skysona™
(elivaldogene autotemcel)
suspension for IV infusion

BILLING & CODING GUIDE

A Resource for Coding, Billing, and Reimbursement Information for SKYSONA™

INDICATION

SKYSONA is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD). Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9.

This indication is approved under accelerated approval based on 24-month Major Functional Disability (MFD)-free survival. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Limitations of Use

SKYSONA does not prevent the development of or treat adrenal insufficiency due to adrenoleukodystrophy.

An immune response to SKYSONA may limit the persistence of descendent cells of SKYSONA, causing rapid loss of efficacy of SKYSONA in patients with full deletions of the human adenosine triphosphate binding cassette, sub family D, member 1 (*ABCD1*) gene.

SKYSONA has not been studied in patients with CALD secondary to head trauma.

Given the risk of hematologic malignancy with SKYSONA, and unclear long-term durability of SKYSONA and human adrenoleukodystrophy protein (ALDP) expression, careful consideration should be given to the appropriateness and timing of treatment for each boy, especially for boys with isolated pyramidal tract disease based on available treatment options since their clinical symptoms do not usually occur until adulthood.

IMPORTANT SAFETY INFORMATION

WARNING: HEMATOLOGIC MALIGNANCY

Hematologic malignancies, including life-threatening cases of myelodysplastic syndrome and acute myeloid leukemia, have occurred in patients treated with SKYSONA. Patients have been diagnosed between 14 months and 7.5 years after SKYSONA administration, and the cancers appear to be the result of the SKYSONA lentiviral vector, Lenti-D, integration in proto-oncogenes. Monitor patients closely for evidence of malignancy through complete blood counts at least every 3 months. Monitor paitients through assessments for evidence for clonal expansion or predominance at least twice in the first year and annually thereafter; consider bone marrow evaluations as clinically indicated.

PLEASE NOTE:

This Billing and Coding Guide is intended to help healthcare professionals understand key billing and coding considerations for SKYSONA and its related services when using SKYSONA for its FDA-approved use during a hospital inpatient admission.

The information provided in this guide is for informational and reference purposes only. The information provided in this guide should not be construed as medical or legal advice. All medical decisions should be made at the discretion of the provider. Providers should exercise independent clinical judgment when selecting codes and submitting claims to accurately reflect the services rendered to individual patients. Coding and coverage policies can change, often without warning. It is the responsibility of the provider to determine coverage, reimbursement, appropriate coding for a particular patient and/or procedure, and to submit accurate claims. The information in this guide is not a guarantee of coverage or reimbursement for any product or service. Please contact your patient's health plan or work with Genetix CARES for additional resources regarding coding for a specific plan.

For reimbursement questions or appeals support, talk with a Patient Navigator at Genetix CARES by calling 1-833-888-6378, emailing support@genetixcares.com, or visiting genetixcares.com.

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We're committed to standing by your patients and caregivers

Genetix CARES is here to help your patients and their caregivers navigate access to Genetix Biotherapeutics gene therapies with resources designed to support their unique treatment paths.

Examples of support range from helping to identify Qualified Treatment Center options for consultation to understanding their insurance benefits and the process for prior authorization.

Patient Navigators could also be your patients' and their caregivers' primary point of contact at **Genetix CARES**. You can rely on the Patient Navigator to offer additional support in a variety of ways, including:

- **Coordination** of care services and logistics, including assistance with travel and lodging
- **Access** to financial assistance and insurance coverage
- **Resources** tailored for every treatment stage, including support for fertility preservation
- **Education** that empowers informed treatment decisions
- **Support** from a dedicated, single point of contact and from other people and families who experienced treatment firsthand

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Patient Care Process

Cell Collection (mobilization and apheresis)

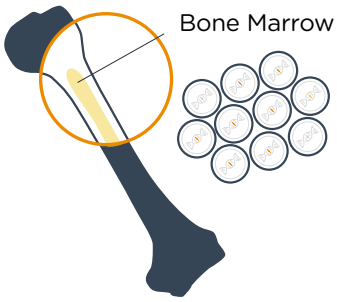
More than one collection may be required to acquire the amount needed for transplanting.[1]

Plerixafor, in combination with G-CSF, can be dosed and documented based on hospital protocol for mobilization.[1]

CONDUCTED IN EITHER HOSPITAL OUTPATIENT OR HOSPITAL INPATIENT SETTING

Full Myeloablative and Lymphodepleting Conditioning[1]

SKYSONA Infusion



Engraftment

CONDUCTED DURING A HOSPITAL INPATIENT ADMISSION

Post-infusion monitoring/Long-term follow-up[1]

Patients should be prepared to remain hospitalized and monitored for up to approximately 2 months after infusion. Patients should be monitored lifelong for hematologic malignancy. For the first fifteen years after treatment with SKYSONA, monitor via complete blood count (with differential) at least every 3 months and via integration site analysis or other testing for evidence of clonal expansion and predominance at least twice in the first year and then annually.

