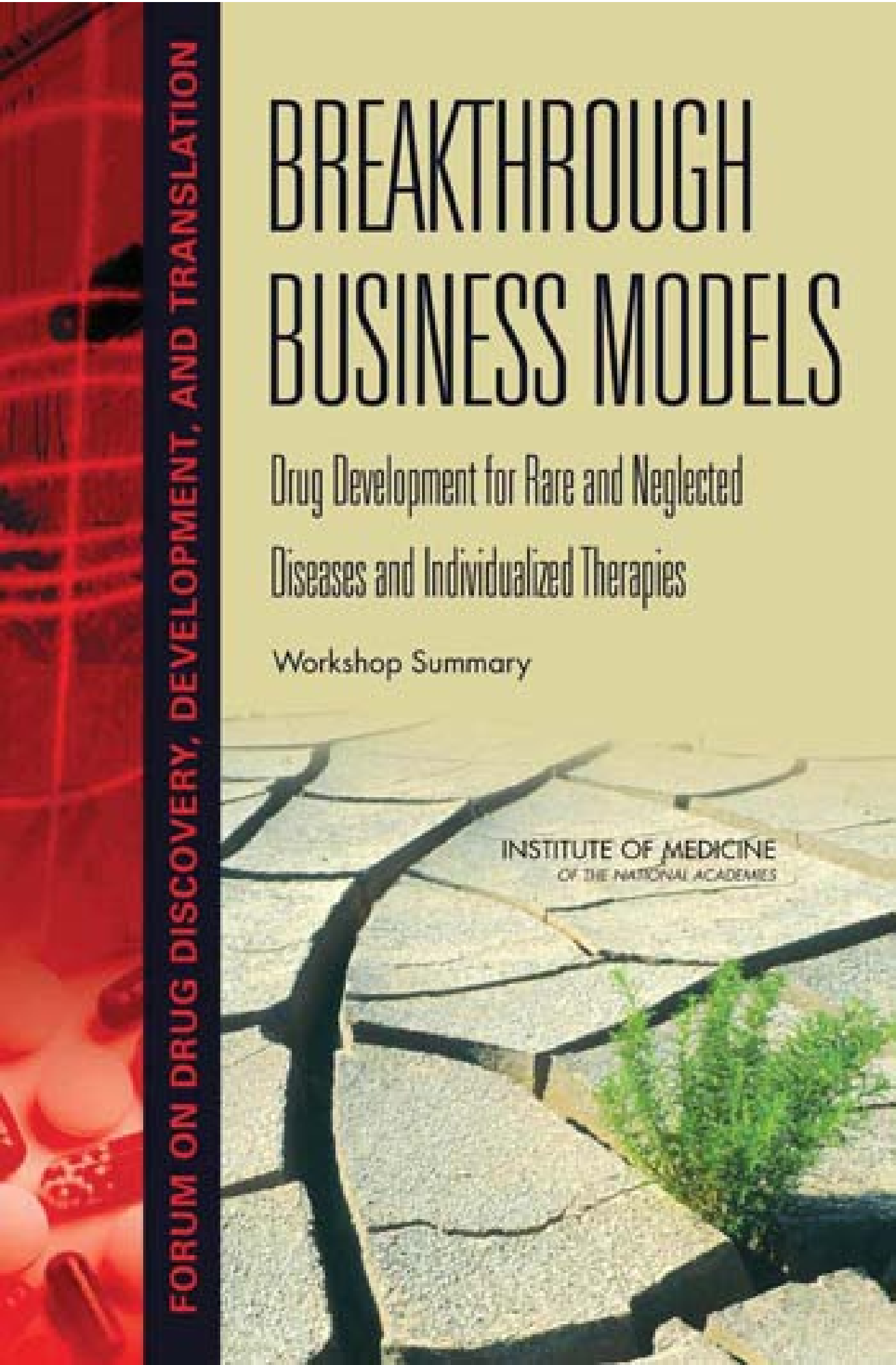

BREAKTHROUGH BUSINESS MODELS

Drug Development for Rare and Neglected Diseases and Individualized Therapies

Workshop Summary

INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

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Workshop Summary

Theresa Wizemann, Sally Robinson, and Robert Giffin

Forum on Drug Discovery, Development, and Translation
Board on Health Sciences Policy

INSTITUTE OF MEDICINE
OF THE NATIONAL ACADEMIES

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"Knowing is not enough; we must apply.
Willing is not enough; we must do."
—Goethe

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This report has been reviewed in draft form by individuals chosen for their diverse perspectives and technical expertise, in accordance with procedures approved by the National Research Council's Report Review Committee. The purpose of this independent review is to provide candid and critical comments that will assist the institution in making its published report as sound as possible and to ensure that the report meets institutional standards for objectivity, evidence, and responsiveness to the study charge. The review comments and draft manuscript remain confidential to protect the integrity of the deliberative process. We wish to thank the following individuals for their review of this report:

