Updates from the Committee of Orphan Medicinal Products
-
COMP

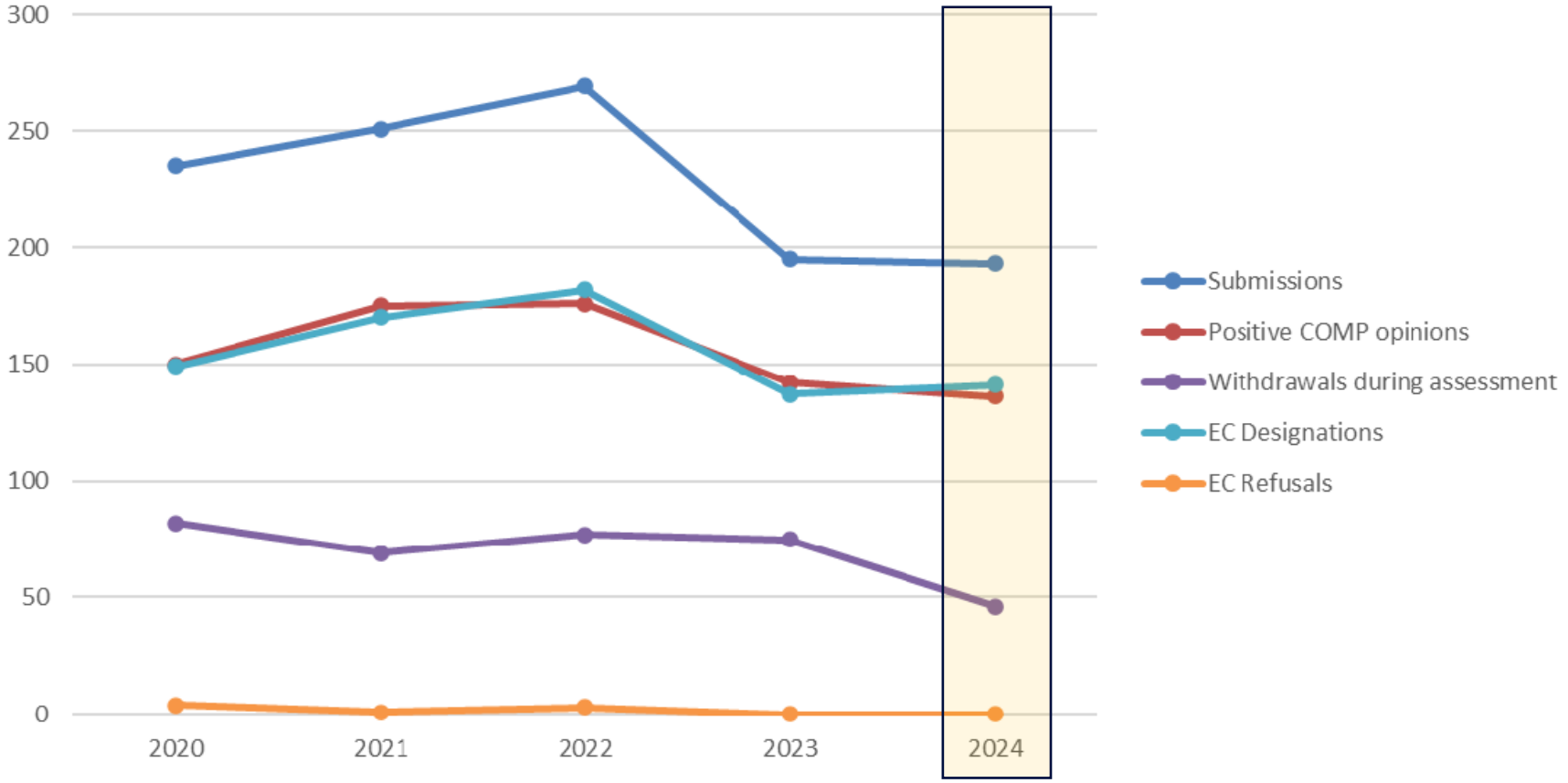
PCWP-HCPWP joint meeting

1 - 2 April 2025

Close-up on

Orphan designations 2024

Status of Applications for Orphan Medicinal Product Designation