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Cell Collection Coding

MOBILIZATION AND APHERESIS

ICD-10-CM CODE[2]	DESCRIPTION
E71.520	Childhood cerebral X-linked adrenoleukodystrophy

When apheresis is conducted during a hospital inpatient admission, the following ICD-10-PCS codes may apply:

ICD-10-PCS CODES[3]	DESCRIPTION
6A550ZV	Pheresis of Hematopoietic Stem Cells, Single
6A551ZV	Pheresis of Hematopoietic Stem Cells, Multiple

Fee schedules, relative value units, conversion factors and/or related components are not assigned by the AMA, are not part of CPT, and the AMA is not recommending their use. The AMA does not directly or indirectly practice medicine or dispense medical services. The AMA assumes no liability for data contained or not contained herein.

CPT is a registered trademark of the American Medical Association.

U.S. Government End Users. CPT is commercial technical data, which was developed exclusively at private expense by the American Medical Association (AMA), 330 North Wabash Avenue, Chicago, Illinois 60611. Use of CPT in connection with this product shall not be construed to grant the Federal Government a direct license to use CPT based on FAR 52.227-14 (Data Rights - General) and DFARS 252.227-7015 (Technical Data - Commercial Items).

Although commonly applied for hospital outpatient mobilization and apheresis, the following CPT procedure codes may also be applicable for inpatient processes as well, based on payer-specific requirements:

CPT® CODES[4]	DESCRIPTION
96372	Therapeutic, prophylactic, or diagnostic injection (specify substance or drug); subcutaneous or intramuscular
38206*	Blood-derived hematopoietic progenitor cell harvesting for transplantation, per collection; autologous

* Report the code for each collection encounter

HCPCS CODES[5]	DESCRIPTION
J2562	Injection, plerixafor, 1 mg
J1442	Injection, filgrastim (G-CSF), excludes biosimilar, 1 mcg
J1447	Injection, tbo-filgrastim, 1 mcg
Q5101	Injection, filgrastim-sndz, biosimilar, (Zarxio), 1 mcg
Q5110	Injection, filgrastim-aafi, biosimilar, (Nivestym), 1 mcg
Q5125	Injection, filgrastim-ayow, biosimilar, (Releuko), 1 mcg

Revenue codes are provided for informational purposes only and may vary by hospital:

REVENUE CODES[6]	DESCRIPTION
0250	Pharmacy
0636	Drugs requiring detailed coding
0871	Cell/Gene Therapy Cell Collection

Payers may vary on the required coding to appropriately reflect mobilization and apheresis for the collection of cells. Providers should confirm with each payer prior to submitting claims.

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Conditioning Regimen Coding

A busulfan/fludarabine or busulfan/cyclophosphamide regimen was administered in clinical trials, for conditioning prior to administration of SKYSONA.[1]

ICD-10-CM CODE[2]	DESCRIPTION
E71.520	Childhood cerebral X-linked adrenoleukodystrophy

ICD-10-PCS CODE[3]	DESCRIPTION
3E03305	Introduction of Other Antineoplastic into Peripheral Vein, Percutaneous Approach

CPT CODES[4]	DESCRIPTION
96413	Chemotherapy administration, intravenous infusion technique; up to 1 hour, single or initial substance/drug
96415	Chemotherapy administration, intravenous infusion technique; each additional hour

HCPCS CODES[5]	DESCRIPTION
J0594	Injection, busulfan, 1 mg
J9185	Injection, fludarabine, 50 mg
J9070	Injection, cyclophosphamide, 100 mg

HCPCS CODES[5]	DESCRIPTION
0250	General Pharmacy
0251	Generic Pharmacy
0260	General IV Therapy
0636	Drug Requiring Detailed Coding

10-DIGIT NDC[8]	NOTES
Varies	Several manufacturers produce busulfan, fludarabine, and cyclophosphamide. Therefore, providers should reference and apply the appropriate NDC code, if required by payers.

Payers may vary on their requirements for detailed coding on the delivery of any conditioning regimen associated with subsequent SKYSONA administration. These requirements can vary based on the setting of care as well as for the patient's specific plan. Providers should confirm requirements with individual payers.

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At this point in time, no product-specific ICD-10-PCS, CPT or HCPCS codes have been assigned to SKYSONA and its administration. Providers may be required to identify SKYSONA with a miscellaneous HCPCS code or general ICD-10-PCS code. These codes should be confirmed with each payer.

ICD-10-CM CODE[2]	DESCRIPTION
E71.520	Childhood cerebral X-linked adrenoleukodystrophy

ICD-10-PCS codes are applied to define specific procedures during a hospital inpatient admission.

ICD-10-PCS CODES[3]	DESCRIPTION
30233C0*	Transfusion of autologous hematopoietic stem/progenitor cells, genetically modified into peripheral vein, percutaneous approach
30243C0*	Transfusion of autologous hematopoietic stem/progenitor cells, genetically modified into central vein, percutaneous approach

MS-DRG alignment—Medicare only. Applying the appropriate ICD-10-PCS code and ICD-10-CM diagnosis codes will align inpatient admissions to MS-DRG 016 or 017 for payment. It is the provider's responsibility to contact each payer for payer-specific coding requirements before submitting any claims.

