

Zhen Dong¹, Bojing Cai³, Shuo Yang³, Dorothy Hsia², Yang Xie³

¹IQVIA Solutions Asia Pte Ltd, Singapore; ²IQVIA Solutions Taiwan Ltd, Taipei, Taiwan; ³IQVIA Solutions Shanghai Ltd, Shanghai, China



Introduction and study objectives

Background: Clinical outcome assessments (COAs) measure aspects of how patients feel, function, or survive and include patient-, clinician-, observer-, and performance-reported measures. In China, CDE issued guidance addressing PROs and patient-centered drug development between 2021 and 2023 (Figure 1). However, the extent to which China-site drug trials register COA-based endpoints remains unclear.

Objective: To estimate the annual proportion of eligible non-generic China-site interventional drug trials initiated during 2020–2025 that registered ≥ 1 classified COA-based primary or secondary endpoint, and to describe endpoint role and distributions by development phase and assigned analysis category.

Interim guidance on PRO use in drug clinical development issued; effective upon release.* announcement/web 4 Jan 2022

2021 Sep

2021 Dec

2023 Jul

Draft guidance on PRO use in drug clinical research released for one-month public consultation

Three interim patient-centered guidelines issued:

- Patient-Centered Clinical Trial **Design** Guideline
- Patient-Centered Clinical Trial **Implementation** Guideline
- Patient-Centered **Benefit-Risk Assessment** Guideline

Figure 1. Evolution of China's Patient-Centered Drug-Development Guidance

Methods

Registry-based descriptive analysis

- ClinicalTrials.gov and the CDE registry via Pharmcube were searched for interventional trials with a start date from 1 January 2020 to 31 December 2025 and at least one China site.
- Duplicate and generic/bioequivalence records were excluded using prespecified rules.
- Candidate outcome measures were identified from registered primary and secondary outcome titles/descriptions using prespecified keywords.
- Two reviewers classified candidates as PRO, ClinRO, ObsRO, PerfO, composite, non-COA, or unclear, with discrepancies adjudicated.
- Prevalence was calculated using all trials meeting the same eligibility and exclusion criteria as the denominator.

Limitation

- Registry entries may be incomplete or updated after study initiation; registered outcome text does not always identify the reporter, instrument version, or actual implementation. Keyword-based classification is therefore subject to both false-positive and false-negative error.

Table 1. Search strategy and eligibility criteria for literature search

Items	Definitions	Items	Definitions
Data sources	• ClinicalTrials.gov • CDE registry data via Pharmcube	Keywords used, not exhaustive	• Generic PRO/COA terms: PRO, COA, patient-reported outcome, patient questionnaire, patient diary, self-reported • Quality-of-life and health-status measures: QoL, HRQoL, EQ-5D, SF-12, SF-36, PROMIS • Symptom and functional assessments: pain, pruritus, fatigue, sleep quality, symptom severity, NRS, VAS • Disease-specific and global instruments: EORTC QLQ-C30, FACT, FACIT, DLQI, KCCQ, IBDQ, PGI, CGI and related measures
Geographic scope	• At least one study site located in China		
Inclusion criteria	• Interventional clinical trials • Trial initiation between 2020–2025 • Conducted in China • Explicitly reported ≥ 1 COA-based endpoint	Analysis approach	• Descriptive aggregation by year, therapeutic area, and trial phase
Exclusion criteria	• Non-interventional studies • Trials without identifiable COA-based endpoints • Duplicate records • Generic drug trials		

Results

- Among **15,567 eligible** non-generic China-site drug trials, **3,427 (22.0%)** registered ≥ 1 classified COA-based primary or secondary endpoint. (Figure 2).

