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Preparing for Future Products of Biotechnology

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Richard M. Murray (Chair),
California Institute of Technology

Martha Krebs, Pennsylvania State University

Raul F. Medina, Texas A&M University

Steven P. Bradbury, Iowa State University

Barbara J. Evans, University of Houston Law Center

Steven L. Evans, Dow AgroSciences

Mary E. Maxon, Lawrence Berkeley National Lab

Richard Amasino, University of Wisconsin, Madison

Jennifer Kuzma, North Carolina State University

Jeffrey Wolt, Iowa State University

David Rejeski, Woodrow Wilson Center for International Scholars

Farren Isaacs, Yale University

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Impetus for the Study

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2015 White House Memorandum calling for modernization of the biotechnology regulatory system:

- Update the Coordinated Framework for the Regulation of Biotechnology
  - Clarify the roles and responsibilities of the agencies that regulate products of biotechnology
- Formulate long-term strategy for biotechnology regulatory system
  - Efficiently assess risks associated with future products of biotechnology
  - Support innovation, protect health and environment, promote public confidence in regulatory process, increase transparency and predictability, reduce unnecessary costs and burdens

- Originally published in 1986
- Orchestrates the responsibilities of NIH, EPA, FDA, USDA, OSHA, … in regulation of biotechnology
- Relies on existing statues: TSCA, FIFRA, FDCA, FDCA, PHSA, PPA, AHPA, MIA, PPIA, …
- Other relevant statues: NEPA, ESA (endangered), APA (admin)
- Updated in 1992 to focus on product rather than process

- Commission an external, independent analysis of the future landscape of biotechnology products

Statement of Task

What will the likely future products of biotechnology be over the next 5-10 years? What scientific capabilities, tools, and/or expertise may be needed by the regulatory agencies to ensure they make efficient and sound evaluations of the likely future products of biotechnology?

(1) Describe the major advances and the potential new types of biotechnology products likely to emerge over the next 5-10 years.

(2) Describe the existing risk analysis system for biotechnology products … and each agency's authorities as they pertain to the products of biotechnology

(3) Determine whether potential future products could pose different types of risks relative to existing products and organisms. Where appropriate, identify areas in which the risks or lack of risks are well understood.

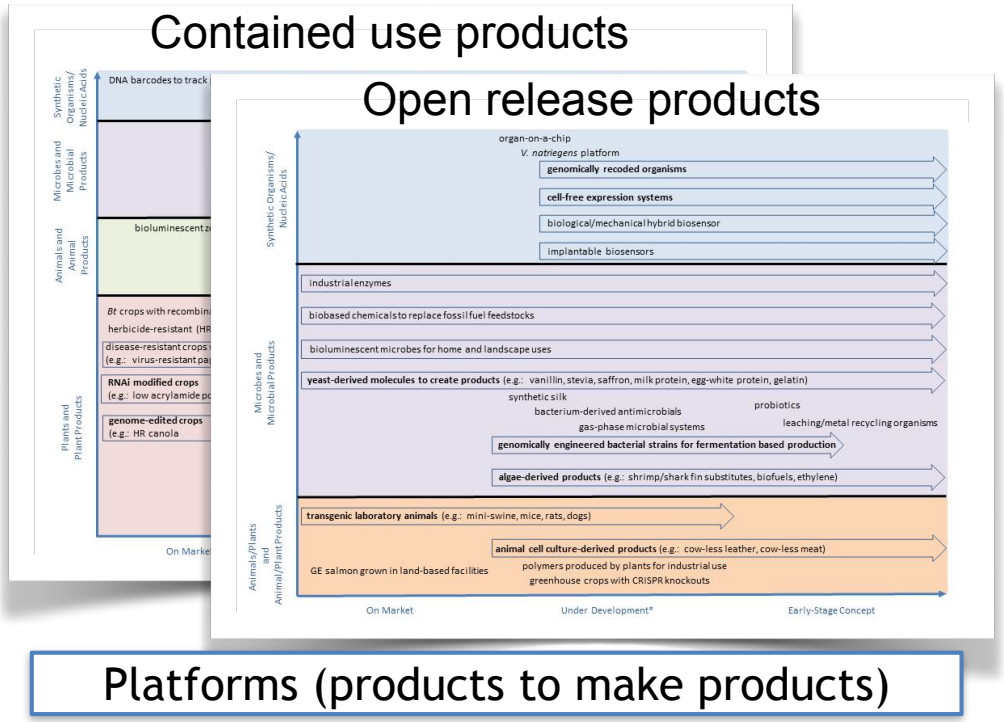
(4) Indicate what scientific capabilities, tools, and expertise may be useful to support oversight of potential future products of biotechnology.

