

The National Academies of
SCIENCES • ENGINEERING • MEDICINE

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

***Enabling Precision Medicine: The Role of Genetics in
Clinical Drug Development – A Workshop***
March 8, 2017

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Our Team

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- Amanda Wagner Gee
- Meredith Hackmann
- Lynn Hudson
- Laura Nisenbaum
- John Orloff
- Elizabeth Patrick-Lake
- Richard Schilsky
- Janet Woodcock
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Challenges and Opportunities

- Drug development faces challenges of **efficiency** and overall **sustainability** due in part to
 - high research costs
 - lengthy development timelines
 - late-stage drug failures
- Utilization of **human genetic** information represents a potentially **disruptive paradigm shift** that could
 - reduce development costs of new medicines
 - improve patient outcomes
 - realize the goals of precision medicine

Enabling Precision Medicine with Human Genetics

March 2016 Workshop:

March 2017 Workshop:

Deriving Drug Discovery Value from Large-Scale Genetic Bioresources

The Role of Genetics in Clinical Drug Development

Understand Disease Mechanism

Discover New Targets

Validate & Prioritize Targets

Discover & Validate Informative Biomarkers

Clinical Trials in Genetic Populations

Realize Precision Medicines

NME Discovery

Longest Time to Impact

Shortest Time to Impact

An Overview of the Workshop

Session I : Overarching Considerations for Implementing Successful Genetics-Based Drug Development

Session II : Case Studies in Precision Drug Development

Session III: Integrating Genetics into the Drug Development Pathway for Complex Diseases

Session IV: Finding Innovative Ways to Integrate Genetics into the Drug Development Process

Session V: Looking Forward – Recap and Conclusions

Today's Meeting Objectives

- To explore how clinical trials with genetically identified participants can enable more efficient and effective drug development and advance precision medicine.
- To highlight ongoing genetics-based clinical trials across a variety of diseases, examining best practices and lessons learned.
- To learn about the logistical challenges and successes associated with genetics-based clinical trial design.
- To examine possible mechanisms to engage participants and improve enrollment into clinical trials based on genetic characteristics.
- **Take-way: The next steps for achieving effective integration of genetics into the drug development process.**

Charge to Speakers and Participants

What is the one thing that could serve as a disruptive force to translate knowledge of human genetics into new **precision** medicines within the next 3-5yrs?

Session I: Overarching Considerations for Implementing Successful Genetics-Based Drug Development

Session Goal: Explore challenges related to the effective utilization of genetic information in the development of precision medicines.

Speakers:

JANE PERLMUTTER, President and Founder, Gemini Group

Accelerating the Pace of Progress in Genetics-Based Drug Development: The Perspective of a Patient and Patient Advocate

ROBERT NUSSBAUM, Chief Medical Officer, Invitae

Clinical Development and Use of Biomarkers for Molecularly Targeted Therapies: Recommendations from a National Academies' Consensus Study

MICHAEL PACANOWSKI, Associate Director for Genomics and Targeted Therapy, Center for Drug Evaluation and Research (CDER) U.S. Food and Drug Administration

Navigating the Regulatory Pathway for Genetic Tests and Biomarkers for Clinical Drug Development

Session I: Overarching Considerations for Implementing Successful Genetics-Based Drug Development

- **Objectives:**
 - Discuss the perspective of patients with regard to the use of genetic data in clinical drug development.
 - Consider challenges and possible solutions associated with the development and use of biomarkers or genetic tests for molecularly-targeted therapies.
 - Explore issues that pertain to the regulatory pathway for genetic tests and biomarkers used in clinical drug development.