Corporate Presentation

January 2016

Craig A. Dionne, PhD
President & CEO

OTC QB: **GNSZ**
www.GenSpera.com

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Corporate Overview

- Publicly listed in 2009 (OTC QB: GNSZ) with IP exclusively licensed from inventors out of John Hopkins
- Deep experience in oncology drug discovery and development
- A leader in prodrug therapeutics for the treatment of cancer
- Robust, global Intellectual Property platform
- Highly-differentiated drugs utilizing natural, plant-derived toxin
- World-class manufacturing and supply partners
- Global barrier to generic entry via exclusive manufacturing and farming controls of raw goods
- Multiple value creation catalysts in 2016

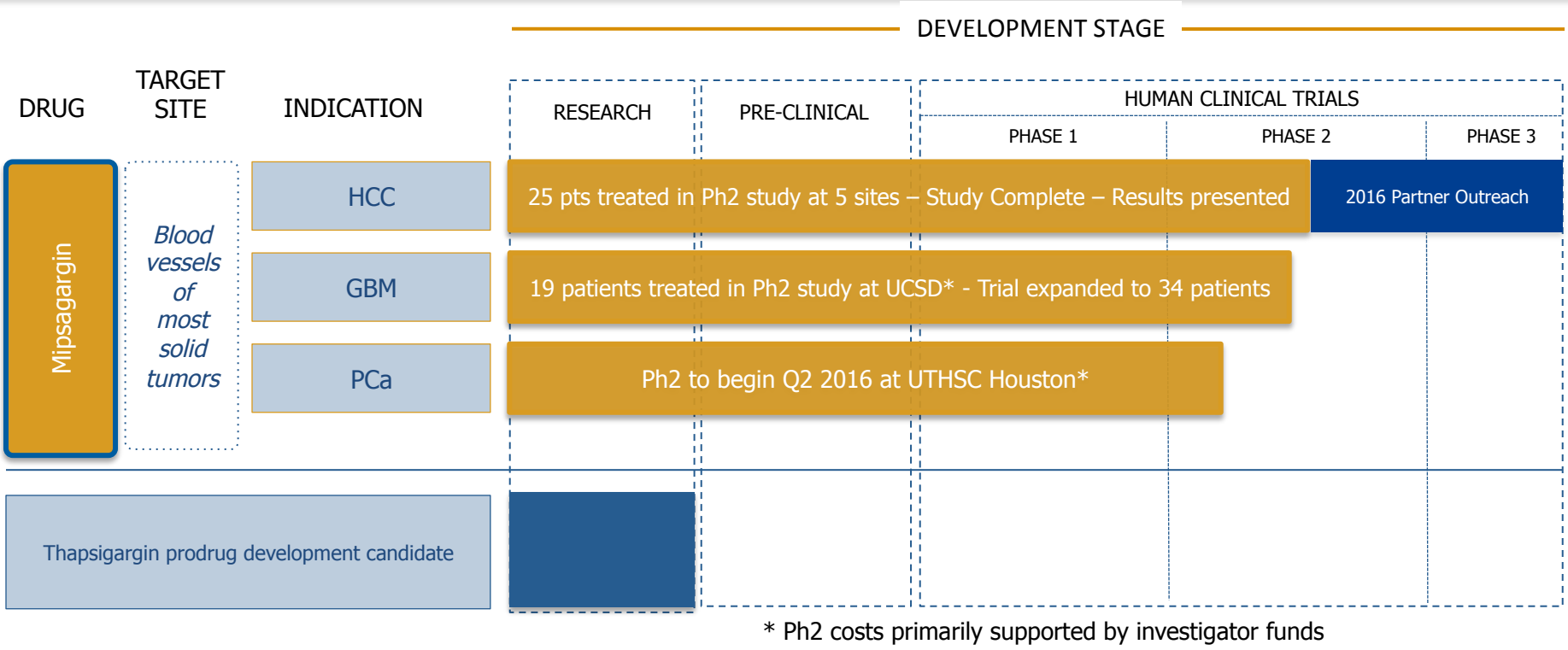
Investment Highlights

- Developing a naturally occurring toxin in a peptide delivery system to target solid tumors without the side-effect profile of chemotherapeutic agents
- Large market opportunities in multiple cancers including liver, brain and prostate with potential for combination with immunotherapy
- Efficacy being demonstrated in mid-stage clinical trials in 2 orphan indications
  - Positive proof-of-concept Phase 2 liver cancer trial
  - Highly encouraging data from ongoing Phase 2 brain cancer trial
- Mechanism of action protected by robust, global patent estate including exclusive license to technology developed at Johns Hopkins University
- Exclusive rights with world-class partners for commercial-scale production and raw material supply
- Management has a history of success in oncology drug discovery and development

Experienced Leadership and Scientific Development Team

Management and Board	(Prior Experience / Affiliations)
Craig A. Dionne, PhD *Chairman and Chief Executive Officer*	Cephalon; Prostate Cancer Foundation
Russell Richerson, PhD *COO and Corporate Secretary*	Abbott; Mannheim Boehringer; Prometheus Therapeutics & Diagnostics; DuPont; Ventana
Peter E. Grebow, PhD *Director*	Cephalon; Optimer Pharmaceuticals, Inc.; HQ Holdings est 1988; Complexa
Bo Jesper Hansen, MD, PhD *Director*	Sobi Swedish Orphan Biovitrum; Orphazyme; Newron Pharmaceuticals; Onxeo; Hyperion Therapeutics; Ablynx
Scott V. Ogilvie *Director*	AFIN International, Inc.; Gulf Enterprises International, Ltd.; CIC group inc.; Hill Farrer
Scientific Advisory Board	
John T. Isaacs, PhD *Chief Scientific Advisor, Co-Founder*	Professor at Johns Hopkins School of Medicine, Co-Inventor of GenSpera's technologies
Samuel R. Denmeade, MD *Chief Clinical Advisor and Co-Founder*	Professor at Johns Hopkins and Board Certified Medical Oncologist, Co-Inventor of GenSpera's technologies
Søren Brøgger Christensen, PhD *Scientific Advisory Board Member*	Professor at University of Copenhagen; expert in chemistry of thapsigargin, exploring further derivatives and manufacturing efficiencies for thapsigargin
Santosh Kesari, MD, PhD *Science Advisory Board Member*	Chair, Department of Translational Neuro-Oncology and Neurotherapeutics, John Wayne Cancer Institute, Board Certified Neurologist and Neuro-Oncologist

