

Orphan Medicinal Product Designation

Overview 2020-2024

Orphan Medicines – Scientific Evidence Generation

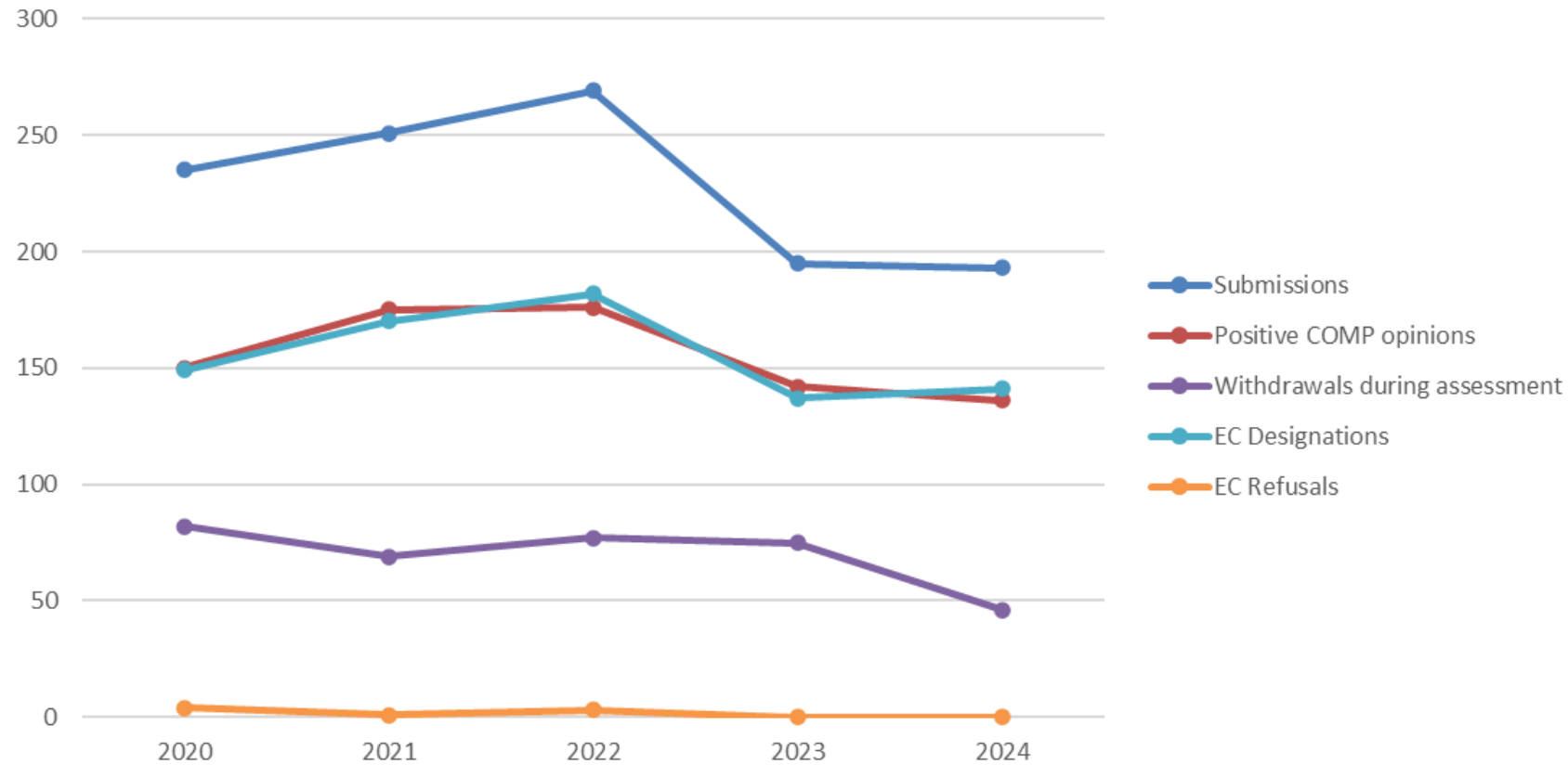


- Overview of orphan medicinal product designations
- Overview of orphan marketing authorisations

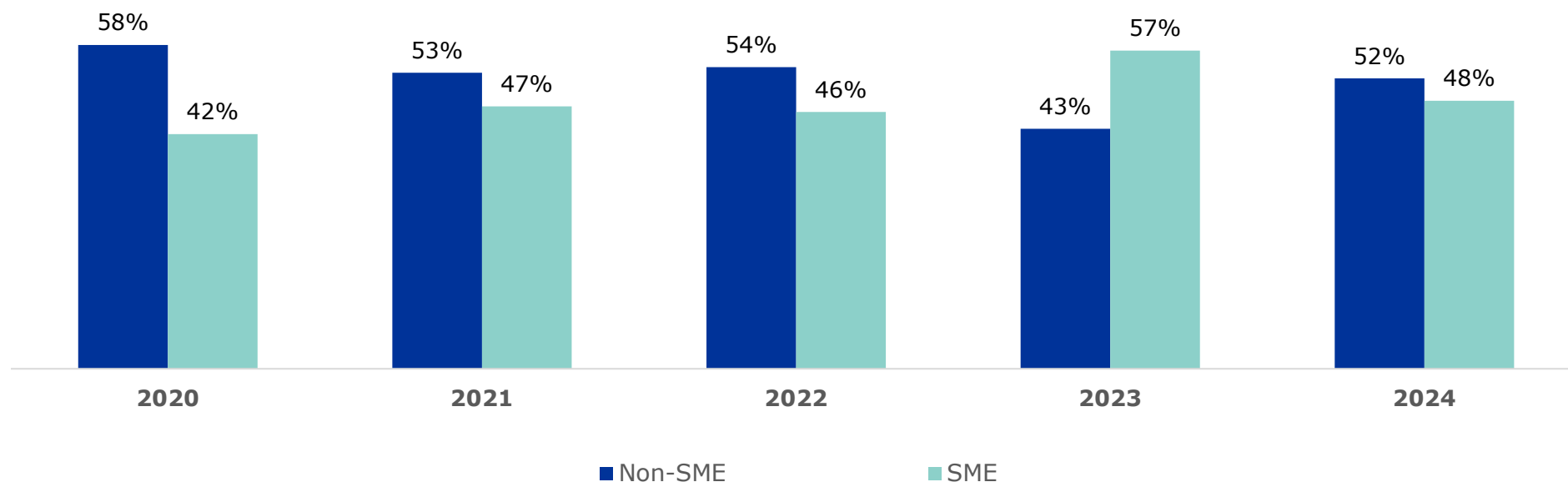
Overview orphan medicinal product designations

