



NEWS RELEASE

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Collaborative Partnership Pioneers Model to Improve Clinical Success of Cancer Immunotherapies *Joint White Paper Outlines New Methodological Framework to Guide Informed, Reproducible Success in Cancer Immunotherapy Clinical Development*

New York, NY, USA, and Mainz, Germany, Oct. 18, 2011 – The Cancer Immunotherapy Consortium (CIC) of the Cancer Research Institute (CRI) and the Association for Cancer Immunotherapy (CIMT) have issued a joint white paper, published in the October 13 issue of [*Nature Biotechnology*](#), outlining a comprehensive roadmap that promises to help improve and facilitate the path forward in the development of novel immunotherapies for cancer. Cancer immunotherapies that can safely and powerfully train patients' immune systems to fight their tumors have the potential to fundamentally change the way that many, if not all, cancers are treated. The enormous promise of these treatments, however, has not translated into large-scale clinical success, in significant part because of inadequate or inappropriate guidelines and tools to evaluate their clinical efficacy. As a result, cancer immunotherapy development has been a complicated enterprise, with negative consequences ranging from decreased investor confidence and industry trepidation to the perceived failure of several therapies due to the application of existing but inappropriate methodologies to evaluate their success.

The conventional framework for cancer drug development has evolved over the past fifty years to test anti-cancer treatments and evaluate their efficacy. This framework is based predominantly on observations of chemotherapeutic agents, which directly target tumor cells. Over the past decade, however, the field has learned that immunotherapies, which target the immune system and exert their anti-tumor effects indirectly, are fundamentally different from chemotherapies in many aspects including how they work, how long they take to work, and the kinds of sustained effects and particular response patterns they induce in patients. These differences have rendered the conventional framework

inadequate for evaluation of cancer immunotherapies and has necessitated the development of improved methodologies to guide the development of this unique class of cancer therapeutics.

“As the scientific understanding of cancer immunology has evolved over the past few decades, it has become necessary, in tandem, to revise the clinical methods used for immunotherapy development,” says Axel Hoos, M.D., Ph.D., co-chairman of the CRI Cancer Immunotherapy Consortium Executive Committee and the primary author of the article. “The new framework provides the missing link between scientific knowledge and clinical development and outlines an improved development path for cancer immunotherapies.”

According to Hoos, these improvements can mean the difference between life and death for cancer patients, as they can help oncologists make more informed treatment decisions based on a better understanding of immunotherapy effects. They can also affect company decisions to develop promising cancer immunotherapies and influence investor choices to finance them.

“The clear clinical development path we have outlined within this new framework increases the likelihood that a well-designed clinical trial using appropriate evaluation criteria can more accurately reflect a cancer immunotherapy’s clinical efficacy,” says Hoos.

“Immunotherapy is bringing about a profound shift in how we think about cancer treatment,” says Jill O’Donnell-Tormey, Ph.D., CRI’s chief executive officer and director of scientific affairs and co-author on the paper. “Just as the paradigm of patient care changed with the introduction of radiation treatment at the turn of the 20th century and the emergence of chemotherapy in the 1950s, immunotherapy is altering the equation in a way that affects all cancer treatment, and this has created a need for new tools to help the field as a whole integrate its knowledge.”

The new framework outlined in the October 13 paper features six components that encompass and address several methodological issues that have hampered cancer immunotherapy development to date, including:

- A new paradigm for clinical development that incorporates knowledge of the unique characteristics of immunotherapies and proposes several considerations for clinical trial design that will enable better evaluation of their therapeutic efficacy.
- International initiatives to harmonize immune monitoring assays to increase the quality of immunological monitoring across the field and enable meaningful aggregation and comparison of immune monitoring data, which will significantly enhance understanding of cancer immunotherapies’ mechanisms of action and facilitate the identification and validation of biomarkers that may help predict clinical outcomes.
- Improved study designs and clinical endpoints that better reflect the biology and time course of cancer immunotherapies and will enable a more accurate and robust analysis of therapeutic efficacy.
- Immune-related response criteria (irRC) that, while based on conventional response criteria, allow for a broader spectrum of responses observed in patients treated with cancer immunotherapies, including initial tumor progression or the appearance of new lesions. These criteria provide more comprehensive guidance to demonstrate and evaluate clinical activity in cancer immunotherapy clinical trials.
- A framework for reporting minimally required clinical immune monitoring data openly and uniformly in academic publications, allowing researchers to interpret and compare data across

clinical trials, enhancing the reproducibility of assays and results, and further supporting ongoing efforts to identify and validate biomarkers correlating with clinical outcomes.

- The development of guidance documents by regulatory authorities, such as the U.S. Food and Drug Administration and the European Medicines Agency, that can provide cancer immunotherapy developers with a clear understanding of the criteria such agencies will use to judge clinical efficacy of cancer immunotherapies, including therapeutic cancer vaccines, and trial considerations that will enable developers to best demonstrate efficacy and meet these criteria as the basis for market approval.

