary endpoints include: the proportion of patients achieving at least a 75% improvement in the Eczema Area and Severity Index (EASI75) – another measurement of disease extent and severity – the proportion of patients (age ≥6 to <12 years) with at least a 4-point improvement in the itch numerical rating scale (NRS4 at Week 8 and time to achieve NRS4). The study also tracked the frequency, duration and severity of adverse events associated with the use of ruxolitinib cream.

For more information about the study, please visit https://www.clinicaltrials.gov/study/NCT04921969.

About Opzelura® (ruxolitinib) Cream

Opzelura (ruxolitinib) cream, a novel cream formulation of Incyte's selective JAK1/JAK2 inhibitor ruxolitinib, approved by the U.S. Food & Drug Administration for the topical treatment of nonsegmental vitiligo in patients 12 years of age and older, is the first and only treatment for repigmentation approved for use in the United States. Opzelura is also approved in the U.S. for the topical short-term and non-continuous chronic treatment of mild to moderate atopic dermatitis (AD) in non-immunocompromised patients 2+ years of age whose disease is not adequately controlled with topical prescription therapies, or when those therapies are not advisable. Use of Opzelura in combination with therapeutic biologics, other JAK inhibitors, or potent immunosuppressants, such as azathioprine or cyclosporine, is not recommended.

In Europe, Opzelura® (ruxolitinib) cream 15mg/g is approved for the treatment of non-segmental vitiligo with facial involvement in adults and adolescents from 12 years of age.

Incyte has worldwide rights for the development and commercialization of ruxolitinib cream, marketed in the United States as Opzelura.

IMPORTANT SAFETY INFORMATION

OPZELURA is for use on the skin only. Do not use OPZELURA in your eyes, mouth, or vagina.

OPZELURA may cause serious side effects, including:

Serious Infections: OPZELURA contains ruxolitinib. Ruxolitinib belongs to a class of medicines called Janus kinase (JAK) inhibitors. JAK inhibitors are medicines that affect your immune system. JAK inhibitors can lower the ability of your immune system to fight infections. Some people have had serious infections while taking JAK inhibitors by mouth, including tuberculosis (TB), and infections caused by bacteria, fungi, or viruses that can spread throughout the body. Some people have been hospitalized or died from these infections. Some people have had serious infections of their lungs while taking OPZELURA. Your healthcare provider should watch you closely for signs and symptoms of TB during treatment with OPZELURA.

OPZELURA should not be used in people with an active, serious infection, including localized infections. You should not start using OPZELURA if you have any kind of infection unless your healthcare provider tells you it is okay. You may be at a higher risk of developing shingles (herpes zoster) while using OPZELURA.

Increased risk of death due to any reason (all causes): Increased risk of death has happened in people 50 years of age and older who have at least 1 heart disease (cardiovascular) risk factor and are taking a medicine in the class of medicines called JAK inhibitors by mouth.

Cancer and immune system problems: OPZELURA may increase your risk of certain cancers by changing the way your immune system works. Lymphoma and other cancers have happened in people taking a medicine in the class of medicines called JAK inhibitors by mouth. People taking JAK inhibitors by mouth have a higher risk of certain cancers including lymphoma and lung cancer, especially if they are a current or past smoker. Some people have had skin cancers while using OPZELURA. Your healthcare provider will regularly check your skin during your treatment with OPZELURA. Limit the amount of time you spend in the sunlight. Wear protective clothing when you are in the sun and use a broad-spectrum sunscreen.

Increased risk of major cardiovascular events: Increased risk of major cardiovascular events such as heart attack, stroke, or death have happened in people 50 years of age and older who have at least 1 heart disease (cardiovascular) risk factor and taking a medicine in the class of medicines called JAK inhibitors by mouth, especially in current or past smokers.

