

Make a Change in cGVHD Treatment Strategy and Provide Rapid, Durable Responses

Niktimvo™ (axatilimab-csfr) is indicated for the treatment of chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.



Learn more about this innovative treatment for cGVHD

IMPORTANT SAFETY INFORMATION

WARNINGS AND PRECAUTIONS

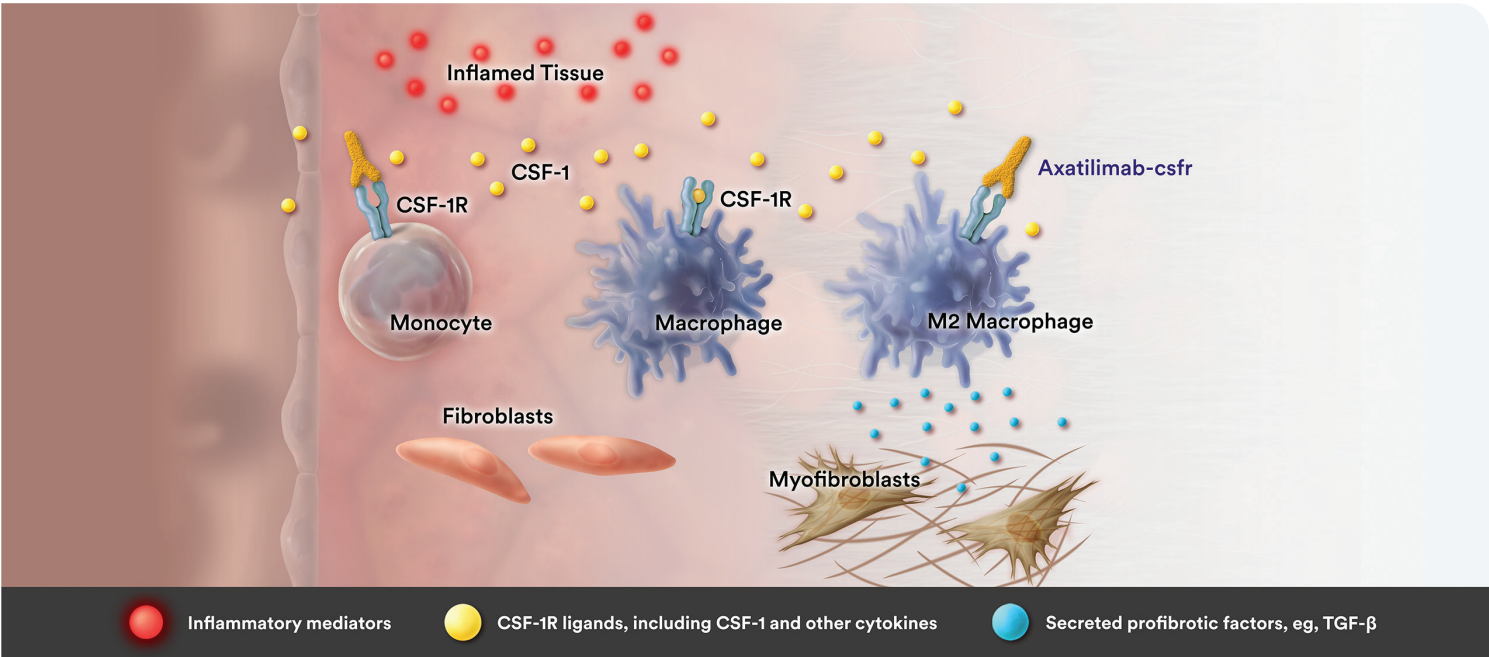
Infusion-Related Reactions

Niktimvo 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.

Please see related and other Important Safety Information on page 9. Please see [Full Prescribing Information](#) for Niktimvo.

