NATIONAL CANCER INSTITUTE
IMMUNOTHERAPY AGENT WORKSHOP
JULY 12TH, 2007

EXECUTIVE SUMMARY

There is an ongoing explosion of knowledge in the immunological sciences with the discovery of many agents that have the potential to serve as immunotherapeutic drugs. For a variety of reasons, few of these are being tested in humans. The workshop developed a ranked list of agents with high potential for use in treating cancer. Despite substantial demonstrated immunological efficacy, these agents are not broadly available for testing in patients with cancer. The ranking by workshop participants was based on the likelihood for efficacy in cancer therapy and was exceedingly well-vetted, with broad and substantial input. The exceedingly broad nature of the consensus behind this list will facilitate subsequent NCI discussions on the availability of clinical grade immunotherapeutic drugs for human trials and will inform other governmental agencies, nongovernmental funding agencies, industry, and individual investigators that these agents have broad appeal to the immunotherapy community and, by consensus, hold particular promise for use in cancer therapy.

Twenty agents are presented on the list, presented in rank order. However, all are considered to have substantial potential for cancer therapy. Criteria essential for inclusion on the list included:

- Potential for use in cancer therapy.
- Perceived need by multiple, independent clinical investigators.
- Potential use in more than one clinical setting (i.e., against different tumor types or as part of multiple therapy regimens).
- Not broadly available for testing in patients.
- Not commercially available or likely to be approved for commercial use in the near future.

The 20 agents were selected from a list of 124 agents suggested to an NCI Web site asking for suggestions and advice about “agents with known substantial immunologic or physiologic activity that have not been tested or have been inadequately tested in cancer patients.” The Web site was publicized widely by the NCI with requests for advice sent to grantees with immunology or immunotherapy grants and to prior recipients of RAID awards, as well as to intramural scientists involved in immunology or immunotherapy. The Web site was further publicized to the membership of the major scientific societies involved in immunology, immunotherapy and cancer research, namely the American Association for Cancer Research (AACR), American Association of Immunologists (AAI), American Society of Oncology (ASCO), American Society of Hematology (ASH), the Cancer Vaccine Consortium (CVC), and the International Society of Biological Therapy of Cancer (iSBTc).

Web respondents expressed particular interest in vaccine adjuvants; T-cell growth factors; agents to inhibit immune checkpoint blockade; functional antibodies, cytokines, ligands, and receptors; including agents "left on the shelf" by drug companies as well as suggestions for specific antigens for vaccines and antigen-specific antibodies.

The organizing committee winnowed the list of agents to the top 30 for presentation and ranking by the Workshop. The committee focused on agents with the greatest potential for broad usage in multiple types of regimens, thereby excluding specific antigens for vaccines and antigen-specific antibodies desired by individual investigators and groups of investigators, regardless of their attractiveness or potential utility.

The workshop participants were selected from suggestions by the AACR, AAI, ASCO, ASH, CVC, and iSBTc, and by the NCI intramural and extramural programs. The participants broadly represented academia, industry, and the NCI. The workshop was open to the public. Observers from industry, the NCI, and the FDA were invited and asked to comment during the proceedings. The final ranked list derived from discussions of each agent. Agents at the top of the list were considered the most desirable based on current evidence. It was well recognized by the participants that many agents with less data, including agents not currently on the list, may ultimately prove to be more important than those at the top of the list. Although the ranking is well vetted and based on the cumulative knowledge of the broad immunotherapy and cancer research communities, the choice and desirability of individual agents will undoubtedly change with new knowledge. Because the priorities are based on incomplete knowledge, the process should be a dynamic, ongoing one that can be revised as more data appear. A common suggestion was that a mechanism should be developed to continually update the list.

Possible positive outcomes of having a well-vetted ranked list based on a broad consensus of the immunology and immunotherapy community should include encouragement of (1) RAID applications for manufacture, (2) NCI distribution of company-manufactured agents, and (3) reinvigoration of pharma/biotech efforts to develop them. Future availability of these agents for broad testing and development will provide a benchmark for the strength and resolve of the national cancer therapy development enterprise.