INODIFTAGENE

Recombinant DNA Gene Therapy for Bladder Cancer

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Inodiftagene for Non-Muscle Invasive Bladder Cancer

INODIFTAGENE

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

Initiating 2 pivotal trials, each of which could lead to approval

Data from phase 2 clinical trials show complete responses indicating strong efficacy

Non-Muscle Invasive Bladder Cancer (NMIBC)

A large and underserved population

$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

Three Completed NMIBC Trials Support Pivotal Study Designs

Inodiftagene clinical strategy

CLINICAL PROGRAM

Trial	Status	Result
Phase 1/2 Monotherapy	Complete; N = 18	Favorable safety, no DLT, no MTD; 22% complete response rate in marker lesions
Phase 2 Monotherapy	Complete; N = 47	33% complete responses in marker lesions; 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

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 2018

5TH MOST COMMON CANCER OVERALL IN EU

EU and Worldwide[2]

141,000
EU NEW CASES IN 2020

403,000
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

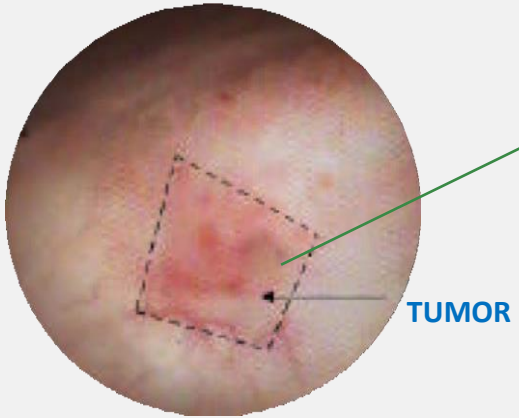
0

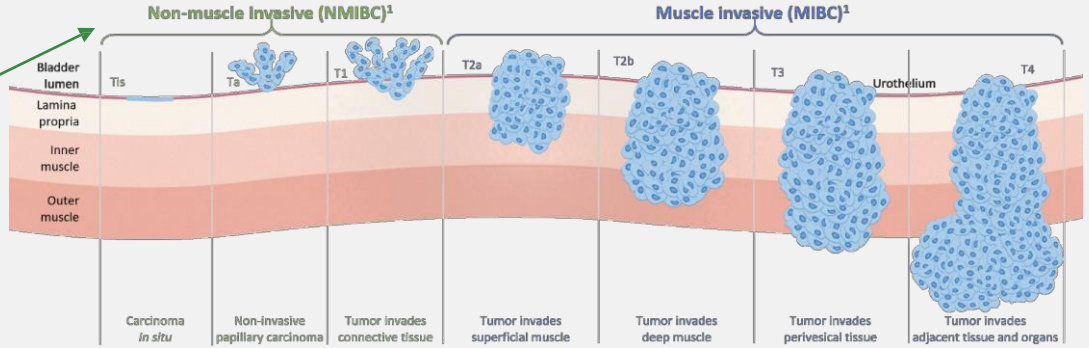
Drugs approved by FDA since 1998 for NMIBC

1. ACS Cancer Facts and Figures 2018, www.cancer.org
2. https://ec.europa.eu/jrc/en/publication/epidemiology-bladder-cancer-europe

