



Corporate Presentation

Rodman & Renshaw

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Forward-Looking Statements

This presentation contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act with respect to the outlook for expectations for future financial or business performance, strategies, expectations and goals. Forward-looking statements are subject to numerous assumptions, risks and uncertainties, which change over time. Forward-looking statements speak only as of the date they are made, and no duty to update forward-looking statements is assumed. Actual results could differ materially from those currently anticipated due to a number of factors. Such factors, among others, could have a material adverse effect upon our business, results of operations and financial condition. Additional information about the factors and risks that could affect our business, financial condition and results of operations, are contained in our filings with the U.S. Securities and Exchange Commission, which are available at www.sec.gov.

Company Overview - NASDAQ:CASI

- **U.S.** biopharmaceutical company focused on the acquisition, development, and commercialization of innovative therapeutics addressing cancer and other unmet medical needs for the global market, with a focus on delivering innovative drugs to China and the U.S.
- **Headquartered** in Rockville, Maryland, U.S.A. with a R&D Center in Beijing, China
- **2012** new investors and refocus of the company's R&D focus and commercial approach
 - **Strong financial backing** from long-term, mission-driven shareholders (IDG-ACCEL, KPCB, others)
- **Experienced Management** with track record of accomplishment in both North America and China

Company Overview - Corporate Structure



Beijing R&D Center

- Discovery Research
- Regulatory Affairs
- Clinical Development
- Business Development
- Financing
- Commercialization



Corporate Headquarters

- Clinical Development
- Regulatory Affairs
- Business Development
- Accounting & Internal Controls
- Corporate Administration
- Legal
- Investor Relations

Company Overview - Growing Pipeline

- **A growing product pipeline** developed under dual model of in-license and internal development:
 - In-license of cancer therapeutics – development & commercialization in China:
 - EVOMELA® (CE-Melphalan)
 - MARQIBO® (vinCRISTine sulfate LIPOSOME)
 - ZEVALIN® (ibritumomab tiuxetan)
 - Internal development of new chemical & biologic entities:
 - ENMD-2076 for the treatment of multiple solid tumors
 - Immuno-Oncology targets

China R&D Core Competencies

- Beijing-based operations experienced in all aspects of IND/CTA submissions, clinical trials, safety reporting and project management to navigate successful regulatory filings with the China FDA
 - Strong relationships with CFDA: 6 CTAs filed with CFDA over past 2 years; 4 approvals, each within 12 months
- Conducting clinical trials in China in full compliance with ICH and CFDA GCP standards
 - High quality data and safety reporting - utilized for global development and regulatory filings
 - Rapid patient recruitment that can accelerate trials and reach proof-of-concept inflection points or expand into additional indications

China Core Competencies (2)

- CASI's oncology clinical network and top Chinese KOLs as principal study investigators
- Strong relationships with preferred, quality contract organizations (CMC, preclinical) and distributors who have established market presence and can provide cost-effective, competitive programs for commercial success

Value Proposition

Leverage CASI's US/China oncology expertise and resources for the development and commercialization of innovative therapeutics

- Extensive experience/capabilities in China/US/EU to support global development programs
- Experienced Beijing-based R&D group to navigate successful regulatory filings with the CFDA and execution of the clinical trials
- Cost-effective development in China to reach POC inflection points quickly to support local/global development programs
 - Rapid patient recruitment, strong relationships with KOLs and major cancer hospitals in China
- Rapid entry into China's oncology market via the Import Drug Registration approach or Local Development path
 - Green Channel/fast track status from CFDA
- Tech transfer of CMC for local manufacturing can reduce production costs and be primary or secondary source of API/drug product

Financial Highlights

	12 Months Ended December 31,		Six Months Ended June 30	
	2016	2015	2017	2016
Total revenues	\$ -	\$ 47,712	\$ -	\$ -
Research & development	4,645,560	4,075,572	2,776,694	2,381,433
General & administrative	4,775,050	3,118,269	1,335,585	2,679,877
Net loss	(9,453,488)	(7,206,423)	(4,108,764)	(5,086,236)
Net loss per share attributable to common shareholders	\$ (0.17)	\$ (0.22)	\$ (0.07)	\$ (0.12)
Weighted average shares outstanding (basic and diluted)	55,869,205	32,445,811	60,196,574	41,556,911
Cash & cash equivalents	\$ 27,092,928	\$ 5,131,114	\$ 23,360,529	\$ 18,534,467