	2000-2005	2006-2010	2011-2015	2016-2020	2021	2022	2023	2024	TOTAL
Submissions	548	686	1151	1293	251	269	195	193	4586
Positive COMP Opinions	348	500	759	790	175	176	142	136	3026
EC Designations	343	485	768	786	170	182	137	141	3012
EC Refusals	8	6	9	12	1	3	0	0	39
Withdrawals after submission	150	144	313	476	68	74	75	46	1346

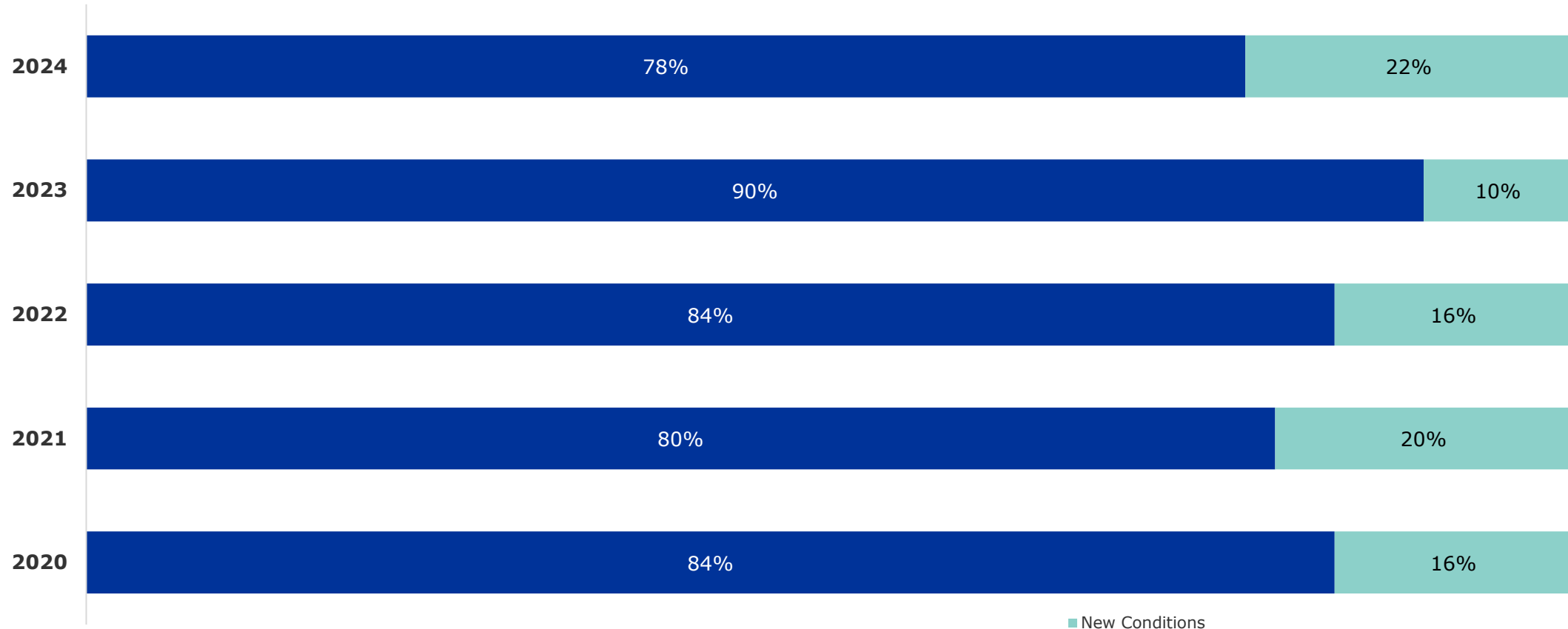
Overview orphan medicinal product designations



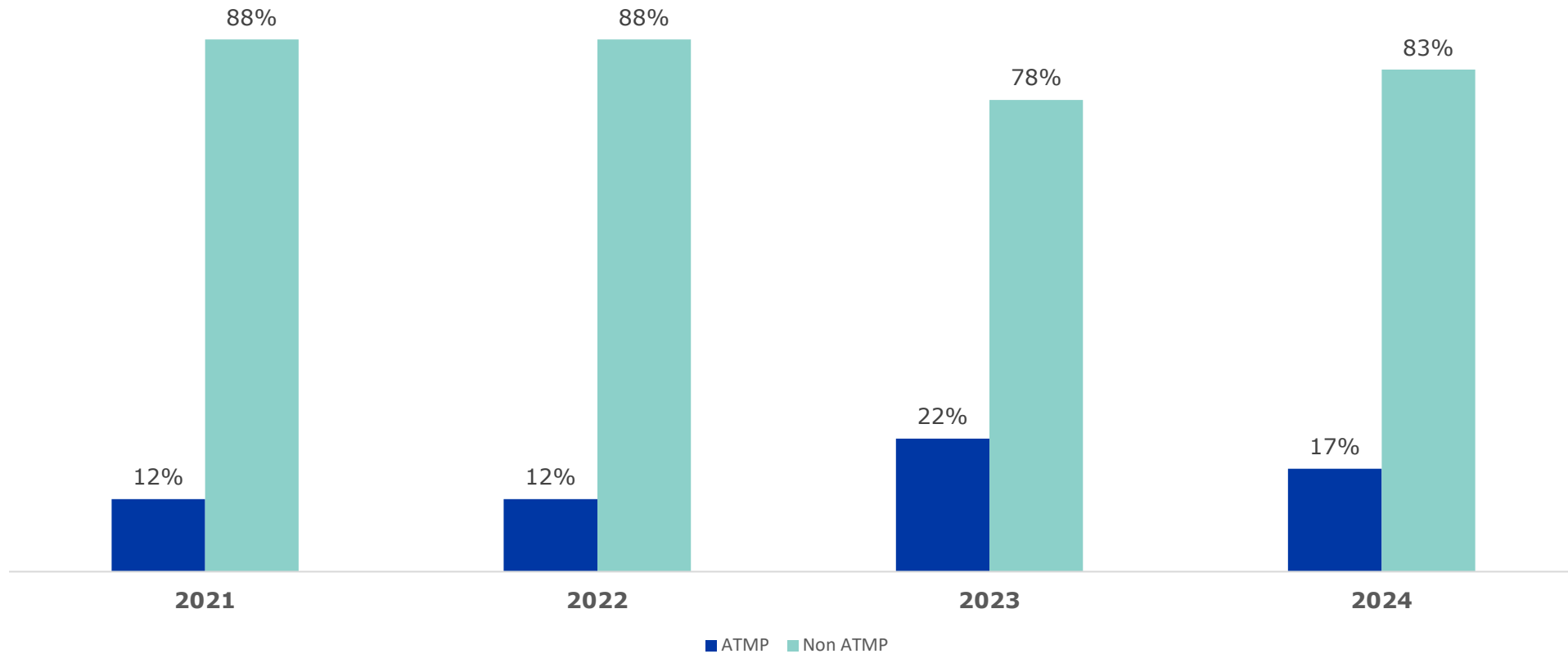
Orphan designation submissions from SME sponsors



