



Corporate Presentation

H.C. Wainwright Global Life Sciences Conference

Monte Carlo, Monaco

April 10, 2018

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.

Investment Highlights

- **Management:** Experienced team with accomplished track record in North America and China
- **Diversified product portfolio:**
 - ***In-licensed from Spectrum Pharmaceuticals*** EVOMELA[®] (CE-Melphalan), MARQIBO[®] (vinCRIS^tine sulfate LIPOSOME), ZEVALIN[®] (ibritumomab tiuxetan); EVOMELA[®] granted priority review by China Food and Drug Administration (CFDA), MARQIBO[®] and ZEVALIN[®] CFDA review in progress
 - ***Acquired from Sandoz Inc.*** 25 US FDA-approved ANDAs, including Entecavir, Ondansetron, Methimazole, and Repaglinide
 - ***Internally developed*** ENMD-2076 - Phase 2 small molecule multi-kinase inhibitor targeting triple-negative breast cancer: enrollment complete; biomarker/genomic analysis ongoing
- **Compelling business model:** Capitalizing on critical U.S./China synergies & competitive advantages for efficient/cost-effective drug development and commercialization
 - Beijing-based 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

- 1.4 billion people in China + 325 million people in the US 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

Bi-Cultural Board & Management Team

BOARD OF DIRECTORS

Dr. Wei-Wu He, 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 Ex-Chairman & CEO, Spectrum Pharmaceuticals, Inc.

CEO, Crown Biosciences, Inc.

President, National Foundation for Cancer Research

SENIOR MANAGEMENT

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

Alexander A. Zukiwski, M.D.

Cynthia W. Hu, J.D.

Jim Goldschmidt, Ph.D.

Sara B. Capitelli

Director and Chief Executive Officer

Chief Medical Officer

Chief Operating Officer, General Counsel & Secretary

SVP, Business Development

VP, Finance, Principal Accounting Officer

Scientific/Clinical Advisors

Dr. Tak W. Mak, Ph.D. Lead Scientific Advisor

Dr. Mak served as a Director of the Company from February 2012 – May 2016. Dr. Mak is currently the Director of the Campbell Family Institute for Breast Cancer Research at the Princess Margaret Hospital and a University Professor in the Department of Medical Biophysics and Department of Immunology, University of Toronto. Dr. Mak's career includes serving as VP of Research at Amgen, Inc. and Director of the Amgen Institute in Toronto from 1993-2002. He is best known as the leading scientist of the group that first cloned the genes of the human T cell antigen receptor.

Ghassan K. Abou-Alfa, M.D.

Dr. Abou-Alfa is Associate Professor of the Gastrointestinal Oncology Service at Memorial Sloan-Kettering Cancer Center and Weill Cornell Medical College in New York. Dr. Abou-Alfa specializes in the treatment of gastrointestinal malignancies and in particular, hepatocellular carcinoma, fibrolamellar carcinoma, and biliary cancers. Dr. Abou-Alfa serves as the chair of the National Cancer Institute (NCI) Task Force for Hepatobiliary Cancers and the chair of the AIDS Malignancy Consortium (AMC) Non-AIDS Defining Malignancies (NADC) Liver/GI Task Force.

S. Gail Eckhardt, M.D.

Dr. Eckhardt, MD, is a visionary cancer leader, educator and research innovator, and is the inaugural director of the LIVESTRONG Cancer Institutes of the Dell Medical School. She is overseeing the creation of a transdisciplinary cancer research program at UT Austin, one that will lead to new models of prevention, treatment, patient-centered cancer care; and new models of teaching and training future doctors. Prior to joining the LIVESTRONG Cancer Institutes of the Dell Medical School, Dr. Eckhardt was a professor in the Division of Medical Oncology at the University of Colorado School of Medicine where she held the Stapp/Harlow Endowed Chair for Cancer Research. Dr. Eckhardt has served on numerous committees/study sections, including the ASCO Molecular Oncology Task Force, the ASCO Board of Directors, the FDA Oncology Drugs Advisory Committee, and the NCI Cancer Centers Study Section. She is a member of the NCI Investigational Drug Steering Committee and the NCI Colorectal Cancer Task Force.

