INODIFTAGENE

Gene Therapy for Bladder Cancer

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Investment Highlights

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

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

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

Strong balance sheet and financing plan: $23M private financing closed in June; F-1 filed with SEC 1Q2019

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

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

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 phase 2 clinical trials show complete responses indicating strong anti-tumor activity

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

Inodiftagene Clinical Development Program

Six completed clinical trials in NMIBC, ovarian and pancreatic cancer

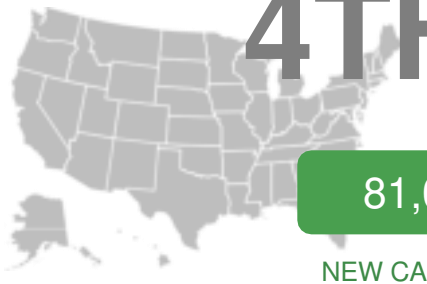
INDICATION	PRODUCT CANDIDATE	TRIAL
NMIBC	Inodiftagene	Phase 1/2
		Phase 2
		Pivotal Codex trial initiated
	Inodiftagene with BCG	Phase 2
		Pivotal Leo Phase 3 planned
Ovarian cancer	Inodiftagene	Phase 2
Pancreatic cancer	Inodiftagene	Phase 1/2
	Inodiftagene with gemcitabine	Phase 2

Responses in all three cancer types in various clinical settings

Focus on NMIBC for pivotal development

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

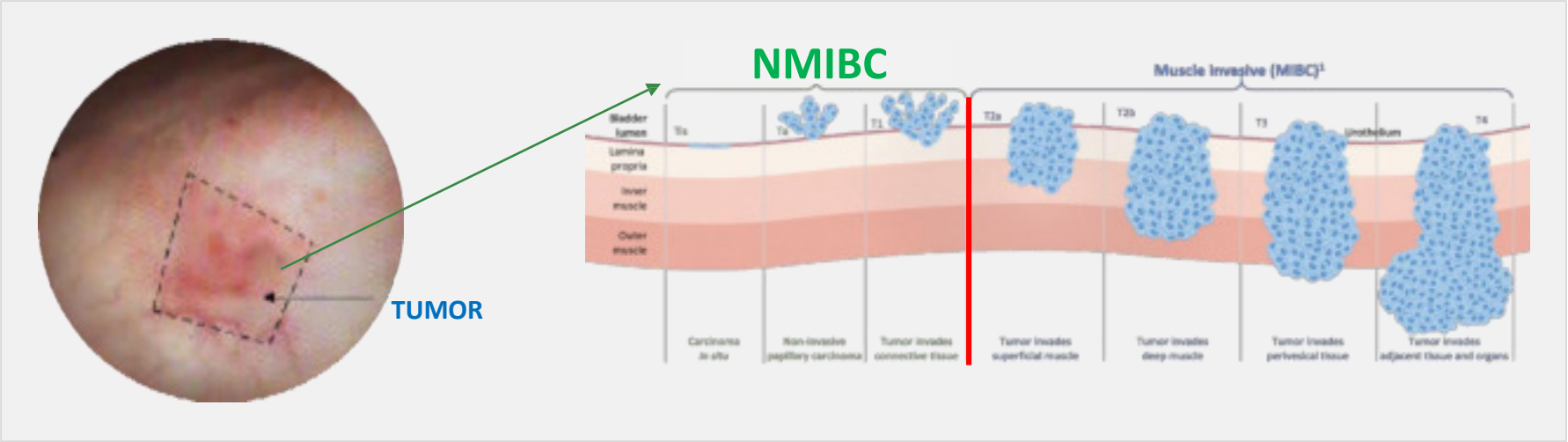
0

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.