NMIBC Classification and Treatment

Recurrence leads to progression and metastasis



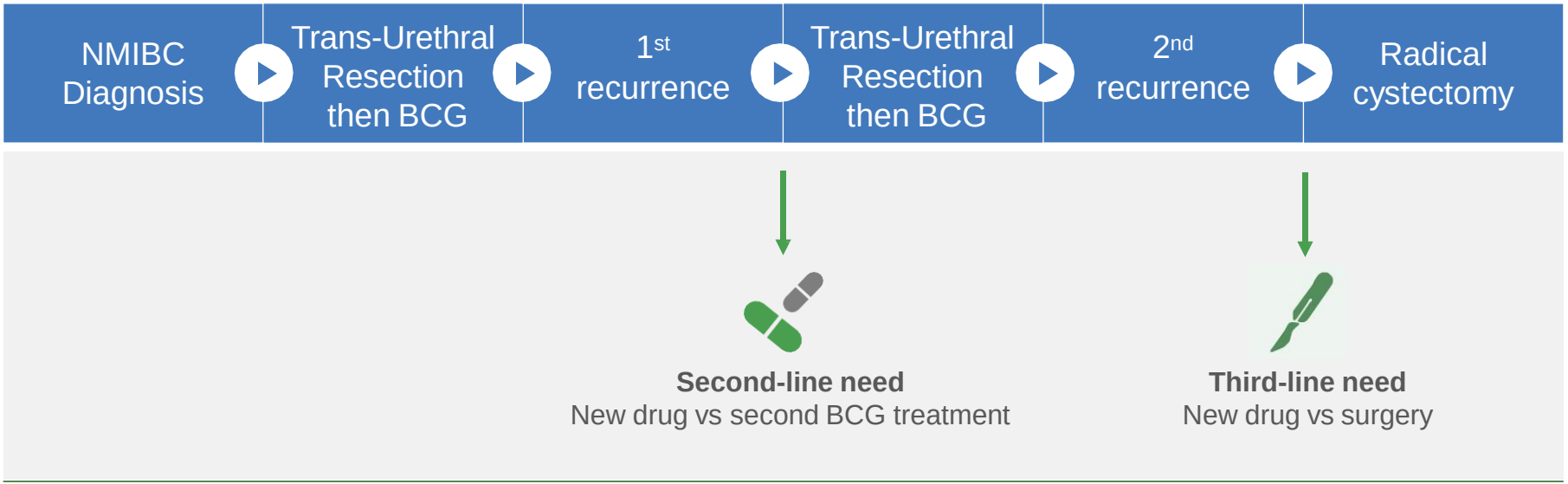


DIAGNOSIS	LOCALIZATION	THERAPY
Patients are diagnosed and evaluated via cystoscope	Tumors are identified on the inner surface of the bladder, resected and classified by depth	NMIBC patients initially receive Bacillus Calmette Guerin (BCG) and are the focus of inodiftagene therapy

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

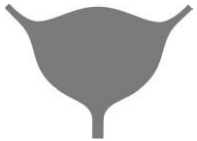


Patients whose tumors recur after BCG therapy are those who need inodiftagene:

the goal is to prevent or delay recurrence and cystectomy

Over 270,000 NMIBC Patients Are Eligible for Treatment Annually

NMIBC market



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

~75%
Proportion of bladder cancer that is NMIBC. **187,000** cases

~70%
Proportion of NMIBC patients who suffer recurrence after BCG treatment. **85,000**

272,000
Total number of incident and recurrent NMIBC cases who are eligible for treatment annually

Of these **60,000 recurrent NMIBC cases** after BCG treatment are eligible for inodiftagene treatment as second- or third-line therapy

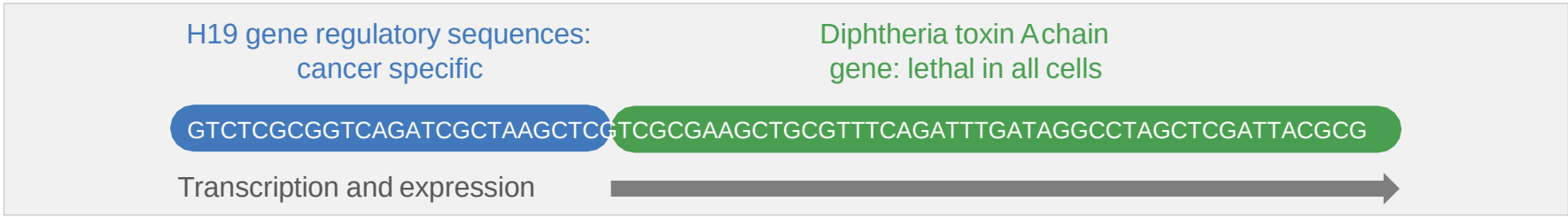
Sources: Decision Resources

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

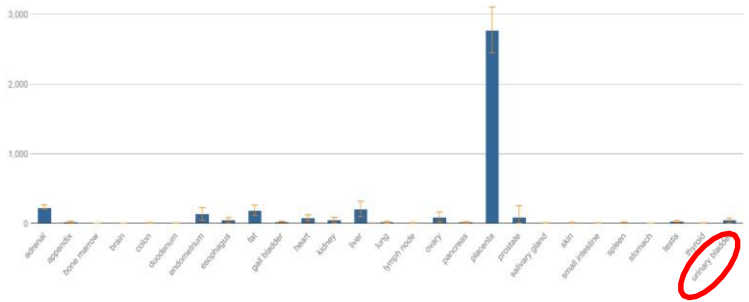
Plasmid facilitates high transfection efficiency. In vitro uptake in 85% of cells after a single exposure; in clinic detectable in bladder more than 48 hours after instillation. Engineered to prevent transfer of toxin between cells