Niktimvo Works Differently: A High-Affinity Monoclonal Antibody Targeting CSF-1R to Potentially Address Both Inflammation and Fibrosis¹⁻³



About Niktimvo in cGVHD

Niktimvo reduces nonclassical monocytes^{1,2}

- Niktimvo modulates the monocyte profile in peripheral blood by reducing the prevalence of nonclassical CD14⁺CD16⁺⁺ monocytes

Niktimvo reduces skin macrophage density and profibrotic cytokines²

- Niktimvo has been shown to decrease skin CSF-1R⁺ macrophage infiltration after 2 cycles of therapy
- Response to Niktimvo is associated with a decrease in key profibrotic macrophage cytokines

Niktimvo works to inhibit the inflammatory process²

- Suppresses the activity of monocytes and macrophages, reducing proinflammatory cytokine secretion

Niktimvo works to inhibit the fibrotic process²

- Reduces monocyte levels, suppressing their differentiation into profibrotic macrophages
- Inhibits macrophages, disrupting the TGF-β-mediated development of fibrosis

Correlation with clinical effect has not been established.

CSF-1, colony-stimulating factor-1; CSF-1R, CSF-1 receptor; TGF-β, transforming growth factor beta.

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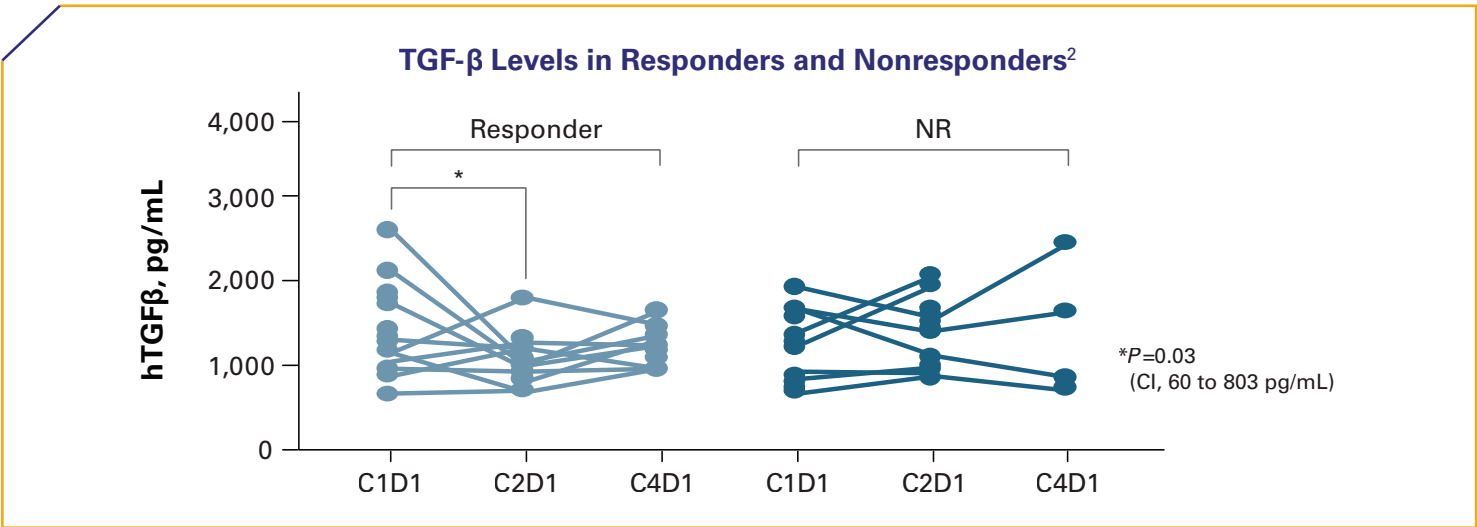
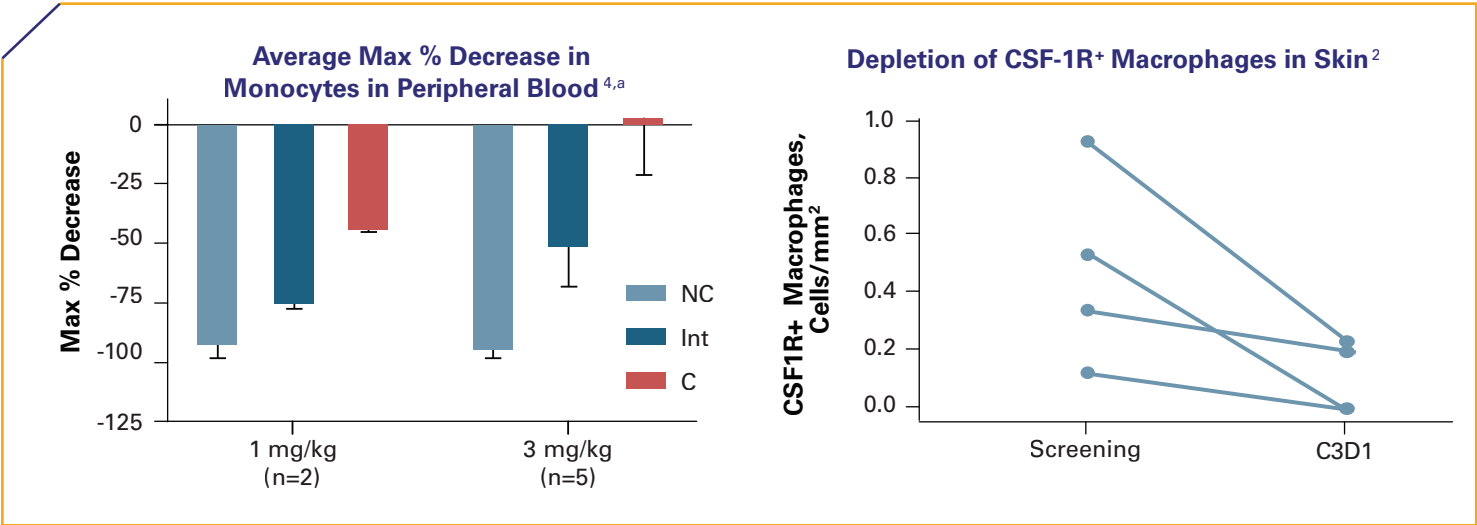
Embryo-Fetal Toxicity

