

Orphan Medicinal Product Designation

Overview 2020-2025

Orphan Medicines – Scientific Evidence Generation

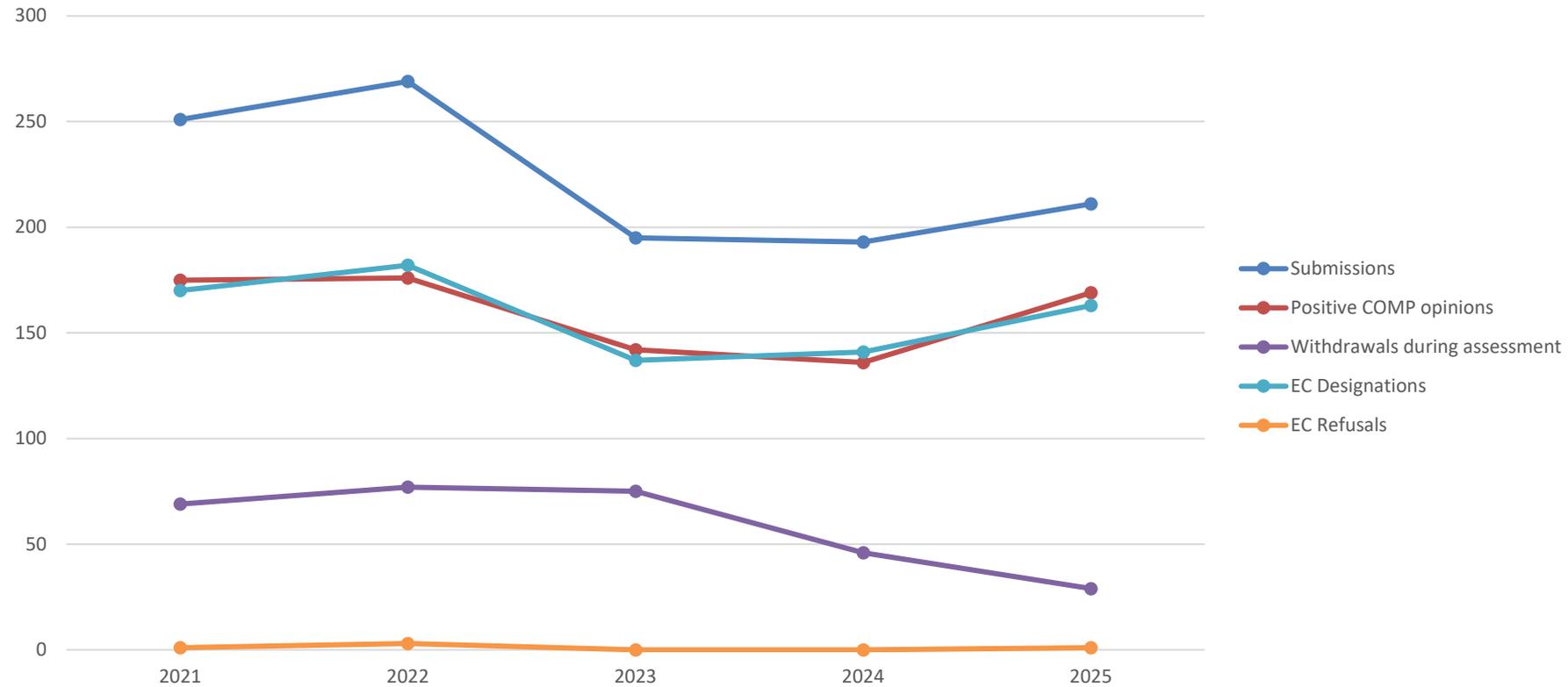


- Overview of orphan medicinal product designations
- Overview of orphan marketing authorisations

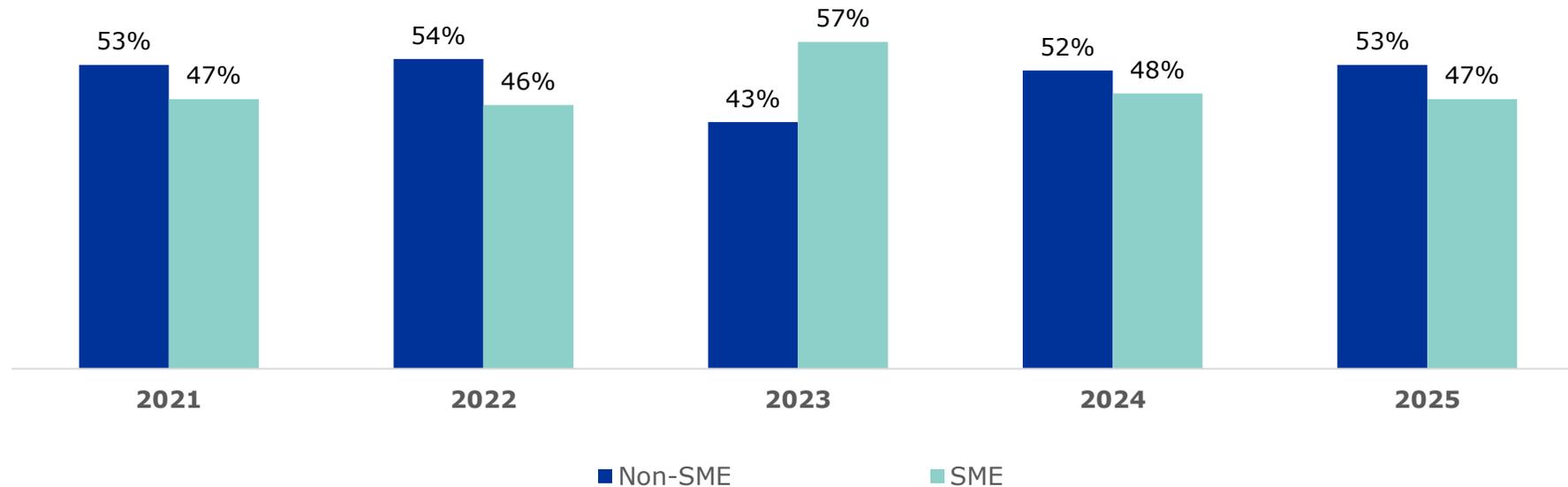
Overview orphan medicinal product designations