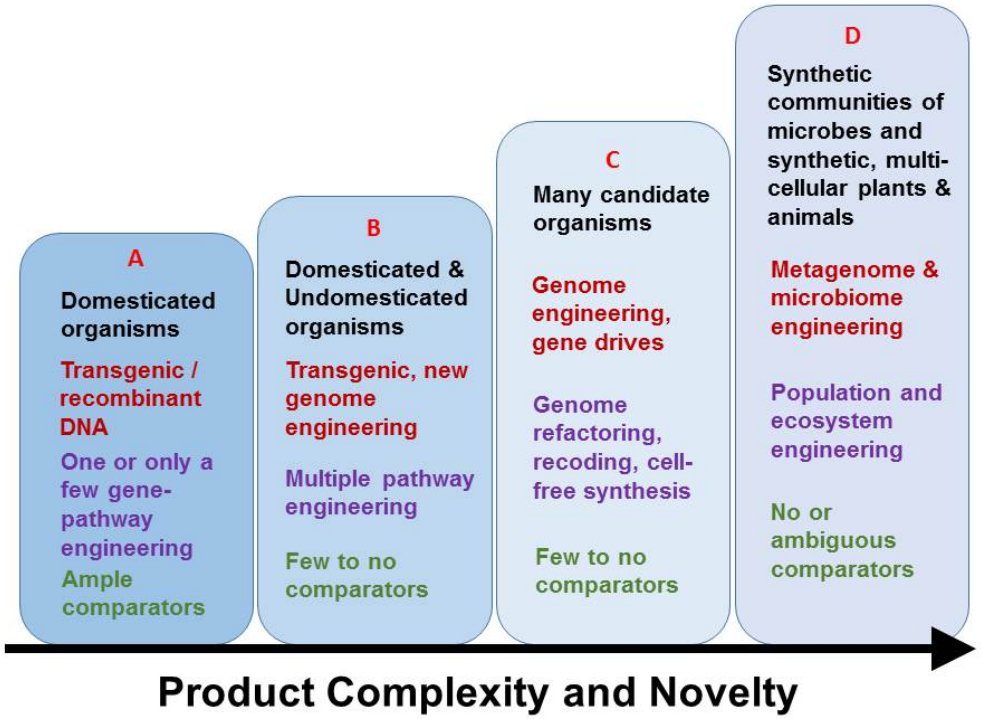
(Human drugs and medical devices are not in the purview of the study.)

Major Advances and New Types of Products

(1) Describe the major advances and the potential new types of biotechnology products likely to emerge over the next 5-10 years

- The *scale, scope, complexity, and tempo* of biotechnology products are *likely to increase* in the next 5–10 years. Many products will be similar to existing biotechnology products, but may be created through new processes, and *some products may be wholly unlike products that exist today*