Evaluating Multiple Oncology Indications



* Ph2 costs primarily supported by investigator funds

- Glioblastoma Phase 2 trial ongoing in 2016
- Data with IP = increased corporate valuation
- Provides expanded options for partnering
- Global market = partnership opportunities driven by disease type

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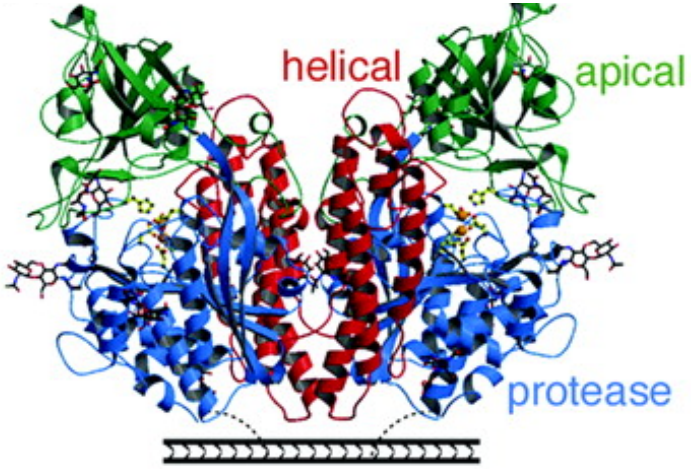
Scientific Background & IP

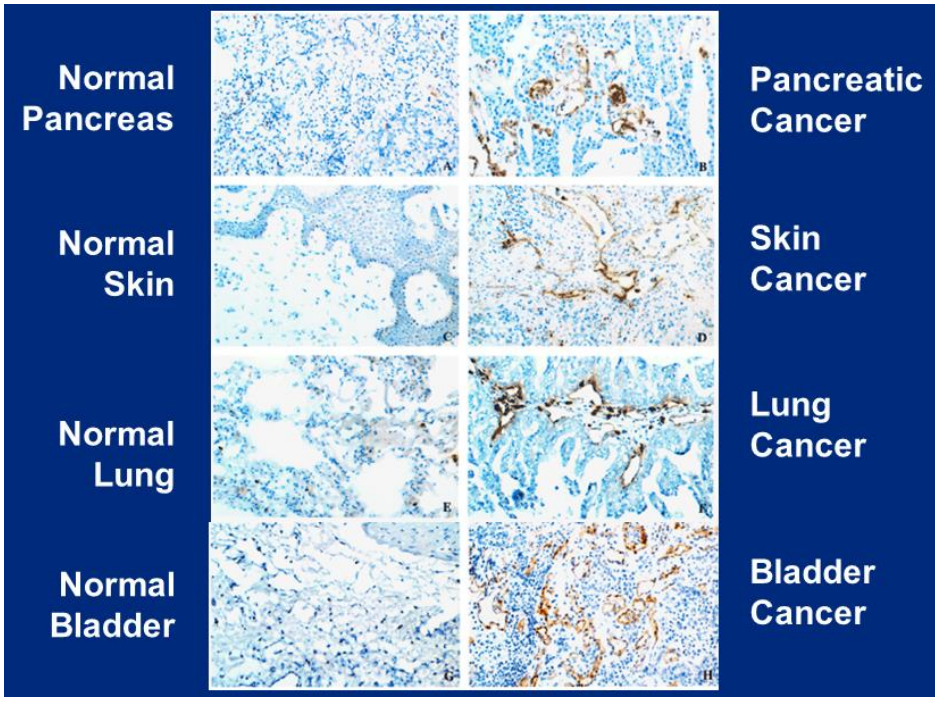
Platform Overview

- GenSpera's platform is based on exclusively licensed technology developed at Johns Hopkins University and the University of Copenhagen
- Platform combines a cytotoxin with a protective peptide, creating a blood soluble prodrug that remains inactive until in the presence of a specific enzyme
  - Selected enzyme is chosen due to its high level of expression in tumors
  - The peptide helps solubilize the prodrug and prevents its internalization into cells until the peptide is removed
  - The enzyme cleaves the peptide from the prodrug and releases the active ingredient only within the tumor, resulting in tumor death
- Unique mechanism of action with enhanced anti-tumor activity
- Targeted and localized cytotoxic presence minimizes side effects
- Target cells unable to acquire resistance

Prostate-Specific Membrane Antigen (PSMA): Pan-Tumor Target

PSMA





PSMA is expressed by tumor vasculature

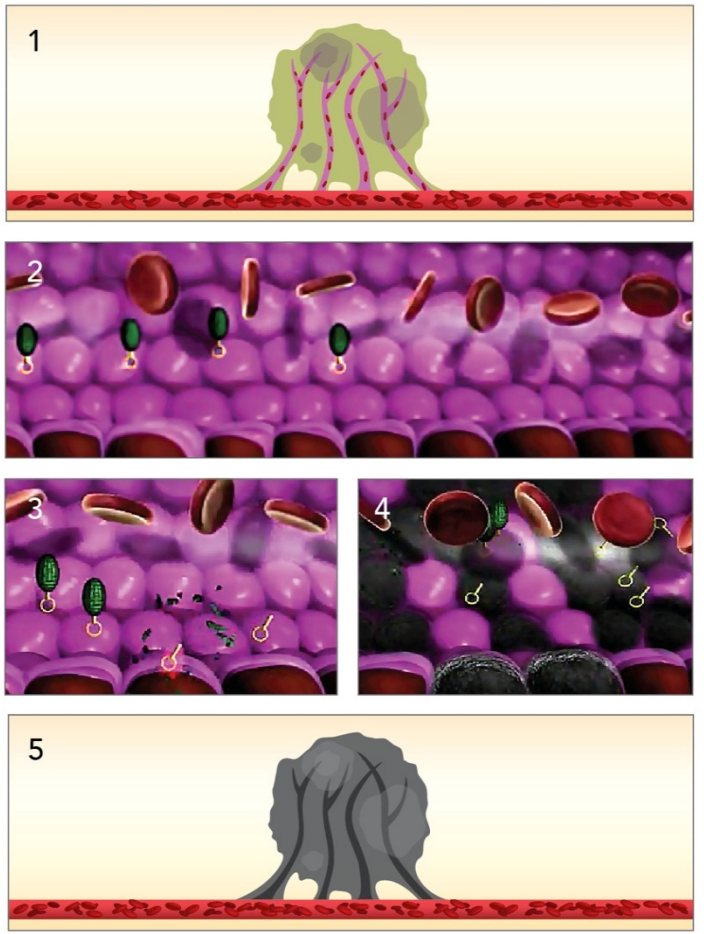
- Type II transmembrane glycoprotein
- Unique proteolytic activity
- Highly expressed in normal and malignant prostate epithelial cells
- Selective expression in the vascular endothelium of solid tumors – both established and new growth

