Expansion of the Humanitarian Use Device Program under the 21st Century Cures Act

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The 21st Century Cures Act, signed into law in December 2016, ushered in a number of changes that will affect the Food and Drug Administration's product development programs. One such example relates to the expansion of the FDA's Humanitarian Use Device Program (HUD Program).

The new law provides FDA with the authority to apply the HUD program to devices that treat diseases and conditions that affect up to 8,000 patients per year in the United States. The current cap is 4,000. In addition, the new law requires the agency to issue draft guidance to define the criteria for establishing "probable benefit," as that term is used in the Federal Food, Drug, and Cosmetic Act.

As these changes are expected to encourage more medical device development for rare conditions and diseases, we will provide a high-level overview of the HUD program, the new law's changes, and AGG observations.

The HUD Program in a Nutshell

- The HUD Program essentially centers around the ability of FDA to approve a Premarket Approval Application (PMA) for a humanitarian use device (HUD) without a demonstration of efficacy so long as the following requirements are met:
  - An applicant must first demonstrate to FDA's satisfaction that the device qualifies as a humanitarian use device (HUD).
  - The applicant must then submit a Humanitarian Device Exemption (HDE), which allows FDA to exempt the device from the efficacy requirements typically required for a PMA provided it is shown that the device will not expose patients to an unreasonable or significant risk of illness or injury, and the probable benefit to health from the device's use outweighs the risk of injury or illness from its use, taking into consideration the probable risks and benefits of currently-available devices or alternative forms of treatment.[1]
- If FDA approves an HDE, the applicant may market the HUD, but there are significant profit and use restrictions.
- With limited exceptions (not discussed here), HUDs approved under an HDE cannot be sold for profit, and can only be used in a facility after an Institutional Review Board has approved the use in the facility (although there are exceptions in certain emergencies).
- User fees are waived for HDE applications.
- Since 1997, the FDA has approved 65 medical devices through the HDE/HUD process.[2]

The Cure's Act Changes to the HUD Program

- As noted, the HUD program was intended to benefit those with rare diseases or conditions, i.e. affecting fewer than 4,000 individuals in the U.S.

1 See, e.g., 21 U.S.C. § 360j(m) § and 21 C.F.R. § 814.3(m); see also Humanitarian Device Exemption (HDE): Questions and Answers-- draft guidance for HDE Holders, Institutional Review Boards, Clinical Investigators, and the Food and Drug Administration Staff (March 2014), http://www.fda.gov/RegulatoryInformation/Guidances/ucm389154.htm

2 http://www.fda.gov/MedicalDevices/ProductsandMedicalProcedures/DeviceApprovalsandClearances/HDEApprovals/ucm161827.htm

- The new law doubles the number from 4,000 to 8,000 patients, thereby increasing the patient population eligibility criteria for orphan diseases or conditions.
- The new law requires the FDA to issue a draft guidance document within 18 months of the new law's enactment to define criteria for establishing a "probable benefit" to health (in the context of use risk/benefit analysis).

AGG Observations

- Congress has attempted to pave the way for increased development of medical devices for orphan diseases and conditions.
- With the doubling of the patient population eligibility criteria for orphan diseases, medical device companies may take a more renewed interest in developing products for a limited audience, where it is not required to establish a product's efficacy and a user fee waiver is possible (compared to a PMA user fee payment of more than $230,000).
- A clarification by FDA of the "probable benefit," standard, as required by the new law, may help a medical device firm evaluate whether its specific product may meet the HDE/ HUD threshold.
- The clarity of the draft guidance document, to be issued within 18 months, will be the critical factor in the success of this expansion of the HUD program.
- Consequently, it is too early to tell if Congressional hope turns into reality, but the new law is a step in the right direction.

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