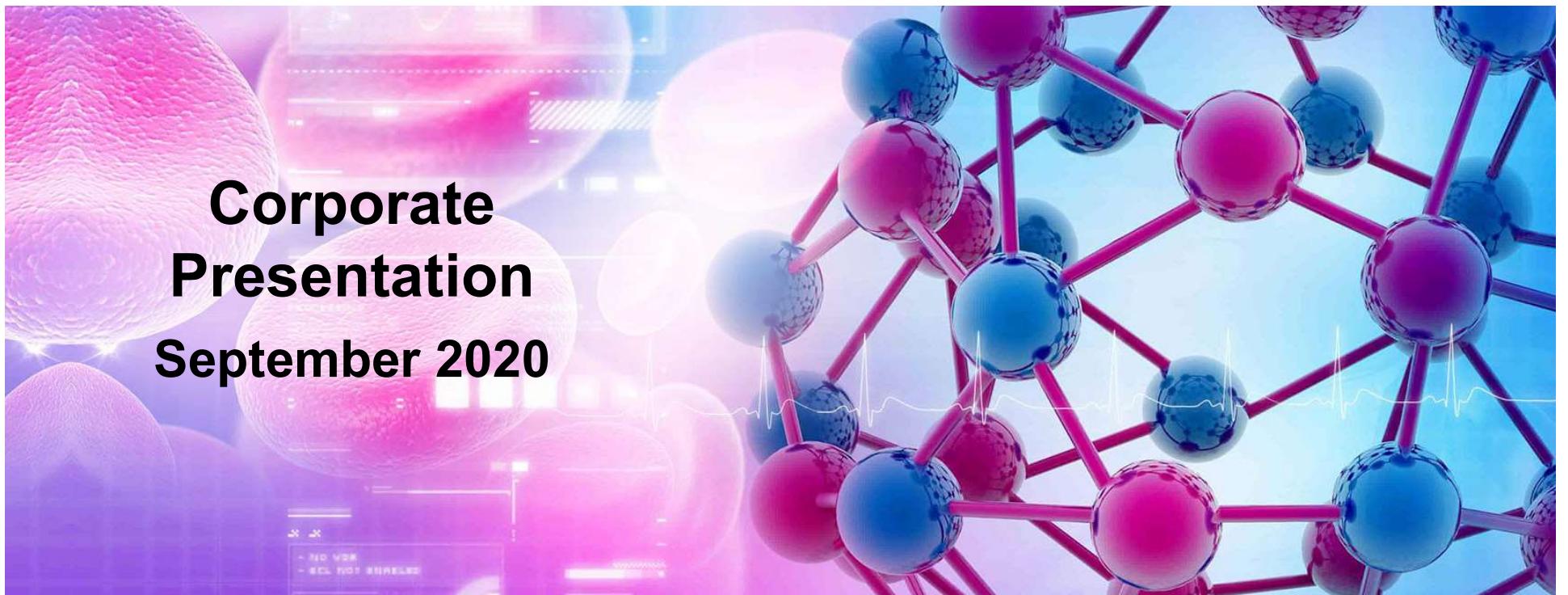




**Corporate
Presentation
September 2020**



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Overview

- We develop transformational therapeutics targeting serious diseases with significant unmet medical needs
- Our lead drug candidate, CpG-STAT3siRNA (“CO-sTiRNA™”), is a Phase 1-ready immuno-oncology gene therapy for the treatment of multiple cancers
- Phase 1 clinical trial for B-cell non-Hodgkin’s lymphoma (“NHL”) is slated to begin in Q1 2021
- CO-sTiRNA is a ground-breaking, first-of-its-kind, dual-action STAT3 inhibitor with game-changing potential in the treatment of cancer
- STAT3 is a gene long-known to play a fundamental role in cancer
- Effective treatments targeting STAT3 have been “elusive” to date, leading STAT3 to be described as “undruggable”
- CO-sTiRNA is a highly selective and targeted immuno-oncology gene therapy designed to simultaneously:
 - “silence” STAT3 activity through RNA interference; and
 - stimulate TLR9 receptors to activate the body’s immune system to recognize and kill cancer cells
- Pre-clinical results have demonstrated reductions in cancer growth and metastasis

Overview (Cont.)

- Pre-clinical testing on-going for cutaneous T-cell lymphoma and other solid tumors
- Additional Phase 1 clinical trials for B-cell NHL and other forms of cancer in combination with immune checkpoint inhibitors and/or CAR-Ts are planned for 2021 and H1 2022
- We have an exclusive worldwide license for CO-sTiRNA from City of Hope National Medical Center (“COH”), a world-renowned research and cancer center near Los Angeles
- We are also collaborating in continuing research and development of CO-sTiRNA with additional IP being generated pursuant to Scopus-sponsored research agreement with COH
- Our approach is to capitalize on groundbreaking research at leading medical, scientific and academic institutions. We have additional exclusive licenses with the National Institutes of Health (“NIH”) and The Hebrew University of Jerusalem (“Hebrew University”)
- Our second lead drug candidate, MRI-1867, is licensed on an exclusive worldwide basis from NIH
 - Initial indication for MRI-1867 is systemic sclerosis, a fibrotic disease without FDA-approved treatment
 - Recent developments in biopharma industry underscore value of MRI-1867