Precision Targeting by Design

Mechanism of Action

1. Blood vessels feed living tumor
2. Drug circulates in bloodstream in benign fashion
3. PSMA enzyme within tumor blood vessels activates drug
4. Activated drug kills blood vessels *and* tumor cells
5. Death of tumor

→ *Potential for Complete Tumor Kill*
→ *Fewer Side Effects*



Intellectual Property

- Exclusive license to use technology developed at Johns Hopkins University and the University of Copenhagen
- Current infusion composition
  - Patent coverage in U.S. to 2023 – additional years via patent restoration and ODD
  - Data exclusivity outside of U.S. – up to 10 years after drug approval in EU
- New composition-of-matter Patent Application
  - Worldwide exclusivity expected through 2033
- Other applications filed for methods of use, formulations, process improvements
- Exclusive partnership with Phyton Biotech for commercial-scale thapsigargin production
- The name mipsagargin has novel stem – new drug class differentiation value

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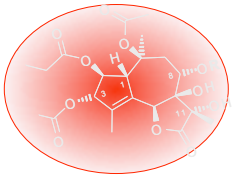
Mipsagargin:
First-in-class Oncologic Agent

Mipsagargin – Lead Drug Candidate

- A prodrug that becomes active only at tumor site
  - Plant-based toxin (thapsigargin derivative, 12ADT) is combined with a protective peptide
  - Peptide targets the PSMA enzyme, which is highly expressed on the surface of tumor vasculature
  - PSMA removes the peptide, releasing toxin that kills blood vessels feeding tumor and also cancer cells
- Targeted drug delivery significantly reduces side effects
  - No apparent effect on bone marrow – combination with immunotherapy is viable
  - Manageable side effect profile
- Orphan drug designation for liver cancer; pending for brain cancer
- Prolonged disease stabilization (via RECIST) in a significant percentage of liver cancer patients

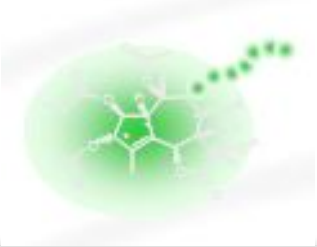
Extraction and chemical modification

12ADT



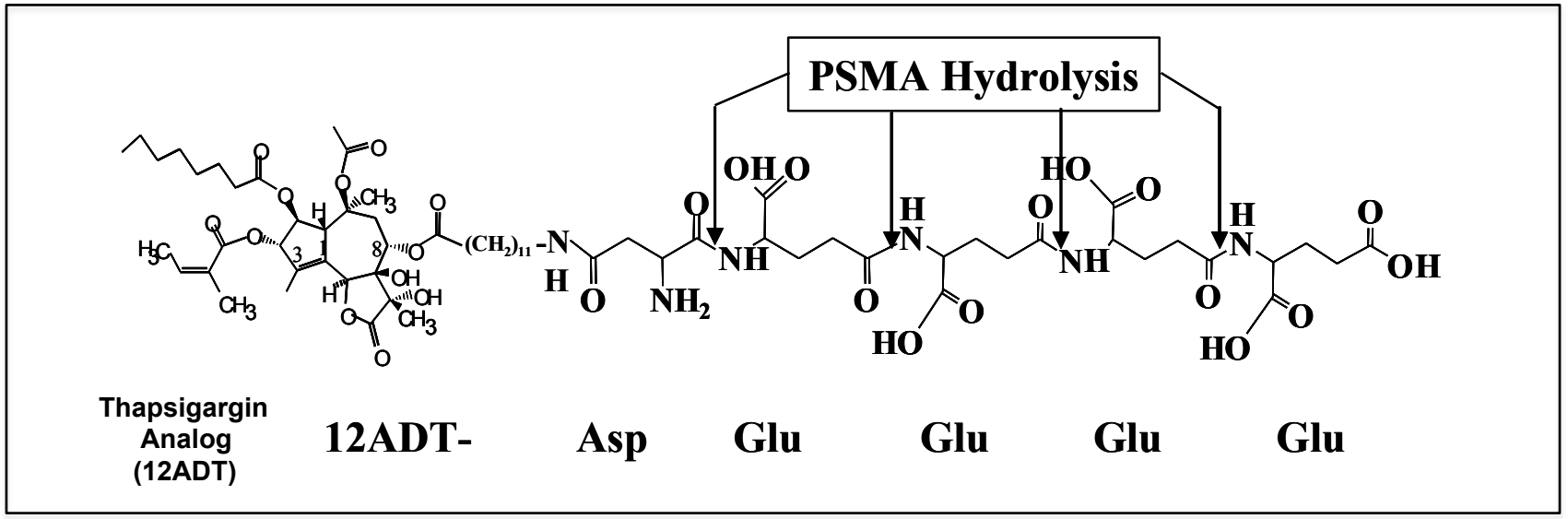
Masking and targeting peptide attached

PSMA removes peptide, releasing toxin at tumor site



Mipsagargin – Chemistry

- Mipsagargin consists of a thapsigargin derivative, 12ADT, coupled to a peptide that helps solubilize the prodrug and prevents its internalization into cells until the peptide is removed
- PSMA (an enzyme highly expressed on the surface of cancer tumor vasculature) recognizes and removes the peptide, releasing 12ADT only within the tumor, bringing about cell death



Mipsagargin – Compelling Preclinical Data

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Efficacy Pharmacology

- Complete tumor regression with monotherapy in mouse tumor models
  - No need to combine with other drugs
  - Fewer side effects
- Safety margin of 7- to 10-fold in mouse
- No acquired resistance to mipsagargin

Bioanalytical

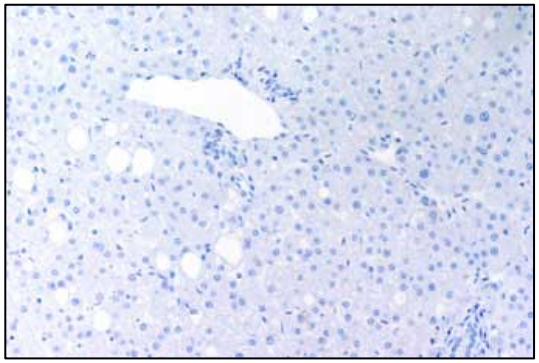
- In blood, can only find intact mipsagargin and no released active 12ADT
- 12ADT is found at very high levels in tumors, less found in liver and kidneys, not found elsewhere -*Targeted Delivery!*

