



State of California—Health and Human Services Agency
Department of Health Care Services



GAVIN NEWSOM
GOVERNOR

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TO: All County California Children's Services Program and Genetically Handicapped Persons Program Staff, Medical Consultants, Statewide Consultants, and Integrated Systems of Care Division Staff

SUBJECT: California Children's Services Program and Genetically Handicapped Persons Program Policy on Coverage of Experimental and Investigational Services

I. PURPOSE

This Numbered Letter (N.L.) updates policy on the coverage of experimental and investigational drugs, biological products, and devices under the California Children's Services (CCS) Program and the Genetically Handicapped Persons Program (GHPP).

The CCS Program publishes this N.L. under the program's authority to authorize services that are medically necessary to treat CCS-eligible conditions.^{1,2,3}

II. BACKGROUND

In general, the U.S. Food and Drug Administration (FDA) must approve a new drug for general use.⁴ Prior to approving a new drug for general use, the FDA may authorize its use in a clinical investigation. A clinical investigation is an experiment in which a drug is administered to one or more human subjects and consists of three phases.^{5,6} A new drug under a clinical investigation is an "investigational drug."

The FDA may also authorize the use of an investigational drug to treat exceptionally ill patients under limited circumstances.^{7,8} Additionally, federal law authorizes the use of investigational drugs outside of the FDA-approval process in other limited circumstances.⁹

The State of California's Right to Try Act also authorizes the use of investigational services outside of the FDA-approval process under limited circumstances.¹⁰ Moreover, the State of California's Right to Try Act also authorizes a health care plan to cover investigational services.¹⁰

For the purposes of this document, the following terms are defined as follows:

A. Investigational service:

A drug, biological product, or device that has successfully completed phase one of a clinical investigation approved by the FDA, but that has not been approved for general use by the FDA and remains under investigation in an FDA approved clinical investigation.

B. Experimental services:

Drugs, equipment, procedures or services that are in a testing phase undergoing laboratory and/or animal studies prior to testing in humans. Experimental services are not undergoing a clinical investigation.⁵

III. POLICY

A. The CCS Program and GHPP will not provide coverage for experimental services unless specifically authorized by law.

B. The CCS Program and GHPP may provide coverage for an investigational service if:

1. It is approved by the FDA under any Investigational New Drug (IND) Application; or
2. It is authorized for use under the State of California's Right to Try Act; and
3. Its use is consistent with its FDA-approved IND Application or the State of California's Right to Try Act;

C. Additional criteria that will be considered in the adjudication process include:

1. Conventional therapy will not adequately treat the intended patient's condition;
2. Conventional therapy will not prevent progressive disability or premature death;
3. The provider of the proposed service has a record of safety and success with it or equivalent to that of other providers of the investigational services;
4. Other criteria (e.g., cost and availability) may be considered in the adjudication of a given request in cases in which more than one investigational service is available;

5. There is reasonable expectation that the investigational service will significantly prolong the patient's life or will maintain or restore a range of physical and social function suited to activities of daily living; and
6. The service is not being performed as part of a research study protocol. For a beneficiary with cancer who participates in a clinical trial for cancer, California Health and Safety Code (HSC) § 1370.6 requires that all routine patient care costs related to the clinical trial be covered if the beneficiary's CCS-paneled treating physician recommends participation in the clinical trial after determining that participation in the clinical trial has a meaningful potential to benefit the enrollee. The coverage does not include investigational services that have not been approved by the FDA and that are associated with the clinical trial.

D. The CCS Program and GHPP will authorize inpatient care associated with the administration of the investigational service only in situations in which the patient's underlying clinical status requires the medical necessity of acute hospital care.

E. Whole Child Model (WCM) Counties

For CCS clients who are enrolled in a Medi-Cal managed care plan (MCP) and reside in a WCM county, the client's MCP shall be responsible for authorizing, coordinating, and covering experimental and investigational services. MCPs operating in WCM counties should use the authorization guidelines described in this N.L., or utilize the MCP's existing experimental and investigational services policies, whichever is less restrictive.

