



**Corporate Presentation**

**2018 Annual Stockholder's Meeting**

**June 11, 2018**

**Rockville, Maryland**

# Forward-Looking Statements

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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](http://www.sec.gov).

# Investment Highlights (1)

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- **Management:** Experienced team with accomplished track record in North America and China
- **Diversified product portfolio:**
  - ***In-licensed FDA approved products for greater China from Spectrum Pharmaceuticals***
    - EVOMELA® (CE-Melphalan), granted priority review by China Food and Drug Administration (CFDA)
    - MARQIBO® (vinCRISTine sulfate LIPOSOME), CFDA review in progress
    - ZEVALIN® (ibritumomab tiuxetan), CFDA review in progress
  - ***Acquired from Sandoz Inc.*** 25 U.S. FDA-approved ANDAs, including Entecavir, Bisoprolol Fumarate, Desvenlafaxine SR, Aripiprazole and Cilostazol
  - ***Internally developed ENMD-2076***
    - Phase 2 small molecule multi-kinase inhibitor targeting triple negative breast cancer and fibrolamellar carcinoma, enrollment complete, biomarker/genomic analysis ongoing

## Investment Highlights (2)

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- **Compelling business model:** Capitalizing on critical U.S./China synergies & competitive advantages for efficient/cost-effective drug development and commercialization
  - Rockville, MD
    - Corporate office, senior management, finance, accounting, compliance, regulatory operations
  - Beijing
    - Operations dedicated to all aspects of IND/CTA submissions, clinical trials, patient safety and project management to navigate successful regulatory filings with CFDA
- **Well-capitalized:** Strong financial position, backed by successful venture funds; under careful financial management with low burn rate

# Business Premise

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- 1.4 billion people in China + 325 million people in the U.S. demand high quality and affordable medicines
- Recent CFDA reforms allow for expedited approvals of FDA ANDAs and innovative products
- CASI is in a unique position to leverage the CFDA reforms by introducing FDA-approved products (ANDAs and NDAs) into China
- CASI has a successful record of licensing/acquisition of approved NDAs & ANDAs to be brought into the China market place.
- Lower cost of goods produced in China can be leveraged on a worldwide basis

# Financial Highlights

	<u>12 Months Ended December 31,</u>		<u>Three Months Ended March 31</u>	
	<u>2017</u>	<u>2016</u>	<u>2018</u>	<u>2017</u>
Total revenues	\$ -	\$ -	\$ -	\$ -
Research & development	7,595,182	4,645,560	1,697,232	1,049,287
General & administrative	3,156,138	4,775,050	1,303,122	644,163
Net loss	(10,770,202)	(9,453,488)	(3,590,910)	(1,700,182)
Net loss per share attributable to common shareholders	\$ (0.18)	\$ (0.17)	\$ (0.05)	\$ (0.03)
Weighted average shares outstanding (basic and diluted)	61,513,988	55,869,205	71,215,000	60,196,574
Cash & cash equivalents	\$ 43,489,935	\$ 27,092,928	\$ 49,887,600	\$ 25,528,952

- Received \$50 million financing in 2018 from long-term mission-driven investors
- Prudent management of financial resources; cash runway through next 12-18 months
- Independent Board committees include Audit Committee and Compensation Committee
- Sarbanes Oxley 404 compliant
- Corporate policies and controls include confidentiality policies, restrictive trading policies, and Code of Ethics



# Bi-Cultural Board & Management Team

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## BOARD OF DIRECTORS

**Dr. Wei-Wu He, Executive Chairman**

**Dr. Quan Zhou**

**James Huang**

**Dr. Rajesh C. Shrotriya**

**Dr. Y. Alexander Wu**

**Franklin C. Salisbury, Jr.**

IDG-ACCEL China Fund Venture Partner; Managing Director of ETP Global Fund, L.P.; Chairman & CEO, OriGene Technologies, Inc. Founder, Managing Director and General Partner at IDG Capital Partners

General Partner, Kleiner Perkins Caufield & Byers China Chairman & CEO, Spectrum Pharmaceuticals, Inc., 2000-2017 CEO, Crown Biosciences, Inc., 2006-2017 President, National Foundation for Cancer Research