Toxicology

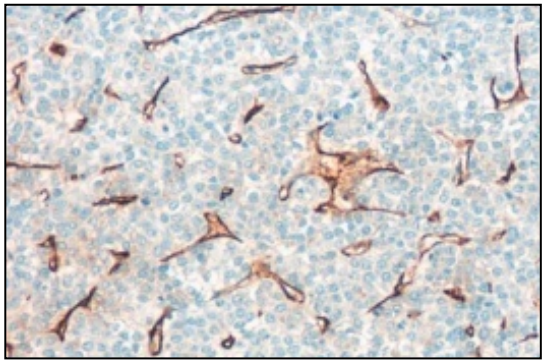
- No effect on liver or cardiovascular system
- No effect on bone marrow – no immunosuppression or anemia
- Kidney is the affected organ at high doses – dose-dependent, non-cumulative, easily monitored and 100% rapidly reversible

Evidence of PSMA Expression in HCC and GBM

PSMA Staining in Normal and Malignant Liver Tissue

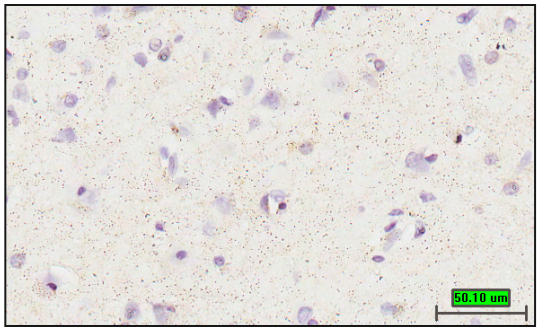


Normal liver tissue

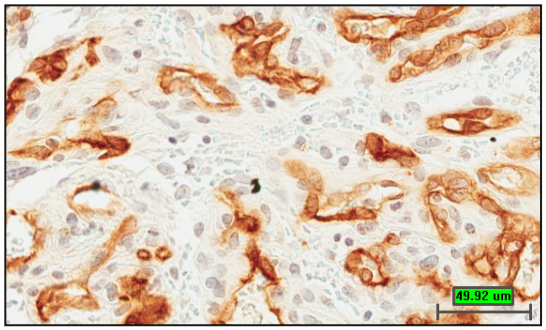


PSMA positive vasculature in HCC

PSMA Staining in Normal and Malignant Brain Tissue



Normal brain tissue



PSMA positive vasculature in GBM

Mipsagargin – Market Focus

- Mipsagargin attacks a universal attribute of all solid tumors – PSMA
- Tremendous global unmet need in numerous types of cancers
- Indications eligible for FDA orphan drug designation (HCC granted, GBM pending)
- GBM is potential stepping stone to larger indications (e.g., breast and lung cancer metastases to brain)

Hepatocellular Carcinoma (HCC)

- 3rd largest cancer-killer worldwide
- Over 600k annual deaths worldwide
- Over 700k new cases diagnosed annually worldwide
- $1.4B market size by 2019

Glioblastoma Multiforme (GBM)

- 13K deaths annually in the US
- Temodar reached peak sales of > $1B

Prostate Cancer (PCa)

- 221K new cases diagnosed annually in the US
- $8.6B market size by 2022

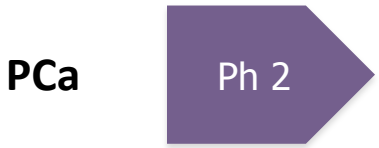
Renal Cell Carcinoma (RCC)

- 61K new cases diagnosed annually in the US
- 14K deaths annually in the US

Mipsagargin Clinical Development

- Current Phase 2 results expected Q4'16
- Phase 2b/3 trial to be conducted at numerous sites worldwide

- East Asia dose-escalating Phase 2a to begin in Q4'16
- Phase 2b/3 study sites focused in East Asia, with some in US and EU



- Phase 2 trial to be initiated in Q2'16

Note: Clinical development plan dependent on access to capital. Phase 2b/3 trial designs not yet finalized or discussed with FDA.

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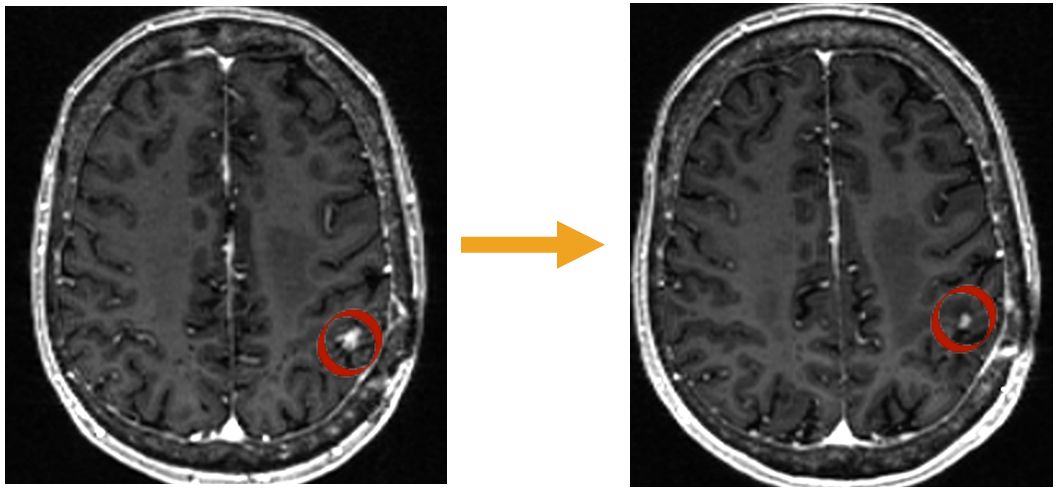
Mipsagargin for Glioblastoma