Well-understood mechanism-of-action

Uses H19 to Target Cancer Cells Avoiding Normal Cells

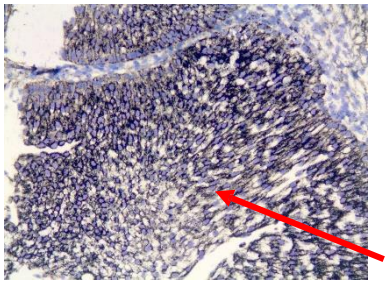
Inodiftagene mechanism of action

A



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

B



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

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**

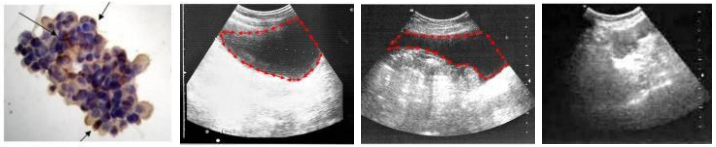
H19 is expressed in all subtypes of NMIBC, including carcinoma in situ (CIS)

In our phase 2 study of inodiftagene, 47 patients were tested for H19 upon entry into the trial, and **all 47 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



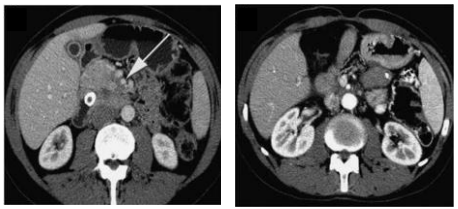
Left to right: H19-positive ovarian cells from ascites; ultrasound of abdomen at baseline, prior to 5th treatment, and after 10th treatment. Red border demarcates ascites, resolved at right

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	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 Studies Support Approvability in >$1B Population

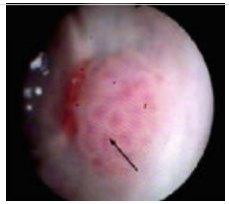
Inodiftagene Clinical Data in Bladder Cancer

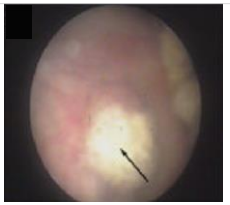
Inodiftagene results in 33% complete responses in marker papillary tumors, 86% CRs in CIS alone and with BCG

Monotherapy durability surpasses historical and competitor experience:

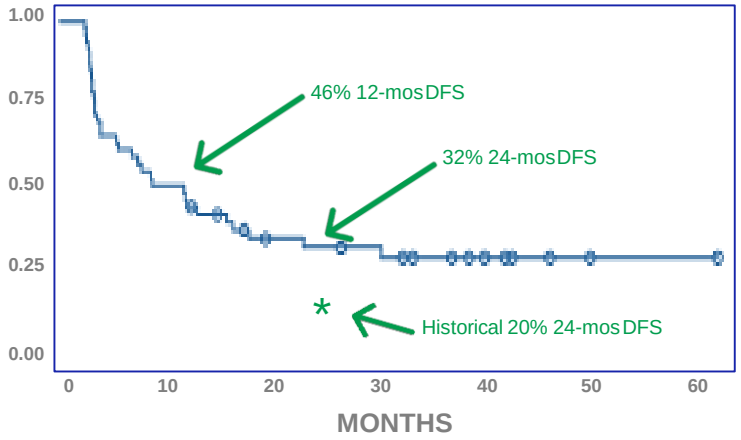
- FDA specified in CIS 30% recurrence-free rate at 18-24 months, excluding 20%, as being an approvable endpoint enpoint[1]
- Phase 2 study demonstrates 18- and 24- month rates are >30% (right)
- 46% 12-month rate compares to competitors' rates of 35% and 15% at 12 months

