Incyte and Syndax Announce U.S. Food and Drug Administration (FDA) Approval of Niktimvo™ (axatilimab-csfr) 9 mg and 22 mg Vial Sizes

January 15, 2025

– U.S. launch expected in early February –

– Niktimvo is the first and only approved treatment for chronic GVHD that targets CSF-1R to reduce the drivers of inflammation and fibrosis –

– Pivotal data from the AGAVE-201 trial supporting FDA approval show treatment with Niktimvo resulted in durable responses across all organs studied and patient subgroups –

WILMINGTON, Del. and WALTHAM, Mass., Jan. 15, 2025 /PRNewswire/ -- Incyte (Nasdaq:INCY) and Syndax Pharmaceuticals (Nasdaq:SNDX) today announced that the U.S. Food and Drug Administration (FDA) has approved Niktimvo™ (axatilimab-csfr) in 9 mg and 22 mg vial sizes. The Companies expect product to be available for order in the U.S. in early February. Niktimvo is approved for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs). Niktimvo is the first and only FDA-approved prescription treatment for chronic GVHD that targets CSF-1R to reduce the drivers of inflammation and fibrosis.

"We are thrilled to build on our strong commitment to the GVHD community with the U.S. launch of Niktimvo, a first-in-class therapeutic agent that has demonstrated remarkable responses in patients with chronic GVHD whose response was suboptimal after at least two prior lines of systemic therapy," said Hervé Hoppenot, Chief Executive Officer, Incyte. "Our deep understanding of chronic GVHD and our connections in the clinical community will support a successful launch, in partnership with Syndax, of this important medicine for patients."

Niktimvo was approved by the FDA on August 14, 2024. The approval was based on positive data from the global AGAVE-201 trial, which were published in the *New England Journal of Medicine* in September 2024.[1] The trial met the primary endpoint across all cohorts receiving Niktimvo with 75% of patients who received 0.3 mg/kg every two weeks achieving a response at six months of treatment (N=79).

"As the first and only FDA-approved anti-CSF-1R antibody targeting the drivers of inflammation and fibrosis in chronic GVHD, Niktimvo represents a major breakthrough for patient care," said Michael Metzger, Chief Executive Officer, Syndax. "Together with Incyte, we look forward to executing a robust commercial launch and advancing the treatment paradigm for patients with chronic GVHD who have progressed after at least two lines of systemic therapy."

Serious adverse reactions occurred in 44% of patients who received Niktimvo (N=79). Serious adverse reactions in > 2 patients included infection (pathogen unspecified) (14%), viral infection (14%), and respiratory failure (5.1%). Permanent discontinuation of Niktimvo due to an adverse reaction occurred in 10% of patients and dose reduction due to adverse reaction occurred in 8% of patients. Dose interruptions due to an adverse reaction occurred in 44% of patients. The adverse reactions leading to dose interruption in >2 patients were viral infection, infection (pathogen unspecified), bacterial infection, musculoskeletal pain and pyrexia.

The most common (≥15%) adverse reactions, including laboratory abnormalities, were increased aspartate aminotransferase (AST), infection (pathogen unspecified), increased alanine aminotransferase (ALT), decreased phosphate, decreased hemoglobin, viral infection, increased gamma glutamyl transferase (GGT), musculoskeletal pain, increased lipase, fatigue, increased amylase, increased calcium, increased creatine phosphokinase (CPK), increased alkaline phosphatase (ALP), nausea, headache, diarrhea, cough, bacterial infection, pyrexia and dyspnea.

The approved dose of Niktimvo for adults and pediatric patients weighing at least 40 kg is 0.3 mg/kg, up to a maximum dose of 35 mg, as an intravenous infusion over 30 minutes every two weeks. Niktimvo will be available for healthcare providers to order through a network of specialty distributors in both 9 mg vial and 22 mg vial sizes to facilitate patient dosing.

Incyte and Syndax are committed to supporting patients and removing barriers to ensure access to Niktimvo. Eligible patients in the U.S. who are prescribed Niktimvo have access to IncyteCARES (Connecting to Access, Reimbursement, Education and Support), a comprehensive program offering personalized patient support, including financial assistance for eligible patients and ongoing education and additional resources. More information about IncyteCARES is available by visiting http://www.incytecares.com or calling 1-855-452-5234.

On August 30, 2024, axatilimab-csfr (Niktimvo) was added to the latest NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) as a category 2A recommendation for the treatment of chronic GVHD after the failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.[2] Treatments are classified as category 2A when there is uniform NCCN consensus that the intervention is appropriate, based on lower-level evidence. The updated NCCN guidelines are available at www.nccn.org.

In the U.S., Incyte and Syndax are co-commercializing Niktimvo. Incyte has exclusive commercialization rights for Niktimvo outside of the U.S.

