

Summary of themes SPOT/Dx Working Group

SUSTAINABLE PREDICTIVE ONCOLOGY THERAPEUTICS AND DIAGNOSTICS



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SPOT/Dx third meeting: Charting a course for the future

On July 15-16, 2014, the Sustainable Predictive Oncology Therapeutics and Diagnostics Working Group (SPOT/Dx) convened its third meeting in Arlington, Virginia. Please see the Appendix for a list of meeting participants. The group reaffirmed its commitment to cancer patients, outlined a pilot focused on enhancing the quality of future molecular diagnostics, continued to explore the merits of facilitated drug access/outcomes registry programs, and considered what future contribution the SPOT/Dx group could make to accelerate and safeguard the implementation of precision medicine in oncology.

Summary of themes

- **The paradigm for molecular diagnostics in oncology is shifting toward a future where the value of a test is defined and rewarded based on its impact on the health system.** At a time where fiscal constraints and demand for quality care are simultaneously increasing, several acknowledge that the future of oncology care will require high quality molecular tests whose performance is easily tracked and consequently rewarded. The value of such tests will be defined by tracking patient outcomes and understanding the impact of the molecular diagnostic on achieving a given patient outcome whether favorable or not. A cost-effective approach to oncology care will potentially shift the emphasis from what one diagnostic developer referred to as a *“companion diagnostic approach to a companion therapeutic model in which multiplex platforms are used to unearth a broad swathe of information – a subset of which is actionable to a course or combination of targeted treatments.”*

Increased complexity in oncology molecular diagnostics and treatment pathways creates tension within *“old regulatory, reimbursement and clinical systems.”* In one clinician’s words, *“the challenge is integrating new technology into clinical oncology practice in a way that is efficient, valuable, and transforms health system care delivery.”* A number of public-private partnerships have risen to this challenge, focusing on areas from innovative clinical trial design (e.g. LUNG-MAP¹) to bridging the gap between the science and clinical implementation (e.g. Actionable Genome Consortium). A number of SPOT/Dx participants are eager to track these efforts and possibly contribute to them in the future. However, the broad question remains of how to integrate the disparate sources of information surrounding patients as they pass through care (from diagnostics to patient history, *“genotypes to phenotypes”*).

- **There is broad multistakeholder support to move forward with the “quality” pilot to assure patients, providers, payers, and laboratories of the accuracy of molecular diagnostic test results.** Many SPOT/Dx participants strongly support the development of robust reference materials prior to regulatory approval for a given biomarker(s) in order to help harmonize test results across various

¹ The Lung Cancer Master Protocol (Lung-MAP) trial is a multi-drug, multi-arm, biomarker-driven clinical trial for patients with advanced squamous cell lung cancer. The trial will use genomic profiling to match patients to one of several different investigational treatments that are designed to target the genomic alterations found to be driving the growth of their cancer. This innovative approach to clinical testing should both improve access to promising drugs for patients and ease the significant recruitment and infrastructure burdens on researchers involved in traditional clinical trials. [“Lung-MAP Launches: First Precision Medicine Trial From National Clinical Trials Network”](#)

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diagnostic platforms and methodologies once a companion therapeutic hits the market. While open questions remain with regard to transparency, funding, enforcement, and unintended consequences of taking on a new quality assurance model, a number of participants affirmed the need for a new approach that would have value even in a world where all companion diagnostics were reviewed by the FDA. Regulators clarified that industry's working with labs ahead of drug/diagnostic licensure would not run afoul of promotional rules so long as it was clear that the drugs/devices are not purported to be safe and effective. Laboratory participants were enthusiastic about adding the pilot's contrived samples to their current practice of exchanging patient samples, but noted their limited resources and the need to avoid a negative financial impact. To this point, participants discussed how some form of transparency would be necessary in order for doctors and patients to select high-performing tests and for payers to reward demonstrated quality. Participants also noted the potential benefit of exploring laboratory quality practices outside the United States, such as the use of ring trials in proficiency testing. Lastly, the group considered the need to broaden the proposed proficiency testing pilot to include some means of outcomes tracking. Many participants felt that, if feasible, incorporating an outcomes-tracking IT component would make the approach more adaptable in terms of where the diagnostics field is going, rather than locking in a dated framework. The urgency for getting this underway was strongly voiced, from payers - *"This is needed yesterday,"* - to patients and providers - *"It should be self-evident that patients and providers want high quality testing and to have confidence that the test is going to inform a meaningful clinical decision. So we're in favor of this approach."*

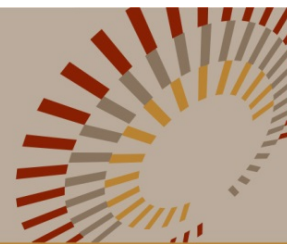
- **The facilitated access/outcomes registry proposals pave the way for “new research approaches” that generate learning outside of the traditional clinical trial paradigm.** The SPOT/Dx group explored the traits of an “ideal” facilitated drug access/outcomes registry model using the ASCO and MED-C proposals (both of which are focused on the off-label use of marketed drugs) as starting points for discussion. Many participants feel strongly that, in the words of an oncologist, *“it is time to start learning from the 95% of patients who are treated outside of clinical trials.”* However, SPOT/Dx participants (perhaps in step with their individual stakeholder groups) have different opinions as to where the needle should fall on the spectrum between observational and hypothesis-driven learning. Perhaps most conservatively, one payer asked whether these real world evidence models should await the results of ongoing basket trials² across cancer centers before moving forward since these trials *“may serve as proof or disproof of concepts.”* In response, the group discussed how these basket trials are themselves in fact very anecdotal because every trial is set up differently.

