Opportunities and Challenges in Clinical Research under China's Scientific Regulatory System: Focusing on Innovative Drug Development

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*The opinions presented are that of the speaker and not of CDE,NMPA

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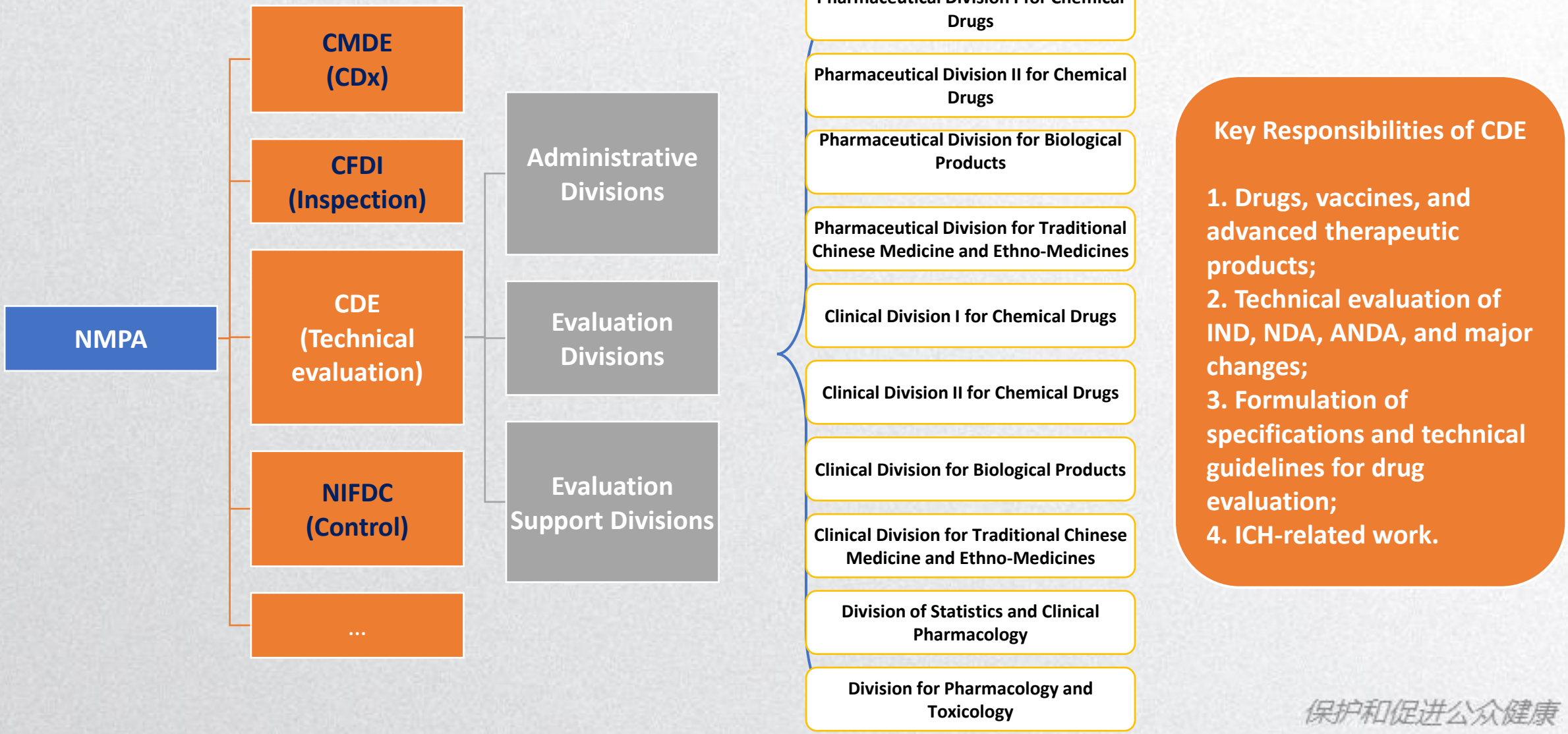
Outline