Highlights

- Potential game-changing treatment making STAT3 “druggable” for the first time
- Highly-differentiated scientific approach for treating cancer encompassing both immunotherapy and gene therapy
- Q1 2021 initiation of Phase 1 clinical trial for initial CO-sTiRNA indication
 - Orphan Drug Designation and/or other accelerated FDA pathways possible
- Targeted cancer indications represent multi-billion-dollar market opportunities
- Strong strategic partnerships
 - Close collaboration and working relationships with leading experts on STAT3 inhibition
- Robust IP protections create high barriers to entry
- Moderate post-IPO capital requirements for implementation of near-term business strategy
- Premium valuations and strong investor demand for promising immuno-oncology and gene therapy companies, with CO-sTiRNA representing a unique value proposition
- Near-term milestones provide multiple opportunities to drive value and attract increasingly larger institutional investors
- Highly-accomplished leadership team with exceptional scientific, medical and business experience

Transformational Therapeutics

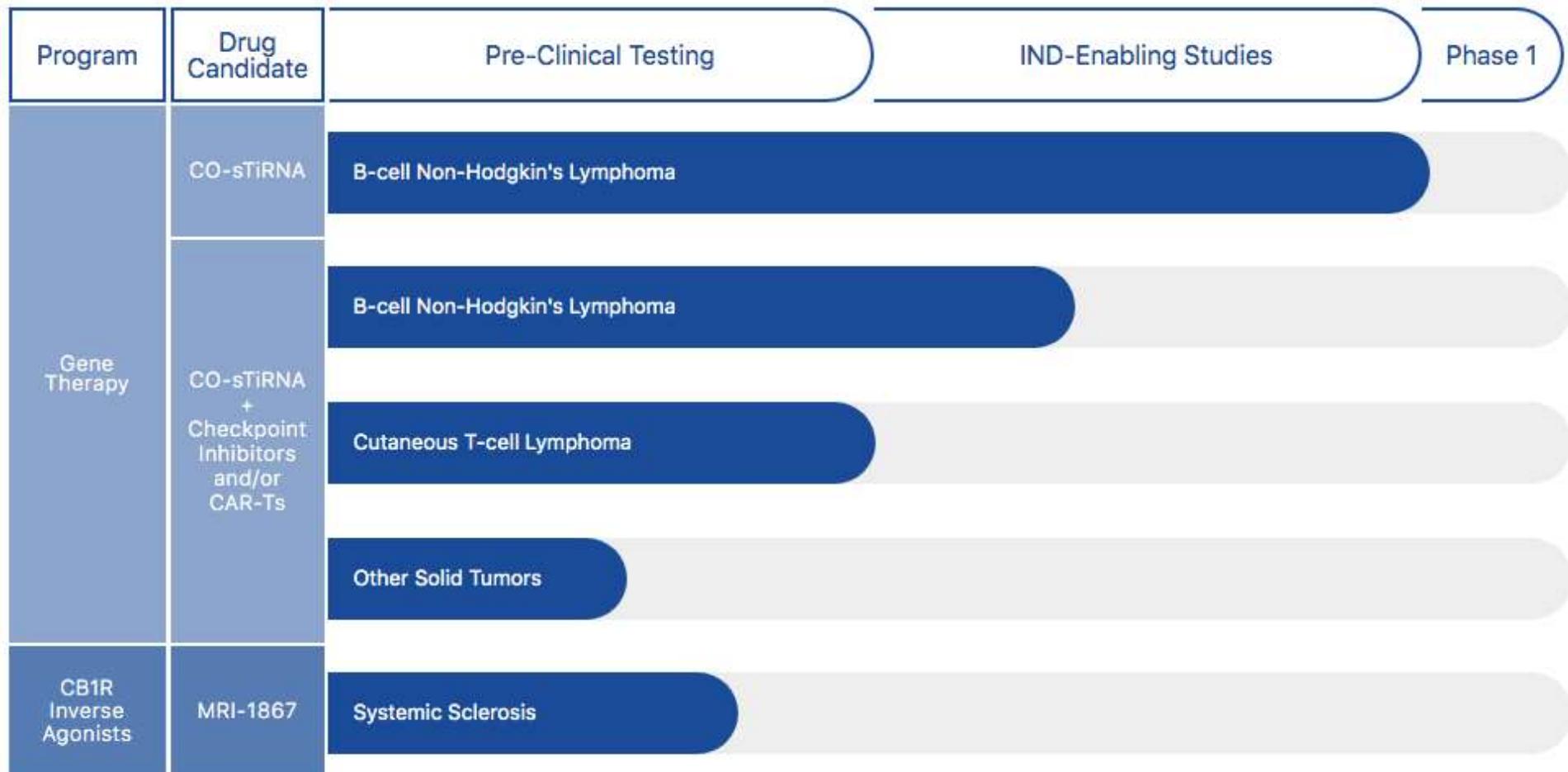
CO-sTiRNA

- Our lead gene-silencing cancer immunotherapy candidate
- A novel, first-in-class drug targeting an undruggable cancer gene, STAT3, which causes tumors to grow and protects them from detection by the immune system
- A new and revolutionary immuno-oncology treatment with a unique dual-mechanism of action
- Highly specific targeting only tumor cells and tumor-associated immune cells