	2000-2005	2006-2010	2011-2015	2016-2020	2021	2022	2023	2024	2005	TOTAL
Submissions	548	686	1151	1293	251	269	195	193	211	4797
Positive COMP Opinions	348	500	759	790	175	176	142	136	169	3195
EC Designations	343	485	768	786	170	182	137	141	163	3175
EC Refusals	8	6	9	12	1	3	0	0	1	40
Withdrawals after submission	150	144	313	476	68	74	75	46	29	1375

Overview orphan medicinal product designations



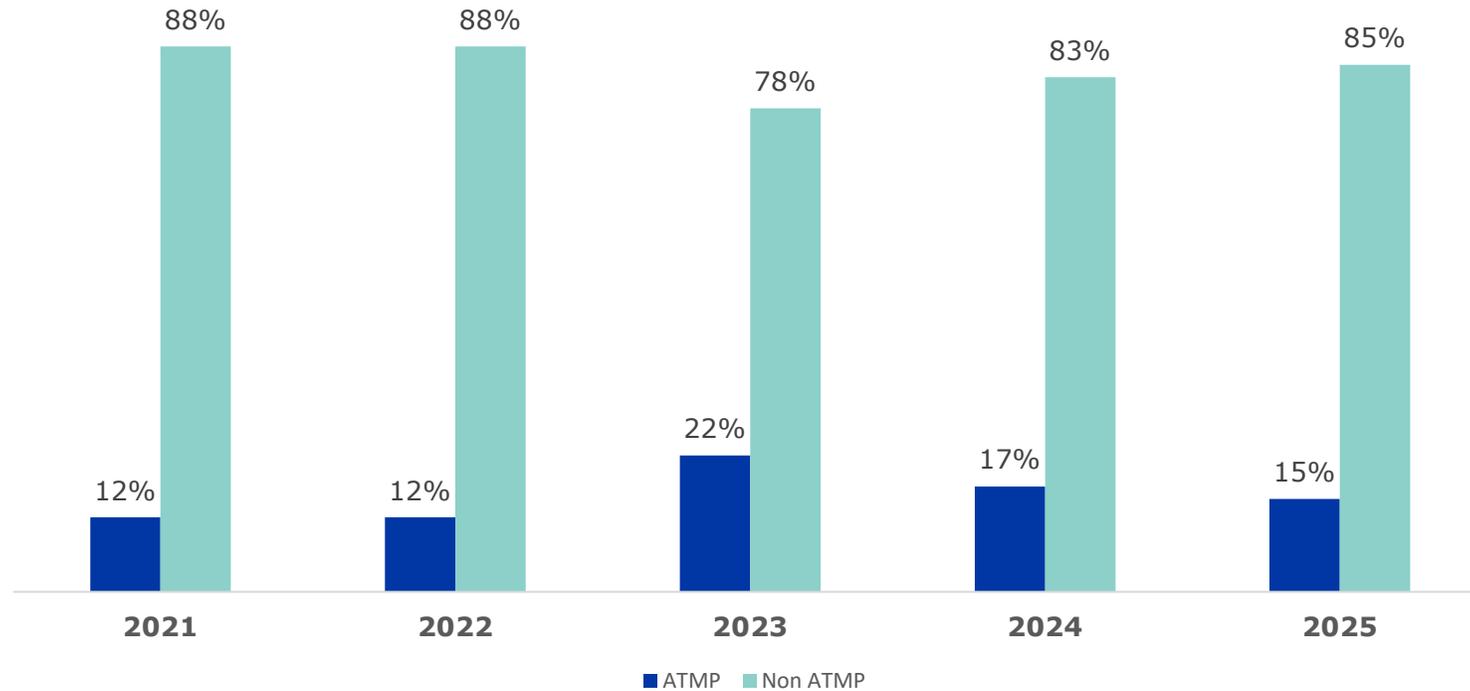
Orphan designation submissions from SME sponsors



