

Proposal for Building an FDA Center of Excellence (COE) for Rare Diseases



2018
Scientific
Workshop

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Background

- **Since enactment of ODA in 1983:**
increased investment in R&D for rare diseases
- **Many unique regulatory challenges:**
requires unique regulatory expertise
- **On this 35th anniversary of ODA:** warrants consideration of reforms for approaches to regulation



Statutory requirement to establish COEs

21st Century Cures Act, Section 3073: Establishment of FDA Intercenter Institutes

- ...shall establish one or more Intercenter Institutes within FDA for a major disease area or areas
- ...shall establish at least one Institute within 1 year



First COE Established

- Friends of Cancer Research proposed COEs at FDA with pilot in oncology
- 6/29/2016: VP Biden announced FDA Oncology COE as part of Cancer Moonshot
- 12/13/16: 21st Century Cures Act enacted
- 7/27/17: FDA issued notice in *Federal Register* establishing new organizational structure for Oncology COE



First COE Established

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- Evaluating FDA's Approach to Cancer Clinical Trials
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Oncology Center of Excellence

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About the Oncology Center of Excellence

The FDA Oncology Center of Excellence leverages the combined skills of regulatory scientists and reviewers with expertise in drugs, biologics, and devices (including diagnostics). This Center of Excellence will help expedite the development of oncology and hematology medical products and support an integrated approach in the clinical evaluation of drugs, biologics, and devices for the treatment of cancer. The OCE works with centers and offices across the FDA; visit [Who We Are](#) for more information.

Mission and Vision Statements

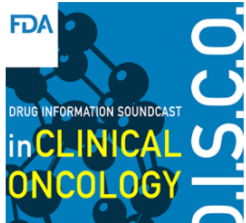
Mission Statement: The mission of the Oncology Center of Excellence is to achieve patient-centered regulatory decision-making through innovation and collaboration.

Vision Statement: We seek to create a unified and collaborative scientific environment to advance the development and regulation of oncology products for patients with cancer.



Richard Pazdur, M.D.
[Director, Oncology Center of Excellence](#)

Spotlight



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What is a COE?

- Organizational unit within the Office of Medical Products and Tobacco
- Leverages the combined skills of regulatory scientists and reviewers with expertise in major disease areas in drugs, biologics, and devices (including diagnostics)
- Helps expedite the development of a medical products and support an integrated approach in the clinical evaluation of drugs, biologics, and devices for the treatment of major disease areas
- Works with CDER, CBER, and CDRH, as well as other offices across FDA (e.g., OPT)

Why a Rare Disease COE?



- Exciting new therapies are changing the way we prevent, diagnose, and treat rare diseases
- Traditional regulatory processes have become more complicated with the reliance on combinations of therapies, genomics, diagnostic tests, and precision medicine
- Numerous parts of the regulatory system need to work together
- Myriad of challenges in rare disease medical product development remain
- Navigating these issues requires its own set of expertise (inconsistently distributed within review divisions)



Proposal for a COE for Rare Diseases

■ Combination of 3 overarching organizational changes:

1. Establishment of a COE organizational unit within the Office of Medical Products and Tobacco with cross-Center responsibilities
2. Establishment of a Deputy Director for Rare Diseases within each review office/division across CDER, CBER, and CDRH
3. Establishment of a Rare Disease Advisory Committee



Rare Disease COE: Cross-Center Activities

- Established in accordance with an Inter-Center Agreement
- Alternatives to Oncology COE Model:
 - ▷ Delegation of signatory authority for clinical portion limited to NMEs and novel biologics only
 - ▷ No departure from existing signatory authority
- However, other important functions independent of review function:
 - ▷ Harmonization of disease area-specific regulatory approaches
 - ▷ Coordination of disease area-specific regulatory science initiatives and outreach
 - ▷ Implementation of cross-Center disease area-focused meetings
 - ▷ Stakeholder engagement to the external community & international regulatory agencies



Rare Disease COE: Added Office/Division Deputy Directors for Rare Diseases

- Allow for dedicated staff person to manage day-to-day oversight and review of rare disease applications
- Coordinate with Rare Disease COE staff and each other
- Similar model employed by CDER review divisions with Deputy Dir. for Safety & Associate Dir. for Labeling
- Additional FTEs would increase review capacity & create greater opportunities for advancement



Rare Disease COE: Rare Disease AdComm

- Provide FDA with access to external advisors who authorities knowledgeable and experienced in rare diseases
- Similar to how Drug Safety & Risk Management AdComm is convened jointly with other AdComms
- Would complement the specialized medical expertise of FDA disease area-specific AdComms



A Complement to Ongoing Reorg Efforts

- In June 2018, Dr. Janet Woodcock, Director of CDER, proposed a reorganization of the center's Office of New Drugs
- Among other things, would increase number review divisions from 11 to 30, to be grouped into seven offices
- We believe this will advance review capacity for drugs for rare diseases by creating greater disease expertise
- However, greater explicit focus on rare diseases needed by *each* Division, as well as greater cross-Division and cross-Center coordination



Conclusion

- We stand on threshold of new era of scientific advances
- Possibility of profound improvements, bordering on “cures”
- Harvesting these transformative therapies and bringing to patients can be encouraged by increased visibility and enhanced regulatory consistency provided by:
 - ▷ Rare Disease COE
 - ▷ Standing Rare Disease Advisory Committee
 - ▷ Office/Division leadership dedicated to rare disease review
- Most of all, initiates dialogue that may result in other related developments beyond this proposal such as:
 - ▷ (1) increased visibility for rare disease therapies generally
 - ▷ (2) increased Congressional appropriations for FDA rare disease FTEs
 - ▷ (3) enhanced prominence of surrogates and Accelerated Approval



THANKS!

Any questions?

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