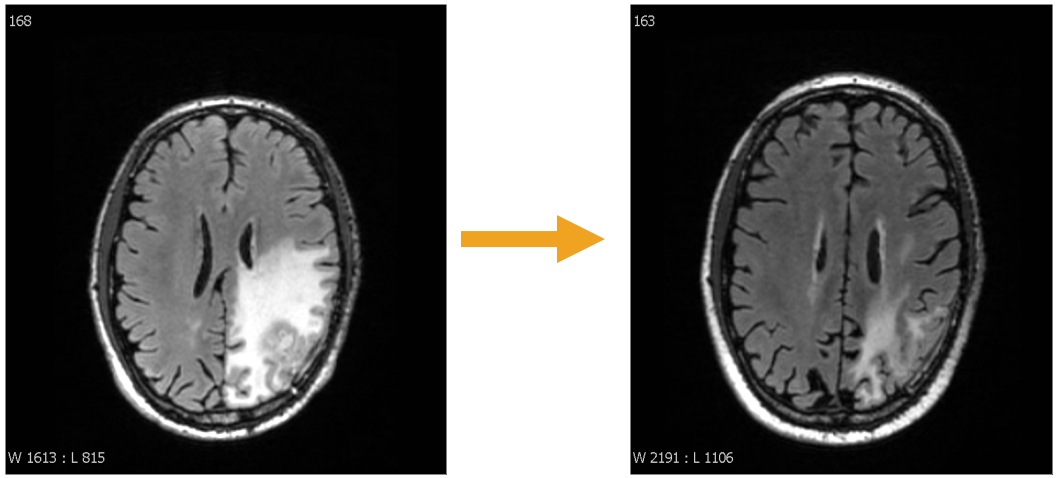
GBM Phase 2 Trial Interim Data

- Recurrent GBM patients (prior surgery, radiation, temozolomide)
- Simon two-stage design
  - Data in first 12 patients exceeded criteria required for expansion
- Variable expression of PSMA – only 60% have 2+ or 3+ staining
  - Biomarker development funded by FDA grant
- 3 of 11 evaluable patients experience clinical benefit
  - SD for at least 2 months, reduction in tumor volume
  - All 3 pts have $\geq$2+ staining
- 1 patient with partial response at 11 months
  - Dramatic reduction in edema, off steroids
- Side-effect profile similar to HCC trial

GBM Initial Results: MRI Scans



Pt 012
T1-MRI



Pt 007
FLAIR-MRI

Baseline

One Month

Proposed (Phase 2/3) Trial Design

- **Indication:** GBM
- **Geography(s)**: Worldwide
- **Partners:** TBD
- **KOLs:** Santosh Kesari, MD, PhD
- **Possible Trial Design**: Two stage adaptive trial design
  - Stage 1: 75 patients in a Phase 2 trial (20 sites)
  - Stage 2: 400 patients in a Phase 3 (100 sites)
- **Primary Study Endpoints**: Overall Survival
- **Expected Timing**:
  - Initiation: Stage 1 to begin in Q4'16
  - Completion: Stage 2 component expected to conclude by 2H'20

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Mipsagargin for HCC

Clinical Trials of Mipsagargin

Phase 1 First-in Human Study

- Multi-center, dose escalation, 3+3 design
- Advanced solid tumors
- One-hour intravenous infusion in saline on Days 1, 2 and 3 of 28-day cycle
- Protocol-defined MTD not reached; 66.8 mg/m^2 established as MTD with 40 mg/m^2 on Day 1 to avoid infusion-related reactions
- 44 patients treated, including 5 with HCC at 40/66.8/66.8 mg/m^2
- Prolonged disease stabilization in HCC (12 and 25 months) observed in 2 of 5 patients

Phase 2 Study in HCC after Progression on Sorafenib

- 25 patients treated
- 40 mg/m^2 on Days 1, 2 and 3 accepted for this patient population
  - 40 mg/m^2 on Days 1, 2 and 3 for patients 1-3 and 10-25
  - 40/66.8/66.8 mg/m^2 on Days 1, 2 and 3 for patients 4-9; dose reduction due to SAEs
- Study complete

Patient Demographics – Phase 2

Indication	**Hepatocellular carcinoma, progressed on sorafenib (25, 100%)**
Dose and Schedule	**40 mg/m^2 on Days 1, 2 and 3 of 28-day cycle (19 pts)** **40 mg/m^2 on Day 1, 66.8 mg/m^2 on Days 2 and 3 of 28-day cycle (6 pts)**
Performance Status	**ECOG 0 (8 pts, 32%) ECOG 1 (17 pts, 68%)**
Child-Pugh Score	**A5 or A6 (18, 72%) B7 or B8 (7, 28%)**
Extra-Hepatic Disease	**No (6 pts, 24%) Yes (19 pts, 76%)**
Age	**Mean 64.5 years (range 51 - 73)**
Gender	**Male (18 pts, 72%) Female (7 pts, 28%)**

Safety Observations – Phase 2

Most Frequently-Occurring Mipsagargin-Related AEs Grade ≥ 2 *	Number (%) of Patients
Creatinine increased	8 (32%)
Fatigue	7 (28%)
ALT increased	6 (24%)
AST increased	6 (24%)
Bilirubin increased	6 (24%)
LDH increased	5 (20%)
Blood urea increased	5 (20%)
Thrombocytopenia	4 (16%)
Hyperkalemia	3 (12%)

* Observed in at least 3 patients

Safety Observations – Phase 2

Mipsagargin-Related SAEs

All Mipsagargin-Related Serious Adverse Events	Number (%) of Patients	Mipsagargin Dose (Day 1 / Day 2 / Day 3)
Acute renal failure/acute renal injury*	3 (12%)	40/66.8/66.8
Congestive cardiac failure*	1 (4%)	40/40/40
Chest pain*	1 (4%)	40/40/40

