Anchiano therapeutics

INODIFTAGENE

Gene Therapy for Bladder Cancer

March 2019

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Inodiftagene for Non-Muscle Invasive Bladder Cancer

INODIFTAGENE

First-in-class, DNA-based gene therapy moving into registrational development in early stage bladder cancer

Data from six clinical trials show activity in pancreatic, ovarian and bladder cancer, with complete and durable responses in NMIBC

Conducting 2 pivotal trials, each of which could lead to approval. The first is open to enrollment, the second planned for 2019

Non-Muscle Invasive Bladder Cancer (NMIBC)

A large and underserved population

More than $1.5 billion commercial global opportunity

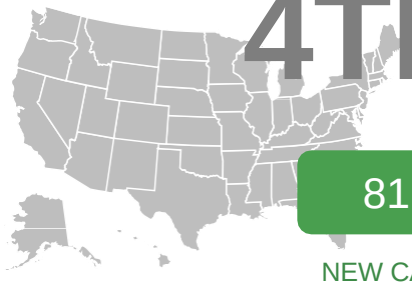
Current standard-of-care is a therapy introduced in the 1970s; patients who relapse go on to radical surgery or distant metastasis

Potential market of

$1.5 billion

Non-Muscle Invasive Bladder Cancer: NMIBC

NMIBC is a common cancer in need of new therapies



4TH MOST COMMON CANCER IN MALES BEHIND ONLY LUNG, COLON, PROSTATE

In the United States[1]

81,000
NEW CASES IN 2018

708,000
PREVALENT CASES IN 2015

5TH MOST COMMON CANCER OVERALL IN EU

EU and Worldwide[2,3]

141,000
EU EXPECTED NEW CASES IN 2020

550,000
WORLDWIDE NEW CASES/YEAR

Quality of Life Issues

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

No New Drugs in 20 Years

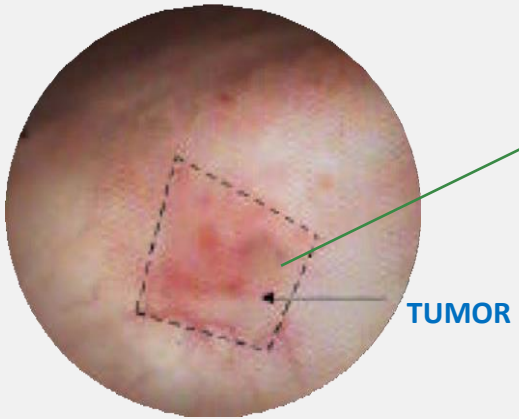
0

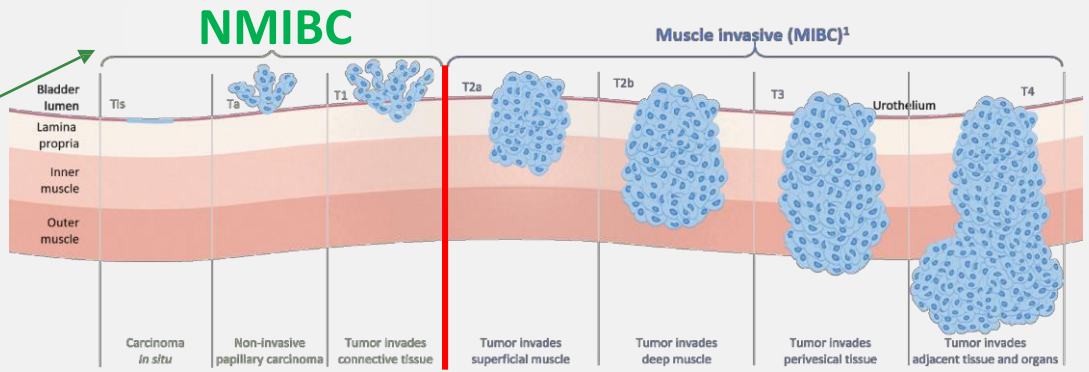
Drugs approved by FDA since 1998 for NMIBC

1. ACS Cancer Facts and Figures 2018, www.cancer.org; 2. https://ec.europa.eu/jrc/en/publication/epidemiology-bladder-cancer-Europe; 3.https://wcrf.org/dietandcancer/cancer-trends/bladder-cancer-statistics

