Orphan Drugs – Regulatory Landscape

Basel Epidemiology Seminar
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Regulatory Affairs & Drug Development Solutions
IQVIA

Regulatory incentives linked to Orphan Drug designation

- Prevalence of condition in the EU not more than 5 in 10,000 or unlikely that the medicine's commercial returns would justify investement
- Treatment, prevention or diagnosis of life threatening or chronically debilitating condition
- No satisfactory method approved or, if such a method exists, the product will be of significant benefit to those affected.

- Condition affecting fewer than 200,000 persons in USA or will not be profitable within 7 years of approval.
- Competitor products containing a similar drug to an authorised orphan drug product, may be awarded orphan designation if a plausible explanation of clinical superiority over the existing drug product is presented.

Regulatory Incentives

Incentive	EU	US
Assistance during product development	Protocol Assistance	Research study design assistance coordinated by Office of Orphan Product Development
Tax breaks/financial incentives for development	No	Subject to derogations. Up to 50% tex credit for clinical research undertaken by sponsor. Grant funding available.
Fee waivers	Fee waivers / reductions for SME and academics	Exemption from user fee
Market exclusivity	10 years market protection +2 years after completion of PIP	7 years – same drug, same use or indication

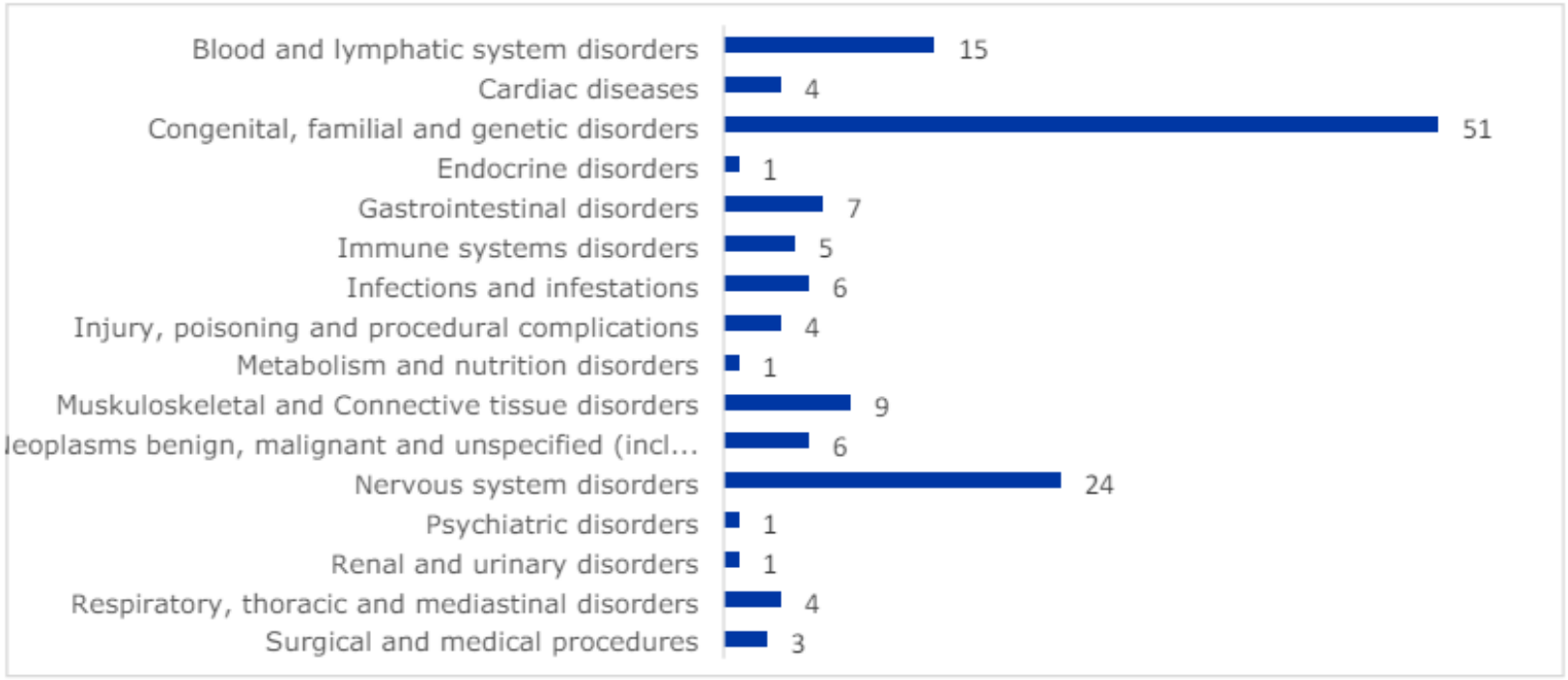
EMA orphan designations 2000-2023

	2000 – 2010	2010 - 2020	2021	2022	2023	Total
Applications for designation submitted	1234	2444	251	269	195	4.393
Commission Decisions on designation	828	1554	170	182	137	2,871

https://www.ema.europa.eu/en/documents/report/annual-report-use-special-contribution-orphan-medicinal-products-2023_en.pdf

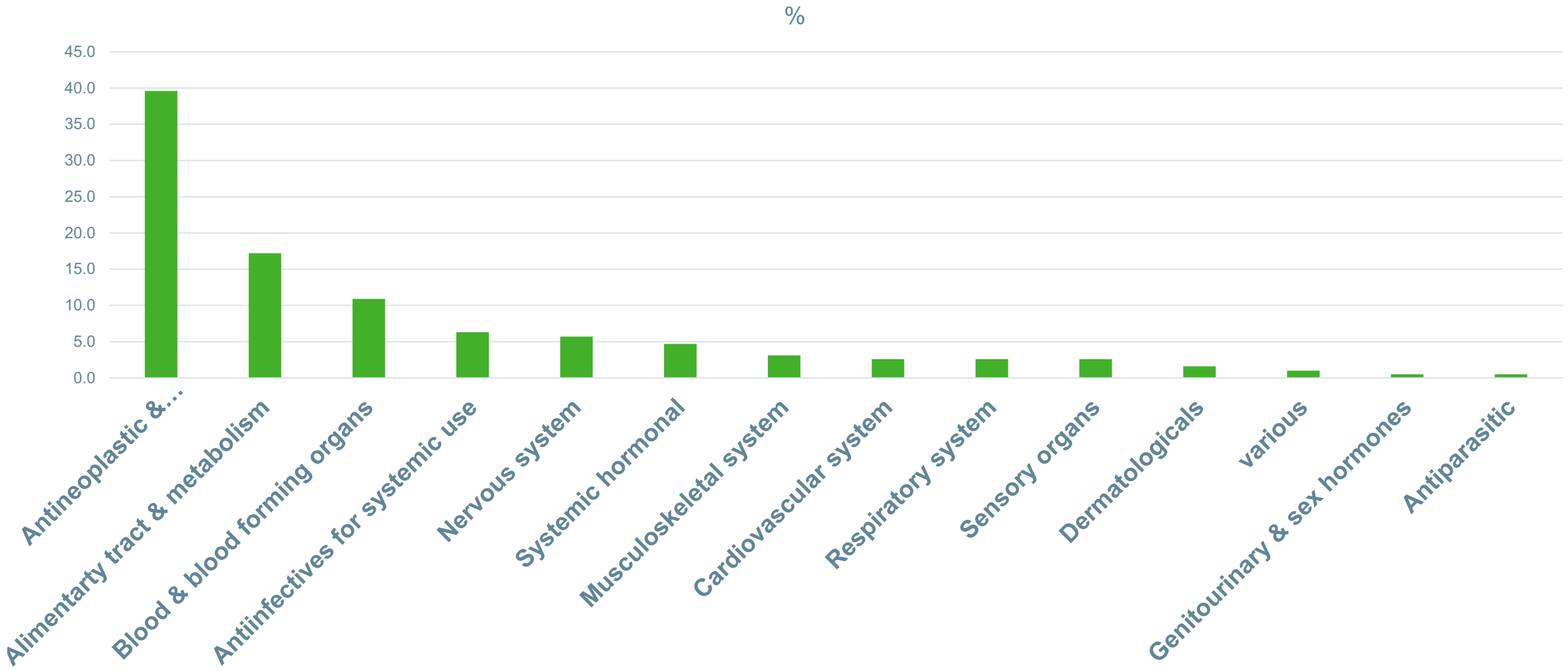
EMA orphan designations by therapeutic area 2023

