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MEDICAID DRUG REBATE PROGRAM NOTICE **Release No. 185**

For State Technical Contacts

State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway

This release specifies that a drug approved by the Food and Drug Administration (FDA) under its "accelerated approval" pathway, which is the approval program authorized under section 506(c) of the Federal Food, Drug, and Cosmetic Act (FFDCA), (https://www.fda.gov/ForPatients/Approvals/Fast/ucm405447.htm), must be covered by state Medicaid programs, if the drug meets the definition of "covered outpatient drug" as found in Section 1927 of the Social Security Act (the Act).

Section 1927(k)(2)(A)(i) the Act defines a covered outpatient drug, to include a drug "…which is approved for safety and effectiveness as a prescription drug under section 505 or 507 of the [FFDCA]…" Since section 506(c)(1)(A) of the FFDCA provides that an accelerated approval for a drug product is an approval under section 505(c) of the FFDCA, such a drug meets the definition of covered outpatient drug, under section 1927(k)(2) of the Act when used for a medically accepted indication as defined in section 1927(k)(6) of the Act. Section 506(e)(2) of the FFDCA further provides that section does not alter the standards of evidence required under section 505(c) for approval, including the standards regarding whether a product is safe and effective. We note that the FDA accelerated approval process also applies to products licensed under section 351(a) of the Public Health Service Act, which are generally biological products, including vaccines. Such biologicals would also fall under the definition of covered outpatient drug at section 1927(k)(2)(B) of the Act. However, we note that as indicated in section 1927(k)(2)(B),vaccines do not fall under the definition of covered outpatient drug under section 1927(k) of the Act.

Therefore, as with any other drug, if the drug is labeled by a manufacturer that has signed a Medicaid National Drug Rebate Agreement, and the drug meets the definition of covered outpatient drug, then the drug is covered by the Medicaid Drug Rebate Program (MDRP) and is to be covered by state Medicaid programs. If the FDA subsequently withdraws its approval, the drug would no longer meet the definition of a covered outpatient drug and would not be covered under the MDRP. Also, section 1927(k)(6) of the Act defines medically accepted indication, in part, to mean "any use for a covered outpatient drug which is approved under the Federal Food, Drug, and Cosmetic Act," and section 1927(k)(3) of the Act specifically limits the definition of covered outpatient drug to exclude when a drug is "used for a medical indication which is not a medically accepted indication."

In summary, this release clarifies that drugs that are granted "accelerated approval" are drugs approved by FDA under section 505(c) of the FFDCA, and are able to satisfy the definition of covered outpatient drug, and if used for a medically-accepted indication, then the drug must be covered by state Medicaid programs if the manufacturer has an applicable signed Medicaid national drug rebate agreement for participation in the MDRP. States can use utilization management mechanisms such as prior authorization to assure appropriate use of these medications.

Background on FDA's Accelerated Approval Program

Section 506(c) of the FFDCA allows the FDA to grant accelerated approval to a drug for a serious or life-threatening disease or condition. Part of the criteria for accelerated approval under section 506(c) is a demonstrated effect on either:

a. A surrogate endpoint that is reasonably likely to predict a clinical benefit, taking into account severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments, or
b. A clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

Drugs granted accelerated approval by FDA under the process described in 506(c) of the FFDCA are approved under section 505(c) of the FFDCA and must meet the same statutory evidentiary standards for safety and effectiveness as those granted traditional approvals. See section 506(e)(2) of the FFDCA. Thus, as noted above, at the time a product is granted accelerated approval, FDA has based such an approval on a determination that the drug has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint other than survival or irreversible morbidity.[1]

If you have any questions regarding this topic, please contact RxDrugPolicy@cms.hhs.gov.

Sincerely,

/s/

Michael Nardone
Director
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[1] See sections 505(a), 505(c), 506(c), and 506(e)(2) of the FFDCA; see also 21 CFR 314.510