NMIBC Classification and Treatment

Recurrence leads to progression and metastasis



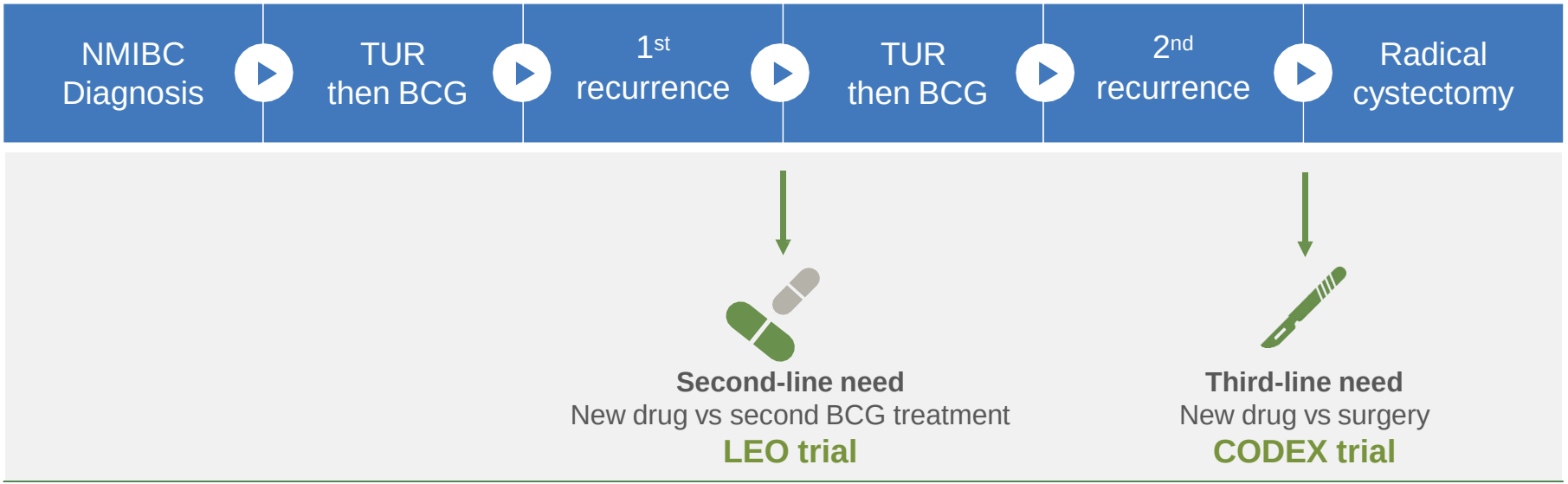


DIAGNOSIS	LOCALIZATION	THERAPY
Patients are diagnosed and evaluated via cystoscope	Tumors are identified on the inner surface of the bladder, resected and classified by depth	NMIBC patients initially receive Bacillus Calmette Guerin (BCG) and are the focus of inodiftagene therapy

Sources: Knowles MA and CD Hurst, 2015. *Nature Rev Cancer*, 15: 25-41, Link. : NCCN Bladder Cancer Guidelines, v. 1.2017.

Two Unmet Needs in NMIBC Therapy

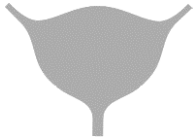
Inodiftagene addresses both



Patients whose tumors recur after one or two courses of BCG are those who are eligible for inodiftagene

Over 90,000 NMIBC Patients Are Eligible for Inodiftagene Annually

285,000 constitute NMIBC global incident and recurrent population



260,000

Number of incident bladder cancer cases in 2017 in US, EU, and Japan

195,000

Cases of bladder cancer that is NMIBC, about 70-80% of total

90,000

Annual number of NMIBC patients who suffer recurrence after treatment

285,000

Total number of incident and recurrent NMIBC cases who are eligible for treatment annually

Of these, approximately 90,000 intermediate and high-risk patients whose first-line or second-line BCG therapy has failed are eligible for therapy with inodiftagene