MS-DRG[6]	DESCRIPTION
016	Autologous bone marrow transplant with cc/mcc
017	Autologous bone marrow transplant without cc/mcc

CPT CODES[4]	DESCRIPTION
96413	Chemotherapy administration, intravenous infusion technique; up to 1 hour, single or initial substance/drug
96415	Chemotherapy administration, intravenous infusion technique; each additional hour

HCPCS CODES[5]	DESCRIPTION
J3490	Unclassified drugs
J3590	Unclassified biologics

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Administration (cont'd)

At this point in time, no product-specific ICD-10-PCS, CPT or HCPCS codes have been assigned to SKYSONA and its administration. Providers may be required to identify SKYSONA with a miscellaneous HCPCS code or general ICD-10-PCS code. These codes should be confirmed with each payer.

CELL/GENE THERAPY REVENUE CODES[11]	DESCRIPTION
0872	Cell/Gene Therapy Specialized Biologic Processing and Storage–Prior to Transport
0873	Cell/Gene Therapy Storage and Processing after Receipt of Cells from Manufacturer
0874	Cell/Gene Therapy Infusion of Modified Cells
0892	Special Processed Drugs–FDA Approved Gene Therapy–Charges for Modified Gene Therapy

Many payers require that the SKYSONA NDC code be reported on the claim, using an 11-digit format (5-4-2) to comply with the electronic claims transaction provisions of the Health Insurance Portability and Accountability Act of 1996 (HIPAA).[9] Requirements may vary by payer. For example, certain payers may require the 10-digit NDC code; others may require the N4 format.

10-DIGIT NDC[8]	11-DIGIT NDC[9]	CLAIM REPORTING REQUIREMENTS[10]
73554-2111-1	73554-2111-01	N473554211101UN

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HOSPITAL REVENUE CODES

One or more of the following revenue codes may apply to services associated with SKYSONA. Each payer's acceptance of and associated claim documentation for these codes should be verified.

REVENUE CODES[11]	DESCRIPTION
0871	Cell/Gene Therapy Cell Collection
0872	Cell/Gene Therapy Specialized Biologic Processing and Storage—Prior to Transport
0873	Cell/Gene Therapy Storage and Processing after Receipt of Cells from Manufacturer Cells
0874	Cell/Gene Therapy Infusion of Modified Cells
0892	Special Processed Drugs—FDA Approved Gene Therapy—Charges for Modified Gene Therapy

NDC INFORMATION

Payers may require that either a 10-digit or 11-digit format NDC code be documented on claims for SKYSONA. The table below outlines the format options, including potential requirement of an NDC qualifier (N4).

10-DIGIT NDC[8]	11-DIGIT NDC[9]	DESCRIPTION[10]
73554-2111-1	73554-2111-01	N473554211101UN

VALUE CODE

Select payers may require a Value Code to document the invoice price for SKYSONA.

VALUE CODE[11]	DESCRIPTION
87	Invoice/acquisition cost of modified biologics. For use with Revenue Category 0892

CPT® CODE

Currently, there is no gene therapy-specific infusion code. Therefore, the following CPT® code may be applicable for SKYSONA administration.

CPT® CODE[4]	DESCRIPTION
96413	Chemotherapy administration, intravenous infusion technique; up to 1 hour, single or initial substance/drug

ICD-10-CM DIAGNOSIS CODE

Providers should code to the level of specificity documented in the patient's medical record, which could fall under the ICD-10-CM diagnosis code below.

ICD-10-CM CODE[2]	DESCRIPTION
E71.520	Childhood cerebral X-linked adrenoleukodystrophy

HCPCS LEVEL II PRODUCT CODES

SKYSONA does not currently have a unique HCPCS code. SKYSONA may be reported by using one of the following unclassified HCPCS codes per payer requirements.

HCPCS CODES[5]	DESCRIPTION
J3490	Unclassified drugs
J3590	Unclassified biologics

ICD-10-PCS INPATIENT PROCEDURE CODES

ICD-10-PCS codes are applied to define specific procedures during a hospital inpatient admission. CMS recommends using 30233C0 or 30243C0; however, coding should be verified by payer.

ICD-10-PCS CODES[3]	DESCRIPTION
30233C0	Transfusion of autologous hematopoietic stem/progenitor cells, genetically modified into peripheral vein, percutaneous approach
30243C0	Transfusion of autologous hematopoietic stem/progenitor cells, genetically modified into central vein, percutaneous approach

In all cases, providers should verify claims coding requirements by payer.