Figure 1. Distribution of COMP opinions in 2023 – MedDRA classification



https://www.ema.europa.eu/en/documents/report/annual-report-use-special-contribution-orphan-medicinal-products-2023_en.pdf

Orphan Medicinal Products Approved 2010-2022 by ATC code



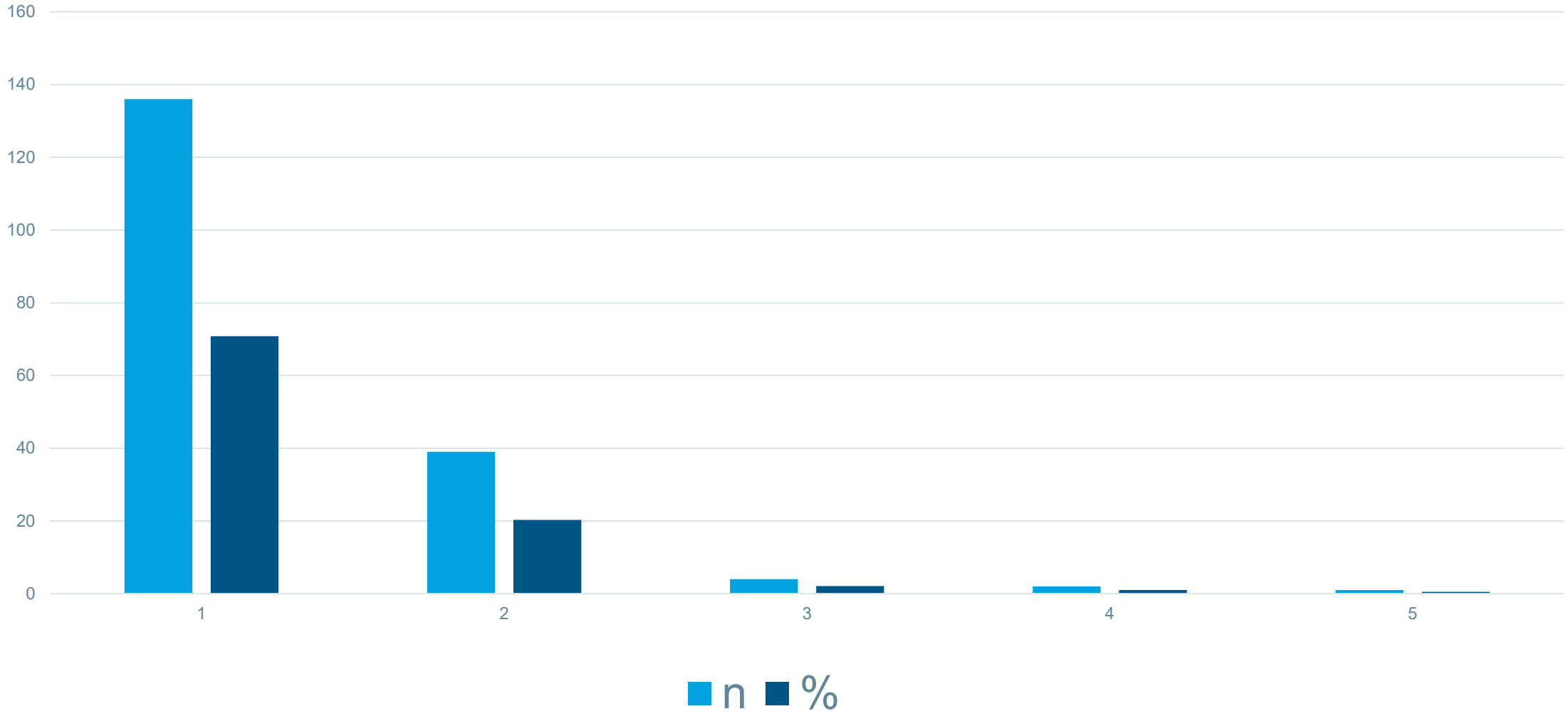
Bouwman et al. Orphanet Journal of Rare Diseases (2024) 19:91. https://doi.org/10.1186/s13023-024-03095-z

New active substance approved with orphan designation 2022

Reg Authority	% of new drugs approved with orphan status (2022)	Median approval time (days) – orphan v non-orphan MA (2018-22)
EMA	42%	**422** v 435
FDA	56%	**301** v 364
PMDA	39%	**272** v 329
Swissmedic	38%	**418** v 424
TGA	33%	**291** v 351

https://cirsci.org/wp-content/uploads/dlm_uploads/2023/07/CIRS-RD-Briefing-88-6-agencies-v.1.4.pdf

