Policy:	GG.1102
Title:	**Experimental and Investigational Service Coverage**
Department:	Medical Management
Section:	Utilization Management
CEO Approval:	*/s/ Michael Hunn 01/29/2024*
Effective Date:	02/01/2002
Revised Date:	12/31/2023
Applicable to:	☒ Medi-Cal ☒ OneCare ☐ PACE ☐ Administrative

I. PURPOSE

This policy defines the benefit coverage for Experimental and Investigational Services under the CalOptima Health program.

II. POLICY

A. Experimental Services are not covered under the Department of Health Care Services (DHCS) Medi-Cal program, Whole Child Model (WCM) program, or the Centers for Medicare & Medicaid Services (CMS) Medicare or Duals Demonstration programs, except as specified in this Policy or specifically authorized by law.

B. Coverage of Investigational Services in the Medi-Cal program.

1. Investigational Services require Prior Authorization, and are covered when it is clearly documented that all of the following apply:

   a. The Member has a life threatening or seriously debilitating disease or medical condition;

   b. There is a medically reasonable expectation that the Investigational Service will significantly prolong the intended Member's life or will maintain or restore a range of physical and social function suited to Activities of Daily Living (ADL);

   c. Conventional therapy will not prevent progressive disability or premature death, nor adequately treat the intended Member's condition;

   d. The provider of the proposed service has a record of safety and success, which is equivalent or superior to that of other providers of the Investigational Service;

   e. The Investigational Service is the lowest cost item or service that meets the Member's medical needs and is less costly than all conventional alternatives; and

   f. The service is not being performed as a part of a research study protocol.

2. For WCM Members, CalOptima Health may provide coverage for an Investigational Service if:

    a. It is approved by the Federal Drug Administration (FDA) under any Investigational New Drug (IND) Application; or

    b. It is authorized for use under the state of California's Right to Try Act; and

    c. Its use is consistent with its FDA-approved IND Application or the state of California's Right to Try Act.

C. Coverage of Investigational Services in the OneCare program

1. Category A and Category B Investigational Device Exemption (IDE) studies require Prior Authorization, and are covered when the following requirements are met:

    a. The principal purpose of the study is to test whether the device improves health outcomes of appropriately selected patients;

    b. The rationale for the study is well supported by available scientific and medical information, or it is intended to clarify or establish the health outcomes of interventions already in common clinical use;

    c. The study results are not anticipated to unjustifiably duplicate existing knowledge;

    d. The study design is methodologically appropriate, and the anticipated number of enrolled subjects is adequate to confidently answer the research question(s) being asked in the study;

    e. The study is sponsored by an organization or individual capable of successfully completing the study;

    f. The study is in compliance with all applicable Federal regulations concerning the protection of human subjects found in Title 21 of the Code of Federal Regulations (CFR), Parts 50, 56, and 812, and Title 45, CFR, Part 46;

    g. Where appropriate, the study is not designed to exclusively test toxicity, or disease pathophysiology, in healthy individuals. Studies of all medical technologies measuring therapeutic outcomes as one (1) of the objectives may be exempt from this criterion, only if the disease or condition being studied is life threatening and the patient has no other viable treatment options;

    h. The study is registered with the National Institutes of Health's National Library of Medicine's ClinicalTrials.gov;

    i. The study protocol describes the method and timing of release of results on all pre-specified outcomes, including release of negative outcomes, and that the release should be hastened if the study is terminated early; and

    j. The study protocol must describe how Medicare beneficiaries may be affected by the device under investigation, and how the study results are or are not expected to be generalizable to the Medicare beneficiary population. Generalizability to populations eligible for Medicare due to age, disability, or other eligibility status must be explicitly described.

D. CalOptima Health shall cover cancer Clinical Trials, in accordance with CalOptima Health Policy GG.1125: Cancer Clinical Trials.

E. Coverage for routine costs of qualifying Clinical Trials are covered upon approval by the Chief Medical Officer (CMO) or their Designee, unless the coverage of the service is otherwise defined as a non-covered service or item in Title 22, California Code of Regulations (C.C.R).