About Chronic Graft-Versus-Host Disease (GVHD)

Chronic GVHD is a serious condition that can occur after an allogeneic stem cell transplant (the transfer of stem cells from a donor) in which the donated cells initiate an immune response and attack the transplant recipient's organs. Chronic GVHD is a leading cause of significant morbidity and mortality after an allogeneic stem cell transplant and is estimated to develop in approximately 42% of transplant recipients, affecting approximately 17,000 patients in the U.S.[3] Of those patients who develop chronic GVHD, nearly 50% require at least three lines of treatment, emphasizing the need for additional effective treatment options.[4]

About AGAVE-201

The global AGAVE-201 dose-ranging trial evaluated the efficacy, safety, and tolerability of axatilimab in 241 adult and pediatric patients with recurrent or refractory active chronic GVHD (GVHD) whose disease had progressed after two or more prior therapies. Patients were randomized to one of three treatment groups that investigated a distinct dose of axatilimab administered at 0.3 mg/kg every two weeks, 1.0 mg/kg every two weeks or 3.0 mg/kg every four weeks. The trial's primary endpoint was the proportion of patients in each dose group who achieved an objective response as defined by 2014 NIH Consensus Criteria for chronic GVHD by cycle 7 day 1. Secondary endpoints included duration of response, percent reduction in daily steroid dose, organ specific response rates and validated quality-of-life assessments using the Modified Lee Symptom Scale.

For more information about AGAVE-201, visit https://www.clinicaltrials.gov/study/NCT04710576.

About Niktimvo™ (axatilimab-csfr)

Niktimvo (axatilimab-csfr) is a first-in-class colony stimulating factor-1 receptor (CSF-1R)-blocking antibody approved for use in the U.S. for the treatment of chronic graft-versus-host disease (GVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg (88.2 lbs).

In 2016, Syndax licensed exclusive worldwide rights to develop and commercialize axatilimab from UCB. In September 2021, Syndax and Incyte entered into an exclusive worldwide co-development and co-commercialization license agreement for axatilimab in chronic GVHD and any future indications.

Axatilimab is being studied in frontline combination trials in chronic GVHD – a Phase 2 combination trial with ruxolitinib (NCT06388564) and a Phase 3 combination trial with steroids (NCT06585774) are underway. Axatilimab is also being studied in an ongoing Phase 2 trial in patients with idiopathic pulmonary fibrosis (NCT06132256).

Niktimvo is a trademark of Incyte.

All other trademarks are the property of their respective owners.

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS
Infusion-Related Reactions
Niktimvo™ (axatilimab-csfr) can cause infusion-related reactions. Infusion-related reactions, including hypersensitivity reactions, occurred in 18% of patients who received Niktimvo in the clinical trial (AGAVE-201), with Grade 3 or 4 reactions in 1.3%.

Premedicate with an antihistamine and an antipyretic for patients who have previously experienced an infusion-related reaction to Niktimvo. Monitor patients for signs and symptoms of infusion-related reactions, including fever, chills, rash, flushing, dyspnea, and hypertension. Interrupt or slow the rate of infusion or permanently discontinue Niktimvo based on severity of the reaction.

Embryo-Fetal Toxicity
Based on its mechanism of action, Niktimvo may cause fetal harm when administered to a pregnant woman. Advise pregnant women of the potential risk to the fetus. Advise females of reproductive potential to use effective contraception during treatment with Niktimvo and for 30 days after the last dose.

ADVERSE REACTIONS
Serious adverse reactions occurred in 44% of patients who received Niktimvo (N=79). Serious adverse reactions in >2 patients included infection (pathogen unspecified) (14%), viral infection (14%) and respiratory failure (5.1%). Permanent discontinuation of Niktimvo due to an adverse reaction occurred in 10% of patients and dose reduction due to adverse reaction occurred in 8% of patients. Dose interruptions due to an adverse reaction occurred in 44% of patients. The adverse reactions leading to dose interruption in >2 patients were viral infection, infection (pathogen unspecified), bacterial infection, musculoskeletal pain, and pyrexia.

The most common (≥15%) adverse reactions, including laboratory abnormalities, were increased aspartate aminotransferase (AST), infection (pathogen unspecified), increased alanine aminotransferase (ALT), decreased phosphate, decreased hemoglobin, viral infection, increased gamma glutamyl transferase (GGT), musculoskeletal pain, increased lipase, fatigue, increased amylase, increased calcium, increased creatine phosphokinase (CPK), increased alkaline phosphatase (ALP), nausea, headache, diarrhea, cough, bacterial infection, pyrexia, and dyspnea.

Clinically relevant adverse reactions in <10% of patients who received Niktimvo included:

- *Eye disorders:* periorbital edema
- *Skin and subcutaneous skin disorders:* pruritus
- *Vascular disorders:* hypertension

Immunogenicity: Anti-Drug Antibody–Associated Adverse Reactions
Across treatment arms in patients with cGVHD who received Niktimvo in clinical trials, among the patients who developed anti-drug antibodies (ADAs), hypersensitivity reactions occurred in 26% (13/50) of patients with neutralizing antibodies (NAb) and in 4% (2/45) of those without NAb.

USE IN SPECIFIC POPULATIONS
Lactation
Because of the potential for serious adverse reactions in a breastfed child, advise women not to breastfeed during treatment and for 30 days after the last dose of Niktimvo.