Robert J. Mayer, M.D.

Dr. Mayer is Faculty Vice President for Academic Affairs at the Dana-Farber Cancer Institute, Senior Physician at the Brigham and Women's Hospital, and the Stephen B. Kay Professor of Medicine at the Harvard Medical School where he is also the Faculty Associate Dean for Admissions. He directed the Institute's Medical Oncology Fellowship Program for 36 years. Dr. Mayer established the Center for Gastrointestinal Oncology at the Dana-Farber Cancer Institute and is the past Chair of the Gastrointestinal Cancer Committee of the Cancer and Leukemia Group B. He has served as an Associate Editor for the New England Journal of Medicine and the Journal of Clinical Oncology, and is a past President of the American Society of Clinical Oncology.

Diversified Product Portfolio

Integral Player in China Well-Positioned for Rapid Growth

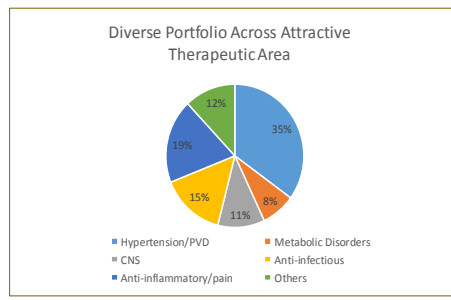
Greater China Rights

Acquired From SPPI



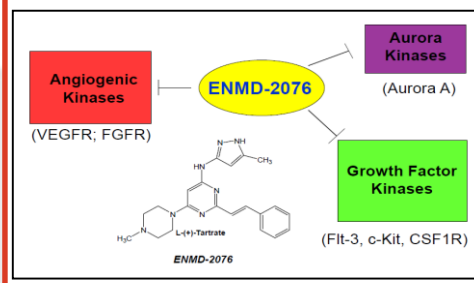
FDA ANDAs

Acquired From Sandoz
25 FDA Approved ANDAs
4 FDA Pending ANDAs



ENMD-2076, Global Rights

Innovative Drug Candidate
Target Therapy for Solid Tumors



Licensed Products

- CASI entered into licensing agreement with Spectrum Pharmaceuticals (Nasdaq: SPPI) for China development & commercialization of hematological oncology products
- **Exclusive rights to three commercial products:**
 - **EVOMELA®** *Conditioning treatment prior to autologous stem cell transplant for patients with multiple myeloma; **PRIORITY REVIEW: Advisory committee meeting set for Apr 25***
 - **MARQIBO®** *Treatment of acute lymphoblastic leukemia; **CFDA review in progress***
 - **ZEVALIN®** *Treatment of non-Hodgkin's Lymphoma; **CFDA review in progress***
- **CASI responsible for development and commercialization in China, including Taiwan, Hong Kong, and Macau**
 - Import drug registration applications
 - Confirmatory clinical studies
 - Premarketing and commercial launches

- **Status: CFDA priority review; Advisory Committee meeting set for Apr 25-26**
- 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 AEs)*
 - *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 \$35m, assuming $\geq 10\%$ self-pay**

*<http://globocan.iarc.fr/Pages/online.aspx>

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

[International Agency for Research on Cancer](#)



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 in China estimated at 75k cases with mortality of ~53k cases in 2015**
 - **Annual peak sales estimated > \$49m, assuming \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

