



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Orphan Medicinal Product Designation

Overview 2000-2023





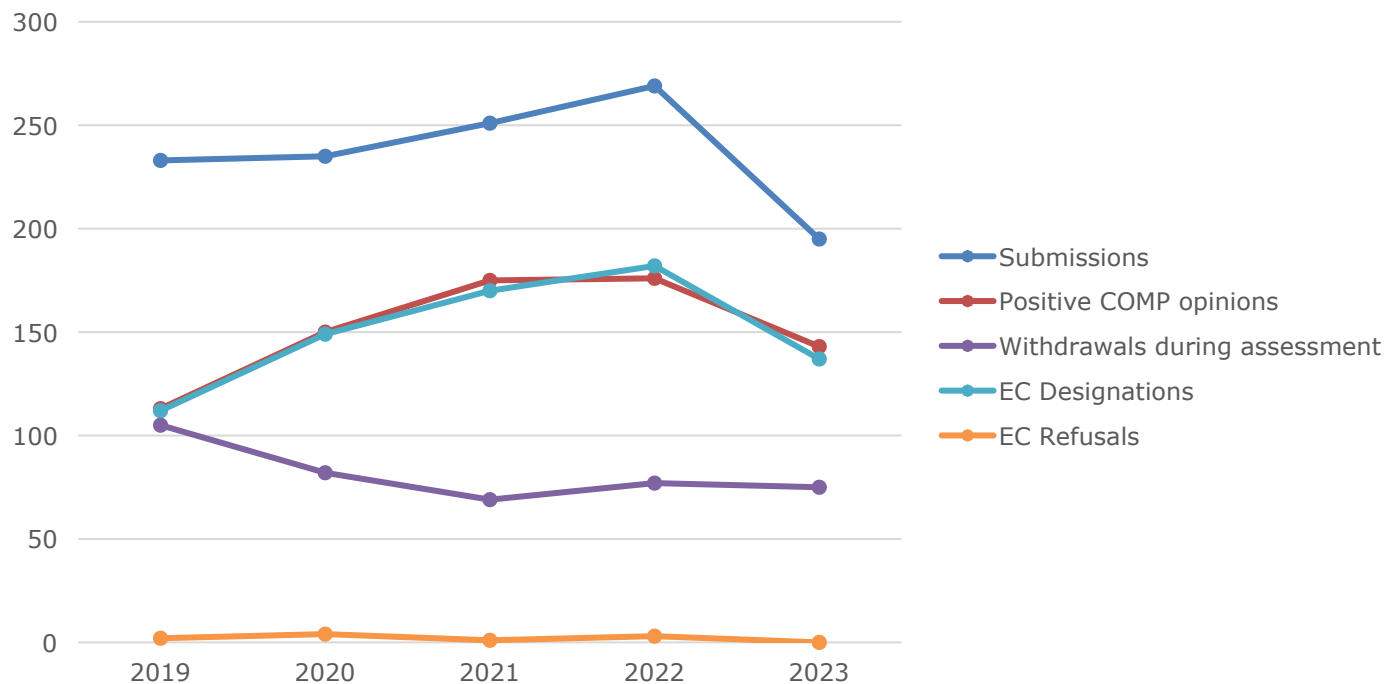
ORPHAN MEDICINAL PRODUCT DESIGNATION Overview

	2000-2005	2006-2010	2011-2015	2016-2020	2021	2022	2023	TOTAL
Submissions	548	686	1151	1293	251	269	195	4393
Positive COMP Opinions	348	500	759	790	175	176	142	2890
EC Designations	343	485	768	786	170	182	137	2871
EC Refusals	8	6	9	12	1	3	0	39
Withdrawals after submission	150	144	313	456	69	77	75	1284