MRI-1867

- Proprietary, dual-action, peripherally-restricted CB1 receptor inverse agonist and iNOS inhibitor
- Over activation of CB1 and iNOS has been implicated in the pathophysiology of systemic sclerosis, including fibrosis of the skin, lung, kidney and heart
- Published in vivo studies conducted by the NIH demonstrated that MRI-1867 successfully prevented and treated fibrosis in lungs and liver

Pipeline



Value Building Milestones

2020 and 2021

Q4 2020

- File IND for CO-sTiRNA in B-cell NHL
- Complete clinical lot manufacturing
- Generate initial pre-clinical results for CO-sTiRNA + checkpoint inhibitors

H1 2021

- Begin Phase 1 clinical trial for CO-sTiRNA in B-cell NHL
- Generate initial pre-clinical results in cutaneous T-cell lymphoma and melanoma
- Pre-IND meeting with FDA for CO-sTiRNA + checkpoint inhibitor

H2 2021

- Complete IND-enabling studies for CO-sTiRNA + checkpoint inhibitor
- File IND for CO-sTiRNA + checkpoint inhibitor
- Begin Phase 1 clinical trial of CO-sTiRNA + checkpoint inhibitor

Intellectual Property Portfolio

- Broad and growing portfolio of novel and proprietary compounds is covered by a broad patent portfolio
- Ongoing R&D with strategic partners allows for further IP creation
- We intend to file for Orphan Drug designation for some of our drug candidates which, if granted, provides for 7 years market exclusivity from the date of approval

CO-sTiRNA

- 5 issued and 2 pending covering “Methods and Compositions for the Treatment of Cancer of Other Diseases”

MRI-1867

- 2 issued and 6 filed covering “Cannabinoid Receptor Mediating Compounds”
- 1 issued and 8 filed covering Pyrazole Derivatives and Their Use as Cannabinoid Receptor Mediators

Our Strategic Partners

- Outstanding scientific and medical provenance with research and development based on pioneering discoveries at some of the world's foremost research and academic institutions
- Technologies licensed from City of Hope National Medical Center, National Institutes of Health and The Hebrew University of Jerusalem
 - CO-sTiRNA licensed from City of Hope
 - MRI-1867 licensed from the NIH





Experienced Biopharma Leadership

Paul E. Hopper
Co-Chairman and Director



Ashish P. Sanghrajka
President and Director



Aharon Schwartz, Ph.D.
SAB Chairman
Executive Chairman - Scopus Israel



Morris C. Lesser, M.D.
SAB Member; Director and
Senior Medical Advisor - Scopus Israel



Raphael (Rafi) Hofstein, Ph.D.
Director



Lesley Russell, MB.Ch.B., MRCP
Director



Hua E. Yu, Ph.D.
SAB Member



Marcin Kortylewski, Ph.D.
SAB Member





Experienced Finance and Capital Markets Leadership

Joshua R. Lamstein
Co-Chairman and Director



LEHMAN BROTHERS

Robert J. Gibson, CFA
Vice Chairman and Director



BALANCE POINT CAPITAL



David S. Battleman, M.D.
Director



David A. Buckel, CMA
Director



SharpSpring



Ira Scott Greenspan
Director



BLANKROME

SIDLEY

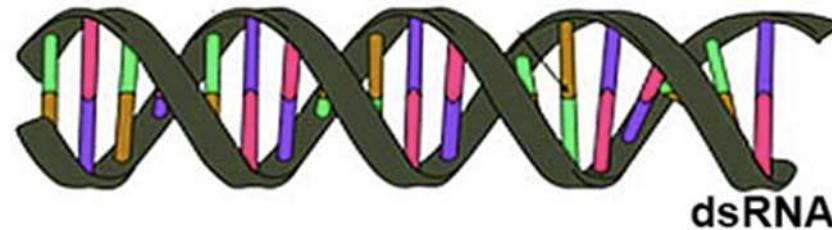
David Weild IV
Director

WEILD&CO.



