Exploring the Efficacy, Safety and Regulatory Approval of Priority Reviewed Innovative Drugs in China (2015-2023)

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Background

China implemented the priority review program (PRP) since 2015 to accelerate the approval of innovative drugs with substantial clinical value. However, comprehensive evaluations comparing the clinical value of drugs approved through the PRP with those approved through the non-priority review pathway remain limited.

Aim

We assess whether the PRP more efficiently facilitates the timely delivery of cancer drugs with greater clinical value to patients by comparing approval times, efficacy, and safety between the two pathways.

Methods

Our research analyzed innovative cancer drugs approved by the National Medical Products Administration (NMPA) from 2015 to 2023, using a public database.

Inclusion criteria: Chemical and biological products registered as Class 1 in drug registration classification.

Exclusion criteria: Traditional Chinese medicine, supportive therapies (such as for the complications of cancer) and contrast agents.

Extracting **NDA/BLA review time**, **efficacy outcomes** (overall survival(OS), progression-free survival(PFS), and response rate(RR)), and **safety outcomes**(Grade ≥ 3 adverse events(AEs) and treatment-related serious adverse events(SAEs)).

A meta-analysis was conducted to assess the **hazard ratio for PFS**, as well as **RR**, **treatment-related SAEs**, and **Grade ≥ 3 AEs**.

A subgroup analysis was performed to evaluate the impact of the new policy changes introduced in 2020 on the PRP.

Results

Characteristics

From January 1, 2015, to December 31, 2023, the NMPA approved 40 innovative cancer drugs for 54 indications, with no approvals occurring between 2015 and 2017.

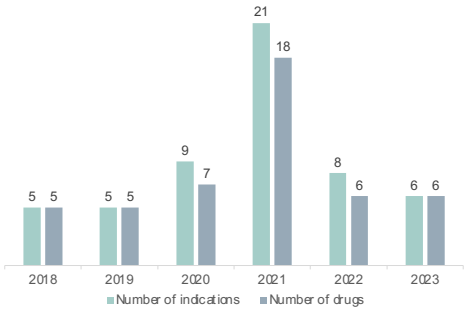

Figure 1: The annual number of innovative cancer drugs and indications approved for market by NMPA*

*Drugs are categorized by year of approval; the total number may exceed 40 because the same drug can be approved for different indications in different years and is counted multiple times.

Among the 54 approved indications, 34(62.96%) were approved primarily based on RR, 13(24.07%) on PFS, and 4(7.41%) on OS. Among the 39 priority-reviewed indications, 28(71.79%) were approved based on RR, 5(12.82%) on PFS, and 3(7.69%) on OS.

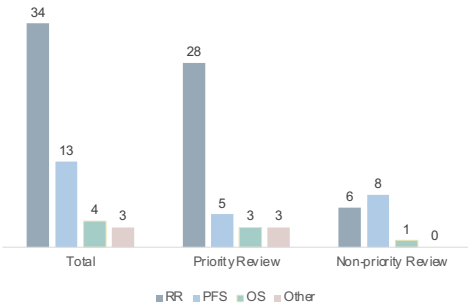

Figure 2. The distribution of primary trial end point

Lung cancer was the most common type, accounting for 33.33% of indications, followed by lymphoma(22.22%), breast cancer(5.56%) and ovarian cancer(5.56%).

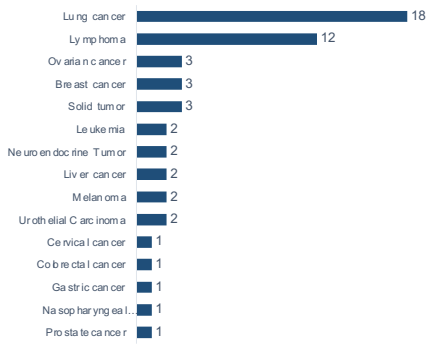

Figure 3. Cancer Types of Indications

Approval Time

The median NDA/BLA review time for priority-reviewed indications was marginally shorter than that for non-priority-reviewed indications(381 vs 404 days, p = 0.636). After the implementation of the new policy in 2020, the review time for priority-reviewed indications was significantly reduced from 405 to 284 days.

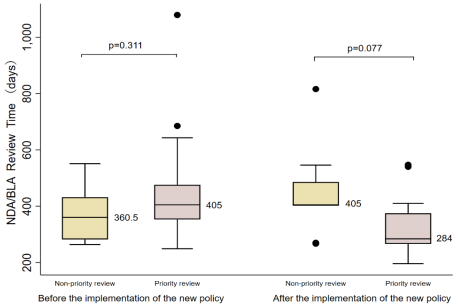

Figure 4. NDA/BLA review times for priority and non-priority review indications for new policy implementation.

Efficacy & Safety

Efficacy analysis revealed no significant differences in RR and PFS between priority and non-priority-reviewed indications.

Safety analysis indicated a higher incidence of Grade ≥ 3 AEs and treatment-related SAEs in drugs approved through the PRP.

Table 1: Efficacy Outcomes of NMPA -Approved Priority-Reviewed Versus Non-Priority-Reviewed Drug Indications, 2015-2023

	Priority Review drug indications (n=39)	Non-Priority Review drug indications (n=15)	P value
single-arm trials			
Response rate, %			
Median (IQR)	62.6(31.3,74.1)	67.6(20.2,89.4)	0.889
Pooled estimate(95%CI)	54.9(45.7,63.9)	58.0(23.5,88.6)	0.869
randomized controlled trials			
Overall survival			
months, median(IQR)	9.6(9.3,12.1)	15.3	/
Clinically meaningful improvement, No. (%)	3 of 5(60%)	1 of 1(100%)	1.000
Progression-free survival			
months, median(IQR)	13.6(8.44,14.7)	8.95(8.25,9.75)	0.379
Pooled hazard ratio (95%CI)	0.50(0.44,0.57)	0.51(0.45,0.57)	0.864
Clinically meaningful improvement, No. (%)	7 of 9 (77.78%)	5 of 9(55.56%)	0.310

Table 2: Safety Outcomes of NMPA -Approved Priority-Reviewed Versus Non-Priority-reviewed Drug Indications, 2015-2023

	Priority Review Drug Indications (n=39)	Non-Priority Review Drug Indications (n=15)	P value
randomized controlled trials			
Grade ≥3 AEs (%)	1408 of 2395(58.79)	1318 of 2384(55.29)	0.948
The risk ratio for the pooled Grade≥3 AEs	1.96(1.36,2.82)	1.07(0.99,1.17)	0.002
treatment-related SAEs (%)	226 of 1898(11.91)	356 of 1449(24.57)	0.036
The risk ratio for the pooled treatment-related SAEs	1.80(1.41,2.30)	1.31(1.12,1.54)	0.035
single-arm trials			
Grade ≥ 3 AEs (%)	1311 of 3401(38.55)	191 of 837(22.82)	0.057
the pooled Grade ≥3 AEs (%)	39.6(31.9,47.5)	22.6(15.9,30.1)	0.002
treatment-related SAEs (%)	483 of 2550 (18.94)	68 of 752(9.04)	0.121
the pooled treatment-related SAEs (%)	20.4(13.9,27.9)	8.9(7,11.1)	0.001

Conclusion

Although the PRP expedites patient access to innovative drugs, evidence remains insufficient to demonstrate significant additional benefits in efficacy and safety.

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