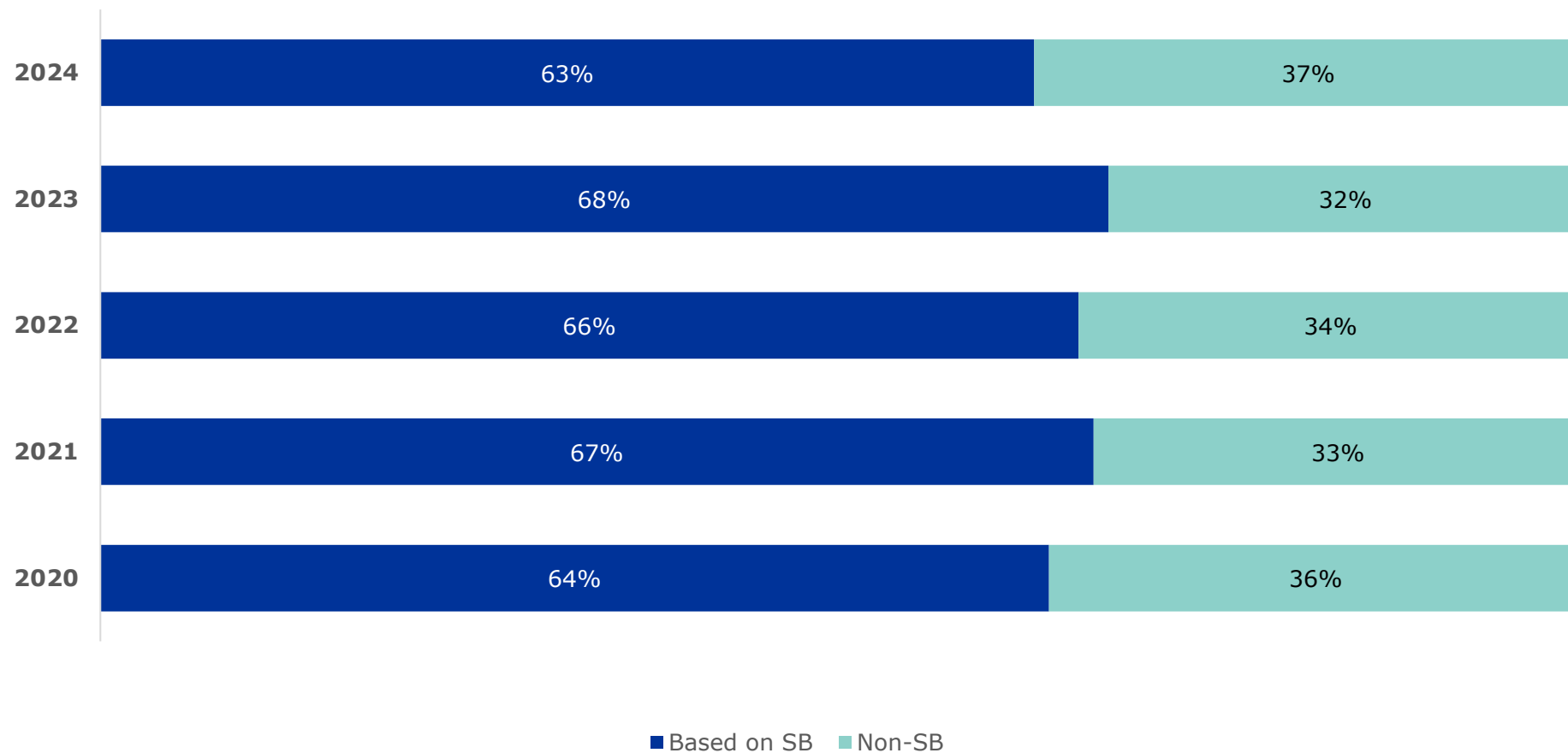
Orphan designation new designated conditions