ORPHAN MEDICINAL PRODUCT DESIGNATION

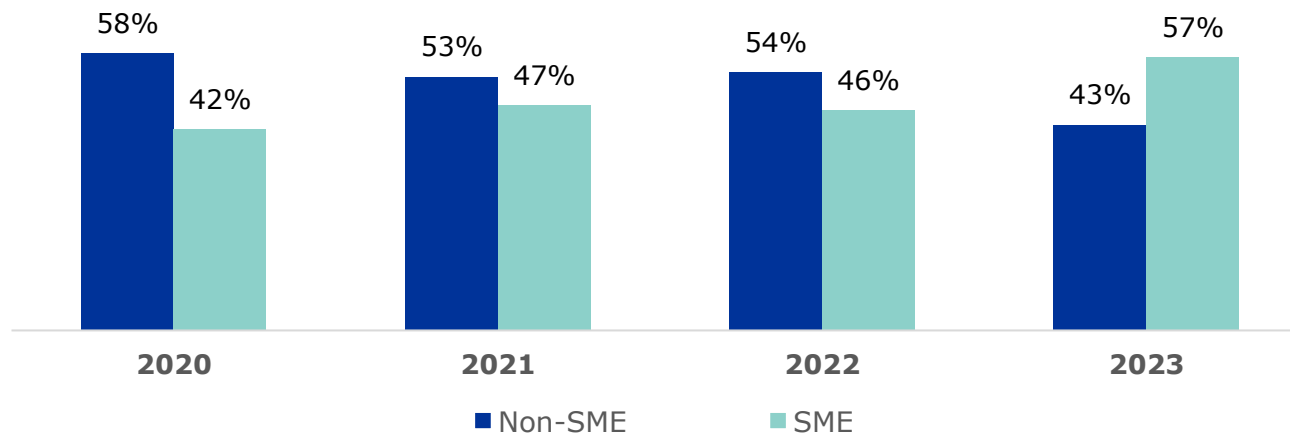
Overview





ORPHAN MEDICINAL PRODUCT DESIGNATION

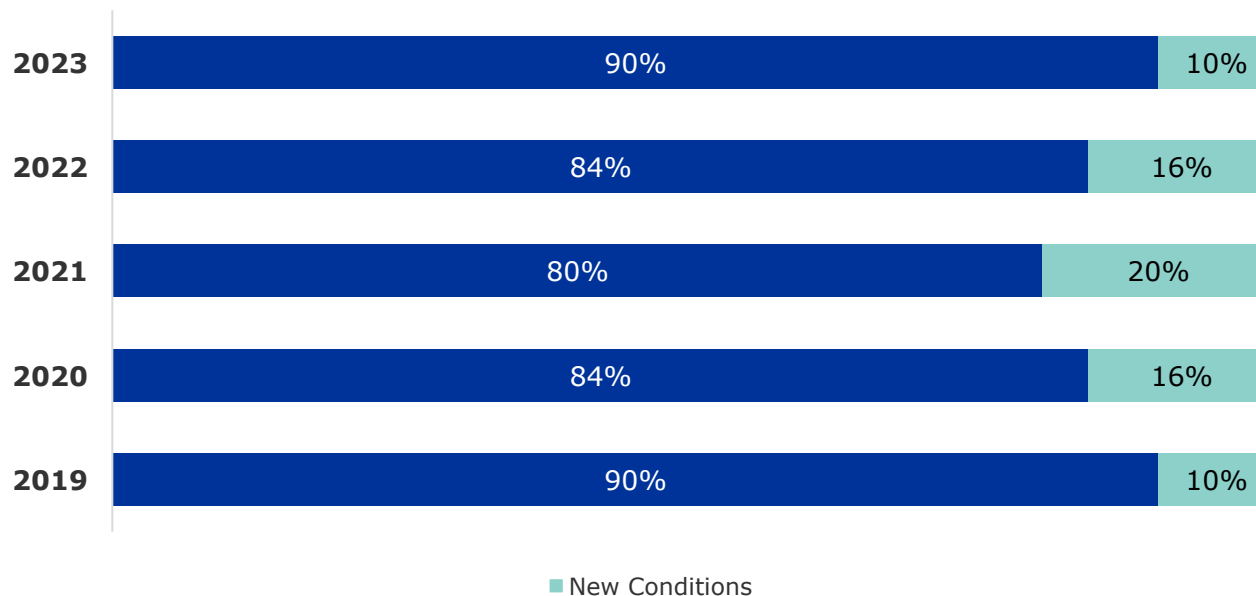
Submissions from SME sponsors





ORPHAN MEDICINAL PRODUCT DESIGNATION

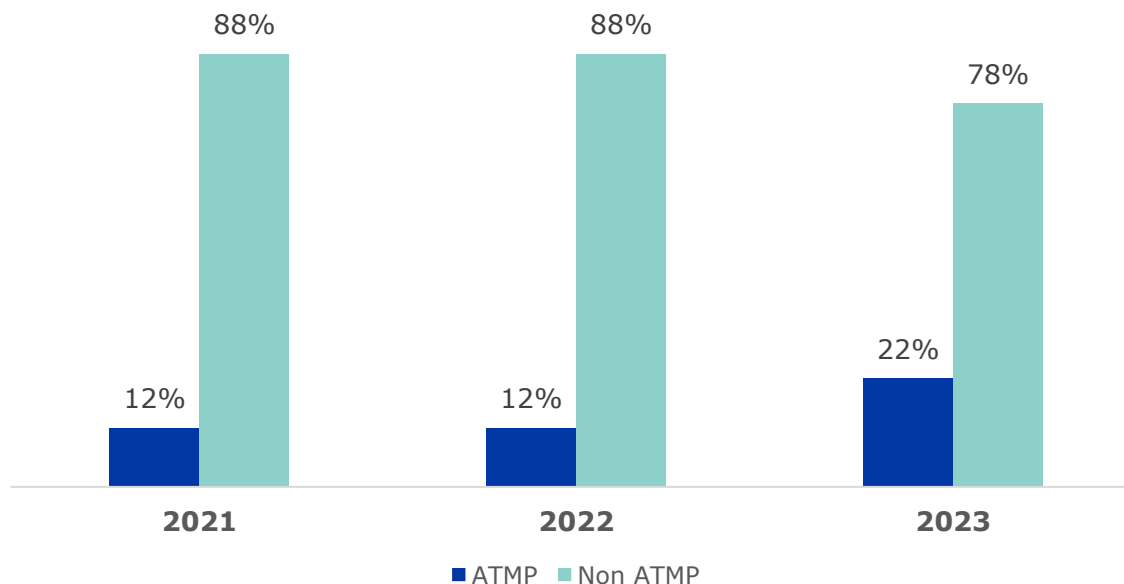
New designated conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