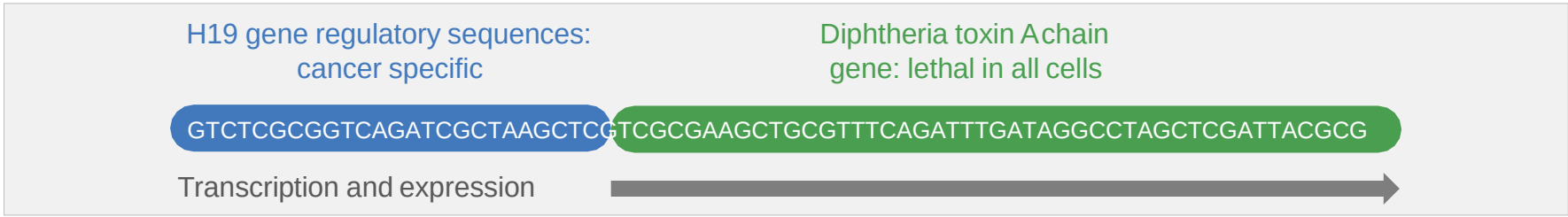
Sources: The Nemetz Group, Decision Resources

First-in-Class, First-of-its-Kind Treatment

Inodiftagene vixteplasmid gene therapy

Targeted gene therapy

Inodiftagene is a recombinant DNA molecule containing regulatory sequences from the H19 gene driving expression of diphtheria toxin A chain gene **only in malignant cells**



Diphtheria toxin gene: efficient delivery

Plasmid facilitates high transfection efficiency. In vitro uptake demonstrable in 85% of cells after a single exposure; in clinic detectable in bladder more than 48 hours after instillation, and administered weekly to every third week for up to 3 years

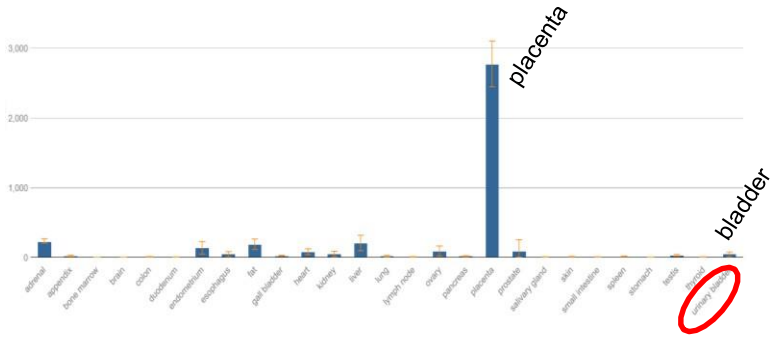
Well-understood and validated mechanism-of-action

Lethal inhibition of protein synthesis

Uses H19 to Target Cancer Cells Avoiding Normal Cells

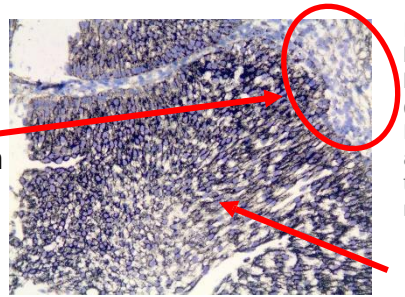
Inodiftagene mechanism of action

A



H19 expression in a panel of 27 human tissue samples. See https://www.ncbi.nlm.nih.gov/gene/283120

B



No H19 expression in normal tissue

In situ hybridization (ISH) of H19 in bladder cancer. Histopathology of bladder cancer stained to demonstrate H19 expression. Black grains (arrow) demonstrate abnormal expression. Normal tissue (top right of section) does not stain for H19.

H19 expression in tumor

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

Figure A: Shows virtually **no H19 expression in normal human tissues** including in normal bladder (in red circle)

Figure B: H19 expression has been identified broadly in human cancers, including **especially bladder carcinoma**

H19 is expressed in all subtypes of NMIBC, including carcinoma in situ (CIS)

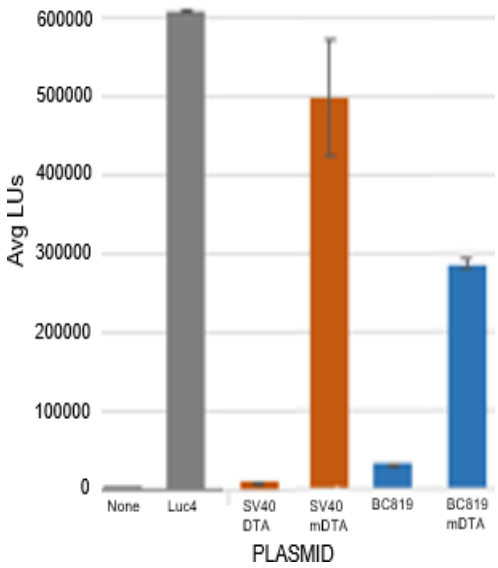
In our phase 2 study of inodiftagene, 96% of screened patients expressed H19, and **all 47 entered patients demonstrated H19 expression**.