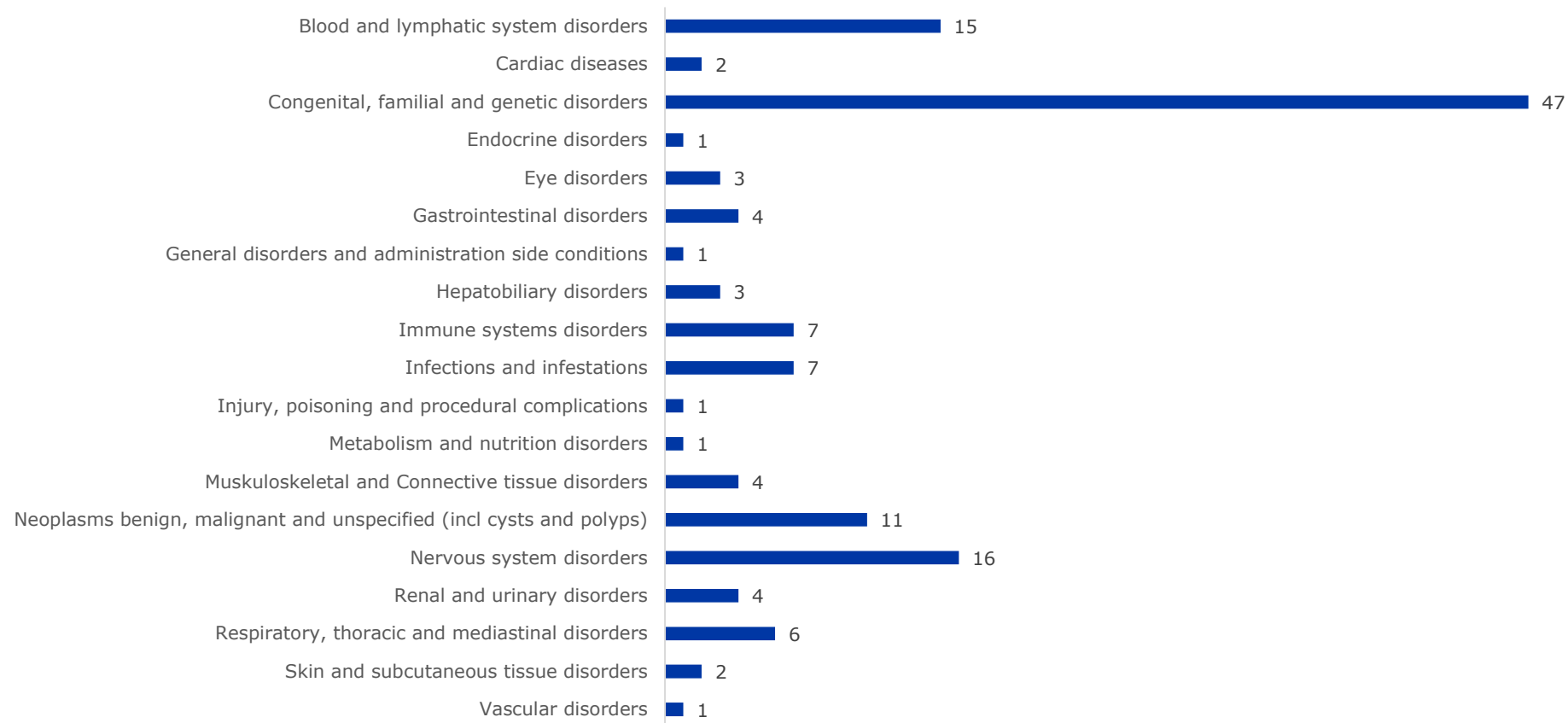
Orphan designation submissions with and ATMP like substance



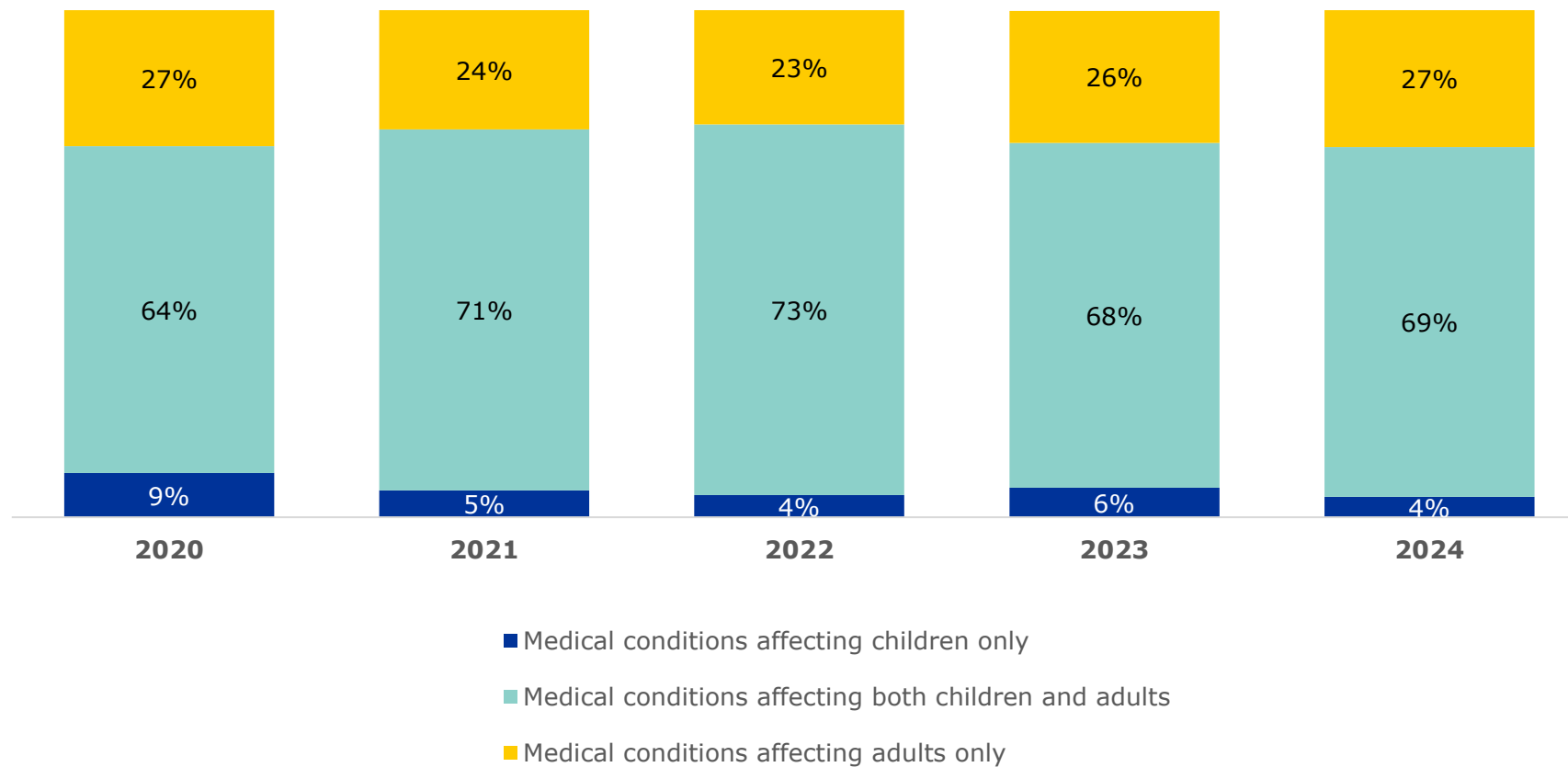
Orphan designations based on significant benefit