IV. POLICY IMPLEMENTATION

A. Experimental and investigational services are not included in Service Code Grouping authorizations.¹¹ Providers should submit a separate Service Authorization Request (SAR) and all supporting documentation in the following manner:

1. For clients residing in a county covered by the WCM, SARs for investigational services shall be submitted to, and processed by, the MCP.
2. For clients residing in all other counties, SARs for investigational services should be submitted to the California Children's Services Network (CMS Net) for initial review. After confirming that all required supporting documents are present, county CCS personnel will notify ISCD via email at

ISCD-MedicalPolicy@dhcs.ca.gov or to secure RightFax number,

(916) 440-5768.

B. The SAR shall include the following information:

1. Documentation of each criterion listed in section III.B and III.C.
2. A complete description of the investigational service being requested:
 - a. Name of service.
 - b. Phase of clinical testing.
 - c. Manufacturer name, contact information, and approval of product use in this beneficiary.
 - d. Specific administration parameters, e.g., dosing and duration.
 - e. IND letter from the FDA for individuals who have received an FDA-approved IND Application.
3. Name of CCS-paneled sub-specialist physician who will administer the investigational service.
4. Name of CCS-approved Special Care Center or facility at which administration of the investigational service will occur.

If you have any questions regarding this N.L., please contact the ISCD Medical Director or designee at ISCD-MedicalPolicy@dhcs.ca.gov.

Sincerely,

ORIGINAL SIGNED BY

Roy Schutzengel
Medical Director
Integrated Systems of Care Division

¹ 22 Cal. Code Regs. § 41515.1 et. seq. Determination of Medical Eligibility
<https://govt.westlaw.com/calregs/Document/I28E30090D4B811DE8879F88E8B0DAAAE?viewType=FullText&originContext=documenttoc&transitionType=CategoryPageItem&contextData=%28sc.Default%29>

² 22 Cal. Code Regs. § 41700 Availability
[https://govt.westlaw.com/calregs/Document/I2F1A7E70D4B811DE8879F88E8B0DAAAE?viewType=FullText&originContext=documenttoc&transitionType=CategoryPageItem&contextData=\(sc.Default\)&bhcp=1&ignorebhwarn=IgnoreWarns](https://govt.westlaw.com/calregs/Document/I2F1A7E70D4B811DE8879F88E8B0DAAAE?viewType=FullText&originContext=documenttoc&transitionType=CategoryPageItem&contextData=(sc.Default)&bhcp=1&ignorebhwarn=IgnoreWarns)

³ 22 Cal. Code Regs. § 41740 Eligibility for Treatment Services
[https://govt.westlaw.com/calregs/Document/I2FDD8050D4B811DE8879F88E8B0DAAAE?viewType=FullText&originContext=documenttoc&transitionType=CategoryPageItem&contextData=\(sc.Default\)&bhcp=1&ignorebhwarn=IgnoreWarns](https://govt.westlaw.com/calregs/Document/I2FDD8050D4B811DE8879F88E8B0DAAAE?viewType=FullText&originContext=documenttoc&transitionType=CategoryPageItem&contextData=(sc.Default)&bhcp=1&ignorebhwarn=IgnoreWarns)

[tionContext=documenttoc&transitionType=StatuteNavigator&contextData=%28sc.Default%29](#)

⁴ 21 U.S.C. § 355 New Drug

<https://www.law.cornell.edu/uscode/text/21/355>

⁵ 21 C.F.R. § 312.3(b) Drugs for Human Use

<https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcr/CFRSearch.cfm?fr=312.3>

⁶ 21 C.F.R. § 312.21 Drugs for Human Use

<https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcr/CFRSearch.cfm?fr=312.21>

⁷ 21 C.F.R. § 312.23

<https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcr/CFRSearch.cfm?fr=312.23>

⁸ 21 C.F.R. § 312.310

<https://www.accessdata.fda.gov/scripts/cdrh/cfdocs/cfcr/CFRSearch.cfm?fr=312.310>

⁹ 21 U.S.C. § 360bbb-0a(b).

<https://www.law.cornell.edu/uscode/text/21/360bbb>

¹⁰ Health & Safety Code § 111548.2

https://leginfo.ca.gov/faces/codes_displayText.xhtml?lawCode=HSC&division=104.&title=&part=5.&chapter=6.&article=4.5

¹¹ Service Authorization Request Tools

<https://www.dhcs.ca.gov/services/ccs/cmsnet/Pages/SARTools.aspx#service>