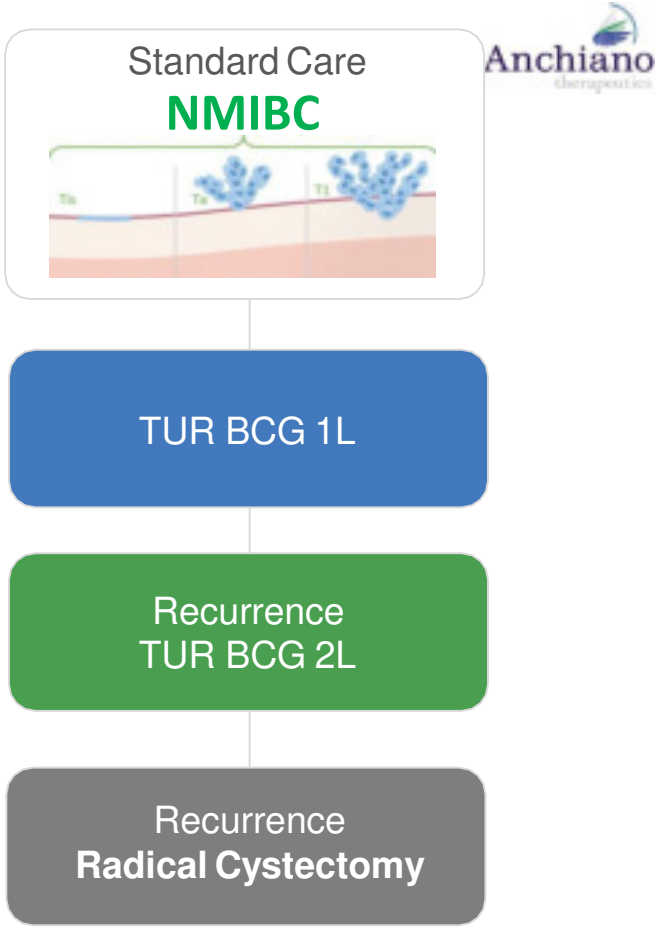
NMIBC Classification and Treatment

Recurrence is a life-changing outcome

Treatment for NMIBC is trans-urethral resection, or TUR (surgery by cystoscope) to remove all papillary tumors, then therapy with BCG administered into the bladder. BCG is live attenuated tuberculosis bacteria

BCG is recommended for initial therapy after TUR, then again after first recurrence. 70% of patients' tumors ultimately fail BCG treatment

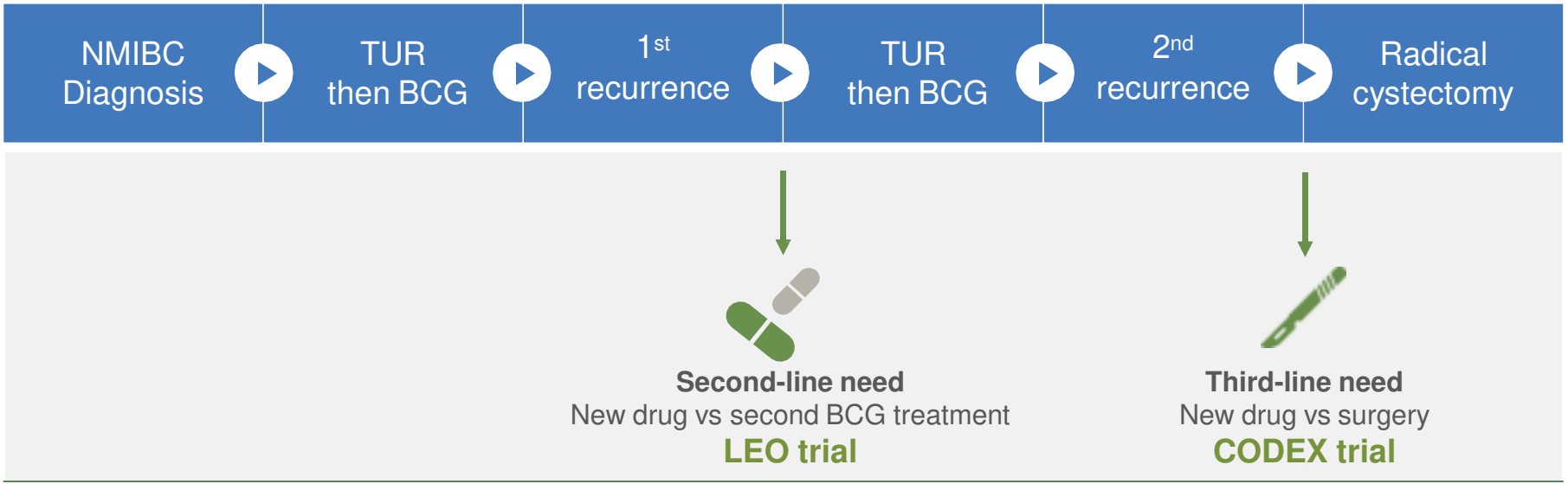
After two courses of failed BCG therapy **radical cystectomy** is recommended, which is a life-changing surgical procedure