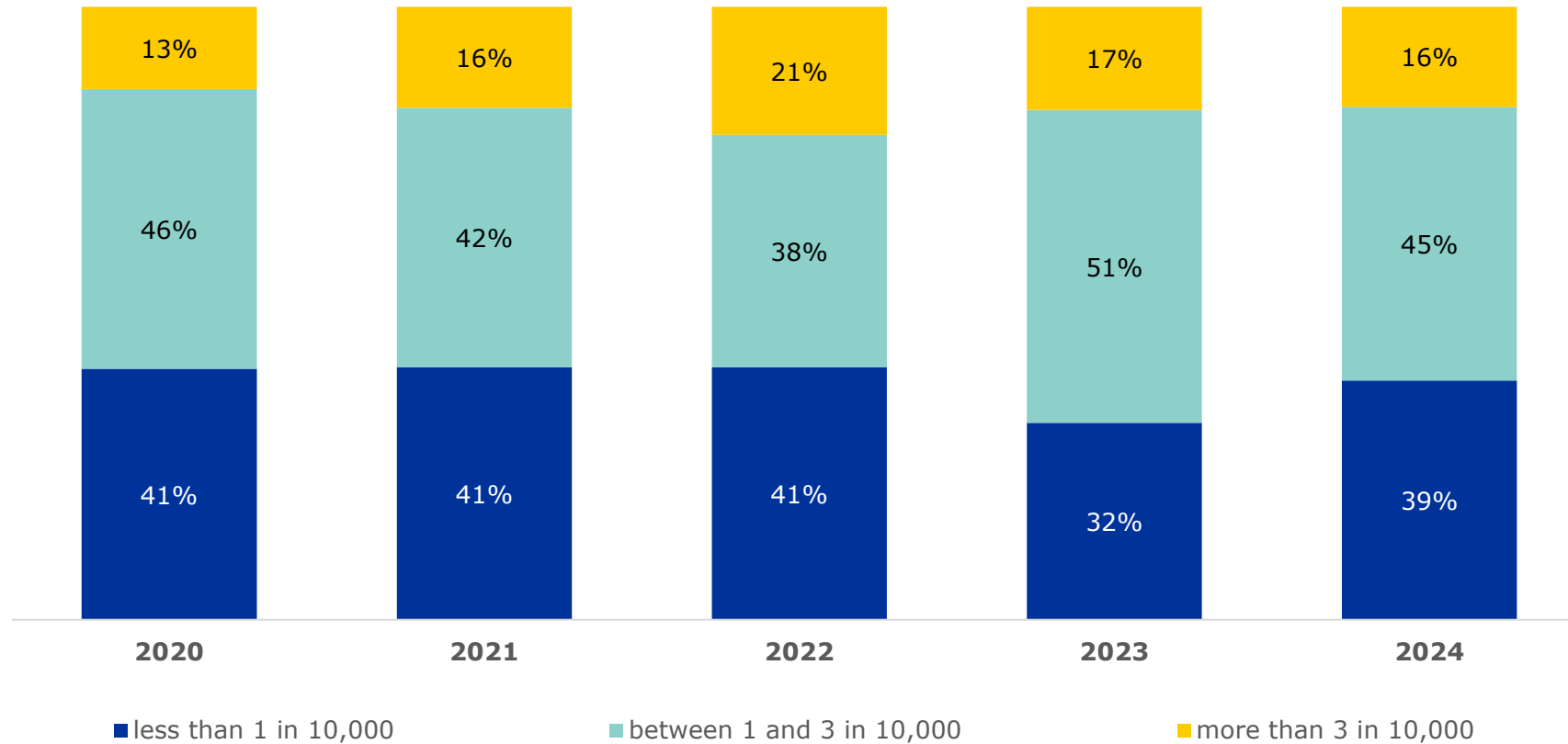
COMP opinions on orphan designation 2024 by MedDRA classification