With respect to the scope of permissible diagnostic tests in these concepts, some participants saw value in the original ASCO proposal which allows doctors to choose from the entire universe of tests. One subject matter expert explained that capturing the test that was used and its performance characteristics would help payers know which tests to reimburse and would allow all stakeholders to compare the utility of approved versus off-label tests. The challenge is in whether such test details are currently successfully captured in EMR data. However, at the other end of the spectrum, some participants believe that both

² A basket trial is a histology-agnostic trial that administers a drug to patients with different tumor types that share the same molecular aberration. For example, [NCT01219699](#) is a phase I trial of solid tumors that have an alteration of the PIK3CA gene.

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the diagnostic test and the treatment protocols must be prospectively defined in order to limit variables and generate high quality data in the most expeditious manner. In the words of a diagnostic developer, *“The move toward this new paradigm of learning from clinical practice needs to be carefully constrained.”*

A number of questions remained in the minds of many stakeholders regarding these concepts from feasibility to the outcomes they would generate. Both ASCO and Palmetto value the group’s feedback as they continue to shape these models. They are also committed to partnering with others to move these innovative proposals forward.

Next steps

A majority of participants acknowledged their commitment to carry the “quality” pilot forward. Several called for the development and distribution of a written proposal codifying stakeholder perspectives on how such a pilot should be designed and implemented. Additionally, a steering committee must be identified and convened to discuss the proper governance required, finalize the pilot design and process, and identify initial biomarker candidates for the pilot. More broadly, the group also discussed the possibility of maintaining the SPOT/Dx Working Group as a multistakeholder troubleshooting body that would meet periodically to advise on and share lessons from existing pilots, as well as to develop solutions and new pilot processes for additional challenges within precision medicine in oncology. The Tapestry team will integrate the discussions of this group, and that of the broader stakeholder landscape, in the development of these proposals and return further details on next steps to participants of the working group later this summer.

The meeting closed with participant reflections on the collective power of the Working Group (*“a diversity of perspectives that you just don’t get anywhere else”*) and the rapidity of an action-orientation to the group discussions highlighted by the commitments to move forward on the quality pilot. A diagnostic developer summed up the experience: *“Bringing together disparate organizations to a common purpose of improving patient care has led to a great deal of education – we are all speaking a common language now.”* A patient representative added: *“Patients are in an interesting position – they are at the core of this topic but do not have visibility into a number of these challenges embedded into the healthcare system that may compromise their care. I’m encouraged by this group’s efforts to remove friction from the system and improve the quality of patient care.”*

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

Participants

Patient/policy advocates

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

Payers

- Naomi Aronson, Executive Director, Clinical Evaluation, Innovation and Policy, Blue Cross and Blue Shield Association *
- Mike Barlow, Vice President, Palmetto GBA
- Dane Dickson, Director of Clinical Science, MolDx, Palmetto GBA *
- Elaine Jeter, Pathologist and Medical Director, Palmetto GBA *
- Penny Keller, Division of Laboratory Services, Center for Clinical Standards and Quality, Centers for Medicare and Medicaid Services
- 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, Lead Medical Officer, Coverage and Analysis Group, Center for Clinical Standards and Quality, Centers for Medicare and Medicaid Services

Regulators

- Pamela Bradley, Personalized Medicine Staff, Office of In Vitro Diagnostics and Radiological Health, FDA – CDRH *
- Robert Klees, Research Scientist, New York State Department of Health *
- 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
- Erasmus Schneider, Associate Director for Research and Technology, New York State Department of Health *

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Participants

continued

Subject matter experts/technology specialists

- Steve 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
- 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
- Mary Williams, Executive Director, Association for Molecular Pathology
- Matt Zubiller, Vice President, Strategy and Corporate Development, McKesson

Sponsor representatives

- Ken Bloom, Chief Medical Officer, GE Healthcare – Clariant Diagnostic Services
- Cindy Collins, Chief Executive Officer, Clariant, GE Healthcare
- 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
- Richard Klausner, Senior Vice President and Chief Medical Officer, Illumina
- Ron Mazumder, Global Head, Research and Product Development, Janssen Diagnostics, Pharmaceutical Companies of Johnson & Johnson
- 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, Johnson & Johnson *
- Peter Sandor, Vice President, Therapeutic Area Head, Oncology Global Marketing, Amgen
- Pamela Swatkowski, Director, Regulatory Affairs, Abbott Molecular

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Participants

continued

Dinner speaker guests

- Joe Frassica, VP & Chief Medical Informatics Officer, Chief Technology Officer, Philips Healthcare
- Hans Hofstraat, Vice President, Philips Research
- Nila Banerjee, Senior Research Staff Member, Philips Healthcare

() Indicates participant or sponsor representative is unable to attend the July meeting.*

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