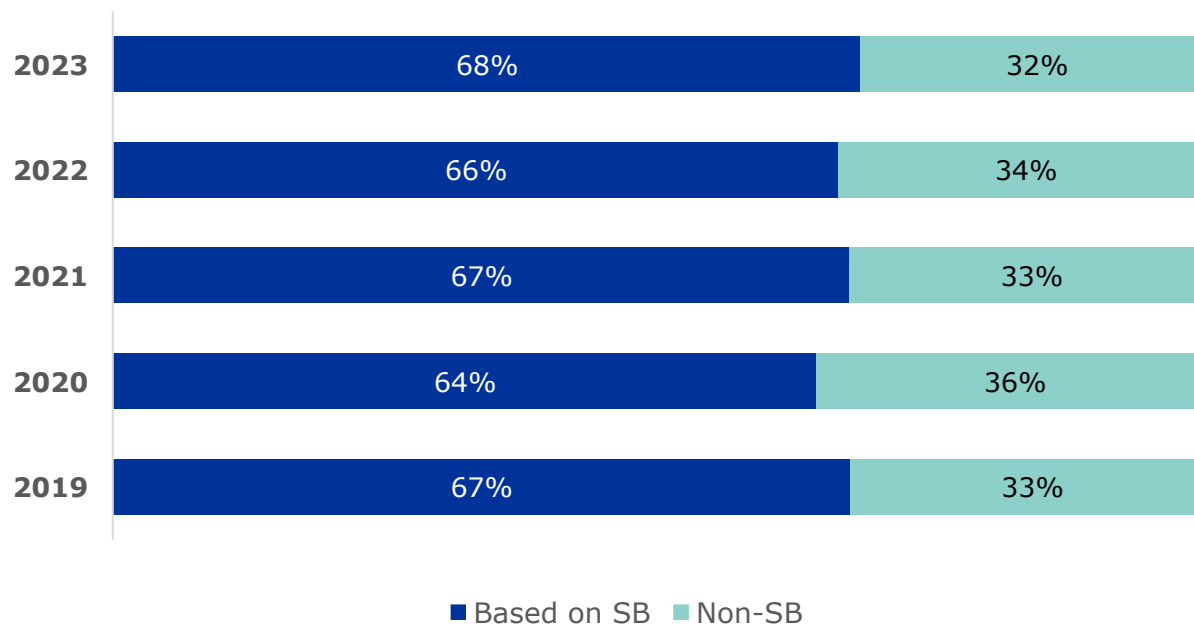
Submissions with and ATMP like substance





ORPHAN MEDICINAL PRODUCT DESIGNATION

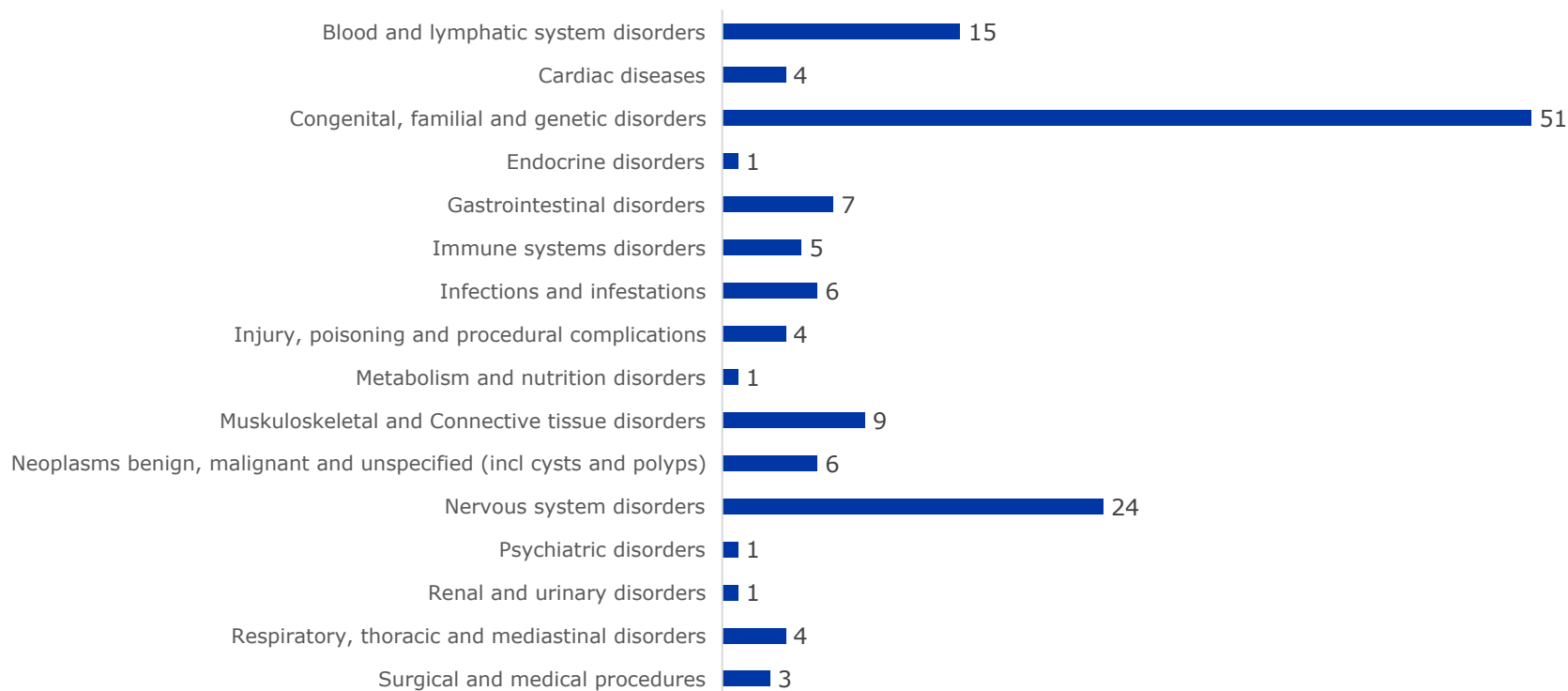
Designations based on significant benefit (SB)





