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CENTER FOR LIFE SCIENCES INNOVATION

Investment in Clinical Trials and the Expectation of a Financial Return: Developing Better Evidence to Inform Biopharmaceutical Policy and Regulation

What: ITIF Symposium

When: October 10, 2024

Where: Information Technology and Innovation Foundation, Washington, DC

Who: Academics in finance, economics, health services research; federal agency and congressional staff; think tank scholars; biopharmaceutical investors and industry representatives. (Invitation only.)

OVERVIEW

There is a lack of credible and recent evidence that is sufficient to inform biopharmaceutical policy. The weakness in the evidence base has been demonstrated in the rhetoric revolving around recent and significant changes in U.S. law that affect biopharmaceutical investment, including the Inflation Reduction Act (IRA) and discussion about expanding federal price intervention or reducing intellectual property protection. There is a need for evidence to better understand how the expectation of a financial return from investment in clinical development in biopharmaceuticals affects the amount of investment in all stages of clinical development and for different types of potential drugs such as rare disease, oncology, or vaccines.

Estimates from the Congressional Budget Office (CBO) to inform how the IRA would affect drug development relied on published studies that were years (even decades) old regarding the impact of market size on investment in clinical study and drug development and depended on simulation models that do not necessarily reflect the practical tradeoffs in real investment decisions or the full impact of the policy on existing drug development or on particular therapeutic areas. Moreover, some significant recent policies have lacked a robust evidence base, like the 9-year implementation of maximum fair prices for small molecules and 13 for large molecules in the IRA, or the criteria CMS is directed to use when setting a maximum fair price.

PURPOSE

As the IRA and other related laws will no doubt evolve, addressing and filling the evidence gaps will better inform policy decisions that affect investment in science for the next generation of medicines. We will consider the current state of evidence that shows the strength of the relationship between investments in clinical development for drugs and the expected financial return from that investment, how the evidence is used, as well as how the perception of that relationship affects biopharmaceutical policy. We will consider approaches to increase the base

of evidence and improve models, including announcing a new grant program for research and a special issue of *Health Affairs Scholar*.

PARTICIPANTS

- Academics in finance, economics, health services research.
- Federal employees, including staff members in Congress considering policy and its impact and those who work in agencies that implement or have oversight of policy.
- Think tank scholars and consultants.
- Biopharmaceutical investors and industry representatives.

AGENDA

9:00 **Welcome and Introduction**

- Stephen Ezell and Sandra Barbosu, ITIF

Announcement of a new grant program and special issue in *Health Affairs Scholar*.

- Kirsten Axelsen, DLA Piper and AEI

9:15–9:55 **Current state of the evidence related to financial return and clinical development.**

How it informs policy for biopharmaceuticals, gaps in existing evidence, thoughts on data and information needed.

- Graham Cookson, OHE
- Q&A and discussion

9:55–10:45 **How investors consider financial return when they make investments.**

What is the expectation of financial return? How does it change as a result of policy or reimbursement changes? How does this affect decision making? And what is the effect on different therapeutic areas?

- Julie Grant, Canaan Partners
- Tess Cameron, RA Capital
- David Beier, Bay Area Capital
- Stephen Ezell, ITIF (Moderator)

10:45–11:05 **Coffee**

11:05–11:30 **Evolution of drug development.**

What has changed in the last 10 years, and how has it affected investment and risk?

- Murray Aitken, IQVIA

11:30–12:30 Data and information to guide policy.

Considering the problem—and knowing what motivates interest in new types of biopharmaceutical investigation—what types of data and what information will be most useful to guide policy?

- Lou Garrison, University of Washington
- Kosali Simon, Indiana University
- Dan Crippen, Former Congressional Budget Office Director
- Ge Bai, Johns Hopkins University
- Luca Mani, Harvard University
- Sandra Barbosu, ITIF (Moderator)

12:30–1:30 Lunch panel on post-market development.

How are decisions made to pursue additional indications or uses of a drug, and how does the expectation of future development affect early investment decisions?

- Christoph Glaetzer, Johnson & Johnson Innovative Medicine
- Mark Stencik, Merck
- Henry Grabowski, Duke University
- Genia Long, Independent Consultant
- Kirsten Axelsen, DLA Piper and AEI (Moderator)