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Incyte Announces U.S. FDA Approval of Opzelura™ (ruxolitinib) Cream for the Treatment of Vitiligo

July 19, 2022

- *Opzelura is the first and only FDA-approved product for repigmentation in nonsegmental vitiligo*
- *Phase 3 data supporting the approval show treatment with Opzelura resulted in improvements in facial and total body repigmentation*
- *Fifty-two week data demonstrate continued improvements in repigmentation with longer duration of treatment*
- *Investor conference call and webcast scheduled for July 19, 2022, at 8:00 a.m. EDT*

WILMINGTON, Del.--(BUSINESS WIRE)--Jul. 18, 2022-- Incyte (Nasdaq:INCY) today announced that the U.S. Food and Drug Administration (FDA) has approved Opzelura™ (ruxolitinib) cream 1.5% for the topical treatment of nonsegmental vitiligo in adult and pediatric patients 12 years of age and older. Opzelura is the first and only FDA-approved treatment for repigmentation in patients with vitiligo, and the only topical formulation of a Janus kinase (JAK) inhibitor approved in the United States. Vitiligo is a chronic autoimmune disease characterized by depigmentation of skin.

This press release features multimedia. View the full release here: <https://www.businesswire.com/news/home/20220718005819/en/>



“With the approval of Opzelura in nonsegmental vitiligo, Incyte has once again delivered a treatment to patients with high unmet medical need who previously had no approved therapies,” said Hervé Hoppenot, Chief Executive Officer, Incyte. “We are proud of Incyte’s scientists and development teams that have made this milestone possible, and we’re pleased that eligible vitiligo patients now have a choice to address repigmentation.”

In patients with non-segmental vitiligo, Opzelura is approved for continuous topical use twice daily to affected areas of up to 10% body surface area. Satisfactory patient response may require treatment with Opzelura for more than 24 weeks.

The FDA approval was based on data from the pivotal Phase 3 TRuE-V clinical trial program (TRuE-V1 and TRuE-V2), evaluating the safety and efficacy of Opzelura versus vehicle in more than 600 people with nonsegmental vitiligo, age 12 and older. In the studies, treatment with

Image of person with nonsegmental vitiligo (Photo: Business Wire)

Opzelura resulted in significant improvements in VASI scores, which represent improvements in facial and total body repigmentation at Week 24 (primary analysis) compared to vehicle (non-medicated cream) and in an open-label extension at Week 52.

Results at Week 24, which were consistent across both studies, showed that approximately 30% of patients treated with Opzelura achieved $\geq 75\%$ improvement from baseline in the facial Vitiligo Area Scoring Index (F-VASI75), the primary endpoint, compared to approximately 8% and 13% of patients treated with vehicle in TRuE-V1 and TRuE-V2, respectively. At Week 52, approximately 50% of Opzelura-treated patients achieved F-VASI75.

Additionally, at Week 24, more than 15% of patients treated with Opzelura achieved $\geq 90\%$ improvement from baseline in F-VASI (F-VASI90), compared to approximately 2% of patients treated with vehicle. At Week 52, the percentage of Opzelura-treated patients who achieved F-VASI90 doubled to approximately 30%.

In the vehicle controlled period of the Phase 3 studies, the most common adverse reactions (incidence $\geq 1\%$) are application site acne, application site pruritus, nasopharyngitis, headache, urinary tract infection, application site erythema, and pyrexia¹. The labeling for Opzelura includes a Boxed Warning for serious infections, mortality, malignancy, major adverse cardiovascular events and thrombosis. See additional Important Safety Information below.

Week 52 data from the Phase 3 TRuE-V studies were [featured](#) in an oral presentation at the late-breaking abstract session at the American Academy of Dermatology (AAD) Annual 2022 Meeting.

Vitiligo is a chronic autoimmune disease characterized by depigmentation of skin that results from the loss of pigment-producing cells known as melanocytes. Over-activity of the JAK signaling pathway is believed to drive inflammation involved in the pathogenesis and progression of vitiligo. In the United States, more than 1.5 million people are diagnosed with vitiligo². The overall prevalence of the condition is estimated to be approximately

2-3 million³, with the majority of patients (approximately 85%) suffering from nonsegmental vitiligo⁴. Vitiligo can occur at any age, although many patients with vitiligo will experience initial symptoms before the age of 30⁵.

"Vitiligo is an immune-mediated disease that can be unpredictable, making it particularly difficult to treat," said David Rosmarin, M.D., Vice Chair of Research and Education, Department of Dermatology at Tufts Medical Center. "There have been no FDA-approved therapies available to date and the approval of Opzelura therefore marks a significant milestone. I welcome a medical treatment that helps my patients with nonsegmental vitiligo who are interested in potentially reversing the depigmentation caused by their disease."

In September 2021, Opzelura was approved by the FDA for the topical short-term and non-continuous chronic treatment of mild to moderate atopic dermatitis (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.

Incyte is committed to supporting patients and removing barriers to access medicines. Eligible patients in the United States who are prescribed Opzelura have access to IncyteCARES (Connecting to Access, Reimbursement, Education and Support), a program offering patient support, including financial assistance and ongoing education and resources to eligible patients. For more information about IncyteCARES, please visit www.incytecares.com or call 1-800-583-6964, Monday through Friday, from 8 a.m. to 8 p.m. EDT.

