Is China leading in RWE? A Scoping Review of RWE Generated from China Hainan Boao Lecheng Pilot Zone

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OBJECTIVE

Since its implementation in 2019, Hainan Boao Lecheng International Medical Tourism Pilot Zone (Pilot Zone) has enabled early clinical use of innovative drugs and medical devices in China and facilitated the generation of real-world evidence (RWE) to support regulatory decision-making. This study aims to systematically evaluate the characteristics and quality of RWE generated within the Pilot Zone, and to identify opportunities for improvement under the newly established RWE-based Comprehensive Value Assessment (RCVA) framework — a national health technology assessment (HTA) framework developed to support dynamic pricing and reimbursement decision-making.

BACKGROUND

RWE generated from clinical practice within the Pilot Zone has been increasingly used to support accelerated regulatory approval and market access. As of December 2025, 525 specially licensed pharmaceuticals and medical devices had been introduced into the Pilot Zone of which 22 technologies have achieved regulatory approval.

The RCVA framework aims to identify key concerns and uncertainties in regulatory and reimbursement decision-making, and to assess long-term clinical effectiveness and safety in real-world settings through real-world studies (RWS), including but not limited to interventional, non-interventional, or observational studies. Throughout the lifecycle of a health technology, the RCVA framework further assesses economic value, health equity, accessibility, innovation, and patient reported outcomes to reflect comprehensive value. This pilot program will carry out in the 11 pilot provinces and gradually roll out to nation-wide by the end of 2027.

METHODS

A targeted literature search was conducted in MEDLINE, Cochrane Library, ClinicalTrials.gov, Chinese database Yiigle and Wanfang Data. Eligible studies included RWS of innovative health technologies conducted in the Pilot Zone and published in English or Chinese since 2019. Study characteristics were extracted. Market approval and national reimbursement status were confirmed through separate searches. The last search was updated in May 2026.

KEY FINDINGS

- 15 health technologies were identified through the search: 11 are medicinal treatments and 4 are medical device/surgical platform.
- **Disease areas**: Among the 15 studies, the most common therapeutic areas were oncology (3 studies), followed by ophthalmology (2), dermatology (2), cardiovascular disease (2), and neurology/CNS disorders (2). Additional areas included oncology supportive care (1), hematology/immunology (1), urology (1), and otolaryngology/ENT (1).
- **Study design**: Sixty percent (9/15) of the studies were prospective observational studies, while the remaining 40% were retrospective studies, including chart review and retrospective observational analyses. Three studies were multicenter. One study was part of an international registry study. Two studies incorporated external control arm or historical cohort.
- **Outcomes assessed**: All studies evaluated effectiveness and safety, with one study additionally collected healthcare resource use (HCRU), two collected subsequent treatment, two collected disease-specific quality of life (QoL) outcomes, and one collected caregiver burden.
- Most of studies reported interim effectiveness and safety results at the time of publication, therefore limited sample size exists across all studies.
- **Analysis methods**: 60% of studies employed descriptive approaches including pre/post and longitudinal analyses, with the remaining 40% applying more advanced statistical methods such as propensity score matching (PSM), time-to-event analysis, causal inference or correlation analysis. One study used a Bayesian borrowing approach to integrate RWD and global trial data while balancing covariates through inverse probability of treatment weighting (IPTW).
- **Regulatory & reimbursement outcomes**: Among the 15 innovative products, 8 were subsequently included in the National Reimbursement Drug List (NRDL). One product was included in the first Commercial Insurance Innovative Drug List. The average time from RWS initiation to regulatory approval was 17.7 months. The duration from regulatory approval to reimbursement listing ranged from a few months to more than three years, with an average of 21 months.

DISCUSSION

The Pilot Zone has enabled innovative products not yet available in China to accelerate their regulatory pathways by generating country-specific RWE while simultaneously addressing urgent unmet patient needs. However, the quality of RWE generated within the Pilot Zone has varied substantially, particularly in terms of study design, matching, and confounding adjustment. Common limitations across studies included short follow-up periods, small sample sizes, and potential selection bias.