Risk Analysis System

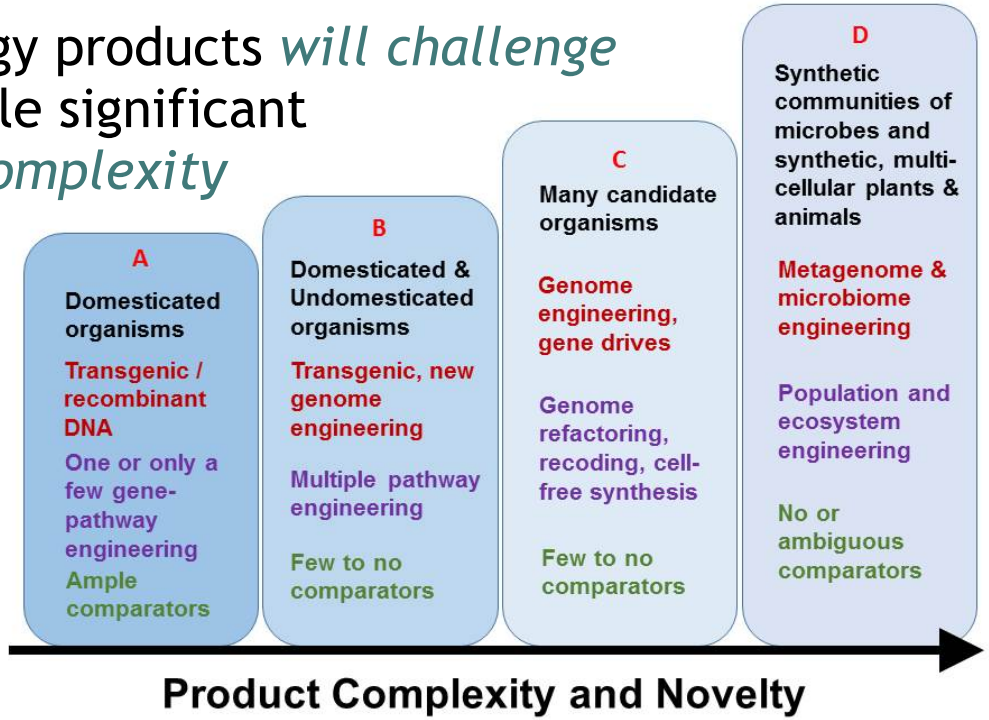
(2) Describe the existing risk analysis system for biotechnology products including, but perhaps not limited to, risk analyses developed and used by EPA, USDA, and FDA, and describe each agency's authorities as they pertain to the products of biotechnology

- The Coordinated Framework for Regulation of Biotechnology appears to have *considerable flexibility* to cover a wide range of biotechnology products, though in some cases the *jurisdiction of the agencies has the potential to leave gaps in regulatory oversight* (for future products)
- The current biotechnology regulatory system is *complex and fragmented*, resulting in a system that can be difficult for individuals, nontraditional organizations, and small- and medium-size enterprises to navigate, that *might cause uncertainty and a lack of predictability* for developers of future biotechnology products, and that has the *potential for loss of public confidence* in regulation of future biotechnology products

