NATIONAL CENTER FOR HEALTH RESEARCH
The Voice For Prevention, Treatment And Policy

MDUFA: A Patient-Centered Public Health Perspective

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Disclosures

The National Center for Health Research is a nonprofit think tank that focuses on the safety and effectiveness of medical and consumer products and does not accept funding from companies that make those products.

FDA Approval

FDA requires evidence that devices are reasonably safe and reasonably effective, defined as having benefits that outweigh the risks for most patients. How do MDUFA performance goals and other funded activities premarket and postmarket ensure those criteria are met?

Is MDUFA Patient-Centered?

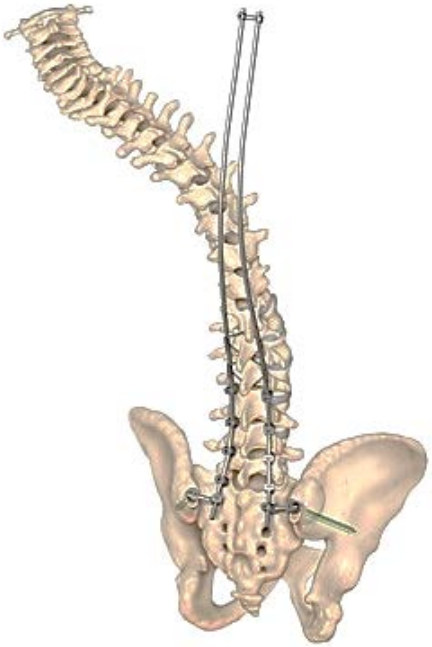
- **Performance data are currently based on speed. Those standards are usually met.**
- **Performance data should also be based on patient-centered outcomes**
- **MDUFA does NOT address many issues most important to patients**

Is MDUFA Patient-Centered?

- **510(k) clearance rarely requires evidence of safety or effectiveness and almost never includes clinical trials. How to make informed choices?**
- **De Novo and PMA usually include case studies or clinical trials, but often have no comparison groups**
- **Are user fees sufficient to support scrutiny by FDA scientific staff?**

Substantial Equivalence: DePuy VIPER Spinal System

Differences: added or modified parts, new complex systems have not been tested



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Substantial Equivalence: Nonthermal Shortwave Diathermy Devices for Pain

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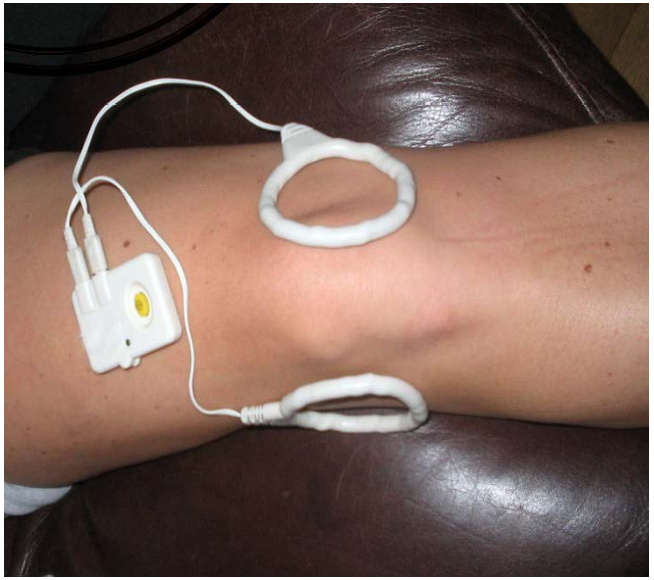

Are these substantially equivalent?

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How can MDUFAimprove ?

