

EVALUATING THE METHODOLOGICAL QUALITY OF PIVOTAL CLINICAL TRIALS' PUBLICATIONS FOR ORPHAN DRUGS AUTHORISED IN 2018. ARE THEY RELIABLE?

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Background and importance

CONSORT 2010
Declaration
Guidelines

Most decisions made in clinical practice are based on the clinical trials (CT) publications

Tool widely used for the evaluation of methodological quality of publications of randomized clinical trials (RCTs)

They are a checklist of 25 items that allow evaluating the publications of RCTs from the point of view of the transparency, its design, the abstract, the flowchart of the participants and the analysis of the results

Aim and objectives

The main objective is to evaluate the methodological quality of all pivotal RCT publications of orphan drugs authorized during 2018 in the European Union

Material and methods

The pivotal CT publications were found in the ClinicalTrials.gov and PubMed databases on February 2019

The methodological quality was examined using the guidelines of the CONSORT 2010 statement on the publications of the RCTs, assigning a score of 0 or 1 to each of the sections that comprise it

They were also evaluated following the CONSORT for Abstracts guidelines because many clinical decisions are made based on the conclusions from these sections

Results

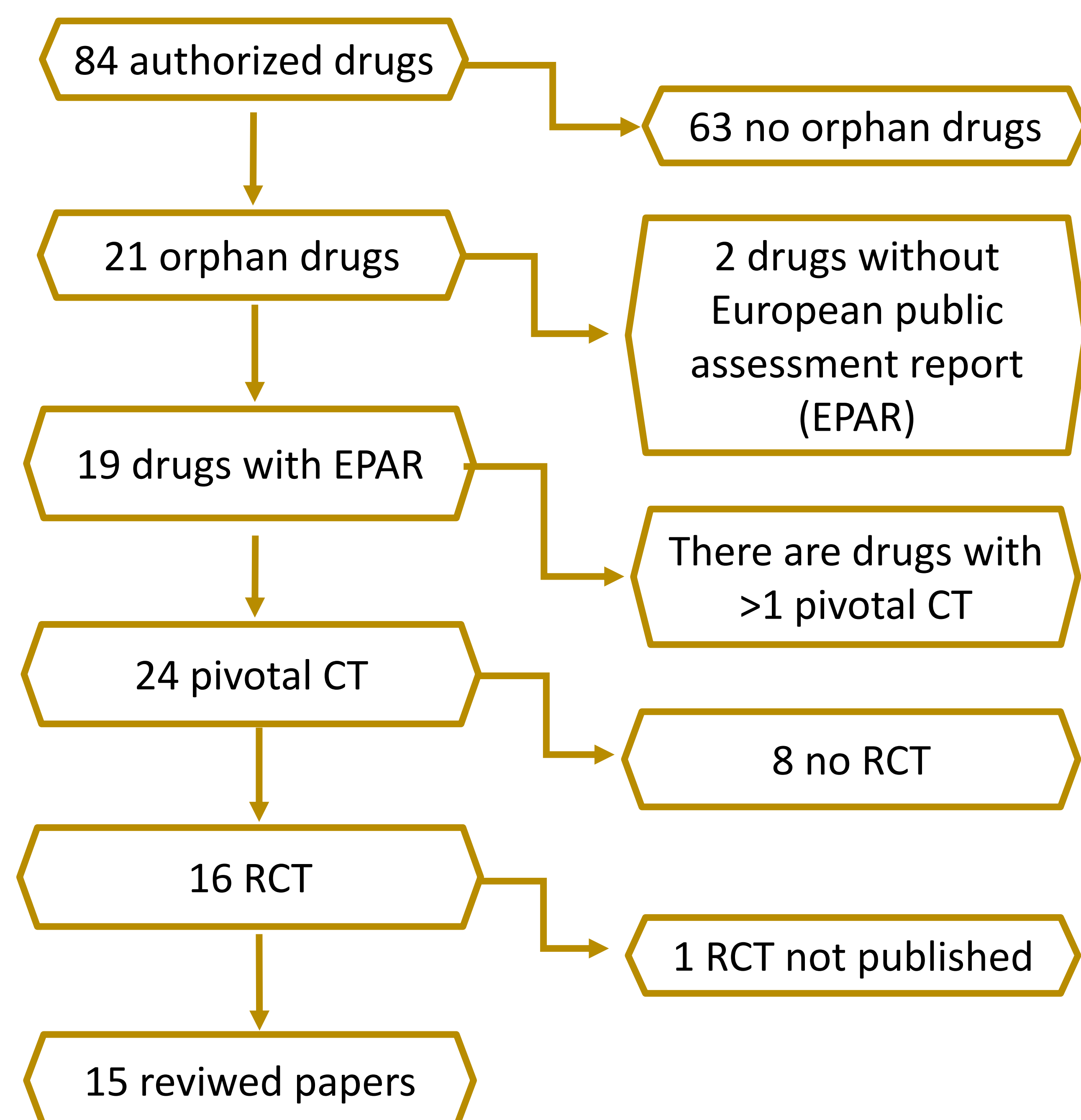
Of the 21 orphan drugs authorized in 2018, 24 pivotal CT were located and 33% of them were not randomized

The pivotal RCTs analyzed comply only 66.13% of the items in the CONSORT guidelines, compared with 82% in high-impact journals. 60% of abstracts analyzed fulfill more than 70% of the items in the CONSORT for Abstracts Declaration

Only 26.6% of the RCTs describe the randomization method selected

Regarding masking, only 40% of the RCTs detail who remained blinded after performing the corresponding interventions

As for the access information to the complete protocol of the RCT, only 20% collects where it can be located



Conclusions and relevance

The pivotal RCT publications of orphan drugs meet most of the items in the CONSORT 2010 guidelines, particularly the abstracts

However, many pivotal CT are not randomized and compliance with the guidelines is not as high as that of other high-impact journal publications

Therefore, more quality and transparency criteria should be required in the pivotal CT publications of orphan drugs, such as randomization, detailed masking or where to locate the complete protocol