ORPHAN MEDICINAL PRODUCT DESIGNATION

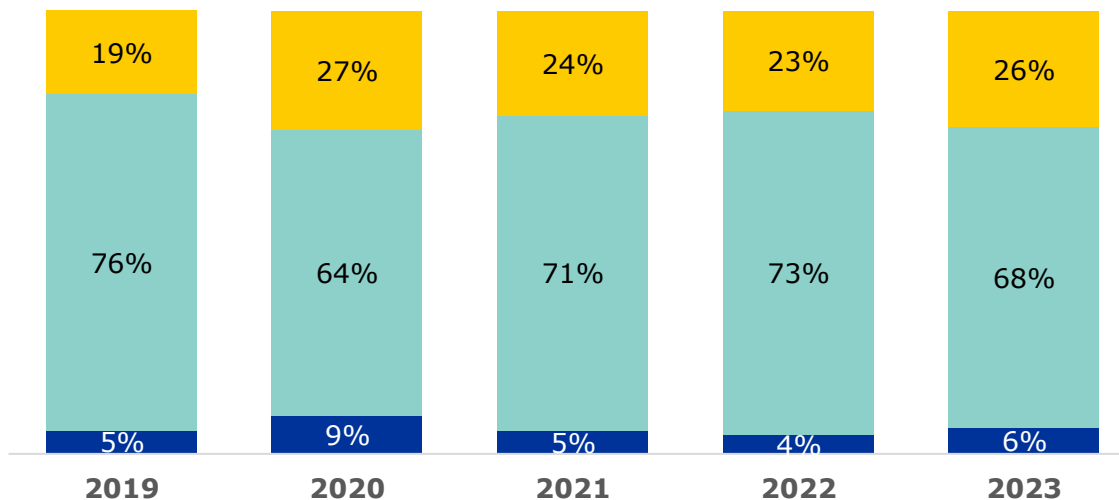
COMP opinions 2023 – MedDRA classification