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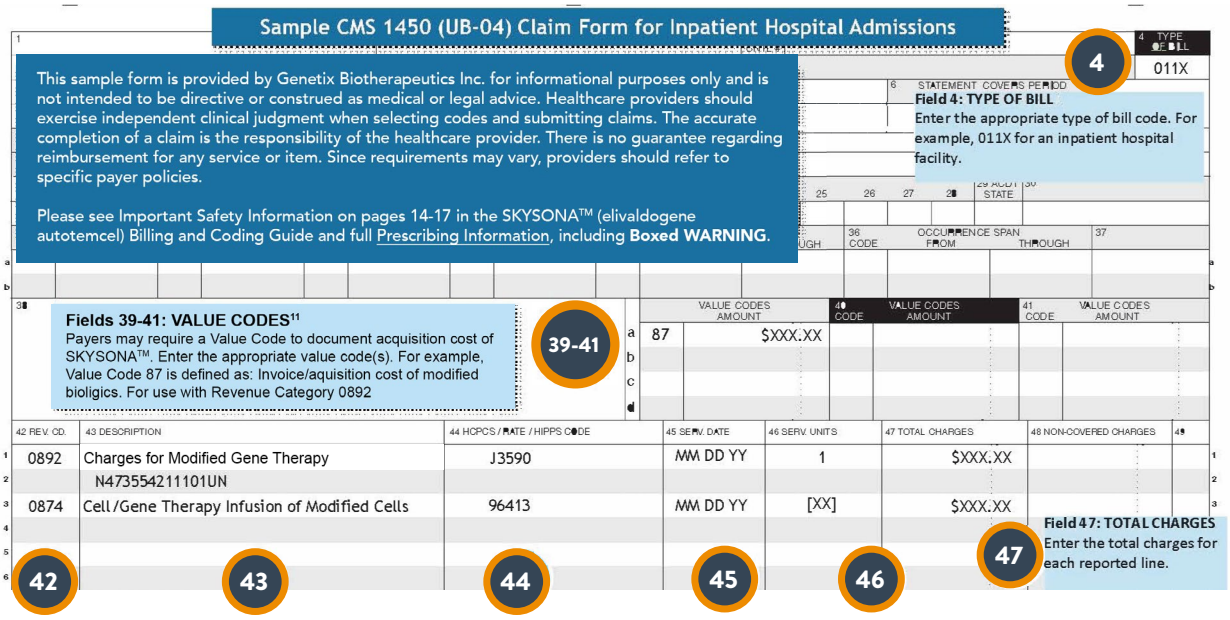
Sample CMS-1450 (UB-04) Claim Form for Inpatient Hospital Admissions

Clicking on the image below will open the Sample Claims Form in a new window.



4 TYPE OF BILL

Enter the appropriate type of bill code. For example, 011X for an inpatient hospital facility.

39-41 VALUE CODES[11]

Payers may require a Value Code to document acquisition cost for SKYSONA. Enter the appropriate value code(s). For example, Value Code 87 is defined as: Invoice/acquisition cost of modified biologics. For use with Revenue Category 0892.

42 REVENUE CODE[11]

Enter the appropriate revenue code for each reported line. For example, 0892 Charges for Gene Therapy.

43 DESCRIPTION[10,11]

Enter the 11-digit NDC with an appended N4 qualifier (electronic equivalent Loop 2410, LIN02). Enter the appropriate description for corresponding revenue codes.

44 HCPCS[5]

Enter the appropriate HCPCS Level II code, along with the applicable modifier. For example, J3590 or J3490. In lieu of a gene therapy-specific code for the infusion, the 96413 CPT code may be required.

45 SERVICE DATE

Enter the corresponding date(s) of service.

46 SERVICE UNITS

Enter appropriate units of service. For unclassified codes, such as J3490 and J3590, 1 unit of service is typically reported.

47 TOTAL CHARGES

Enter total charges for each reported line.

Sample forms are for informational purposes only. The accurate completion of a claim is the responsibility of the healthcare provider. There is no guarantee regarding reimbursement for any service or item. Since requirements may vary, providers should refer to specific payer policy.