Number of pivotal efficacy studies supporting orphan medicinal product approval in EU (2010-22)



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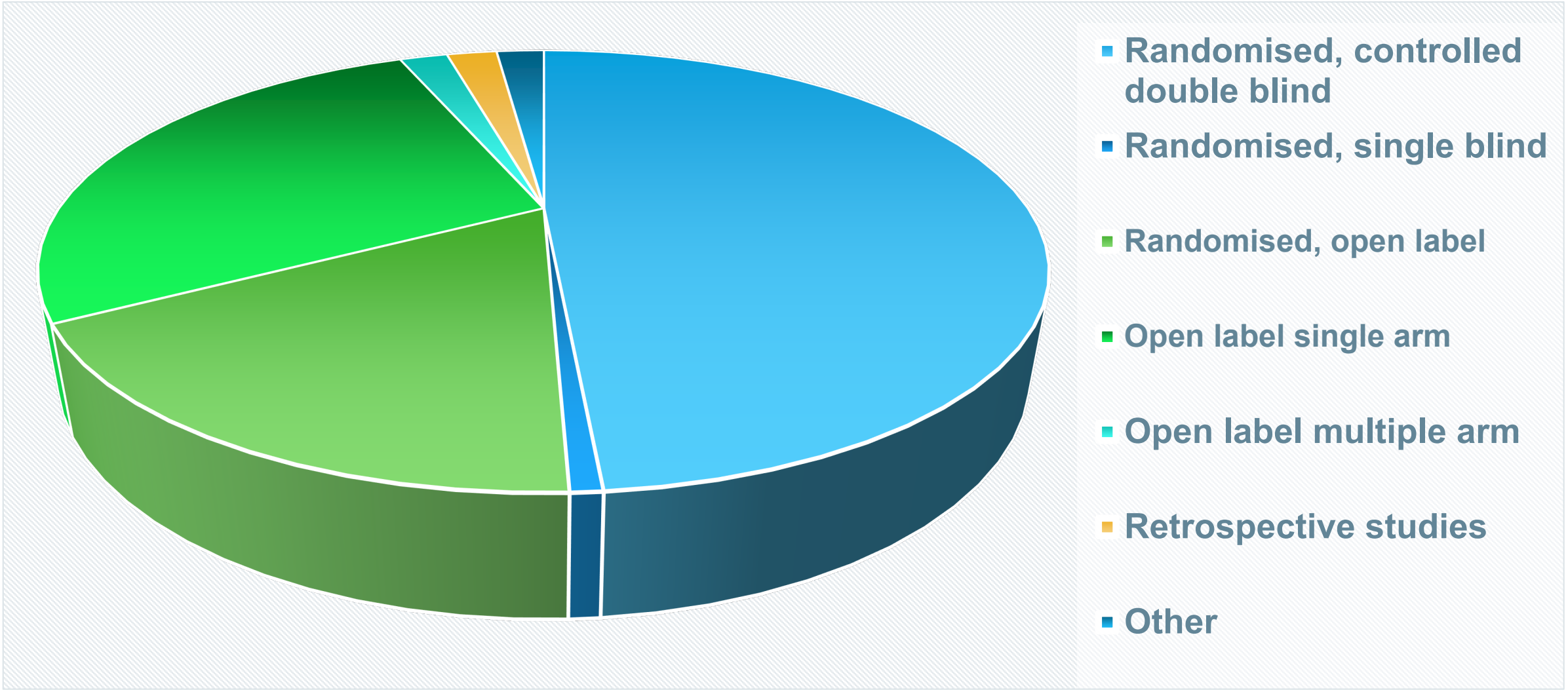
Main clinical trial designs supporting EU orphan medicinal product approvals, 2010-2022