## SENIOR MANAGEMENT

**Wei-Wu He, Ph.D.**

**Ken K. Ren, M.D., Ph.D.**

**Alexander A. Zukowski, M.D.**

**Cynthia W. Hu, J.D.**

**Jim Goldschmidt, Ph.D.**

**Sara B. Capitelli, CPA**

Executive Chairman

Director and Chief Executive Officer

Chief Medical Officer

Chief Operating Officer, General Counsel & Secretary

SVP, Business Development

VP, Finance, Principal Accounting Officer

# Diversified Product Portfolio

**Through Acquisitions, In-licensing and Internally Developed Innovative, Targeted Oncology Therapeutics and High Quality ANDAs**

## Greater China Rights

Acquired From SPPI

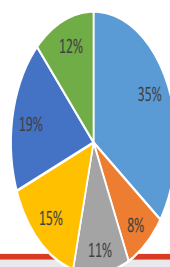


## US FDA ANDAs

Acquired From Sandoz

- 25 FDA Approved ANDAs
- 4 FDA Pending ANDAs

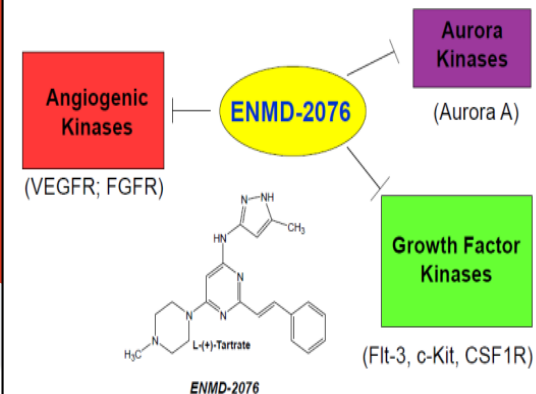
Diverse Portfolio Across Attractive Therapeutic Area



- Hypertension/PVD
- Metabolic Disorders
- CNS
- Anti-infectious
- Anti-inflammatory/pain
- Others

## ENMD-2076, Global Rights

Innovative Drug Candidate  
Targeted Therapy for Solid Tumors





# Licensed Products

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- CASI entered into licensing agreement with Spectrum Pharmaceuticals for China development & commercialization of three hematological oncology products
- **Exclusive rights to three commercial products:**
  - **EVOMELA**<sup>®</sup> *Conditioning treatment prior to autologous stem cell transplant for patients with multiple myeloma and as a palliative treatment of patients with multiple myeloma;*
    - **Priority Review granted 2017**
    - **Expert Clinical Advisory Committee meeting April 26, 2018**
  - **MARQIBO**<sup>®</sup> *Treatment of acute lymphoblastic leukemia*
  - **ZEVALIN**<sup>®</sup> *Treatment of non-Hodgkin's Lymphoma*
- **CASI responsible for development and commercialization in greater China**
  - Import drug registration applications
  - Confirmatory clinical studies
  - Premarketing and commercial launches

# Evomela<sup>®</sup>: China Market Potential



- **Status: CFDA priority review; Expert Clinical Advisory Committee meeting took place on April 26, 2018**
- Import drug application for EVOMELA granted **priority review** by CFDA, underpinning high unmet medical need and advantages to currently available therapeutics
  - *Lack of propylene glycol solvent (related to certain adverse events)*
  - *Captisol formulation allows for increased stability when reconstituted for patient administration*
- **No form of melphalan, branded or generic, currently available in China – EVOMELA addresses urgent medical need**
- Annual incidence of multiple myeloma in China: ~12k cases with case mortality of ~9k in 2015\*
- **Annual peak sales estimated over \$75m**

[\\*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](#)



# Marqibo®: China Market Potential



- **Status: CFDA review in progress**
- Proprietary liposomal formulation of vincristine which improves pharmacokinetics and pharmacodynamics of vincristine\*
  - *Higher maximum tolerated dose, superior antitumor activity and higher amounts of active drug delivered to target tissues*
- Annual incidence of leukemia (all types) in China estimated at 75k cases with mortality of ~53k cases in 2015\*\*
  - **Annual peak sales estimated > \$49m**

\*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.