According to Cedrik M. Britten, M.D., CIMT executive director of translational medicine and co-author on the paper, this new framework for immune-oncology was only made possible by intensive trans-continental cooperation among networks with unique expertise in the fields of technology, immunology, oncology, industrial drug development, and regulatory aspects.

“Developing new treatments to address the huge unmet medical need in oncology is a global challenge,” says Britten. “Fortunately, we have reached a deeper understanding of the peculiar features of immune therapies, and we now have the tools to make the next leap forward toward more effective immunotherapies and their broader use.”

References:

Axel Hoos, Cedrik M. Britten, Christoph Huber, and Jill O’Donnell-Tormey. [A methodological framework to enhance the clinical success of cancer immunotherapy](#). *Nature Biotechnology* 29, 867-870 (2011).

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About Cancer Immunotherapy

Cancer immunotherapies are designed to boost the immune system’s ability to find and destroy cancer cells and control tumor growth. These immunotherapies—including immune-modulating monoclonal antibodies, therapeutic cancer vaccines, and other interventions to overcome tumor-induced immunosuppression or to potentiate the anti-tumor immune response—have the potential to be more targeted, more effective, and less toxic than today’s standard approaches to fighting this disease.

In the past two years, two active immunotherapies for cancer—the therapeutic cancer vaccine Provenge® for advanced prostate cancer and the Yervoy™ monoclonal antibody “immune checkpoint blockade” for metastatic melanoma—have received approval by the U.S. FDA, and several dozen others are in the pipeline, with an expected total of five active immunotherapies to receive FDA approval by 2015. For more information on the promise and pipeline of cancer immunotherapies, visit <http://www.cancerresearch.org>.

About the Cancer Immunotherapy Consortium of the Cancer Research Institute

The Cancer Immunotherapy Consortium (CIC), a program of the Cancer Research Institute, is an international association of more than 100 pharmaceutical and biotechnology companies and academic institutions that share a common interest in immunotherapy research and development.

CIC's mission is to improve patient care by making cancer immunotherapies part of the standard-of-care in oncology. CIC provides a platform that allows its stakeholders to advance the field by working together to achieve solutions to scientific and developmental challenges creating a single and powerful voice within the biomedical research community. From these collaborative, community-wide efforts, CIC has developed and published a number of landmark recommendations for practical solutions to key challenges in the field, including the development of new immune related response criteria and clinical trial endpoints for evaluation of cancer immunotherapy impact on patient health.

Founded in 2002 as the Cancer Vaccine Consortium (CVC), CIC amended its name in early 2010 to represent more accurately the broader diversity of the many promising clinical developments in immune-based treatment of cancer beside vaccines, including monoclonal antibodies and other modulators of immunity.

About the Association for Cancer Immunotherapy (CIMT)

The Association for Cancer Immunotherapy (CIMT) is an international networking and education platform aiming to facilitate the development of new immunotherapies against cancer for the benefit of patients. CIMT was founded in 2002, and has its home office in Mainz, Germany. The association hosts the largest European expert meetings on cancer immunotherapy, publishes therapeutic guidelines and has established two topic-specific working groups: the CIMT Regulatory Research Party, focusing on the regulatory aspects of drug development, which is pioneering the regulatory framework of actively personalized vaccines for single patient use only; and the CIMT Immunoguiding Program (CIP), which focuses on the quality control and harmonization of immunomonitoring assays. To reach its goals, CIMT closely cooperates with various partners, among them the Cancer Research Institute's Cancer Immunotherapy Consortium (CRI-CIC). Visit www.cimt.eu for more information.

About the Cancer Research Institute

The Cancer Research Institute (CRI), established in 1953, is the world's only nonprofit organization dedicated exclusively to transforming cancer patient care by advancing scientific efforts to develop new and effective immune system-based strategies to prevent, treat, and cure cancer. Guided by a world-renowned Scientific Advisory Council that includes three Nobel laureates and thirty-one members of the National Academy of Sciences, CRI has invested more than \$200 million in support of research conducted by immunologists and tumor immunologists at the world's leading medical centers and universities, and has contributed to many of the key scientific advances that demonstrate the potential for immunotherapy to change the face of cancer treatment.

To accelerate the pace of progress in the field, CRI convenes and coordinates global collaborations among academics, industry scientists and decision makers, regulatory representatives, and health research associations focused on discovery, development, and refinement of new cancer immunotherapies. A founding visionary and scientific leader in tumor immunology, CRI is helping to shape the emerging field of immuno-oncology, and is ushering in a new era of medical progress to bring more treatment options to cancer patients sooner.

The Cancer Research Institute has one of the lowest overhead expense ratios among nonprofit organizations, with more than 85 percent of its resources going directly to the support of its science,

medical, and research programs. CRI meets or exceeds all 20 standards of the Better Business Bureau Wise Giving Alliance, the most comprehensive U.S. charity evaluation service, and has earned the GuideStar Exchange Seal, indicating our commitment to the transparency of our organizational information to donors, funders, those we serve, the public, and regulators. CRI has also received an 'A' grade for fiscal disclosure and efficiency from the American Institute of Philanthropy, as well as top accolades from other charity watchdog organizations. For more information, visit <http://www.cancerresearch.org>.

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