

Strength of clinical evidence leading to approval of novel cancer medicines in Europe: a systematic review and data synthesis.

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Abstract

Aim We aimed to evaluate the quality of clinical evidence that substantiated approval of cancer medicines by European Medicines Agency (EMA) in the last decade. **Methods** We performed a systematic review and data synthesis of EMA documents in agreement with PRISMA guidelines. We included European Public Assessment Reports, Summaries of Product Characteristics, and published randomized controlled trials (RCTs) on anti-cancer drugs approved by EMA from 2010 to 2019, and excluded drugs not indicated for targeting solid or hematological tumors and non-innovative treatments. We synthesized frequencies of approvals differentiating between unblinded and blinded RCTs with and without overall survival (OS) as predefined primary outcome measure. We assessed frequency of post-approval RCTs for indications without at least one RCT at the time of approval. **Results** Of 199 approvals, 159 (80%) were supported by at least one RCT, 63 (32%) by at least one RCT having OS as the primary or co-primary endpoint, 74 (37%) by at least one blinded RCT, and 30 (15%) by at least one blinded RCT having OS as the primary or co-primary endpoint. Whereas 40 approvals (20%) were not supported by any RCT and, of those, 9 (22%) were followed by a post-approval RCT. **Discussion** While the majority of approvals of cancer medicines approved by EMA was supported by at least one RCT, we noted substantial methodological heterogeneity of the studies.

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Evidence supporting cancer drugs in EU.docx available at <https://authorea.com/users/725887/articles/708766-strength-of-clinical-evidence-leading-to-approval-of-novel-cancer-medicines-in-europe-a-systematic-review-and-data-synthesis>

Figure 1

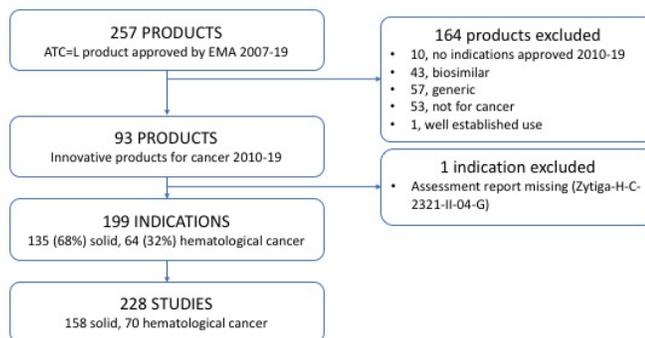


Figure 2.

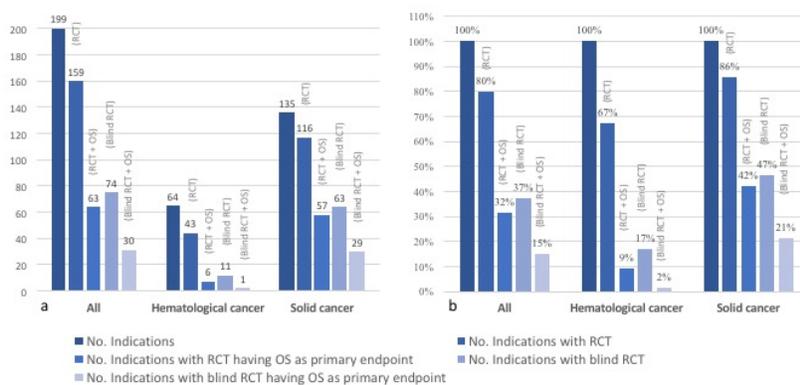


Figure 3.