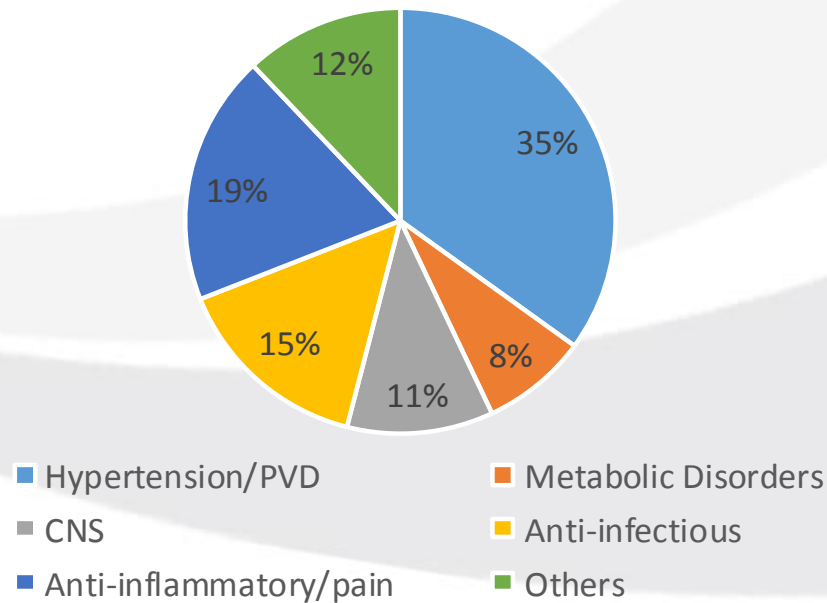
**Chen W, Zheng R, Baade PD, Zhang S, Zeng H, Bray F, Jemal A, Yu XQ, He J. Cancer statistics in China, 2015. CA Cancer J Clin. 2016 Mar-Apr;66(2):115-132.

- ***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
- 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, assuming ≥ 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.

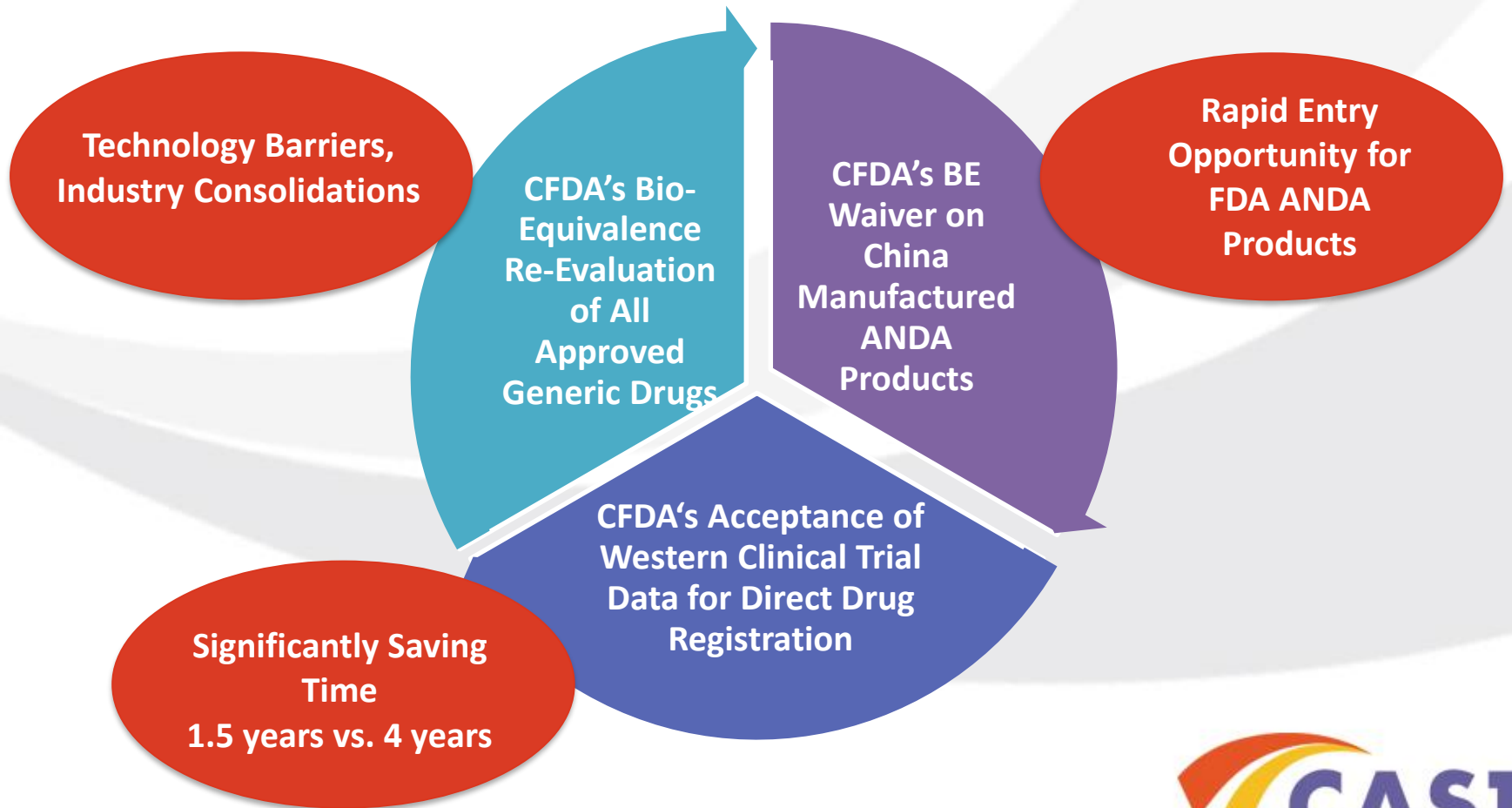
25 FDA Approved, 4 Pending FDA Approval ANDAs