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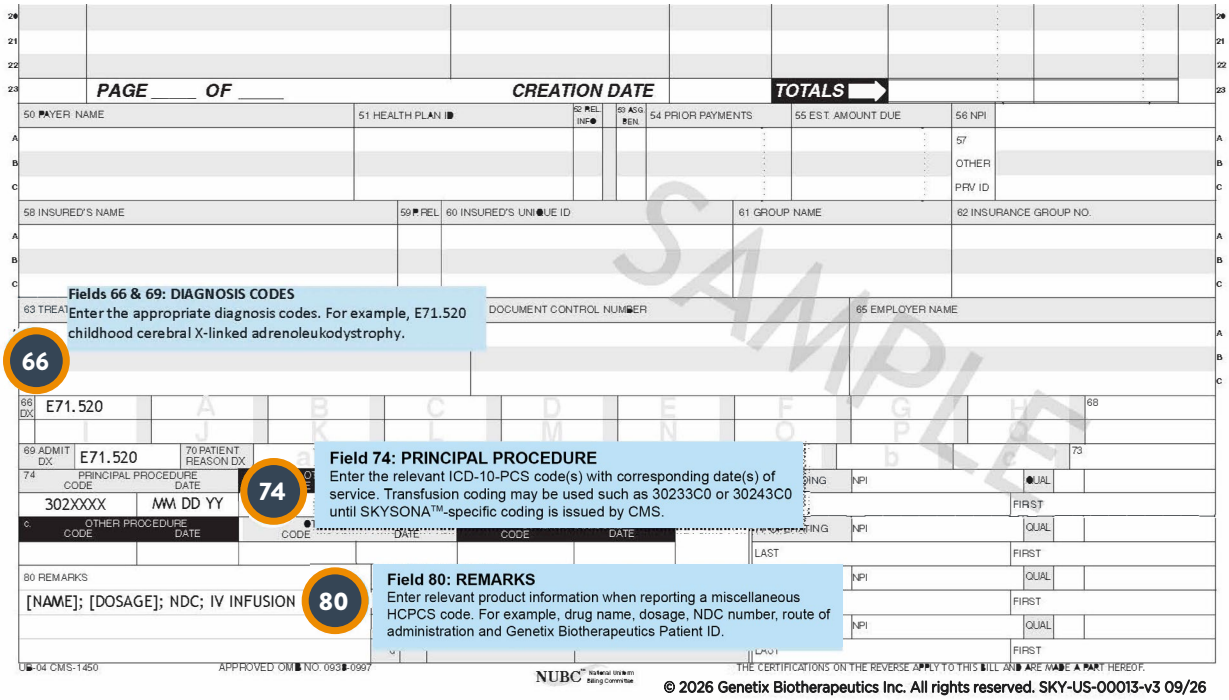
Sample CMS-1450 (UB-04) Claim Form for Inpatient Hospital Admissions (cont'd)



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66 DIAGNOSIS CODE(S)[2]

Enter the appropriate ICD-10-CM diagnosis code(s) for patient condition(s). For example, E71.520 Childhood cerebral X-linked adrenoleukodystrophy.

74 PRINCIPAL PROCEDURE[3]

Enter relevant ICD-10-PCS procedure code(s) with corresponding date(s) of service. Genetix Biotherapeutics intends to request a product-specific ICD-10-PCS code for SKYSONA in an upcoming application cycle with CMS. Prior to that date, CMS recommends using 30233C0 and 30243C0; however, required interim coding should be verified by payer.

80 REMARKS

Enter relevant product information when reporting a miscellaneous HCPCS code. For example, drug name, dosage, NDC number and route of administration, and Genetix Biotherapeutics Patient ID.

Sample forms are for informational purposes only. The accurate completion of a claim is the responsibility of the healthcare provider. There is no guarantee regarding reimbursement for any service or item. Since requirements may vary, providers should refer to specific payer policy.

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PLANNED ADMISSION

If the initial cell collection is conducted during an inpatient hospital admission and patient is discharged prior to subsequent admission for administration of SKYSONA, it may be necessary to confirm with each payer that the subsequent admission is a planned admission for gene therapy administration.

SKYSONA

Some payers may establish prior authorizations based on clinical trial inclusion and exclusion criteria, which may require documentation or physician attestation for specific diagnostic testing and clinical history.

It is recommended that you verify all authorization requirements and/or review any established medical policies with the individual payers.

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Sample Letter of Medical Necessity

SKYSONA

Clicking on the image below will open the Sample Letter of Medical Necessity in a new window.

SKYSONA™ (elivaldogene autotemcel)
Sample Letter of Medical Necessity

To the Treating Physician:

This sample letter, provided by Genetix Biotherapeutics is for informational purposes only, providing an example of language that may be required or helpful when responding to a request from a patient's health plan. Use of this information does not constitute medical or legal advice and does not guarantee reimbursement for coverage. It is not intended to be a substitute for, or an influence on, the independent clinical decision of the prescribing healthcare professional. Please note that some payers may have specific forms that must be completed in order to request prior authorization or to document medical necessity. When sending this information to a third-party payer for review, ensure that you submit under your practice/individual physician letterhead.

The following pages are a sample that may be customized to use as a statement of medical necessity/appeal for your patients. Use of this sample letter is not required.

Indication

SKYSONA is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD) without an available human leukocyte antigen (HLA)-matched donor for allogeneic hematopoietic stem cell transplant. Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9.
This indication is approved under accelerated approval based on 24-month Major Functional Disability (MFD)-free survival. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

Limitations of Use
SKYSONA does not prevent the development of or treat adrenal insufficiency due to adrenoleukodystrophy.

An immune response to SKYSONA may limit the persistence of descendent cells of SKYSONA, causing rapid loss of efficacy of SKYSONA in patients with full deletions of the human adenosine triphosphate binding cassette, sub family D, member 1 (*ABCD1*) gene.

SKYSONA has not been studied in patients with CALD secondary to head trauma.