Pipeline and Development Status

LATE-STAGE DRUG CANDIDATES FOR CHINA REGION ONLY

	INDICATION	IN-LICENSED FROM SPECTRUM	CTA FILING & REVIEW	CONFIRMATORY TRIAL	NDA FILING & REVIEW	LAUNCH IN CHINA
EVOMELA®	Multiple Myeloma	[Progress bar: ~80%]				
MARQIBO®	Acute Lymphoblastic Leukemia	[Progress bar: ~90%]				
ZEVALIN®	Non-Hodgkin's Lymphoma	[Progress bar: ~40%]				

CLINICAL DRUG CANDIDATE

	INDICATION	PRE-CLINICAL	IND/CTA	PHASE 1	PHASE 2	PHASE 3
ENMD-2076	Triple-Negative Breast Cancer	[Progress bar: ~95%]				
	Fibrolamellar Carcinoma	[Progress bar: ~85%]				

PRECLINICAL CANDIDATES IN IMMUNO-ONCOLOGY

	INDICATION	PRE-CLINICAL	IND/CTA	PHASE 1	PHASE 2	PHASE 3
CASI-001	Oncology	[Progress bar: ~60%]				
CASI-002	Oncology	[Progress bar: ~50%]				

In-licensed Pipeline for the China Market

Products Under Development for Import Drug Registration in China			
Product	Description	Indication	Status
EVOMELA[®]	Captisol [®] Enabled (propylene glycol-free) melphalan	Multiple myeloma (MM) transplant setting	Import Drug Registration application filed with China FDA; Priority Review Granted
MARQIBO[®]	vincristine sulfate LIPOSOME injection	Advanced adult Ph-acute lymphoblastic leukemia (ALL)	Import Drug Registration application filed with China FDA
ZEVALIN[®]	ibritumomab tiuxetan injection, a cd20-directed radiotherapeutic antibody	Advanced non-Hodgkin's lymphoma (NHL)	Import Drug Registration filing in process with China FDA

EVOMELA[®], MARQIBO[®], ZEVALIN[®]

- Significant competitive advantage in greater China's marketplace due to its strong IP position and significant technology barriers.
- Minimal clinical and regulatory risks, although it may take time to get marketing approval.
- The value of each of the products will increase incrementally as marketing approval approaches, and we expect to see sustained growth in market share once CFDA approval is granted.
- The direct costs for having those products approved in China are significantly lower, compared with development of new NCEs in China.



Collectively, \$148 million annual peak sales are forecast

- **No form of melphalan, branded or generic, is currently available in China – EVOMELA will address an urgent medical need**
- CASI's import drug application for EVOMELA was granted **priority review** by the China FDA, acknowledging the urgent medical need and EVOMELA's therapeutic advantages to currently available therapeutics.
 - Lack of propylene glycol solvent which is related to certain safety events
 - Captisol formulation allows for increased stability when reconstituted for patient administration
- Annual incidence of multiple myeloma in China is estimated at 12,197 cases with a case mortality of 9,038 in 2015*
- **Annual peak sales are estimated to be over \$35 million, assuming ≥ 10% self-pay**

[*http://globocan.iarc.fr/Pages/online.aspx](http://globocan.iarc.fr/Pages/online.aspx)

GLOBOCAN 2012: Estimated Cancer Incidence,
Mortality and Prevalence Worldwide in 2012

International Agency for Research on Cancer



- Proprietary liposomal formulation of vincristine which improves the pharmacokinetics and pharmacodynamics of vincristine*
 - Higher maximum tolerated dose, superior antitumor activity and delivered higher amounts of active drug to target tissues
- Annual incidence of leukemia in China is estimated at 75,300 cases with a mortality of 53,400 cases in 2015**
- **Annual peak sales are estimated to be over \$49 million, assuming a $\geq 10\%$ self-pay**

*Silverman, J and Deitcher S. Marqibo (vincristine sulfate liposome injection) improves the pharmacokinetics and pharmacodynamics of vincristine Cancer Chemother Pharmacol (2013) 71:555–564

**Chen W, Zheng R, Baade PD, Zhang S, Zeng H, Bray F, Jemal A, Yu XQ, He J. Cancer statistics in China, 2015. CA Cancer J Clin. 2016 Mar-Apr;66(2):115-132.