Diverse Portfolio Across Attractive
Therapeutic Area



Historical Opportunity for FDA-Approved ANDAs in China

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



China Market for 6 Key ANDA Products

Key Products by 2016 (Estimated) Generic Sales in China

	Product	Indication	2016E Sales (in US\$)
1	Entecavir tablet	HBV Infection	1,490,000,000
2	Repaglinide tablet	Diabetes	225,000,000
3	Bisoprolol Fumarate tablet	Hypertension	205,000,000
4	Cilostazol tablet	peripheral vascular disease	65,000,000
5	Methimazole tablet	Hyperthyroidism	29,000,000
6	Tizanidine tablet	Muscular Relaxant	20,895,000
		Total Estimated Sales	US \$2.2 billion

* Source: China's PDB Database

U.S. Market for 8 Key ANDA Products

Key Products by 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
		Total Sales in US\$	\$62 million	\$694 million

* Source: IMS

China Regulatory Environment

China Regulatory Environment – An Evolving Landscape

Historic challenges:

- Issues with comparative quality (international standards vs local products & manufacturers)
- Longer timeframe for review & new drug approval
- Lack of capacity in regulatory bodies / application backlog

China State Council issued Reforms* in 2015 to bring marketed products in line with international standards (e.g. efficacy, safety, quality).

Objectives:

- Eliminate existing backlog of registration applications
- Establish environment for maximizing generic drug quality
- Create framework in China encouraging new drug R&D in line with global development
- Improve quality and transparency of review and approval process

* *Opinions on Reforming Review and Approval System for Drugs & Medical Devices*

China Regulatory Environment – An Evolving Landscape

CFDA published “restricted” and “promoted” categories of generic drugs, signaling move toward **more logical control of generics industry**

- Historically **strong competition** in generics area: local companies have been unmotivated to invest in innovative new drugs
- Traditionally, generics cos relied on **bioequivalence (BE) trials** for generic drug registration. These **companies now face CFDA’s new requirements** for generic drug quality and efficacy consistency evaluation
- Previously; marketing authorization holder (MAH) had to be owner of drug manufacturing plant; NOW separate; creates more flexible, modern framework in which **companies can be MAH and contract manufacture to another party**

Sources:

Nature – Sept 2017, Volume 14: Zhou et al

Regulatory Rapporteur –July/August 2017, Vol 14, No 7/8: Bill Wang, Alistair Davidson

China Regulatory Environment – An Evolving Landscape

Recent Reforms & Implications:

- **Shortened IND / NDA review timelines:** Increased human capacity at CDE resolving current backlog; **goal is for IND/CTA timelines to be ~six months by YE2018** with zero backlog
- **Increasing transparency and globalization:** CFDA encouraging foreign sponsors to undertake global studies in China and recommending local clinical sites join global studies
- **Stronger quality controls:** Beneficial to new drug innovators planning to conduct meaningful scientific trials
- **Anticipated re-shuffling of key players: 90% of 1,600+ applications withdrawn or rejected in 2016.** Local CROs now challenged to enhance quality; expected budget for BE study increased tenfold. Sponsors motivated to use reliable CROs with established standards
- **Minimizing drug lag:** Foreign drug registration process changed from “3-submission-3-approval” to “2-submission-2- approval”; enabling drug approval in China in parallel with the US / EU.

Sources:

Nature – Sept 2017, Volume 14: Zhou et al

Regulatory Rapporteur –July/August 2017, Vol 14, No 7/8: Bill Wang, Alistair Davidson

China Regulatory Environment – Immediate Impact of Reforms

- **Self-inspection:** Applicants, CROs & clinical sites required to self-inspect registration applications
- **Priority review:** Target approval time from submission **within six months**
- **Additional CDE capacity:** From ~70 reviewers handling 7k+ drug applications in 2015 to 600+ by YE 2016 and growing
- **Rationalization of MAH system & new classification/definition of drugs:**
 - Local drug innovators can hold **marketing authorization independently**; encourages R&D focus
 - **New classification/definition of new drugs – more globally aligned**
 - **Generic drug quality and efficacy consistency:** Requires generic drug manufacturers to conduct drug consistency research.
- **Opening of first-in-human (FIH) Phase I trials to global devt:** Foreign applicants now have full clinical development plan inside China, starting from FIH to POC
- **Simplified process:** Sponsor now has 2 submissions (v. 3): shortens approval process by **ONE YEAR+**
- **No need for CPP for NDA/MAA submission:** China NDA/MAA submission & approval can now be in parallel with (or earlier than) foreign MAA approval

Appendix

China Market for EVOMELA[®], ZEVALIN[®], MARQIBO[®]

- **Strong IP position, greater China exclusivity, significant technology barriers**
- **Minimal clinical, regulatory, financial risks:** FDA approved products sold in U.S.
- Product value increases incrementally as applications get closer to marketing approval, **sustained growth anticipated** after market entry
- **Addresses unmet medical needs:** Superior safety and efficacy benefits compared to existing products; CFDA's priority review status, *i.e. Evomela*, could potentially lead to fast track approval



Collectively, \$148 million annual peak sales are forecast

Market for Entecavir

- Hepatitis B virus (HBV) infection remains severe public health problem worldwide; ~2b people currently infected, ~360m suffer from chronic infections; ~600k deaths annually from HBV-related liver disease or hepatocellular carcinoma.*
- In China, ~ 120m hepatitis B surface antigen (HBsAg) carriers, 20m suffering from chronic hepatitis B, and ~ 300k deaths / year caused by HBV-related infections.*
- Entecavir is first line therapy for HBV infection; average cost of treatment is ~\$800 dollars / patient in China
- **~\$16 b market potential for ~20m patients**