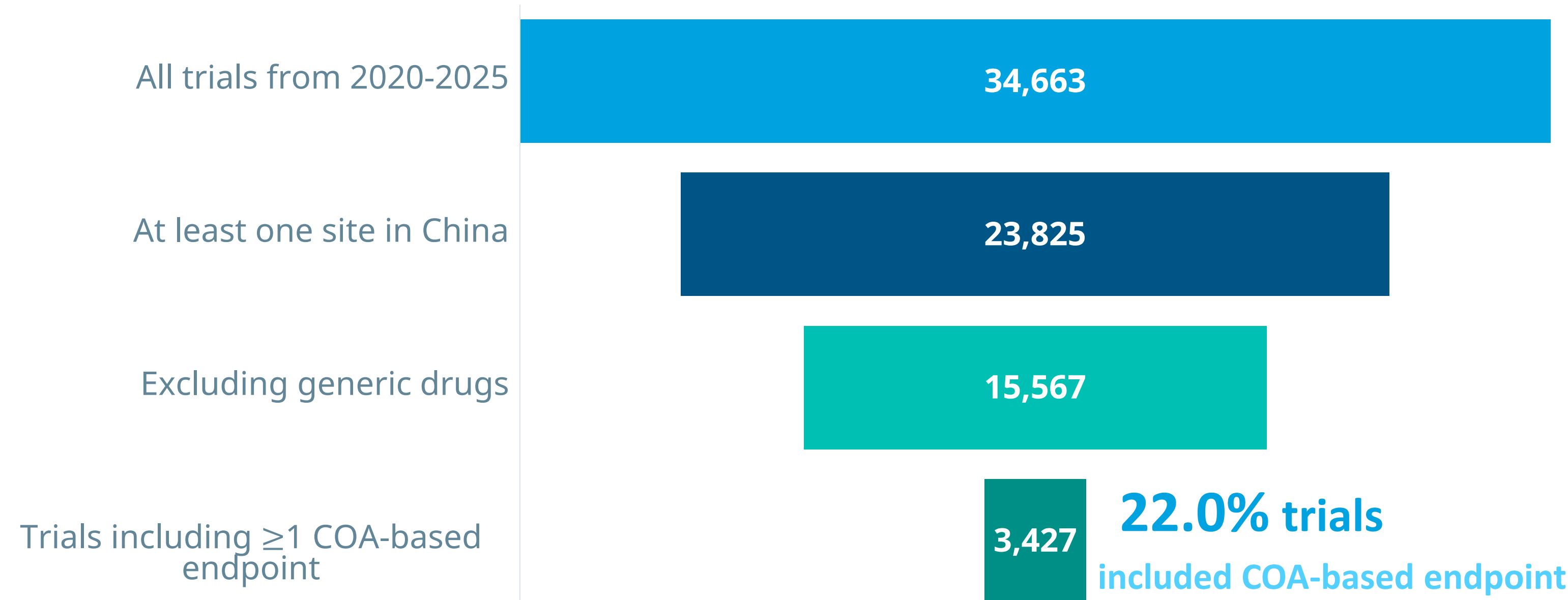


Figure 2. Study-selection flow

- Among COA-positive trials, 83.2% were Phase II or III; 71.0% included COA-based endpoints only as secondary endpoints (Figure 4, 5).