- Latest advancements in scientific regulatory system
- Navigating Challenges and Seizing Opportunities in a Changing Landscape
- Conclusion and outlook

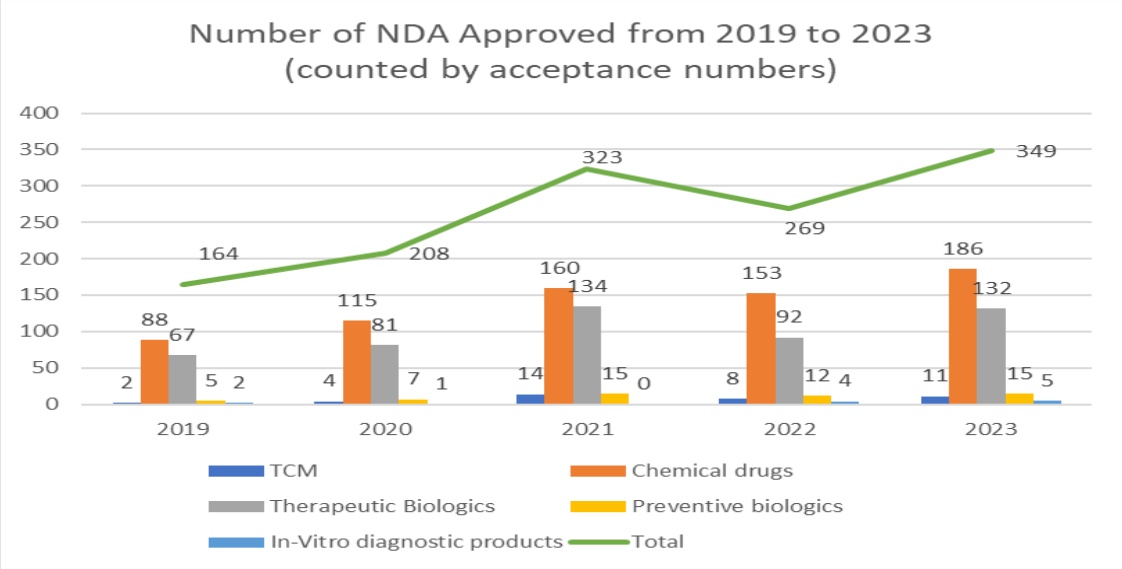
Organizational Framework of NMPA and CDE

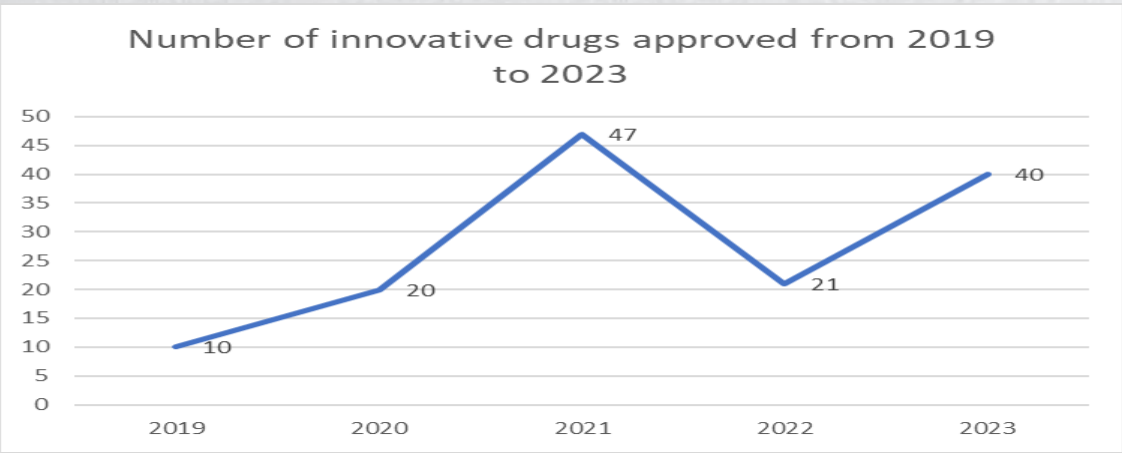


Key Responsibilities of CDE

1. Drugs, vaccines, and advanced therapeutic products;
2. Technical evaluation of IND, NDA, ANDA, and major changes;
3. Formulation of specifications and technical guidelines for drug evaluation;
4. ICH-related work.