ORPHAN MEDICINAL PRODUCT DESIGNATION

Orphan designations adult/paediatic

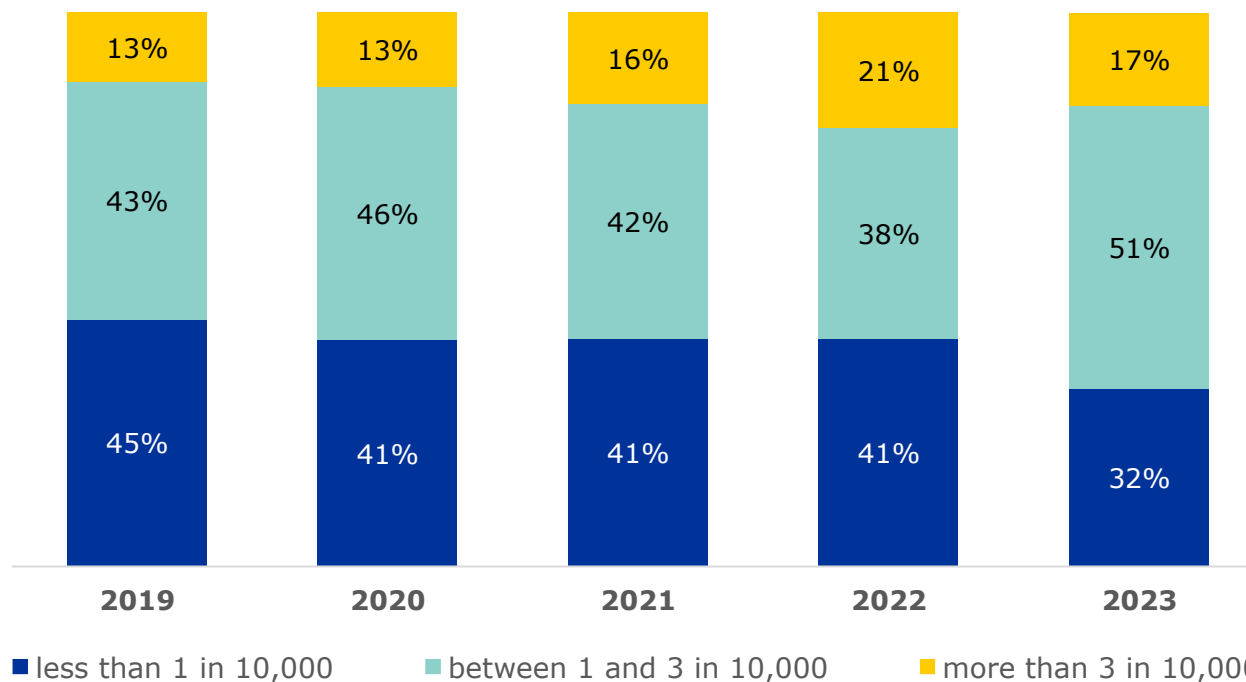


- Medical conditions affecting children only
- Medical conditions affecting both children and adults
- Medical conditions affecting adults only



ORPHAN MEDICINAL PRODUCT DESIGNATION

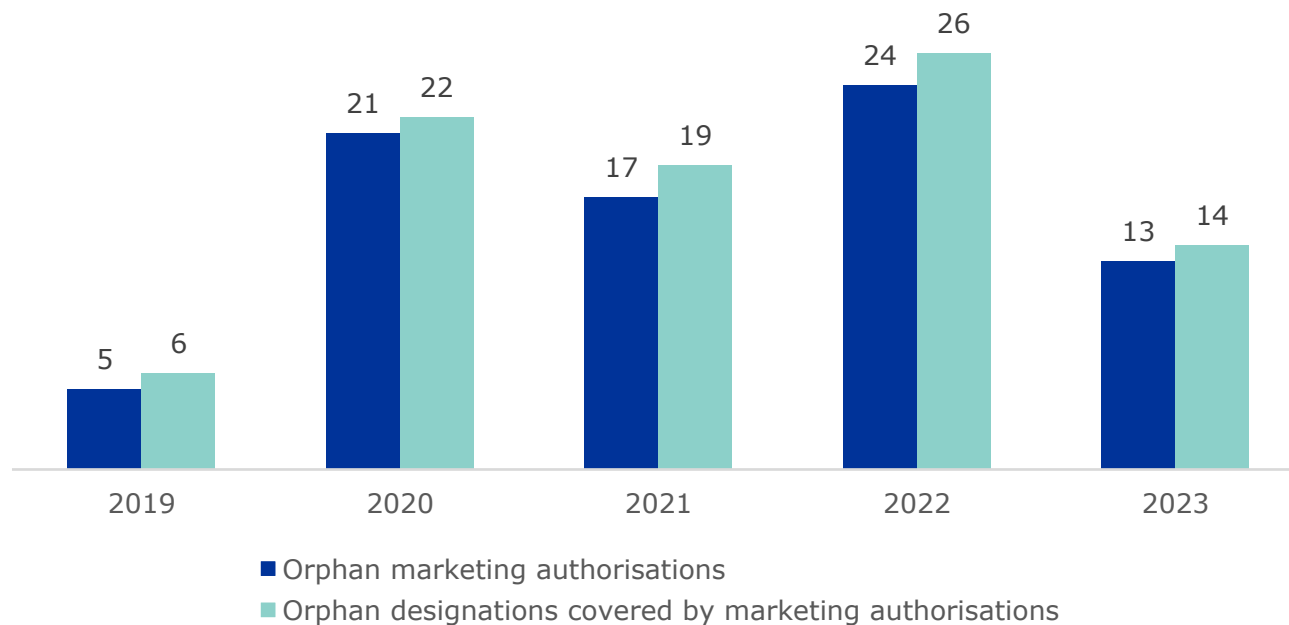
Prevalence for designated orphan conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

