NIKTIMVO™ (axatilimab-csfr) in Chronic Graft-Versus-Host Disease After Failure of ≥ 2 Prior Lines of Therapy

Prescribing Information

MSL-AXA-US-0010 09/26

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Notice

- Some information contained in this presentation may not be included in the approved Prescribing Information for NIKTIMVO. This presentation is not intended to offer recommendations for any administration, indication, dosage, or other use for NIKTIMVO in a manner inconsistent with the approved Prescribing Information

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Indications and Usage[1]

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

Please see the Full Prescribing Information, including Warnings and Precautions, and Patient Information

NIKTIMVO™ (axatilimab-csfr). Prescribing Information. Incyte Corporation; June 2026.

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Presentation Overview

SECTION 1 — Chronic GVHD Disease State

SECTION 2 — Axatilimab-csfr Clinical Efficacy and Safety in cGVHD

SECTION 3 — Axatilimab-csfr Dosage and Administration in cGVHD

SECTION 4 — Warnings, Precautions, and Use in Specific Populations

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cGVHD, chronic graft-versus-host disease.

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Chronic GVHD Disease State

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Clinical Presentation and Pathophysiology of cGVHD

- cGVHD has a characteristic clinical presentation that is distinct from that of acute GVHD[5]

Acute GVHD

- Strong inflammatory component with tissue damage[1]
- Mediated primarily by mature donor T cells[2]
- Primarily affects the skin, gut, and liver[1]

Chronic GVHD[5]

- Complex immune-mediated pathology involves T cell dysregulation, innate immune system and B cell activation, and fibroblast proliferation[2,3]
- Displays autoimmune/inflammatory and fibrotic features[1]
- Affects multiple organ sites, such as eyes, lungs, mouth, muscles, fascia, joints, skin, GI tract, and genitalia[1,4-6]

cGVHD, chronic graft-versus-host disease; HSCT, hematopoietic stem cell transplantation.

1. Blazar BR, et al. *Nat Rev Immunol*. 2012;12:443-458. 2. Cutler CS, et al. *Blood*. 2017;129(1):22-29. 3. Cooke KR, et al. *Biol Blood Marrow Transplant*. 2017;23:211-234; 4. Jagasia MH, et al. *Biol Blood Marrow Transplant*. 2015;21(3):389-401; 5. Vukic T, et al. *Croat Med J*. 2016;57(3):266-275. 6. Inamoto Y, et al. *Arthritis Rheumatol*. 2014;66(4):1044-1052.

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Chronic GVHD Epidemiology and Burden of Disease

cGVHD is a common long-term complication of allo-HSCT[1]

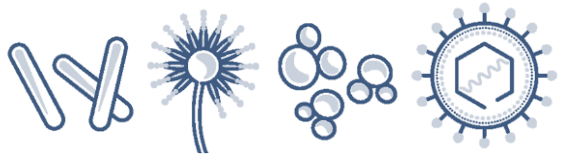
30-50%

Of allo-HSCT recipients experience cGVHD[1]

cGVHD leads to a significantly lower health-related QoL, including[2-7]

- Decreased functional status
- Inability to work or resume social roles

cGVHD is associated with significant morbidity[8-11]



cGVHD is associated with severe and frequent infections[8-10]

cGVHD leads to debilitating fibrotic organ damage that can be irreversible[11]

cGVHD is a leading cause of NRM following allo-HSCT[12]

38%

of NRM is associated with cGVHD

NRM from cGVHD is frequently associated with infection and organ failure

NRM, non-relapse mortality; QoL, quality of life; RFS, relapse-free survival.

1. Lee SJ. *Best Pract Res Clin Haematol*. 2010;4:529-535. 2. Kitko CL, et al. *Transplant Cell Ther*. 2021;27:545-557. 3. Lee SJ, et al. *Haematologica*. 2018;103:1535-1541. 4. Wong FL, et al. *Blood.* 2010;115:2508-2519. 5. Bevans M, et al. *Biol Blood Marrow Transplant*. 2017;23:538-551. 6. Yu J, et al. *Cancer Med*. 2023;12:3623-3633. 7. Kurosawa S, et al. *Biol Blood Marrow Transplant*. 2019;25:1851-1858. 8. Arai S, et al. *Biol Blood Marrow Transplant*. 2015;21:266-74. 9. Socié G, Ritz J. *Blood.* 2014;124:374-384. 10. Blau O, et al. *Int J Transplant Red Med.* 2017;3:033. 11. Hill GR, et al. *Annu Rev Immunol*. 2021 Apr 26;39:19-49. 12. deFilipp Z, et al. *Blood Adv*. 2021;5:4278-4284.

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First-Line Corticosteroid Therapy Fails in the Majority of Patients, Leading to Steroid-Refractory Chronic GVHD

50-60% of patients require additional therapy within 2 years of initial systemic therapy[1]

In clinical trials, SR cGVHD should be considered in case of:[2]

Progressive disease

1-2 weeks after treatment with ≥1 mg/kg/day of prednisone

No improvement

Stable GVHD with prednisone ≥0.5 mg/kg/d (or 1 mg/kg every other day) for 1-2 months

Steroid dependence

Inability to taper prednisone <0.25 mg/kg/day in ≥2 unsuccessful attempts separated by ≥8 weeks

SR, steroid-refractory.

1. Flowers MED, Martin PJ. *Blood.* 2015;125:606-615. 2. Schoemans HM, et al. *Bone Marrow Transplant*. 2018;53:1401-1415.

