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Department of Health Care Services
Children's Medical Services Network
(CMS Net) - Information Bulletin #344

SARs for Investigational Drugs Under an Investigational New Drug (IND) Application - Revised

On August 13, 2009, the Food and Drug Administration (FDA) published 21 Code of Federal Regulation Part 312 and 316: Charging for Investigational Drugs Under an Investigational New Drug Application; Expanded Access to Investigational Drugs for Treatment Use; Final Rules.

This final rule, effective October 2009, revises and clarifies circumstances for appropriate charging for investigational drugs in clinical trials and describes criteria for charging, under new rules for expanded access to investigational drugs under an IND application.

The Medi-Cal Program has requested clarification and direction from the Centers for Medicare & Medicaid Services. Until clarification is received, the Department is advising that non-FDA approved investigational use of drugs, when medically necessary, be authorized as an Early and Periodic Screening, Diagnostic and Treatment (EPSDT) Supplemental Services (SS). Non-FDA approved drugs will not have a NDC (National Drug Code). Some of these drugs must be purchased outside the United States and will require specific instructions on the SAR.
At the time of issuance of this document, drugs that are currently being authorized are:

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- Omegaven – an omega-3 fish oil emulsion used in total parenteral nutrition (TPN) dependent infants and children with cholestatic liver disease. This product is very effective in reversing the liver disease and, in many cases, avoiding the path to liver transplant.
- ~~Erwinase (erwinia chrysanthemi L-asparaginase for injection) used to treat patients with acute lymphoblastic leukemia (ALL) who have allergic reactions to currently available L-asparaginase preparations.~~
- ~~Clobazam – antiepileptic drug used primarily for patients with Lennox-Gasteaux Syndrome and Dravet Syndrome~~
- Stiripentol – antiepileptic drug used in Dravet Syndrome

When a SAR request is for a non-FDA approved drug, the following steps shall be taken:

- CCS county staff shall
  - obtain documentation as required by NL #37-1292 and NL #09-0899 to allow determination of medical necessity for the requested drug,
  - verify that the request is for outpatient use only.
- CCS county medical consultant and/or designee shall review the request and information obtained to assure that
  - the request is related to the CCS eligible medical condition,
  - medical reports supplied are appropriate for the request and indicate dosing and/or plan for administration.
- County CCS case management staff shall
  - enter pending EPSDT-SS SAR requiring state approval for Medi-Cal clients or state approved SAR for Healthy Families or a CCS-Only client that is only for the drug(s) being requested.
    - Choose ”Investigational Services” from the drop down box
    - Check the EPSDT box for those who have Medi-Cal eligibility, full scope, no share of cost,

        - Use code Z5999.
        - Enter number of units to be authorized.
        - In Special Instructions
            - state what the unit is equivalent to. For example, Omegaven 1 unit = 100 ml vial,
            - enter EPSDT-SS specific instructions,
            - enter the following text only for requests requiring purchase out of the United States: "Provider to include both original invoice and payment transaction showing payment in U.S. dollars and conversion from Euros" (or the supplying countries currency if different).
    - Complete EPSDT-SS worksheet
    - Forward to the Chief, Medical Policy & Consultation Section (fax to: 916-440-5312)
        - medical information after review by the local medical consultant,
        - completed EPSDT-SS worksheet.

- Chief, Medical Policy & Consultation Section shall review submitted material to determine
    - medical necessity,
    - whether the requested drug meets criteria for investigational new drug and EPSDT-SS,
    - whether the requesting provider has submitted appropriate documentation as required by the FDA.
- Chief, Medical Policy & Consultation Section will coordinate with the county's designated case management liaison for this request if additional documentation or information is needed from the requesting provider.

- If there is medical necessity and appropriate FDA documentation, Chief, Medical Policy & Consultation Section will
    - document case review in CMS-Net case notes,
    - issue authorization directly or via designee,
    - notify CCS county liaison that authorization has been issued. County CCS program is responsible for printing and distributing the authorization.

Any subsequent requests for the drug after the initial authorization ends shall follow the same procedure as above. Under no circumstances shall county CCS staff issue authorizations for these drugs without state medical consultant review.

Any related requests for supplies or equipment shall be authorized separately by the county after notification to the county that the investigational drug has been authorized.

- If the prescriber/provider is unfamiliar with the FDA requirement to submit a request for approval to charge for investigational drugs, inform the provider of this requirement. Some helpful links to the FDA website:

  http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm172492.htm

  http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm107434.htm

  http://www.fda.gov/Drugs/DevelopmentApprovalProcess/HowDrugsareDevelopedandApproved/ApprovalApplications/InvestigationalNewDrugINDApplication/ucm071073.html

If you have any questions, please contact:

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