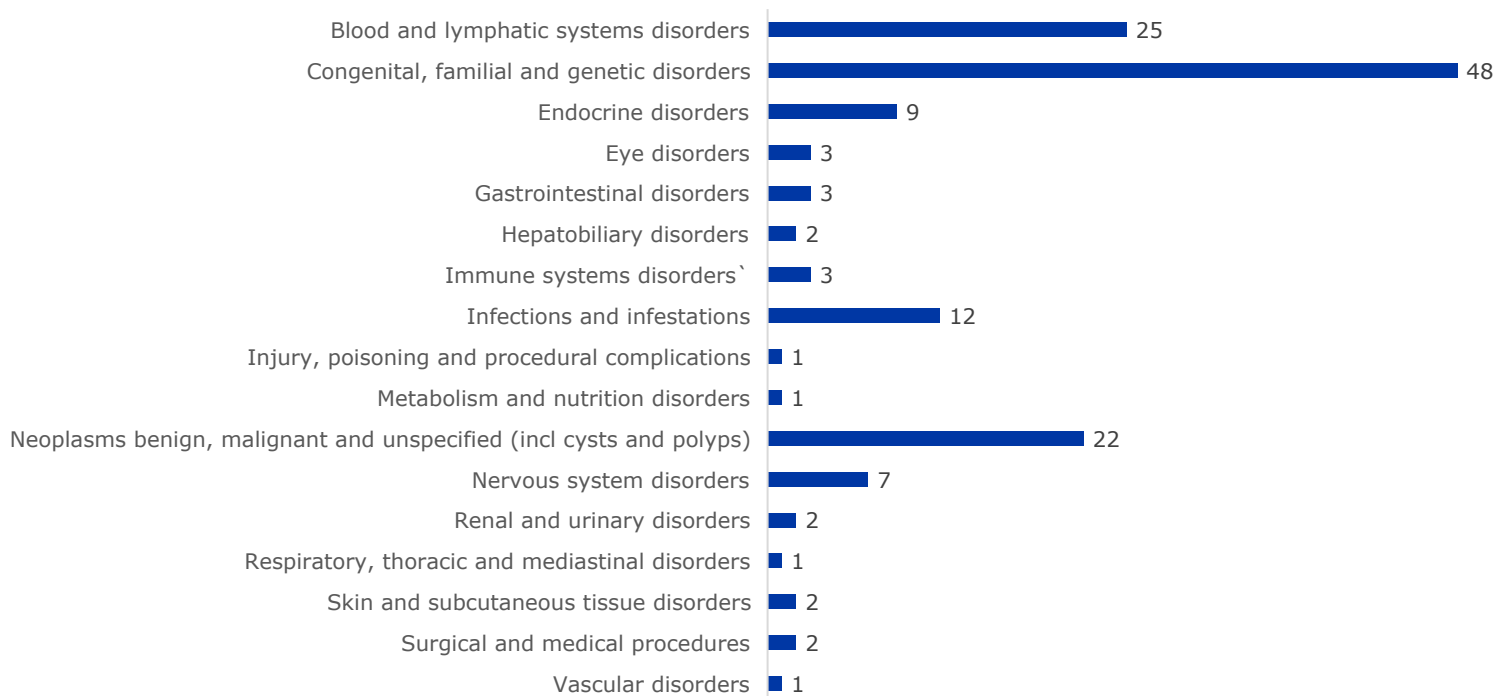
Authorised orphan medicinal products





ORPHAN MEDICINAL PRODUCT DESIGNATION

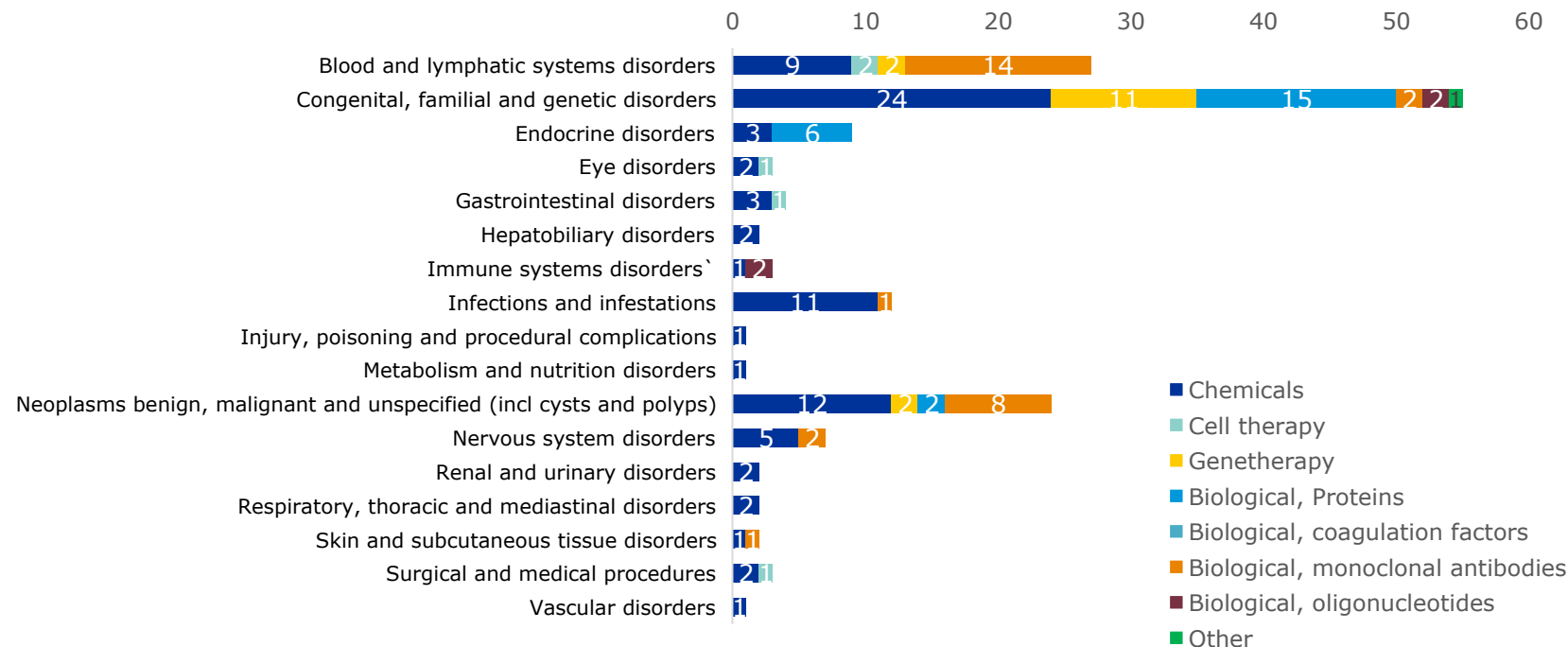
Active orphan MAA – MedDRA classification