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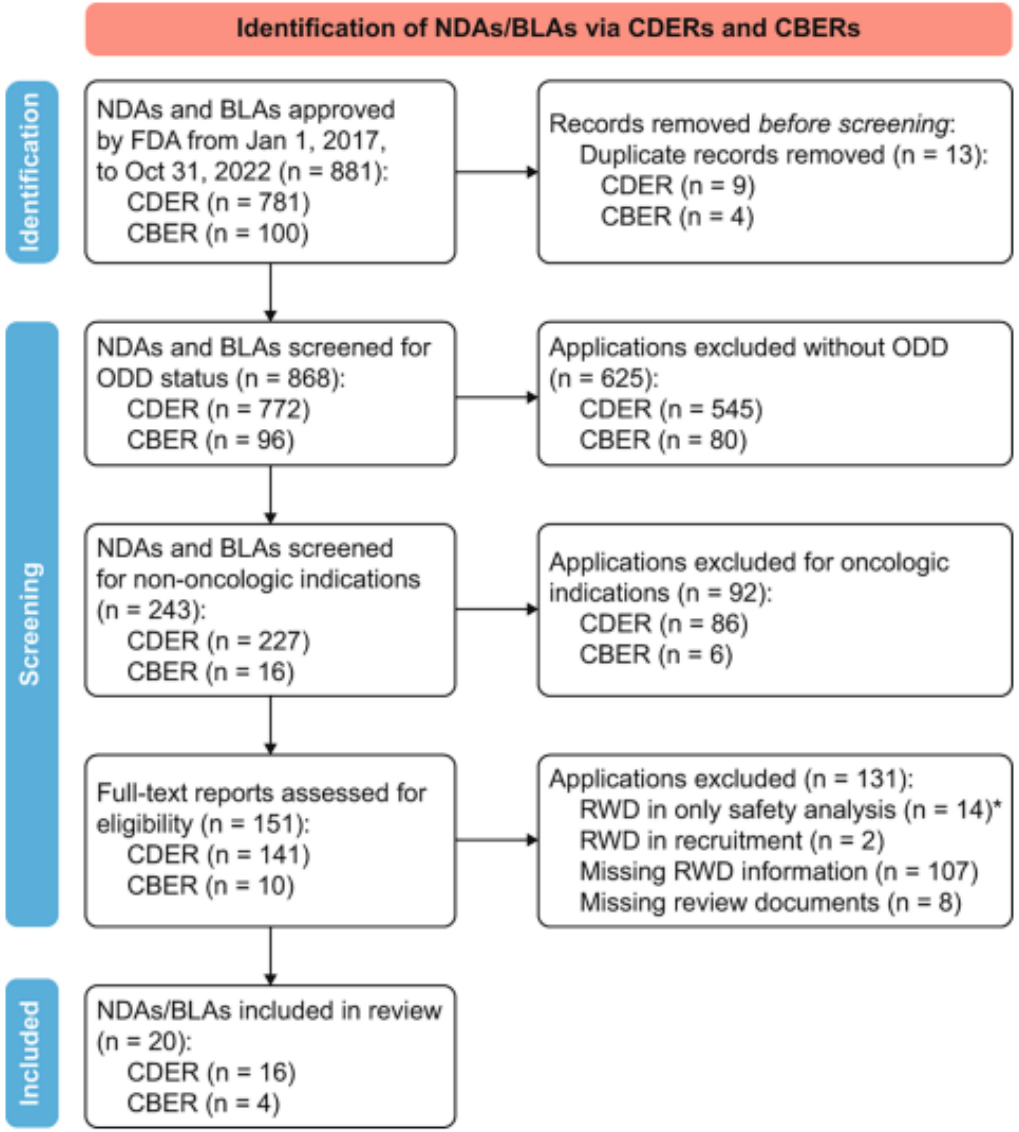
Vaghela *et al. Orphanet Journal of Rare Diseases* (2024) 19:117
https://doi.org/10.1186/s13023-024-03111-2