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.

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Correlative Studies From a Phase 1/2 Study Assessed the Antifibrotic Activity of Niktimvo

40 patients had undergone allogeneic HSCT and had active cGVHD
Phase 1: Dose escalation (n=17) | **Phase 2:** Expansion with 1.0 mg/kg Q2W dose (n=23)²



Correlation with clinical effect has not been established.

^aMax decrease defined as the greatest reduction at any time in the first cycle.
C, classical (CD14⁺CD16⁻); C1D1, cycle 1 day 1; C2D1, cycle 2 day 1; C3D1, cycle 3 day 1; C4D1, cycle 4 day 1; CI, confidence interval; HSCT, hematopoietic stem cell transplant; hTGF-β, human transforming growth factor beta; Int, intermediate (CD14⁺CD16⁺); NC, nonclassical (CD14⁺CD16⁺⁺); NR, nonresponder; Q2W, every 2 weeks.

IMPORTANT SAFETY INFORMATION

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.

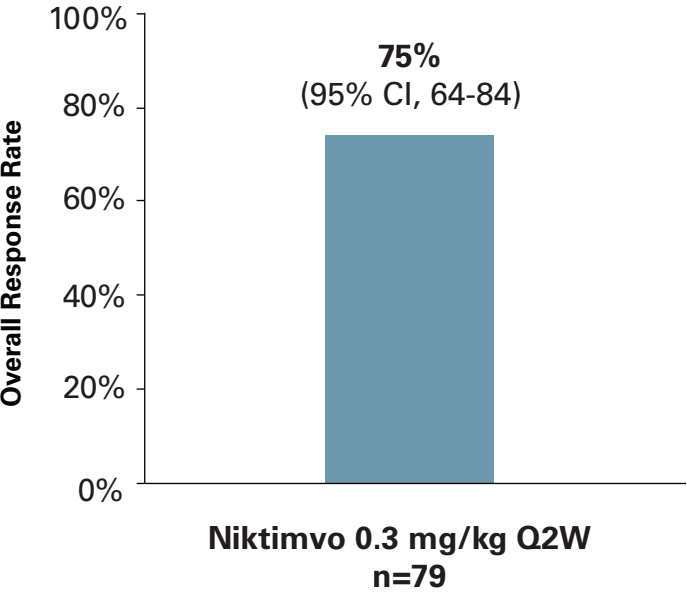
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AGAVE-201 Was a Randomized, Open-Label, International, Multicenter Phase 2 Trial^{1,5,6}