ORPHAN MEDICINAL PRODUCT DESIGNATION

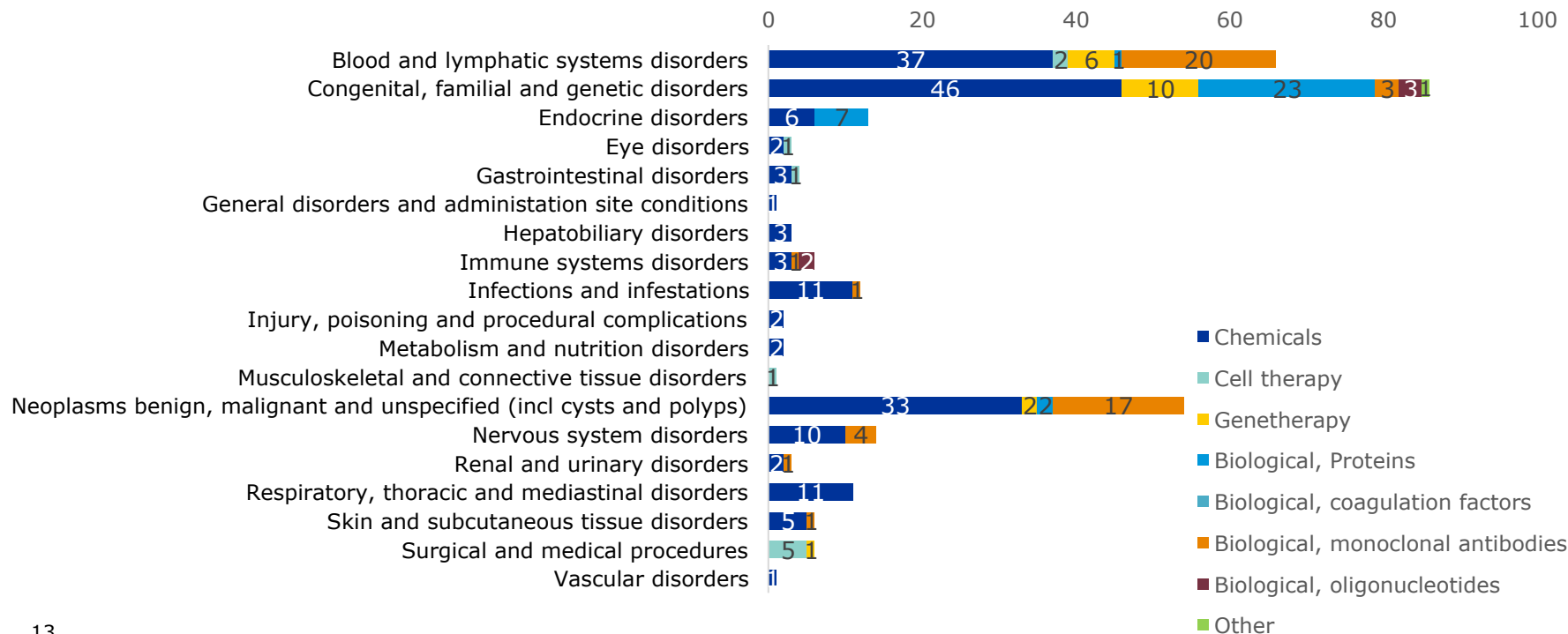
Authorisations by type of product





ORPHAN MEDICINAL PRODUCT DESIGNATION

Authorisations by type of product including extensions of indication





ORPHAN MEDICINAL PRODUCT DESIGNATION

Overview of orphan marketing authorisations granted to date

244 initial marketing authorisations granted

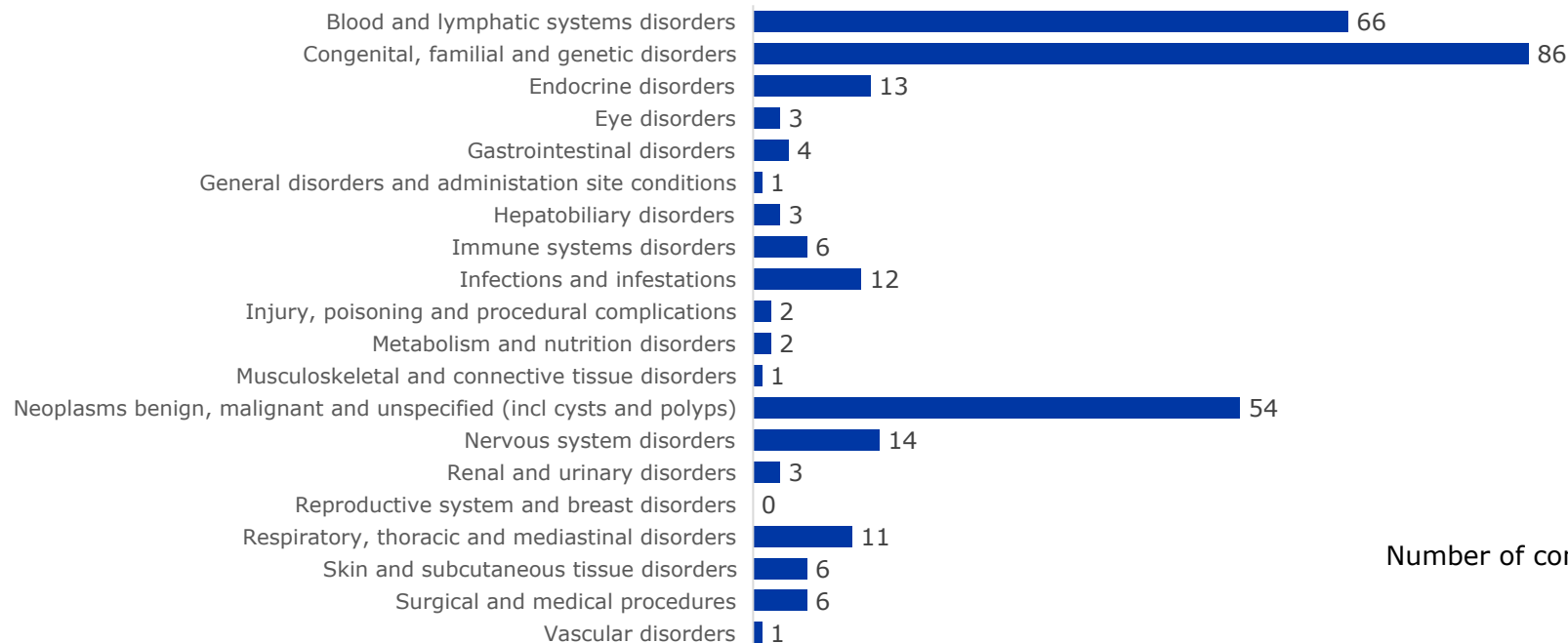
50 extensions of indication

- **144 active initial authorisations**
- **30 active extensions of indication**
- **29 withdrawals** from the register of orphan medicinal products (including 11 ext. of indication)
- **12 withdrawals** from register medicinal products human use/ orphan status expired