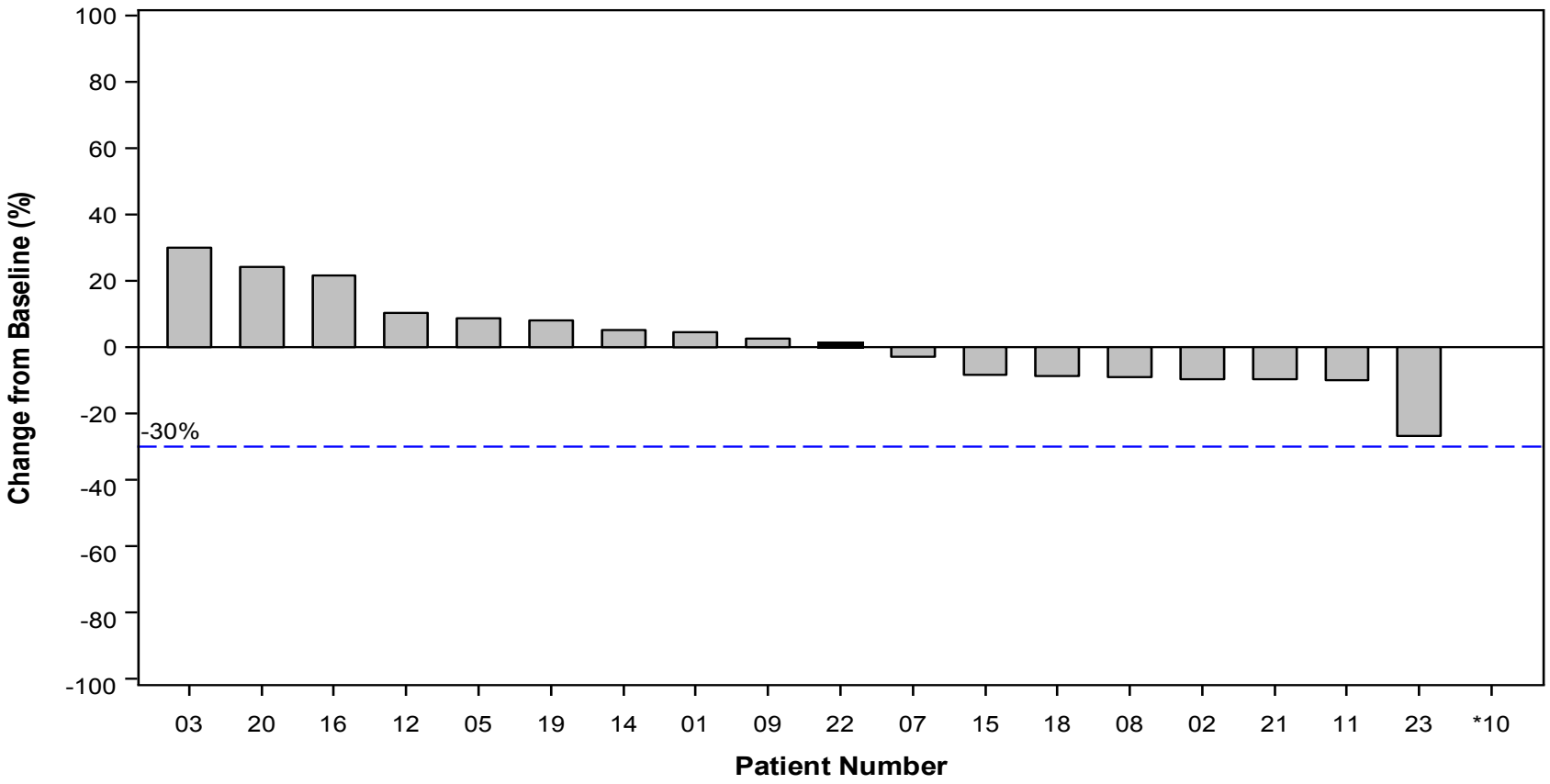
* Grade 3 SAE

Clinical Observations – Phase 2

Number of Cycles (n=25 patients)	**75 total (average 3 per pt; range 1 - 9)** **63 cycles at 40/40/40** **12 cycles at 40/66.8/66.8**
Evaluable for Response (EE Population)	**19 patients (76%)**
Best Response (EE Population)	**CR + PR 0 pts SD 12 pts (63%)**
SD ≥ 5 cycles	**6 pts (24%)**
TTP (EE Population)	**134 days* (4.5 months; 95% CI: 58.0, NE)****
OS (EE Population)	**205 days (6.8 months; 95% CI: 138, NE)*****
PFS (EE Population)	**129 days (4.3 months; 95% CI: 58.0, 205.0)******

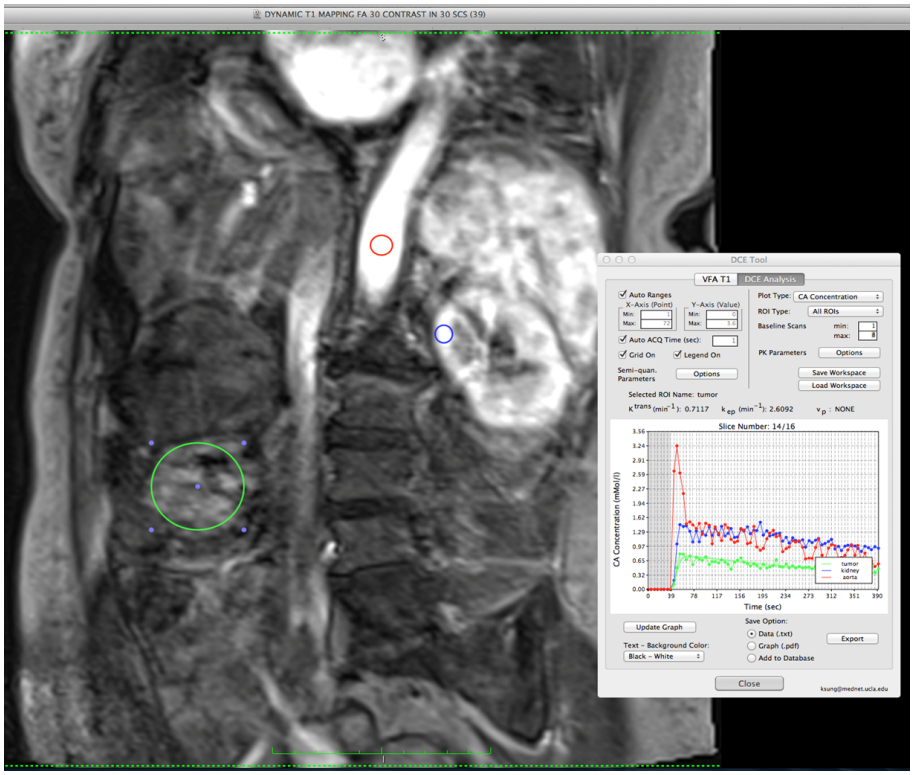
* p < 0.001 vs. historic control of 2.1 months
** 7 patients censored
*** 8 patients censored
**** 2 patients censored

Best Change in Tumor Size – Phase 2



Note: *Patient 010 exhibited new metastatic disease and was removed from study; post-treatment target lesion measurement not performed.

