Biotechnology Industry Organization

October 23, 2013

Committee on Strategies for Responsible Sharing of Clinical Trial Data
Institute of Medicine of the National Academies
2101 Constitution Avenue NW
Washington, DC 20418

Re: Biotechnology Industry Organization Public Testimony for the Committee on Strategies for Responsible Sharing of Clinical Trial Data – Meeting One

Dear Sir/Madam:

The Biotechnology Industry Organization (BIO) thanks the Institute of Medicine's (IOM's) Committee on Strategies for Responsible Data Sharing (the Committee) for the opportunity to submit written testimony for Meeting One, scheduled for October 23, 2013.

BIO represents more than 1,100 biotechnology companies, academic institutions, state biotechnology centers and related organizations across the United States and in more than 30 other nations. BIO members are involved in the research and development of innovative healthcare, agricultural, industrial and environmental biotechnology products, thereby expanding the boundaries of science to benefit humanity by providing better healthcare, enhanced agriculture, and a cleaner and safer environment.

BIO and its member companies are committed to modernizing regulatory science and enhancing public health through advancements that fill knowledge gaps in drug development. To that end, BIO supports voluntary, responsible sharing of clinical trial data as one potential way to develop new knowledge or scientific insights. BIO member companies routinely publish clinical research results and collaborate with academic researchers, while both adhering to applicable privacy laws and regulations and protecting confidential commercial information.

I. Guiding BIO Principles on Clinical Trial Results Disclosure:

It is important to recognize that issues of transparency and disclosure of regulatory data are extremely complex and are currently being debated and evaluated globally in various venues. We urge the Committee to consider these issues in the context of other transparency initiatives, including the rulemaking process to expand the ClinicalTrials.gov database as directed by the Food and Drug Administration Amendments Act (FDAAA) enacted in 2007. As stated in BIO's April 2009 testimony on the ClinicalTrials.gov expansion, it is critical to ensure that data to be interpreted outside of the regulatory review process have scientific merit and can enhance the treatment and safety of patients.[1] Accordingly, the purpose of disclosure of such data, as well as appropriate recipients of such data, must be clearly and transparently identified.

[1] BIO Comments on Public Meeting on Expansion of the Clinical Trial Registry and Results Databank (2009) http://www.bio.org/sites/default/files/BIO_Comments_NIH_2009_0002.pdf

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In addition, it is important to strike a balance in achieving dual public health goals: the goal of enhancing scientific knowledge to advance public health and patient care, as well as the goal of protecting confidential and trade secret information, in order to maintain the incentives necessary for further investment in and development of new treatments for patients. With regard to the latter, the trade secret and commercially confidential nature of clinical data may remain even when such data are masked or pooled with data from other products.

BIO's longstanding policies on clinical trial disclosure and results dissemination state that all confirmatory trials that evaluate both safety and efficacy in patients who have the disease or condition to be treated, diagnosed, or prevented should be registered in a comprehensive listing, readily accessible by the public, no later than 21 days post initiation of enrollment.[2] Additionally, all confirmatory trials for marketed drugs, regardless of whether results are considered "positive" or "negative," as well as all pivotal confirmatory clinical trials for a product that has been discontinued in development for all indications when such trials were terminated due to safety reasons, should have summary results posted in a clinical trials database one year after the completion of the analysis of trial results.[3]

II. Proposed Committee Focus Areas:

With respect to responsible, voluntary sharing of clinical trial data, BIO recommends that the Committee first conduct a formalized review of voluntary data sharing initiatives, such as the Cardiac Safety Research Consortium[4] and Project Data Sphere[5], to share lessons learned, evaluate whether these initiatives have been productive and efficient in addressing scientific questions, and identify the gaps the Committee's own recommendations could address.

- Ensure patient confidentiality and respect the terms of informed consent: To promote robust patient participation in clinical trials, it is important that study participants have confidence that their personal medical information and privacy will be respected and protected from public disclosure. Any approach to data sharing should have adequate safeguards in place to ensure that personally identifiable data is not inadvertently disclosed, and further, that any data disclosed cannot be traced back ad hoc to individual patients. This includes respect for and compliance with informed consent procedures.

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[2] BIO Position Statement on Clinical Trial Registries and Dissemination of Clinical Trial Results (2005) http://www.bio.org/articles/bio-position-statement-clinical-trial-registries-and-dissemination-clinical-trial-results

[3] BIO Comments on Expansion of the Clinical Trial Registry and Results Data Bank (2009) http://www.bio.org/sites/default/files/20090622.pdf

[4] Cardiac Safety Research Consortium (2006-present) https://www.cardiac-safety.org/

[5] Project Data Sphere, LLC, CEO Roundtable on Cancer's Life Sciences Consortium (2013) http://projectdatasphere.org

- Employ clearly defined criteria to evaluate requests for data on a case-by-case basis: It will also be important to provide study sponsors and academics with guidelines for developing and evaluating requests for patient-level data. Consistent guidelines across industry for evaluating requests can help to ensure that analyses are conducted according to rigorous and widely accepted scientific and statistical standards, and the final results are not misleading to patients and physicians.

- Protect incentives for innovation: Modern drug development is a highly complex, costly, and competitive endeavor. For innovative biotechnology companies to be able to attract the investment necessary to fund a drug or biologic program over the course of a decade or more, it is important that data not be disclosed prematurely in a manner that can undercut a company's competitive standing in the marketplace and provide an undue advantage to other firms.

Additionally, BIO believes that responsible data sharing may impact the following areas of drug development and should be further evaluated by the Committee:

- Investigating orphan indications or other disease states where there are small populations that may benefit from pooling data across multiple studies;

- Establishing surrogate endpoints and developing biomarkers;

- Understanding the course of disease in the untreated arm to help identify better treatment intervention and endpoints;

- Modeling disease outcomes using multivariate approaches;

- Predicting patient populations more or less likely to respond to treatment, and identifying specific circumstances likely to impact response.

III. Conclusion

BIO is committed to working with stakeholders to develop a framework for responsible sharing of clinical trial data that will benefit public health and regulatory science while protecting the incentives for innovation. BIO appreciates this opportunity to submit testimony for the IOM's Committee on Strategies for Responsible Sharing of Clinical Trial Data – Meeting One on October 23, 2013. We would be pleased to provide further input or clarification of our testimony, as needed.

Sincerely,

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Andrew W. Womack, Ph.D.
Director, Science and Regulatory Affairs
Biotechnology Industry Organization (BIO)