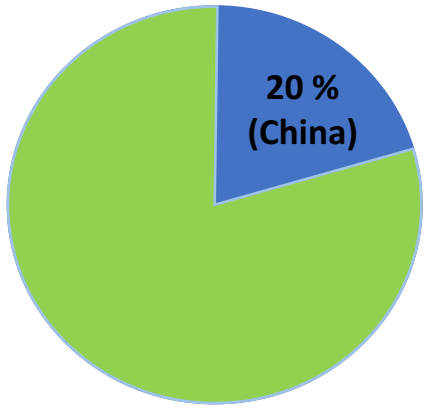
The Need for Expedited Approval: Policy Recommendation for Oncology and Orphan Drugs in China

Shuang Chen
The Dartmouth Institute
May 30, 2017

Policy Systematic Review

Cancer in China and the United States

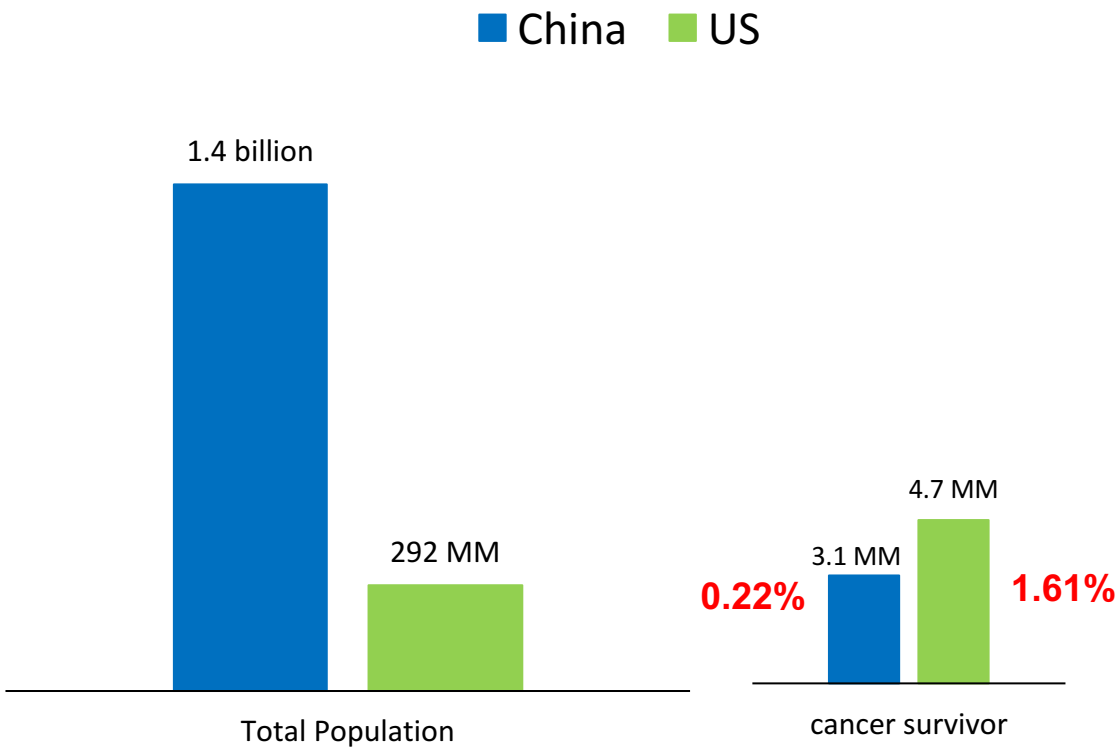


World New Cancer Cases in 2015

China had **4.3 million** new cancer cases in 2015

Cancer is a leading cause of death, it is responsible for **28%** of deaths in China

3.1 million people living with cancer in China vs US has **4.7 million** cancer survivors



Rare Diseases in China and the United States

A **rare disease** is any disease that affects a small percentage of the population.

