

Aurora Health Care

Clinical Trials

**Emergency Use (of an Investigational Device or Drug) Coverage/Billing
(Often incorrectly referred to as Compassionate Use)**

Date: March, 2004

PROCEDURE STATEMENT:

All Aurora Health Care employees have a responsibility to maintain a high level of integrity in the clinical trial process, including the accuracy of claims submitted for hospital services to the Part-A Fiscal Intermediary or the Part-B Carrier for Medicare related to the emergency use of an investigational device or drug.

PURPOSE OF PROCEDURE:

The purpose of this procedure is to assist individuals involved in the billing of research related claims as pertaining specifically to the concept or terminology "emergency use." (Emergency use is sometimes referred to as "compassionate use" though there is no compassionate use terminology in billing, only emergency use.)

ACCOUNTABILITY:

The Central Business Office (CBO) Special Projects Representative (SPR) through whom all research related claims flow, in conjunction with a Clinical Research Coordinator who identifies the "emergency (compassionate) use" of a drug or device with a patient. Additionally the Medical Audit Coverage Analyst will make a final determination as to the accuracy of claim submission to Medicare.

BACKGROUND:

Although the term "compassionate use" has been used in the past to refer to the provision of investigational drugs, biologics and devices outside of an ongoing clinical trial to a limited number of patients who are desperately ill and for whom no standard alternative therapies are available, the term "compassionate use" does not appear in FDA or DHHS regulations. Further, CMS regulations do not contemplate coverage for "compassionate" use outside of an emergency. Rather, the medical necessity and reasonableness of these claims are considered on a case-by-case basis. For purposes of this billing policy, use of the term compassionate use to refer to emergency situations and various access mechanisms causes more confusion than it does assistance. It is preferable, instead, to use the names of the specific access programs when discussing the use of investigational articles outside of formal clinical trials.

First, for FDA-regulated studies, generally the physician or investigator must obtain prospective approval from the Aurora IRB and meet specific requirements in order to conduct research in emergency situations. Thus, compliance with all Aurora IRB policies is required. However, the FDA human subjects regulations do allow for a test article to be used in emergency situations without prior IRB approval provided that the emergency use is reported to the IRB within five working days; subsequent use of the test article must be reviewed by the IRB [21 CFR 56.104].

Second, various FDA regulations and policies allow certain persons **not enrolled in clinical trials** to obtain access to investigational drugs, biologics and devices:

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- The emergency use of an investigational drug, device or biologic requires an IND or IDE. If the intended subject does not meet the criteria of an existing study protocol, or if an approved study protocol does not exist, the usual procedure is to contact the manufacturer and determine if the drug, device or biologic can be made available for the emergency use under the company's IND or IDE.
- A Treatment IND (Investigational New Drug) is a treatment protocol that is added to an existing investigational new drug application, which allows physicians to treat qualifying patients according to the protocol, and which provides additional data on the drug's safety and effectiveness. Treatment INDs are available for patients with life-threatening or other serious diseases for which no satisfactory alternative drug or other therapy exists [21 CFR 312.34].
- A "single patient use" allows a physician to obtain access to an investigational drug for the treatment of a single patient. Usually, the patient is in a desperate situation and unresponsive to other therapies, or in a situation where no approved or generally recognized treatment is available. Further, there is usually little evidence that the proposed therapy is useful, but may be plausible on theoretical grounds or anecdotes of success. Access to investigational drugs for use by a single, identified patient may be gained either through the sponsor under a treatment protocol, or through the FDA, by first obtaining the drug from the sponsor, and then submitting a treatment IND to the FDA requesting authorization to use the investigational drug for treatment use [21 CFR 312.35].
- The Parallel Track mechanism makes available promising investigational agents as quickly as possible to persons with AIDS and other HIV-related diseases while generating data on the safety and effectiveness of the drug [Federal Register 57 (April 15, 1992): 13250-13259]. Under the FDA policy, persons with AIDS and HIV-related diseases who are not able to take standard therapy or for whom standard therapy is no longer effective, and who are not able to participate in ongoing controlled clinical trials would have access to promising investigational drugs. Applications for consideration of experimental therapies for Parallel Track expanded availability must be submitted to the FDA as amendments to existing INDs.
- IDE supplements

See Guidebook Chapter 2, Section B, "Food and Drug Administration Regulations and Policies" for a more detailed description of these mechanisms.

Third, CMS regulations also generally require an IND or IDE before consideration for coverage of the investigational item and/or related services can be made. Cross-reference to policies you have on billing investigational drugs and devices in general or cite to the NCD and category B regs?

In summary, a physician may request emergency (incorrectly referred to as "compassionate") use of a drug or device by seeking approval from both the drug or device manufacturer and the FDA. A physician would make an emergency use (compassionate) request when s/he believes that the use of an investigational drug or device is in the best interest of his/her patient and without an emergency (compassionate) use exception, the patient would not have access to a potentially lifesaving drug/device.

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DEFINITIONS:

- **Clinical Research Coordinator** – Most often the nurse (or health care professional) assigned to or assisting the Principal Investigator responsible for carrying out the defined protocol of the clinical trial.
- **CMS** - Centers for Medicare & Medicaid (formerly known as HCFA; Health Care Financing Administration)
- **Compassionate Use** – An often inappropriately used term, incorrectly used interchangeably with the term “emergency use.” Note also that Medicare does not recognize the term “compassionate use” as it relates to the billing for “compassionate” or “emergency” use drugs or devices.
- **Emergency** - An emergency is defined as a life-threatening situation in which no standard acceptable treatment is available and in which there is not sufficient time to obtain IRB approval [21 CFR 56.102(d)]. [See Guidebook Chapter 2, Section B, "Food and Drug Administration Regulations and Policies."]
- **Life-threatening** – At immediate risk of death

PROCEDURES:

1. It is the responsibility of the physician to obtain manufacturer, FDA and IRB approval to use a drug or device in an emergency situation. (See also IRB Policy xx-xxx for IRB guidelines.) Because from a practical standpoint, it is often the physician’s Clinical Research Coordinator (CRC) who is involved in this process, the CRC is likely to participate in the preparation and submission of the approval documentation.
2. It is a requirement for the CRC to also notify the Special Billing Representative (SBR) in Aurora’s Central Business Office regarding the emergency (compassionate) use of a drug or device. In order to prevent a possible incorrect claim from producing electronically in an automated format, the CRC must notify the SBR within 24-hours of use.
3. In order to determine whether the drug or device employed during the applicable emergency is billable to a federal program, the SBR will flag the patient account using the established research account protocol, appending a “99” code to the account. (This stops all automated claims processing.)
4. The CRC will then notify the Medical Auditor Research Analyst of the emergency (compassionate) use of a device or drug and ask for a coverage determination.
5. The Medical Auditor Research Analyst will determine whether this particular emergency (compassionate) use of the drug or device is appropriate for billing to a federal program following the detailed procedures set-forth in Aurora’s Coverage Analysis Process and taking into consideration the individual facts and circumstances behind the emergency use.

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6. The Medical Auditor will forward to the SPR appropriate billing instructions from the claim analysis.
7. SPR will submit claim(s) for emergency (compassionate) drug or device use to Medicare in accordance with the guidance provided by the Medical Auditor's direction.
8. The SPR will follow all applicable, existing investigational billing procedures to accurately complete the UB claim form.