Niktimvo was approved by the FDA for cGVHD after 2 lines of systemic therapy in adults and pediatric patients weighing at least 40 kg, based on positive results from the AGAVE-201 trial



AGAVE-201 Primary Endpoint: ORR by cycle 7, day1^{1,†,‡}



High and Rapid Response Rates Were Observed in AGAVE-201

- Median time to first response in patients who had an overall response was **1.5 months**¹

*Patients were randomized to 1 of 3 treatment groups that investigated a distinct dose of Niktimvo administered at 0.3 mg/kg Q2W, 1.0 mg/kg Q2W, and 3.0 mg/kg Q4W until disease progression, lack of response by 9 months, or unacceptable toxicity.^{1,6}

[†] ORR was defined as the proportion of patients who achieved a complete or partial response as defined by 2014 NIH consensus criteria. The ORR was assessed by the number of participants with objective response by cycle 7 (upon the first six 28-day cycles), day 1. Range of time to first response was 0.9 to 5.1 months.¹

[‡] Statistical significance was achieved if the lower bound of the 95% CI exceeded 30%.⁶

FDA, US Food and Drug Administration; IV, intravenously; NIH, National Institutes of Health; ORR, overall response rate; Q4W, every 4 weeks.

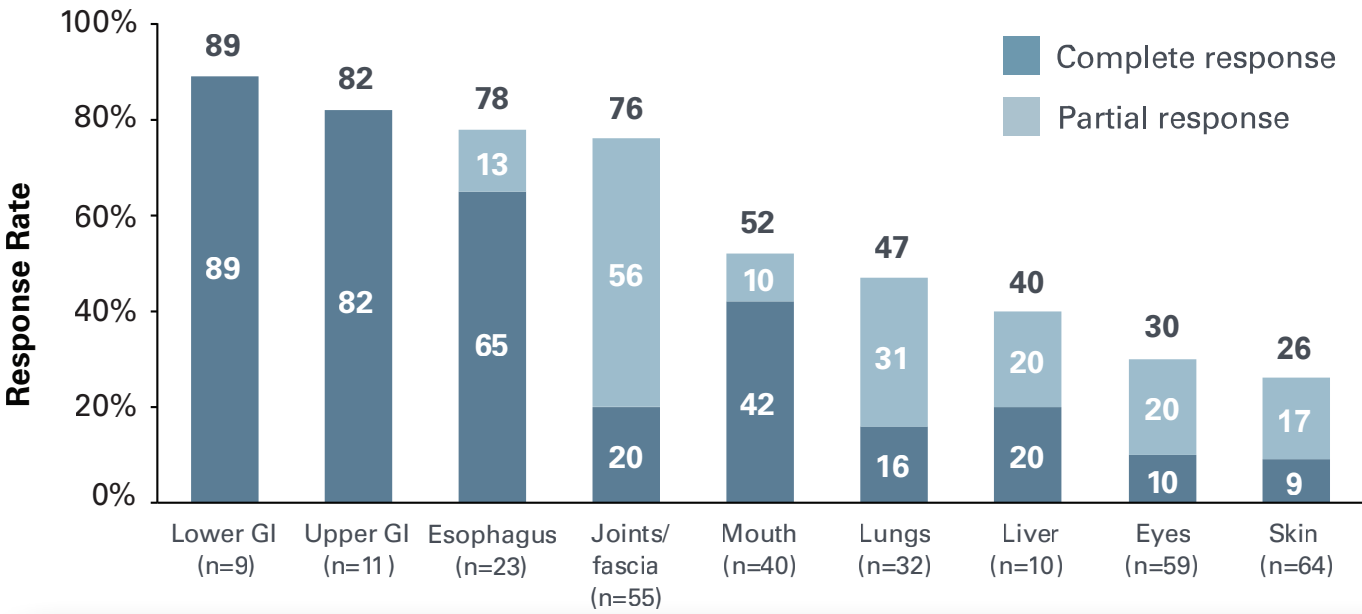
IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

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.