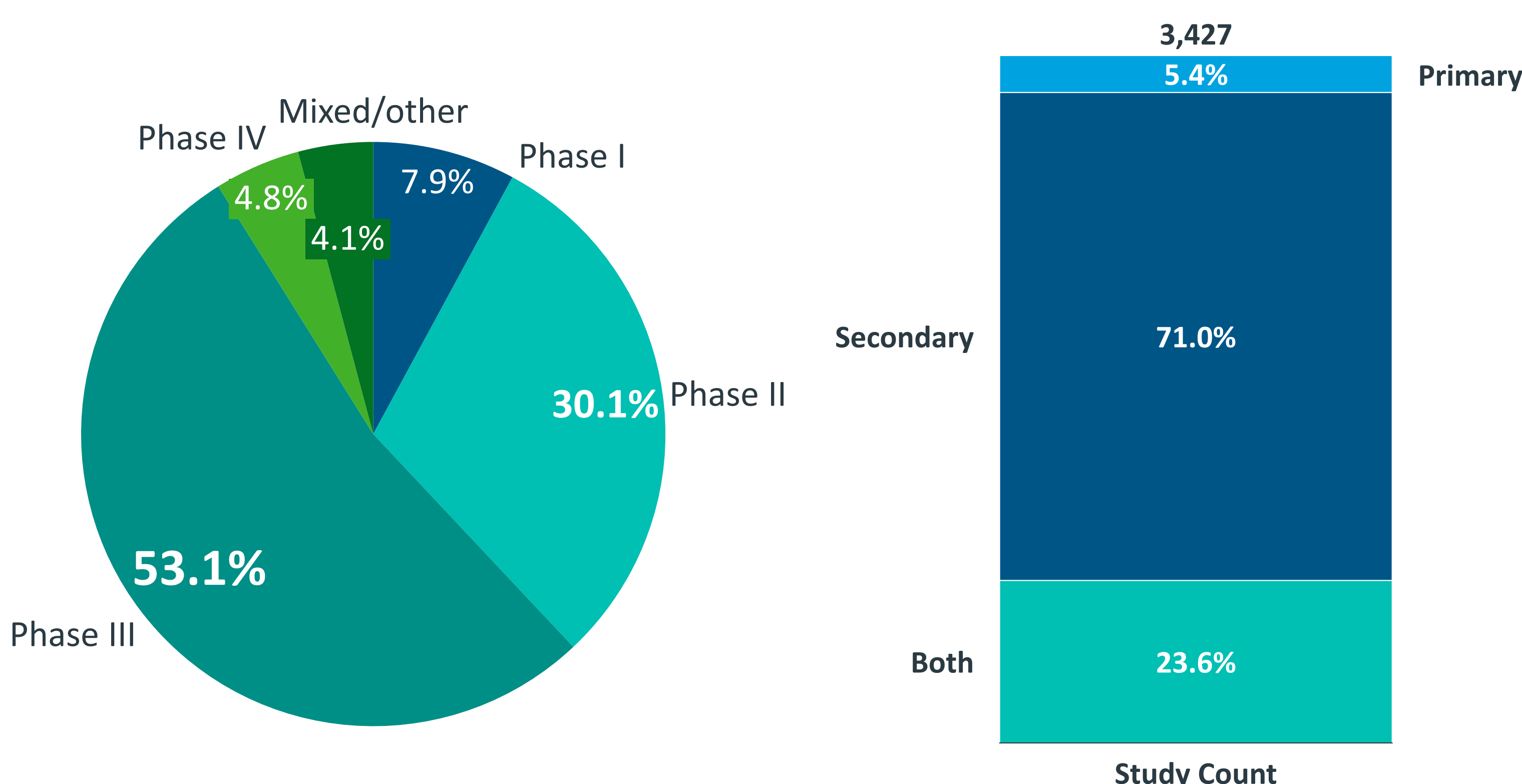


Figure 4. Distribution of Trials including COA-based Endpoints by Development Phase