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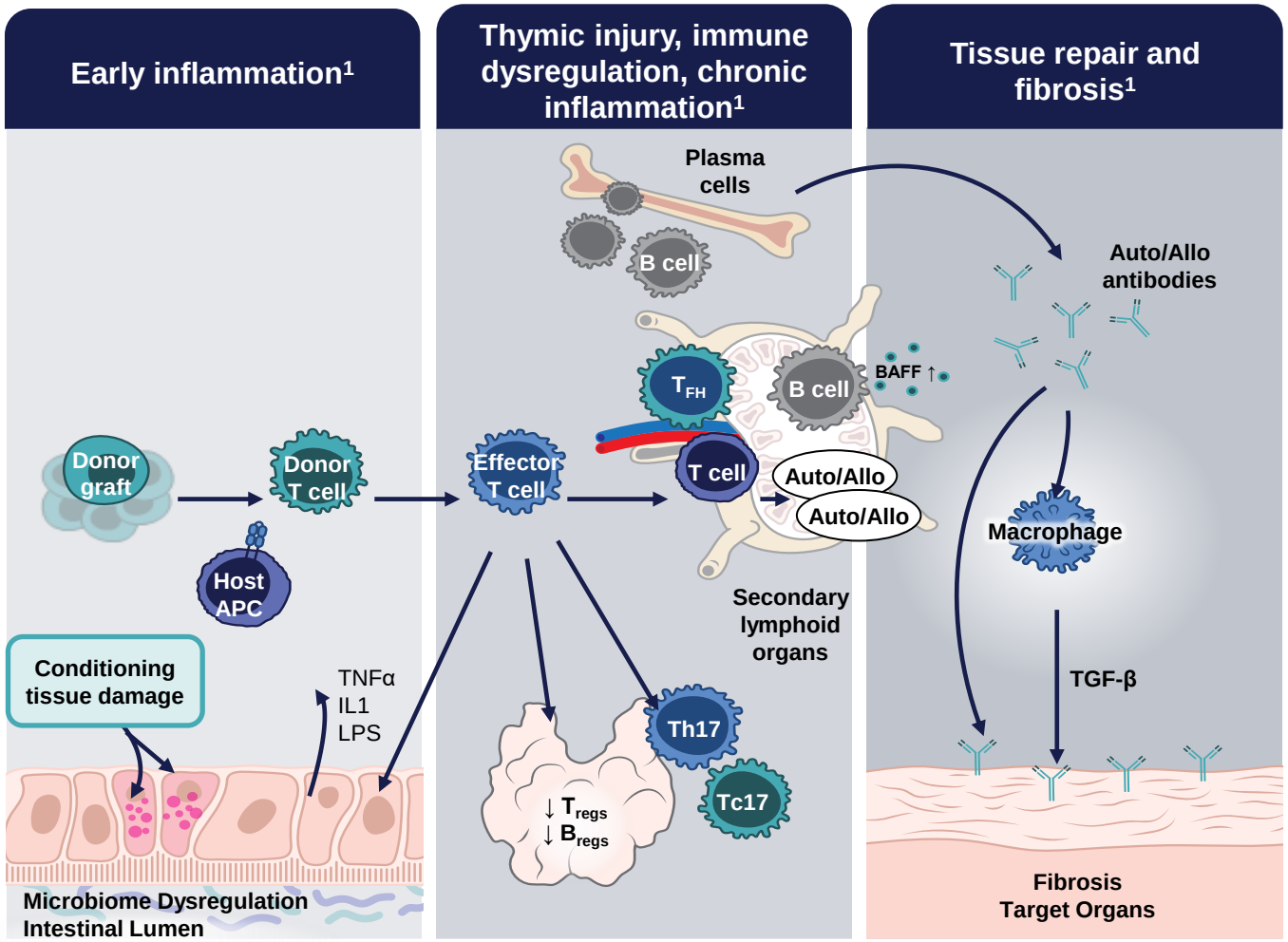
The Pathophysiology of cGVHD Occurs in Phases



Activated Macrophages

- Secrete cytokines to trigger early inflammation[2]
- Regulate tissue repair and remodeling[1,2]
- Depend on CSF-1 for development, function, and survival[1-4]
- Produce TGF-β and PDGF, resulting in fibroblast activation and cGVHD manifestations such as scleroderma or bronchiolitis obliterans[1]

Figure used with permission of American Society of Hematology (ASH) from "Updates in chronic graft-versus-host disease", Betty K. Hamilton, 2021 (1), Pages 648–654; permission conveyed through Copyright Clearance Center, Inc.

APC, antigen-presenting cell; BAFF, B cell activating factor; IL1, interleukin 1; LPS, lipopolysaccharide; PDGF, platelet-derived growth factor; Tc17, IL-17 secreting CD8 T cells; T_{FH}, T follicular helper cell; TGF-β, transforming growth factor beta; Th17, T helper 17; TNFα, tumor necrosis factor alpha.

1. Hamilton BK. *Hematology Am Soc Hematol Educ Program.* 2021;2021(1):648–654. 2. Hong YQ, et al. *World J Clin Cases*. 2020;8(10):1793-1805. 3. Alexander KA, et al. *J Clin Invest*. 2014;124(10):4266-4280. 4. Hume DA, MacDonald KP. *Blood*. 2012;119(8):1810-1820.