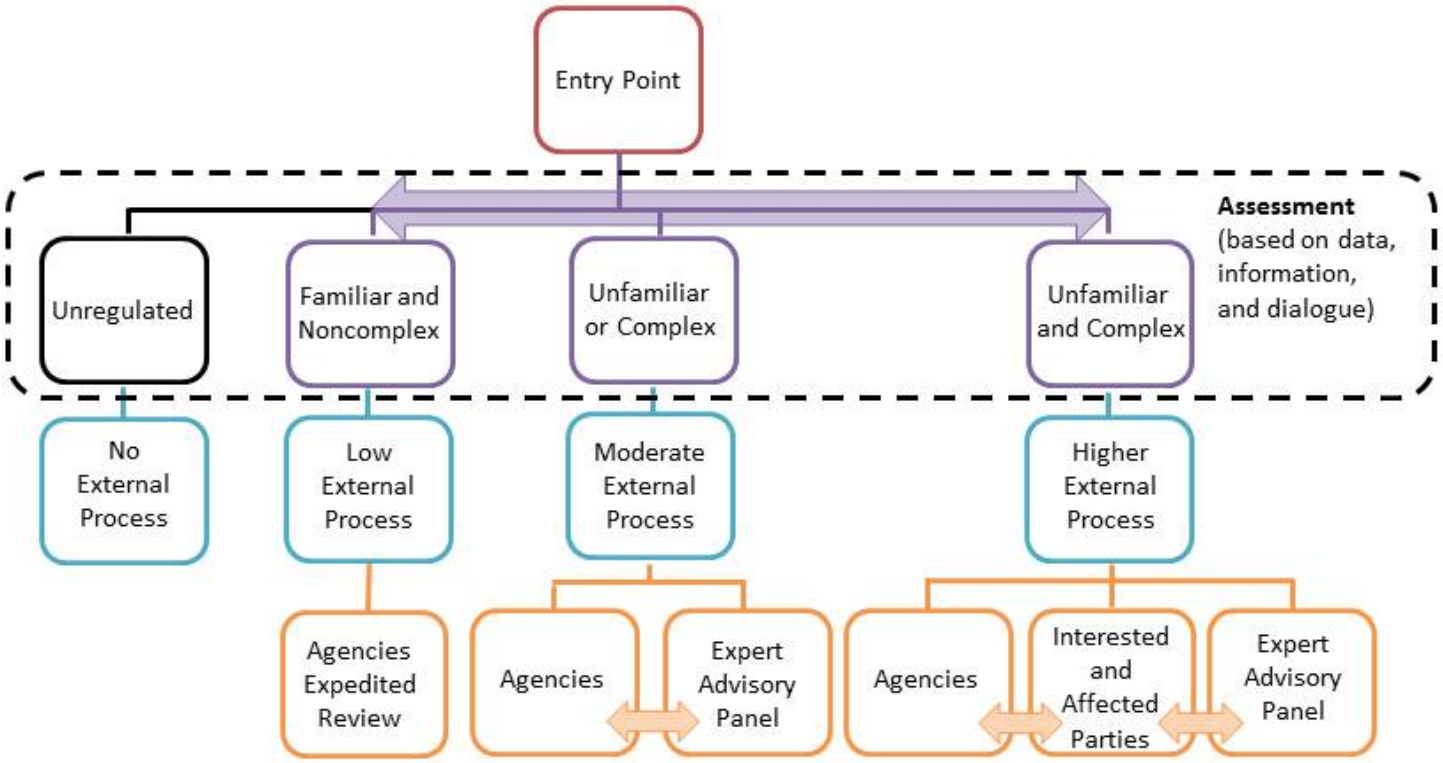
Future Products Under the Coordinated Framework

(3) Determine whether potential future products could pose different types of risks relative to existing products and organisms. Where appropriate, identify areas in which the risks or lack of risks relating to the products of biotechnology are well understood

- *The risk-assessment endpoints are not new*, but the pathways to those endpoints have the *potential to be very different in terms of complexity*.
- The *profusion* of future biotechnology products *will challenge* the federal agencies' ability to handle significant *increases in the rate, number and complexity* of biotechnology products, and *the diversity of actors*
- To enable effective regulation, it would be beneficial to have a *single point of entry* into the regulatory system



A Single Point of Entry



- *Example mechanism* to handle scope, scale, complexity and tempo + public, actors
- Operate with the agencies' existing statutory authorities
- Determine if a product falls under regulation + initial "read" on the regulatory pathway
- Provide an accessible public face for the regulatory system regulatory process