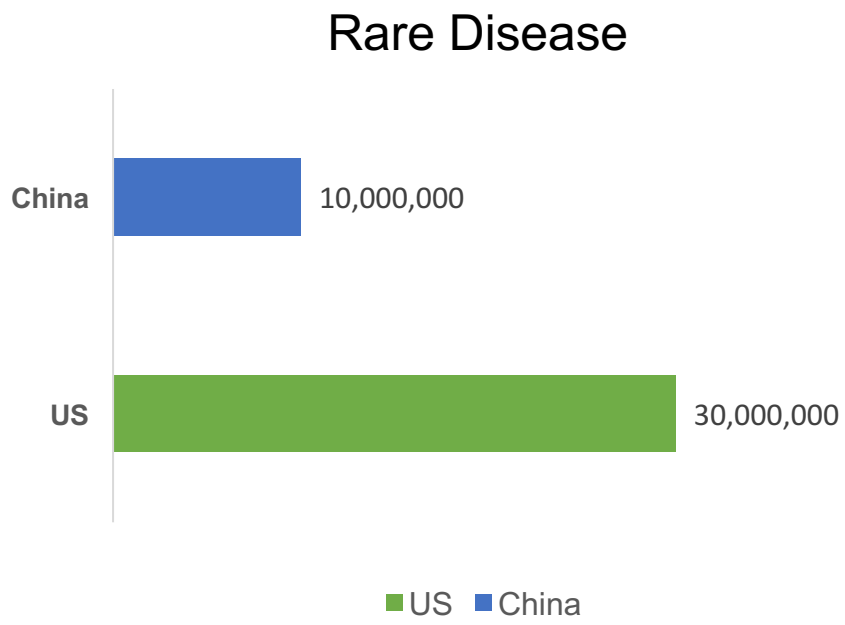
In the US, rare disease refers to disease affect patient population smaller than 200,000 people.

In China, a disease can be classified as a rare disease if it is prevalent in fewer than 1/500,000.

Orphan drug refers to the drug treating rare disease

There are **25-30 million** patients with rare diseases in the United States

There are more than **10 million** patients with rare diseases in China *(underestimation)*



“Drug backlog”

Top 10 most sold cancer drugs worldwide			
Drug Name	**First Approval in US**	**First Approval in China**	**Delay Time(years)**
Rituximab	1997	2001	**4**
Bevacizumab	2004	2010	**6**
Trastuzumab	1998	2002	**4**
Imatinib	2001	2001	**0**
Pegfilgrastim	2002	Not Available in China	-
Lenalidomide	2005	Not Available in China	-
Pemetrexed	2004	2005	**1**
Bortezomib	2003	Not Available in China	-
Cetuximab	2004	2006	**2**
Abiraterone	2011	2015	**4**

“ Innovative oncology drugs approved in the United States more than 5 years before they became available on the Chinese market ”

“ more than 21,000 drugs awaiting approval.”

Zhen Wu, Vice Minister of CFDA

Unmet demand

- In 2004-2014, **291** new drugs were approved by the FDA. In the same time, **183** new drugs were approved by CFDA.
- Of the 291 drugs approved in the United States, **27%** were approved in China.

Even though some drugs have been in the market for several years in the US, Europe and other developing countries, Chinese patients have no access to it due to the “drug backlog”.

Some patients have no choice and turn to unofficial channels and purchase drugs in the gray market.

Is this behavior illegal?

Dose the quality of these drug is assured?