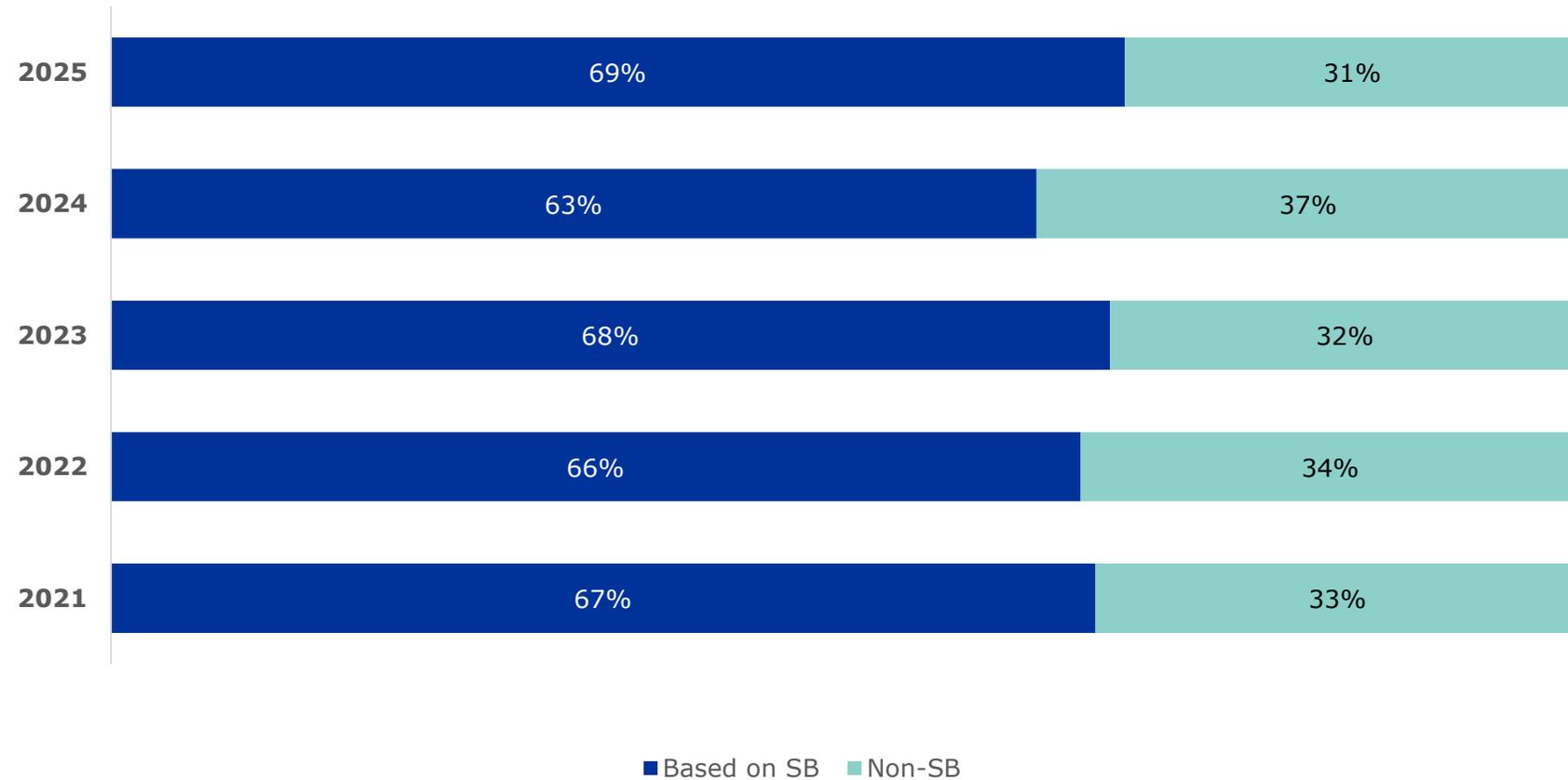
Orphan designation new designated conditions



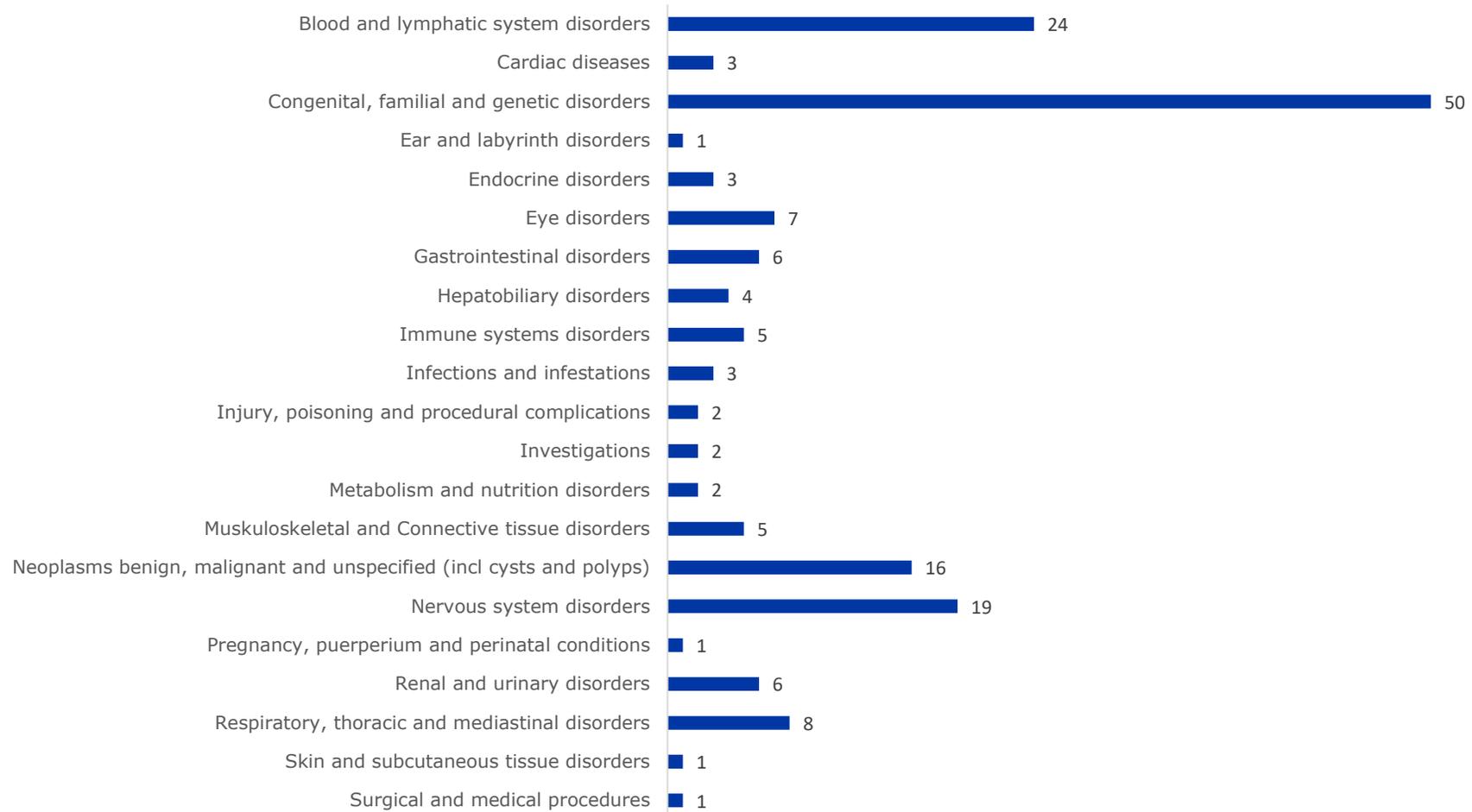
Orphan designation submissions with and ATMP like substance



