

Comparative Effectiveness Research Strategic Plan Moffitt Cancer Center, Tampa, Florida

The success of Comparative Effectiveness Research to improve the quality and cost-effectiveness of national healthcare requires focused efforts to:

- Expand information technology infrastructures that can integrate large data sets from various sources
- Establish experienced research teams that can perform data mining and modeling
- Establish healthcare networks that are capable of reducing new CER methods to practice
- Work with the Federal, State and insurance payers to embrace new healthcare models.

To enhance the national CER program, the creation of regional CER consortia that can focus on a specific class of disease, develop research methodologies, create validation networks and encourage partnerships with industry leaders would be the most effective strategy for defining new standards of care that will revolutionize patient care.

Moffitt Total Cancer Care – A Model Infrastructure for CER

Cancer is the second leading cause of death in the United States with an estimated cost of \$171.6 billion from treatment, morbidity and mortality. The costs to Florida alone are estimated to be approximately \$16 billion per year. **The Moffitt Cancer Center & Research Institute** has developed a visionary approach called Total Cancer Care™ (TCC) that leverages partnerships with industry, academia and private foundations to provide a unique infrastructure and model for Comparative Effectiveness Research. TCC is a comprehensive approach to a cancer patient's life journey, while integrating research and improving access to information for patients, researchers and clinicians. A key component of TCC is a prospective research study that is recruiting tens of thousands of patients who consent to: 1) donate tumor and tissue samples for research that will molecularly characterize cancer diseases, personalize cancer care and reveal targets that can be used for the development of new treatments 2) the ongoing collection of clinical data throughout the patient's lifetime, and 3) be contacted throughout their lives about new treatments or research that is relevant to their disease.

A state-of-the-art, data warehouse is being utilized to capture the data and allow secure access by patients, researchers and clinicians to the data being collected on over 30,000 patients who have consented to participate in this innovative research project to-date. Currently, approximately 100 patients are consented daily throughout the United States and by 2012 over 100,000 patients should be enrolled in the TCC trial.

The TCC National Consortium

The ability to conduct comparative effectiveness research requires a study of clinical outcomes across a variety of settings and patient populations, examining racial, ethnic and geographic variations that affect health outcomes. Although TCC was conceived by the Moffitt Cancer Center (MCC), in Tampa Florida, the Total Cancer Care Consortium includes ten Florida hospitals, including MCC, and seven national sites that recruit patients for the study, ship tissues to MCC for molecular analysis and banking, and provide patients' medical data for integration into the data warehouse. Additionally, Moffitt has established a network of 15 affiliated partners across the State of Florida that are able to conduct clinical trials to both increase patient accrual and to deliver investigative treatments locally to the patients.

Strategic Plan for Comparative Effectiveness Research

Moffitt Cancer Center proposes to establish a Florida Comparative Effectiveness Research Consortium that will focus on cancer diseases. The TCC project provides new technologies and methodologies to discover new ways to more effectively treat patients. This prospective, observational study is only a single component of this effort. The creation of a statewide CER Consortium would require the coordination of centralized information systems that would integrate public and private data from resources such as SEER (Surveillance, Epidemiology and End Results), Medicare, Medicaid, private insurance, clinical trial data, Florida Cancer Registry data, data from observational studies such as TCC and data created through systematic reviews of the medical literature. The integration of data should occur at a central site that can be accessed by all members of the consortium for CER analyses.

The first level of analysis would be data mining of the integrated data to find subpopulations that can be defined by various factors, including treatment responders and non-responders, the efficacy of neoadjuvant therapies or combination therapies, as well as the effectiveness of screening to identify disease at early stages. With the advent of molecular technologies, the possibility to expand the knowledge derived from classic data mining studies will have the potential to greatly impact CER research to develop more effective tools for prevention and screening, diagnosis and matching the right patient to the right treatment. This unifying methodology of combining diverse data sets that have been collected for unique uses will provide the foundation of CER research to more accurately derive knowledge that will lead to novel models of care that have the potential to dramatically improve patient care.

The next step in the process to create a new standard of care would be the development of innovative disease and treatment models that would be the basis for reducing to practice the knowledge derived through data mining. First, mathematical models that mimic new treatment models will need to be tested *in silico* to verify that the new methodologies can be independently validated. This is a pivotal step before creating new clinical trials. The mathematical modeling necessary requires unique expertise from the computer science and mathematics fields that will need to work in teams with epidemiologists, informaticists, clinicians, medical systems and basic scientists to meld the vast biological knowledge with the clinical, treatment and outcomes data to model the system in ways that are able to predict how particular interventions and screening methodologies will alter the disease state. In addition, mathematical models based on data mining results will provide a tool that allows minor perturbations of the various parameters of the model that reveals new insights to the mechanisms of disease and treatment targets. This knowledge can spawn new basic research that can validate the effectiveness and usefulness of the models, but also can strengthen the new treatment paradigms for the next generation of patient care.

To accurately assess the methods of the first stages of CER research it is necessary to create clinical trials that can be deployed across a broad healthcare network that includes primary care facilities, specialty hospitals and physician practices. By using existing networks that have been created by members of a CER consortium, the role out of new methodologies of patient care will be most effective as the parts of the network will have a vested interest, with respect to improving patient care. And with the combination of Health Information Technology (HIT) to support interoperability of information systems, the potential to leverage these resources increases significantly.

Considering a Florida CER Consortium, there is a wealth of expertise to create this venture. Through the combined efforts of the Moffitt Cancer Center, and public, private collaborators across the State of Florida and other potential partners from industry, all facets of the strategy could be timely brought together towards this focused approach. By concentrating on cancer, where Florida is uniquely positioned to have vast amounts of data due to several demographic and risk factor characteristics, CER can more efficiently discover new treatment paradigms that will be able to positively impact patient care.

This model is the general strategy proposed for CER. The creation of a National CER Center and Regional CER Consortium will focus on creating a data warehouse for specific diseases that are based on patient demographics, and overall outcomes that will be used to successfully conduct a large focused CER project. The Department of Health and Human Services will create a network that will improve patient care by harnessing the expertise of academia and industry to develop new paradigms for patient care and screening and have the ability to disseminate this techniques and methodologies to all levels of health providers. This structure will facilitate innovative research, model validation from a research and healthcare perspective and provide the necessary evidence to compare outcomes that will allow new standards of care. Through these efforts, the patients will benefit from evidence-based approaches that are able to personalize treatments and screening techniques to fit the disease and patient, providing better outcomes that should ultimately reduce economic burdens currently plaguing the healthcare enterprise.