Notably, only one study collected HCRU data to inform economic modeling, and very few studies captured subsequent treatment patterns or QoL outcomes. This highlights an important gap, suggesting that much of the RWE previously generated in the Pilot Zone may not be considered sufficiently comprehensive under the new RCVA framework, which requires evidence on HCRU and QoL in addition to effectiveness and safety outcomes.

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Table 1 . Characteristics of studies identified

Treatment	Disease area	Type of technology	Study Design	Additional clinical trial in China	Reported Sample Size	Interim Study period	Population (local/national)	Outcomes collected	Analysis	Marketing approval status	NRDL status
Fluocinolone Acetonide Intravitreal[1]	Chronic Noninfectious Uveitis Affecting the Posterior Segment of the Eye (NIU-PS)	Ccorticosteroid implant	Prospective multicentre RWS, the first phase of injection was conducted in Boao	Yes	74	Oct 2020 – Dec 2027, ongoing	National	Effectiveness, safety, concomitant treatments, HCRU, subsequent therapy	External control arm using propensity score matching (PSM); Published cost-effectiveness study based on the RWS	Approved in June 2022	Included in 2025
Lecanemab[2]	Mild cognitive impairment (MCI) or mild Alzheimer's disease	Medicine	Prospective, observational, single-arm study	Yes	64	Commenced in Nov 2023, ongoing	National	Safety and 6-month clinical outcomes; biomarker; MRI;PET; longitudinal cognitive and functional outcomes collected in long-term follow-up; burden of caregivers	Longitudinal analysis	Approved in Jan 2024	Included in China's first Commercial Insurance Innovative Drug Lis in Dec 2025.
Trilaciclib Hydrochloride for Injection[3]	ES-SCLC myeloprotection	Medicine	Prospective observational single-arm study	Yes	30	August 2021 -December 2022	National	Effectiveness (myeloprotective effect and antitumor) and safety	Safety analysis and time-to-event analysis	Approved in July 2022	Included in 2024
Lurbinectedin[4]	ES-SCLC	Medicine	Multicentre retrospective study	Yes	47	Commenced in Aug 2022	National	Effectiveness and safety	Descriptive and time-to-event analysis	Approved in Dec 2024	Not included/initiated
Tepotinib[5]	METex14 skipping NSCLC	Medicine	Case series/retrospective systematic chart review	Yes	4	January 2022 – Nov 2023	National	Effectiveness and safety	Per-case discussion	Approved in Dec 2023	Included in 2024
Belumosudil[6]	Chronic Graft-versus-Host Disease	Medicine	Retrospective single-arm study	Yes	20	May 2023 - March 2024	National	Effectiveness and safety	Time-to-event analysis	Approved in Aug 2023	Included in 2024
Unilateral Cochlear implantation[7]	Hearing loss	Medical Device	Prospective, Single-arm study	Yes	78	Sep 2020 - Dec 2021	National	Effectiveness and safety	Pre/post analysis	Approved in Sep 2022	Participated in 2024 national procurement
Secukinumab[8]	Psoriasis	Medicine	Retrospective multicentre observational study	Yes	81	Commenced in July 2018	National/Local	Effectiveness, QoL and safety	Statistical analysis; causal analysis of the association between outcomes and baseline variables	Approved in March 2019	Included in 2020
Symplicity Spyral™ renal denervation (RDN) system[9]	Hypertension	Medical Device/surgical platform	Prospective international registry study	No	54	Nov 2022 – May 2024, ongoing	National	Effectiveness and safety, antihypertensive medication usage collected through 6 months	Pre/post analysis	Approved in May 2024	Not included
3D OCT Femtosecond Laser-Assisted Cataract Surgery[10]	Cataract	Medical Device/surgical platform	Prospective observational study	Yes	113	November 2019 -January 2021	Local	Pre/post-operative visual outcomes, success rate and safety	Pre/post analysis	Approved in Jan 2021	Not included
Rezūm water vapor thermal therapy system[11]	Benign prostatic hyperplasia	Medical Device/surgical platform	Retrospective observational study	Yes	22	Dec 2020 – Jan 2021	National	Effectiveness, operation time, QoL and safety	Descriptive analysis	Approved March 2022	Not included
1.5% ruxolitinib cream[12]	Non-segmental vitiligo	Topical cream	Retrospective observational study	No	996	Aug 2023 – May 2025	National	Long-term Effectiveness and safety	Descriptive analysis; clinical phenotype correlation analysis	Approved in Jan 2026	Not initiated
Inclisiran[13]	Hyperlipidemia	Medicine	Prospective observational study	Yes	28	Jan 2022 – Apr 2023	National	Short-term effectiveness and safety	Pre/post analysis	Approved in Aug 2023	Included in 2025
Isatuximab, pomalidomide, and dexamethasonesatuximab (Isa-Pd)[14]	Relapsed and/or refractory multiple myeloma	Medicine	Prospective observational study	No	24	Commenced in Aug 2022	National	Effectiveness and safety	Bayesian borrowing approach – this RWS as a bridging study; time-to-event endpoint analysis; External historical cohort was used. IPTW was used to balance confounding factors	Approved in Jan 2025	Included in 2025
Pitolisant[15]	Adult narcolepsy	Medicine	Prospective observational study	No	30	Commenced in May 2021	National	Effectiveness and safety	Longitudinal analysis	Approved in June 2023	Included in 2023