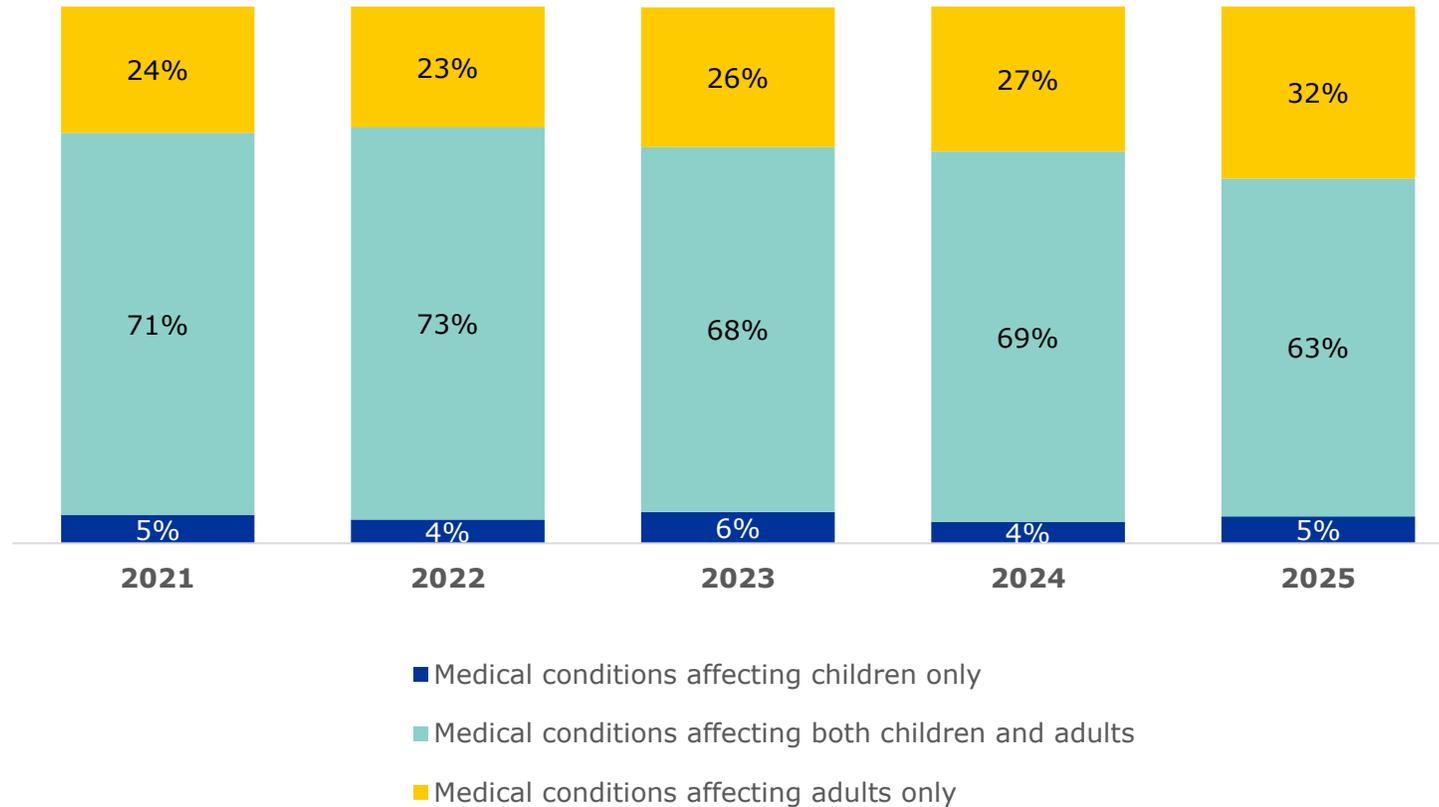
Orphan designations based on significant benefit