# Zevalin®: China Market Potential



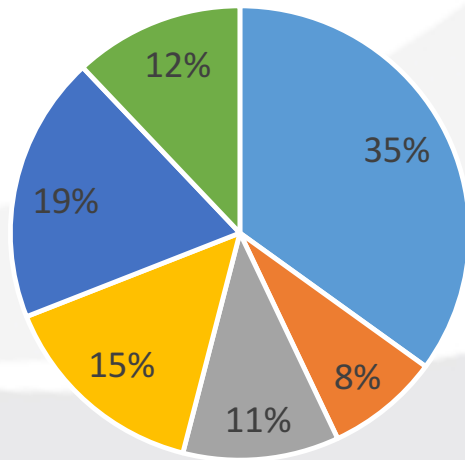
- **Status: CFDA review in progress**
- Anti-CD20 antibody coupled to Yttrium-90
  - 83% ORR, 39% CR in relapsed or refractory, low-grade or follicular B-cell NHL (FNHL)
- Annual incidence of NHL in China estimated at ~84k new cases with mortality of ~50k cases in 2015\*
- Expected to rapidly penetrate Chinese market as centralized structure of patient care results in less fragmented delivery system relative to other countries. Hospitals are central facility of oncology patient care; physicians likely to refer patients for treatment within same hospital.
- **Annual peak sales estimated to be \$64m for FNHL**

\*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.

# 29 ANDA Products & Pipeline

**25 FDA Approved, 4 Pending FDA Approval ANDAs**

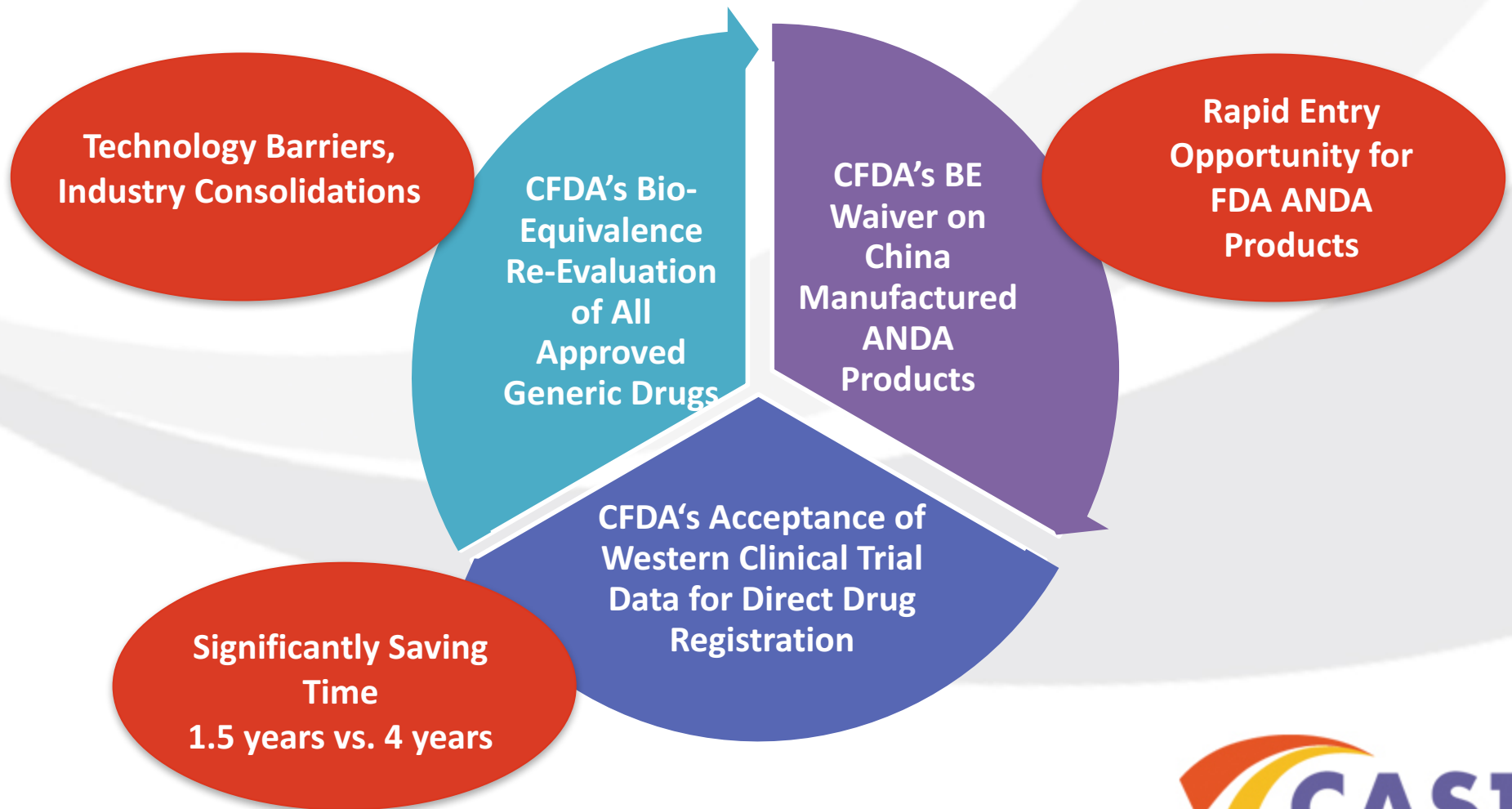
Diverse Portfolio Across Attractive Therapeutic Area