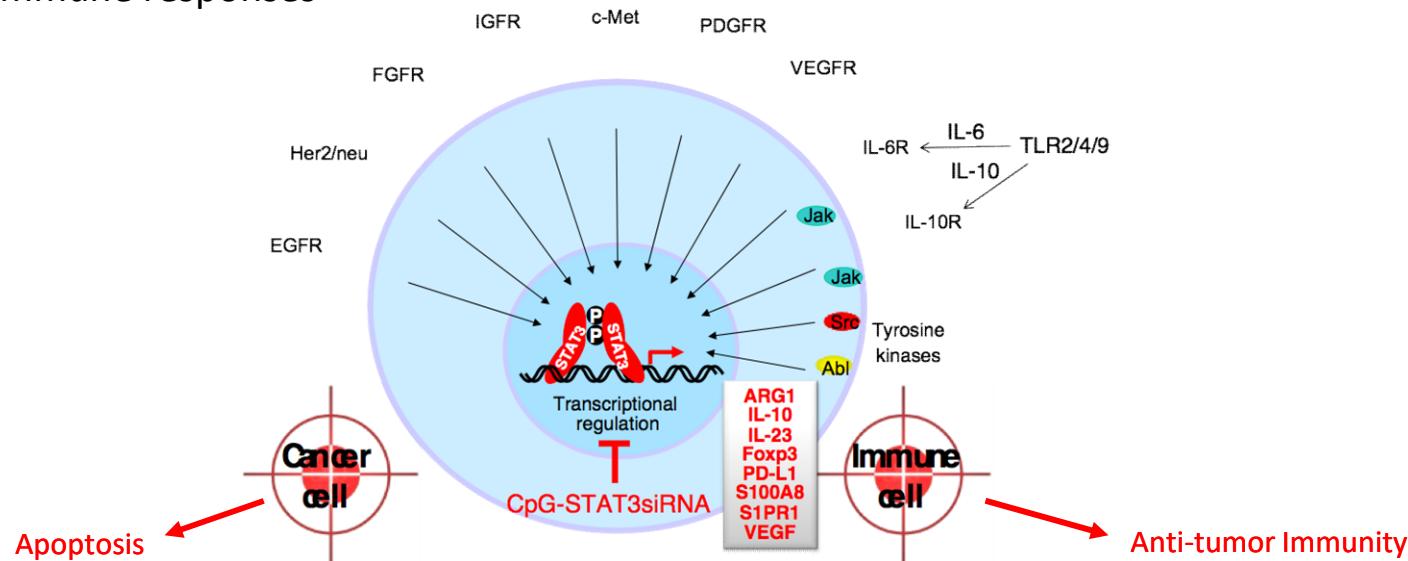
CO-sTiRNA is a Drug That Switches Off the STAT3 Gene

- CO-sTiRNA acts by silencing STAT3 based on the gene therapy called RNA interference (RNAi)
- CO-sTiRNA is a targeted therapy directly to the cells of interest: the cancer cells and the associated immune cells
- CO-sTiRNA results in potent antitumor immunity



Why Targeting STAT3 is So Important for Cancer Therapy

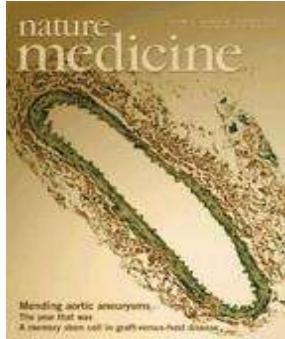
- STAT3 is a critical convergence point for many cancer-causing pathways, promoting cancer cell survival, proliferation and invasion by regulating several genes involved in cell growth and division
- When STAT3 proteins are activated by various extracellular signals, they move into the cell nucleus and bind to specific areas of DNA to regulate expression of various cancer promoting genes
- STAT3 suppresses detection and elimination of cancer cells by the immune system by inducing production of multiple immunosuppressive proteins and blocking those that are helping antitumor immune responses



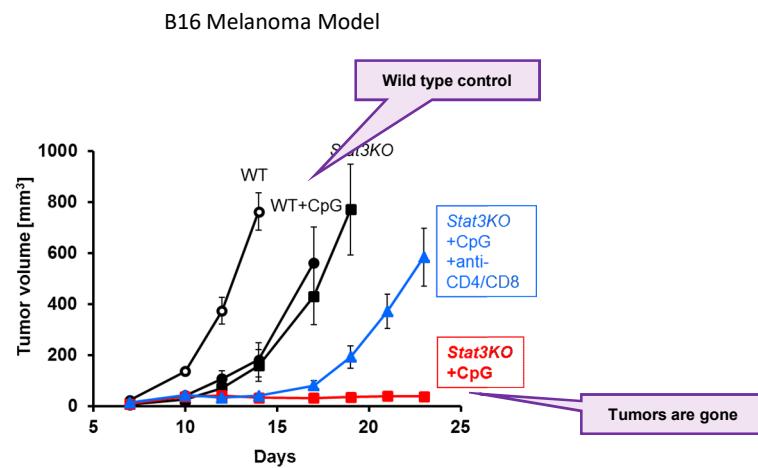
Blocking STAT3 in the Presence of CpG Induces Potent Antitumor Immune Responses

Proof-of-concept study in genetic model of STAT3 deletion (Mx-Cre/STAT3^{flox}):

STAT3 was removed from immune cells such as myeloid cells (DC, TAM, MDSC) and B cells but not from T cells



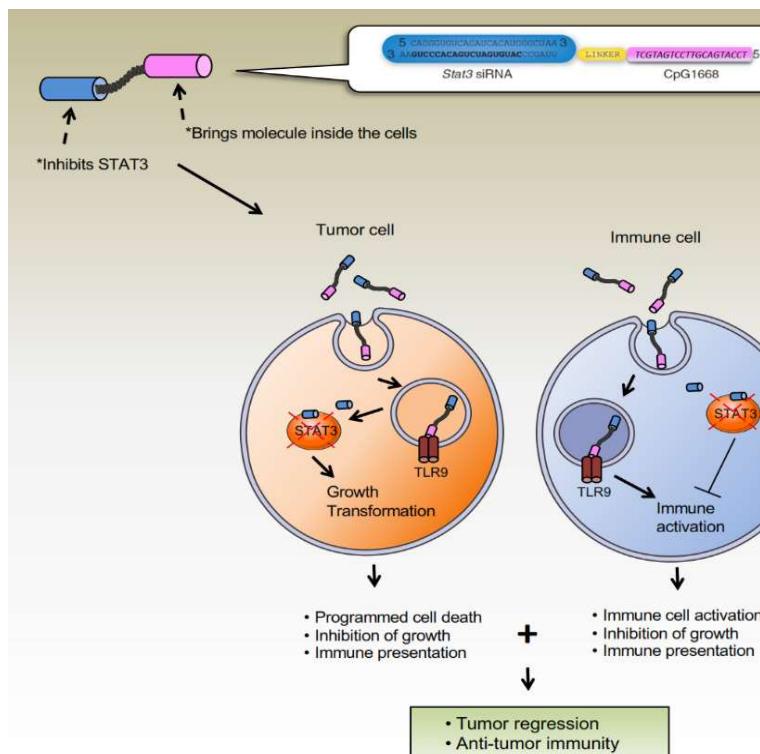
Kortylewski M, et al.
Nature Med, 2005;
Cancer Res, 2009