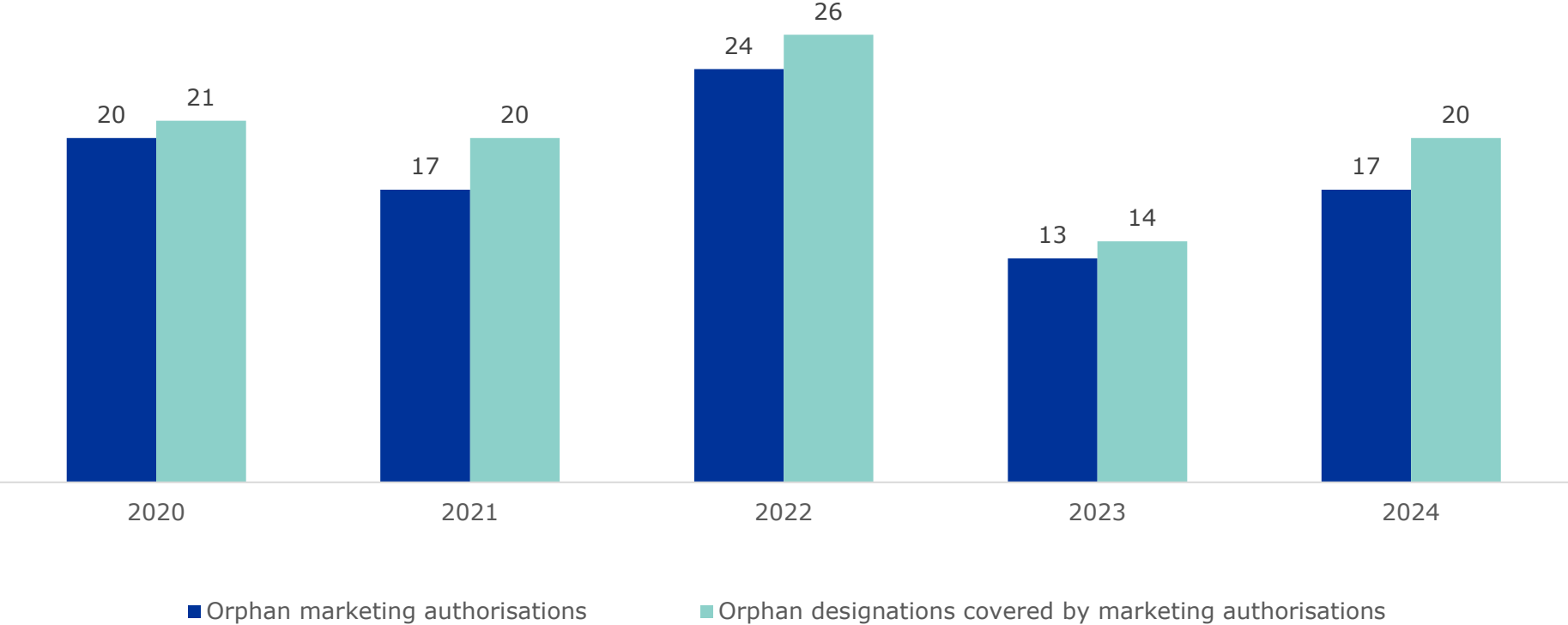
Orphan designations by age range



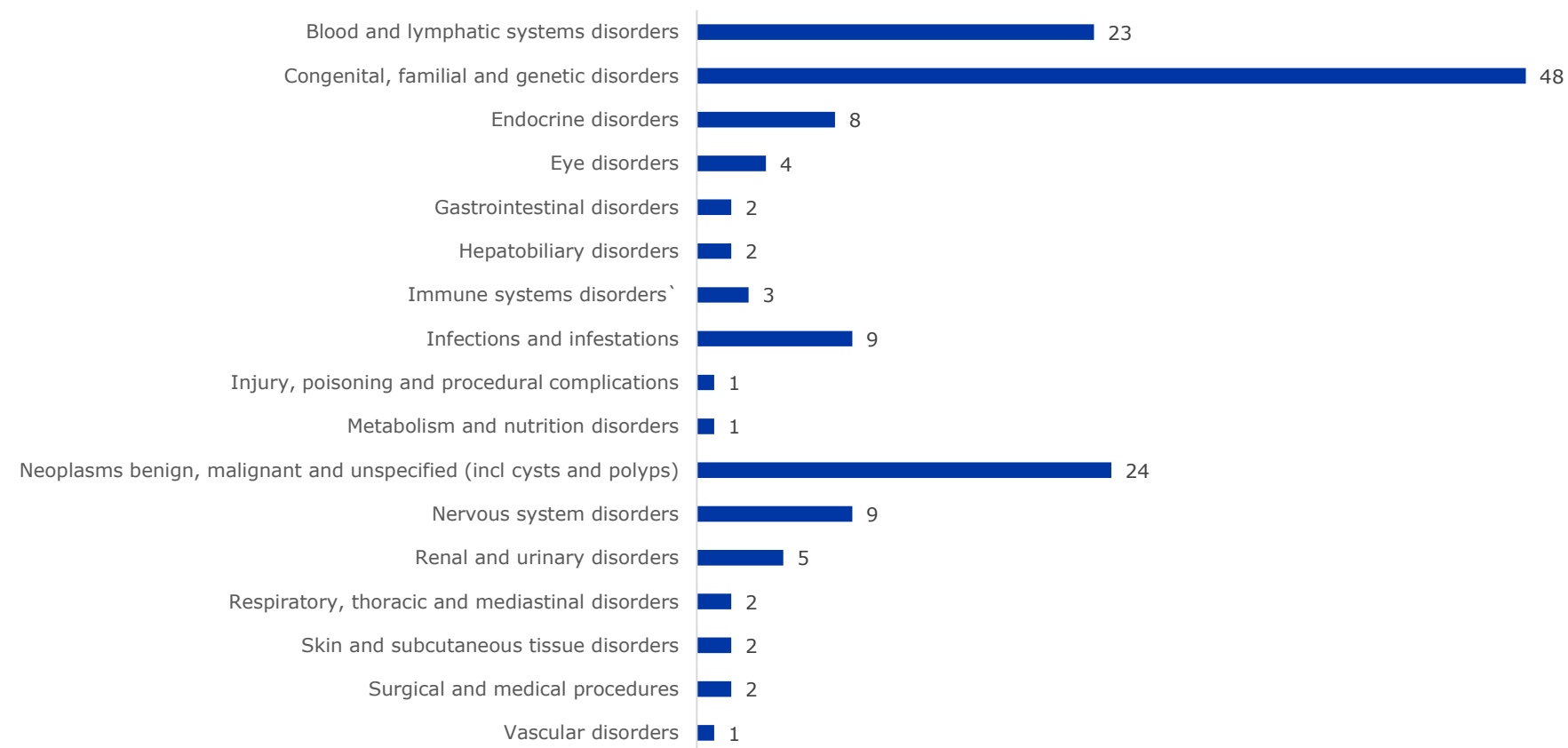
Prevalence for designated orphan conditions