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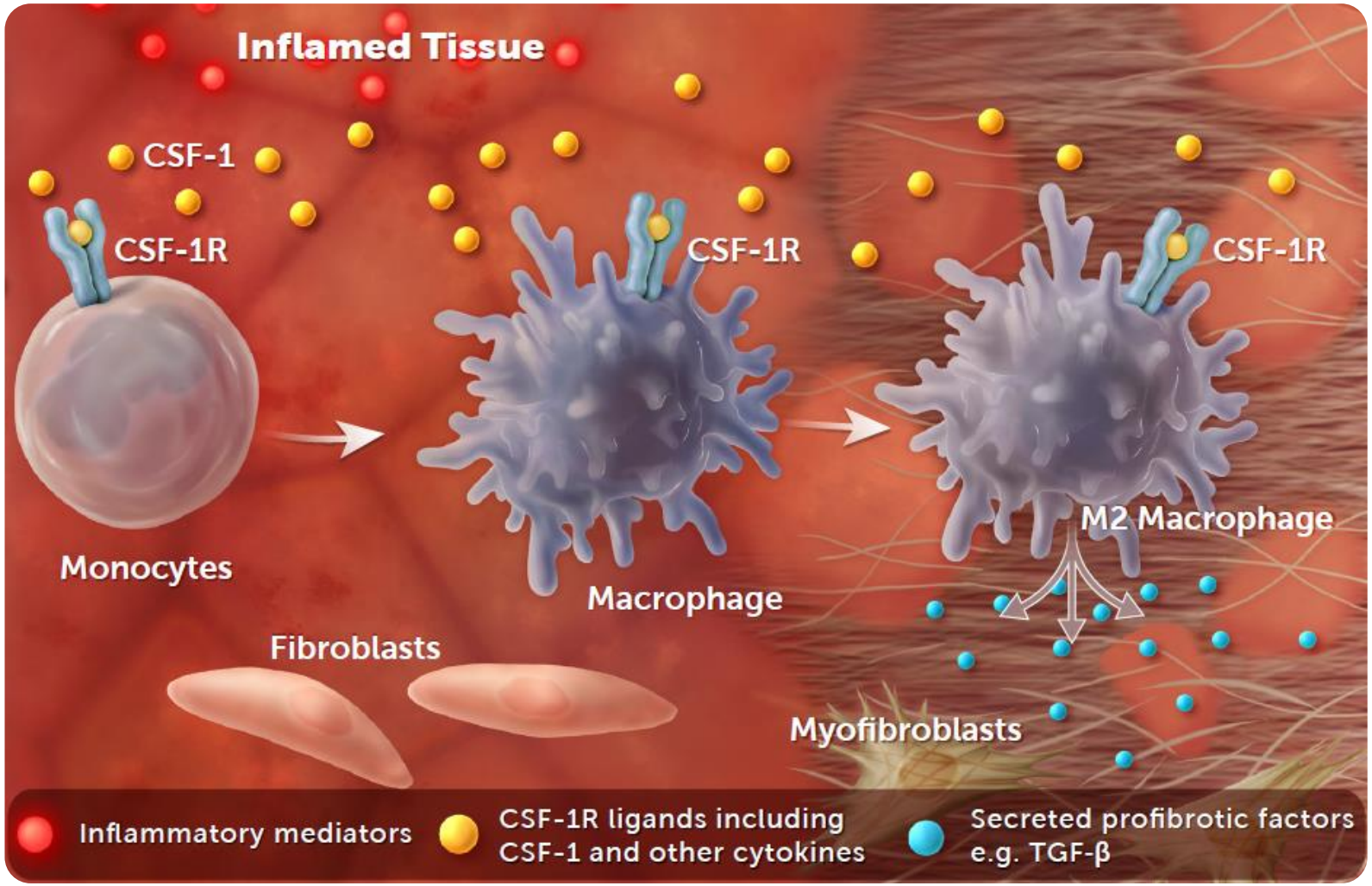
In Tissue, CSF-1R-Dependent Macrophages Promote Fibrosis and Inflammation During cGVHD



- Activated circulating monocytes are present at the onset of cGVHD and remain elevated in patients with active disease[1,6]
- Macrophages release proinflammatory cytokines, increasing the recruitment of immune cells into the tissue[1,2]
- In cGVHD, CSF-1R-dependent activated profibrotic macrophages mediate the production of TGF-β[3-5]

1. Huma DA and MacDonald KP. *Blood*. 2012;119(8):1810-1820. 2. Hong YQ, et al. *World J Clin Cases*. 2020;8(10):1793-1805. 3. MacDonald KP, et al. *Blood*. 2010;116(19):3955-3963. 4. Alexander KA, et al. *J Clin Invest*. 2014;124(10):4266-4280. 5. Banovic T, et al. *Blood*. 2005; 106(6):2206-2214; 6. Hakim FT, et al. *J Immunol*. 2016;197(9):3490-3503.

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Axatilimab-csfr Efficacy and Safety

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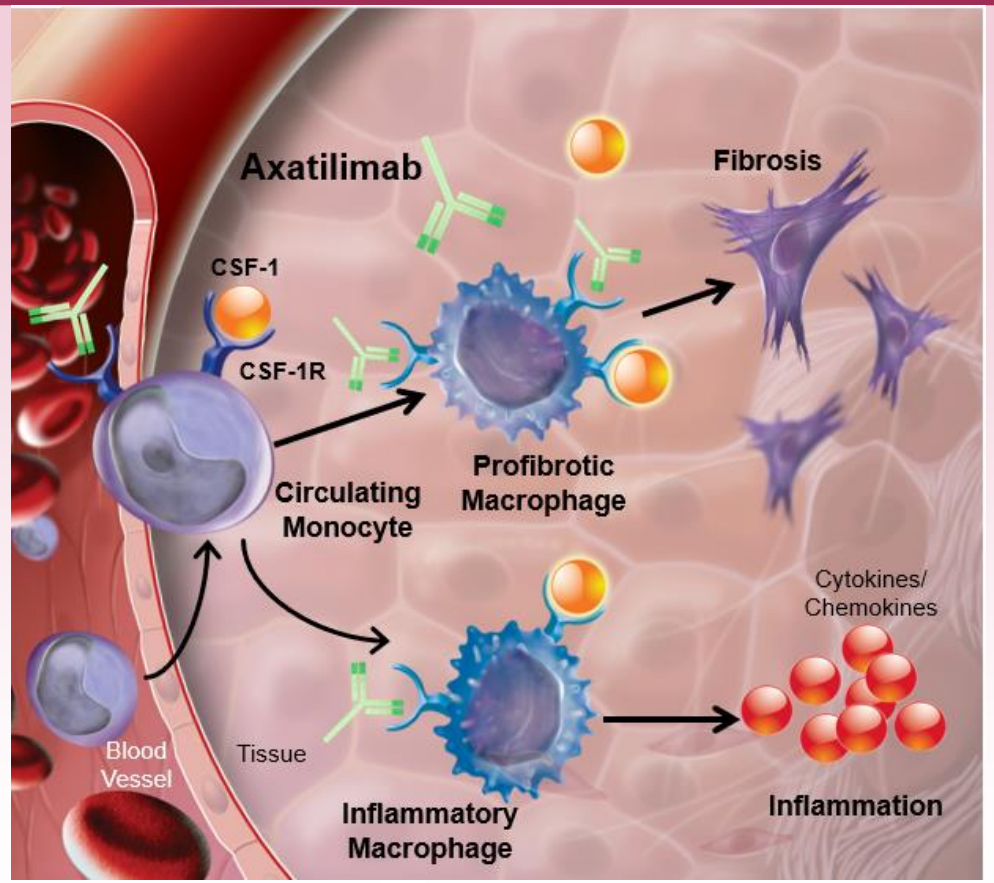
Axatilimab-csfr is an Anti-CSF-1R Monoclonal Antibody