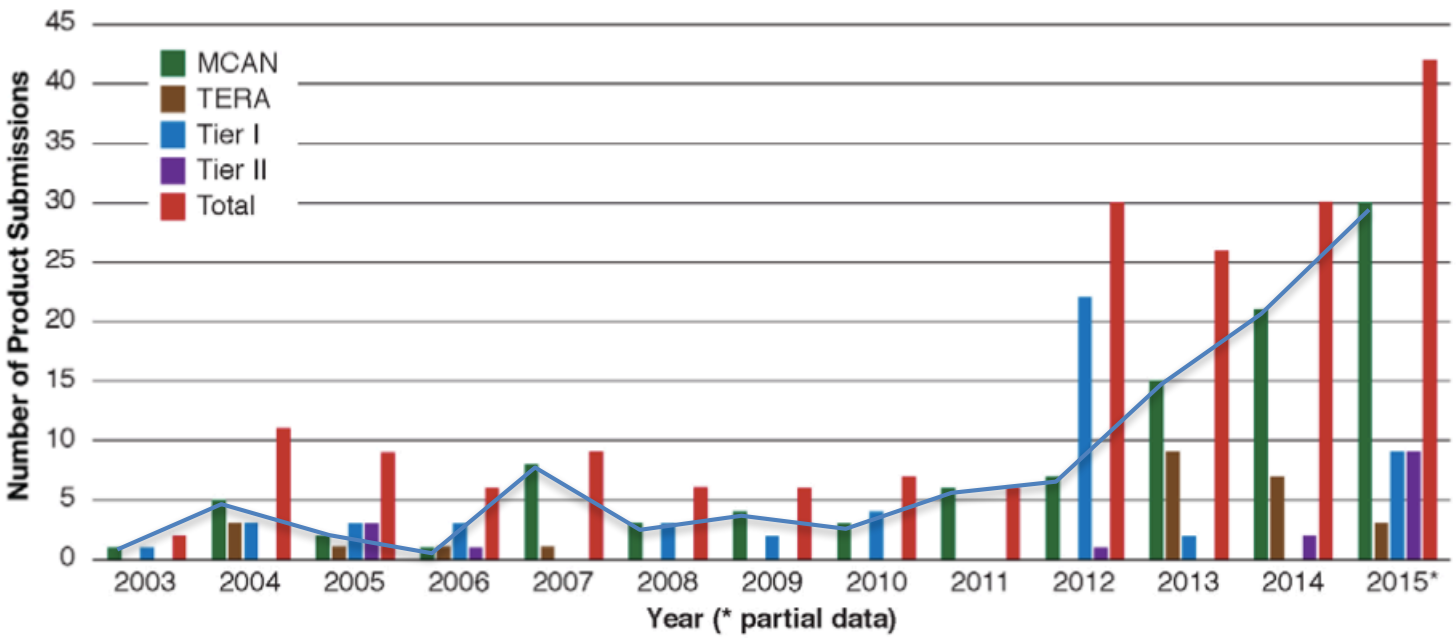
- Enable the federal agencies to decide early in the product development cycle which authorities are relevant
- Over time product types might "move" from right to left, as experience gained in evaluating additional products

Many different ways to implement similar ideas (this is just one)

Opportunities for Enhancement

(4) Indicate what scientific capabilities, tools, and expertise may be useful to the regulatory agencies to support oversight of potential future products of biotechnology

- *The staffing levels, expertise, and resources* available in EPA, FDA, USDA, and other agencies that have interests related to future biotechnology products *may not be sufficient to handle the expected scope and scale* of future biotechnology products



Increase in Toxic Substances Control Act (TSCA) biotechnology product submissions to U.S. Environmental Protection Agency (EPA), 2003–15.

- MCAN = Microbial commercial activity notices;
- TERA = TSCA experimental release applications.
- Tier I exemption requires certain certifications and recordkeeping.
- Tier II exemption requires certain certifications and a notification to EPA and EPA review of specific physical containment and control technologies.

Report Recommendations

1. EPA, FDA, USDA and other agencies involved in regulation of future biotechnology products should *increase scientific capabilities, tools, expertise, and horizon scanning in key areas of expected growth* of biotechnology, including *natural, regulatory, and social sciences*
2. EPA, FDA, and USDA should increase their use of *pilot projects* to *advance understanding and use of ecological risk assessments and benefit analyses* for future biotechnology products that are unfamiliar and complex and to *prototype new approaches for iterative risk analyses that incorporate external peer review and public participation*
3. The National Science Foundation, the Department of Defense, the Department of Energy, the National Institute of Standards and Technology, and other agencies that fund biotechnology research with the potential to lead to new biotechnology products should *increase their investments in regulatory science and link research and education activities to regulatory-science activities*

Summary and Acknowledgements

Key takeaways

- Balanced approach required
- Profusion could overwhelm; agencies need to be agile
- Regulatory science investments are needed

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