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 285,000 NMIBC Patients Are Eligible for Treatment Annually

90,000 constitute NMIBC global market

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

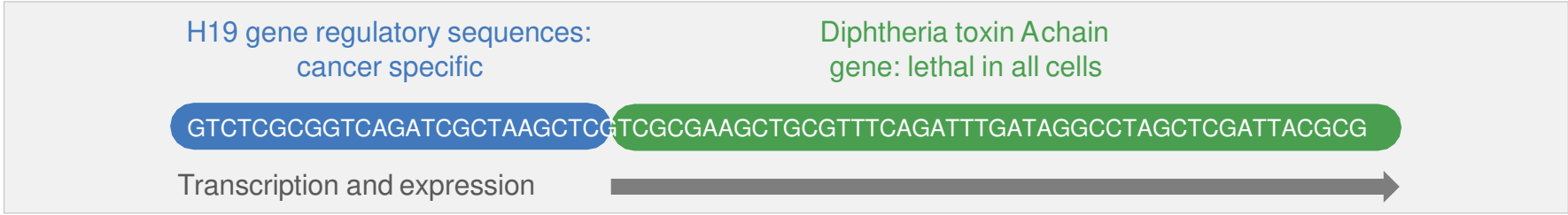
Sources: The Nemetz Group, Decision Resources

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

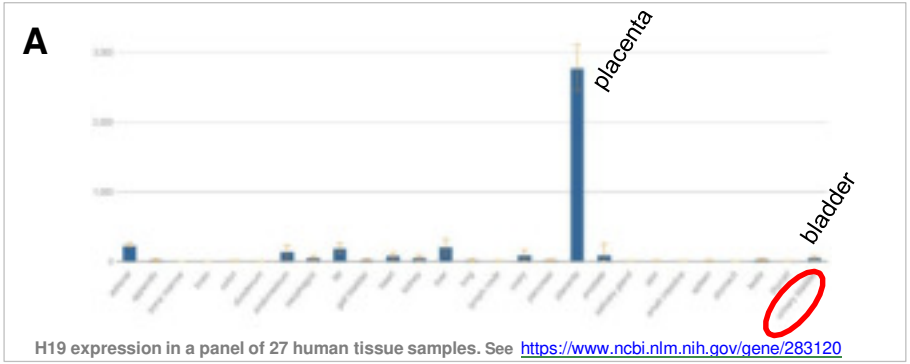
Plasmid facilitates high transfection efficiency. In vitro uptake demonstrable in 85% of cells after a single exposure; in clinic detectable in bladder more than 48 hours after instillation. Engineered to prevent transfer of toxin between cells

Well-understood and validated mechanism-of-action

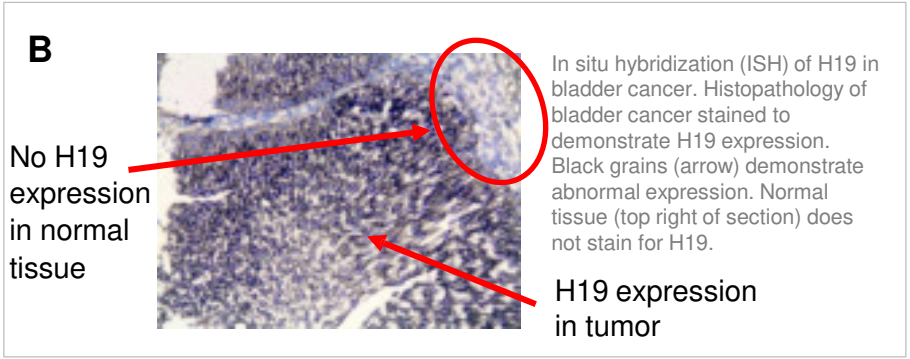
Lethal inhibition of protein synthesis

Uses H19 to Target Cancer Cells Avoiding Normal Cells

Inodiftagene mechanism of action



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



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 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**