Axatilimab-csfr blocks CSF-1R on circulating proinflammatory and profibrotic monocytes and macrophages thereby targeting multiple underlying mechanisms of the manifestations of cGVHD[1,2]



In early phase clinical studies, axatilimab-csfr:

- Depleted nonclassical monocytes from circulation[3]
- Reduced CSF-1R+ macrophages in cGVHD target tissues[3]
- Led to a decrease in profibrotic cytokines[3]

1. NIKTIMVO™ (axatilimab-csfr). Prescribing Information. Incyte Corporation; June 2026; 2. Wolff et al. ASH 2023, Plenary Abstract 1; 3. Kitko CL, et al. *J Clin Oncol*. 2023;41(10):1864-1875.

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AGAVE-201 – Key Trial Considerations

AGAVE-201: An Open-Label, Randomized, Phase 2 Study Design[1-3]

ELIGIBILITY CRITERIA

Inclusion

- ≥2 years of age[a]
- HSCT with refractory[b] or recurrent[c] active cGVHD despite at least 2 lines of systemic therapy (no limit)
- No limit of lung function severity

Exclusion

- Taking agents for treatment of cGVHD other than corticosteroids or either a CNI or mTOR inhibitor is prohibited

TREATMENT PERIOD

Randomization 1:1:1

0.3 mg/kg IV q2w n=79

1 mg/kg IV q2w n=81

3 mg/kg IV q4w n=80

Treatment for up to 2 years
until disease progression or unacceptable toxicity

Primary Endpoint

- ORR in the first 6 cycles (up to day 169) as defined by the 2014 NIH Consensus Development Project on Criteria for Clinical Trials in cGVHD

Key Secondary Endpoint

- Proportion of patients with a >5-point improvement in mLSS

Other Secondary Endpoints

- DOR[d]
- SRR[e]
- Organ-specific response rate[f]
- Number of patients with TEAEs

The median duration of treatment was 10.3 months (range: 0.5 to 28.6 months), and 73.4% were treated for more than 6 months[3]

[a] Age inclusion criteria differ by country. [b] Defined as meeting all of the following criteria: 1) development of 1 or more new sites of disease while being treated for cGVHD 2) progression of existing sites of disease despite at least 1 month of standard or investigational therapy for cGVHD 3) no response within 3 months on prior therapy for cGVHD and treating physician believes a new systemic therapy is required. [c] Active, symptomatic disease (after an initial response to prior therapy) as defined, based on the NIH 2014 consensus criteria, by organ-specific or global assessment or for which the physician believes that a new line of systemic therapy is required. [d] DOR is defined as the time from initial partial response or complete response until documented progression of cGVHD, start of new therapy, or death for any reason. [e] SRR is defined as the proportion of participants with objective response lasting for at least 20 weeks (140 days) from the time of initial response. Responses by organ system will be assessed based on the 2014 NIH Consensus Development Project on Clinical Trials in cGVHD. [f] Organ-specific response is defined as the proportion of participants with objective response for the nine individual organs based on 2014 NIH Consensus Development Project on Criteria for Clinical Trials in cGVHD (skin, eyes, mouth, esophagus, upper GI, lower GI, liver, lungs and joints and fascia).

DOR, duration of response; KPS, Karnofsky Performance Scale; mLSS, modified Lee Symptom Scale; PS, performance score; SRR, sustained response rate; TEAE, treatment-emergent AE.

1. ClinicalTrials.gov. Accessed January 25, 2024. https://clinicaltrials.gov/ct2/show/NCT04710576 2. Wolff D, et al. Presentation at ASH Annual Meeting. December 10, 2023; 3. NIKTIMVO™ (axatilimab-csfr). Prescribing Information. Incyte Corporation; June 2026.

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AGAVE-201: Baseline Characteristics (0.3 mg/kg q2w cohort)[1]

Patient Characteristic	0.3 mg/kg q2w (N=79)
Age, median (min, max), years	50 (7, 76)
Age ≥ 65 years, n (%)	21 (27)
Sex, males, n (%)	46 (58)
Race, White, n (%)	
White	67 (85)
Asian	4 (5)
Black	2 (3)
Other	1 (1)
Not reported	5 (6)
Time from cGVHD diagnosis to randomization, median (range), months	47 (4, 211)
≥ 4 organs involved, n (%)	45 (57)
Patients with severe cGVHD, n (%)	63 (80)
Global severity rating, median (range)	7 (2, 10)
Modified Lee Symptom Scale Score at baseline, median (range)	24 (4, 55)
Median (range) corticosteroid dose at baseline (PE/kg)	0.21 (0.04, 2.12)

Patient Characteristic	0.3 mg/kg q2w (N=79)
Number of prior lines of therapy, median (range)	4 (2, 12)
Number of prior lines of therapy, n (%)	
2	11 (14)
3	14 (18)
4	17 (22)
≥ 5	37 (47)
Prior ibrutinib, n (%)	27 (34)
Prior ruxolitinib, n (%)	57 (72)
Prior belumosudil, n (%)	16 (20)
Refractory to last therapy, n (%)	37 (47)

- 71% of patients were receiving systemic corticosteroids at baseline, at a mean dose of 18.8 mg[2]

kg, kilogram; mg, miligram; PE/kg, prednisone equivalents/kilogram/day; q2w, every 2 weeks.