Orphanet Journal of Rare Diseases

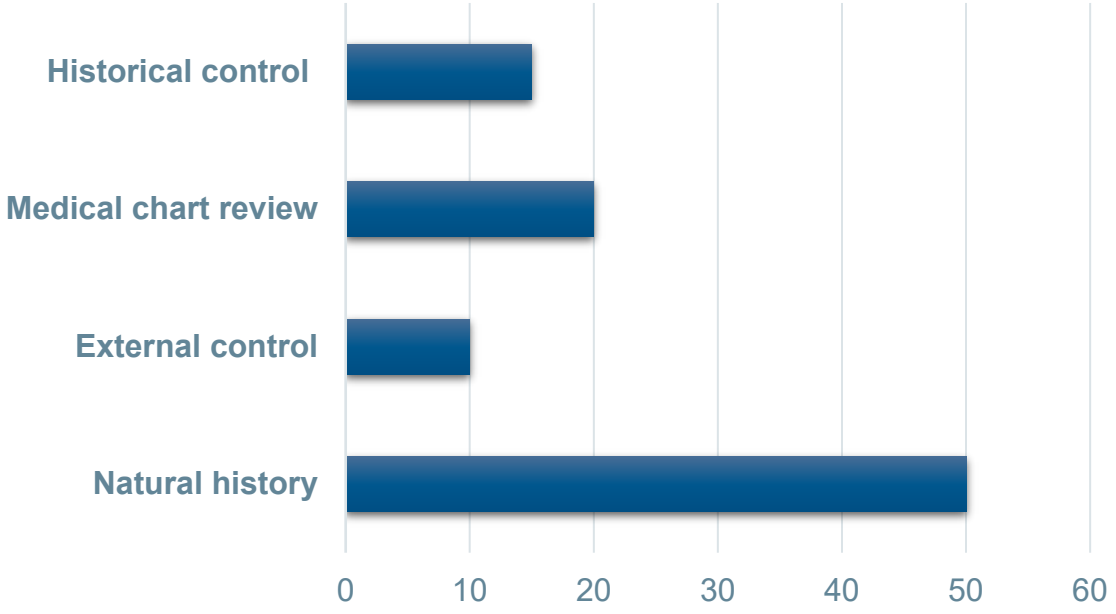
REVIEW Open Access

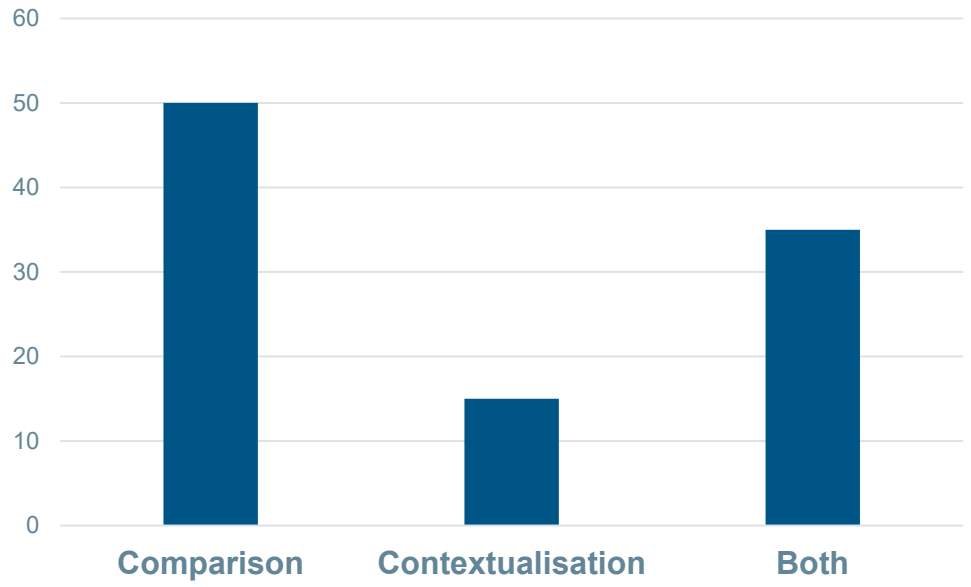
A systematic review of real-world evidence (RWE) supportive of new drug and biologic license application approvals in rare diseases

Shailja Vaghela[1], Kaniz Afroz Tanni[2], Geetanjoli Banerjee[3*] and Vanja Sikirica[3]



RWD study design & purpose of RWD included in FDA non-oncologic products 2017-22 (%)





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Parliament adopts its position on EU pharmaceutical reform

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- Ensuring safe, efficient and quality medicinal products
- Fostering innovation and development of medicines to address unmet medical needs
- Boosting research in novel antimicrobials to fight antimicrobial resistance (AMR)

Further information

- Adopted texts will be available here (10.04.2024)
- Recording of the plenary debate (10.04.2024)
- Procedure file (directive)
- Procedure file (regulation)
- Legislative train - Revision of the EU pharmaceutical legislation
- EP Research: Revision of the EU pharmaceutical legislation (April 2024)
- Free photos, videos and audio material

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