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FDA Issues Draft Guidance on Diagnostic Tests Used With Targeted Drug Therapies

On July 14, 2011, the U.S. Food and Drug Administration (FDA) issued a new draft guidance to facilitate the development and review of companion diagnostics—tests designed to be used by healthcare professionals to select the appropriate patient for a particular therapy or to optimize a dosing regimen.[1] Given that the diagnostic device is "essential" to the safe and effective use of a particular therapeutic product, and that the device will be included in the therapeutic product's labeling, the FDA does not expect, as a general rule, to approve such therapeutic product applications unless the device is also approved.

These *in vitro* companion diagnostic devices (IVD companion diagnostic devices) are increasingly attractive to drug developers because of the emergence of new technologies that make it possible to personalize medical therapies for individual patients. Indeed, when an appropriate scientific rationale supports such an approach, the FDA encourages development of therapeutic products that depend on the use of approved or cleared IVD companion diagnostic devices.[2]

The draft guidance recommends early engagement between the FDA and developers. It explains that sponsors developing either the therapeutic product or the IVD companion diagnostic device should consult with both relevant device and therapeutic review divisions within the FDA to ensure the product development plans will produce sufficient data to meet safety and efficacy approval requirements for the product pair. Meeting this approval standard is critical because inadequate performance of an IVD companion diagnostic device can produce results that would lead to withholding appropriate therapy or to administering inappropriate therapy.

The draft guidance provides that when the use of an IVD companion diagnostic device is essential for the safe and effective use of a novel therapeutic product, the IVD companion diagnostic device and therapeutic product

1 *Draft Guidance for Industry and Food and Drug Administration Staff /* In Vitro *Companion Diagnostic Devices*. See 76 Fed. Reg. 41,506 (July 14, 2011). The draft guidance, when finalized, will represent the FDA's current thinking on the topic, but it does not operate to bind the FDA or the public.

2 One common type of companion diagnostic looks for whether a patient has a specific gene amplification or protein over-expression that could predict whether a drug might benefit the patient or lead to harm. For example, in 1998, the FDA approved Herceptin (trastuzumab), a breast cancer drug that targeted the HER2 gene amplification or HER2 protein expression. The drug was approved with a companion test and today testing is routinely performed on women diagnosed with breast cancer to help physicians determine whether the patient should receive Herceptin.

should be approved or cleared contemporaneously by the FDA. However, the proposed guidance also identifies instances where the FDA may approve a targeted medicine in the absence of a cleared or approved companion diagnostic. For example, where the therapeutic product is intended to treat a serious or life-threatening disease or condition for which there is no available or satisfactory treatment and when the potential benefits outweigh the risks of not having a cleared or approved companion diagnostic, the therapy could be approved even though the IVD companion diagnostic device is still awaiting approval or clearance. In those circumstances, when the IVD companion diagnostic device is subsequently approved, the therapeutic product label will be revised to include the IVD companion diagnostic device.

The draft guidance also:

1. Clarifies the FDA's definition of an IVD companion diagnostic device.

2. Declares that the FDA will generally not approve a supplement to stipulate the use of an IVD companion diagnostic device for an approved therapeutic product until the IVD companion diagnostic device has been approved or cleared.

3. Notes that the FDA will expect a therapeutic product sponsor to address the need for an approved or cleared IVD companion diagnostic device in its therapeutic product development plan. The sponsor can either develop its own diagnostic device or partner with a diagnostic device sponsor. Alternatively, the sponsor can explore modification of an existing IVD companion diagnostic device, either its own or another sponsor's, to accommodate the appropriate intended use.

4. Explains that the FDA will apply a risk-based approach to determine the regulatory pathway for IVD companion devices, as it does with all medical devices.

5. Confirms that the labeling for an IVD companion diagnostic device must specify the therapeutic product(s) or class of therapeutic product for which it has been approved or cleared.

6. Addresses labeling for therapeutic products and IVD companion diagnostic devices.

7. Specifies that diagnostic devices and therapeutic products which are to be studied together to support their respective approvals can be evaluated in the same investigational study.

The FDA is seeking public comment on the draft guidance for 60 days. Comments should be submitted to the FDA online or in writing by September 12, 2011.