Inodiftagene Inhibits Protein Translation and Cell Proliferation

In vitro studies demonstrate mechanism of action

A

Inodiftagene (BC-819) potently inhibits protein translation and requires functional DTA



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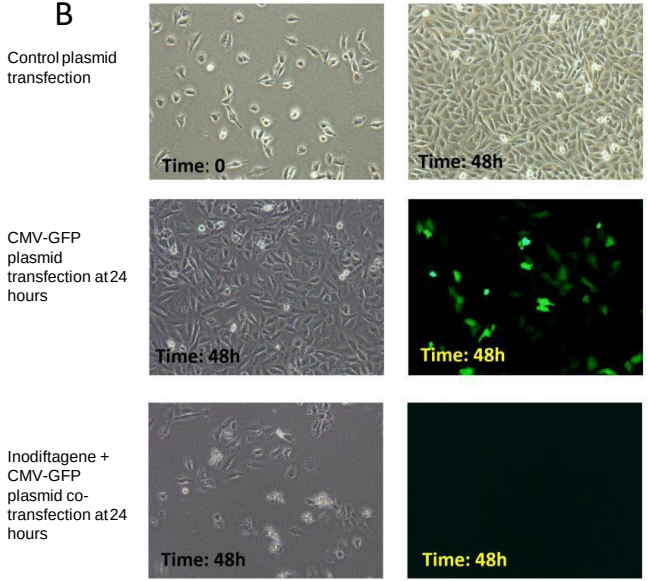


Inhibition of protein translation and proliferation by inodiftagene in tissue culture

Inodiftagene potently inhibits luciferase translation and is specific to the activity of functional DTA (A)

Inodiftagene inhibits translation and bladder cancer cell proliferation (B)

Inodiftagene activity was investigated in tissue culture. Bladder cancer cells were transfected with plasmid/PEI under various conditions. (A) Co-transfection of a luciferase reporter plasmid with inodiftagene (BC-819 in the figure) and control plasmids shows inhibition of luciferase activity by inodiftagene and DTA plasmid BC-821 but not by a variety of plasmids lacking DTA. (B) DTA expressed from an SV40 promoter or in inodiftagene (BC-819) potently inhibits luciferase. Constructs containing attenuated DTA carrying point mutations (mDTA) are less effective, demonstrating specificity of inhibition to active DTA. (C) Inodiftagene co-transfected with a green fluorescent protein (GFP)-producing plasmid into bladder cancer cells demonstrates inhibition of proliferation and abrogation of translation of GFP.

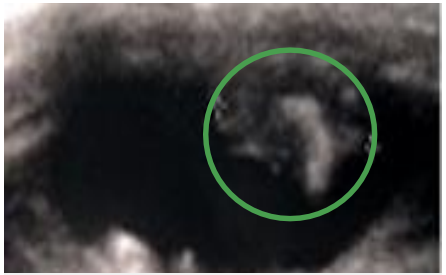
Effective in Eliminating Experimental Bladder Cancer

Inodiftagene in vivo

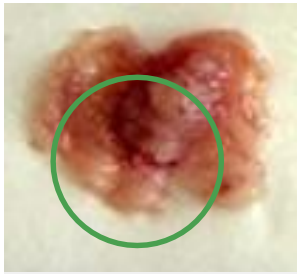
Animal model data demonstrate that intravesical instillation of inodiftagene eliminates rat bladder cancers

Analysis of inodiftagene-treated rat bladders by ultrasound and at necropsy shows progression of experimentally induced tumor when treated with control vector (left) but absence of tumor when treated with inodiftagene (right)