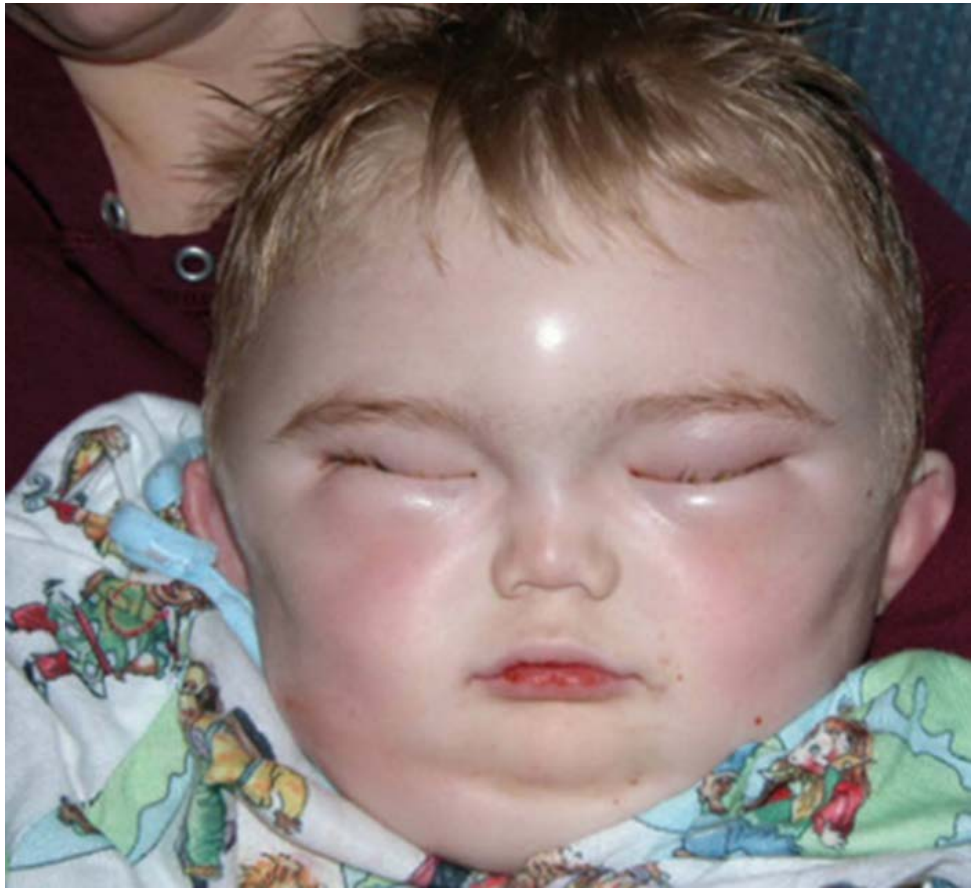
- **When good data are lacking prior to clearance, granting De Novo, or PMA, post-market surveillance are especially important.**

- **Device user fees are inadequate to support excellent post-market surveillance**

How else can MDUFA improve ?

- **Are clinical studies too small or short-term?**
- **How good are safety or effectiveness data for male and female patients of different ages and race/ethnicity?**
- **Are DTC ads misleading? Other promotional activities?**

2-year old Treated with Infuse
St. Louis Children's Hospital
2008 published case report



Current Insufficient FDA Warning

Product:

Certain recombinant proteins and synthetic peptides mimic bone growth substances normally found in the body and may be added to a carrier or scaffold to be used as bone graft substitutes. Once combined, these products are surgically implanted in a patient with a bone defect to promote new bone growth or to replace or heal existing bone.

Recommendations:

The FDA recommends against routine use of these products in patients under age 18 because their safety and effectiveness has not been reviewed or approved for use in this population.

How can MDUFAimprove (cont'd)?

- **MDUFA should support FDA staff to improve safeguards for off-label use. FDA should develop patient and provider materials to explain off label risks and target off-label uses that are known to be ineffective or unsafe.**

- **Are detailing activities, DTC ads, or ads to doctors directly or indirectly promoting inappropriate off label use?**

How can MDUFAimprove info for patients and providers?

- **MDUFA should provide support for FDA staff to create Patient Booklets, Informed Consent Checklists, etc.**
- **MDUFA should support comprehensive postmarket surveillance**
- **MDUFA should support FDA “Dear Doctor” letters, warnings to patients**

- **Enforce clinical trial requirements**

- **Adverse event reports, registries, and other real world data can supplement not replace controlled clinical trials**

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