

Regulatory Considerations in the Development of Medical Devices for Rare Diseases

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Humanitarian use Devices or HUD is defined as medical devices used for purposes of diagnosis or treatment of diseases affecting no more than 8000 patients in the U.S. per year. The HUD application facilitates marketing of devices for use in the rare disease population. The FDA approves the investigation of the device by the manufacturer, sponsor and individual investigator in collecting safety and efficacy data. The FDA recognizes the challenge of obtaining sufficient and statistically meaningful data in a small population.

If the device is not intended to be widely marketed, then the Human Device holder may obtain an approval from the FDA for a Human Device Exemption or HDE. The HDE restricts profitability and lessens the burden of efficacy data collection prior to IRB approval. The FDA and IRB must approve significant risk devices prior to use (21 CFR 812) [1,2]. Some examples of significant risk devices (SR) include sutures, shunts, and orthopedic implants.

The FDA office of orphan products (OOPD) advances the evaluation and development of products, including biologics, devices and medical foods that hold promise in the treatment of rare disease. The office encourages the discovery and use of such devices and may help to fund their development. This effort is in part related to the difficulty in establishing efficacy when the device is used only by a small number of patients in the U.S.

There are different regulatory distinctions between devices used for clinical use only, without intent to market the device, and the pre-marketing studies or investigations required pre-marketing for FDA approval. If the device is only intended for clinical use, the applicant is required to have IRB approval and to provide a summary of safety and benefits of the device when used clinically. A patient information packet is provided to the patient, and is a key resource describing the device. If no packet is available, information that includes an explanation that the HUD is designed to diagnose or to treat the condition is written. The physician describes known risks, postulated mechanisms and rationale of use, and then may acknowledge that effectiveness has not been established.

The Medical Device Reporting or MDR requirements 21 CFR 803 [3]. 2014, specify that clinicians and user facilities provide medical device reports to the HDE holder or sponsor, FDA and IRB. If there has been a serious injury while the HUD is being used, the HDE provides reports that are sent to the FDA and all reviewing IRBs.

The IRB and the sponsor-investigator each have roles in assessing risk for the HUD. If there has been an HDE approved, and there is no pre marketing FDA application, the IRB assesses the risk of use for a new indication. If there are severe risks, then the FDA will be contacted prior to use in a non-emergent situation.

In conclusion, the development of novel medical devices to treat and to diagnose rare conditions involves coordinated efforts between the Sponsor-Investigator, IRB and FDA. There are federal regulatory requirements specific to the use and development of humanitarian medical devices for the rare disease population, as well as funding incentives to encourage future progress in the treatment of special disease populations.

References

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2. FDA. 21 CFR 814. Department of Health and Human Services, United States.
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