Given the risk of hematologic malignancy with SKYSONA, and unclear long-term durability of SKYSONA and human adrenoleukodystrophy protein (ALDP) expression, careful consideration should be given to the appropriateness and timing of treatment for each boy, especially for boys with isolated pyramidal tract disease since their clinical symptoms do not usually occur until adulthood.

Please see Important Safety Information on pages 2-5 and full Prescribing Information, including **Boxed WARNING**.
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Additional support for medical necessity may be required for new patients. Resources available through Genetix CARES include the above Letter of Medical Necessity sample, which can be downloaded and adapted to reflect your patient's prior treatment journey and clinical rationale for treatment with SKYSONA.

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Indication and Important Safety Information[1]

INDICATION

SKYSONA is indicated to slow the progression of neurologic dysfunction in boys 4-17 years of age with early, active cerebral adrenoleukodystrophy (CALD). Early, active cerebral adrenoleukodystrophy refers to asymptomatic or mildly symptomatic (neurologic function score, NFS ≤ 1) boys who have gadolinium enhancement on brain magnetic resonance imaging (MRI) and Loes scores of 0.5-9.

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An immune response to SKYSONA may limit the persistence of descendent cells of SKYSONA, causing rapid loss of efficacy of SKYSONA in patients with full deletions of the human adenosine triphosphate binding cassette, sub family D, member 1 (*ABCD1*) gene.

SKYSONA has not been studied in patients with CALD secondary to head trauma.

Given the risk of hematologic malignancy with SKYSONA, and unclear long-term durability of SKYSONA and human adrenoleukodystrophy protein (ALDP) expression, careful consideration should be given to the appropriateness and timing of treatment for each boy, especially for boys with isolated pyramidal tract disease based on available treatment options since their clinical symptoms do not usually occur until adulthood.

IMPORTANT SAFETY INFORMATION

WARNING: HEMATOLOGIC MALIGNANCY

Hematologic malignancies, including life-threatening cases of myelodysplastic syndrome and acute myeloid leukemia, have occurred in patients treated with SKYSONA. Patients have been diagnosed between 14 months and 7.5 years after SKYSONA administration, and the cancers appear to be the result of the SKYSONA lentiviral vector, Lenti-D, integration in proto-oncogenes. Monitor patients closely for evidence of malignancy through complete blood counts at least every 3 months. Monitor patients through assessments for evidence for clonal expansion or predominance at least twice in the first year and annually thereafter; consider bone marrow evaluations as clinically indicated.

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Important Safety Information[1] (cont'd)

Hematologic Malignancy

Hematologic malignancies, including myelodysplastic syndrome (MDS) and acute myeloid leukemia (AML) have developed in patients treated with SKYSONA in clinical studies between 14 months and 7.5 years after SKYSONA administration. Malignancies are life-threatening and death related to treatment for malignancy has occurred. As of April 2024, hematologic malignancies have been diagnosed in 6/67 (9%) clinical study patients. At diagnosis, all patients had high-frequency integrations in oncogenes; most of which were in *MECOM*. Pathological diagnoses ranged between MDS-unilineage dysplasia to acute myeloid leukemia. Most patients required chemotherapy with or without allogeneic hematopoietic stem cell transplant.

SKYSONA Lenti-D lentiviral vector integration into proto-oncogenes, including *MECOM*, appears to have mediated the cases of hematologic malignancy. All patients treated with SKYSONA in clinical studies have integrations into *MECOM*; it is unknown which integrations into *MECOM* or other proto-oncogenes are likely to lead to malignancy.

Because of the risk of hematologic malignancy, carefully consider alternative therapies including allogeneic hematopoietic stem cell transplant for patients who have a suitable, willing, and available matched sibling donor, prior to the decision to treat a child with SKYSONA.

Consider consultation with hematology experts prior to SKYSONA treatment to inform benefit-risk treatment decision and to ensure adequate monitoring for hematologic malignancy. Consider performing the following baseline hematologic assessments: complete blood count with differential, hematopathology review of peripheral blood smear, and bone marrow biopsy (core and aspirate) with flow cytometry, conventional karyotyping, and next generation sequencing (NGS) with a molecular panel appropriate for age and including coverage for gene mutations expected in myeloid and lymphoid malignancies; and testing for germline mutations that are associated with hematologic malignancy.

Early diagnosis of hematologic malignancy can be critically important, therefore, monitor patients treated with SKYSONA lifelong for hematologic malignancy. For at least the first fifteen years after treatment with SKYSONA, monitor via complete blood count (with differential) at least every 3 months and via integration site analysis or other testing for evidence of clonal expansion and predominance at least twice in the first year and then annually. Consider appropriate expert consultation and additional testing such as more frequent complete blood count (with differential) and integration site analysis, bone marrow studies, and gene expression studies in the following settings after treatment with SKYSONA:

- Delayed or failed engraftment of platelets or other cell lines (while all patients are at risk for hematologic malignancy, patients who do not achieve unsupported platelet counts of ≥ 20 × 10^9/L on or after Day 60 appear to be at higher risk); or
- New or prolonged cytopenias; or
- Presence of clonal expansion or predominance (e.g., increasing relative frequency of an integration site, especially if ≥ 10% and present in *MECOM* or another proto-oncogene known to be involved in hematologic malignancy).