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





L: Baseline: papillary tumor
R: 3 weeks following 6th instillation of inodiftagene: necrosis

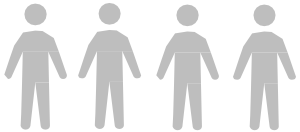


1. Jarow et al., J. Urology, 2014

Pathway to Registration in Two Discrete Indications

Inodiftagene registrational program

Codex

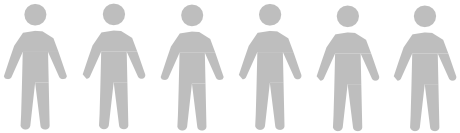


Codex phase 2 pivotal study
trial is a single-arm path with FDA concurrence to full approval in third line patients

Monotherapy, 140 patients, single arm

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

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 approval in two separate (but related) indications

Codex Study (204 Trial): Initial Registrational Trial Design

Inodiftagene phase 2 trial in third-line patients

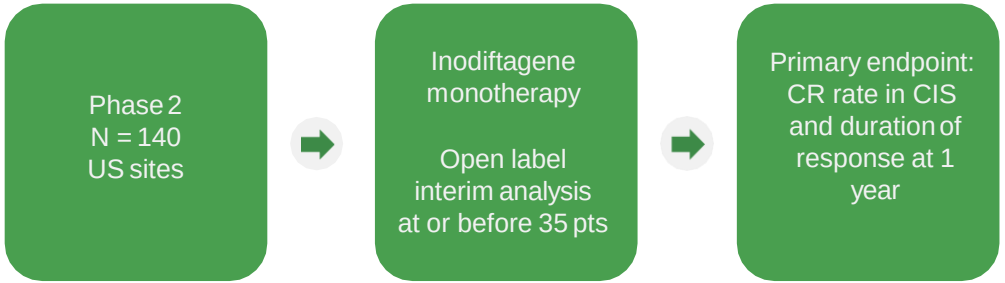
SINGLE ARM TRIAL
For approval

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 supportive

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

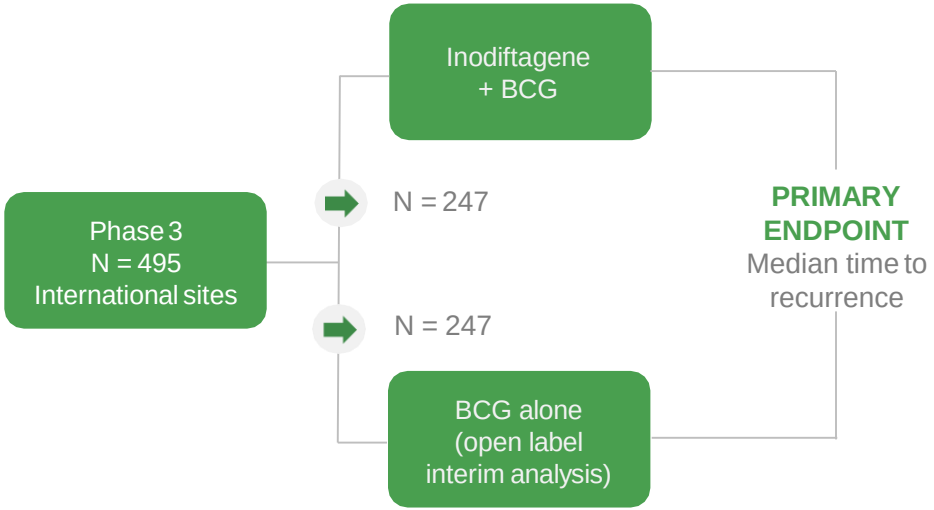
RANDOMIZED TRIAL
For approval

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

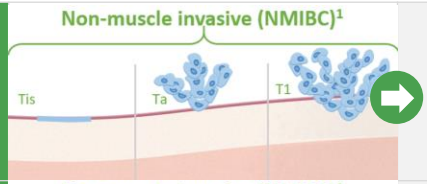
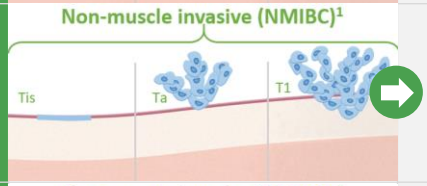
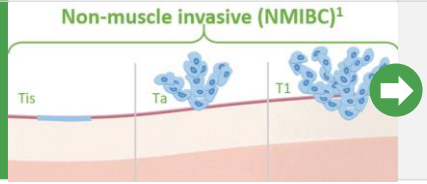
SPANISH, GERMAN, CANADIAN, UK AND FRENCH REGULATORS
support study as well