The growing momentum of innovative drug development





Data extracted from the "Annual Drug Review Report" (2019-2023)

Number of drug clinical trials registered from 2020 to 2023

	2020	2021	2022	2023
Total number of clinical trials	2602	3358	3410	4000
Clinical trials of innovative drug N (%)	1473 (57.0%)	2033 (60.5%)	1974 (57.9%)	2323 (58%)
Sponsored by domestic pharmaceutical companies N (%)	1142 (78.0%)	1678 (78.9%)	3018 (88.5%)	3668 (91.7%)
Sponsored by foreign pharmaceutical companies N (%)	331 (22.0%)	355 (21.1%)	392 (11.5%)	332 (8.3%)
MRCTs of innovative drugs N (%)	208 (14.1%)	321 (15.8%)	292 (14.8%)	290 (6.7%)
Domestic clinical trials of innovative drugs N (%)	1265 (85.9%)	1712 (84.2%)	1677 (85.0%)	3988 (92.7%)
Phase I trials N (%)	643 (43.7%)	872 (42.9%)	848 (43.0%)	978 (42.1%)
Phase II trials N (%)	359 (24.4%)	410 (20.2%)	368 (18.6%)	440 (18.9%)
Phase III trials N (%)	293 (19.9%)	474 (23.3%)	402 (20.4%)	489 (21.1%)

Data extracted from the "Annual Report on the Current Status of Clinical Trials for New Drug Registration in China" (2020, 2021, 2022, 2023)

A Robust Regulatory System: the Key to Safe, Effective, and Timely Drug Innovation

Science based regulation

Regulatory decisions should be evidence-based, emphasizing the role of scientific data

Transparency in Regulation

Regulatory processes should be transparent to enhance public trust

Efficiency in the Regulatory Process

Streamlining the approval process to accelerate review timelines

A regulatory framework serves as the final safety net for drug safety and efficacy.

Revamping Drug Regulation: NMPA's Approach

Strengthening the legal framework

continuously improve legal provisions in areas such as pre-market drug review and approval procedures, supervision of drug production and distribution, and drug adverse reaction monitoring

Enhancing risk management

regulate the pharmacovigilance activities throughout the lifecycle of drugs

Improving the drug review and approval process

introduce a series of new policies and measures, such as the adoption of a nationwide marketing authorization holder system, implementing clinical trial Implicit approval and bioequivalence notification Systems, establishing systems for accelerating market launch to support clinical-value-oriented drug innovation etc

Leveraging technology for better oversight

establish a national drug electronic supervision platform to realize the traceability of the entire process of drug production, circulation and use, and improved supervision efficiency

Refining the regulatory requirements for key areas and special drugs

formulate more detailed regulations for drugs in specific fields or types such as anti-tumour drugs, advanced therapies, drugs for special populations, drugs for rare diseases, narcotic psychotropic drugs, radioactive diagnostic and therapeutic drugs.

Clinical value oriented and Public engagement

focus on clinical value throughout the drug development process and ensure patients have a say in the development of innovative drugs

Accelerating market launch to support clinical-value-oriented drug innovation

Special Review and Approval Procedure

When a public health emergency breaks out, special review and approval for corresponding therapeutic and prophylactic drugs may be carried out based on the decision of NMPA in accordance with the law.

BTD

Innovative drugs or modified new drugs used for the prevention and treatment of serious life-threatening diseases or diseases that seriously affect the quality of life but no effective means of prevention and treatment is available, or significantly clinically superior over current treatments with sufficient evidence is demonstrated

Priority Review and Approval Procedure

(1) Drugs with urgent clinical needs in shortage, innovative drugs and modified new drugs for the prevention and treatment of major infectious diseases or rare diseases;
(2) New products, dosage forms and strengths of paediatric drugs complying with the physiological characteristics of children;
(3) Vaccines urgently needed for disease prevention and control, and innovative vaccines;
(4) Drugs included into the breakthrough therapy drug procedure;
(5) Drugs included into the conditional approval procedure;
(6) Other circumstances of priority review and approval specified by the NMPA.

