Promotion of Early Patient Access to Medical Devices in the U.S. and Japan – US Regulatory View on Current and Prospective Activities

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CDRH Overview

• CDRH’s commitment to ensuring patients and providers have timely and continued access to safe, effective, and high-quality medical devices does not occur in a vacuum

• Engage with global regulatory, clinical, and industry stakeholders to leverage available resources and information and promote regulatory consistency and predictability
CDRH Strategic Priorities: 2018 - 2020

• Employee Engagement, Opportunity, and Success
• Simplicity
• Collaborative Communities

Our Measure of Success:
“By December 31, 2020, more than 50 percent of manufacturers of novel technologies for the U.S. market intend to bring their devices to the U.S. first or in parallel with other major markets.”
What About Japan?

• Similar to US in many ways
  – Large markets
  – Strict regulatory systems
  – Comparable levels of clinical care

• Similar focus on improving the environment for medical device development and access
How Can We Promote Patient Access Together Throughout the Total Product Life Cycle?
Early Feasibility Studies

• Promote early-stage clinical research in US
  – Small number of subjects
  – Device design not necessarily final
  – “Just-in-time” non-clinical testing

• Additional focus on streamlining non-regulatory processes
  – IRB approval and site initiation
  – Reimbursement
Early Feasibility Studies

Through HBD, promoted EFS pathway in Japan

How can we best utilize multi-national EFS studies and leverage these experiences?

*Do differences in clinical practice, clinical study infrastructure, patient/physician attitudes matter?*
Clinical Study Harmonization

- Multiple efforts to enhance the quality and utility of clinical data
  - Acceptance of non-IOUS data
  - Standardization of clinical definitions and endpoints
  - Use of real-world evidence (RWE)
Special Report

Clinical Trial Design Principles and Endpoint Definitions for Transcatheter Mitral Valve Repair and Replacement

A Consensus
Part 1: Evaluation and Treatment of Patients With Lower Extremity

Registry Assessment of Peripheral Intervene Devices (RAPID) — Registry Assessment of Peripheral Intervene Devices Core Data Elements —

Harmonization by Doing Proposal for Global Clinical Trial Designs for Endovascular Devices for Treatment of Critical Limb Ischemia: The United States Food and Drug Administration Perspective

LETTER TO THE EDITOR

Harmonization by Doing Proposal for Global Clinical Trial Designs for Endovascular Devices for Treatment of Critical Limb Ischemia: The United States Food and Drug Administration Perspective — Reply —
Clinical Study Harmonization

Japan and US moving in similar directions

What additional steps can be taken to promote US-Japanese clinical collaboration?
Are there non-regulatory issues to consider?
Breakthrough Devices / Safer Technologies (STEP) Programs

• Intended to facilitate access to devices that:
  – Offer advantages compared to existing treatments for a debilitating or life-threatening disease (BD)
  – Treat less serious disease and offer an improved safety profile (STEP)

• Incorporate more frequent discussions, novel clinical and non-clinical approaches, pre/post-market data shifts, …
Breakthrough Devices / Safer Technologies (STEP) Programs

In Japan, similar “Sakigake” program exists

Can these programs be complementary for companies seeking both U.S. and Japanese approval?
Can lessons learned from one program be applied to the other?
Regulatory Decision-Making

• 510(k): Alternative pathway for clearance based not on substantial equivalence, but on:
  – Intended use/technology equivalent to predicate
  – Performance testing following FDA-specified methods/criteria
    • Based on recognized standards, historical performance of comparable products
Regulatory Decision-Making

- **Least Burdensome principle**: the minimum amount of information necessary to address a regulatory issue through the most efficient manner, at the right time
  - Benefit-risk considerations
  - Acceptable of uncertainty
  - Pre-market/post-market balance
Regulatory Decision-Making

• Very similar principles developed and implemented in Japan

• IMDRF developing a process for recognition of third-party marketing decisions

Can we exploit the synergies between U.S./Japanese review approaches? Are there additional opportunities for bilateral collaboration and reviews?
Points to Consider/Discuss

• Are there opportunities to optimize EFS use in each country?
• Can we develop best practices for collecting/analyzing RWE?
• Is there value in post-market collaboration/information-sharing?
• What infrastructure/non-regulatory issues can we address?
• Are HBD and other collaborations accessible for small business?

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Conclusions

• In recent years, both U.S. and Japan have taken steps to facilitate patient access throughout the product life cycle.
• Further US-Japanese collaborations make particular sense now.
• Consider opportunities to use previous learnings and initiate new projects as part of your regulatory and clinical strategies.

• How can HBD build on its successes and enter new areas?
Thank You!

ご清聴ありがとうございました！