Citrine Medicine

Building the first rare disease ecosystem in China

January 2021

Imagining new futures for people living with rare diseases

Our Mission

To provide individuals and families in China affected by rare diseases with access to the best, most effective medicines and treatment protocols available in the world.

Our Approach

Building the first rare disease ecosystem in China to bring a support network and much-needed treatments to individuals and their families affected by rare disease.

Vision

To become the leading company supporting the rare disease community in China.

Executive summary: Building the first rare disease ecosystem in China

Unmet need

- More than 800 rare disease drugs have been approved by the U.S. Food and Drug Administration
- Less than 20 percent of these are available in China
- 20 million people in China suffer from rare diseases

High impact portfolio

- In-licensing clinically-validated assets developed in the U.S. and EU for the Chinese market
- WAKIX® (pitolisant) is a narcolepsy treatment that Citrine has in-licensed from the French Biotech Bioprojet for use in China
- Therapeutic focus on RARE neurology, hepatology/nephrology, endocrine/metabolic, and pediatric oncology

Integrated care platform for rare disease patients in China

Building an integrated platform that can support scalable development and commercialization of Rare Disease Drugs by:

- Leveraging digital platforms to improve diagnosis and treatment
- Creating access to rare disease drugs through creative distribution systems
- Improving reimbursement and payment systems
- KOL engagement to drive better outcomes and expedited regulatory approvals for rare disease patients

Strategic funding overview

- $80 Million Series A completed in July 2020
- Strong cross-border syndicate including Vivo Capital, F-Prime Capital, Eight Roads, Quan, 3H Health, Wu Capital

Who is Citrine?

Strong, cross-border syndicate

$80 Million
Series A
completed
in July 2020

Leadership team with deep rare and orphan disease expertise

A best-in-China rare disease focused team with expertise across rare and orphan diseases, business development, regulatory affairs, and product development and commercialization.

Citrine has offices in Shanghai, Beijing, Hong Kong, Boston, and Zurich.

Since its establishment in 2019, Citrine has established a strong executive team with rich pharma experiences including:

Abbott, FDA, McKinsey & Company, abbvie, IQVIA, SANOFI, gsk, Lilly

Since the announcement of its first licensed-in product, Citrine is in negotiation with many other top multi-national companies for partnership opportunities in rare diseases in China.

Citrine has a team of ~30 people and is quickly expanding.

Citrine Strategy

The rare disease community in China:

High unmet need for physician education, therapeutics, and patient support networks

20M+ People

affected by rare disease in China

121 rare diseases

designated by Chinese government as high priority

3M Chinese

rare disease patients affected on this list

800+ rare disease drugs

approved by the U.S. Food and Drug Administration

Less than 20%

of these are available to Chinese patients

The market opportunity: Chinese regulatory changes enabling rapid approval of rare disease drugs

YEARS PAST

Multiple Barriers to entry

AVAILABILITY

Complex regulatory requirements for rare disease drug approval

▼

HIGH COST OF ENTRY

▼

Few drugs available

ACCESSIBILITY

Lack of infrastructure

▼

HIGH COST TO ENABLE ACCESS

▼

Few patients treated

Very few multinationals were willing to invest in RD due to ***poor return on investments***

PRESENT (2017-2020)

The time has come and the market calls for execution

中华人民共和国国家卫生健康委员会
National Health Commission of the People´s Republic of China

- National Expert Committee
- National Rare Disease Registry (NRDRS)

国家药品监督管理局
National Medical Products Administration

- National Rare Disease List
- Urgently Needed Drug List
- Priority Review / Conditional Approval

国家医疗保障局
National Healthcare Security Administration

- National roll-out of “4+7” volume based procurement to give room for effective specialty drugs
- First Orphan Drugs added to 2019 NRDL
- Orphan Drugs in National Reimbursement List (NRDL) 2020.

Our strategy is to build the first rare disease ecosystem in China

Citrine is building an integrated platform to support the scalable development and commercialization of rare disease drugs in China

- In-licensing rare and orphan disease drugs to China
- Leveraging China's expedited regulatory pathways for rapid approval
- Engaging physicians and the rare disease community to create greater awareness
- Developing digital platform technologies to accelerate patient identification, diagnosis, and treatment
- Creating affordable solutions for rare disease therapies
- Advancing clinical trials for U.S. and European global clinical studies
- Developing rare disease research and development platforms in China and the US

Building the first rare disease ecosystem in China

Market Readiness: From Research & Development to Regulatory Approval

Identify potential patients

Genetic testing

Digital platforms

Advocacy networks

Collect and evaluate clinical data

Global clinical study participation

NMPA local studies

Named patient programs

Evaluate and share data

Rare Disease Waiver

Faster rare disease trials

Regulatory approval

China

US

EU

Building the first rare disease ecosystem in China

Commercializing Rare Disease Treatments

Educate about unmet need

Disease education

KOL support

Patient advocacy support

National guidelines

Reimbursement

Value dossier

Budget burden report

National Drug Reimbursement Listing

Commercial platform

Direct sales force effort

Top hospital targeting

Patient treatment

Broad access

Affordability

Patient disease management

Building the first rare disease ecosystem in China

The patient journey: Treatment initiation & monitoring

App & call center

Citrine patient Solution platform

Financial

PAP

OOP assistant

Insurance, charities, etc.