- **2 revoked** from register medicinal products human use/ orphan status expired
- **68 removals of orphan status for initial MAA** from register after expire of the market exclusivity period plus for **10 extensions of indication**



ORPHAN MEDICINAL PRODUCT DESIGNATION

Initial orphan marketing authorisations and extension of indication granted to date

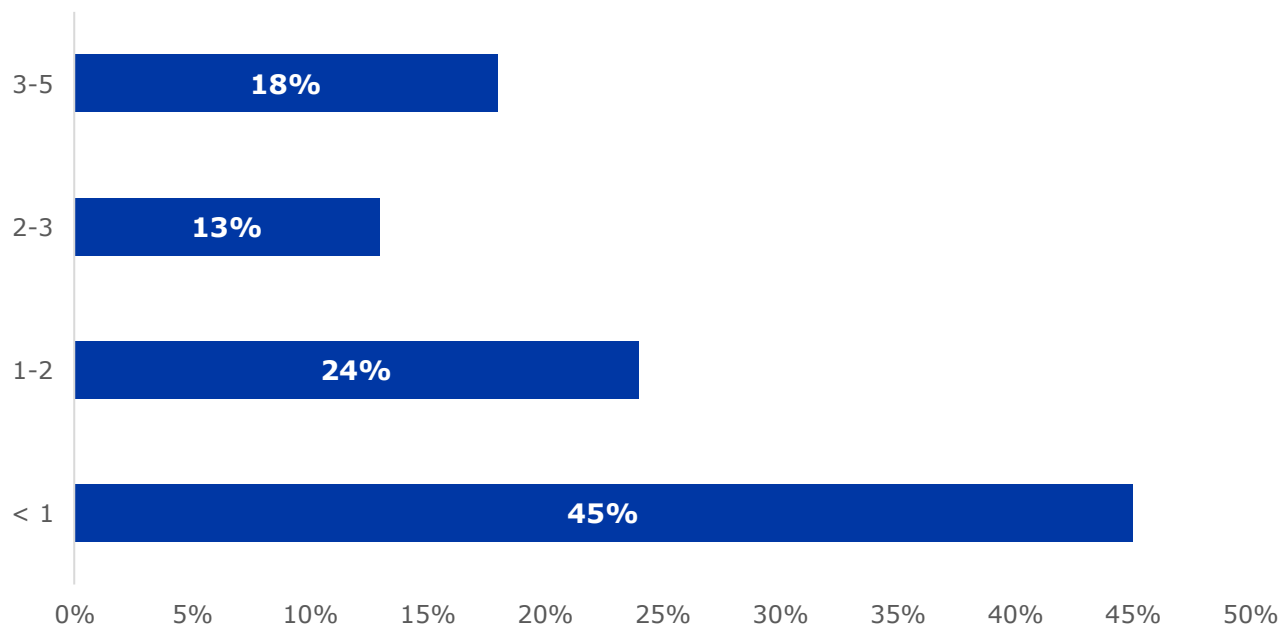


Number of conditions: **166**



ORPHAN MEDICINAL PRODUCT DESIGNATION

Overview of prevalence for all orphan marketing authorisations to date





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Further information

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