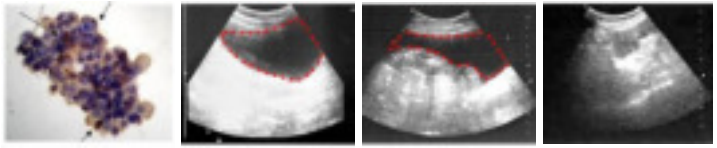
H19 is expressed in all subtypes of NMIBC, including carcinoma in situ (CIS)

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

Responses in Advanced Ovarian and Pancreatic Cancer

Inodiftagene activity in solid tumors validates mechanism of action

Complete resolution of ascites following instillation of inodiftagene



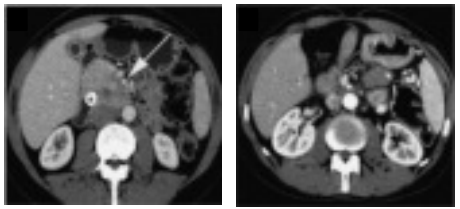
Left to right: H19-positive ovarian cells from ascites; ultrasound of abdomen at baseline, prior to 5th treatment, and after 10th treatment. Red border demarcates ascites, resolved at right

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	603	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 response rate in marker
Phase 2 Monotherapy	Complete; N = 47	33% complete responses; 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

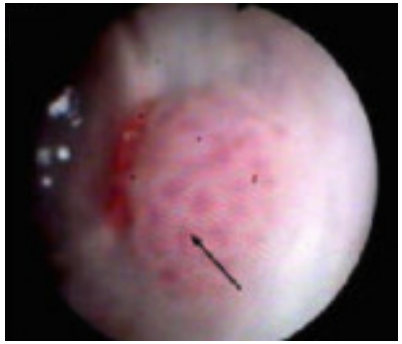
Inodiftagene consistently showed clinically meaningful anti-cancer activity

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

Proof of concept requires ability to destroy macroscopic tumor. Patients in two phase 2 trials underwent complete resection of existing papillary lesions except a single marker tumor, assessed at 12 weeks: 30% had CRs

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

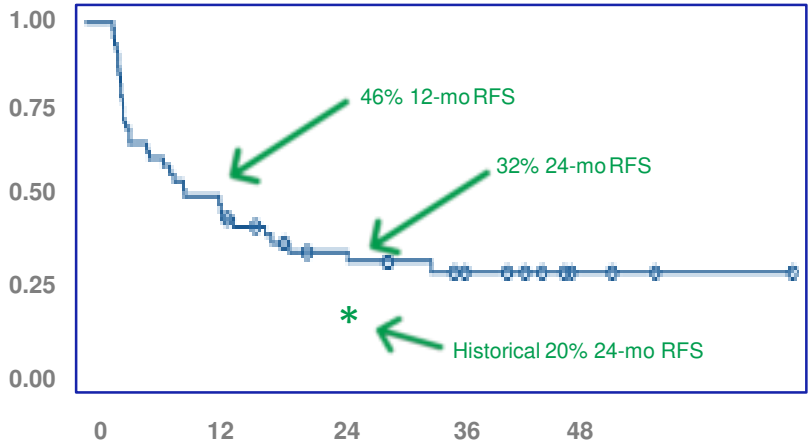
Inodiftagene phase 2 monotherapy results

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

3-Month RFS Favorable Results Demonstrated in Phase 2 Combination Trial

Inodiftagene phase 2 combination results

MONTH	RECURRENCE-FREE SURVIVAL	PROGRESSION-FREE SURVIVAL
3	94.6	100
6	78.4	94.6
9	73.0	89.2
12	67.6	89.2
18	62.2	83.8
Overall (24 month)	54.1	75.7
Median	Not Reached	Not Reached

Inodiftagene plus BCG **3-month RFS 95%**

Historical 3-month RFS for BCG therapy alone ranges from **50.7% to 85% in CIS** and **57%** in papillary disease[1-3]

The combination trial did not test maintenance, only induction

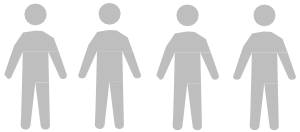
3-month RFS may be best indication of inodiftagene effect on BCG-treated patients