F. Coverage for Clinical Trials is restricted to participating hospitals and physicians in California, unless the protocol for the Clinical Trial is not provided for at a California hospital or by a California physician.

G. For the Medi-Cal program, CalOptima Health or a Health Network shall provide coverage for routine costs for Investigational Services associated with a Clinical Trial, if the Clinical Trial meets the following requirements:

   1. The subject or purpose of the trial must be the evaluation of an item or service that falls within the benefit category of the Medicaid/Medicare program (e.g., Physician's service, Durable Medical Equipment (DME), diagnostic test) and are not statutorily excluded from coverage (e.g., cosmetic surgery).

   2. The trial must not be designed exclusively to test toxicity, or disease pathophysiology. It must have therapeutic intent and be considered a Phase III Clinical Trial in the United States.

   3. Trials of therapeutic interventions must enroll patients with a diagnosed disease, rather than healthy volunteers. Trials of diagnostic interventions may enroll healthy patients, in order to have a proper control group.

H. The requirements in Section II.G. of this Policy are insufficient by themselves to qualify a Clinical Trial for the Medicare Clinical Trial registry and OneCare coverage for routine costs. A Clinical Trial shall have the following characteristics:

   1. The principal purpose of the Clinical Trial is to test whether the intervention potentially improves the Member's health outcomes;

   2. The Clinical Trial is well-supported by available scientific and medical information, or is intended to clarify, or establish, the health outcomes of interventions already in common clinical use;

   3. The Clinical Trial does not unjustifiably duplicate existing studies;

   4. The Clinical Trial design is appropriate to answer the research question being asked in the Clinical Trial;

   5. The Clinical Trial is sponsored by a credible organization or individual capable of executing the proposed Clinical Trial successfully;

   6. The Clinical Trial is in compliance with Federal regulations relating to the protection of human subjects; and

   7. All aspects of the Clinical Trial are conducted according to the appropriate standards of scientific integrity.

I. Payment will not be authorized for Investigational Services that do not meet the criteria set out in this Policy, or for associated inpatient care, when a Member needs to be in the hospital primarily because they are receiving such non-approved Investigational Services. For non-covered items and services, only the treatment of complications arising from the delivery of the non-covered item or service, and unrelated reasonable and necessary care is a covered benefit.

III. PROCEDURE

A. All requests for Investigational Services or Experimental Services, including Clinical Trials, must be reviewed and authorized by the CMO or their Designee prior to services being provided to Members.

1. For WCM Members, requests shall be evaluated for Medical Necessity in accordance with California Children's Services (CCS) guidelines as provided in CCS Numbered Letters.

2. In addition, for WCM Members, CalOptima Health shall authorize Investigational Services or Experimental Services in accordance with GG.1508: Authorization and Processing of Referrals or CCS Numbered Letters, whichever is least restrictive and as applicable.

B. Determination that a service is Experimental or Investigational is based on:

1. Relevant Federal regulations or guidance, such as those contained in Title 42, CFR, Chapter IV, and Title 21, CFR, Chapter I, Food and Drug Administration (FDA) 510k approvals, and certain other categories of FDA approval for limited use or continuing research, do not represent full and unrestricted FDA acceptance and would still be considered investigational. Further, devices or therapies which are fully FDA approved for some indications but are being used for indications other than those for which there is an FDA approval may also be deemed investigational;

2. Verification that the Clinical Trial meets the criteria for being registered with the Medicare clinical trials registry;

3. Consultation with provider organizations, academic and professional specialists pertinent to the specific medical service; and

4. Current medical literature in the United States.

C. Routine costs in Clinical Trials include:

1. Items or services that are typically provided absent a Clinical Trial (e.g., conventional care);

2. Items or services required solely for the provision of the Investigational Service or item (e.g., administration of a non-covered chemotherapeutic agent);

3. The clinically appropriate monitoring of the effects of the item or service, or the prevention of complications; and

4. Items or services needed for reasonable and necessary care arising from the provision of an investigational item or service, in particular, for the diagnosis or treatment of complications.