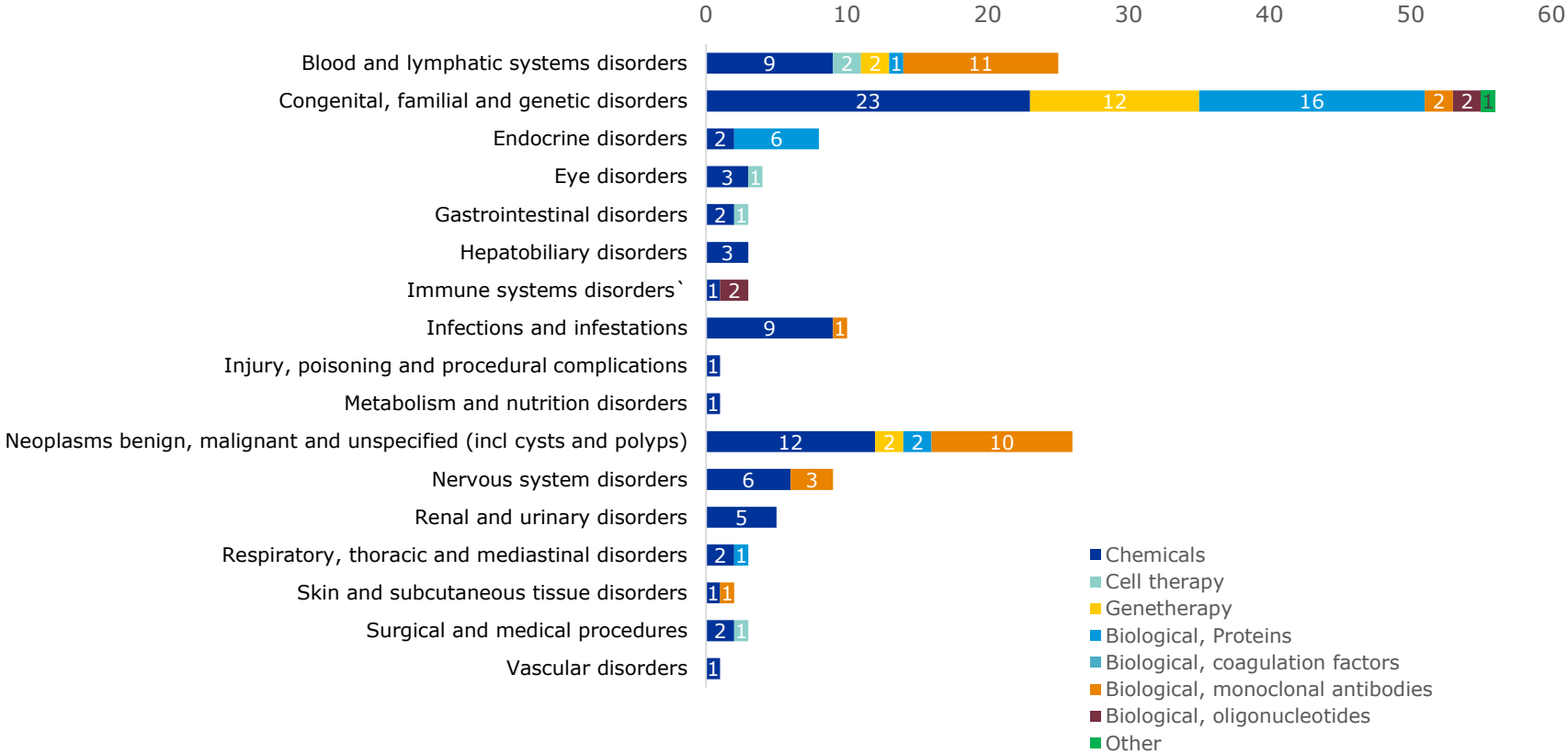
Authorised orphan medicinal products



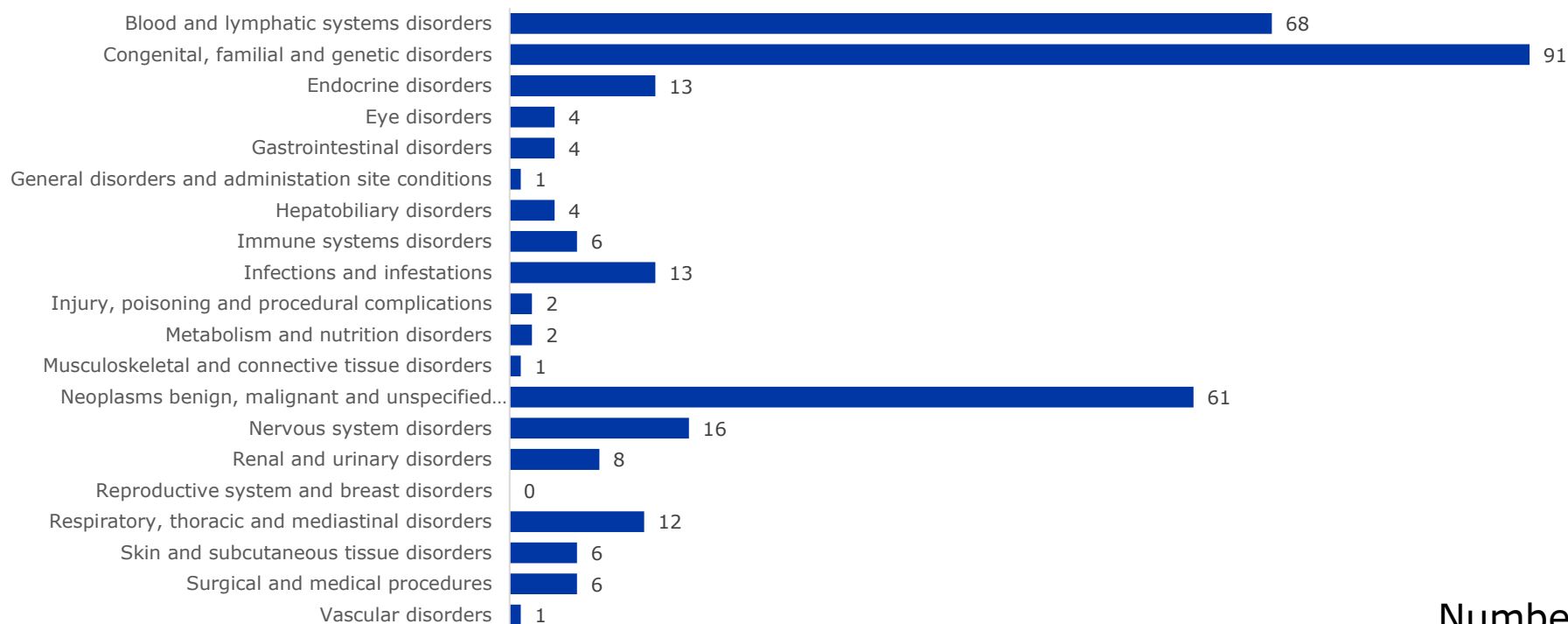
Active authorised orphan MAA by MedDRA classification