1. Lamm et al., J Urol. 163: 1124, 2000; 2. Herr and Dalbagni, J. Urol. 169:1706, 2003; 3. DiLorezno et al., Cancer 2010, April 15, p. 1893

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

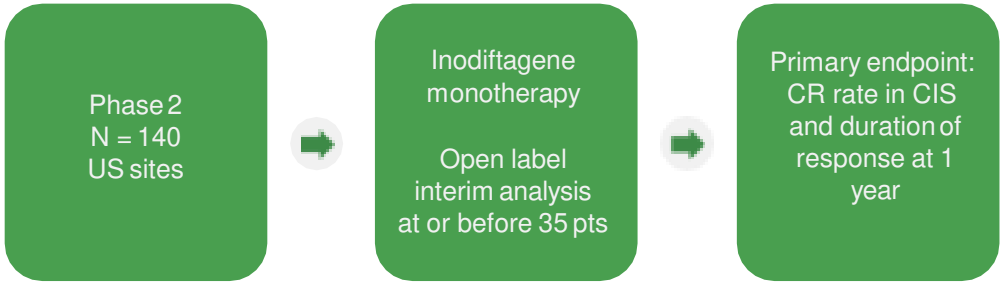
OPEN TO ENROLLMENT
Actively recruiting

INTENSIFIED SCHEDULE
10 week induction then every 3 weeks replaces every 3 months in prior trials

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



Leo Study (301 Trial): Second Registrational Trial Design

Inodiftagene phase 3 trial in second-line patients

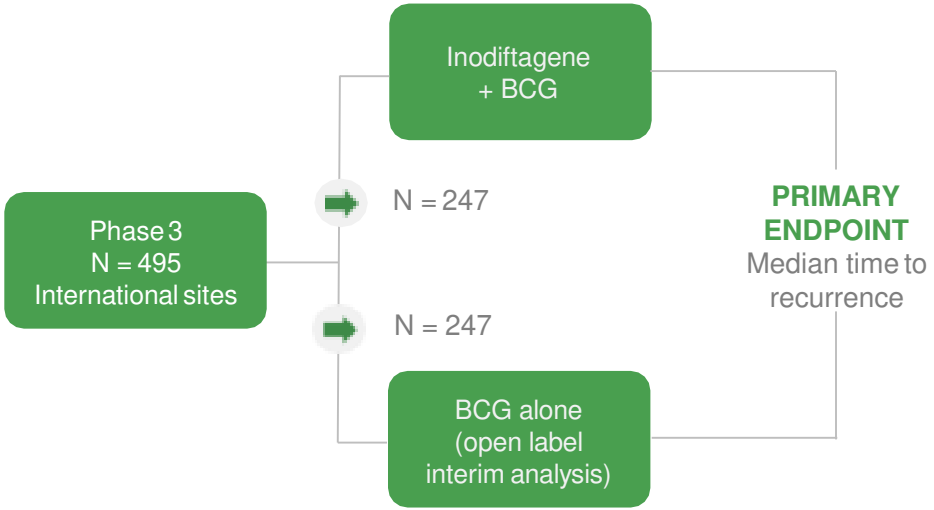
RANDOMIZED TRIAL
For approval

INTENSIFIED SCHEDULE
10 week induction then every 3 weeks as in Codex trial

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

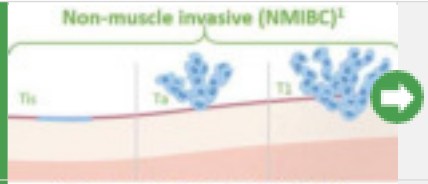
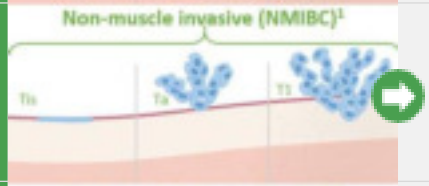
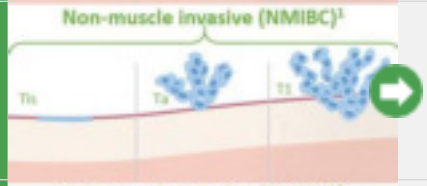
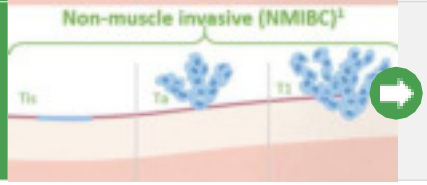
Second-line patients: intermediate or high risk NMIBC after one failed course of BCG
N = approximately 495 patients



