



FerGene Inc.

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Revolutionizing the treatment of bladder cancer

Gene therapy company FerGene Inc. is developing products for the treatment of urologic cancers, the company's lead product, nadofaragene firadenovec, uses a non-replicating adenovirus vector to deliver the gene for interferon alfa-2b into bladder cells for the treatment of bladder cancer.

In the US, there are around 81,400¹ new cases of bladder cancer diagnosed each year. Non-muscle-invasive bladder cancer (NMIBC) makes up around 75%² of all new cases. Bladder cancer has high rates of recurrence and is the most expensive cancer to treat². Patients with high-risk NMIBC are commonly treated with an induction dose of the immunotherapeutic BCG (Bacillus Calmette-Guérin) instilled directly into the bladder. Due to a severe BCG shortage, guidelines now recommend adjuvant BCG usage for only high-risk NMIBC patients³.

Although 80% of patients will achieve an initial complete response (CR) to a course of induction and maintenance BCG, within the first year, over 50% of patients with an initial response to BCG will experience recurrence and progression, and many will develop disease that is BCG-unresponsive⁴.

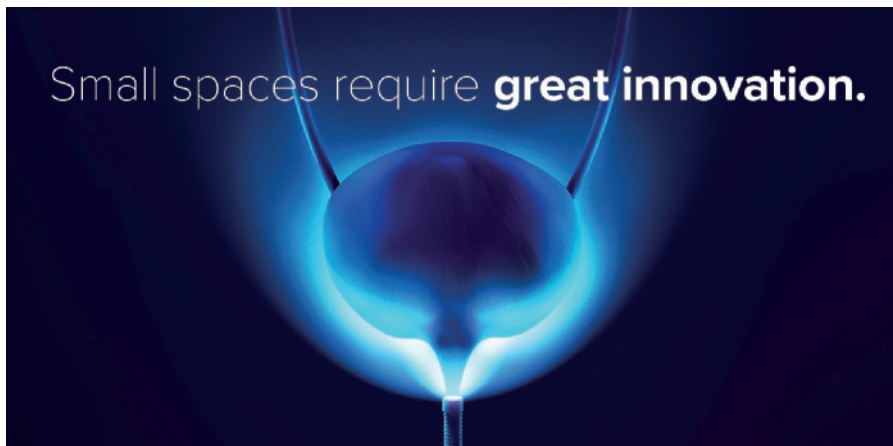
"Bladder cancer is a large area of medical need, particularly in patients where BCG has failed," said Vijay Kasturi, MD, Senior Vice President, Scientific Affairs. "There have been few advances in the standard-of-care in the past twenty years. Once patients stop responding to BCG, there are few alternatives. One of those alternatives is removal of the bladder which offers the most definitive cancer treatment; however, it is associated with high morbidity, and many patients are unwilling or unable to undergo the procedure."

FerGene Inc., a new gene therapy company, aims to develop and market a portfolio of products for the treatment of urologic cancers, starting with what the company hopes will be the world's first bladder cancer gene therapy to reach the market. The company was launched in 2019 with investment of more than \$570 million.

"We believe collaboration breeds innovation and we are committed to working with partners that share our vision," said David Meek, President and CEO.

Creating a new treatment for bladder cancer

FerGene's lead product is nadofaragene firadenovec, a gene therapy that uses a non-replicating adenovirus vector to deliver the gene for interferon alfa-2b into the bladder cells. After treatment, the cells begin to secrete interferon alfa-2b. The results of a phase 3 trial of nadofaragene firadenovec in BCG-unresponsive NMIBC has been recently reported in *The Lancet Oncology*⁴. The study was conducted in collaboration with the Society of Urologic Oncology Clinical Trials Consortium (SUO-CTC), a clinical research investigator network and serves as an ideal model for clinical trials. The drug is being developed by urologists for urologists.



"Bladder cancer that recurs after BCG has historically been very difficult to treat," said Stephen A. Boorjian, MD, Professor of Urology at the Mayo Clinic, Rochester, US, and principal investigator of the phase 3 study. "Nadofaragene firadenovec promises to be a clinician- and patient-friendly and well-tolerated treatment option. It is instilled once every three months directly into the bladder and can be administered in an outpatient setting," Boorjian explained.

FerGene has rights for nadofaragene firadenovec in bladder cancer in the US. A Biologics License Application has been submitted to the U.S. Food and Drug Administration (FDA) for the treatment of high-grade BCG-unresponsive NMIBC. The FDA has granted breakthrough therapy designation.

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David Meek, President and CEO, FerGene

The next steps for uro-oncology

FerGene is looking at new indications for nadofaragene firadenovec. These include as a monotherapy as first-line treatment for NMIBC, which could be important as BCG is in short supply in the US and elsewhere.

"Addressing the needs of BCG-naïve patients will give us access to another 35 or 40% of the market," said Ambaw Bellele, Chief Operating

Officer. Bellele said, "We have a robust lifecycle management program for nadofaragene firadenovec in place."

FerGene's leadership team is seeking complementary products in bladder cancer or other uro-oncologic indications, such as prostate cancer, to add to its pipeline and portfolio. As well as licensing in or co-developing earlier stage products, it also can offer co-promotion through its field-based specialist uro-oncology sales team.

There is an opportunity to combine nadofaragene firadenovec with other therapies and FerGene has already been approached by companies following the publication of its pivotal phase 3 data in *The Lancet Oncology* in November 2020.

"While we strongly believe our lead product alone is enough to be successful, we want to build the company up as a sustainable biotech for urologic cancers," said Meek. "We will continue to develop new treatment options because meeting the needs of patients comes first."

1. National Cancer Institute. Cancer Stat Facts: Bladder Cancer. SEER Cancer <https://seer.cancer.gov/statfacts/html/urinb.html> (2020).

2. Mossanen, M. et al. *World J. Urol.* **37**, 2059–2065 (2019).

3. American Urological Association. BCG shortage info—American Urological Association. <https://www.auanet.org/about-us/bcg-shortage-info>

4. Boorjian, S. et al. *Lancet Oncol.* **22**, 107–117 (2020).

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