DCE-MRI – Decreased Vascular Enhancement



DCE-MRI on 28 Apr 2014; measured K_{trans} = 0.72-0.76 min^{-1}

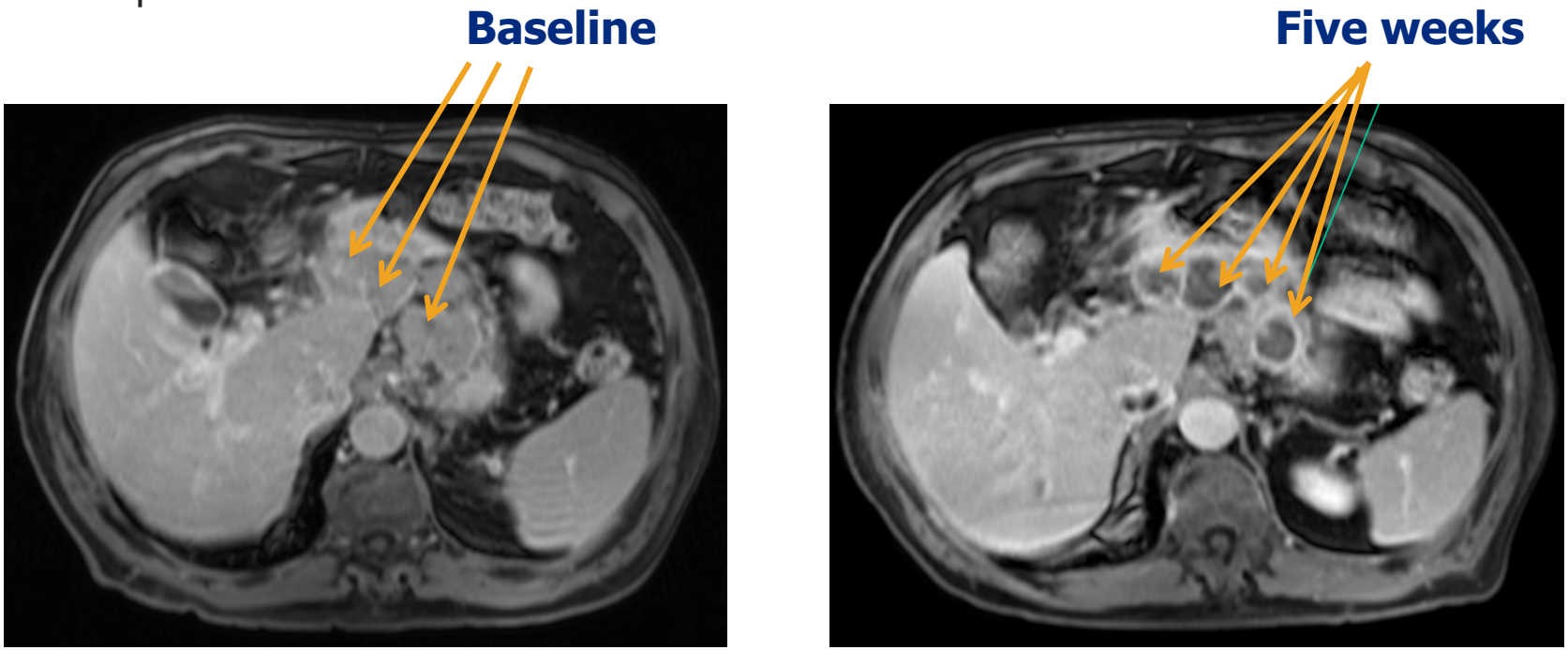
DCE-MRI on 02 Jun 2014; measured K_{trans} = 0.14-0.16 min^{-1}

Red: aorta; blue: kidney; green: tumor.

Range reflects use of larger and smaller regions of interest (ROIs) within the lesion.

HCC – Pharmacodynamic Proof of Principle via DCE-MRI

- RECIST criteria is limited by lack of tumor viability information
- HCC patient with gastrohepatic metastatic lymph node involvement (gold arrows)
- Increased hypoenhancement after mipsagargin treatment (right panel) suggests response



Mipsagargin vs Sorafenib

	Mipsagargin Phase 2a Study (25 pts)	Sorafenib SHARP Study (299 pts)
Population	**Sorafenib Failures**	**Chemo Naïve**
ECOG 0/ 1 or 2 (%)	**32/68**	**54/46**
Child-Pugh A/B (%)	**72/28**	**95/5**
Extra hepatic disease (%)	**76**	**53**
DCR (Disease Control Rate) (%)	**63***	**43****
Increase in TTP symptomatic (months)	**2.4*****	**0.8******
Side effects	**Minimal, easily managed**	**Significant**

***** **At 56 days**
****** **At 28 days**
******* **p < 0.001 vs historical control of 2.1 months**
******** **Not significant vs placebo**

Source: Llovet, JM, et.al., "Sorafenib in Advanced Hepatocellular Carcinoma," The New England Journal of Medicine 359:4 (2008): 378-90.

Phase 2 HCC Trial Results Overview

Impressive clinical activity

- Highly differentiated from other anti-cancer approaches
- Outstanding clinical safety profile
- Minimal and manageable side effects
  - Fatigue, nausea, rash, reversible creatinine increase
  - No apparent effect on bone marrow
- Data strongly suggestive of clinical activity in second-line HCC
  - Study participants experienced a median time to progression of 4.5 months ($p<0.001$), more than twice the time demonstrated in prior studies with placebo or ineffective agents
  - 63% of patients experienced disease stabilization (via RECIST) at 2 months
  - Prolonged disease stabilization in a significant percentage of patients – 24% with stable disease > 5 months
- Additionally, mipsagargin demonstrated decreased blood flow in liver tumors as measured by DCE-MRI

Next HCC Clinical Development Steps

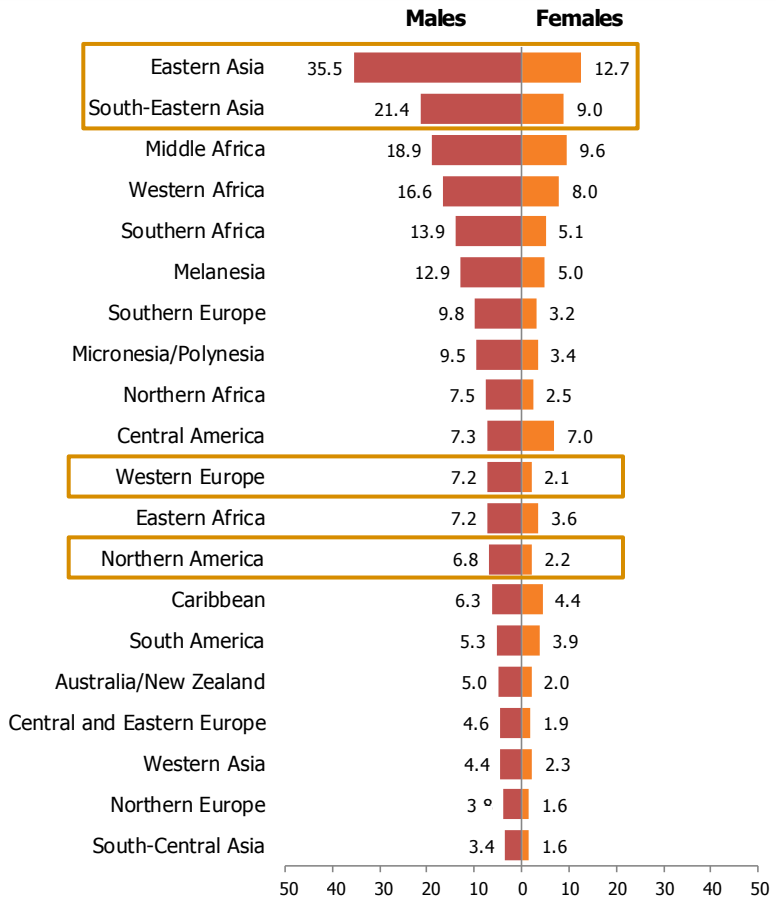
Next Steps: Asia Represents Advantageous Environment for Clinical Development