1. NIKTIMVO™ (axatilimab-csfr). Prescribing Information. Incyte Corporation; June 2026. 2. Data on file. Incyte Corporation.

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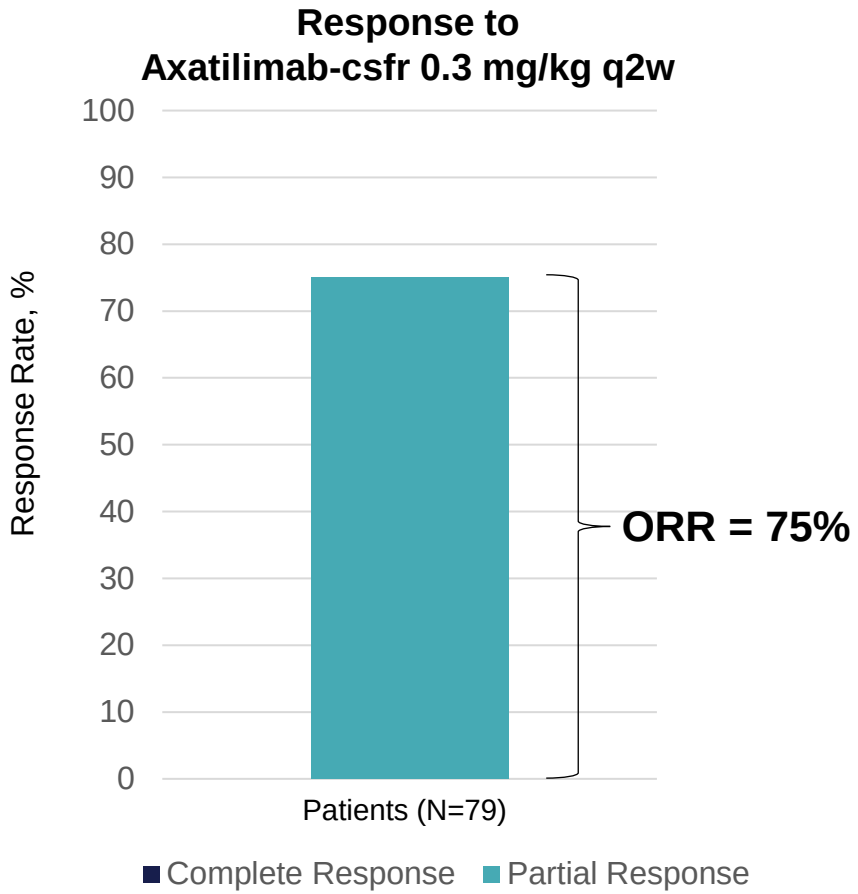
Overall Response Rate and Duration of Response

- Efficacy was based on ORR through Cycle 7 Day 1, where overall response included complete response or partial response according to the 2014 NIH Consensus Development Project on Response Criteria



Patients (N=79)	
Overall response rate, n (%)	**59 (75%)**
(95% CI)	(64%, 84%)
Complete response rate	0 (0%)
Partial response rate	59 (75%)
Duration of Response[a]	
Median (95% CI) in months	1.9 (1.6, 3.5)

- In patients who achieved a response, no death or new systemic therapy initiation occurred in 60% (95% CI: 43, 74) of patients for at least 12 months since response
- The median time to first response was 1.5 months (range, 0.9-5.1)
- Exploratory analysis showed that 56% (95% CI: 44, 67) of patients had a ≥ 7-point decrease in mLSS through Cycle 7 Day 1

[a] Calculated from first response to progression, death, or new systemic therapies for cGVHD.

mLSS, modified Lee Symptom Scale; ORR, overall response rate.

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Adverse Reactions in ≥ 10% of Patients With cGVHD Who Received Axatilimab-csfr

Adverse Reactions	Axatilimab-csfr 0.3 mg/kg intravenously Q2W (N = 79) All Grades (%)	Grade 3-4 (%)
Infections and Infestations		
Infection (pathogen unspecified)[a]	57	14
Viral infection[b]	43	15
Bacterial infection[c]	15	8
Musculoskeletal and Connective Tissue Disorders		
Musculoskeletal pain[d]	35	3
General disorders and administration site conditions		
Fatigue[e]	32	4
Pyrexia	15	1
Edema[f]	13	1

- Dose reduction due to ARs occurred in 8% of patients
- Dose interruptions due to an AR occurred in 44% of patients
- ARs leading to dose interruption in > 2 patients were viral infection, infection (pathogen unspecified), bacterial infection, musculoskeletal pain, and pyrexia
- Clinically relevant ARs in < 10% of patients included
  - Periorbital edema
  - Pruritus
  - Hypertension