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

Differentiating Strategy for Inodiftagene Approval in Two Indications

Inodiftagene clinical development strategy

Standard of care		**TUR BCG 1L**	**Recurrence TUR, BCG 2L**	**Recurrence Cystectomy**
Competition		TUR BCG	Recurrence TUR, BCG	Recurrence Competitor 3L 1 yr RR 15-35%
Codex		TUR BCG	Recurrence TUR, BCG	Recurrence **Inodiftagene 3L**
Leo		TUR BCG	Recurrence TUR, BCG **Inodiftagene 2L**	Recurrence Cystectomy

Large Potential Market of up to $1.8 Billion

Over 90,000 potential inodiftagene patients with NMIBC in major markets

285,000
NMIBC Patients Eligible For Drug Treatment, **US, EU and Japan**

90,000
Patients Eligible For Inodiftagene Treatment, US, EU and Japan

Approximately $1B
Projected Year-5 US, EU and Japan Sales for both indications

Nearly $1.8 Billion
Projected Peak US, EU and Japan Sales

260,000 new cases of bladder cancer in 2017 in US, EU, and Japan

195,000 of those patients present with NMIBC, **90,000** patients recur with NMIBC annually

Thus **285,000** incident and recurrent NMIBC are eligible for drug treatment

~**90,000** of all drug treatable patients either failed or unresponsive are eligible for inodiftagene therapy

Company-estimated market penetration at year 5:

BCG failure 2L Leo population: **20-24%**

BCG unresponsive 3L Codex population: **20–24%**

Assumes cost per patient per year of ~**$80,000**

Source: Nemetz Group Revenue Forecast and Market Research

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

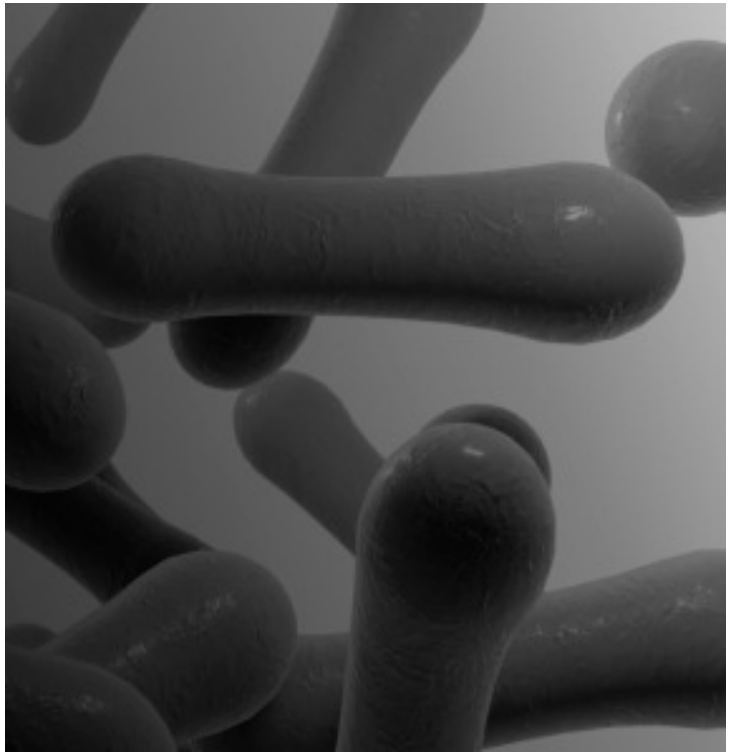
2Q 2018: Completed a $23M private financing round. Will fund phase 2 registrational study through early open-label data

4Q 2018: Codex trial initiated for registration of inodiftagene

1Q 2019: Publicly filed F-1 with US SEC

2Q 2019: Open label data will become available

3Q 2019: Complete 35 patient enrollment for interim analysis



Key Takeaways

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

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