Conference Call Information

Incyte will host an analyst and investor conference call and webcast on July 19, 2022 at 8:00 a.m. EDT. The live and archived webcast will be available via Investor.incyte.com.

To access the conference call, please dial 877-407-3042 for domestic callers or 201-389-0864 for international callers. When prompted, provide the conference identification number, 13730931.

If you are unable to participate, a replay of the conference call will be available for 30 days. The replay dial-in number for the United States is 877-660-6853 and the dial-in number for international callers is 201-612-7415. To access the replay you will need the conference identification number, 13730931.

The live webcast with slides can be accessed at Investor.Incyte.com and will be available for replay for ninety days.

About TRuE-V

The TRuE-V clinical trial program includes two Phase 3 studies, TRuE-V1 ([NCT04052425](https://clinicaltrials.gov/ct2/show/study/NCT04052425)) and TRuE-V2 ([NCT04057573](https://clinicaltrials.gov/ct2/show/study/NCT04057573)), evaluating the safety and efficacy of ruxolitinib cream in patients with vitiligo. Each study enrolled approximately 300 patients (age ≥12 years) who have been diagnosed with nonsegmental vitiligo.

About Opzelura™ (ruxolitinib) Cream 1.5%

Opzelura, a novel cream formulation of Incyte's selective JAK1/JAK2 inhibitor ruxolitinib, is the first and only topical JAK inhibitor approved for use in the United States, indicated for the topical treatment of nonsegmental vitiligo in adult and pediatric patients 12 years of age and older and the topical short-term and non-continuous chronic treatment of mild to moderate atopic dermatitis (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. Use of Opzelura in combination with therapeutic biologics, other JAK inhibitors, or potent immunosuppressants, such as azathioprine or cyclosporine, is not recommended.

In October 2021, Incyte announced the validation of the European Marketing Authorization Application (MAA) for ruxolitinib cream as a potential treatment for adolescents and adults (age >12 years) with nonsegmental vitiligo with facial involvement.

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

Opzelura is a trademark of Incyte.

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). If needed, your healthcare provider will 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.
- 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 treated for atopic dermatitis include: common cold (nasopharyngitis), diarrhea, bronchitis, ear infection, increase in a type of white blood cell (eosinophil) count, hives, inflamed hair pores (folliculitis), swelling of the tonsils (tonsillitis), and runny nose (rhinorrhea).

The most common side effects of OPZELURA in people treated for nonsegmental vitiligo include: acne at the application site, itching at the application site, common cold (nasopharyngitis), headache, urinary tract infection, redness at the application site, and fever.

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.

INDICATIONS AND USAGE

OPZELURA is a prescription medicine used on the skin (topical) for:

- short-term and non-continuous chronic treatment of mild to moderate eczema (atopic dermatitis) in non-immunocompromised adults and children 12 years of age and older whose disease is not well controlled with topical prescription therapies or when those therapies are not recommended
- the treatment of a type of vitiligo called nonsegmental vitiligo in adults and children 12 years of age and older

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 12 years of age with atopic dermatitis or nonsegmental vitiligo.

About Incyte Dermatology

Incyte's science-first approach and expertise in immunology has formed the foundation of the company. In Dermatology, the Company's research and development efforts are focused on leveraging our knowledge of the JAK-STAT pathway to identify and develop topical and oral therapies with the potential to modulate immune pathways driving uncontrolled inflammation and help restore normal immune function.

Currently, Incyte is exploring the potential of JAK inhibition for additional immune-mediated dermatologic conditions with a high unmet medical need, including hidradenitis suppurativa. To learn more, visit the [Dermatology section of Incyte.com](#).

About Incyte

Incyte is a Wilmington, Delaware-based, global biopharmaceutical company focused on finding solutions for serious unmet medical needs through the discovery, development and commercialization of proprietary therapeutics. For additional information on Incyte, please visit [Incyte.com](#) and follow [@Incyte](#).

Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding whether and when Opzelura might provide a successful treatment option for patients with vitiligo, Incyte's TRuE-V clinical program and Incyte's Dermatology program generally, contain predictions, estimates and other forward-looking statements.

These forward-looking statements are based on the Company's current expectations and 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; the effects of the COVID-19 pandemic and measures to address the pandemic on the Company's clinical trials, supply chain, other third-party providers and development and discovery operations; determinations made by the FDA and other regulatory authorities; the efficacy or safety of the Company's products; the acceptance of the Company's products in the marketplace; market competition; sales, marketing, manufacturing and distribution requirements; and other risks detailed from time to time in the Company's reports filed with the Securities and Exchange Commission, including its annual report and its quarterly report on Form 10-Q for the quarter ended March 31, 2022. The Company disclaims any intent or obligation to update these forward-looking statements.

1Opzelura Prescribing Information. Wilmington, DE. Incyte Corporation.

2Bergqvist C, Ezzedine K. Vitiligo: A Review. *Dermatology* 2020;236:571-592.

3Gandhi K, et al. Prevalence of vitiligo among adults in the United States. *JAMA Dermatol.* 2022;158(1):43-50.

4Ezzedine K, et al. Seminar: Vitiligo. *Lancet* 2015;386:74-84.

5Frisoli M, et al. Vitiligo: mechanisms of pathogenesis and treatment. *Annu. Rev. Immunol.* 2020;38(1):621-648.

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