BC-819
RECOMBINANT DNA THERAPY FOR BLADDER CANCER
BioCanCell
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BioCanCell is Developing a Phase 3 Asset with a >$1.5 Billion Market Potential

Lead product BC-819, gene therapy for early stage bladder cancer

- Data from three clinical trials demonstrate results that surpass published FDA guidance for approvability
- First-in-class approach uses recombinant engineering to specifically target cancer

Early stage bladder cancer a large and underserved population

- Standard of care utilizes agent introduced in the 1970’s, FDA actively identified unmet need
- Large majority of patients relapse and go on to radical surgery or distant metastasis
- BC-819 market size upwards of $1.5 billion

Poised to launch FDA-approved pivotal program targeting two indications

- Two clinical studies prepared to initiate
- Trials are independent paths to approval: two shots on goal
- Randomized study is unique, has an SPA from FDA, and is without competition
BC-819 CLINICAL DATA IN BLADDER CANCER

Three Studies Support Approvability in >$1B Population

BC-819 results in 33% complete responses in marker papillarity tumors, 86% CRs in CIS alone and with BCG

Monotherapy durability surpasses historical and competitor experience:

• FDA specified in CIS 30% recurrence-free rate at 18-24 months, excluding 20%, as being an approvable endpoint

• Phase 2 study demonstrates 18- and 24-month rates are >30% (right)

• 46% 12-month rate compares to competitors’ rates of 35% and 15% at 12 months

BC-819 in combination with BCG shows 3-mo and 6-mo DFS of 95% and 74%


MONTHS

46% 12-mos DFS
32% 24-mos DFS

Historical 20% 24-mos DFS
NON-MUSCLE-INVASIVE BLADDER CANCER: NMIBC

NMIBC is a Common Cancer in Need of New Therapies

### In the United States

**4th** MOST COMMON CANCER IN MALES

- **77,000** NEW CASES PER YEAR
- **570,000** PREVALENT CASES

### Quality of Life Issues

- Repeated recurrence
- Repeated cystoscopy, surgery and drug treatment cycles
- Lifelong cystoscopy follow-up
- Most expensive cancer to treat

### Worldwide

**9th** MOST COMMON CANCER

- **150,000** EU NEW CASES/YEAR
- **420,000** WORLDWIDE NEW CASES/YEAR

### No New Drugs in 20 years

- **0** Drugs approved by FDA since 1998 for NMIBC
NMIBC CLASSIFICATION AND TREATMENT

Recurrence Leads to Progression and Metastasis

- Patients are diagnosed and evaluated via cystoscope, at left
- Tumors are identified on the inner surface of the bladder and classified by depth

- Non-muscle invasive bladder cancer (NMIBC) patients are the focus of BC-819 therapy

• Treatment for NMIBC is surgery to remove the small tumors, then treat with BCG. BCG is live attenuated tuberculosis bacteria.
• 70% of patients’ tumors ultimately fail BCG, necessitating radical cystectomy.
• Patients whose tumors fail BCG are those who need BC-819.

Over 200,000 Existing US Patients Could Benefit From BC-819

- **75%** of prevalent bladder cancer cases are NMIBC (non-muscle-invasive bladder cancer)
- **85%** are intermediate and high risk
- **~80%** of intermediate and high risk cases are treated with BCG
- **70%** of NMIBC patients recur after BCG treatment

BioCanCell U.S. patient population:
- **205,000** recurrent NMIBC cases after BCG treatment

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2 All figures refer to NMIBC (non-muscle-invasive bladder cancer)
2 Decision Resources
3 Source: IMS data 2011
BC-819 GENE THERAPY

Unique Technology, Potent Mechanism

A first-in-class, first-of-its-kind gene therapy

• Targeted recombinant DNA-based gene construct for bladder cancer, engineered to express lethal toxin specifically in malignant cells

Plasmid based, specifically targeted

• BC-819 is a recombinant DNA plasmid containing H19 regulatory sequences driving expression of diphtheria toxin A chain only in malignant cells

Potent lethal payload, efficient delivery

• Diphtheria toxin is delivered to the cells by the plasmid: one molecule may kill a cell, and it is engineered to prevent transfer between cells

No need to prove new mechanism of action

• Unlike most cancer therapies, for BC-819 there is no need to validate a new target or pathway; mechanism is well understood
EXPRESSION OF H19 IN NORMAL AND MALIGNANT CELLS

BC-819 Uses H19 to Target Cancer, but not Normal Cells

H19 is not normally expressed in adult tissues, but is expressed in a variety of human cancers

Virtually no expression in normal human tissues (Fig. A, left)

H19 expression has been identified broadly in human cancers, including especially bladder carcinoma (Fig B)

In our phase 2 study of BC-819, at entry 47 patients were tested for H19, and all 47 demonstrated H19 expression.