Blood clots: Blood clots in the veins of your legs (deep vein thrombosis, DVT) or lungs (pulmonary embolism, PE) can happen in some people taking OPZELURA. This may be life-threatening. Blood clots in the vein of the legs (deep vein thrombosis, DVT) and lungs (pulmonary embolism, PE) have happened more often in people who are 50 years of age and older and with at least 1 heart disease (cardiovascular) risk factor taking a medicine in the class of medicines called JAK inhibitors by mouth.

Low blood cell counts: OPZELURA may cause low platelet counts (thrombocytopenia), low red blood cell counts (anemia), and low white blood cell counts (neutropenia). Your healthcare provider may do a blood test to check your blood cell counts during your treatment with OPZELURA and may stop your treatment if signs or symptoms of low blood cell counts happen.

Cholesterol increases: Cholesterol increase has happened in people when ruxolitinib is taken by mouth. Tell your healthcare provider if you have high cholesterol or triglycerides.

Before starting OPZELURA, tell your healthcare provider if you:

- have an infection, are being treated for one, or have had an infection that does not go away or keeps coming back
- have diabetes, chronic lung disease, HIV, or a weak immune system
- have TB or have been in close contact with someone with TB
- have had shingles (herpes zoster)
- have or have had hepatitis B or C
- live, have lived in, or have traveled to certain parts of the country (such as the Ohio and Mississippi River valleys and the Southwest) where there is an increased chance for getting certain kinds of fungal infections. These infections may happen or become more severe if you use OPZELURA. Ask your healthcare provider if you do not know if you have lived in an area where these infections are common.
- think you have an infection or have symptoms of an infection such as: fever, sweating, or chills, muscle aches, cough or shortness of breath, blood in your phlegm, weight loss, warm, red, or painful skin or sores on your body, diarrhea or stomach pain, burning when you urinate or urinating more often than usual, feeling very tired.
- have ever had any type of cancer, or are a current or past smoker
- have had a heart attack, other heart problems, or a stroke
- have had blood clots in the veins of your legs or lungs in the past
- have high cholesterol or triglycerides
- have or have had low white or red blood cell counts
- are pregnant or plan to become pregnant. It is not known if OPZELURA will harm your unborn baby. There is a pregnancy exposure registry for individuals who use OPZELURA during pregnancy. The purpose of this registry is to collect information about the health of you and your baby. If you become exposed to OPZELURA during pregnancy, you and your healthcare provider should report exposure to Incyte Corporation at 1-855-463-3463 or www.opzelura.pregnancy.incyte.com.
- are breastfeeding or plan to breastfeed. It is not known if OPZELURA passes into your breast milk. Do not breastfeed during treatment with OPZELURA and for about 4 weeks after the last dose.

After starting OPZELURA:

- Call your healthcare provider right away if you have any symptoms of an infection. OPZELURA can make you more likely to get infections or make worse any infections that you have.
- Get emergency help right away if you have any symptoms of a heart attack or stroke while using OPZELURA, including:
  - discomfort in the center of your chest that lasts for more than a few minutes, or that goes away and comes back
  - severe tightness, pain, pressure, or heaviness in your chest, throat, neck, or jaw
  - pain or discomfort in your arms, back, neck, jaw, or stomach
  - shortness of breath with or without chest discomfort
  - breaking out in a cold sweat
  - nausea or vomiting
  - feeling lightheaded
  - weakness in one part or on one side of your body
  - slurred speech
- Tell your healthcare provider right away if you have any signs and symptoms of blood clots during treatment with OPZELURA, including: swelling, pain, or tenderness in one or both legs, sudden, unexplained chest or upper back pain, or shortness of breath or difficulty breathing.
- Tell your healthcare provider right away if you develop or have worsening of any symptoms of low blood cell counts, such as: unusual bleeding, bruising, tiredness, shortness of breath, or fever.

Tell your healthcare provider about all the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

The most common side effects of OPZELURA in people 12 years of age and older treated for atopic dermatitis include: common cold (nasopharyngitis), bronchitis, ear infection, increase in a type of white blood cell (eosinophil) count, hives, diarrhea, inflamed hair pores (folliculitis), swelling of the tonsils (tonsillitis), and runny nose (rhinorrhea). For people 2-11 years: upper respiratory tract infection, COVID-19, application site reaction, fever, white blood cell count decreased.