Superficial Tumor

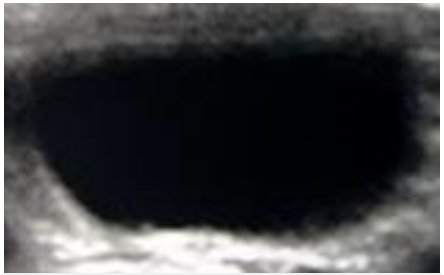


Ultrasound



Resected Bladder

Tumor Response



Ultrasound

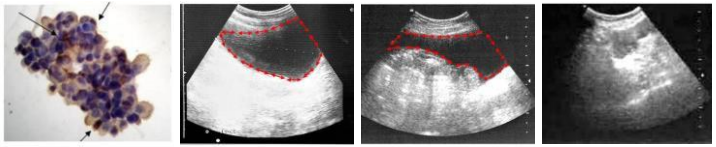
Resected Bladder

Wistar rats received N-butyl-N(4-hydroxybutyl) nitrosamine (BBN), a potent carcinogenic alkylating agent, in drinking water for 5-30 weeks. Tumors were evident by 10 weeks, with superficial invasion evident by 15 weeks and typically deep invasion by 20 weeks. At 19 weeks 100 ug of control luciferase vector (left) or inodiftagene (right) was instilled weekly for 5 weeks intravesically.

Responses in Advanced Ovarian and Pancreatic Cancer

Inodiftagene activity in solid tumors validates mechanism of action

Complete resolution of ascites following instillation of inodiftagene



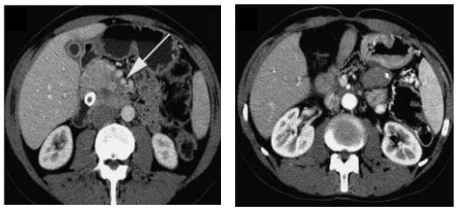
Left to right: H19-positive ovarian cells from ascites; ultrasound of abdomen at baseline, prior to 5th treatment, and after 10th treatment. Red border demarcates ascites, resolved at right

Advanced pancreatic cancer responses to monotherapy: 2 partial responses with inodiftagene alone

Table 5. Subject status at 3 months follow-up

Cohort #	Subject ID	End of study at 4 weeks	3 Months	Other treatments
1	201	PD	PD	None
1	202	PD	SD	Chemotherapy
1	602	SD	PD	Chemotherapy
2	204	PD[a]	PR[a]	None
2	205	SD	PR	Chemotherapy
2	301	PD	SD	Chemotherapy
2	501	SD	PD	Radiation
2	604	SD[b]	PR[b]	None
2	1102	SD	SD	Chemoradiation - Complete Resection at 3 months

Complete resection of advanced pancreatic cancer following inodiftagene, chemoradiation and surgery



Left baseline tumor; right complete resection of tumor following inodiftagene and multimodality therapy

Complete resolution of refractory malignant ascites in ovarian cancer patient who received inodiftagene injected intra-abdominally as compassionate use[1]

Partial responses observed in 2/9 patients with advanced localized pancreatic cancer who received only inodiftagene intratumoral injection; third patient had complete control of tumor following chemo-radiation and resection (shown)[2]. In additional trial with gemcitabine, 1/12 partial responses

1. Mizrahi et al., J. Med. Case Reports 2:228, 2010 http://www.jmedicalcasereports.com/content/4/1/228; 2. Ohana et al., Cancer Gene Therapy 19:374, 2012.

Three Completed NMIBC Trials Support Pivotal Study Designs

Inodiftagene clinical strategy

CLINICAL PROGRAM

Trial	Status	Result
Phase 1/2 Monotherapy	Complete; N = 18	Well tolerated, no DLT or MTD identified at doses tested; 22% complete responses in marker tumors
Phase 2 Monotherapy	Complete; N = 47	33% complete responses in marker tumors; 46% durable response rate at 1 year
Phase 2 Combination with BCG	Complete; N = 38	3 month DFS 95%; 6 month DFS 78%; median time to progression not yet reached

Trial Results Support Path to Approval Based on FDA Guidance

DLT: dose limiting toxicity; MTD: maximum tolerated dose

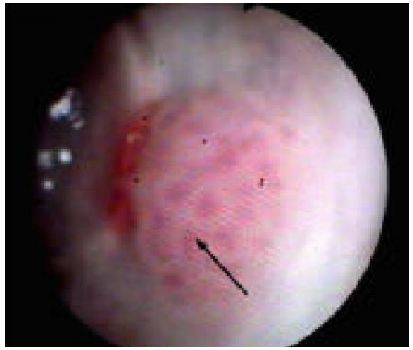
Complete Responses in all Three NMIBC Trials