Females and Males of Reproductive Potential
Pregnancy Testing
Verify pregnancy status in females of reproductive potential prior to initiating Niktimvo.

Contraception

Females
Advise females of reproductive potential to use effective contraception during treatment with Niktimvo and for 30 days after the last dose of Niktimvo.

DOSAGE AND ADMINISTRATION
Dosage Modifications for Adverse Reactions
Monitor aspartate aminotransferase (AST), alanine aminotransferase (ALT), alkaline phosphatase (ALP), creatine phosphokinase (CPK), amylase, and lipase prior to the start of Niktimvo therapy, every 2 weeks for the first month, and every 1 to 2 months thereafter until abnormalities are resolved. See Table 1 in the Prescribing Information for more recommendations.

Please see the full Prescribing Information for Niktimvo.

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.

About Syndax

Syndax Pharmaceuticals is a commercial-stage biopharmaceutical company developing an innovative pipeline of cancer therapies. Highlights of the Company's pipeline include Revuforj® (revumenib), an FDA-approved menin inhibitor, and Niktimvo™ (axatilimab-csfr), an FDA-approved monoclonal antibody that blocks the colony stimulating factor 1 (CSF-1) receptor. Fueled by our commitment to reimagining cancer care, Syndax is working to unlock the full potential of its pipeline and is conducting several clinical trials across the continuum of treatment. For more information, please visit www.syndax.com/ or follow the Company on X (formerly Twitter) and LinkedIn.

Incyte Forward-Looking Statements

Except for the historical information set forth herein, the matters set forth in this press release, including statements regarding whether Niktimvo might provide a successful treatment option for patients with chronic GVHD: expectations regarding the launch of Niktimvo; and the potential for axatilimab to treat additional conditions, contain predictions, estimates and other forward-looking statements.

These forward-looking statements are based on Incyte'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; determinations made by the U.S. FDA and other regulatory authorities outside of the U.S.; the efficacy or safety of Incyte and its partners' products; the acceptance of Incyte and its partners' products in the marketplace; market competition; sales, marketing, manufacturing and distribution requirements; and other risks detailed from time to time in Incyte's reports filed with the Securities and Exchange Commission, including its annual report on form 10-Kand its report on form 10-Q for the quarter ended September 30, 2024. Incyte disclaims any intent or obligation to update these forward-looking statements.

Syndax Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995. Words such as "may," "will," "expect," "plan," "anticipate," "estimate," "intend," "believe" and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements. These forward-looking statements are based on Syndax's expectations and assumptions as of the date of this press release. Each of these forward-looking statements involves risks and uncertainties. Actual results may differ materially from these forward-looking statements. Forward-looking statements contained in this press release include, but are not limited to, statements about the progress, timing, clinical development and scope of clinical trials, the reporting of clinical data for Syndax's product candidates, the acceptance of Syndax and its partners' products in the marketplace, sales, marketing, manufacturing and distribution requirements, and the potential use of our product candidates to treat various cancer indications and fibrotic diseases. Many factors may cause differences between current expectations and actual results, including: unexpected safety or efficacy data observed during preclinical or clinical trials; clinical trial site activation or enrollment rates that are lower than expected; changes to Niktimvo's commercial availability; changes in expected or existing competition; changes in the regulatory environment; failure of Syndax's collaborators to support or advance collaborations or product candidates; and unexpected litigation or other disputes. Other factors that may cause Syndax's actual results to differ from those expressed or implied in the forward-looking statements in this press release are discussed in Syndax's filings with the U.S. Securities and Exchange Commission, including the "Risk Factors" sections contained therein. Except as required by law, Syndax assumes no obligation to update any forward-looking statements contained herein to reflect any change in expectations, even as new information becomes available.

Incyte Contacts:

Media	Investors
media@incyte.com	ir@incyte.com

Syndax Contact:
Sharon Klahre
sklahre@syndax.com
Tel 781.684.9827

[1] Wolff D, et al. Axatilimab in Recurrent or Refractory Chronic Graft-versus-Host Disease. *N Engl J Med* 2024;391:1002-14. DOI: 10.1056/NEJMoa2401537.
[2] NCCN Clinical Practice Guidelines in Oncology (NCCN Guidelines®) for Hematopoietic Cell Transplantation (HCT). Version 2.2024 – August 30,

2024. NCCN makes no warranties of any kind whatsoever regarding their content, use or application and disclaims any responsibility for their application or use in any way.

[3] Data on file.

[4] Bachier, CR. et al. ASH annual meeting 2019; abstract #2109 Epidemiology and Real-World Treatment of Chronic Graft-Versus-Host Disease Post Allogeneic Hematopoietic Cell Transplantation: A U.S. Claims Analysis.

SNDX-G

View original content:https://www.prnewswire.com/news-releases/incyte-and-syndax-announce-us-food-and-drug-administration-fda-approval-of-niktimvo-axatilimab-csfr-9-mg-and-22-mg-vial-sizes-302352411.html

SOURCE Syndax Pharmaceuticals; Incyte