These are not all of the possible side effects of OPZELURA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088. You may also report side effects to Incyte Corporation at 1-855-463-3463.

Please see the Full Prescribing Information, including Boxed Warning, and Medication Guide for OPZELURA at Opzelura.com.

INDICATION AND USAGE

OPZELURA is a prescription medicine used on the skin (topical) for the short-term and non-continuous chronic treatment of mild to moderate eczema (atopic dermatitis) in non-immunocompromised adults and children 2 years of age and older whose disease is not well controlled with topical prescription therapies or when those therapies are not recommended.

The use of OPZELURA along with therapeutic biologics, other JAK inhibitors, or strong immunosuppressants such as azathioprine or cyclosporine is not recommended.

It is not known if OPZELURA is safe and effective in children less than 2 years of age with atopic dermatitis.

About Incyte Dermatology

Incyte's science-first approach and expertise in immunology has formed the foundation of the company. Today, we are building on this legacy as we discover and develop innovative dermatology treatments to bring solutions to patients in need.

We strive to identify and develop therapies to modulate immune pathways driving uncontrolled inflammation to help restore normal immune function and bring the body closer to homeostasis. Specifically, our efforts in dermatology are focused on a number of immune-mediated dermatologic conditions with a high unmet medical need, including atopic dermatitis, vitiligo, hidradenitis suppurativa, lichen sclerosus, and prurigo nodularis.

To learn more, visit the Dermatology section of Incyte.com.

About Incyte

A global biopharmaceutical company on a mission to *Solve On.*, Incyte follows the science to find solutions for patients with unmet medical needs. Through the discovery, development and commercialization of proprietary therapeutics, Incyte has established a portfolio of first-in-class medicines for patients and a strong pipeline of products in Oncology and Inflammation & Autoimmunity. Headquartered in Wilmington, Delaware, Incyte has operations in North America, Europe and Asia.

For additional information on Incyte, please visit Incyte.com or follow us on social media: LinkedIn, X, Instagram, Facebook, YouTube.

Incyte Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding whether Opzelura will provide a successful treatment option for children ages 2-11 with atopic dermatitis, contain predictions, estimates, and other forward-looking statements.

These forward-looking statements are based on our current expectations and are subject to risks and uncertainties that may cause actual results to differ materially, including unanticipated developments in and risks related to: unanticipated delays; further research and development and the results of clinical trials possibly being unsuccessful or insufficient to meet applicable regulatory standards or warrant continued development; the ability to enroll sufficient numbers of subjects in clinical trials and the ability to enroll subjects in accordance with planned schedules; determinations made by the FDA and other regulatory agencies; the efficacy or safety of our products; the acceptance of our products in the marketplace; market competition; unexpected variations in the demand for our products; the effects of announced or unexpected price regulation or limitations on reimbursement or coverage for our products; sales, marketing, manufacturing, and distribution requirements, including our ability to successfully commercialize and build commercial infrastructure for newly approved products and any additional new products that become approved; and other risks detailed from time to time in our reports filed with the U.S. Securities and Exchange Commission, including our annual report on Form 10-K and our quarterly report on Form 10-Q for the quarter ended June 30, 2025. We disclaim any intent or obligation to update these forward-looking statements.

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[1] https://investor.incyte.com/news-releases/news-release-details/incyte-announces-new-data-ruxolitinib-cream-opzelurar-children

[2] U.S. Census Bureau (2020). 2020 Decennial Census. Retrieved from https://data.census.gov/cedsci/table?q=Populations%20and%20People&tid=DECENNIALPL2020.P1 [data.census.gov].

[3] Data on file.

[4] Boguniewicz M, et al. Ann Allergy Asthma Immunol. 2018;120(1):10-22.

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