The Market Potential in China

- Anti-CD20 antibody coupled to Yttrium-90
 - 83% ORR, 39% CR in relapsed or refractory, low-grade or follicular B-cell NHL
- Annual incidence of NHL in China is estimated at 83,790 new cases with a mortality of 50,100 cases in 2015*.
- ZEVALIN is expected to rapidly penetrate the Chinese market as the centralized structure of patient care in China results in a much less fragmented delivery system relative to other countries. In China, hospitals are the central facility of oncology patient care and physicians are likely to refer their patients for ZEVALIN treatment within the same hospital.
- **Annual peak sales are estimated to be \$64 million for FNHL, assuming a ≥ 10% self-pay**

*Chen W, Zheng R, Baade PD, Zhang S, Zeng H, Bray F, Jemal A, Yu XQ, He J. Cancer statistics in China, 2015. CA Cancer J Clin. 2016 Mar-Apr;66(2):115-132.

EVOMELA[®], MARQIBO[®] and ZEVALIN[®] Projected Timelines

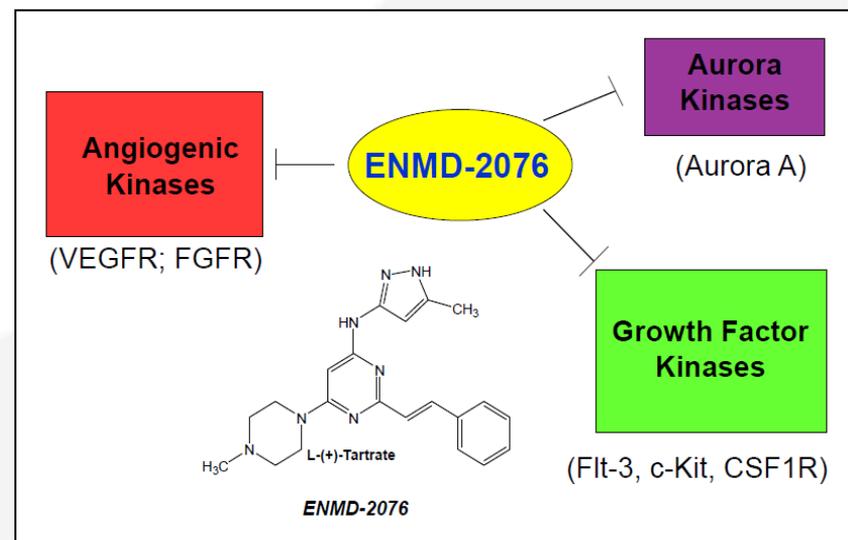
- **EVOMELA**
 - CTA Approval: *December 2017 – January 2018*
 - Confirmatory trial start: *Q2-Q3 2018*
 - Commercial launch: *Q4 2019*

- **MARQIBO**
 - CTA Approval: *Q4 2017*
 - Confirmatory trial start: *Q2 2018*
 - Commercial launch: *Q4 2020*

- **ZEVALIN**
 - CTA Approval: *Q4 2018*
 - Confirmatory trial start: *Q3 2019*
 - Commercial launch: *Q4 2021*

ENMD-2076 - A NCE for Solid Tumors

- **Unique MOA:** An orally active, small molecule, multi-targeted kinase inhibitor with selected inhibition of Aurora A, VEGFR, FGFR, and GF, and a Mechanism of Action against angiogenesis, proliferation and the cell cycle.
- **Strong Preclinical Data:** demonstrated strong anti-tumor activities *in vitro* and in multiple *in vivo* animal models.
- Good pharmaceutical PK/PD property
- Good safety profile.
- Low cost and easy manufacturing
- Multiple publications in international journals.
- **FDA Orphan Drug Designations** in HCC, ovarian cancer, multiple myeloma and AML.
- **Strong Global IP** with the first patent issued in the U.S. in 2009 and in China in 2013.