Conditional Approval Procedure

(1) Drugs used for treating serious life-threatening diseases for which no effective treatment is available, whose efficacy has been verified by data in drug clinical trials and whose clinical values can be predicted;
(2) Drugs that are urgently needed for public health, whose efficacy has been demonstrated by data in drug clinical trials and whose clinical values can be predicted;
(3) Vaccines urgently needed for response to critical public health emergencies or urgently needed as identified by the National Health Commission, whose benefits outweigh risks as evaluated.

Evolving Regulatory System in Alignment with ICH

- The NMPA joined the International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) in 2017 and was elected twice to the ICH Management Committee, in 2018 and 2021. This year, NMPA was re-elected as a member of the ICH Management Committee for the third time.
- As of the ICH Fukuoka Conference in June 2024, NMPA has adopted and implemented all 69 ICH guidelines and has played an active role in shaping regulatory rules from an international perspective.
- The adoption of ICH guidelines has been expedited through targeted training sessions and the publication of interpretative documents, which have facilitated their integration into drug research, development, registration, and approval processes.
- NMPA has strengthened its exchanges and collaboration with other member states by actively participating in various ICH activities, thereby enhancing its influence in global drug regulation.

https://www.cde.org.cn/ichWeb/index.jsp

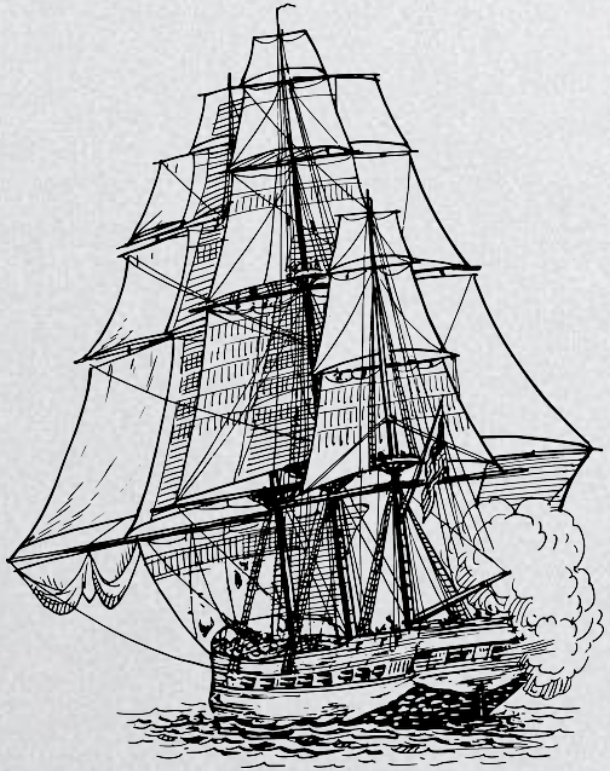
Status of the issuance of domestic guidelines

Category		Guideline	Implementation Date
Top-level Design		General Considerations in Drug Clinical Trials	2017-01-18
Evaluation	**Efficacy**	Comprehensive analysis of the effectiveness of drug clinical studies (for Trial Implementation)	2021-12-30
	Safety	Clinical Safety Evaluation of New Drugs	2023-12-01
	Comprehensive Assessment	Benefit-Risk Assessment of New Drugs	2023-06-25
Situations Requiring Flexible Regulation	**Oncology**	Clinical development of anti-tumour drugs oriented by clinical value	2021-11-19
		Suitability of single-arm clinical trials to support marketing applications for anti-tumour drugs	2023-03-14
		Clinical Trials of Anti-tumour Drugs	2012-05-15
	Rare Diseases	Clinical Development of Drugs for Rare Diseases	2022-01-06
		Application of Decentralized Clinical Trials in the Development of Drugs for Rare Diseases	2024-05-30
	MRCT	International Multi-Center Drug Clinical Trials	2015-01-30
		Acceptance of Overseas Clinical Trial Data	2018-07-11
	Animal Rule	Drug Research Based on the Animal Rule (for Trial Implementation)	2023-04-07

