

Medical Devices for Rare Diseases: FDA/NIH Needs Assessment Project

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What we will cover today...

- Overview of the FDA/NIH Needs Assessment Project
 - Why now?
 - Goals ?
 - Who will oversee the project and who can contribute?
 - What has been done so far?
 - What does the current proposal look like?
- Conversation about the Needs Assessment Project
 - Consider the relevant questions together
 - Gather input to improve the current proposal

Catalyst for Conducting a Needs Assessment Now

- Institute of Medicine (IOM) Rare Diseases and Orphan Products Report (2010)
 - *RECOMMENDATION 7-1: FDA and NIH should collaborate on an assessment of unmet device needs and priorities relevant to rare diseases. That assessment should focus on the most plausible areas of unmet need, identify impediments to meeting these needs, and examine options for overcoming impediments and stimulating high priority innovations.*
- FDA Section 740 Rare and Neglected Diseases Report to Congress (2011)



What are the goals of the Needs Assessment Project?

- Big Picture: Help address unmet needs for rare disease patients
- How?
 - Begin to identify unmet medical device needs (including therapeutics and diagnostics) for rare diseases
 - Sub-focus on pediatric population
 - Humanitarian use device considerations
 - Understand the extent of the need in rare disease populations
 - Generate meaningful data to inform patients/practitioners/developers
 - Increase public awareness of device needs for rare diseases
 - E.g., Publish findings

Leads for Project: FDA/NIH



FDA

- Office of Orphan Products Development
- Center for Devices and Radiological Health
- Office of Planning and Policy

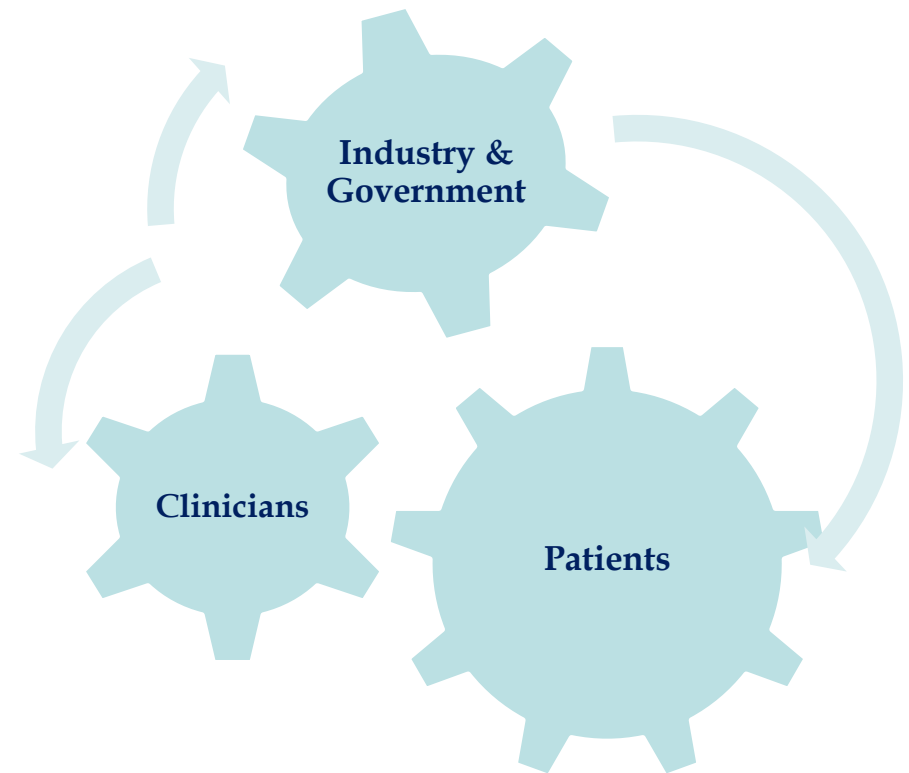


NIH

- NCATS Office of Rare Disease Research

Additional Participating Stakeholders

- Advanced Medical Technology Association
- American Academy of Pediatrics
- American Medical Association
- Medical Devices Manufacturers Association
- National Organization for Rare Disorders



(Note: ICF International contractor for implementation)

What is a “rare disease”?

- For purposes of the Needs Assessment Project, “rare disease” is defined as < 200,000 persons in the U.S.
 - Same definition as the Orphan Drug Act (as opposed to the Humanitarian Use Devices (HUD) definition of < 4,000 persons/year)
 - Same as the eligibility criteria for funding device clinical trials through the OOPD grant program
 - Helps assess how well the HUD criteria addresses rare disease device needs
 - Allows inclusion of diagnostics for rare diseases

What is an “unmet need”?

Potential Definition for Needs Assessment Project...

- When there are no approved devices for the treatment or diagnosis of a disease or condition or when a novel device could provide a significant clinically meaningful advantage over existing approved devices
 - *Question under consideration:* Is there still an unmet need for purposes of this needs assessment if there are approved drugs or biologics?



Work to Date



- Determined scope and primary focus
 - Scope: Evaluate rare disease device needs across a broad spectrum of diseases/body systems, rather than focusing in-depth on limited areas (e.g., cardiovascular device needs)
 - Primary Focus: Identifying device needs for rare diseases, rather than focusing on barriers to development
- Developed a concept paper and initial methods for approaching the needs assessment
 - Considered Paperwork Reduction Act and other issues

Work to Date



- Held a kickoff stakeholder meeting and, with ICF's assistance, conducted an Expert Elicitation
 - Obtained feedback on how to conduct the needs assessment (e.g., what the goals are, who to target for this information, how to obtain it, etc.)
- Based on research and feedback received to date, drafting a proposed plan on how to perform the needs assessment

Moving Forward – Proposed Plan

- Draft study plan for conduct of assessment (*January 2014*)
- Focus Group/Interviews (*end of 2014*)
 - Advisory Committees (AC) and FDA/NIH experts
 - Broad/deep expertise in devices from academic/clinical/industry/patient representatives
 - Pediatric Device Consortia/Medical Device Innovation Consortium (MDIC)
 - Access to broad device development stakeholders
 - Additional Stakeholder Focus Groups as appropriate
- Public Meeting/Stakeholder Workshop (*end of 2014*)
 - Federal Register (FR) notice that solicits input
 - Allows for transparency, inclusion, and new perspectives
- Analysis and Draft Publication (*June 2015*)



Summary

- Seeking collaborative effort with many stakeholders to identify unmet medical device needs for rare diseases
- Generate meaningful data that can inform patients/practitioners/developers
- Time for conversation on these topics...



Federal Register Questions...

Conversation starters

- Describe the parameters that should be used in determining priority areas of development of devices, including both therapeutic and diagnostic devices in pediatric rare diseases
 - Challenge: How best to formulate and prioritize key questions as we conduct a cross-cutting assessment of needs
- What is the best approach to conduct needs assessment of medical devices required for use with pediatric rare diseases?
 - Challenge: How to perform a cross-cutting analysis but include a sub-focus on pediatric devices and humanitarian use device questions

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