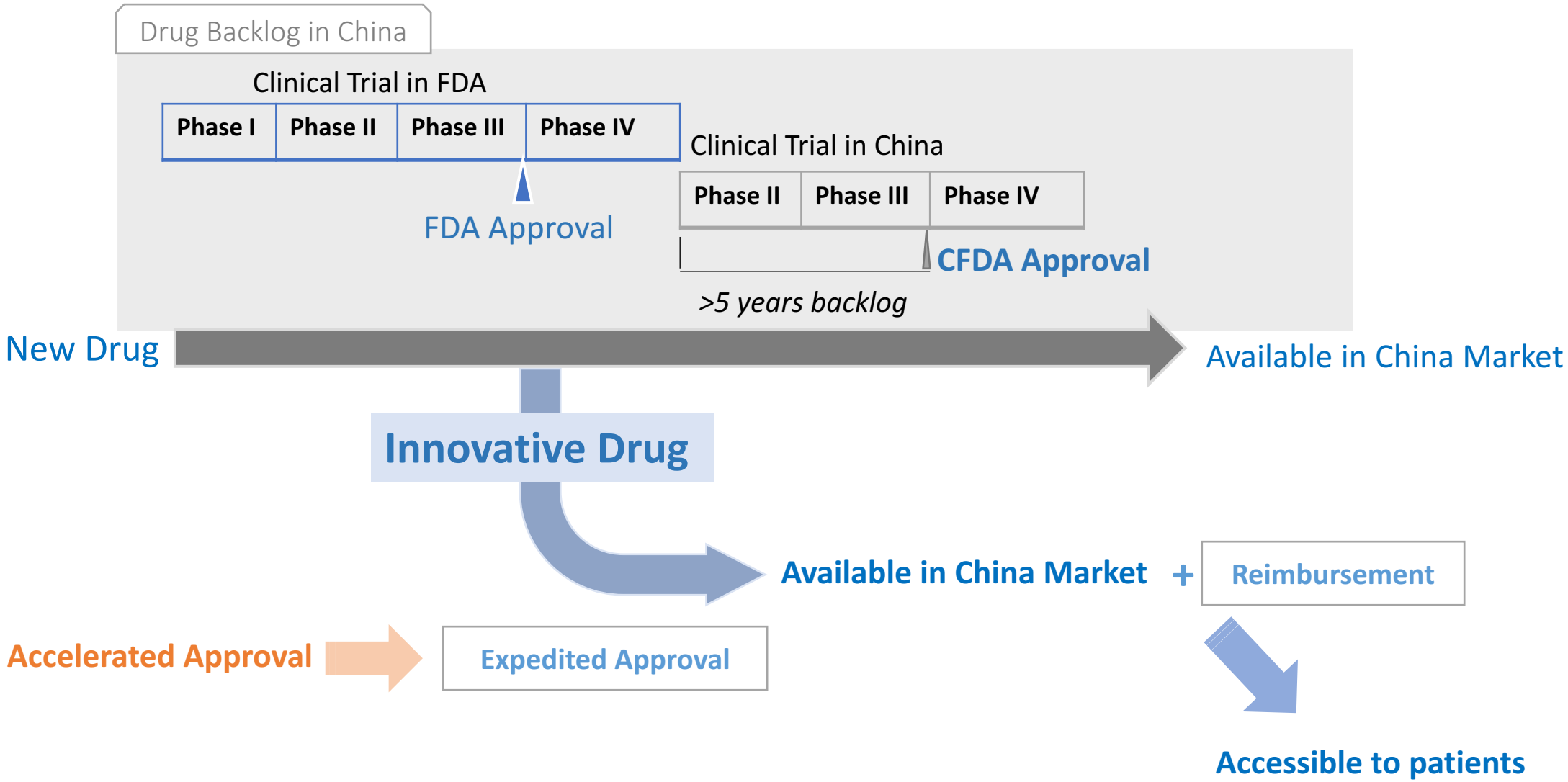
This social issue related to drug backlog is of great concern to the whole society in China.

Policy Question

- How to respond to the urgent demand of patients with a life-threatening disease?
- How to solve the problem of “drug backlog”?

Should CFDA introduce regulations to speed up the approval of oncology and orphan drugs?

Conceptual Model



* CFDA: China Food and Drug Administration

Inclusion & Exclusion Criteria

Inclusion Criteria

1. Mainly discussed about mechanism or characteristics of expedited approval programs (including Fast Track, Accelerated Approval, Priority Review, Breakthrough Therapy).