Data extracted from
https://www.cde.org.cn/zdyz/listpage/9cd8db3b7530c6fa0c86485e563f93c7

Category		Guideline	Implementation Date
Design Elements Impacting Clinical Trial Quality	**Study Population**	Enrichment Strategies and Design in Drug Clinical Trials (for Trial Implementation)	2020-12-31
	Treatment Description	No Guideline	—
	Control Group Selection	Based on ICH E10: Choice of Control Group and Related Issues in Clinical Trials	—
	Response Variables	Endpoints in Clinical Trials of Anti-tumour Drugs	2012-05-15
		Imaging Evaluation Procedures in Anti-tumour Drug Clinical Trials	2023-07-19
		Application of Patient-Reported Outcomes in Drug Clinical Research (for Trial Implementation)	2022-01-04
	Bias Reduction Methods	Randomization in Drug Clinical Trials (for Trial Implementation)	2022-01-07
		Blinding in Drug Clinical Trials (for Trial Implementation)	2022-12-30
	Statistical Analysis	Biostatistics in Drug Clinical Trials	2016-06-03
		Non-Inferiority Design in Drug Clinical Trials	2020-07-24
		Statistical design of clinical trials of anti-tumour drugs (for Trial Implementation)	2020-12-31
		Adaptive Design in Drug Clinical Trials (for Trial Implementation)	2021-01-29
		Statistics in clinical studies of drugs for rare diseases (for Trial Implementation)	2022-06-06
	Research Data Management	Data Management and Statistical Analysis Plans in Drug Clinical Trials	2022-01-04

Rising Tide, Rising Issues: Innovative drug development in a Changing Landscape



01. A Pressing Need for Groundbreaking Innovation

Original innovation is the driving force behind drug development; Increased investment in basic research and encouragement of original thinking are crucial to avoid product homogeneity

02. Elevating the Foresight of Technological Standards

Establishing a forward-looking and dynamic system of technological standards is necessary

03. Strengthening International Collaboration for Global Impact

Strengthen international exchange and cooperation to contribute to International harmonization of technical requirements; Jointly promote global drug development and accelerate simultaneous drug approval

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Fostering Innovation in Drug Development: Navigating Challenges and Seizing Opportunities

Fostering innovation in drug development requires a collaborative approach among governments, enterprises, and researchers. Despite challenges, China's drug R&D offers significant opportunities:

- Strong Government Support
- Regulatory reforms
- Growing scientific capabilities
- Large patient pool
- Vast Unmet Clinical Needs
- Rapidly Growing Pharmaceutical Market
- A developing innovation ecosystem

And many more…

Conclusion and outlook

- Align drug review standards with international best practices, actively participate in global regulatory harmonization efforts, and foster a robust environment for drug research and development
- Continuously deepen the reform of the evaluation and review & approval system and accelerate the marketing of clinically urgent drugs, especially those used to prevent and treat severe life-threatening diseases
- prioritize the oversight of innovative drugs and advancing therapies and intensify focus on drug quality and safety
- Encourage global simultaneously R&D
- Encourage the R&D of new drugs for special groups, such as rare disease drugs and paediatric drugs
- Commit to promoting regulatory science research and exploring innovative supervision methodologies
- Leverage new technology to enhance the intelligence and efficiency of drug supervision.
- Strengthen relationships with regulatory agencies worldwide and foster collaboration among government, academia, and industry to promote innovation, and increase support for the development of new drugs
- Build a strong talent base and to create a diverse and dynamic ecosystem for drug innovation.

We are dedicated to advancing the field of innovative drug development,
meeting evolving clinical demands and protecting public health.

Thank you!