- Hypertension/PVD
- CNS
- Anti-inflammatory/pain
- Metabolic Disorders
- Anti-infectious
- Others

# Historical Opportunity for FDA-Approved ANDAs in China

*Rapidly Establishing Leading Position in China's >\$100B Small Molecule Drug Market*



# China Market for 5 Key ANDA Products

## Key Products Based on 2016 (Estimated) Generic Sales in China

	Product	Indication	2016E Sales (in US\$)
1	Entecavir tablet	Chronic HBV Infection	1,490,000,000
2	Bisoprolol Fumarate tablet	Hypertension	205,000,000
3	Desvenlafaxine SR tablet	Depression	170,000,000
4	Aripiprazole tablet	Schizophrenia	108,000,000
5	Cilostazol tablet	Peripheral vascular disease	65,000,000
		<b>Total Estimated Sales</b>	<b>US \$2,038,000,000</b>

\* Source: China's PDB Database

# U.S. Market for 8 Key ANDA Products

## Key ANDAs 2016 U.S. Sales\*

	Product Name	Indications	Sandoz U.S. Sales 2016 (IMS data)	Overall U.S. Generic Sales 2016 (IMS data)
1	Midodrine	Hypotension	19,800,000	56,900,000
2	Triamterene / HCTZ cap	Hypertension, diuretic	4,600,000	54,000,000
3	Lisinopril tab	Hypertension	15,000,000	93,000,000
4	Bisoprolol Fumarate	Hypertension	8,400,000	22,000,000
5	Cilostazol tab	peripheral vascular disease	2,200,000	13,000,000
6	Diclofenac Potassium tab	OA, RA	5,400,000	27,000,000
7	Diclofenac Sodium tab	OA, RA	2,400,000	406,177,392
8	Nabumetone	RA	4,200,000	22,000,000
		<b>Total Sales in US\$</b>	<b>\$62 million</b>	<b>\$694 million</b>

\* Source: IMS



# ENMD-2076 Update (1)

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- *Clear Cell Ovarian Carcinoma (CCOC)*
  - Phase 2 data presented at the 2017 Annual Meeting of the American Society of Clinical Oncology. 1/38 patients responded, several patients with protracted stable disease
  - *Biomarker studies ongoing*
- *Soft Tissue Sarcoma (STS).*
  - Phase 2 data presented at the 2015 Annual Meeting of the Connective Tissue Oncology Society. 2/23 patients responded, several patients with protracted stable disease
- *Triple Negative Breast Cancer (TNBC).*
  - Phase 2 data presented at the 2017 San Antonio Breast Cancer Symposium. 2/36 patients responded, several patients with protracted stable disease
- Based on the clinical data, CASI has determined it will **not** pursue company sponsored development of single agent ENMD-2076 in CCOC, STS or TNBC

## ENMD-2076 Update (2)

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- *Fibrolamellar Carcinoma (FLC)*. The accrual to this ongoing Phase 2 study is complete and is in data clean up and analysis phase. It is anticipated that the data from this study will be submitted to a medical conference for presentation in 2018.
- Phase 1/2 study in patients with TNBC in China. The dose escalation component has been completed and data analysis is ongoing.

# Summary

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- **On track with a Diversified product portfolio:**
  - ***In-licensed FDA approved products for greater China from Spectrum Pharmaceuticals***
    - EVOMELA® (CE-Melphalan), granted priority review by China Food and Drug Administration (CFDA)
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  - ***Acquired from Sandoz Inc.*** 25 U.S. FDA-approved ANDAs, including *Entecavir*, *Bisoprolol Fumarate*, *Desvenlafaxine SR*, *Aripiprazole* and *Cilostazol* – across therapeutic areas
- **Active business development activities to further expand product portfolio**
- **Well-capitalized:** Strong financial position, backed by successful venture funds; under careful financial management with low burn rate

Thank you



NASDAQ: CASI

[www.casipharmaceuticals.com](http://www.casipharmaceuticals.com)

