Supporting the Cures Acceleration Network: What *CAN* we do differently and better?

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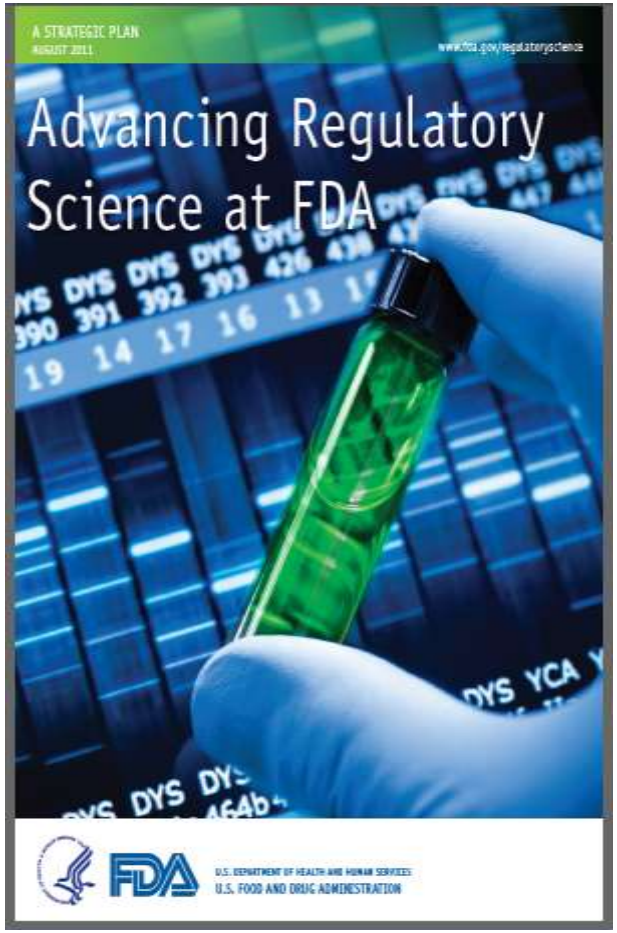
Overview

- CAN is well positioned to spur and support collaboration to address, *as an explicit part of its project's work*, gaps in regulatory science that could, as filled, both accelerate and improve predictability in product development
- Parallels to HHS, White House and FDA Medical Countermeasure Reviews and Initiatives (MCMi) in CAN's "high need cures" where "incentives in the commercial market are unlikely to result in its adequate or timely development"
  - Key findings include rapid, flexible funding, end to end project management and support, working closely with sponsors, dual use approaches, early FDA engagement and identifying and developing regulatory science to address gaps and uncertainties
- Scientific, leadership and management opportunities

CAN – time to support regulatory science tools needed for real translation



"FDA will advance regulatory science to speed innovation, improve regulatory decision-making, and get safe and effective products to people in need. 21st Century regulatory science will be a driving force as FDA works with diverse partners to protect and promote the health of our nation and the global community"

- For CAN – A rare chance to look at project proposals and plans for opportunities to *build the regulatory science tool box*, to *enhance better understanding and measures of diseases*, disease areas and outcomes, beyond single products, and, potentially, across multiple projects in CAN portfolio
- Should stimulate such opportunities through project design and evaluation criteria
- In addition to "cures" look for opportunities to support cross-cutting pre-competitive consortia and relevant data efforts/sharing that can enable them

Advancing Regulatory Science: FDA Priority Areas Relevant to CAN

- Modernize Toxicology to Enhance Safety
- Stimulate Innovation in Clinical Evaluation & Personalized Medicine
- Support new Approaches to Improve Product Manufacturing and Quality
- Ensure Readiness to Evaluate Emerging Technologies
- Harness Diverse Data through Information Sciences to Improve Health Outcomes
- Facilitate Development of Medical Countermeasures to Protect US and Global Health and Security
- Strengthen Social and Behavioral Science to Help Consumers and Professionals Make Informed Decisions

Engaging the ecosystem for success: thoughts to consider for CAN: valuing applied regulatory science

- Development and use of new evaluation tools
- New approaches to clinical studies
  - Value use of relevant populations, comparator arms and clinically meaningful outcomes (e.g. survival, major quality of life, patient reported)
  - Value development/measurement of relevant biomarkers
  - Link to and help strengthen clinical trial infrastructures
- Data creation and sharing:
  - Value development/use of strong natural history data
  - Leverage data from related products/studies
  - Creation of data that will be available and contribute to field and help patients even if product fails

Engaging the ecosystem for success: thoughts to consider for CAN: FDA

- Engage with FDA early and in a continuing manner
  - » Define a product development pathway and identify gaps/risks, including regulatory science questions
- The ultimate approach to ‘de-risking’ is real product benefit – wherever possible seek it up front
  - » If something fails because it does not have much benefit, FDA has done its job, it is the project team and investment that have failed
- Such engagement resource intensive for FDa – (MCMi supported positions help enable interactive review/policy development by Centers/teams) - can CAN help?
- Don’t forget about the product
  - » Especially for biologics/devices – need to make and characterize, again, consistent with and after trials

Engaging the ecosystem for success: thoughts to consider for CAN; *big picture*

- Can the program find a “sweet spot” where industry is not investing but yet there truly is promise and possibility, and, thus, catalyze other interest?
- Value higher risk applications that have promise for more than incremental benefit and/or targets and approaches that can affect multiple diseases
- Project business and management proposals with timeline/milestones
- Broadening evaluation criteria and the evaluators beyond study section model – include clinicians, business/VC, patient representatives
- Consider expectations for applicant contributions and how NIH/USG/patients benefit from investments (if cost-free will it be valued?)

Big Picture: thoughts to consider…continued

- For funded projects – project management teams with broad representation, including decisional oversight
- Including periodic review by a diverse independent multiagency team with the expectation and power to advise project termination or changes
- Requiring grantees to share their experiences in a CAN community
- Focusing not just on what grantees will do, but how they plan to do it (e.g. the tools and knowledge they will develop)
- *Different values, management, and team approaches - combined with developing new tools, methods and public knowledge – would help make the CAN investment uniquely valuable and sustainable*
- *FDA looks forward to working with CAN and all its partners*
- Thanks!