

Summary of themes SPOT/Dx Working Group

SUSTAINABLE PREDICTIVE ONCOLOGY THERAPEUTICS AND DIAGNOSTICS



April 11, 2014

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SPOT/Dx second meeting: Exploring the value of quality molecular diagnostics and real world data in the face of constrained resources

On March 31 – April 1, 2014, the Sustainable Predictive Oncology Therapeutics and Diagnostics Working Group (SPOT/Dx) convened for its second meeting in Arlington, Virginia. Please see the Appendix for a list of meeting participants. The meeting took place amidst an increasingly dynamic political and economic period for healthcare where patient need and quality care must contend with resource constraints. On the heels of the newly passed Protecting Access to Medicare Act of 2014, participants discussed the law's impact on their ability to deliver personalized oncology treatment in a sustainable and valuable way. The meeting was also an opportune time for participants to consider new models for: 1) improving molecular diagnostic quality and 2) harnessing real world evidence to advance the development and administration of precision oncology and improve patient outcomes. The meeting enabled participants to clarify outstanding concerns and begin shaping roadmaps in service of these topics. This document summarizes key themes from the meeting.

Summary of themes

- **A patient-centered approach is integral to delivering on the promise of precision medicine.** The development of new molecular techniques and the identification of new biomarkers are dramatically increasing the scope and value of molecular diagnostics. However, some participants have identified a gap between clinical research and patient care and question how best to bridge that gap. To address this challenge, SPOT/Dx invited Joe Selby, the executive director of the Patient-Centered Outcomes Research Institute (PCORI) to comment on the future of real world data, comparative effectiveness research, and the implications for how we think about precision medicine in oncology. Selby highlighted an opportunity to transform cancer care by focusing greater attention on outcomes studies that have incorporated patient perspectives into their design. *“[Patients newly diagnosed with cancer] want to know not just survival rates but the impact of different therapies on their quality of life or ability to work. Such outcomes matter to patients, but research often fails to address them.”* Allowing patient voices to be heard when designing outcomes-focused studies may lead to data that can more appropriately inform treatment decisions and address patient need. PCORI is committed to funding large-scale observational studies that include active participation by relevant patient organizations, professional organizations, and/or payer organizations.¹
- **Fiscal constraints and political pressures are creating a climate that may, on one hand, challenge competition and local provision of testing while, on the other, place greater emphasis on the value of testing to patient outcomes.** In addition to extending current Medicare reimbursement for physician services (i.e. the “doc fix”), the Protecting Access to Medicare Act of 2014 creates a new process for adjusting reimbursement rates for the Clinical Laboratory Fee Schedule.² In brief, beginning in 2017, Medicare's rates for laboratory tests will be benchmarked off of a weighted

¹ [“Introducing a New PCORI Research Funding Initiative – Large Pragmatic Clinical Trials”](#) (Washington, DC, 2013).

² See section 216 of [“Public Law 113-93: Protecting Access to Medicare Act of 2014.”](#)

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average of private payer rates. SPOT/Dx participants had a mixed reaction to the new law. While many applauded improvements in process transparency, stakeholder groups had different opinions as to the soundness of the overall approach and its anticipated effects on pathology and patient care. Some participants likened Congress' actions to *"squeezing blood from a turnip."* They cautioned that diagnostic test innovation and local provision of laboratory services (and therefore diagnostic utilization) would suffer as a consequence of eroding price support. Others, however, believed the law reflects a larger shift toward paying for performance in healthcare. To accommodate this shift, participants suggested that pathology, like other clinical services, must transition from a transactional model to one in which utilization is driven by value.

- **Opportunities to improve diagnostic quality and equivalence exist within the current infrastructure for proficiency testing (PT). However, perceived variations in specimen collection, lab methodology, result interpretation, and clinical use may warrant a new integrated oversight mechanism.** All SPOT/Dx participants believe there is room for quality improvement within molecular diagnostics. The group considered multiple models to address this issue ranging from improvement within the current PT infrastructure to formulation of alternative paths. Within the former scenario, the group considered the need for increased educational opportunities for lab directors. They also considered the need for upfront analytical performance standards coupled with the development of contrived samples to supplement existing PT with clinical tissue. Participants identified the National Institute of Standards and Technology as one vehicle to create marketable reference standards for a select group of "troublesome" analytes. Finally, understanding that PT only assesses one part of the larger diagnostic process, the group discussed alternative models, including the need for a single entity (or steering committee) to oversee the administration of a standardized registry of tests with proscribed, consistent validation and quality assessment parameters. All agreed that assurance of diagnostic quality is a necessary forerunner to the meaningful collection of outcomes data.
- **Outcomes-focused research tools and models such as CancerLinQ and MED-C offer promising continuous learning vehicles to capture the value of personalized therapy and improve the treatment pathway for oncology patients.** As noted above, reimbursement increasingly comes down to outcomes, whether in terms of the efficacy of drugs or the clinical utility of the diagnostic. In principle, every patient's clinical experience should feed into a continuous quality improvement system. SPOT/Dx participants discussed how numerous stakeholders are either already tracking outcomes or are planning to do so. In addition to the ASCO and MolDx presentations, the group heard about similar industry and private payer efforts. Regarding molecular diagnostics, the real challenge is not defining clinical utility, but finding ways to demonstrate it. A hypothesis-generating diagnostic test may work perfectly, but if the utility of subsequent treatments is not recognized, there is no financial support. SPOT/Dx participants discussed the possibility of placing some form of the MED-C coverage with evidence development framework in front of the CancerLinQ infrastructure. Both drug and diagnostic developers expressed concern over running afoul of the FDA's premarket promotion rules. Additional open questions include the need for patient consent and whether patients would avoid randomized trials in favor of obtaining the experimental therapy off-study. SPOT/Dx participants did

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not view these challenges as insurmountable and expressed an interest in exploring this opportunity further.