- Black lines show CpG and STAT3 alone do not inhibit tumor growth effectively
- Blue line shows that antitumor effects induced by taking away STAT3 and stimulating with CpG molecule are dependent on T cell activities
- The **antitumor effects require T cells** since elimination of these immune cells using anti-CD4 and CD8 antibodies compromises the antitumor effects
- Red line shows **complete tumor regression** when STAT3 is taken away and CpG is added.
- Mice can be rechallenged with B16 cancer cells but protective and long-term immunity

CO-sTiRNA: A Cancer Therapeutic Drug Candidate with Dual Activity

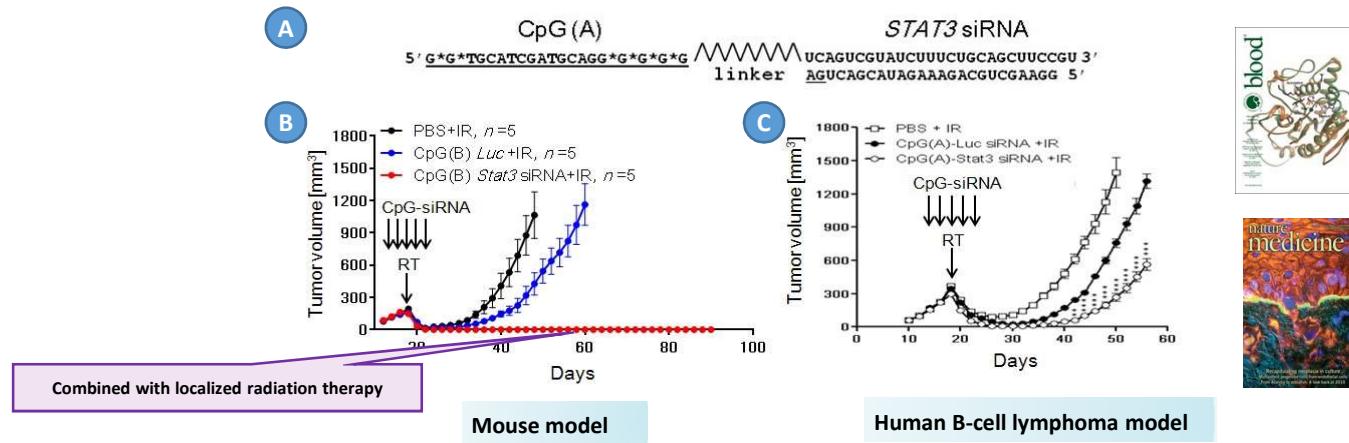
- Dual activity blocks STAT3 in cancer cells and in tumor-associated immune cells while inducing antitumor immune responses



- ✓ Signal Transducer and Activator of Transcription 3 (STAT3) is a highly desirable target for cancer therapy. Importantly, STAT3 inhibition does not effect viability of normal cells.
- ✓ CO-sTiRNA strategy is a gene therapy, which relies on STAT3 silencing through RNA interference (RNAi) and results in potent antitumor immunity.
- ✓ CO-sTiRNA targets cancer cells and tumor-associated immune cells without interfering with memory T cells, thus resulting in a protective, long-term antitumor immunity.
- ✓ CO-sTiRNA conjugates restore T cell activity by disrupting immunosuppressive effects of the tumor microenvironment, and not by direct effect on T cells thus it is safer and less likely to result in autoimmune disorders.

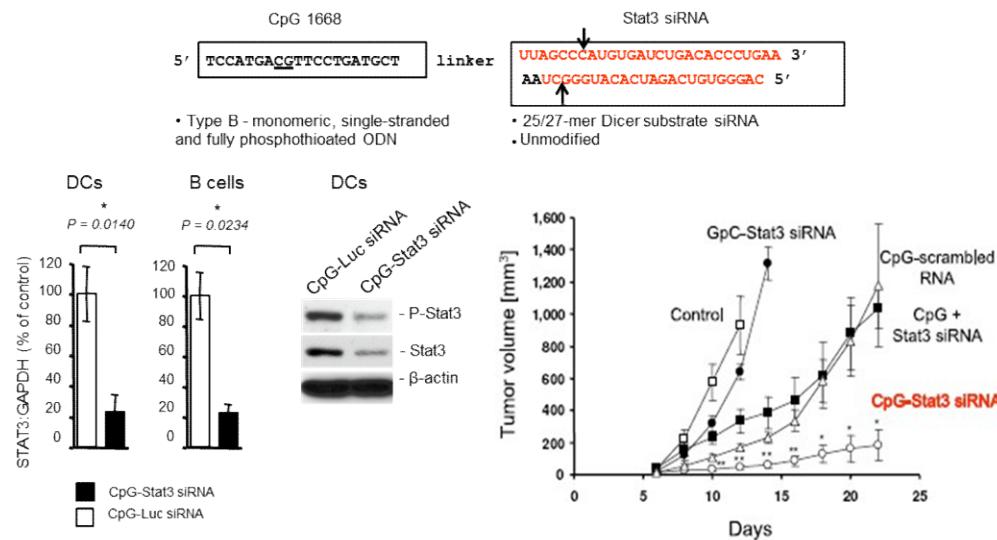
CO-sTiRNA: Preclinical In Vivo Efficacy Studies in B-cell Lymphoma