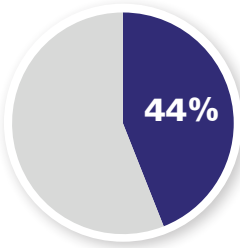
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AGAVE-201 Secondary Endpoint: ORR by Organ Affected^{6,*,†}

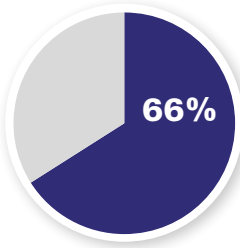


Niktimvo Broadly Demonstrated Disease Control[‡] Across All Organs Studied, Even Those With Difficult-to-Treat Manifestations

The majority of patients with skin involvement had deep sclerotic skin lesions at baseline



44% of patients had reduction in BSA affected by sclerosis



66% of affected patients had improvement in skin- and joint-tightening severity

*Organ-specific responses were evaluated using baseline cGVHD severity as a reference.⁷

[†] ORR by organ affected was a prespecified secondary endpoint that was not powered for statistical significance and is therefore considered exploratory.

[‡] Disease control is defined as complete or partial response to treatment. BSA, body surface area; GI, gastrointestinal.

IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS

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

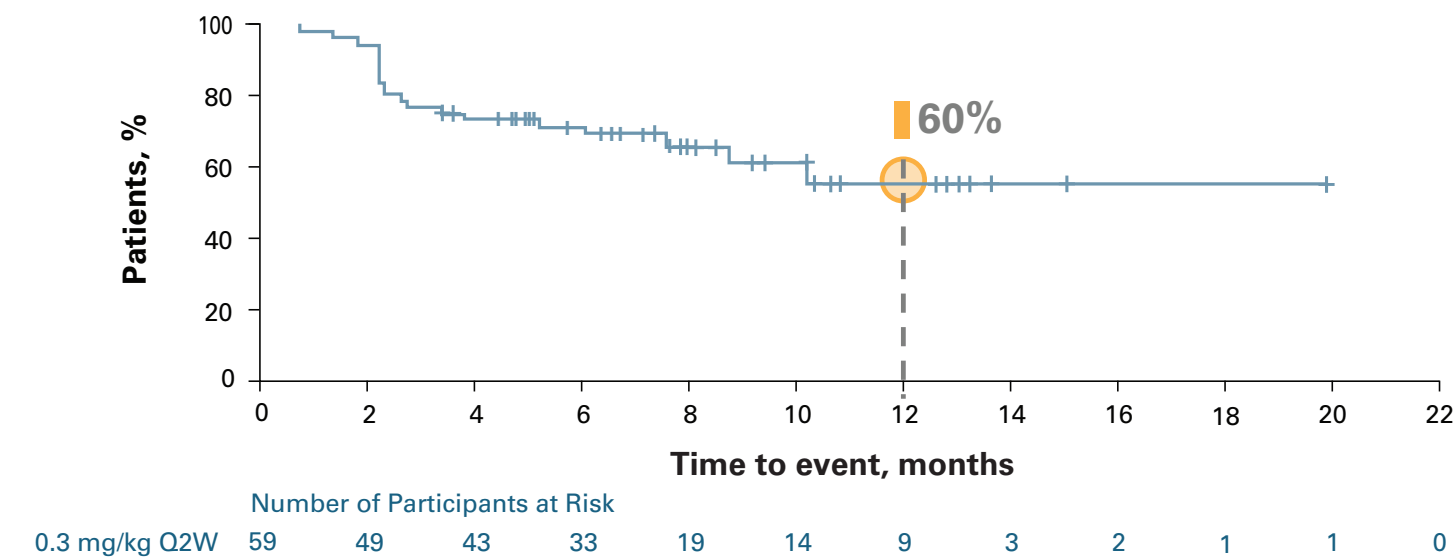
- Eye disorders:* periorbital edema
- Skin and subcutaneous skin disorders:* pruritus
- Vascular disorders:* hypertension

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.