The meeting closed with participant reflections on the collective next steps of the Working Group. A clinician said, *“I’m encouraged by the fact that we can work together to do, in the end, what’s going to be best for patients.”* Tapestry will distribute a more detailed summary of the meeting in the weeks ahead.

About this document

The views expressed in this document represent those of the Sustainable Predictive Oncology Therapeutics and Diagnostics (SPOT/Dx) Working Group, a group of leading stakeholders from the public and private sectors committed to improving patient outcomes by equipping US healthcare leaders with the tools needed to change the diagnosis and treatment of cancer. This document is not intended to represent the particular policies or positions of the Working Group’s individual participants or their affiliated organizations. This material is prepared and copyrighted by Tapestry Networks with all rights reserved. It may be reproduced and redistributed, but only in its entirety, including all copyright and trademark legends. Tapestry Networks and the associated logo are trademarks of Tapestry Networks, Inc.

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Appendix: SPOT/Dx Working Group participants

Patient/policy advocates

- Jeff Allen, Executive Director, Friends of Cancer Research
- Andrea Ferris, President and Chairman, LUNGevity Foundation
- Nancy Roach, Founder and Chairman, Fight Colorectal Cancer

Payers

- Naomi Aronson, Executive Director, Clinical Effectiveness and Policy, Blue Cross and Blue Shield Association
- Mike Barlow*, Vice President of Operations, Palmetto GBA
- Dane Dickson, Director of Clinical Science, MolDx, Palmetto GBA
- Elaine Jeter*, Pathologist and Medical Director, Palmetto GBA
- Michael Kolodziej, National Medical Director, Oncology Solutions, Aetna
- Lee Newcomer, Senior Vice President, Oncology, Genetics and Women's Health, UnitedHealthcare
- Ed Pezalla*, Vice President, National Medical Director, Pharmacy Policy and Strategy, Aetna
- Jeff Roche (Liaison to the Working Group), Lead Medical Officer, Coverage and Analysis Group, Center for Clinical Standards and Quality, Centers for Medicare and Medicaid Services

Regulators (Liaisons to the Working Group)

- Pamela Bradley*, Personalized Medicine Staff, Office of In Vitro Diagnostics and Radiological Health, FDA – CDRH
- Jonathan Jarow, Acting Deputy Office Director, Office of Hematology and Oncology Products, FDA – CDER
- David Litwack, Personalized Medicine Staff, Office of In Vitro Diagnostics and Radiological Health, FDA – CDRH
- Michael Pacanowski, Associate Director, Genomics and Targeted Therapy, Office of Clinical Pharmacology, Office of Translational Sciences, FDA – CDER

Subject matter experts/technology specialists

- Steven Anderson*, Global Head, Clinical Trials; Chief Scientific Officer, Oncology and Genetics, LabCorp Clinical Trials
- Frank Cockerill, Chair, Department of Laboratory Medicine and Pathology; President and Chief Executive Officer, Mayo Medical Laboratories, Mayo Clinic

Subject matter experts/technology specialists continued

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- Helena Duncan, Assistant Director, Economic and Regulatory Affairs, College of American Pathologists
- Stephen Grubbs, Principal Investigator, Delaware Christiana Care CCOP, Medical Oncology Hematology Consultants, PA
- Cliff Hudis, Chief, Breast Cancer Medicine Service, Memorial Sloan-Kettering Cancer Center
- Karen Kaul, Chair, Department of Pathology and Laboratory Medicine, NorthShore University HealthSystem
- Doug Moeller, Medical Director, McKesson Health Solutions
- Richard Schilsky, Chief Medical Officer, American Society of Clinical Oncology
- Matt Zubiller*, Vice President, Strategy and Corporate Development, McKesson

Industry representatives

- Ken Bloom, Chief Medical Officer, GE Healthcare – Clariant Diagnostic Services
- Peter Collins, Vice President, Diagnostics, GlaxoSmithKline
- Nic Dracopoli*, Vice President, Head of Oncology Biomarkers, Chief Scientific Officer, Next Generation CTC Technology, Janssen R&D, Pharmaceutical Companies of Johnson & Johnson
- Chris Jowett, Global Commercial Head, Companion Diagnostics, Abbott Molecular
- Ron Mazumder, Global Head, Research and Product Development, Janssen Diagnostics
- Jonathan Pan, Director, Oncology Companion Diagnostic and Disease Strategy, GlaxoSmithKline
- Scott Patterson, Executive Director, Medical Sciences, Amgen
- Patrik Ringblom*, Global Commercial Strategy Leader, Oncology, Janssen Global Services
- Ryan Saadi, Global Market Access Head, Health Economics and Reimbursement, Oncology, Johnson & Johnson
- Peter Sandor, Vice President, Therapeutic Area Head, Oncology Global Marketing, Amgen
- Pamela Swatkowski, Director, Regulatory Affairs, Abbott Molecular

Dinner guest speaker

- Joe Selby, Executive Director, Patient-Centered Outcomes Research Institute

*Participant was unable to attend March 31 – April 1, 2014 meeting