D. Routine costs exclude the following:

1. The Investigational Service or item;

2. Items and services provided solely to satisfy data collection and analysis needs that are not used in the direct clinical management of the patient (e.g., monthly CT scans for a condition usually requiring only a single scan);

3. Items and services customarily provided by the research sponsors free of charge for any enrollee in the trial; and

4. Services not directly associated with health care, such as travel, housing, companion expenses, and other non-clinical expenses associated with the Clinical Trial.

E. A Member may appeal a CalOptima Health or Health Network decision to a requested service in accordance with CalOptima Health Policies GG.1510: Member Appeal Process, MA.9003: Standard Pre-Service Appeal, MA.9004: Expedited Pre-Service Appeal, and MA.9015: Standard Integrated Appeals.

IV. ATTACHMENT(S)

Not Applicable

V. REFERENCE(S)

A. CalOptima Health Contract with the Centers for Medicare & Medicaid Services (CMS) for Medicare Advantage
B. CalOptima Health Contract with the Department of Health Care Services (DHCS) for Medi-Cal
C. CalOptima Health, Health Network Service Agreement
D. CalOptima Health Policy GG.1125: Cancer Clinical Trials
E. CalOptima Health Policy GG.1508: Authorization and Processing of Referrals
F. CalOptima Health Policy GG.1510: Member Appeal Process
G. CalOptima Health Policy MA.9003: Standard Pre-Service Appeal
H. CalOptima Health Policy MA.9004: Expedited Pre-Service Appeal
I. CalOptima Health Policy MA.9015: Standard Integrated Appeals
J. Centers for Medicare & Medicaid Services, National Coverage Determination for Routine Costs in Clinical Trials, July 9, 2007
K. DHCS CCS Numbered Letter (N.L.) 05-1020: California Children's Services Program and Genetically Handicapped Persons Program Policy on Coverage of Experimental and Investigational Services
L. Final National Coverage Decision-Clinical Trials, Social Security Act, Section 1862 (a)(1)(E)
M. Medicare Managed Care Manual, Chapter 4, Section 10.7.2
N. Medicare Benefit Policy Manual, Chapter 14, Section 20
O. Medicare Approved Clinical Trials/Clinical Research Studies List
P. Title 21, Code of Federal Regulations, Chapter I
Q. Title 22, California Code of Regulations, §§51056.1, 51303 (g) and (h)
R. Title 42, Code of Federal Regulations, Chapter IV
S. Title 42, United States Code, §300gg-8
T. Health and Safety Code §111548.2
U. Welfare and Institutions Code, §14132.98

VI. REGULATORY AGENCY APPROVAL(S)

Date	Regulatory Agency	Response
08/18/2021	Department of Health Care Services (DHCS)	Approved as Submitted

VII. BOARD ACTION(S)

Date	Meeting
06/03/2021	Regular Meeting of the CalOptima Board of Directors

VIII. REVISION HISTORY

Action	Date	Policy	Policy Title	Program(s)
Effective	02/01/2002	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal
Revised	05/01/2007	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal
Revised	09/01/2014	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal
Revised	11/01/2015	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	10/01/2016	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	08/01/2017	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	12/01/2018	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	06/01/2020	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	06/03/2021	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare OneCare Connect
Revised	12/31/2022	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare
Revised	12/31/2023	GG.1102	Experimental and Investigational Service Coverage	Medi-Cal OneCare