[a] Includes abscess jaw, atypical pneumonia, bacteremia, bronchitis, conjunctivitis, cystitis, device-related infection, enterocolitis infectious, gastroenteritis, gastrointestinal infection, groin abscess, hordeolum, liver abscess, nasopharyngitis, otitis media, otitis media acute, pneumonia, respiratory tract infection, rhinitis, sepsis, sinusitis, tooth infection, upper respiratory tract infection, urinary tract infection, and wound infection; [b] Includes adenoviral upper respiratory infection, BK virus infection, COVID-19, coronavirus infection, enterovirus infection, gastroenteritis astroviral, gastroenteritis viral, herpes simplex, herpes zoster, influenza, metapneumovirus bronchiolitis, metapneumovirus infection, norovirus infection, oral viral infection, parainfluenza viral bronchitis, parainfluenza virus infection, respiratory syncytial virus infection, rhinovirus infection, viral infection, and viral upper respiratory tract infection; [c] Includes bacterial diarrhea, bacterial vaginosis, campylobacter gastroenteritis, campylobacter infection, cellulitis, clostridium difficile colitis, clostridium difficile infection, enterococcal infection, erysipelas, hemophilus infection, lower respiratory tract infection bacterial, pseudomonal skin infection, staphylococcal bacteremia, staphylococcal infection, stenotrophomonas infection, streptococcal infection, and urinary tract infection enterococcal; [d] Includes arthralgia, back pain, flank pain, musculoskeletal pain, myalgia, pain in extremity; [e] Includes asthenia, fatigue, and malaise; [f] Includes localized edema and peripheral edema.

AR, adverse reaction.

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Adverse Reactions in ≥ 10% of Patients With cGVHD Who Received Axatilimab-csfr (cont'd)

Adverse Reactions	Axatilimab-csfr 0.3 mg/kg intravenously Q2W (N = 79) All Grades (%)	Grade 3-4 (%)
Gastrointestinal disorders		
Nausea[a]	23	3
Diarrhea[b]	18	5
Nervous system disorders		
Headache[c]	20	1
Dizziness[d]	11	0
Respiratory, thoracic and mediastinal disorders		
Cough[e]	18	0
Dyspnea[f]	15	3
Immune system disorders		
Drug hypersensitivity[g]	13	3

Adverse Reactions	Axatilimab-csfr 0.3 mg/kg intravenously Q2W (N = 79) All Grades (%)	Grade 3-4 (%)
Metabolism and nutrition disorders		
Decreased appetite	11	4
Vascular disorders		
Hemorrhage[h]	11	1
Skin and subcutaneous tissue disorders		
Rash[i]	10	0

- SARs occurred in 44% of patients
  - SARs in > 2 patients included infection (pathogen unspecified), viral infection, and respiratory failure
- Permanent discontinuation of axatilimab-csfr due to an AR occurred in 10% of patients

[a] Includes nausea and vomiting; [b] Includes colitis and diarrhea; [c] Includes headache and migraine; [d] Includes dizziness and dizziness postural; [e] Includes cough and productive cough; [f] Includes dyspnea and dyspnea exertional; [g] Includes bronchospasm, flushing, hot flush, hypersensitivity, infusion-related hypersensitivity reaction, infusion-related reaction, and urticaria; [h] Includes contusion, epistaxis, hematochezia, hematoma, and vaginal hemorrhage; [i] Includes dermatitis bullous, dermatitis exfoliative generalized, rash, and rash maculo-papular.

SAR, serious adverse reactions.

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Immunogenicity

- The observed incidence of ADAs is highly dependent on the sensitivity and specificity of the assay
- Differences in assay methods preclude meaningful comparisons of the incidence of ADAs in the studies described below with the incidence of ADAs in other studies, including those of axatilimab-csfr or of other axatilimab products.
- In 276 patients in the AGAVE-201 trial, 34% (93/276) developed ADAs following a median exposure time of 7.8 months
  - Hypersensitivity reactions occurred in 26% (13/50) of patients with NAb and in 4% (2/45) of those without NAb
  - NAb were detected in 47/93 patients on trial with treatment-emergent ADAs
  - There was no clinically meaningful effect of anti-axatilimab-csfr antibodies or NAb on pharmacokinetics, pharmacodynamics, or effectiveness of axatilimab-csfr

ADA, anti-drug antibodies; NAb, neutralizing antibodies.

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Selected Laboratory Abnormalities

Laboratory Abnormality	Axatilimab-csfr 0.3 mg/kg intravenously Q2W (N = 79)	
	All Grades[a] (%)	Grade 3 or 4[a] (%)
Hematology		
Decreased hemoglobin	48	4
Chemistry		
Increased aspartate aminotransferase	61	5
Increased alanine aminotransferase	51	3
Decreased phosphate	51	NA
Increased gamma glutamyl transferase	39	4
Increased lipase	34	3
Increased amylase	32	0
Increased calcium	31	1
Increased alkaline phosphatase	28	0
Increased creatine phosphokinase	25	0

NA, not applicable.
[a] The denominator used to calculate the rate varied from 78 to 79 based on the number of patients with at least 1 post-treatment value.
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Axatilimab-csfr Dosage and Administration

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Recommended Dosage and Monitoring

- For patients weighing at least 40 kg, administer axatilimab-csfr 0.3 mg/kg, up to a maximum dose of 35 mg, as an intravenous infusion over 30 minutes every 2 weeks until progression or unacceptable toxicity
- Monitor AST, ALT, ALP, CPK, amylase, and lipase prior to the start of axatilimab-csfr, every 2 weeks for the first month, and every 1 to 2 months thereafter until abnormalities are resolved
- For patients that have previously experienced an IRR to axatilimab-csfr, premedicate with an antihistamine and antipyretic
  - Monitor for signs and symptoms of IRRs, including fever, chills, rash, flushing, dyspnea, and hypertension

ALT, alanine aminotransferase; ALP, alkaline phosphatase; AST, aspartate aminotransferase; CPK, creatine phosphokinase; IRR, infusion-related reactions.