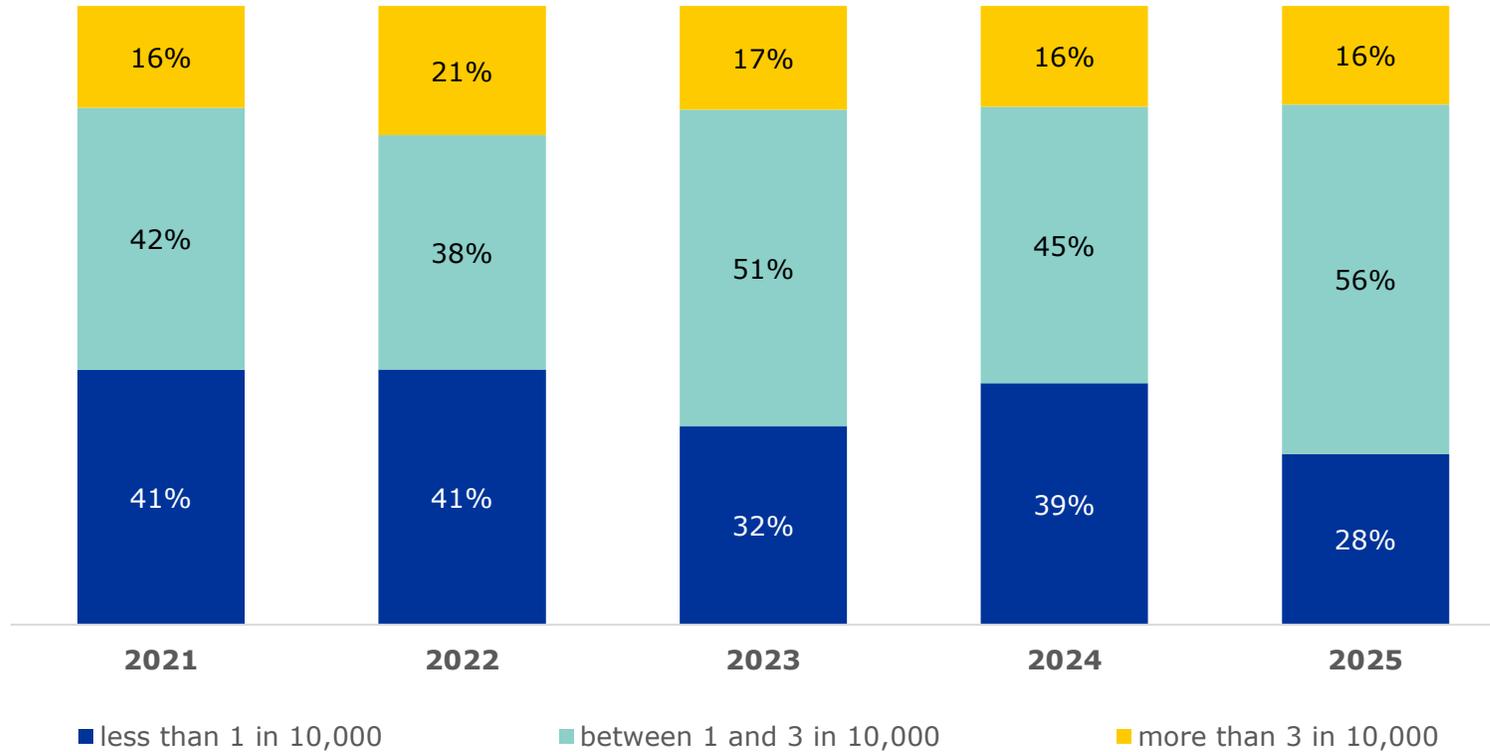
COMP opinions on orphan designation 2025 by MedDRA classification