Please see related and other Important Safety Information on page 9. Please see [Full Prescribing Information](#) for Niktimvo.

AGAVE-201 Secondary Endpoint: Duration of Response^{1,7,*†}



Niktimvo Responses Were Durable, With 60% of Patients Maintaining Responses for More Than 1 Year^{1,7}

- The median duration of response, calculated from first response to progression, death, or new systemic therapies for cGVHD, was 1.9 months (95% CI, 1.6-3.5)¹
- In patients who achieved response, no death or new systemic therapy initiation occurred in 60% (95% CI, 43-74) of patients for at least 12 months since response¹

*Sensitivity analysis with duration of response defined as time from response of complete response or partial response until start of new anti-GVHD systemic therapy or death from any cause, whichever was earlier. Patients who had not started new therapy and were still alive were censored at the last contact date.⁷

[†] DOR was a prespecified secondary endpoint that was not powered for statistical significance and is therefore considered exploratory.⁵
DOR, duration of response.

IMPORTANT SAFETY INFORMATION

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.

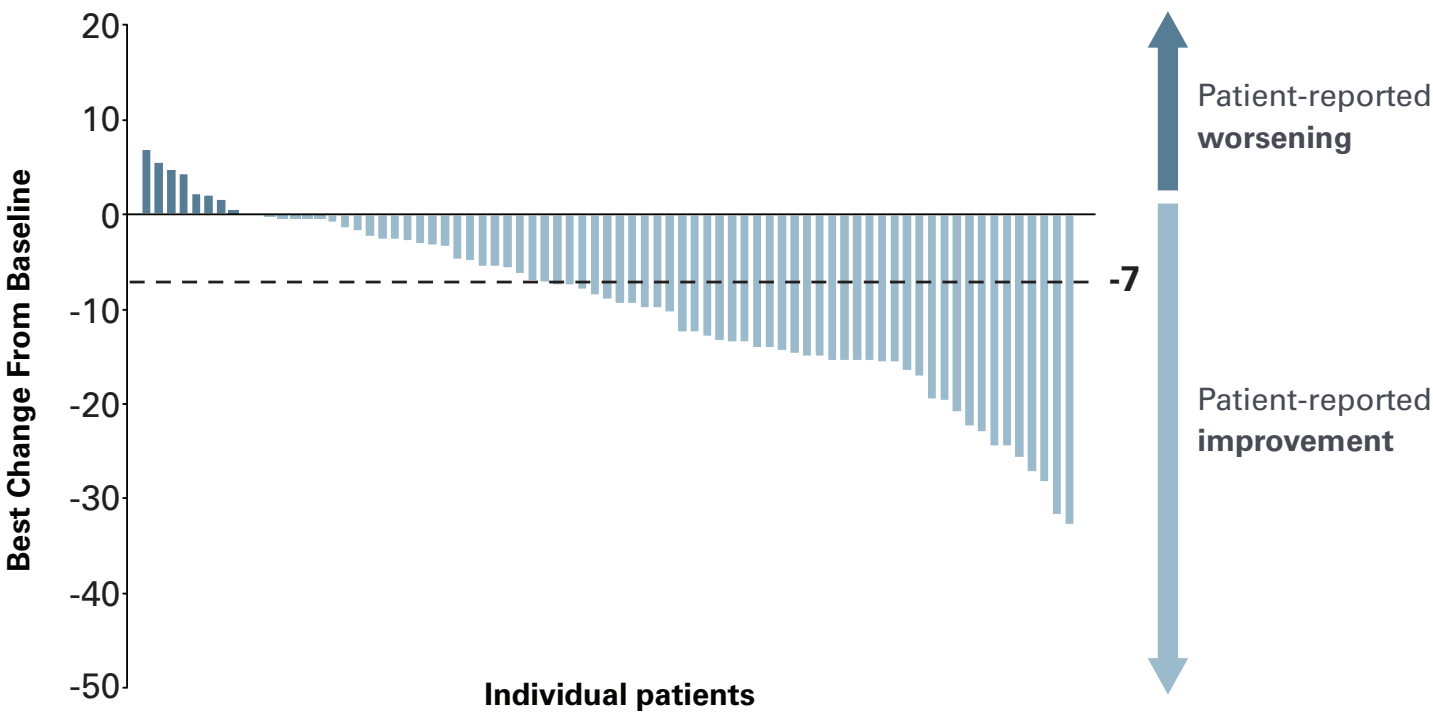
Contraception

Females

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

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AGAVE-201 Exploratory Endpoint: Patient-Reported Improvement in mLSS^{6,*†}



56% of Patients Had a Patient-Reported Improvement of ≥7 Points in mLSS¹

- Median time to ≥7-point improvement on the mLSS was 1.5 months⁶
- ORR results were supported by exploratory analyses of patient-reported symptom bother, which showed at least a 7-point decrease in mLSS through cycle 7, day 1 in 56% of patients (95% CI, 44-67)¹

*The mLSS is a health-related tool to assess and measure symptoms validated by the NIH for use in clinical trials of patients with cGVHD.⁸

[†] mLSS is an exploratory endpoint that was not statistically powered.⁶

mLSS, modified Lee cGVHD symptom scale.

IMPORTANT SAFETY INFORMATION

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.

