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Looking for Magic 'Formula' in Cancer Maintenance Therapy

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Many acute myeloid leukemia (AML) patients who achieve remission after a round of chemotherapy currently have only one medical recourse available: Wait for the cancer to come back.

Giving cytotoxic chemotherapy to a patient deemed in remission isn't really an option. And "there's no approved therapy for the maintenance of remission," said Maurits W. Geerlings, CEO of Formula Pharmaceuticals Inc.

Co-founded in 2009 by Geerlings and another biotech veteran, Giorgio Mosconi, who serves as president and chief business officer, Formula aims to in-license programs – preferably those in clinical stage – to take through early development. An immunotherapy candidate discovered at the Memorial Sloan-Kettering Cancer Center (MSKCC) with the potential as a maintenance treatment in cancers such as AML and ovarian cancer has emerged as the lead candidate, FPI-01.

Other approaches for preventing relapse after response to initial therapy have been hit and miss. In the lung cancer space, chemotherapy drug Alimta (pemetrexed, Eli Lilly and Co.) and EGFR-targeting drug Tarceva (erlotinib, Astellas Pharma Inc. and Roche AG) are approved for maintenance use, though their uptake in that indication has been limited. Roche has seen more success with its anti-CD20 antibody Mabthera (rituximab), which gained approval in the European Union for preventing relapse in follicular lymphoma patients who have responded to induction therapy.

Tarrytown, N.Y.-based EpiCept Corp. managed to gets its AML maintenance candidate Ceplene (histamine dihydrochloride) approved in Europe, but the drug has stumbled in the U.S. In September, the company said the FDA provided guidance for a new registration study testing Ceplene in combination with interleukin-2 in AML patients following first complete remission.

But, as a synthetic multi-peptide immunotherapeutic, FPI-01 could have several advantages.

For starters, it's designed to work by targeting Wilm's tumor 1 (WT-1) antigen. Not just any cancer target, WT-1 recently was ranked by the National Cancer Institute as the most relevant antigenic target for active immunotherapies, Mosconi noted.

It's overexpressed in many blood cancers and tumor solid tumors – by up to 93 percent of patients – and could have use in acute lymphoblastic leukemia, mesothelioma, lung cancer, breast cancer and prostate cancer, as well.

But Formula is going after AML first, a particularly aggressive cancer for which a successful maintenance therapy could translate into clear improvements in survival, Geerlings said.

So far, long-term survival studies have shown that after four and a half years, more than half of AML patients treated with FPI-01 are still alive. That compares to historical controls, which shows median survival rates of less than two and a half years for AML patients after first remission. While those data are small, Geerlings conceded, "they're compelling in nature."

The idea is to administer FPI-01 subcutaneously 12 times over a nine-month period to patients who have achieved complete remission on standard-of-care treatment.

"Essentially, they are waiting to relapse," he told *BioWorld Today*, adding that "the vast majority of patients have remaining cancer cells that have escaped the chemo."

As an immunotherapy, FPI-01 also is designed to overcome the shortcomings of other cancer vaccines, Geerlings said, by eliciting both CD4-positive and CD8-positive T-cell responses and enabling those T cells to overcome immune tolerance. Basically, "it teaches patients' T cells to recognize the native antigen and recognize the cancer as foreign."

Another key feature is its applicability regardless of HLA subtype. That means Formula's clinical trials can enroll all patients whose cancers test positive for the WT-1 antigen.

Phase II studies sponsored by MSKCC already are under way in AML and mesothelioma, and Formula intends to start its own Phase II study in first-remission AML patients. That trial is slated to start in the first half of 2012.

Beyond that, other Phase II studies are in the works to test FPI-01 in ovarian cancer and in patients with minimal residual disease.

Formula's founders funded early operations out of their own pockets. The company closed a Series A round with angel investors earlier this year. After completing Phase II proof of concept, the firm hopes to have attractive enough data in hand to land a collaboration.

For now, FPI-01 is the only product in the pipeline, but Geerlings said the start-up is interested in finding other promising candidates to develop.

"But we'll keep the same philosophy," he said. "We want the right drugs, the right indications and the right approach." ■