HOW CERTAIN ARE WE OF INNOVATION? A REVIEW OF CLINICAL EVIDENCE OF EMA APPROVED NEW INDICATIONS IN 2024

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BACKGROUND AND IMPORTANCE

Therapeutic innovation is continuously expanding leading to a growing number of new active substances (NAS) and indication extensions of preexisting medicines (IEPM) evaluated by the European Medicines Agency (EMA). The need for new therapeutic options has driven many approvals to occur under accelerated procedures or based on preliminary evidence. This raises questions regarding the maturity and robustness of clinical data supporting them. It's crucial to assess the quality of evidence behind these authorizations ensuring informed decision-making by emphasizing hospital pharmacists' role in evidence appraisal and real-world assessment.

AIM AND OBJECTIVES

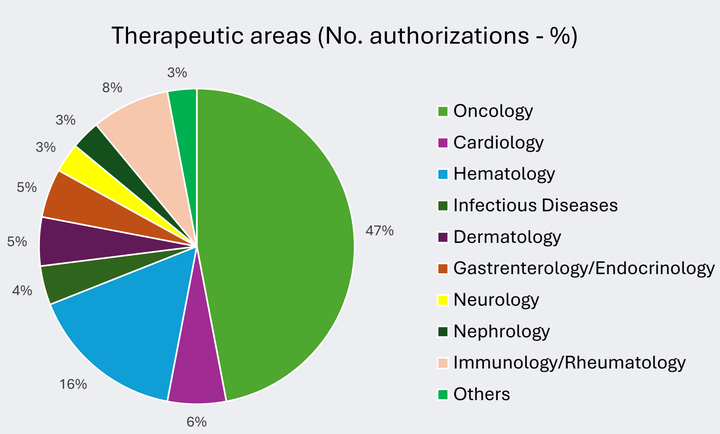
Characterize and assess the level of clinical evidence underlying all positive opinions issued by the Committee for Medicinal Products for Human Use (CHMP) in 2024 for NAS and IEPM.

MATERIALS AND METHODS

Observational, descriptive, retrospective study including all NAS and IEPM receiving a positive CHMP opinion in 2024 [age-based extensions excluded.] Data were retrieved from the European Public Assessment Reports (EPARs). Extracted variables comprised drug characteristics, regulatory designations and approval conditions, clinical evidence parameters, innovation metrics, regulatory conclusions, study limitations, and trial identifiers..

RESULTS

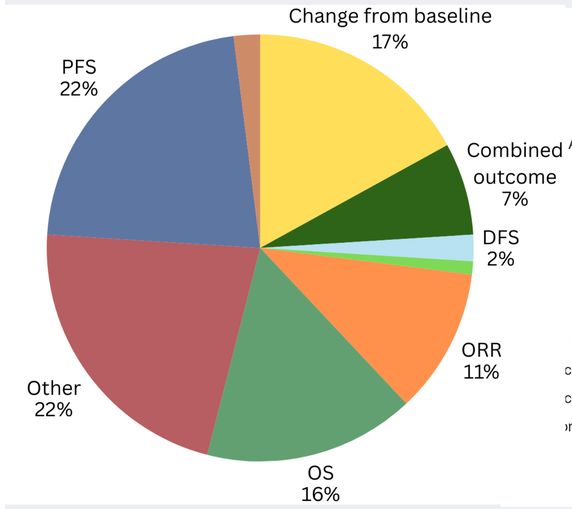
A total of 100 authorizations were included: 42 NAS (46 indications and 35.7% first-in-class) and 54 IEPM across 15 therapeutic areas and 23% held an orphan designation. Additionally, 26% of the NAS were approved under special conditions (CA/AA/EC).



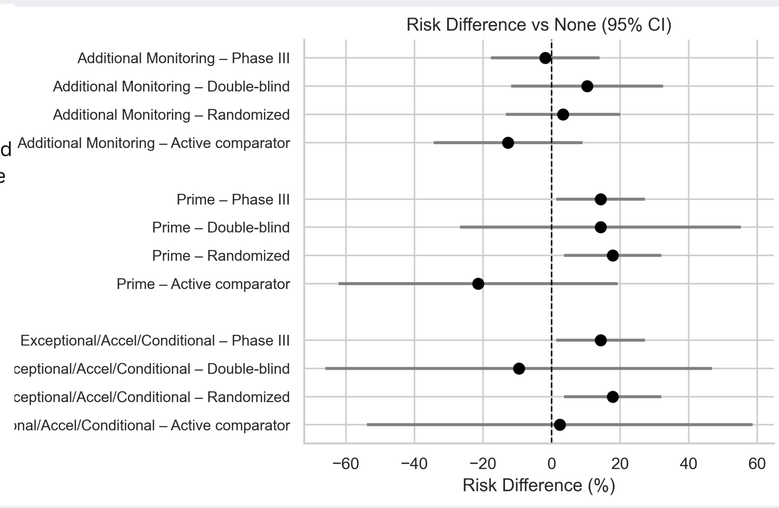
Graphic 1 – Therapeutic areas of each authorization (%)

23% Orphan
7% PRIME
69% Additional Monitoring
13% Conditional approval
4% Exceptional Conditions
4% Accelerated Assessment

The majority were supported by phase III (83%), randomized (85%), active-controlled (54%), open-label (49%).



Graphic 2 – Relative frequency of type of primary outcome (%)

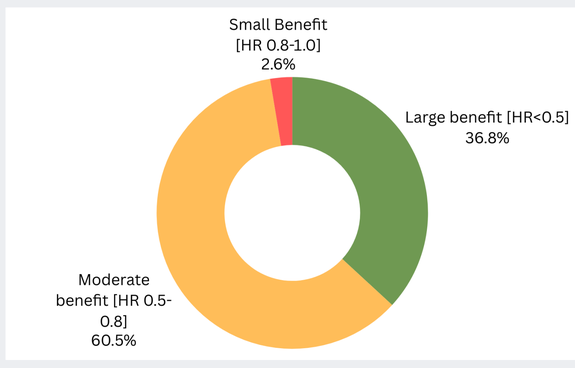


Graphic 3 – Trial characteristics by regulatory group

Oncology

Among oncology therapies (47%), 43% achieved an ESMO-MCBS score ≥4 or A, indicating relevant clinical benefit, although 68% relied on surrogate endpoints and/or with immature overall survival data.

79% Phase III	**64%** Open-label	**81%** Randomized	**74%** Active comparator	**32%** OS as primary outcome	2 AA; 7 CA 2 PRIME 7 Orphan



Graphic 4 – Clinical benefit based on HR value, in Oncology

Nearly half of authorizations relied on trials meeting gold standard criteria.

17% Single-Arm, Phase II

35% Phase III + Randomized + Open-Label

48% Phase III + Randomized + Double-Blind

CONCLUSION AND RELEVANCE

Most 2024 approvals were supported by phase III, randomized, active-controlled trials, however, a considerable proportion relied on preliminary data, particularly within oncology. Accelerated and conditional pathways highlight the need for systematic post-authorisation re-evaluation supported by real-world evidence to confirm clinical benefit and ensure rational use.

REFERENCES

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eahp european association of hospital pharmacists
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