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Niktimvo Safety Profile¹

Adverse reactions in ≥10% of patients	Niktimvo 0.3 mg/kg Q2W (n=79)	
	All grades (%)	Grades 3-4 (%)
Infections and infestations		
Infection (pathogen unspecified)	57	14
Viral infection	43	15
Bacterial infection	15	8
Musculoskeletal and connective tissue disorders		
Musculoskeletal pain	35	3
General disorders and administration site conditions		
Fatigue	32	4
Pyrexia	15	1
Edema	13	1
Gastrointestinal disorders		
Nausea	23	3
Diarrhea	18	5
Nervous system disorders		
Headache	20	1
Dizziness	11	0
Respiratory, thoracic, and mediastinal disorders		
Cough	18	0
Dyspnea	15	3
Immune system disorders		
Drug hypersensitivity	13	3
Metabolism and nutrition disorders		
Decreased appetite	11	4
Vascular disorders		
Hemorrhage	11	1
Skin and subcutaneous tissue disorders		
Rash	10	0

- Selected laboratory abnormalities >30% were decreases in phosphate (51%) and hemoglobin (48%), as well as increases in AST (61%), ALT (51%), GGT (39%), lipase (34%), amylase (32%), and calcium (31%)¹
- Transient increases in serum enzymes were consistent with the known effect of CSF-1R inhibition on macrophage clearance⁷
- For further characterization of each adverse reaction type, please see below Table 2 in section 6.1 (Clinical Trial Experience) of the Full Prescribing Information for Niktimvo
- Permanent discontinuation and dose reduction due to an adverse reaction occurred in 10% and 8% of patients¹

Graded according to National Cancer Institute Common Terminology Criteria for Adverse Events (CTCAE), version 5.0.

ALT, alanine aminotransferase; AST, aspartate aminotransferase; GGT, gamma glutamyl transferase.

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- *Skin and subcutaneous skin disorders:* pruritus
- *Vascular disorders:* hypertension

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INDICATIONS AND USAGE

Niktimvo™ (axatilimab-csfr) is a colony stimulating factor-1 receptor (CSF-1R)-blocking antibody indicated for the treatment of chronic graft-versus-host disease (cGVHD) after failure of at least two prior lines of systemic therapy in adult and pediatric patients weighing at least 40 kg.