2. The type of article could be primary studies, opinion, commentary, review, report or official documents etc.

3. The approved drug discussed in articles should be oncology drugs or orphan drugs, or drugs treating rare disease.

4. The approval process should be in Food and Drug Agency in United State.

Exclusion Criteria

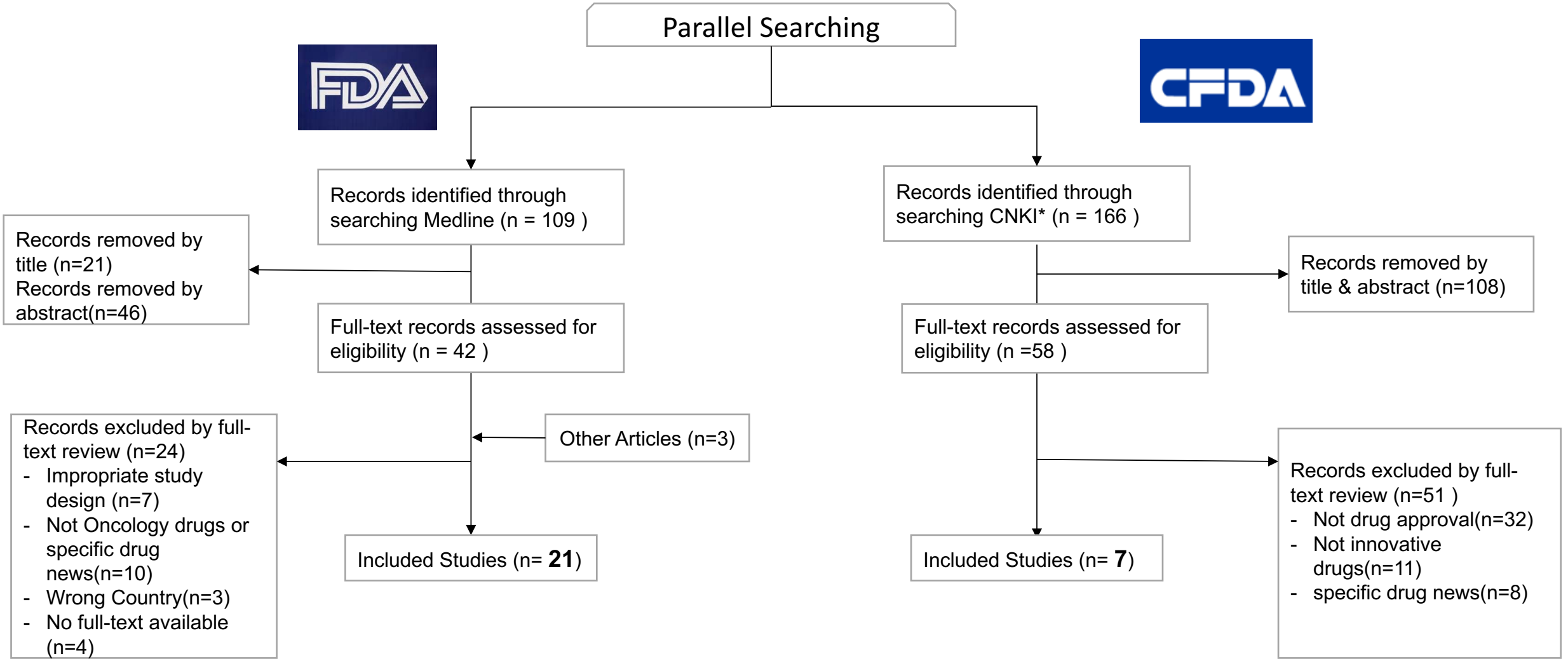
1. Studies mainly talked about approval process in EU, Japan, or any other countries

2. The approved drug is treating HIV or other diseases even they are under the expedited approval programs.

3. It is an official FDA drug approval summary for one specific drug. (Even it contains original and reliable data for the drug, which might be utilized in the future).

4. It is a short announcement to announce one specific drug gain approval or enter the market.

Parallel Searching



* CNKI= China Knowledge Resource Integrated Database. http://gb.oversea.cnki.net/kns55/

Evidence Results: 21 Included studies

- 4 studies focus on drugs treating rare disease, and 17 studies are discussing oncology drugs
- Included studies are diverse in the study design, including case study, cost-effectiveness analysis, commentary, opinion, review, report etc.
- 10 studies are deemed to be of good quality, 7 studies are considered as moderate, while 4 studies considered as poor quality
- 18 studies include disclosure of potential conflicts of interest

Synthesis and Discussion

Synthesis of Study Focus

- 15 studies focus on analyzing expedited approval process itself
- 3 studies hold positive attitude
- 2 studies remain skeptical
- 1 vigorously opposes this regulation

Findings

- Using surrogate endpoint
- Clinical trial design: single-arm, uncontrolled study + Only 1 clinical trial
- Identify subgroup who have higher response
- Post-marketing studies
- Communication mechanism between sponsors and agency
- Outside professional committee

Opportunity

- Government has highly encouraged innovation in drug Research and Development
- Multinational pharmaceutical corporations have motivation in developing oncology and orphan drug

How to translate the scientific advance into practice?

Improve in drug R&D → **Speed up the approval**

Recommendation

CFDA should introduce regulations to expedite access of oncology and orphan drugs immediately.

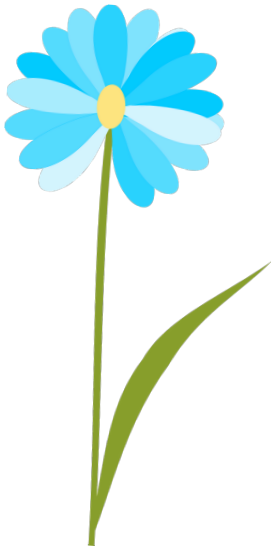
- ❑ CFDA should build a rationale to give guidance for drug sponsors;
- ❑ Develop a dialogue mechanism between sponsors and agency;
- ❑ Post-approval confirmatory studies need to be conducted carefully.

Acknowledgement

First and foremost, I would like to thank Professor Kristina Wolff for her guidance and persistent support through out this capstone, as well as my wonderful team members, Laura Chirite, Christopher Gerace and Juliette Kassas. It was a great pleasure to work with such brilliant and engaging people.

I would like to thank Professor Meghan Longacre, Peter Thurber and Aurora Drew for their good advice.

Thanks to my classmate and professors in TDI. You were always patient and willing to help me.



Q & A

Thank you!