IX. GLOSSARY

Term	Definition
Activities of Daily Living (ADL)	Personal everyday activities including, but not limited to, tasks such as eating, toileting, grooming, dressing, and bathing.
Category A Experimental Device	A device for which absolute risk of the device type has not been established, that is, initial questions of safety and effectiveness have not been resolved, and the Food and Drug Administration (FDA) is unsure whether the device type can be safe and effective.
Category B Non-Experimental/Investigational Device	A device for which the incremental risk is the primary risk in question, that is, initial questions of safety and effectiveness of that device type have been resolved, or it is known that the device type can be safe and effective because, for example, other manufacturers have obtained Food and Drug Administration FDA premarket approval or clearance for that device type.
Clinical Trials	Trials certified to meet the qualifying criteria and funded by National Institute of Health, Centers for Disease Control and Prevention, Food and Drug Administration (FDA), Department of Veterans Affairs, or other associated centers or cooperative groups funded by these agencies. Criteria for Clinical Trials include the following characteristics: 1. The principal purpose of the Clinical Trial is to test if the intervention potentially improves a participant's health outcomes; 2. The Clinical Trial is well supported by available scientific and medical information or is intended to clarify or establish the health outcomes of interventions already in common clinical use; 3. The Clinical Trial does not unjustifiably duplicate existing studies; 4. The Clinical Trial is designed appropriately to answer the research question being asked in the trial; 5. The Clinical Trial is sponsored by a credible organization or individual capable of successfully executing the proposed Clinical Trial; 6. The Clinical Trial complies with federal regulations relating to the protection of human subjects; and 7. All aspects of the Clinical Trial are conducted according to the appropriate standards of scientific integrity.
Designee	A person selected or designated to carry out a duty or role. The assigned designee is required to be in management or hold the appropriate qualifications or certifications related to the duty or role.
Durable Medical Equipment (DME)	Medically Necessary medical equipment that is prescribed for the Member by Provider and is used in the Member's home, in the community or in an institution that is used as a home. DME: 1. Can withstand repeated use. 2. Is used to serve a medical purpose. 3. Is not useful to an individual in the absence of an illness, injury, functional impairment, or congenital anomaly. 4. Is appropriate for use in or out of the patient's home.

Term	Definition
Experimental Services	Drugs, equipment, procedures, or services that are in a testing phase undergoing laboratory or animal studies prior to testing in humans.
Investigational Services	Drugs, equipment, procedures, or services for which laboratory and animal studies have been completed and for which human studies are in progress but testing is not complete (Phase III clinical trials are not yet completed and published), the efficacy and safety of such services in human subjects are not yet established, and the service is not generally accepted by the medical community in the United States or in widespread general medical usage in the United States.
Medically Necessary or Medical Necessity	Medi-Cal: Reasonable and necessary Covered Services to protect life, to prevent significant illness or significant disability, or alleviate severe pain through the diagnosis or treatment of disease, illness, or injury, as required under W&I Code 14059.5(a) and Title 22 CCR Section 51303(a). Medically Necessary services shall include Covered Services necessary to achieve age-appropriate growth and development, and attain, maintain, or regain functional capacity. For Members under 21 years of age, a service is Medically Necessary if it meets the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) standard of medical necessity set forth in Section 1396d(r)(5) of Title 42 of the United States Code, as required by W&I Code 14059.5(b) and W&I Code Section 14132(v). Without limitation, Medically Necessary services for Members under 21 years of age include Covered Services necessary to achieve or maintain age-appropriate growth and development, attain, regain or maintain functional capacity, or improve, support or maintain the Member's current health condition. CalOptima Health shall determine Medical Necessity on a case-by-case basis, taking into account the individual needs of the child. OneCare: Reasonable and necessary medical services to protect life, to prevent significant illness or significant disability, or alleviate severe pain through the diagnosis or treatment of disease, illness, or injury, as required under W&I Code 14059.5(a) and Title 22 CCR Section 51303(a). Medically Necessary services includes Medi-Cal Services necessary to achieve age-appropriate growth and development, and attain, maintain, or regain functional capacity.
Member	A beneficiary enrolled in a CalOptima Health program.
Prior Authorization	Medi-Cal: A formal process requiring a health care Provider to obtain advance approval of Medically Necessary Covered Services, including the amount, duration, and scope of services, except in the case of an emergency. OneCare: A process through which a physician or other health care provider is required to obtain advance approval from CalOptima Health and/or a delegated entity, that payment will be made for a service or item furnished to a Member.