A20 Mouse Lymphoma Model



LUC ([Nat. Biotech. 2009](#)), BCL2L1 ([Blood 2013](#)), NF-KB/RELA ([Clin.Cancer Res. 2015](#)), STAT5, SMAD2/3 ([unpub.](#)), S1PR1 ([Lee et al. Nat.Med. 2010](#)), A20 ([Braun et al. PlosOne 2015](#)).

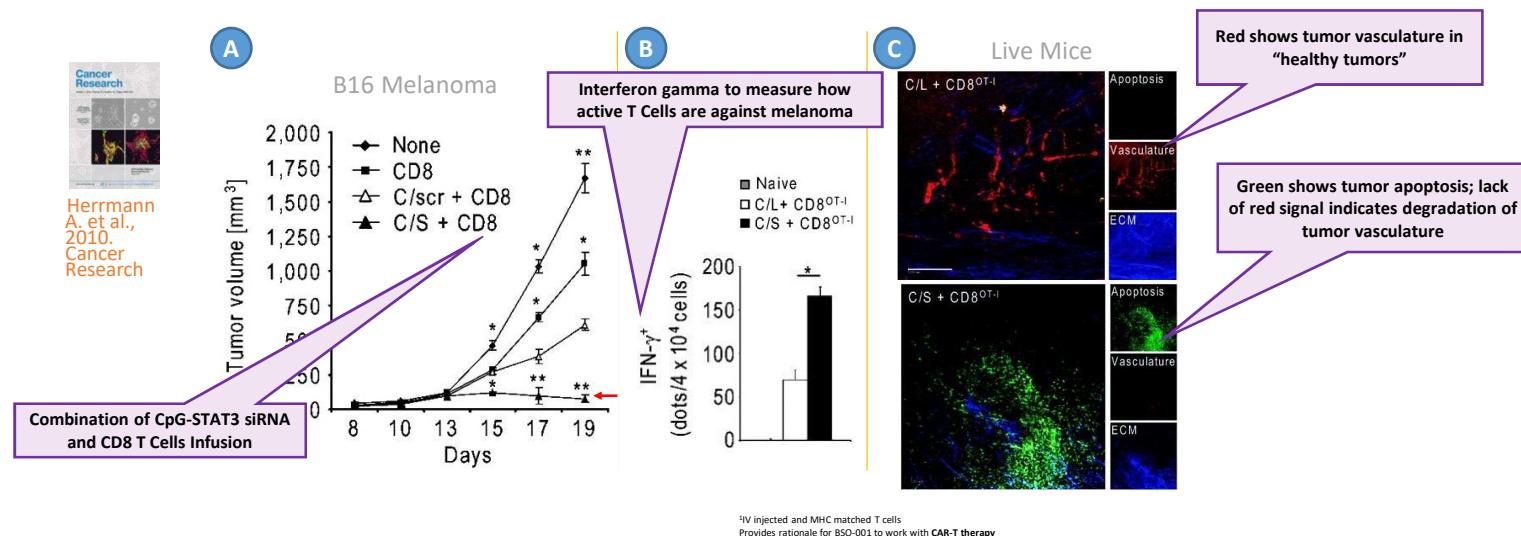
CO-sTiRNA Induces Potent Antitumor Effects in Melanoma and Other Tumor Models



Kortylewski et al., Nat. Biotech., 2009

Source: COH

CO-sTiRNA Treatment Augments In Vivo Anti-TumorActivity of Adoptively Transferred T Cells