ENMD-2076 - Phase I Data

Phase I Data Demonstrated Good Safety Profile and Promising Preliminary Antitumor Activity in Multiple Types of Solid Tumors

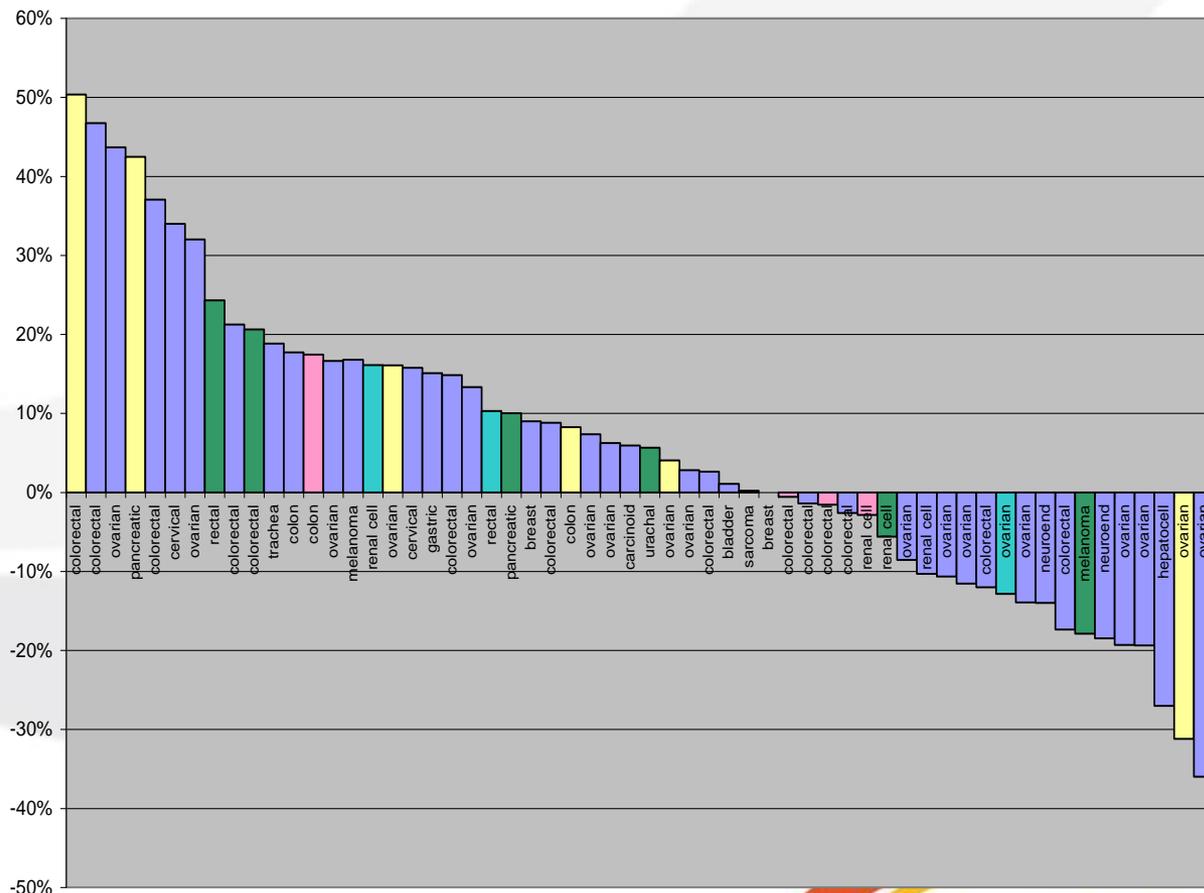
Recommended dose:

- 160 mg/m²/d

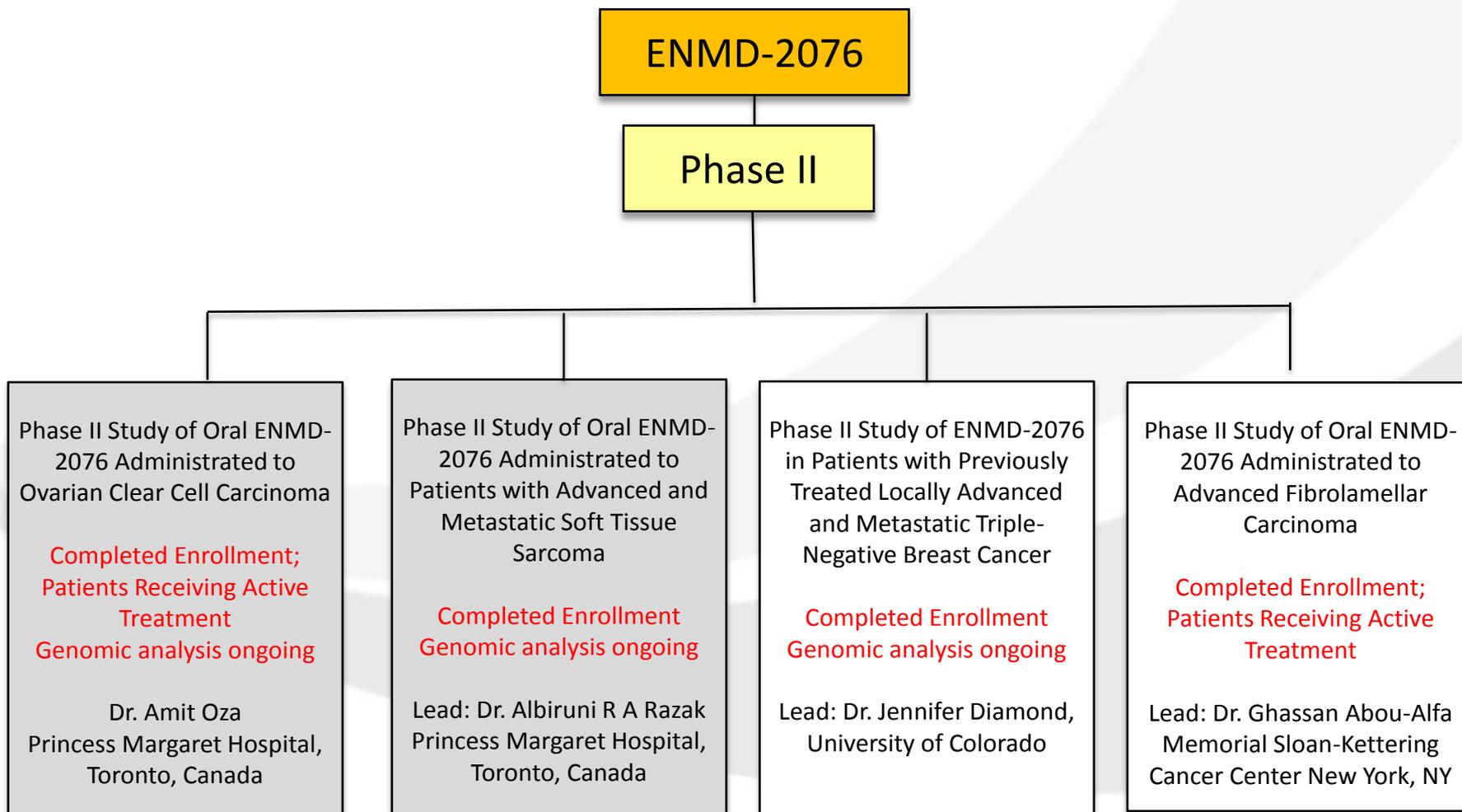
Partial Response/Progression Free Survival 6 months patients include:

- Triple-Negative Breast Cancer (TNBC)
- Soft Tissue Sarcoma (STS)
- Ovarian clear cell carcinoma (OCCC)
- Fibrolamellar Carcinoma (FLC)