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



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 1 yr RR 46%**
Leo		TUR BCG	Recurrence TUR, BCG **Inodiftagene 2L**	Recurrence Cystectomy

Development plan in second-line patients, the Leo patient population, is unique at this time, and addresses the majority of the market potential of NMIBC therapy

Large Potential Market of $1.5 Billion

Over 60,000 potential inodiftagene patients in NMIBC

272,000
NMIBC Patients Eligible For Drug Treatment, **US, EU and Japan**

60,000
Patients Eligible For Inodiftagene 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

187,000 of those patients present with NMIBC, **85,000** patients recur with NMIBC annually

Thus **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 Inodiftagene therapy

Company-estimated market penetration at year 5:

BCG failure 2L **20-24%**

BCG unresponsive 3L **20–24%**

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

Experienced Management Team

US-based clinical development team

	**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

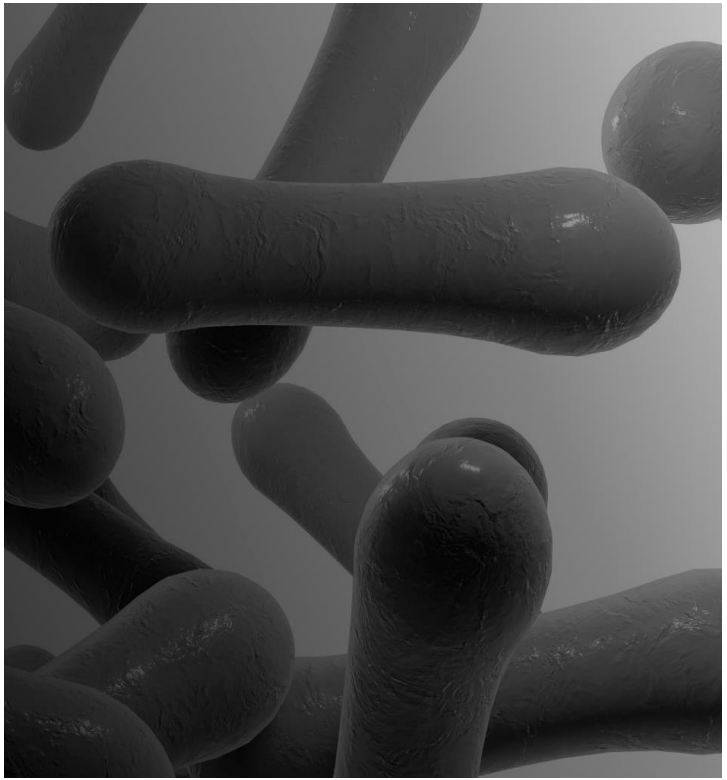
Q2 2018: Completed a $23M private financing round. Will fund phase 2 registrational study through early open-label data

Q4 2018: Enroll first patients in the single arm Codex study

H1 2019: Open label data become available

Q2 2019: Complete 35 patient enrollment for interim analysis

H2 2019: Leo study begins enrolling pending funding



Key Takeaways

Anchiano therapeutics

- Potential for first-of-its-kind DNA-directed cancer therapy in non-muscle invasive bladder cancer (NMIBC), a serious area of unmet need—**inodiftagene vixteplasmid**
- Over $1.5 billion commercial potential serving large global population in need of new therapy and uniquely addressing second line treatment
- Two registrational studies, providing independent routes to approval in two separate, but related, indications
- Private financing of $23M completed; robust balance sheet going forward
- Preliminary data from development program and FDA agreement support direct path to approval with either of two trials
- Strong, experienced management team and newly expanding global organization