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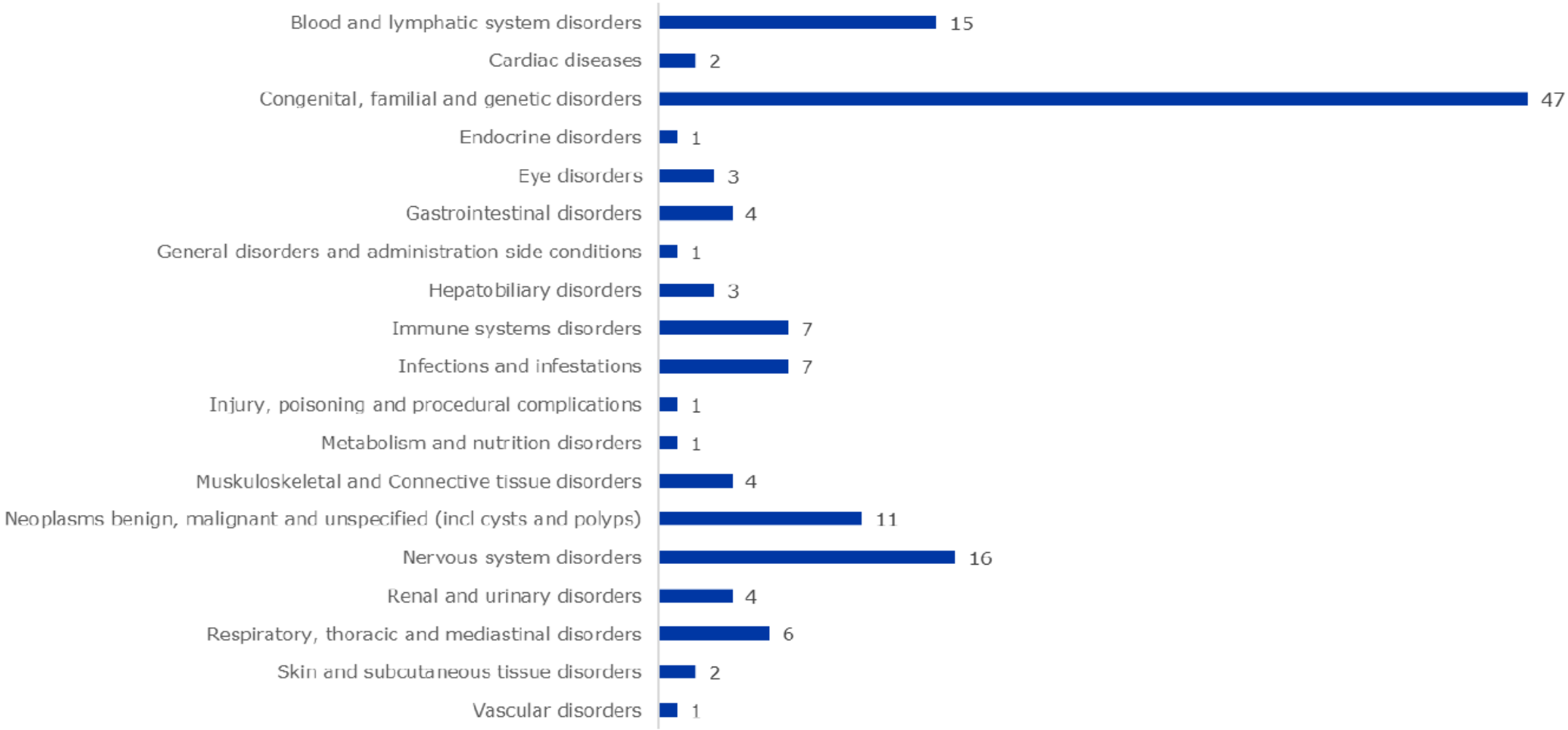
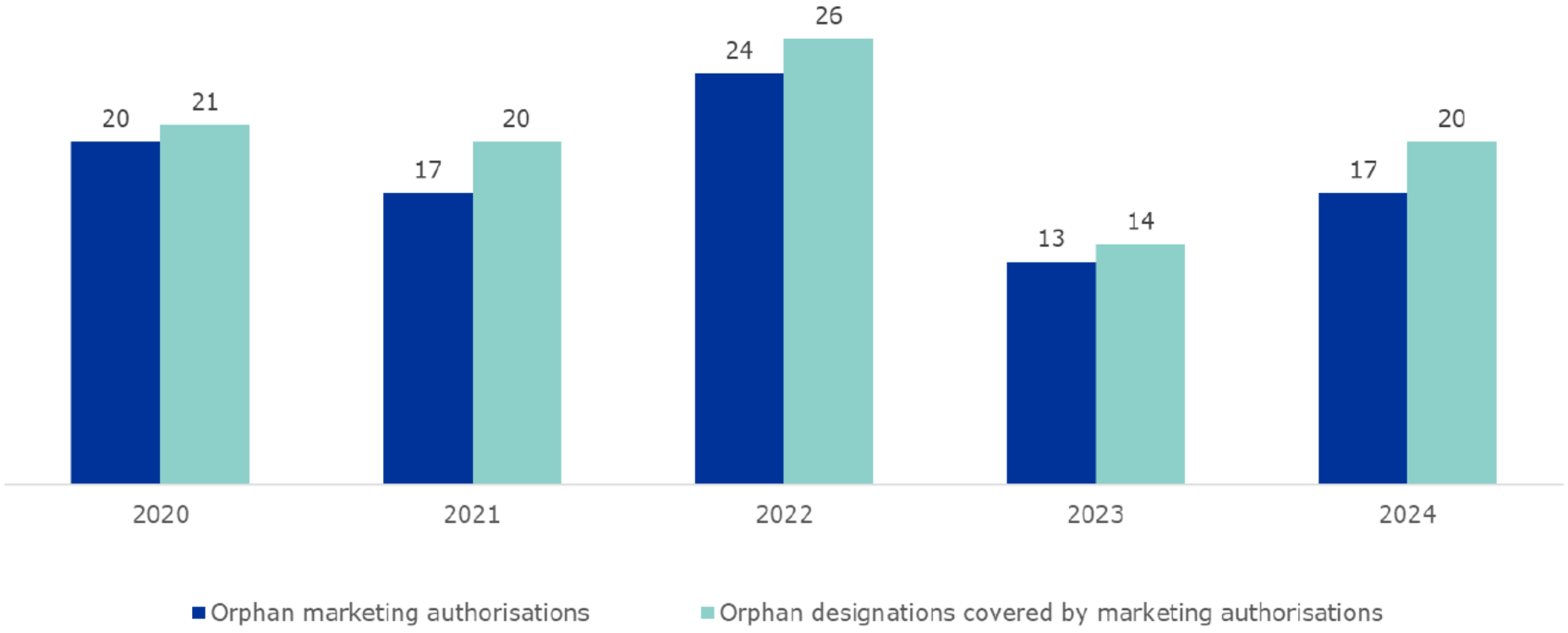
Overview of orphan medicinal product (OMP) activities in 2024