* <https://www.nature.com/articles/srep36186>

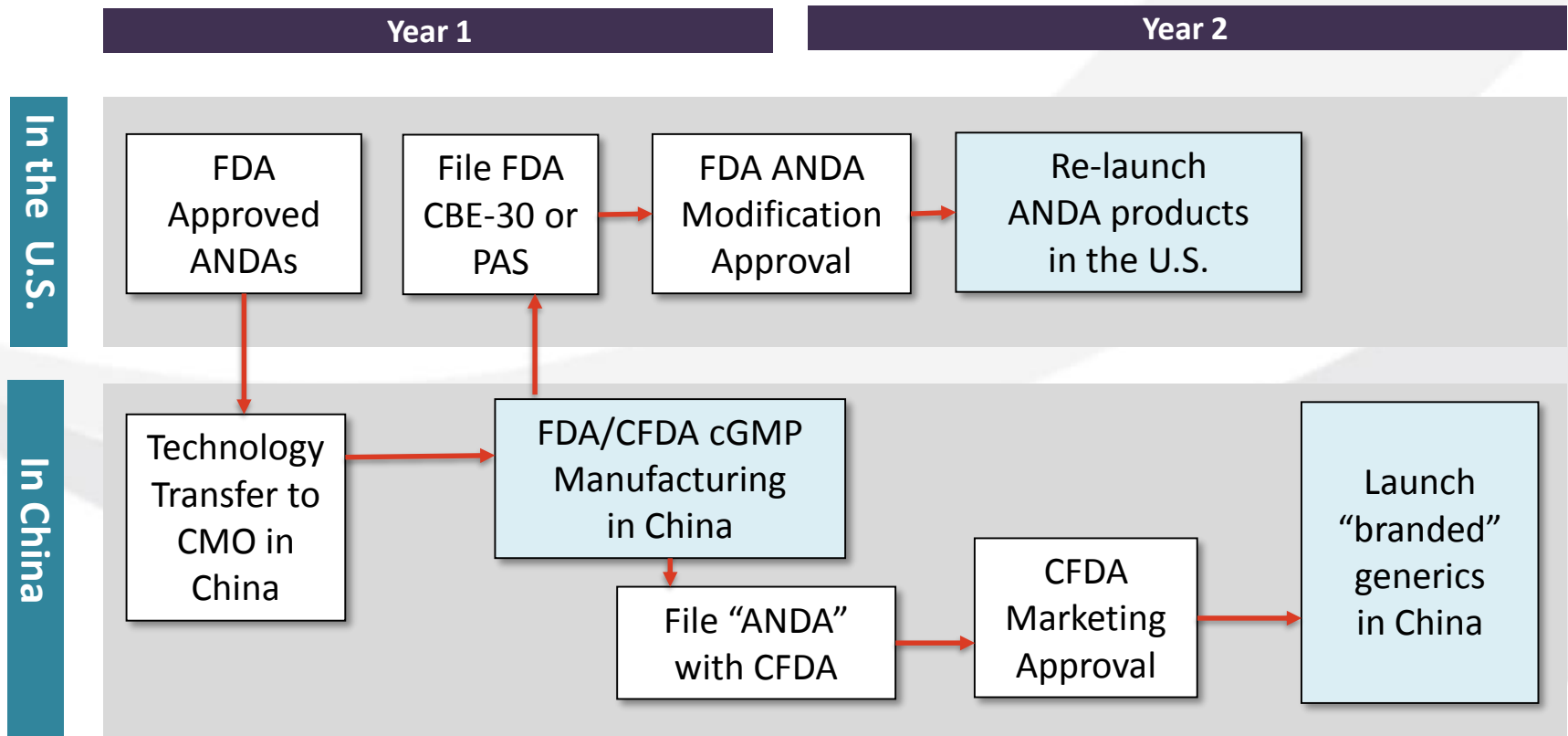
Pharmaceutical Reimbursement in China

- Self Pay
 - Innovative premium priced products (EVOMELA, ZEVALIN, MARQIBO)
 - Supported by expanding middle class (2017 estimate of ~225m)
- National/Provincial government programs
 - National Reimbursement Drug List (NRDL) expanded in 02/17
 - List A: Widely used drugs, essential drugs list (EDL) plus innovative products
 - List B: Administered by provincial authorities (PRDL), tailored to meet local needs. 2017 additions included trastuzumab, rituximab, etc. Reimbursement may not be at 100%
- Private Insurance
 - Supplemental coverage, early stage
- Near universal coverage via major programs
 - *Urban Employee Basic Medical Insurance*
 - *Urban Resident Basic Medical Insurance*
 - *New Cooperative Rural Medical Scheme*