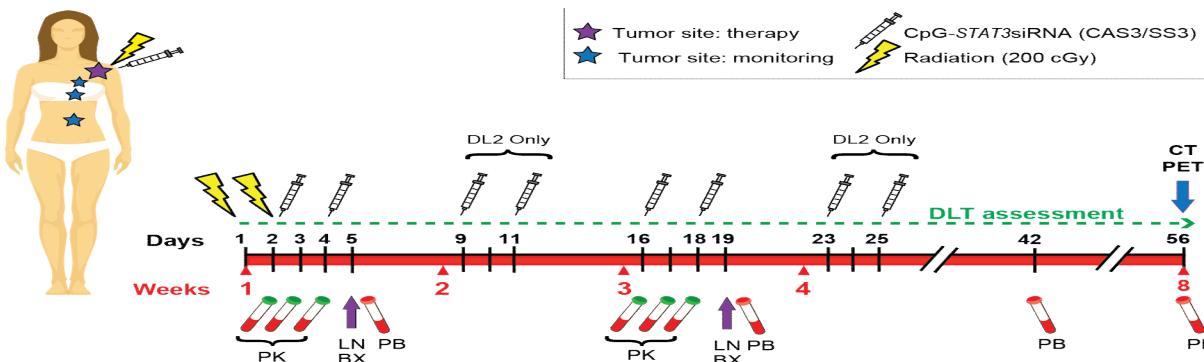


CO-sTiRNA: Pharmacology and Toxicology Studies

- No Cytokine Release Syndrome in vitro (human PBMC), no gross abnormalities in mouse and rat toxicology studies
- Completed initial toxicology study with CO-sTiRNA injected subcutaneously into male and female rats
 - Subcutaneous injection (in rodents) versus proposed local administration in humans - intratumoral in Non-Hodgkin's Lymphoma and intratumoral and intraventricular in GBM
- No toxicity of CO-sTiRNA observed at doses up to 15 mg/kg/injection (equivalent to 2.1 mg/kg dose in humans), exceeding planned clinical trial dosing (0.4 mg/kg/injection)
 - Local delivery proposal gives more confidence in better safety and less possible systemic toxicity
- No gross pathology findings in either terminal phase (i.e., immediately after dosing) or recovery phase animals (i.e., 4 weeks after last dose)
- No safety concerns from non-human primate study
- FDA review at pre-IND meeting with no additional safety concerns noted

Overview of Phase 1 Clinical Trial for CO-sTiRNA

Indication	Non-Hodgkin's Lymphoma
Population/ Indication(s):	Relapsed/Refractory B-Cell Non-Hodgkin's Lymphoma
Phase	Phase 1, with expansion cohort
Segment	Relapsed/ Recurrent
Therapy Status	At initial treatment
Sample Size	15 expected (4-24)
Estimated Study/ Duration	30 months
Participant Duration	Follow-up for at least 6 months or until study termination
Route of Administration	Intratumoral injection
Location	COH, Los Angeles



Treatment Schema. PK: Pharmakokinetic sampling, LN BX: Lymph Node Biopsy of tumor treatment site, PB: Peripheral Blood for research, CT PET: CT or CT/PET scans for tumor response, DLT: dose-limiting toxicity

Phase 1 Clinical Trial Study Design and Proposed Endpoints

- Study design is a modified rolling six dose escalation design to be performed in 2 dose levels, with dose escalation, de-escalation or expansion based on dose-limiting toxicities (DLT)
- There will be a 6-patient expansion at the recommended Phase II dose (RP2D)
- The maximum tolerated dose (MTD) will be the highest dose, among those tested, in which ≤ 1/6 patients experience a DLT. The RP2D will generally be at the MTD but may be less depending on data review

Does Level	CpG plus STAT3siRNA Dose ¹	Radiation Dose 2
-1	15 mg/injection in 1ml volume Schedule: Days 2,4,16, and 18	200cGy/day Schedule: Days 1 and 2
1	30 mg/injection in 1ml volume Schedule: Days 2,4,16, and 18	200cGy/day Schedule: Days 1 and 2
2	30 mg/injection in 1ml volume Schedule: Days 2,4,9,11,16,18, 23 and 25	200cGy/day Schedule: Days 1 and 2

- Proposed Endpoints:

Endpoints	
Primary	<ul style="list-style-type: none"> • Toxicity, according to CTCAE • Determination of MTD and RP2D based on DLT and data review
Secondary	<ul style="list-style-type: none"> • Clinical response (e.g., complete, partial...) and duration of response • Pharmacokinetics and biodistribution • Pharmacodynamic: target gene silencing, local immune response, systemic immune response

CO-sTiRNA Context

CpG Oligonucleotides Alone

- Checkmate Pharmaceuticals (+ anti-PD-1)
- Exicure
- Idera Pharmaceuticals

STAT3 Antisense Oligonucleotides Alone

- Codiak Biosciences (exosome formulation)
- Ionis/AstraZeneca (+ anti-PD-L1)

Small Molecule STAT3 Inhibitors

- Glactone Pharma
- GLG Pharma
- Immix Biopharma
- Janpix
- Kitov Pharmaceuticals
- Kymera Therapeutics
- Moleculin Biotech
- Otsuka Pharmaceutical Co.
- Sumitomo Dainippon Pharma Oncology
- Tvardi Therapeutics
- WPD Pharmaceuticals

