

Head and neck vaccines get 'orphan' go-ahead

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Two experimental vaccines from the University of Maryland, Baltimore (UMB) recently received a US federal orphan drug designation for advanced head and neck cancer, according to a company official.

The vaccine candidates were licensed by Gliknik, Inc., located at the University of Maryland BioPark.

Orphan status for the vaccines means that the startup firm will receive tax credits and marketing incentives from the Food and Drug Administration (FDA), which may hasten its development of the treatments. The vaccines were eligible because they are personalised for a limited number of patients.

"Advanced head and neck cancer is a challenging disease with limited treatment options. Even with chemotherapy, radiation, and surgery, people with advanced head and neck cancer may have a limited life expectancy of six to eight months," says David Block, MD, MBA, co-founder, president, and CEO of Gliknik.

The vaccines were designed in a precise manner to boost the immune system. They were invented by Scott Strome, MD, a professor at the University of Maryland School of Medicine. "The survival of head and neck cancer patients has not really improved in 30 years," says Strome, who included in the two vaccines certain compounds that act as biological recognition points for specific substances associated with some head and neck cancerous tumours.

Head and neck cancer is different from brain cancers and may include cancers of the tongue, tonsils, nasal cavity, sinuses, lips, mouth, salivary glands, throat, and larynx.

Any proposed treatment for the total number of all head and neck cancers would actually be targeting more than the maximum 200,000 patients needed to qualify for the FDA's orphan status.

However, the two vaccines owned by UMB and the Mayo Clinic Strome's former employer are at the vanguard of so-called personalized medicine that targets specific genetic traits of only certain patients. Gliknik's two vaccines are specific to molecular substances, or antigens, of tumors. That reduces the patient population well below 200,000. Also, the FDA's Office of Orphan Products Development determined that the vaccines demonstrated encouraging signs in a pilot study run in 2006 and 2007, says Block.

Strome has worked on such vaccines for seven years. He designed the vaccines while at the Mayo Clinic, filed an Investigational New Drug Application with the FDA, and had the products manufactured. After he arrived at the School of Medicine in 2005, he and Block co-founded the company. An unusual clinical "R01" grant from the National Institute of Dental and Craniofacial Research supports clinical trials of the vaccines.

"Overall, I think we have better odds than usual of making a breakthrough medicine because our science is guided by a highly innovative scientist in Scott, because of the great team we have built, and because as a company we are committed to ensuring that all the products we are working on are commercially feasible," explains Block. Gliknik is working with \$3 million from private investors generated in part through the Maryland biotechnology investment tax credit. Block credits the program as the most important tool in Maryland's arsenal for starting and nurturing young, innovative biotechnology

companies.

He believes the new vaccines have a reasonable chance to help some patients where others have failed. Humans likely form early cancer cells "all the time" says Block, while "our bodies through our immune system takes care of them and keeps them under control or kills those tumor cells." When the immune system cannot overcome the tumors, they begin to grow and eventually get other factors that allow them to metastasize, he explains.

For more than 20 years, medical researchers have pursued a cancer therapeutic vaccine as "the ultimate drug, if you will, against tumors. These trials have all failed so far. Dr. Strome designed these vaccines to learn from these past failures," says Block. In the clinical trial, only if a biopsy uncovers the target antigens of the vaccines, MAGE-A3 or HPV-16, are patients eligible to enroll in the clinical trial testing the new vaccines, he says.

A dose-determining clinical trial of the vaccines in patients with advanced head and neck squamous cell cancer began on July 1, 2008, at the University of Maryland Marlene and Stewart Greenebaum Cancer Center. Strome is the chair of otorhinolaryngology at the School of Medicine and director of tumor immunology at the Greenebaum Cancer Center.

Block was formerly executive vice president of international operations at DuPont Pharmaceuticals, chief operating officer of Celera Genomics, and founder of venture-backed Ruxton Pharmaceuticals, Inc., which was spun out of Johns Hopkins Medicine.

Source: University of Maryland Baltimore