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For patients who have had a suboptimal response to T- and B-cell–modulating therapies^{1,9}

Make a Change in cGVHD Treatment Strategy and Provide Rapid, Durable Responses With Niktimvo

Delivered high and rapid response rates¹

Primary endpoint of ORR by cycle 7, day 1^{*,†}

- 75% (59/79) with Niktimvo at dose 0.3 mg/kg Q2W (95% CI, 64-84)
- Median time to first response was 1.5 months

Responses were durable^{1,7}

- 60% of patients maintained responses for >1 year

IV formulation dosing for patients weighing at least 40 kg¹

- Administer Niktimvo 0.3 mg/kg (max 35 mg) IV Q2W

Broadly demonstrated disease control[†] in all organs studied⁶

Observed response rates included

- Esophagus (78%)
- Joints/fascia (76%)
- Lungs (47%)
- Skin (27%)

*ORR was defined as the proportion of patients who achieved a complete or partial response as defined by 2014 NIH consensus criteria.

The ORR was assessed by the number of participants with objective response by cycle 7 (upon the first six 28-day cycles), day 1.¹

† Statistical significance was achieved if the lower bound of the 95% CI exceeded 30%.⁶

† Disease control is defined as complete or partial response to treatment.

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 **Niktimvo™**
(axatilimab-csfr)
50 mg/mL for injection, for intravenous use

For patients with a suboptimal response to T- and B-cell–modulating therapies¹

Know the Subtle Signs and When to Make a Change in cGVHD Treatment Strategy



JOINTS

- Joint severity is scored based on the impact on range of motion and activities of daily living²
- Consider using P-ROM in the assessment of 4 maneuvers to screen for sclerosis and fasciitis³



GI TRACT

- Weight loss and diarrhea are significant factors in diagnosing GI tract manifestations^{2,3}
- Assessments of weight, appetite, and any changes in daily behaviors are key to assessing the GI tract for signs of cGVHD^{2,3}



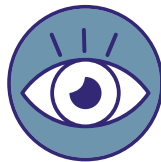
MOUTH

- Range of function is a significant contributor to scoring the severity of oral manifestations²⁻⁴
- Quantify the extent of erythema, lichenoid lesions, ulcers, and mucocoeles using the A,B,C approach²⁻⁴



GENITALS

- Genital manifestations are often associated with the appearance of oral manifestations of cGVHD²
- Be sure to include the patient's genital areas in the comprehensive skin exam and ask questions about sexual health²



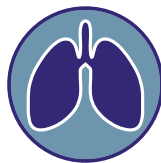
EYES

- Ocular manifestations are scored based on dry eye symptoms and new impairments of vision²
- Consider comprehensive ophthalmic evaluation by an ophthalmologist before and after allogeneic HSCT²



SKIN/HAIR/NAILS

- Scoring of skin severity is determined by degree of coverage and the presence or absence of sclerotic features²
- Consider a “Look, Feel, Move” approach to assess both superficial and deep layers of skin³



LUNGS

- Lung manifestations are scored based on both lung symptoms and lung function²
- Screenings include PFTs, laboratory tests, and coughing/wheezing²

cGVHD, chronic graft-versus-host disease; GI, gastrointestinal; HSCT, hematopoietic stem cell transplant; PFT, pulmonary function test; P-ROM, passive range of motion.

References: 1. Zeiser R, Lee SJ. Three US Food and Drug Administration–approved therapies for chronic GVHD. *Blood*. 2022;139(11):1642-1645. 2. Jagasia MH, Greinix HT, Arora M, et al. National Institutes of Health consensus development project on criteria for clinical trials in chronic graft-versus-host disease: I. The 2014 diagnosis and staging working group report. *Biol Blood Marrow Transplant*. 2015;21(3):389-401.e1. 3. Carpenter PA. How I conduct a comprehensive chronic graft-versus-host disease assessment. *Blood*. 2011;118(10):2679-2687. 4. Lee SJ, Flowers MED. Recognizing and managing chronic graft-versus-host disease. *Hematology Am Soc Hematol Educ Program*. 2008:134-141.