	2000 –2010	2010 - 2020	2021	2022	2023	2024	Total
Applications for designation submitted	1234	2444	251	269	195	193	4,586
Commission Decisions on designation	828	1554	170	182	137	141	3,012

Distribution of COMP opinions in 2024 – MedDRA classification



Designated orphan medicines authorisation activities



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OMP

COMP draft work plan 2025

Progress Work Plan 2025 – Carry over from 2024

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Initiative	Activity / additional information
1.1.1 Optimise the quality of initial orphan designation applications and maintenance by sharing COMP experience with stakeholders	• Emerging discussion on gene independent medicinal products and defining conditions for the purpose of orphan designation. Agreement on a suitable way to currently designate conditions for these type of products. This activity will be subject to new submissions for such products in the first half of 2025. • Mapping the orphan designations for very rare conditions
1.1.1 Explore cases and process options for generation of real-world evidence (RWE) in orphan designation decision making	• Provide expert input from COMP to the yearly review of the experience gained with RWD studies conducted across the regulatory network to support regulatory decision making; • Provide expert input in support of the development of guidance on use of RWE for regulatory purpose; • Provide expert input in the implementation of the recommendations from the HMA/EMA Big Data Steering Group in accordance with the Big Data work plan deliverables for 2024; • Explore the use of real-world data (RWD) sources for disease epidemiology and drug utilisation studies of potentially obsolete products. • Drug utilization study
2.1.1 To maintain and ensure evidence standards in view of methods used to support significant benefit claims.	• Collaboration with the Patient Experience Data initiative • Establishing the use of patient experience data for orphan medicines in regulatory purposes through a patient-validated methodology. • CollaboRARE

Work Plan 2025 - Carry over from 2024 & beyond

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Initiative	Activity / additional information
1.1.1 Ensure consistency, transparency and quality of the grounds of opinions	**2024** • Defining the requirements for **major contribution to patient care** (MCPC) • Publish: orphan drug designations flowchart on the EMA website and a commentary on MAAs **2025** • Review the orphan conditions in the setting of treatment modalities like treatment in "solid organ transplant", "haematopoietic stem cell transplantation", and conditions resulting from invasive/surgical procedures

Work Plan 2025 – New Initiatives Proposal

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Initiative	Activity / additional information
2.1.1 To develop guidance for appropriate methods to perform indirect comparisons	• Contribute to an analysis and scoping exercise to initiate the work towards the development of guidance on indirect comparisons. • Explore feasibility of adequate indirect comparisons in the field of rare diseases and when they should be used in the context of significant benefit. • Explore collaboration and knowledge sharing with HTA colleagues on the assessment of indirect comparisons.

EMA
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Foster patient involvement at the COMP

Action plan

Operationalising patient involvement in COMP

1. orphan designation initial stage (ODi)
2. orphan designation maintenance (ODm)

Support internal (EMA/COMP) patient involvement

- Define potential criteria to identify early patient involvement at ODi/ODm
- Potential use of CollaboRARE at validation stage

Support developers and foster patient involvement

- Include information about patient involvement at the COMP report
- Link to available resources (IRDiRC ODGuidebook, ERNs, etc.)

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Thank you

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