Inodiftagene consistently showed clinically meaningful anti-cancer activity

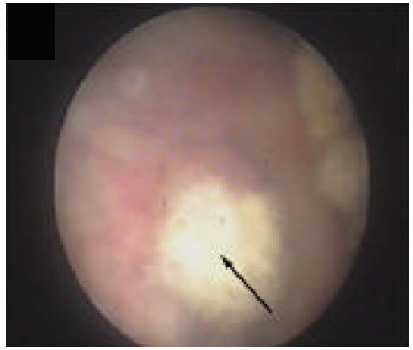
Complete responses observed in 17/57 patients (30%) in two monotherapy trials demonstrating activity against unresected papillary cancer

In addition, **complete responses in 6/7 (86%) of CIS** patients (most in combination study) at 3 months

This is not standard of care: it is an investigative approach to demonstrating anti-tumor activity and is FDA-recommended



Baseline
papillary tumor



3 weeks following 6th instillation of inodiftagene
complete resolution

Durability of Response Demonstrated in Phase 2 Monotherapy Trial

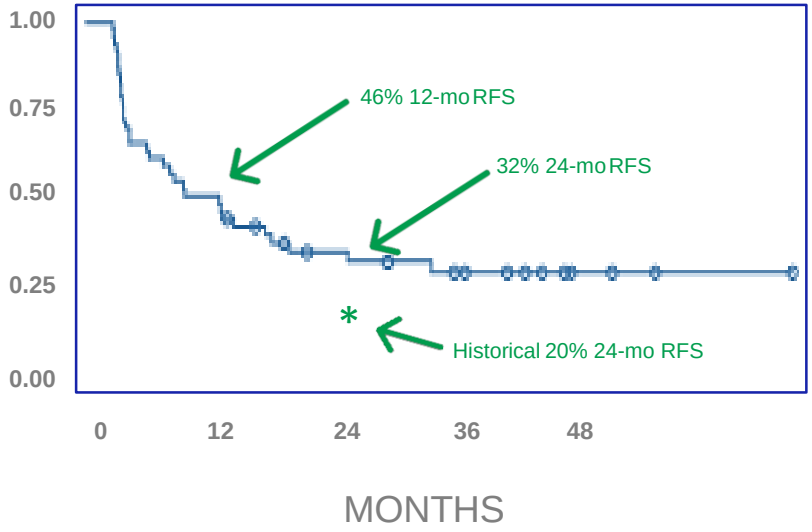
Inodiftagene phase 2 monotherapy results

33% CR rate in marker lesions

46% 12-month RFS rate

23% of patients has adverse events (AE) thought related to treatment; overall **3/47** serious adverse events (SAEs)

18- and 24-month RFS rates are **~32%.** FDA guidance suggests >30% RFS rate at 18-24 months as being approvable in CIS in adequate population.[1]

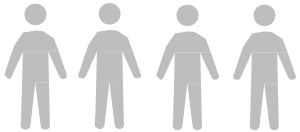


1. Jarow et al., J. Urology 83:262, 2014

Pathway to Registration in Two Discrete Indications

Inodiftagene registrational program

Codex



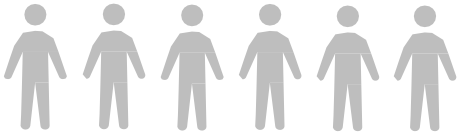
Codex phase 2 pivotal study
trial is a single-arm path with FDA concurrence designed for approval in **third line** patients

Monotherapy, 140 patients, single arm

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

Open to enrollment in US

Leo



Leo phase 3 pivotal study
trial is approved under SPA and will support indication in **second line** patients

Combination therapy, 500 patients, randomized

Trial has been granted an SPA by the FDA

This trial is complementary to the phase 2

These two trials provide independent routes to potential approval in two separate (but related) indications