Figure 1. Duration from RWS initiation to marketing approval

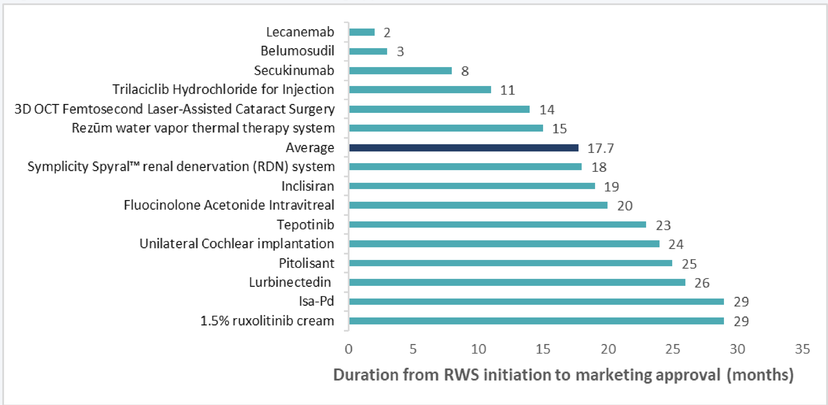
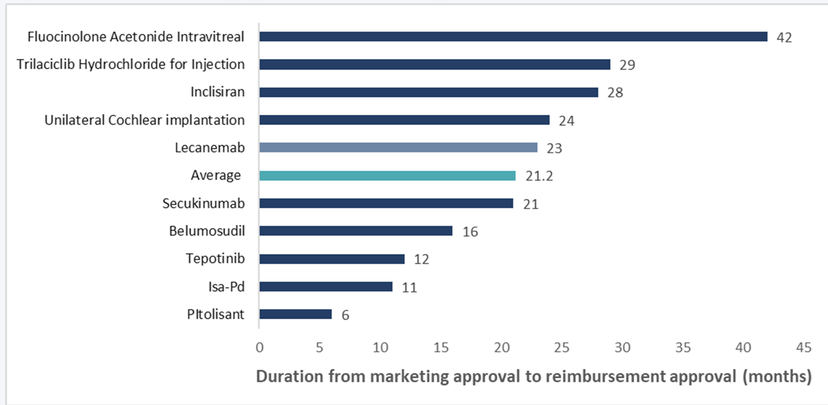


Figure 2. Duration form marketing approval to reimbursement approval



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