H19 is expressed in all subtypes of NMIBC, including CIS
Results from Three Trials Support a Linear Path to Approval Based on FDA Guidance

<table>
<thead>
<tr>
<th>Trial</th>
<th>Status</th>
<th>Result</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Phase 1/2</strong></td>
<td>Complete; N=18</td>
<td>Favorable safety; 22% complete response rate</td>
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<tr>
<td><strong>Phase 2</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Monotherapy</td>
<td>Complete; N=47</td>
<td>33% complete responses; 46% durable response rate at 1 year</td>
</tr>
<tr>
<td><strong>Phase 2</strong></td>
<td>Enrollment Complete; N=38</td>
<td>3 month DFS 95%; 6 month DFS 74%; median time to progression not yet reached</td>
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BC-819 CLINICAL DEVELOPMENT PROGRAM

Pathway to Registration in Two Discrete Indications

204
Phase 2 trial is a single-arm path with FDA concurrence to full approval in third line patients

• Open label, interim analyses at 35 patients essentially allows repeat of phase 2 experience in US

301
Phase 3 trial is approved under SPA and will support indication in second line

• Trial has been granted an SPA by the FDA
• This trial is complementary to the phase 2

These two trials provide independent routes to approval in two separate (but related) indications
BC-819 INITIAL REGISTRATIONAL STUDY: PHASE 2 TRIAL 204

Patients whose Disease is BCG-Unresponsive: Third Line

Patients: High-risk NMIBC in pts with BCG-unresponsive disease; will require approximately 140 patients

- Single arm trial **for approval**
- Open label: interim analysis at 35 CIS patients
- FDA agreement: stated a single-arm study in this population could lead to approval. Spanish, German, Canadian, UK and French regulators also support study
BCG Failure Patients Under SPA From FDA: Second Line

Patients: Intermediate or high risk NMIBC after at least one failed course BCG; will require approximately 495 patients

- FDA reviewed, granted SPA, certifying it could meet condition for full approval
- Spanish, German, Canadian, UK and French regulators support study as well

Phase III 301

N = 247

BC-819 + BCG

BCG alone

Primary endpoint: Median time to recurrence

Open Label Interim analysis
BC-819 CLINICAL DEVELOPMENT

Unique Strategy for BC-819 Approval in Two Indications

- **Standard care**
  - Non-muscle invasive (NMIBC)³
  - Surgery BCG 1L → Recurrence BCG 2L → Recurrence Cystectomy

- **BC 204**
  - Non-muscle invasive (NMIBC)³
  - Surgery BCG 1L → Recurrence BCG 2L → Recurrence **BC-819 3L 1 yr DFS 46%**

- **BC 301**
  - Non-muscle invasive (NMIBC)³
  - Surgery BCG 1L → Recurrence **BC-819 2L** → Recurrence Cystectomy

- Development plan in 2L, the 301 patient population, is unique and at this time without a competitor. Over two-thirds of the market potential of NMIBC therapy is in 2L therapy.
COMMERCIAL MODELING OF NMIBC MARKET

BC-819 Approval Predicts a Market of well over $1 Billion

272,000
NMIBC PATIENTS ELIGIBLE FOR DRUG TREATMENT, US, EU AND JAPAN

60,000
PATIENTS ELIGIBLE FOR BC-819 TREATMENT, US, EU AND JAPAN

Approximately $600M
PROJECTED YEAR-5 US, EU AND JAPAN SALES

Over $1.5 Billion
PROJECTED PEAK US, EU AND JAPAN SALES

- 257,000 new cases of bladder cancer in 2017 in US, EU, and Japan
- 73-81% of those patients present with NMIBC
- 272,000 incident and recurrent NMIBC are eligible for drug treatment
- ~60,000 of all drug treatable patients either failed or unresponsive are eligible for BC-819 therapy
- Company-estimated market penetration at year 5:
  - BCG failure 20-24%
  - BCG unresponsive 20–24%
- Assumes cost per patient per year of ~$80,000
US BASED CLINICAL DEVELOPMENT TEAM

BioCanCell is Building a New Company in Cambridge

Frank Haluska, MD, PhD
President and Chief Executive Officer

Harvard Medical faculty, ARIAD CMO, led global CRD team and two oncology drug approvals

Yan Moore, MD, MBA
Chief Medical Officer and Sr. Vice President of Clinical Development

Extensive pharma experience, multiple product launches

Jonathan Burgin, MBA, CPA
Chief Financial Officer and Chief Operating Officer

Former BC CEO, CFO of TASE and NASDAQ companies

Ron Knickerbocker, PhD
Senior Vice President of Clinical Development and Data Sciences

Designed and analyzed clinical trials for two successful NDAs

Sean Daly
Vice President of Clinical Operations

Conducted trials for two approvals, one globally
CLINICAL OPERATIONS

Funding Plans and Clinical Trial Timelines

• Have announced round of private financing led by Israeli and US investors with present allocation of approximately $23M, priced at 1.35 NIS ($0.38 USD), with 80% warrant coverage and price protections. The transaction values the company at $35M. The round will close in June 2018

• Objective is to undertake US listing/IPO in 2018

• Prepared to initiate clinical trial operations for both registrational studies in 2018 based on funding, enrolling first patients in mid 2018 in the single arm 204 study, and initiating randomized study late 2018

• $23M will support phase 2 registrational study through the data from the interim analysis
Key Takeaways

Potential for first-of-its-kind DNA-directed cancer therapy in non-muscle invasive bladder cancer (NMIBC), a serious area of unmet need—**BC-819**

Strong, experienced management team and newly expanding global organization

Preliminary data from development program and FDA agreement support direct path to approval with either of two trials

Over $1.5 billion commercial potential serving large global population in need of new therapy and uniquely addressing second line treatment

Private financing announced; plan for US IPO later in 2018