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Preparation and Administration

Preparation

- Use aseptic technique to prepare axatilimab-csfr
- Visually inspect the vial for particulate matter and discoloration prior to dilution. Axatilimab-csfr is a slightly opalescent, pale brownish yellow solution. Discard the vial if the solution is cloudy, discolored, or contains visible particles
- Do not shake the vial
- Determine the dose based on the recommended dosage and total volume of axatilimab-csfr solution needed

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Preparation and Administration (cont'd)

Dilution

- Withdraw the calculated volume of axatilimab-csfr solution from the vial and add it into an intravenous infusion bag made of PVC, polyolefin, polyolefin with polyamide, or EVA containing 0.9% Sodium Chloride Injection to achieve a final concentration between the range of 0.24 mg/mL and 0.75 mg/mL
- Discard vial with any unused portion
- Mix diluted solution by gentle inversion. Do not shake
- Parenteral drug products should be inspected visually for particulate matter and discoloration prior to administration, whenever solution and container permit. The diluted solution is a clear to slightly opalescent, colorless solution that may contain trace amounts of translucent to white particles. Discard if the solution is cloudy, discolored, or contains extraneous particulate matter other than trace amounts of translucent to white particles

EVA, ethylene vinyl acetate; PVC, polyvinyl chloride

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Preparation and Administration (cont'd)

Storage of diluted axatilimab-csfr solution

- Immediately use diluted axatilimab-csfr solution. If not used immediately, the diluted solution can be stored:
  - At room temperature [up to 25°C (77°F)] for no more than 4 hours from the time of preparation to the end of the infusion

    OR
  - Refrigerated at 2°C to 8°C (36°F to 46°F) for no more than 24 hours. If refrigerated, allow the diluted solution to come to room temperature prior to administration. The diluted solution must be administered within 4 hours (including infusion time) once it is removed from the refrigerator
- Do not freeze or shake the diluted solution

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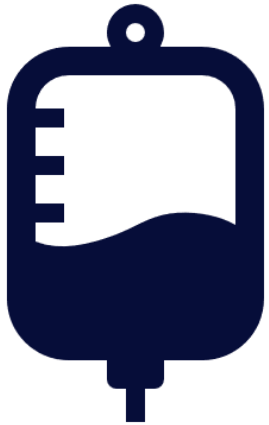
Preparation and Administration (cont'd)



Administration

- Administer diluted axatilimab-csfr solution by intravenous infusion over 30 minutes through a dedicated infusion line that includes a **sterile, low-protein binding 0.2-micron in-line** or **add-on PES filter**
- Do not co-administer other drugs through the same infusion line
- After administration, flush the infusion line with 0.9% Sodium Chloride Injection

PES, polyethersulfone

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Dosage Forms and Strengths

- Axatilimab-csfr injection is a slightly opalescent, pale brownish yellow solution available as:
  - 9 mg/0.18 mL in a single-dose vial
  - 9 mg/0.9 mL in a single-dose vial
  - 22 mg/0.44 mL in a single-dose vial
  - 22 mg/2.2 mL (10 mg/mL) in a single-dose vial

- Prior to preparation, check the vial labels to confirm which concentration is being used to avoid dosing errors, including underdosage and overdosage

Axatilimab-csfr Vial Presentations

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Dosage Modifications for Adverse Reactions: Infusion-Related Reactions[1]

Infusion-Related Reactions	
Severity	**Dosage Modification**
Grade 1[a] or 2[b]	• Temporarily interrupt the infusion until resolution or decrease infusion rate by 50% • Initiate symptomatic treatment (e.g., antihistamines and antipyretics) • For subsequent infusions, premedicate and resume the infusion at 50% of the prior infusion rate
Grade 3[c] or 4[d]	• Permanently discontinue axatilimab-csfr

[a] Grade 1 – mild; asymptomatic or mild symptoms; clinical or diagnostic observations only; intervention not indicated.[2] [b] Grade 2 – moderate; minimal, local or noninvasive intervention indicated; limiting age-appropriate instrumental activities of daily living.[2] [c] Grade 3 – severe or medically significant but not immediately life-threatening; hospitalization or prolongation if hospitalization indicated; disabling; limiting self care activities of daily living.[2] [d] Grade 4 – life threatening consequences; urgent intervention indicated.[2]

1. NIKTIMVO™ (axatilimab-csfr). Prescribing Information. Incyte Corporation; June 2026; 2. Common Terminology Criteria for Adverse Events. Version 5.0. 2017. https://dctd.cancer.gov/research/ctep-trials/for-sites/adverse-events/ctcae-v5-8x11.pdf. Accessed on August 20, 2026.

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Dosage Modifications for Adverse Reactions: Elevated AST or ALT

Elevation of AST or ALT (on the day of dosing)	
Severity	**Dosage Modification**
Grade 3 with total bilirubin ≤ Grade 1	• Withhold axatilimab-csfr until recovery to Grade 2, then resume axatilimab-csfr at 0.2 mg/kg (maximum 23 mg) every 2 weeks
Elevation of AST or ALT (regardless of the time of the reaction)	
ALT or AST ≥ 3 times ULN with total bilirubin ≥ 2 times ULN and ALP < 2 times ULN	• Withhold axatilimab-csfr and investigate for drug-induced liver injury. If confirmed, permanently discontinue axatilimab-csfr
Grade 4	• Permanently discontinue axatilimab-csfr

ULN, upper limit of normal.