Distribution

Distribution logistics

Coordinator for clinical trials

Adherence

Adherence program

Training & education

Clinical monitoring

Safety reporting

Outcome Monitoring

Treatment outcome monitoring

Coordinator for clinical trials

Real world data collection

WAKIX ® for narcolepsy: First in-licensed therapy

Urgent unmet need for narcolepsy treatments in China

No approved drugs for narcolepsy

Need for safer pediatric options

- Existing options are inadequate: Stimulants (cFDA scheduled), antidepressants (suicidal black box for peds)

Key opinion leader interest in better treatment options

Market size

~200K narcolepsy patients in US

~200K narcolepsy patients in EU

Potentially 1 million narcolepsy patients in China

WAKIX (Pitolisant): "First-in-class" novel mechanism H3 receptor antagonist

Excessive daytime sleepiness (EDS) in treatment of adult narcolepsy patients with or without cataplexy.

Potential for label expansion including:

- Pediatric dosing in children for treatment of narcolepsy with or without cataplexy
- Excessive Daytime Sleepiness associated with Obstructive Sleep Apnea

Daily, oral tablet formulation

Efficacy and safety

Compelling clinical data demonstrating dramatic and durable response

Transient, mild side effects (i.e. insomnia, nausea and anxiety)

De-risked, as approved for use in narcolepsy by the FDA and EMA

WAKIX® for narcolepsy: Go-to-market Strategy

Position narcolepsy as a rare disease

Shape the market with concentrated launch

Facilitate national listing to quickly and efficiently scale up WAKIX® business

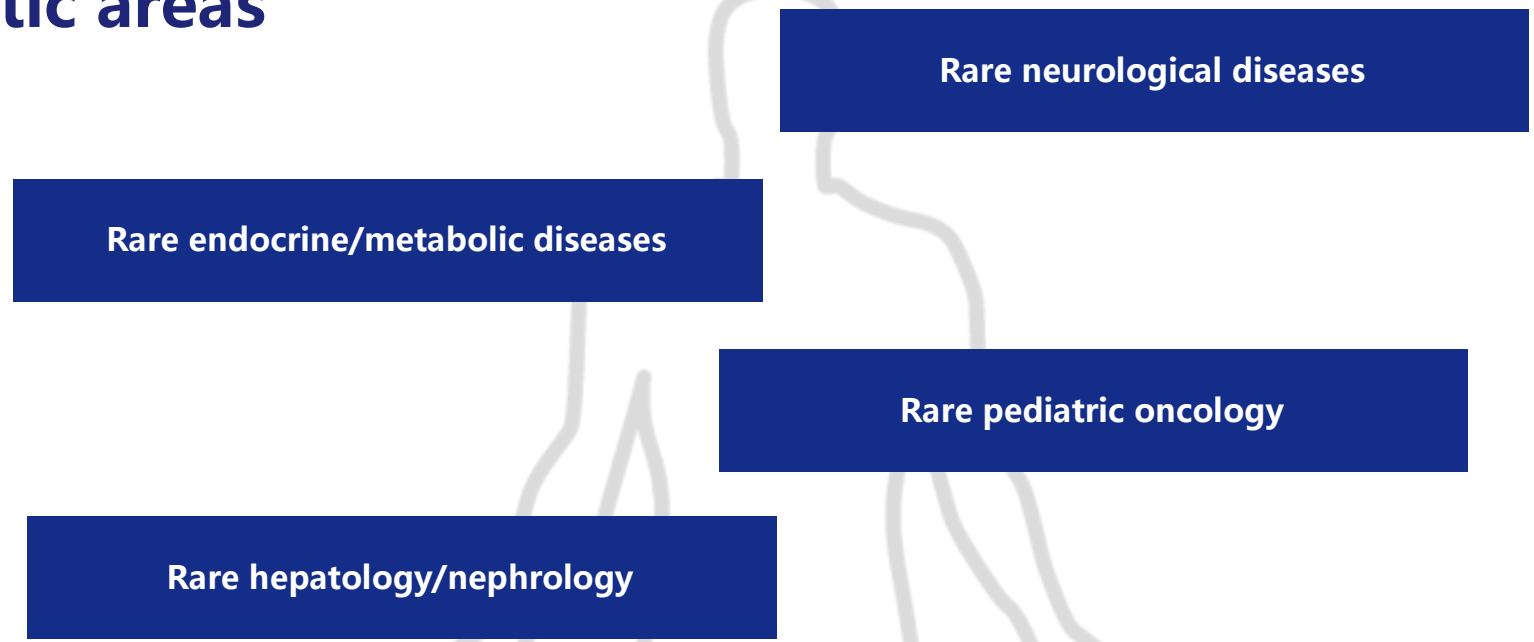
WAKIX ®
DEVELOPMENT TIMELINE

Q2 2021
NDA filing

Q2 2022
Approval

Q3 2022
Launch

Expanding to multiple therapeutic areas



Very few multinationals were willing to invest in RD due to ***poor return on investments***

Partnering with leading biopharma companies in the U.S. and Europe to develop & Commercialize rare and orphan drugs in China

Our people

Our ecosystem

Our platform

CITRINE

EXPERTISE

INFRASTRUCTURE

QUALITY/COMPLIANCE

INVESTMENT

Why partner with Citrine

Why is Citrine the partner of choice in China for global rare disease companies?

1 Speed to market for new ***drugs***. Our **elite development and commercial team** is equipped with best-in-class clinical dev and launch know-how specifically for rare disease drugs.

2 Finding the right ***patients***. Our **ecosystem** allows us to already understand where they are and even who they are.

3 Covering the right ***physicians***. Our **commercialization platform** affords a sizeable rare disease sales force to well cover top centers all over the country.


Thank
You