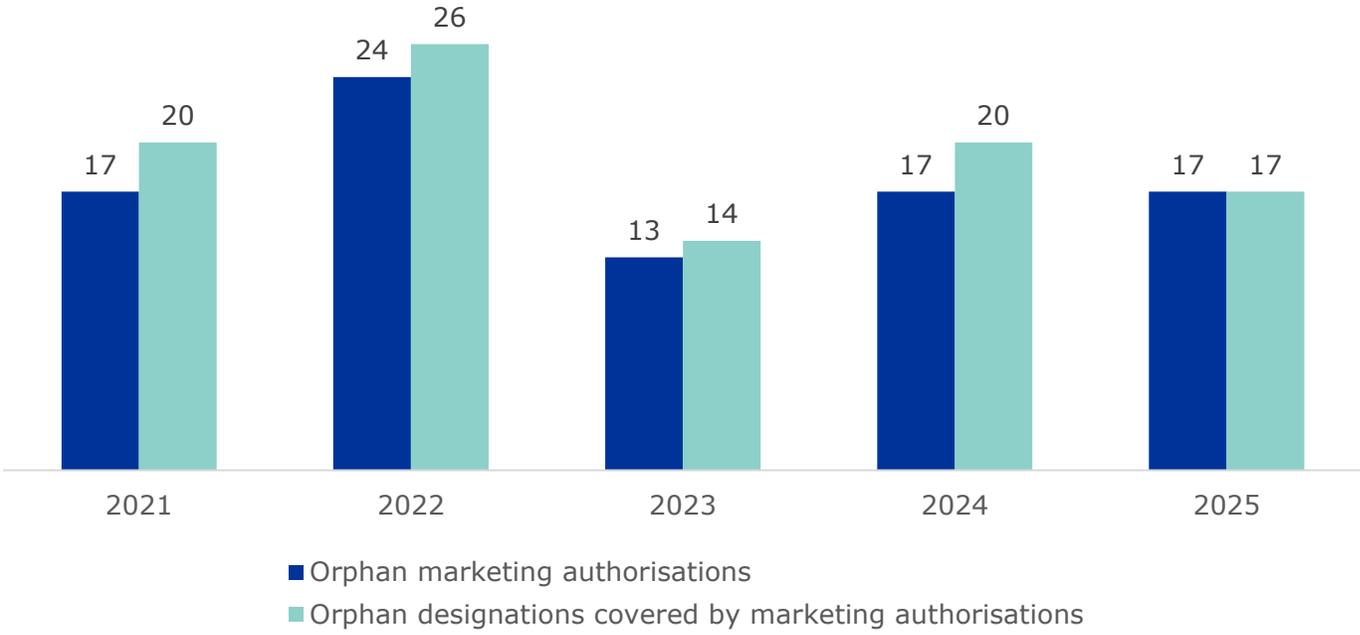
Orphan designations by age range



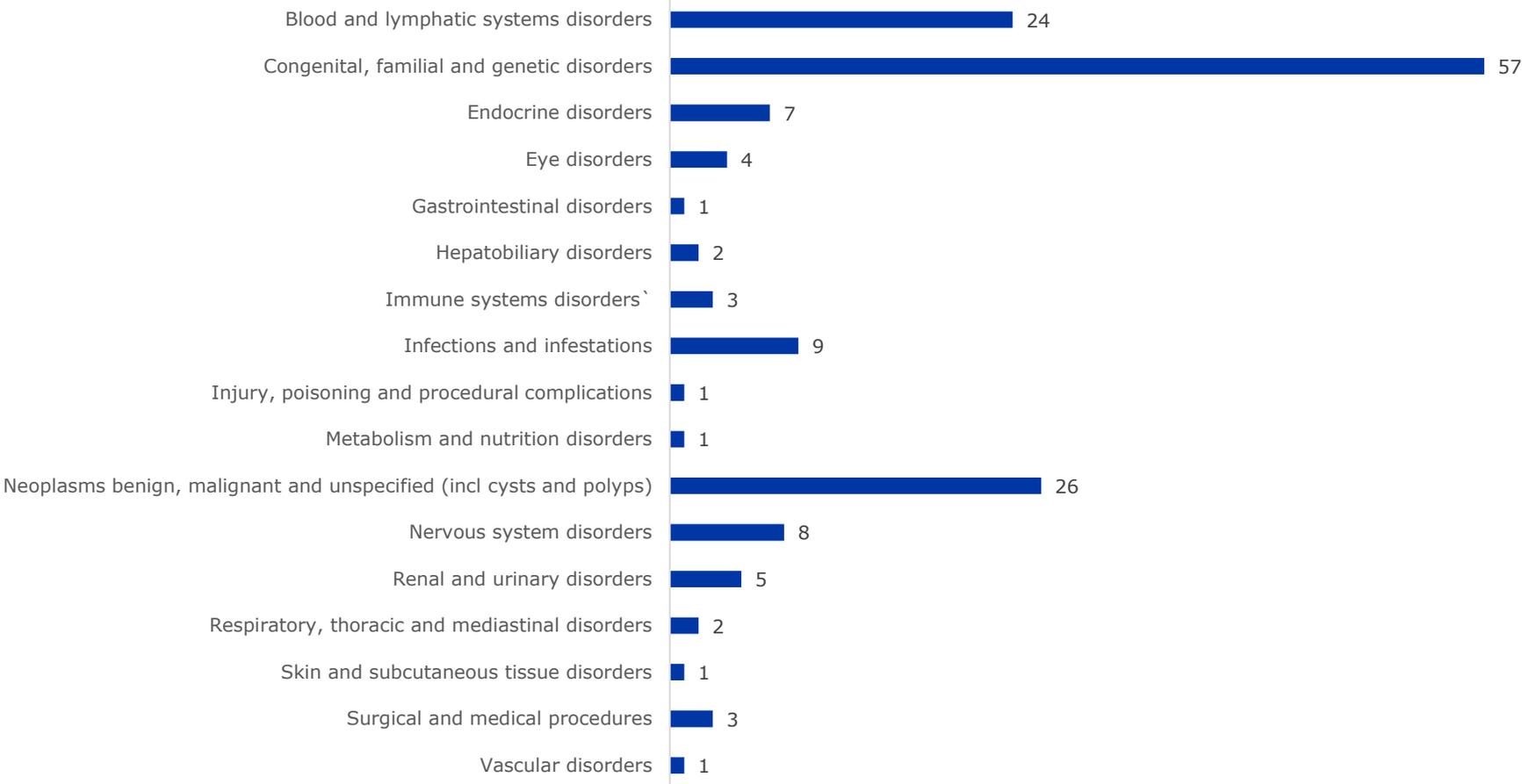
Prevalence for designated orphan conditions