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Dosage Modifications for Adverse Reactions: Elevation Of CPK, Amylase, Or Lipase, And Nonhematologic Adverse Reactions

Elevation of CPK, amylase, or lipase	
Severity	**Dosage Modification**
≥ Grade 3	• If diagnostic evaluation results show no evidence of end-organ damage, continue axatilimab-csfr without dose reduction • If diagnostic evaluation results show evidence of end-organ damage, permanently discontinue axatilimab-csfr
Symptomatic ≥ Grade 3	• Permanently discontinue axatilimab-csfr
Other Nonhematologic Adverse Reactions	

Grade 3	• Withhold axatilimab-csfr until recovery to Grade 2:
	  • If delayed by ≤ 4 weeks from the planned infusion, resume axatilimab-csfr at 0.2 mg/kg (maximum 23 mg) every 2 weeks
	  • If delayed by > 4 weeks from the planned infusion, permanently discontinue axatilimab-csfr

Grade 4	• Permanently discontinue axatilimab-csfr

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Warnings, Precautions, and Use in Specific Populations

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Summary of Warnings and Precautions

Infusion-Related Reactions

- Axatilimab-csfr can cause infusion-related reactions
- Infusion-related reactions, including hypersensitivity reactions, occurred in 18% of patients who received axatilimab-csfr in the clinical trial, AGAVE-201, with Grade 3 or 4 reactions in 1.3% of patients
- Premedicate with an antihistamine and an antipyretic for patients who have previously experienced an infusion-related reaction to axatilimab-csfr
- 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 axatilimab-csfr based on severity of the reaction

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Summary of Warnings and Precautions

Embryo-Fetal Toxicity

- Based on its mechanism of action, axatilimab-csfr 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 axatilimab-csfr and for 30 days after the last dose

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Use in Specific Populations

Pregnancy

- Based on its MOA, axatilimab-csfr may cause fetal harm when administered to a pregnant person
- There are no available data on axatilimab-csfr use in pregnant people to evaluate for a drug-associated risk
- IgG is known to cross the placenta; therefore, axatilimab-csfr has the potential to be transmitted to the developing fetus
- Advise women of the potential risk to the fetus

Lactation

- Advise women not to breastfeed during treatment and for 30 days after the last dose of axatilimab-csfr

Pediatric Use

- The safety and efficacy of axatilimab-csfr for the treatment of cGVHD after failure ≥ 2 prior lines of systemic therapy have been established in pediatric patients weighing at least 40 kg
- It is supported by evidence from clinical trials that included 3 children (between 6 and less than 12 years old) and 5 adolescents (ages 12 to less than 17)
- The safety and efficacy of axatilimab-csfr have not been established in pediatric patients weighing < 40 kg
- Compared to adult and pediatric patients weighing 40 kg and above, patients weighing less than 40 kg had lower maximum concentration, trough concentration, and average concentration at the same weight-based dosage
- Based on findings of thickening of the growth plate and metaphysis and/or degeneration of the growth plate in the femur in animals, monitor bone growth and development in pediatric patients

IgG, human immunoglobulin G; MOA, mechanism of action.

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Use in Specific Populations Cont'd

Geriatric Use

- In AGAVE-201, of the 79 patients that were treated with 0.3 mg/kg q2w with axatilimab-csfr, 26.6% were ≥ 65 years old and 2.5% were ≥ 75 years
- No overall differences in the safety and efficacy of axatilimab-csfr have been observed between patients ≥ 65 years and younger patients

People of Reproductive Potential

- Axatilimab-csfr may cause fetal harm when administered to a pregnant person
- Verify pregnancy status in people of reproductive potential prior to initiating axatilimab-csfr
- Advise people who could become pregnant to use effective contraception during treatment and for 30 days after the last dose of axatilimab-csfr

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Appendix

- Pharmacokinetics & Pharmacodynamics
- Axatilimab-csfr Vial Presentation Transition

Click on bulleted text to access the corresponding backup slides.

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Pharmacodynamics / Pharmacokinetics

Pharmacodynamics: CSF-1 and IL-34

- Axatilimab-csfr caused a dose-dependent increase from 0.15 mg/kg to 6 mg/kg (0.5 to 20 times the approved recommended dosage) in CSF-1 and IL-34 concentrations and a dose-dependent reduction in the levels of nonclassical monocytes in peripheral blood

Pharmacokinetics:

- Axatilimab-csfr pharmacokinetics are presented as geometric mean (%CV) in adult patients with cGVHD following axatilimab-csfr 0.3 mg/kg (maximum 35 mg) every 2 weeks, unless otherwise specified
- Axatilimab-csfr AUC increased in a greater than dose-proportional manner following single-dose administration of axatilimab-csfr over a dose range of 0.15 mg/kg to 3 mg/kg (0.5 to 10 times the approved recommended dosage) in healthy subjects
- There was no axatilimab-csfr systemic accumulation following the approved recommended dosage

CV, coefficient of variation; IL, interleukin.

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Pharmacodynamics / Pharmacokinetics (cont)

Distribution:

- Axatilimab-csfr volume of distribution is 6.06 L (16.3% CV)

Elimination and metabolism:

- Axatilimab-csfr total clearance is 0.07 L/h (38.8% CV). The median (5th to 95th percentile) time to 97% reduction from Cmax after the end of infusion is 4.0 (2.3 to 7.2) days following axatilimab-csfr 0.3 mg/kg (maximum 35 mg)
- The total clearance of axatilimab-csfr is composed of linear and non-linear components such that axatilimab-csfr clearance decreased from 2.32 mL/h/kg to 0.21 mL/h/kg and mean terminal half-life increased from 10.7 hours to 108 hours following single-dose administrations of axatilimab-csfr over a dose range of 0.15 mg/kg to 3 mg/kg (0.5 to 10 times the approved recommended dosage)
- Axatilimab-csfr is expected to be metabolized into small peptides by catabolic pathways

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Axatilimab-csfr Vial Presentation Transition

BACK

Discontinued Vial Presentation	Discontinued NDC	New Vial Presentations	New NDC
9 mg/0.18 mL (50 mg/ml)	50881-034-12	9 mg/0.9 mL (10 mg/mL)	50881-036-17
22 mg/0.44 mL (50 mg/mL)	50881-023-11	22 mg/2.2 mL (10 mg/mL)	50881-047-18

As axatilimab-csfr transitions to the new vial concentrations, please consider the updated vial presentations when preparing doses and refer to the complete Prescribing Information for complete preparation and administration guidance

NDC, National Drug Code.

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