Data supports further Phase II clinical trials



ENMD-2076 - Phase 2 Trials



No further company-sponsored clinical investigation

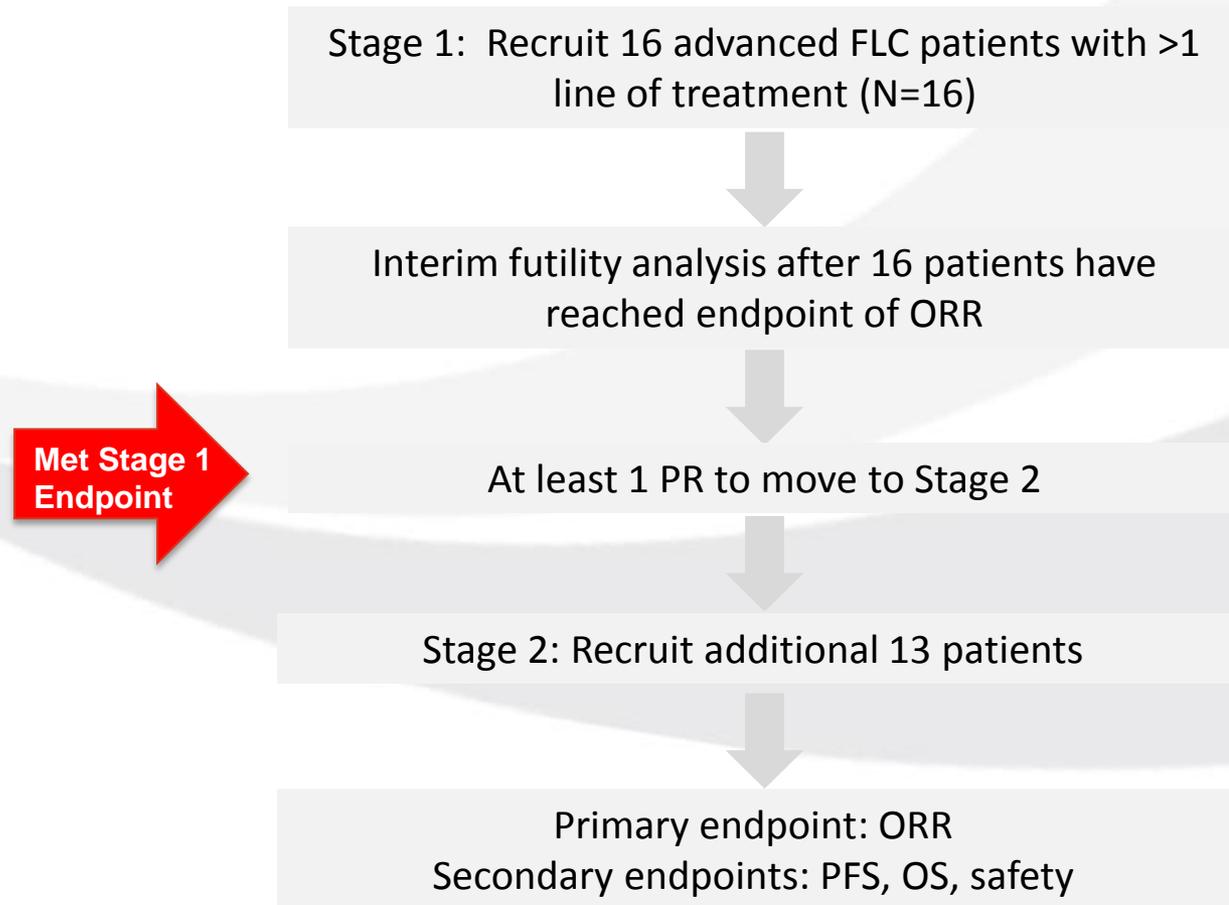
ENMD-2076 – Potential Treatment of FLC

FLC, fibrolamellar carcinoma, a rare type of hepatocellular carcinoma (HCC) liver cancer:

- Accounts for 0.6- 8.6% of HCC. The total incidence of HCC is estimated to be 780,000 each year worldwide based on the World Health Organization.
- Primarily affects adolescents and young adults.
- Is often advanced when diagnosed due to lack of symptoms.
- Surgical resections are the optimal treatment for localized tumors, but has a very high recurrence rate; For post-operative recurrence, resection is often not possible.
- No standard first-line systemic treatment option available.
- Overall prognosis remains poor because of its primary chemo-resistance and early recurrence of metastasis.
- An unmet medical need for development of new therapeutics.

ENMD-2076 – Phase 2 Trial in FLC - Ongoing

A single arm, single agent, 2 stage design multi-center Phase 2 trial of ENMD-2076 for the treatment of advanced FLC patients



Investment Highlights

- **Market:** Well-positioned to pursue significant and unprecedented market opportunities in China and worldwide.
- **Model:** Compelling business model with important North America/China synergies and competitive advantage with efficiency and effectiveness for drug development and commercialization.
- **Product:** A strong and growing product pipeline developed under dual models of in-license and internal development.
- **Management:** An experienced, motivated leadership with track record of accomplishment in both North America and China.
- **Finance:** Strong cash position, backed by successful venture fund and smart money, under careful financial management with low burn rate.
- **Value Drivers:** Significant defined near-term value drivers supporting long term growth.



NASDAQ: CASI

www.casipharmaceuticals.com