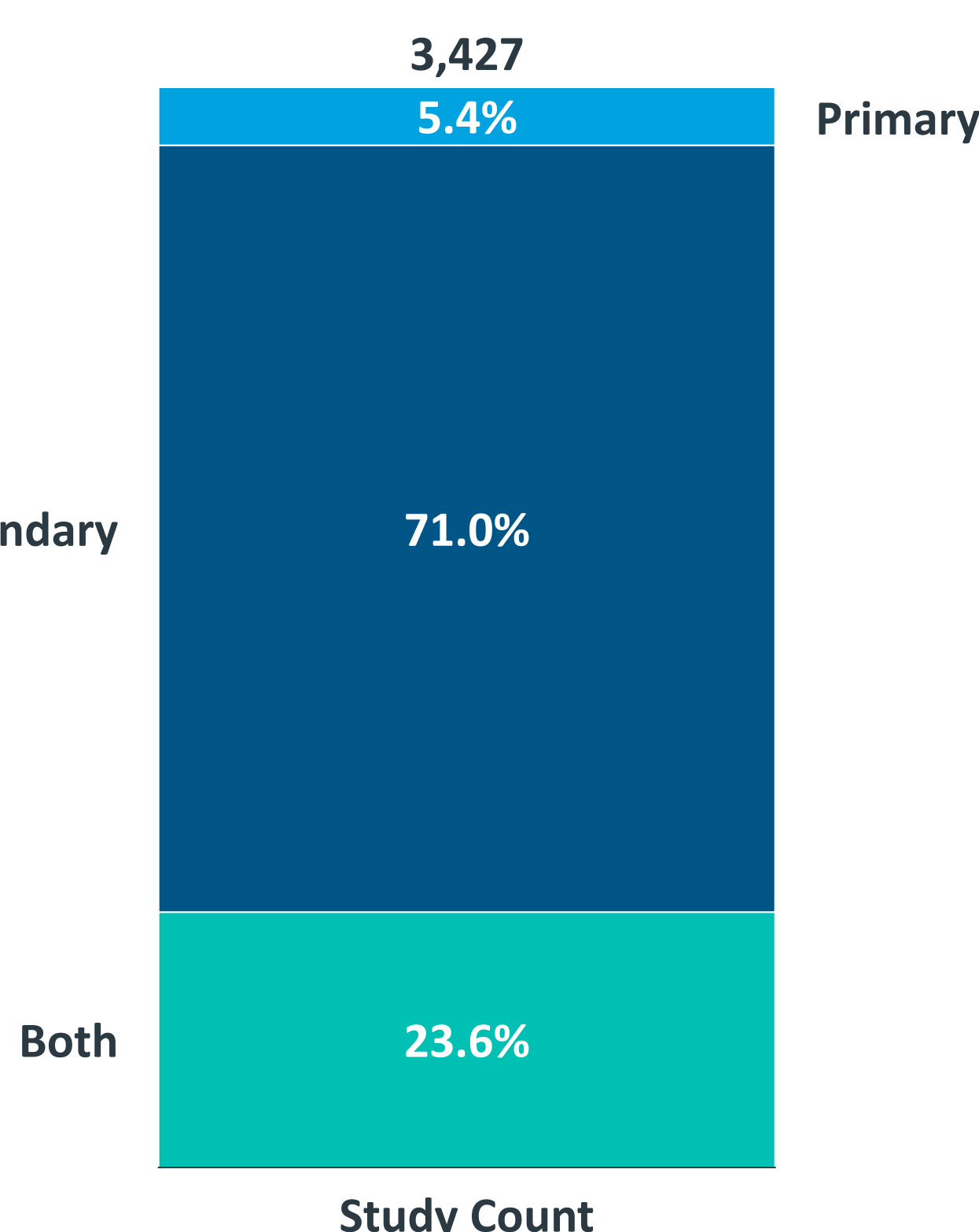


Figure 5. Overall distribution of COA-based endpoints by endpoint role

- Annual counts increased from 338 in 2020 to 826 in 2025, while annual proportions varied modestly from 20.5% to 22.9% (Figure 3).