Russell L. Bromley, Myelin Repair Foundation
Scott Campbell, American Diabetes Association
Mikhail L. Gishizky, Entelos, Inc.
Greg Simon, FasterCures

Although the reviewers listed above have provided many constructive comments and suggestions, they were not asked to endorse the final draft of the report before its release. The review of this report was overseen by **Hellen Gelband**, Resources for the Future. Appointed by the Institute of Medicine, she was responsible for making certain that an independent examination of this report was carried out in accordance with institutional procedures and that all review comments were carefully considered. Responsibility for the final content of this report rests entirely with the authors and the institution.

Preface

The research and development process for new drug and biologic products has become extraordinarily expensive and time-consuming. Even for large pharmaceutical companies working to develop potential blockbuster drugs, many consider the current model to be unsustainable. While developing drugs to treat rare and neglected diseases can be just as expensive and time consuming as it is for blockbuster drugs, the products are often far less commercially viable to certain sectors of the pharmaceutical industry. Recognizing that patient advocacy groups can play a vital role in the development of new drugs to treat rare and neglected diseases, the Forum held a workshop in September 2007 entitled "From Patient Needs to New Drug Therapies: Can We Improve the Pathway." The workshop featured the work of four patient-focused organizations: the National Breast Cancer Coalition, the Cystic Fibrosis Foundation, the Arthritis Foundation, and the American Diabetes Association.

To better understand the innovative approaches being used by these organizations to help advance drug development, the Forum hosted a public workshop on June 23, 2008, titled "Breakthrough Business Models: Drug Development for Rare and Neglected Diseases and Individualized Therapies." Investors, policy makers, and companies seeking to develop therapies for smaller markets came together to discuss innovative strategies being implemented to expedite the development of products for these less commercially viable conditions. The intent of the workshop was first to raise awareness of these new models. Additionally, participants discussed approaches for reducing the risk of such investments by both filling critical

funding gaps along the drug development pathway and pursuing highly targeted approaches to early-phase development.

The workshop had several objectives. The first was to lay a foundation for the discussions by describing the changes that have taken place in the translational research process over the past 10 years, such as the 10-fold increase in investment by philanthropic organizations since 2000. The second objective was to discuss successful "venture philanthropy" models for funding translational research. Beyond new funding models, some philanthropic organizations and for-profit groups have undertaken innovative strategies to help expedite the development of safe and effective drugs for rare and neglected diseases by, for example, funding trials directly, supporting resources such as tissue banks, and negotiating intellectual property. A third objective was to explore whether such strategies are successful and could be implemented more broadly. Finally, workshop participants were asked to examine regulatory, legislative, and institutional policy tools currently in place to help advance the development of therapies for rare or neglected diseases.

The workshop provided an opportunity for participants to share ideas and identify potential collaborative activities. It is our hope that this workshop summary will serve as a resource for all organizations interested in advancing the drug development process for rare and neglected conditions, as well as individualized therapies.

Nancy Sung
Workshop Chair and Member
Forum on Drug Discovery, Development, and Translation

Stephen Groft
Member
Forum on Drug Discovery, Development, and Translation

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Introduction and Overview*

An objective look at current statistics characterizing the state of drug development paints a gloomy picture. The traditional process for developing a new drug or biologic product and bringing it to market has become exceedingly expensive and lengthy—estimated to cost between $800 million and $1.3 billion, and to take approximately 10–15 years. Only 8 percent of investigational new drugs entering Phase I clinical trials run the full course of development and receive U.S. Food and Drug Administration (FDA) approval, and of those, about 4 percent are eventually removed from the market. In addition, the number of new drug approvals has been slowly declining over the last 11 years—from 53 new molecular entities approved in 1996, to an average of 28 per year between 1999 and 2005, and to a mere 17 in 2007. A recent editorial outlines many of these issues and concludes that "the conventional business model appears fallible" and that "both industry and academia are poorly positioned to respond in the [current] financial landscape" (FitzGerald, 2008). Even considering the potential for blockbuster drugs, this lengthy, high-cost, low-success-rate model is likely to prove unsustainable; for those far less commercially attractive drugs used to treat rare and neglected diseases,[1] it is simply infeasible.

*The planning committee's role was limited to planning the workshop, and this summary was prepared by the workshop rapporteur and the Drug Forum staff as a factual summary of what occurred at the workshop.

[1]For the purposes of this report, rare diseases are defined as diseases that affect small patient populations, and neglected diseases are defined as diseases that are concentrated in poor or developing countries.