Source: 1. Southern Medicine Economic Research Institute, 2 FiercePharma <https://www.fiercepharma.com/pharma-asia/china-updates-basic-medical-insurance-drug-list-adds-133-western-style-meds>, 3. Int Soc Pharmacoeconomics Outcomes, <https://www.ispor.org/HTARoadMaps/China.asp>

ANDA Development Strategy and Plan

Self-Development and Partnering with Local Players



Phase I Sites in China

- Selection of phase 1 study sites based on established phase 1 unit, history of participating in global development programs, recognized KOL, institutional influence with CFDA
 - *Cancer Hospital Chinese Academy of Medical Science*
 - Beijing
 - *Beijing Cancer Hospital*
 - Beijing
 - *Fudan University Shanghai Cancer Center*
 - Shanghai

CASI's Network of CMO/GMP Facilities in China

- **CMOs for DS/DP manufacturing**

- Zhejiang Jiuzhou Pharmaceutical Co., Ltd.
 - CFDA, USFDA, TGA, PMDA inspected
- Asymchem Inc.
 - CFDA, USFDA, TGA inspected
- JHL Biotech Inc.
 - CFDA, USFDA, TGA, PMDA inspected
- WuXi AppTec
 - CFDA, USFDA, TGA, PMDA inspected
- MabPlex International, Ltd.
 - Biologic drug R&D and Manufacturing service in full compliance with the U.S. and EU GMP requirements

- **CMO for Formulation manufacturing**

- North China Pharmaceutical Group Corp.
 - Formulation production line includes lyophilized powder for injection, powder for injection, small volume injection, eye drops, capsules, tablets, granules, and oral solutions

CASI's Network of CROs

- **Research/Biomarkers/Proteins/Assays**
 - Genetron Health (Beijing)
 - OriGene (Rockville)
- **Preclinical Pharmacology Efficacy Models and IND Toxicology**
 - Crown Bio (China and US)
 - Joinn-Lab (Beijing)
 - WuXi Apptec (Shanghai)
- **Clinical Trials/Data Management**
 - Target Health (NY)
 - R&G PharmaStudies (Beijing)
 - TigerMed (Beijing, Shanghai, Chongqing, and Guangzhou)

CASI's Clinical & Regulatory Team

Alex Zukiwski, MD – CMO, Medical Oncologist
Executive Oversight and US Point of Contact

Core Internal Team in China

- **Yolanda Zhang**, VP Operations for China and Project Leader. Responsible for overall project management, project coordination, resources allocation, quality, timeline, and budget
- **Cici Xu**, Director, Regulatory Affairs - responsible for CTA filing/approval with CFDA
- **Danyl Dang**, Specialist, Medical & Reg - responsible for dossier preparation
- **Bruce Jiao**, Senior Manager - responsible for CMC
- **Vivian Shi**, Manager for Clinical Research - responsible for Clinical Trial Operations
 - Clinical Trial Team:
 - Ying Liu, PM
 - Nancy Kang, CRA
 - Megan Wu, CRA
 - Vicky Lu, CRA
- **Marry Ma**, Senior Manager Clinical QC
- **Hellen Luo**, Manager, Med Monitor - responsible for Medical Monitoring

Total Beijing Clin/Reg Staff of 20

R&G PharmaStudies, Co. Ltd.

CASI's local CRO partner for clinical & regulatory services (2nd largest CRO in China specializing in Oncology):

- Regulatory Team: 40+ staff
- Clinical and Medical affairs for monitoring, trial coordination and project management: 600+ staff
- BioStat & Data Management: 50+ staff
- Senior Management: 27
- A total of 800 full-time staff