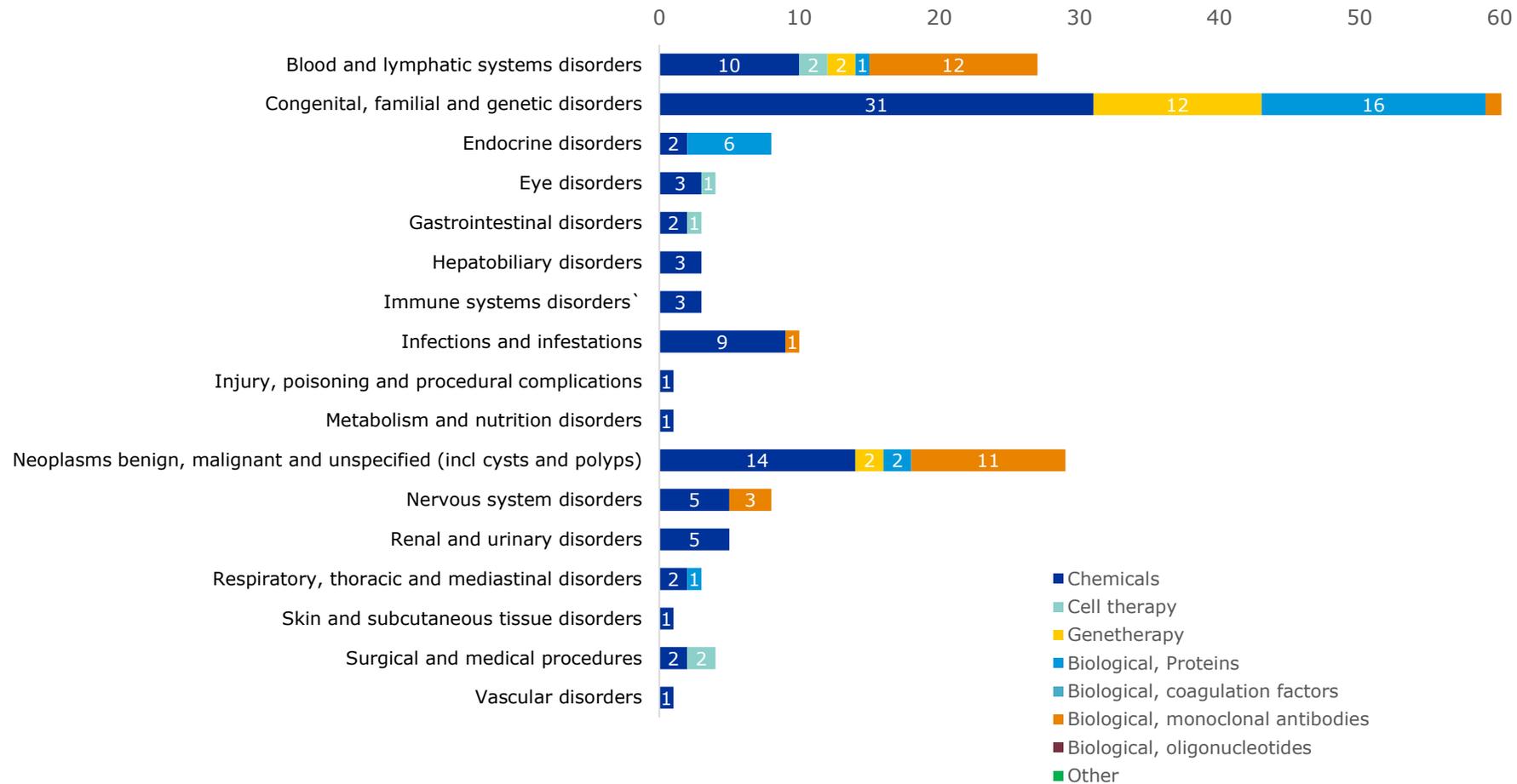
Authorised orphan medicinal products



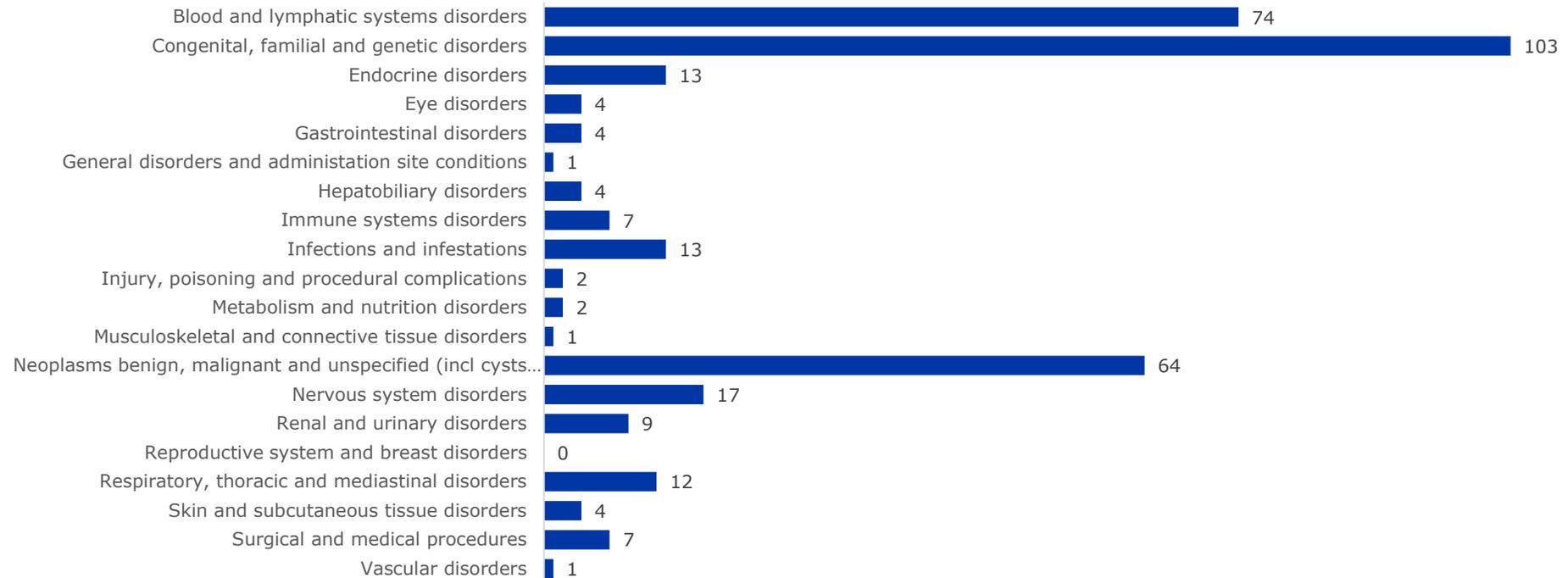
Active authorised orphan MAA by MedDRA classification



