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## FOR IMMEDIATE RELEASE

**Center for Medical Technology Policy, Green Park Collaborative-USA Convene Consortia in Baltimore**

***--Nearly 100 thought leaders in oncology and endocrine-metabolic diseases consider study design guidance in key clinical areas--***

Baltimore, MD—May 2, 2014—The Green Park Collaborative (GPC-USA), a multi-stakeholder forum of the Center for Medical Technology Policy (CMTP), gathered health care leaders from around the country in Baltimore, MD, on April 23-24, 2014. This is the latest in its ongoing efforts to create study design recommendations that guide evidence development and inform complex clinical and payment decisions in oncology and endocrine-metabolic diseases.

The semi-annual meetings of the Oncology and Endocrine-Metabolic Diseases Consortia of GPC-USA featured high level presentations, frank and provocative discussions, and recommendations for each group's work. The Oncology Consortia is working on methodological recommendations and data strategies for evaluating the clinical utility of next generation sequencing in oncology and assessing the best ways to sequence cancer care. The Endocrine-Metabolic group is developing standards on patient-centered outcomes for diabetes treatment.

"Our Consortia continue to make exciting progress," says Elisabeth (Els) Houtsmuller, PhD, Senior Program Director, GPC-USA. "These meetings bring together stakeholders from throughout the drug and device development process—researchers from industry and academia, payers, patient advocates, clinicians, and regulators. It is this diversity that enables GPC-USA to provide guidance on how to produce the "real world" evidence these groups need to make the full range of health care choices and decisions."

***Two Efforts in Oncology***

Led by Donna Messner, PhD, CMTP Research Director, the Oncology Consortium is developing Effectiveness Guidance Documents (EGDs) on two topics.

***Next Generation Sequencing***

The Consortium has initiated a project to establish methodological advice on creating the evidence needed to evaluate promising clinical applications of next generation sequencing (NGS) for personalized cancer diagnosis and treatment. This is a rapidly developing field. NGS quickly produces a breadth and depth of genomic information impossible to achieve with earlier generations of molecular testing, and at increasingly competitive costs. But standards for interpreting and using the mass of data generated are lacking, and developing efficient methods to assess the clinical utility of NGS remains challenging.

The project's EGD will focus on developing methods to evaluate innovative clinical applications of NGS-based test data and render information that is usable and useful to payers, clinicians, and cancer patients.

Participants called for the creation of databases to capture outcomes from off-label use of oncology drugs on biomarker-based targets; establishment of common standards across different data platforms; and development of methodologies to efficiently identify, evaluate, and communicate actionable genomic markers for patient treatment and determination of reimbursement. "Going forward, the only way we can determine appropriate coverage for NGS and other advanced testing is to have established common evidentiary standards for assessing clinical data. We need scientifically based and transparent methods to determine clinical utility," said Dane Dickson, MD, Director of Clinical Science, Palmetto GBA.

"The challenge of the work is discovering how to actually design studies that cost-effectively and reliably determine clinical utility—particularly below the level of randomized clinical trials—with clear and needed outcomes," said CMTP Founder and CEO Sean Tunis, MD, MSc. "What other research methodologies are appropriate and strong enough to convince a reasonable skeptic?"

***Treatment Sequence***

The Oncology Consortium's second EGD, which should be ready later this year, will describe methods and data strategies for determining the optimal sequence and timing of oncology care options (including not only aggressive interventions but also supportive care and therapy "holidays") in order to yield optimal net benefit to patients who have advanced cancer. The document will provide important guidance for research and data collection in two contrasting example disease areas: metastatic breast cancer and renal cell carcinoma.

In advanced cancer, there may or may not be significant differences in the overall survival benefit of different care strategies. But as patient advocate Deborah Collyar, Director of Patient Advocate Research Team Program, Patient Advocates in Research, said, "We need to develop relevant data points that actually matter to real patients. Survival is important, but so is lowering the burdens of cancer so they can spend their time doing what they love. For instance, what care sequence causes the fewest problems? Where does hospice fit? How can we help patients know their family will not be crushed by huge medical bills?"

At the meeting, participants discussed ways to validly collect and assess information on patient burden that include, but go beyond, traditional measures of toxicity to capture other aspects of patient experience, such as out-of-pocket costs for care or missed days of work.

***Endocrinology***

The Endocrine-Metabolic Consortium's first EGD, scheduled for publication in early summer 2014, will focus on guidance for designing trials that provide better information about real-world effectiveness and outcomes that are most meaningful to diabetes patients. "The Consortium developed a set of preliminary recommendations for the EGD from a series of 19 key informant interviews. The diverse group of stakeholders who gathered at CMTP

headquarters provided both confirmation that the guidance addressed the most salient points and outstanding suggestions for edits to the draft guidance," says C. Daniel Mullins, PhD, the Consortium's lead. "That gave us a strong starting point to begin discussions and make refinements at the meeting."

To sharpen the EGD's developing ideas, meeting participants discussed each preliminary recommendation derived from the interviews. The recommendations addressed selection of Patient Reported Outcome (PRO) instruments, determination of study comparators, and inclusion criteria for study populations, as well as methodology for reporting on stratified results.

Participants also discussed possible topics for future EGDs, including treatments for obesity, osteoporosis, diabetes complications, and mobile health technology—all related to this clinical area.

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**About the Green Park Collaborative-USA (GPC-USA)**
GPC-USA is a multi-stakeholder forum that is developing condition-specific study design recommendations to guide the generation of evidence needed to inform coverage and payment decisions in the United States. The GPC-USA serves as a trusted forum where payers, life sciences companies, patient groups, clinicians, researchers, regulators and other stakeholders can engage in dialogue regarding methodological standards for studies intended to demonstrate real world effectiveness and value. For more information click here.

**About CMTP**
The Center for Medical Technology Policy (CMTP) is an independent, non-profit 501(c)(3) organization that aims to make health care more effective and affordable by improving the quality, relevance, and efficiency of health care research. We focus on the design and implementation of comparative effectiveness research to produce information that helps patients, clinicians, and payers make informed treatment and policy decisions. CMTP provides a trusted forum in which a broad range of stakeholders can collaborate to identify important research questions, design appropriate studies, and develop innovative partnerships to implement these studies.

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