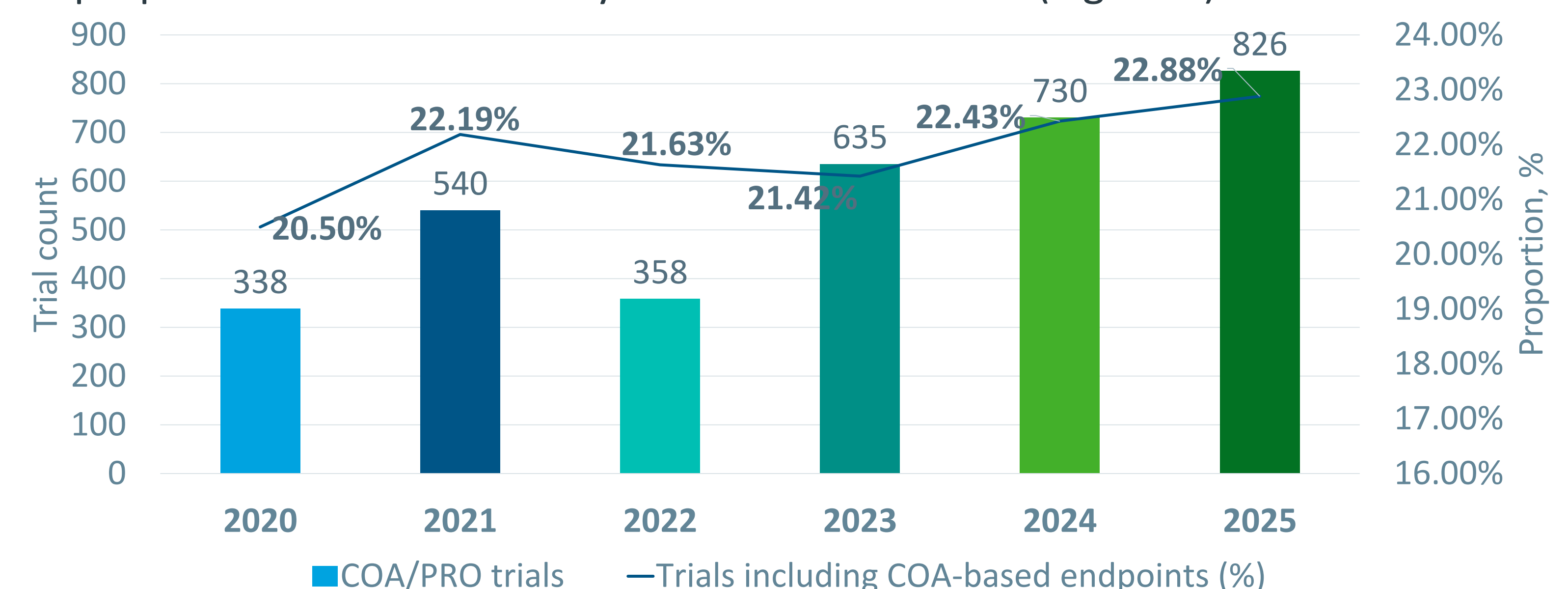


Figure 3. Annual number and proportion of trials including COA-based endpoints

- **Oncology (n=949) and immunology (n=918)** contributed the largest numbers of COA-positive trials, but category-specific prevalence could not be assessed without corresponding eligible-trial denominators (Figure 6).
- **Endpoint-role proportions showed a different pattern:** Despite having the largest trial volume, the most common COA-based endpoint role in oncology was secondary (86.1%). **Primary COA-based endpoint was proportionately highest in infectious disease (13.1%)**, while CNS had the highest proportion of both primary and secondary use (46.2%, Figure 6).

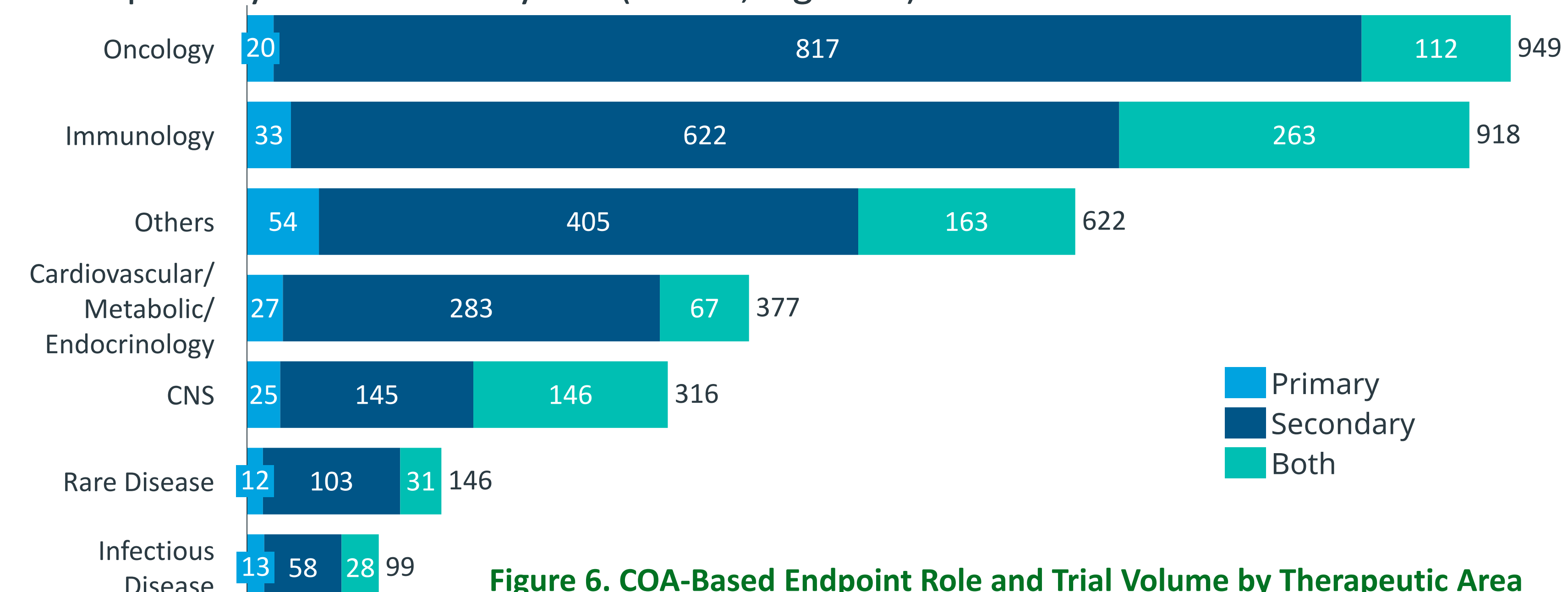


Figure 6. COA-Based Endpoint Role and Trial Volume by Therapeutic Area

Conclusions

Approximately one in five eligible China-site non-generic drug trials registered ≥ 1 classified COA-based endpoint during 2020–2025. The absolute number increased alongside overall trial volume, whereas the annual proportion showed no consistent upward pattern. COA-based endpoints were predominantly secondary, and most endpoint-positive trials were Phase II or III.