Codex Study (204 Trial): Initial Registrational Trial Design

Inodiftagene phase 2 trial in third-line patients

SINGLE ARM TRIAL
For approval

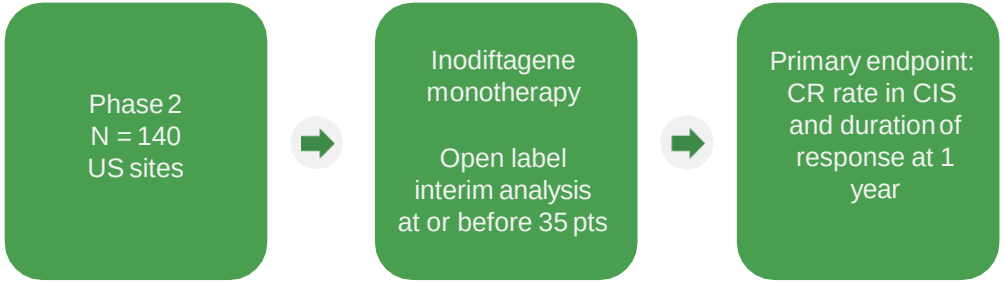
OPEN TO ENROLLMENT
Actively recruiting

OPEN LABEL
interim analysis of CR rate at or before 35 CIS patients beginning at 3 months

FDA AGREEMENT
stated single-arm study could lead to approval. EU and Canadian regulators also support study conduct

Third-line patients: high-risk BCG-unresponsive NMIBC after two failed courses of BCG

N = approximately 140 patients



Inodiftagene administered QW x 10 weeks then Q3W

Leo Study (301 Trial): Second Registrational Trial Design

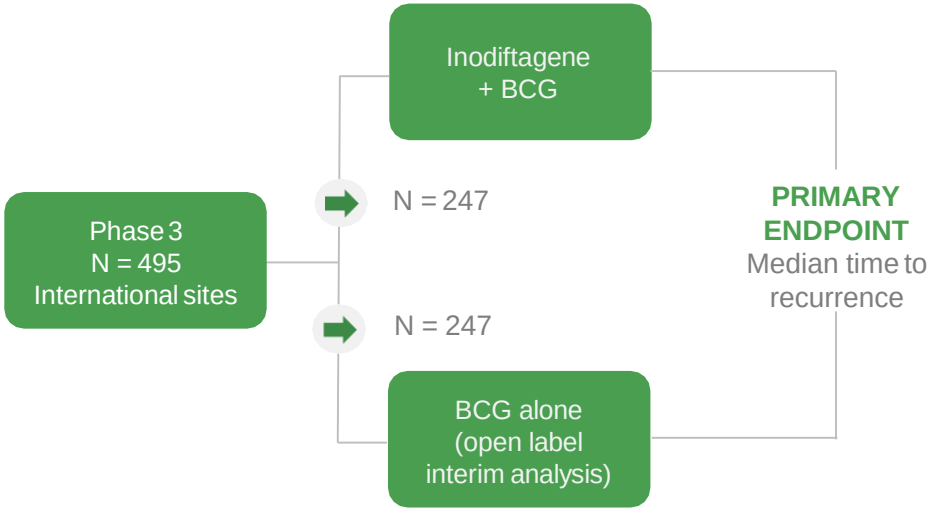
Inodiftagene phase 3 trial in second-line patients

RANDOMIZED TRIAL
For approval

FDA REVIEWED, GRANTED SPA,
certifying it could meet condition for full approval[1]

SPANISH, GERMAN, CANADIAN, UK AND FRENCH REGULATORS
support study conduct as well

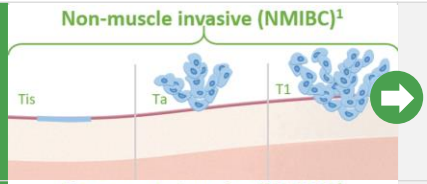
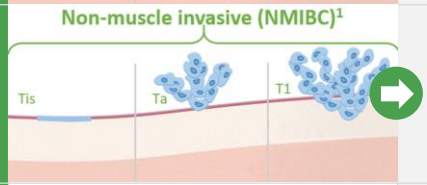
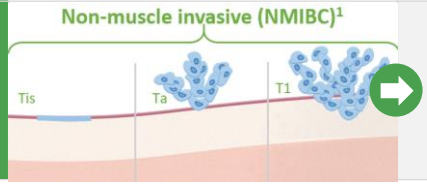
Second-line patients: intermediate or high risk NMIBC after one failed course of BCG
N = approximately 495 patients



