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China FDA Solicits Comments on Conditional Approvals for New Drugs and Compassionate Use of Investigational Drugs

The China Food and Drug Administration ("CFDA") recently proposed two draft circulars for public comments, namely (a) the *Technical Guidelines for Conditional Approvals for Urgently Needed Drugs* (*"Draft Conditional Approvals Guidelines"*) and (b) the *Administrative Measures for Compassionate Use of Investigational Drugs ("Draft Compassionate Use Measures")*. The CFDA is soliciting public comments for these drafts until mid-January 2018. These two drafts echoed the central government's recent reform to encourage innovation in drugs and medical devices. If adopted, these drafts are likely to streamline and accelerate the market access of new drugs and allow Chinese patients to have early access to innovative therapies.

1. 1. Conditional approvals will be granted to provide early access to innovative drugs which address severe life-threatening diseases without any effective cures. The Draft Conditional Approvals Guidelines propose the eligibility criteria and application process for conditional approvals.

    1. Only innovative drugs not yet marketed in China that can treat severe illness, life-threatening diseases, or rare diseases are eligible for conditional approvals.

    2. The CFDA may grant conditional approvals to allow early marketing authorization of the innovative drugs, if any of the following occurs:

        a. The surrogate endpoint or intermediate clinical endpoint indicate the efficacy and clinical values of the innovative drugs;

        b. The early and mid-stage study data indicate the innovative drugs' efficacy and can predict their clinical values (Notes: A confirmatory clinical study must be completed after the conditional approval is issued in this case); or

        c. Any orphan drugs that been marketed out of China.

    3. For any new drug applications ("NDA") that may be eligible for conditional approvals, the applicant may apply for a meeting with the Center for Drug Evaluation ("CDE") to discuss the possibility of conditional approvals and relevant requirements.

    4. The NDA applicant should apply for conditional approval when filing for the NDA.

    5. For an orphan drug that has been marketed in foreign counties, the NDA applicant can apply for conditional approval based on the foreign study data used to support the foreign marketing approval. Once the conditional approval is granted, the applicant should finish the clinical study requirements regarding ethnicity differences as soon as possible.

2. The Draft Compassionate Use Measures set forth the definition, the purposes, the criteria, and the application process. Compassionate use of investigational products is regulated as if it were a clinical study, and can be deemed an expanded access study.

    1. When patients do not meet the enrollment criteria, they may take the investigational drugs through compassionate use.

    2. The target group of the compassionate use program is limited to patients having life-threatening diseases or diseases that have a severe impact on the quality of life and require early intervention due to the lack of any other satisfactory therapies.

    3. Patients may be eligible for the compassionate use, if any of the following occurs:

        a. The patient is not eligible for any ongoing clinical studies of the investigational drugs;

        b. The patient is unable to participate in any clinical studies due to limitations in his/her location or other reasons; or

        c. The clinical studies of the investigational drug were completed, but the investigational drug has not been marketed in China; the available research data can indicate the effectiveness and safety of the investigational drug on relevant patients.

    4. The NDA applicant must submit an application to CDE for an approval regarding the expanded access study. The CDE will come to a decision within 30 days.

    5. For any patient participating in the expanded access study, their physicians must evaluate and conclude that the probable risks to the patient from the investigational drug are no greater than the probable risks from the disease, and the patient must give informed consent to such compassionate use.

    6. The data generated from the expanded access study can be used as supportive safety data in the NDA application dossier.

We recommend that pharmaceutical and medical device companies provide their comments on these two draft rules and closely monitor their implementation.