Active authorised orphan MAA by type of product

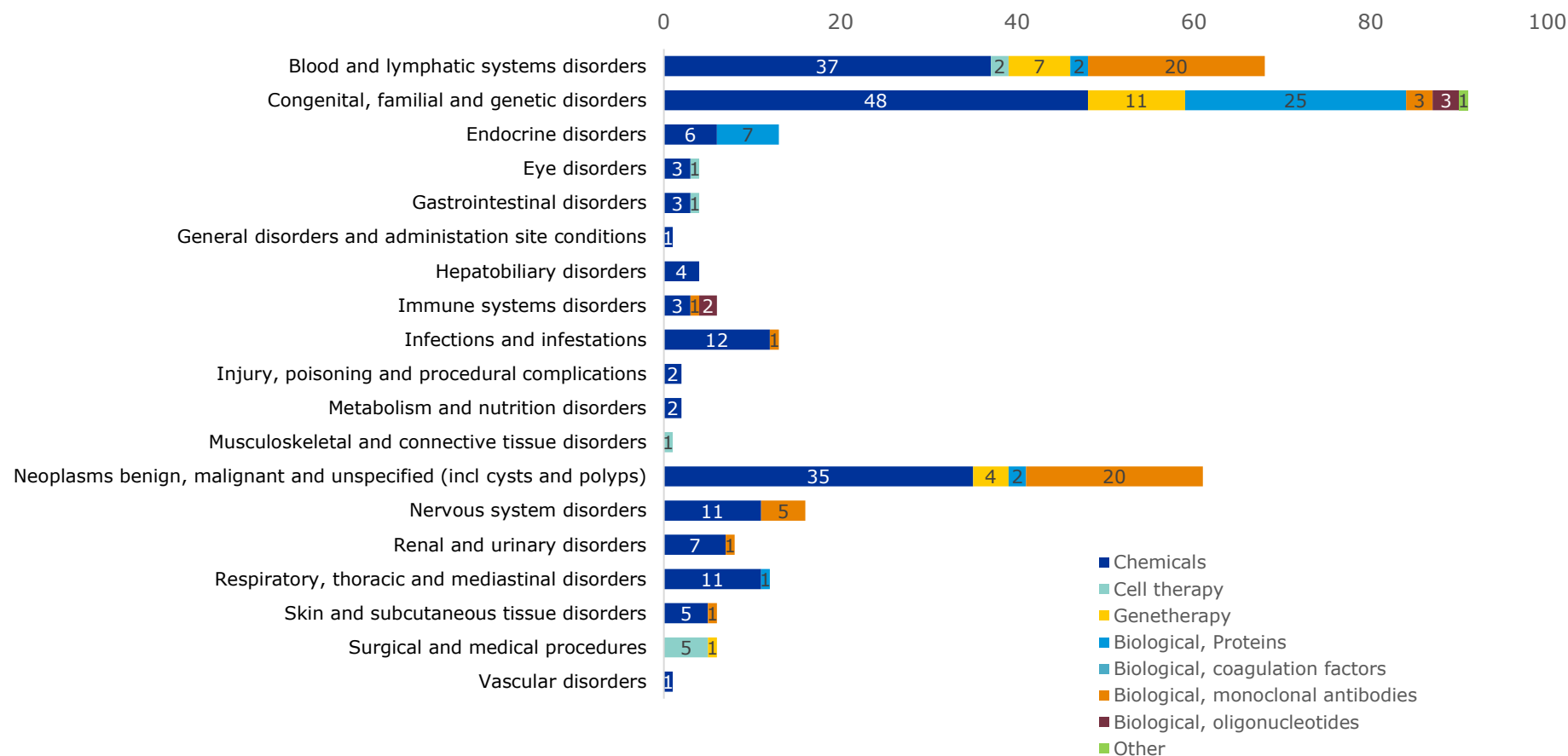


Initial authorised orphan MAA and extensions of indication granted to date by MedDRA classification



Number conditions: 170

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



Orphan marketing authorisations granted

261

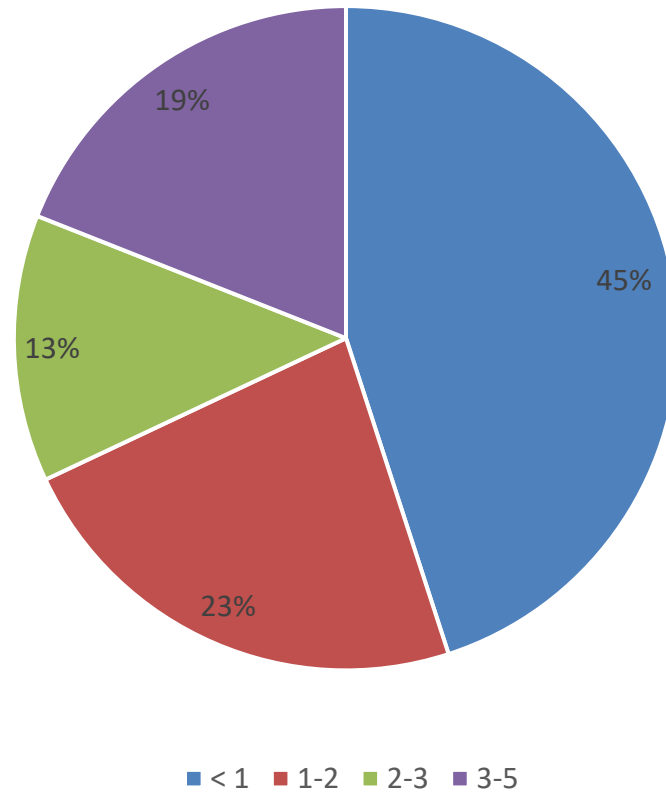
initial marketing
authorisations granted

58

extensions of
indication granted

- **163 active initial authorisations**
- **31 active extensions of indication**
- **35 withdrawals** from the register of orphan medicinal products (including **15 extensions of indication**)
- **13 withdrawals** from register medicinal products human use/ orphan status expired plus
- **3 revoked** from register medicinal products human use/ orphan status expired
- **1 suspended**
- **78 removals of orphan status for initial MAA** from register after expire of the market exclusivity period plus for **12 extensions of indication**

Prevalence for marketing authorisations





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