Active authorised orphan MAA by type of product

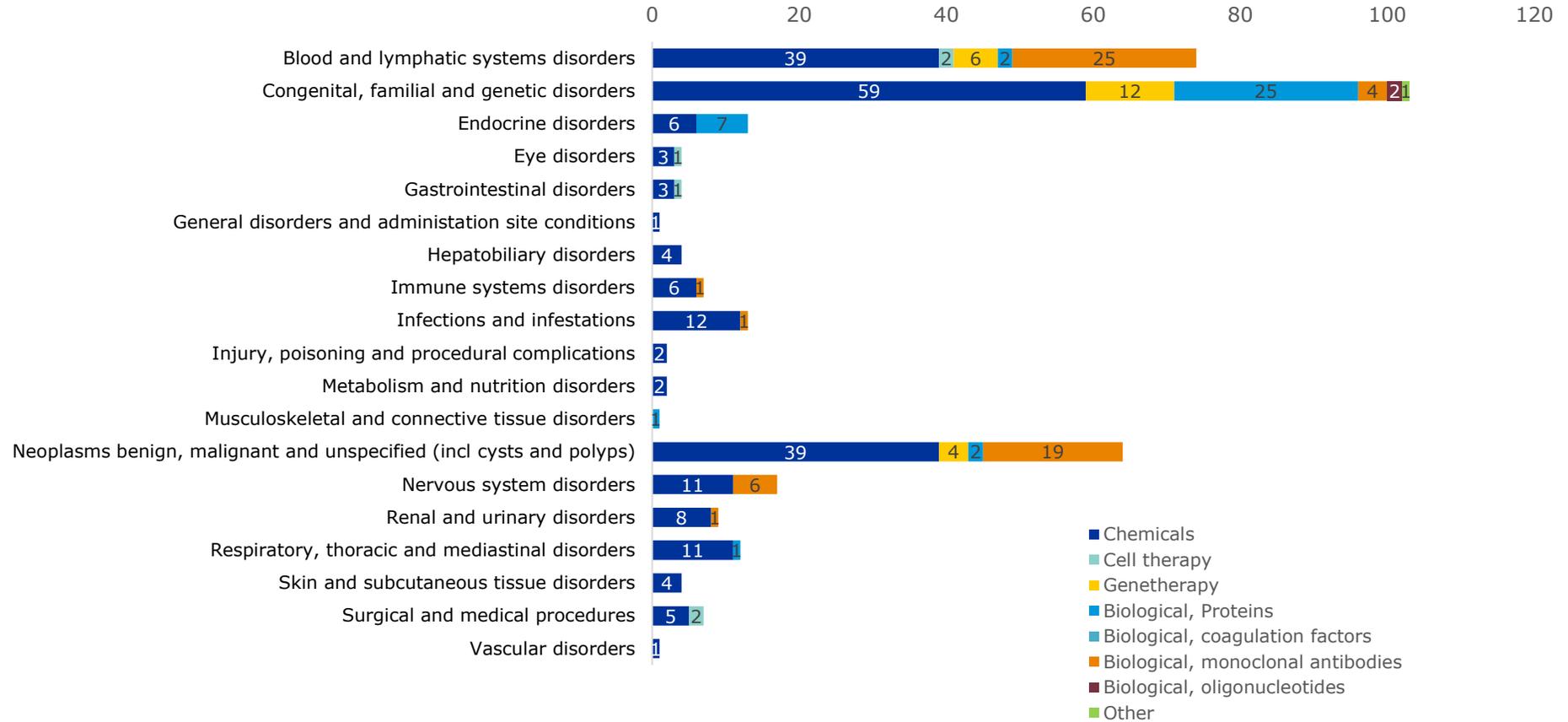


Initial authorised orphan MAA and extensions of indication granted to date by MedDRA classification



Number conditions: 176

Initial authorised orphan MAA and extensions of indication granted to date by type of product



Orphan marketing authorisations granted

278

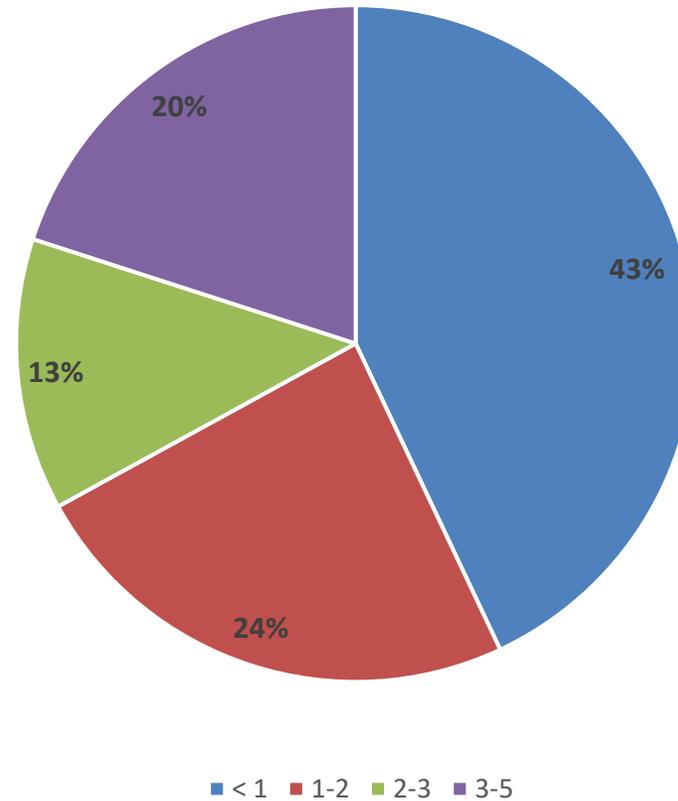
initial marketing
authorisations granted

64

extensions of
indication granted

- **155 active initial marketing authorisations**
- **37 active extensions of indication**
- **35 withdrawals** from the register of orphan medicinal products (including **15 extensions of indication**)
- **14 withdrawals** of marketing authorisations from register medicinal products human use/ orphan status expired
- **3** marketing authorisations **revoked** from the register of medicinal products for human use/ orphan status expired
- **1** marketing authorization **suspended**
- **3** marketing authorisations **non-renewed**
- **82 removals of orphan status for initial MAA** from register after expire of the market exclusivity period plus **12 extensions of indication**

Prevalence for marketing authorisations





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