Significant Investor Demand for Immuno-Oncology and Gene Therapy IPOs in 2020

Company	Ticker	Date	Price	IPO		Current ⁽¹⁾		Return (IPO to Current)
				Gross Proceeds	Post-money Market Cap.	Price	Market Cap.	
Immuno-Oncology								
Inhibrx, Inc.	INBX	08/19/20	\$17.00	\$136.9	\$641.0	\$17.36	\$654.6	2.1%
Checkmate Pharmaceuticals	CMPI	08/07/20	\$15.00	\$75.0	\$321.5	\$11.53	\$247.2	(23.1%)
iTeos Therapeutics	ITOS	07/24/20	\$19.00	\$201.1	\$632.8	\$29.66	\$1,039.3	56.1%
Nurix Therapeutics	NRIX	07/24/20	\$19.00	\$209.0	\$703.7	\$31.21	\$1,087.4	64.3%
Relay Therapeutics	RLAY	07/16/20	\$20.00	\$460.0	\$1,797.5	\$36.93	\$3,322.6	84.7%
ALX Oncology Holdings	ALXO	07/16/20	\$19.00	\$185.7	\$693.2	\$39.24	\$1,450.7	106.5%
Nkarta	NKTX	07/10/20	\$18.00	\$289.8	\$585.5	\$34.58	\$1,129.9	92.1%
Poseida Therapeutics	PSTX	07/09/20	\$16.00	\$224.0	\$989.1	\$9.89	\$611.4	(38.2%)
Legend Biotech	LEGN	06/05/20	\$23.00	\$487.3	\$3,036.1	\$32.40	\$4,187.4	40.9%
Oric Pharmaceuticals	ORIC	04/24/20	\$16.00	\$138.0	\$478.2	\$21.87	\$667.9	36.7%
I-MAB	IMAB	01/17/20	\$14.00	<u>\$114.5</u>	\$809.6	\$39.50	\$2,284.3	182.1%
Sub-Total Immuno-Oncology				\$2,521.3				
Mean								54.9%
Median								56.1%
Gene Therapy								
CureVac	CVAC	08/14/20	\$16.00	\$213.3	\$2,697.5	\$53.13	\$9,455.5	232.1%
Freeline Therapeutics	FRLN	08/07/20	\$18.00	\$158.8	\$624.2	\$17.14	\$595.4	(4.8%)
Akouos	AKUS	06/25/20	\$17.00	\$244.4	\$584.1	\$26.92	\$925.2	58.4%
Repare Therapeutics	RPTX	06/19/20	\$20.00	\$253.0	\$733.0	\$32.86	\$1,207.6	64.3%
Avidity Biosciences	RNA	06/12/20	\$18.00	\$298.1	\$675.2	\$35.94	\$1,348.8	99.7%
Generation Bio	GBIO	06/12/20	\$19.00	\$230.0	\$878.0	\$30.45	\$1,413.8	60.3%
Passage BIO	PASG	02/28/20	\$18.00	\$248.4	\$808.1	\$16.71	\$760.1	(7.2%)
Beam Therapeutics	BEAM	02/06/20	\$17.00	<u>\$207.0</u>	\$758.0	\$27.26	\$1,353.7	60.4%
Sub-Total Gene Therapy				\$1,853.0				
Mean								70.4%
Median								60.3%
Immuno-Oncology and Gene Therapy								
Total				<u>\$4,374.3</u>				
Mean								61.4%
Median								55.7%

⁽¹⁾ As of September 14, 2020

- MRI-1867 is a proprietary, dual-action, peripherally-restricted NCE CB₁ receptor (CB1R) inverse agonist and inhibitor of iNOS
 - Over activation of CB₁ and iNOS has been implicated in the pathophysiology of systemic sclerosis, including fibrosis of the skin, lung, kidney and heart
 - Modulation of two pathways should result in greater efficacy than targeting only one
- There is no FDA-approved treatment for systemic sclerosis
 - MRI-1867 may qualify for Orphan Drug, Accelerated Approval, Fast Track, Breakthrough Therapy and/or Priority Review from the FDA
 - Recent developments in biopharma industry underscore value of MRI-1867
- MRI-1867 has demonstrated numerous positive characteristics in pre-clinical testing to date
 - Published in vivo studies conducted by the NIH demonstrated that MRI-1867 successfully prevented and treated fibrosis in lungs and liver
 - In vivo studies under our CRADA have shown MRI-1867 was effective in treating fibrosis in a bleomycin-induced skin fibrosis animal model
 - Oral delivery with once daily dosing
 - Does not cross the blood/brain barrier, thus eliminating potential adverse central nervous system side effects

Capital Strategy and Structure

- Principal initial strategy for drug candidate portfolio is to in-license assets post-discovery from leading institutions and others
- Acquire/license rights to drug candidates for which significant capital already invested
 - Reduces capital requirements of internal drug discovery
 - Provides preliminary pre-clinical information as to safety and biological activity
 - Mitigates risks of drug discovery and early development
- Leverage the expertise of multi-disciplinary team and their networks of biopharma and other relationships
 - Enables possible advantageous identification and evaluation of drug candidates for potential acquisition, in-licensing, collaboration and/or other strategic relationships
- Advance assets through pre-clinical and clinical development with on-going monitoring of capital requirements and development risks and optimizing asset monetization strategies
 - Potential transactions include out-licensing, co-development, joint venture and asset sales

Capital Strategy and Structure (Cont.)

- Capital structure with embedded flexibility
 - Optionality as to financing amounts, timing and pathways
 - Optimized to drive growth in shareholder value
- Outstanding warrants structured to accommodate dynamic business strategy
 - Advance drugs further along in clinical development
 - Acquire later-stage clinical assets opportunistically (*i.e.*, “below replacement values”)
- No warrant exercisability until October 2021

Key Takeaways

- Potential game-changing treatment making STAT3 “druggable”
- CO-sTiRNA stands out as both immunotherapy and gene therapy
- Value-driving, near-term milestones
- Multi-billion dollar market opportunities
- High barriers to entry
- Attractive drug candidates with moderate near-term capital requirements
- Strong investor interest in immuno-oncology and gene therapy companies with promising new drug candidates
- Highly-motivated leadership team committed to improving patient outcomes and driving shareholder value



Thank You