1. Granting of an SPA does not guarantee approval even if trial is positive

Unique Strategy for Inodiftagene Approval in Two Indications

Inodiftagene clinical development strategy

Standard of care		**TUR BCG 1L**	**Recurrence TUR, BCG 2L**	**Recurrence Cystectomy**
Codex		TUR BCG	Recurrence TUR, BCG	Recurrence **Inodiftagene 3L**
Leo		TUR BCG	Recurrence TUR, BCG **Inodiftagene 2L**	Recurrence Cystectomy

Development plan in second-line patients, the Leo patient population, addresses the majority of the market potential of NMIBC therapy

Experienced Management Team

US-based clinical development team with record of US approvals with FDA

	**Frank G. Haluska, MD, PhD** President and Chief Executive Officer	Former Harvard Medical faculty, ARIAD CMO, led global research team and two oncology drug approvals
	**Jonathan Burgin, MBA, CPA** Chief Financial Officer and Chief Operating Officer	Former Anchiano CEO, CFO of TASE and Nasdaq companies
	**David Kerstein, MD** Chief Medical Officer	Former Takeda Lung Cancer Clinical Portfolio Strategy Lead
	**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	Successfully conducted clinical trials supporting two approvals
	**Michal Gilon, PhD** Vice President of Research and Development	Extensive research experience in the fields of molecular and developmental biology

Funding Plans and Upcoming Milestones

Clinical trial timelines

4Q 2018: Codex trial initiated for registration of inodiftagene

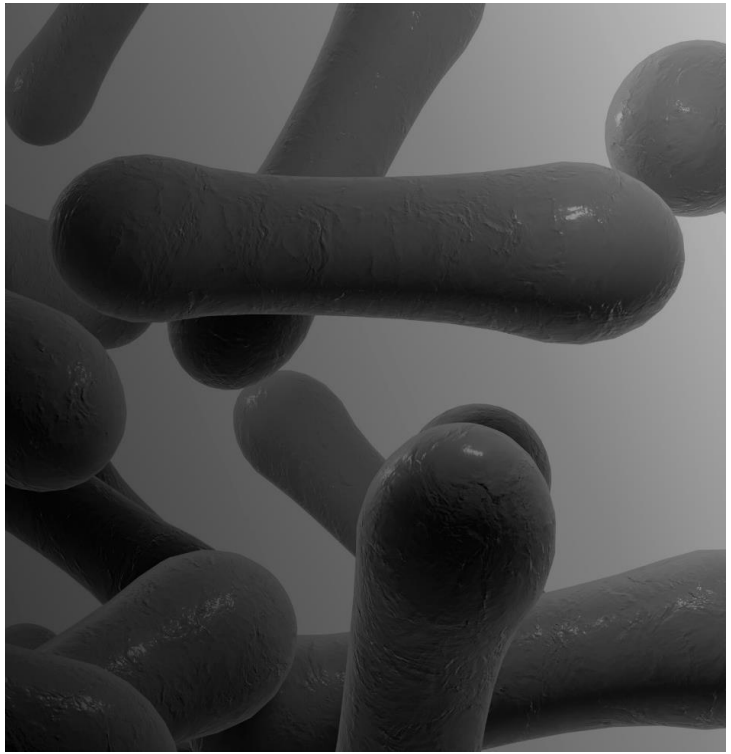
1Q 2019: $30.5M IPO on Nasdaq (ANCN)

2Q 2019: Open label Codex data will begin to become available

3Q 2019: Complete 35 patient enrollment for interim analysis

4Q 2019: Final Codex interim analysis

4Q 2019- 1H2020: Initiation and first patient enrolled in Leo



Key Takeaways

Inodiftagene vixteplasmid is a first-of-its-kind gene therapy in non-muscle invasive bladder cancer (NMIBC)

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

Preliminary data from development program and FDA agreement form foundation for path to approval with either of two trials

Strong balance sheet: $30.5M US IPO (Nasdaq: ANCN) closed in February

Two registrational studies provide independent routes to approval in two separate, but related, indications. The first is open to enrollment

Experienced management team with history of successful drug development and newly expanding global organization