If hematologic malignancy is detected in a patient who received SKYSONA, contact Genetix Biotherapeutics at 1-833-999-6378 for reporting and to obtain instructions on collection of samples for further testing.

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Serious Infections

Severe infections, including life-threatening and fatal infections, have occurred in patients after SKYSONA infusion. Important opportunistic infections that have been diagnosed within the first 3 months after treatment with SKYSONA include BK cystitis, cytomegalovirus reactivation, human herpesvirus-6 viremia, candidiasis, and bacteremias. Opportunistic infections after the first 3 months include an atypical mycobacterium vascular device infection, pseudomonas bacteremia, and Epstein-Barr virus reactivations diagnosed as late as 18 months after treatment with SKYSONA. Serious infections involving adenovirus include a case of transverse myelitis at 6 months that was attributed to adenovirus and entero/rhinovirus infection, and a fatal adenovirus infection at 21 months in a patient with CALD progression who developed multisystem organ failure.

Grade 3 or higher infections occurred in 21% of all patients (12% bacterial, 3% viral, and 6% unspecified). The most common Grade 3 or higher infections were vascular device infections (7% of patients) diagnosed as late as 6 months after treatment with SKYSONA, and bacteremias (6% of patients) diagnosed as late as 8 months after treatment with SKYSONA.

Febrile neutropenia was commonly observed in clinical studies and may be a sign of a serious infection. In the event of febrile neutropenia, evaluate for infection and manage with broad-spectrum antibiotics, fluids, and other supportive care as medically indicated.

Monitor patients for signs and symptoms of infection before and after SKYSONA administration and treat appropriately. Administer prophylactic antimicrobials according to best clinical practices and clinical guidelines.

Avoid administration of SKYSONA in patients with active infections.

Prolonged Cytopenias

Patients may exhibit cytopenias, including pancytopenia, for > 1 year following conditioning and SKYSONA infusion.

Grade 3 or higher cytopenias on or after Day 60 following SKYSONA infusion occurred in 47% of patients and included low platelet count (14%), low neutrophil count (22%), low lymphocyte count (27%), and low hemoglobin (2%). Grade 3 cytopenias persisted beyond Day 100 in 15% of patients and included low platelet count (7%), low neutrophil count (9%), and low lymphocyte count (6%).

Serious adverse reactions of pancytopenia occurred in two patients who required support with blood and platelet transfusions as well as growth factors (G-CSF for up to 6 months and eltrombopag for up to 14 months) after SKYSONA administration. One patient had intercurrent parvovirus infection and his pancytopenia was ongoing at least two years after SKYSONA administration. Pancytopenia in the other patient was ongoing until he was diagnosed with myelodysplastic syndrome approximately two years after SKYSONA administration.

Monitor blood counts until normalization and assess patients for signs and symptoms of bleeding and/or infection prior to and after SKYSONA administration.

Delayed Platelet Engraftment

Delayed platelet engraftment (platelet count ≤ 50×10^9/L beyond 60 days after treatment with SKYSONA) has been observed. Bleeding risk is increased prior to platelet engraftment and may continue after engraftment in patients with prolonged thrombocytopenia.

Patients should be made aware of the risk of bleeding until platelet recovery has been achieved. Monitor patients for thrombocytopenia and bleeding according to standard guidelines. Conduct frequent platelet counts until platelet engraftment and platelet recovery are achieved. Perform blood cell count determination and other appropriate testing whenever clinical symptoms suggestive of bleeding arise.

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Please see additional Important Safety Information on page 17 and full Prescribing Information, including **Boxed WARNING**.

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Risk of Neutrophil Engraftment Failure

There is a potential risk of neutrophil engraftment failure after treatment with SKYSONA. Neutrophil engraftment failure was defined as failure to achieve 3 consecutive absolute neutrophil counts (ANC) ≥ 0.5×10^9 cells/L obtained on different days by Day 43 after infusion of SKYSONA. Monitor neutrophil counts until engraftment has been achieved. If neutrophil engraftment failure occurs in a patient treated with SKYSONA, provide rescue treatment with the back-up collection of CD34+ cells.

Hypersensitivity Reactions

Allergic reactions may occur with the infusion of SKYSONA. The dimethyl sulfoxide (DMSO) in SKYSONA may cause hypersensitivity reactions, including anaphylaxis which is potentially life-threatening and requires immediate intervention.

Anti-retroviral Use

Patients should not take anti-retroviral medications for at least one month prior to mobilization or the expected duration for elimination of the medications, and until all cycles of apheresis are completed. Anti-retroviral medications may interfere with manufacturing of the apheresed cells.

If a patient requires anti-retrovirals for HIV prophylaxis, mobilization and apheresis of CD34+ cells should be delayed until HIV infection is adequately ruled out.