- Liver cancer is the most common type of cancer in Southeast Asia
- Three fourths of worldwide liver cancer cases in males, and two thirds in females, occur in 15 Asian countries
  - Compared to the US and EU, high rates of Hepatitis B virus (HBV) prevalence, and to a lesser extent HCV, are leading factors for higher liver cancer rates
- Regular screening of high-risk HBV carriers in Eastern Asia allows for early detection of liver cancer when potentially curative treatment can still be offered
  - Clinical development program for mipsagargin will initially be focused in East Asia
  - Rapid trial initiation in Singapore, Taiwan and/or Hong Kong because of potential to access US INDs

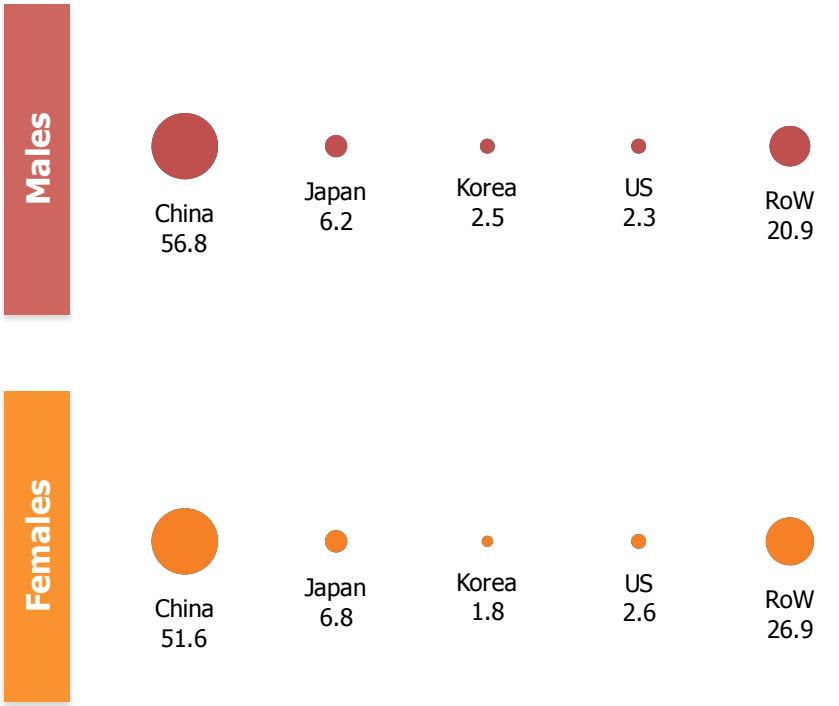
Source: Pfizer Facts: The Burden of Cancer in Asia, 2008.

Market Focus: Liver Cancer in Asia

Age-Standardized Liver Cancer Incidence Rates by Sex and Geography (per 100,000)



Annual WW New Liver Cancer Cases by Country ('000s)



Source: GLOBOCAN 2008.

Phase 2a Dose Escalation Trial

- **Indication:** HCC
- **Geography(s)**: Taiwan, Singapore and potentially Hong Kong
- **Partners:** TBD
- **KOLs:** TBD
- **Trial Design**: Dose escalation study in East Asian population; typical 3+3 design
- **Trial Size**: 9-18 patients
- **Study Endpoints**: Safety, Tolerability, Response Rate
- **Expected Timing**:
  - Initiation: Phase 2a trial to be initiated in Q4'16
  - Results: 2H'17
  - Trial to be followed by Phase 2b/3, scheduled to begin in Q4'17

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Mipsagargin for Prostate

Mipsagargin in PCa

Phase 2 Neoadjuvant PCa Study

- Site: University of Texas Health Science Center in Houston; investigator-sponsored
- Initiate in Q2'16
- Expected completion Q4'16

Prostate Cancer (PCa)

- 221K new cases diagnosed annually in the U.S.
- Est. $8.6B market by 2022*

* Source: Decision Resources

Other Drug Candidates

Prodrug Candidate	Activating Enzyme	Target Location of Active Enzyme	Status
G-115	Prostate-Specific Antigen (PSA)	Prostate Cancer	Pilot toxicology completed; limited preclinical development
G-114	Prostate-Specific Antigen (PSA)	Prostate Cancer	Validated efficacy in preclinical animal models (Johns Hopkins)
G-301	Human Glandular Kallikrein 2 (hK2)	Prostate Cancer	Validated efficacy in preclinical animal models (Johns Hopkins)

GenSpera: Going Forward

Business Strategy

- Optimize value across multiple indications, territories, and technologies
- Continue developing drug candidates independently
- Expand management and staffing
- Continue business development discussions and partnership opportunities
- Develop pipeline through internal efforts, licensing, or acquisition

Clinical Milestones

Mipsagargin Clinical Trials

Liver Cancer		Brain Cancer*		Prostate Cancer*	
Q4'16	Begin Phase 2a (East Asia dose-escalation)	Q4'16	Complete Phase 2	Q2'16	Begin Phase 2
Q3'17	Complete Phase 2a	Q4'16	Begin Phase 2b		
Q4'17	Begin Phase 2b/3	2H'20	Complete Phase 3		
Q4'22	Complete Phase 2b/3				

* Primarily funded by collaborating institution

Investment Highlights

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- Large market opportunities in multiple cancers including liver, brain and prostate with potential for combination with immunotherapy
- Efficacy being demonstrated in mid-stage clinical trials in 2 orphan indications
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- Exclusive rights with world-class partners for commercial-scale production and raw material supply
- Management has a history of success in oncology drug discovery and development