

## [394] GENE THERAPY FOR EARLY STAGE BLADDER CANCER: THE PIVOTAL PROGRAM WITH INODIFTAGENE

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- **Investment Rationale**

Anchiano Therapeutics is developing an investigational gene therapy for early stage, non-muscle invasive bladder cancer (NMIBC), a large market without recent new agents. Preliminary data from our development program, and FDA agreement, form the foundation for a path to potential approval with either of two trials. Interim open label data from the first trial are expected by end 2019.

- **Business Strategy**

NMIBC is an area of substantial unmet need. The last drug approved by the FDA was marketed in 1998, and over 60,000 patients are diagnosed yearly in the US with NMIBC, for which outcomes are poor. We estimate that the market for our technology, if approved in the indications in which we are conducting trials, is well over \$1.5B.

- **Core Technology**

Our technology, inodiftagene vixteplasmid, is a first-in-class gene therapy for NMIBC. Its design utilizes Anchiano's core intellectual property, a gene designated as H19, to direct therapy specifically to bladder cancer cells. Inodiftagene is administered to the bladder lining, where it is taken up by both normal and malignant bladder cells, but exerts its lethal toxicity only against the cancer.

- **Product Profile/Pipeline**

We have carried out six clinical trials of inodiftagene in cancer, including three in NMIBC. Data demonstrate activity against ovarian, pancreatic and bladder cancer. All three NMIBC trials demonstrate complete responses. Two registrational studies will provide independent routes to potential approval in separate, but related, indications. The first trial is ongoing, the second planned for later this year.

- **What's Next?**

Anchiano carried out a US public offering in February, having raised \$30.5M (Nasdaq:ANCN). Proceeds will fund operations into mid-2020. Interim data from the first 35 patients of the registrational study, one quarter of the total enrollment of 140, will be available in Q4 2019. We anticipate initiation of our second, larger randomized study, in late 2019.