Laboratory Test Interference

SKYSONA affects polymerase chain reaction (PCR) assays for HIV due to LVV provirus insertion. A PCR based assay should not be used to screen for HIV infection in patients treated with SKYSONA as a false positive test result is likely.

Adverse Reactions

Most common non-laboratory adverse reactions (≥ 20%): mucositis, nausea, vomiting, febrile neutropenia, alopecia, decreased appetite, abdominal pain, constipation, pyrexia, diarrhea, headache, rash.

Most common Grade 3 or 4 laboratory abnormalities (≥ 40%): leukopenia, lymphopenia, thrombocytopenia, neutropenia, anemia, hypokalemia.

Vaccines

Vaccination is not recommended during the 6 weeks preceding the start of myeloablative conditioning, and until hematological recovery following treatment with SKYSONA. Where feasible, administer childhood vaccinations prior to myeloablative conditioning for SKYSONA.

Males of Reproductive Potential

Advise patients of the risks associated with mobilization and conditioning agents.

Males capable of fathering a child and their female partners of childbearing potential should use an effective method of contraception (intra uterine device or combination of hormonal and barrier contraception) from start of mobilization through at least 6 months after administration of SKYSONA.

Data are available on the risk of infertility with myeloablative conditioning. Advise patients of the option to cryopreserve semen before treatment if appropriate.

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Please see Important Safety Information on pages 14-17 and full Prescribing Information, including Boxed WARNING.

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GENETIX™ Biotherapeutics

Sample CMS-1450 (UB-04) Claim Form for Inpatient Hospital Admissions

This sample form is provided by Genetix Biotherapeutics Inc. for informational purposes only and is not intended to be directive or construed as medical or legal advice. Healthcare providers should exercise independent clinical judgment when selecting codes and submitting claims. The accurate completion of a claim is the responsibility of the healthcare provider. There is no guarantee regarding reimbursement for any service or item. Since requirements may vary, providers should refer to specific payer policies.

Please see Important Safety Information on pages 14-17 in the SKYSONA™ (elivaldogene autotemcel) Billing and Coding Guide and full Prescribing Information, including **Boxed WARNING**.

Field 4: TYPE OF BILL
Enter the appropriate type of bill code. For example, 011X for an inpatient hospital facility.

Fields 39-41: VALUE CODES[11]
Payers may require a Value Code to document acquisition cost of SKYSONA™. Enter the appropriate value code(s). For example, Value Code 87 is defined as: Invoice/aquisition cost of modified bioligics. For use with Revenue Category 0892

42 REV. CD.	43 DESCRIPTION	44 HCPCS / RATE / HIPPS CODE	45 SERV. DATE	46 SERV. UNITS	47 TOTAL CHARGES	48 NON-COVERED CHARGES
0892	Charges for Modified Gene Therapy	J3590	MM DD YY	1	$XXX.XX	
	N473554211101UN					
0874	Cell/Gene Therapy Infusion of Modified Cells	96413	MM DD YY	[XX]	$XXX.XX	

Field 42: REVENUE CODE
Enter the appropriate revenue code for each reported line. For example, 0892 Charges for Gene Therapy

Field 43: DESCRIPTION
Enter the 11-digit NDC with an appended N4 qualifier (electronic equivalent: Loop 2410, LIN02). Enter the appropriate description for corresponding revenue code(s).

Field 44: HCPCS
Enter the appropriate HCPCS Level II code(s) and applicable modifier(s). For example, J3490 or J3590. In lieu of a gene therapy-specific code for the infusion, CPT code 96413 may be required.

Field 45: SERVICE DATE
Enter the corresponding date(s) of service.

Field 46: SERVICE UNITS
Enter the appropriate unit(s) of service. For unclassified HCPCS, such as J3490 & J3590, 1 unit may be required.

Field 47: TOTAL CHARGES
Enter the total charges for each reported line.

Fields 66 & 69: DIAGNOSIS CODES
Enter the appropriate diagnosis codes. For example, E71.520 childhood cerebral X-linked adrenoleukodystrophy.

66 DX: E71.520

69 ADMIT DX: E71.520

Field 74: PRINCIPAL PROCEDURE
Enter the relevant ICD-10-PCS code(s) with corresponding date(s) of service. Transfusion coding may be used such as 30233C0 or 30243C0 until SKYSONA™-specific coding is issued by CMS.

74 PRINCIPAL PROCEDURE CODE: 302XXXX DATE: MM DD YY

Field 80: REMARKS
Enter relevant product information when reporting a miscellaneous HCPCS code. For example, drug name, dosage, NDC number, route of administration and Genetix Biotherapeutics Patient ID.

80 REMARKS: [NAME]; [DOSAGE]; NDC; IV INFUSION

SAMPLE

UB-04 CMS-1450 APPROVED OMB NO. 0938-0997 NUBC™ National Uniform Billing Committee THE CERTIFICATIONS ON THE REVERSE APPLY TO THIS BILL AND ARE MADE A PART HEREOF.

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