



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Orphan Medicinal Product Designation

Overview 2000-2022





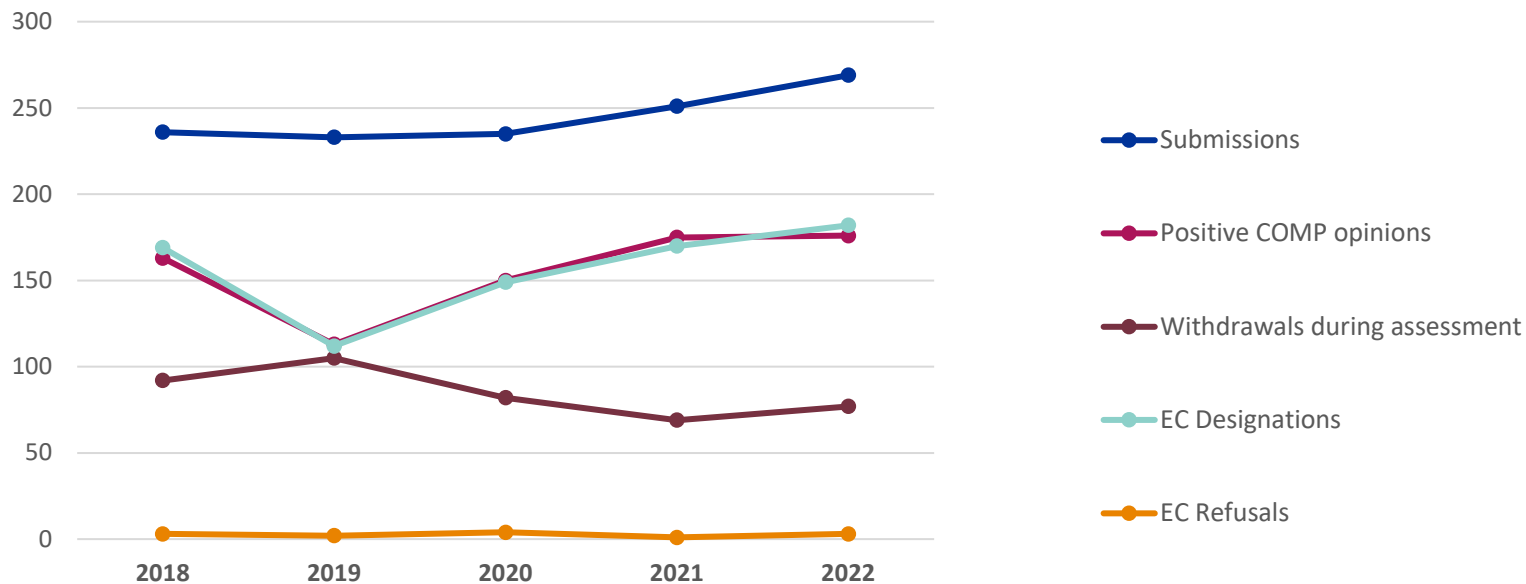
ORPHAN MEDICINAL PRODUCT DESIGNATION Overview

	2000-2005	2006-2010	2011-2015	2016-2020	2021	2022	TOTAL
Submissions	548	686	1151	1293	251	269	4198
Positive COMP Opinions	348	500	759	790	175	176	2748
EC Designations	343	485	768	786	170	182	2734
EC Refusals	8	6	9	12	1	3	39
Withdrawals after submission	150	144	313	456	69	77	1209



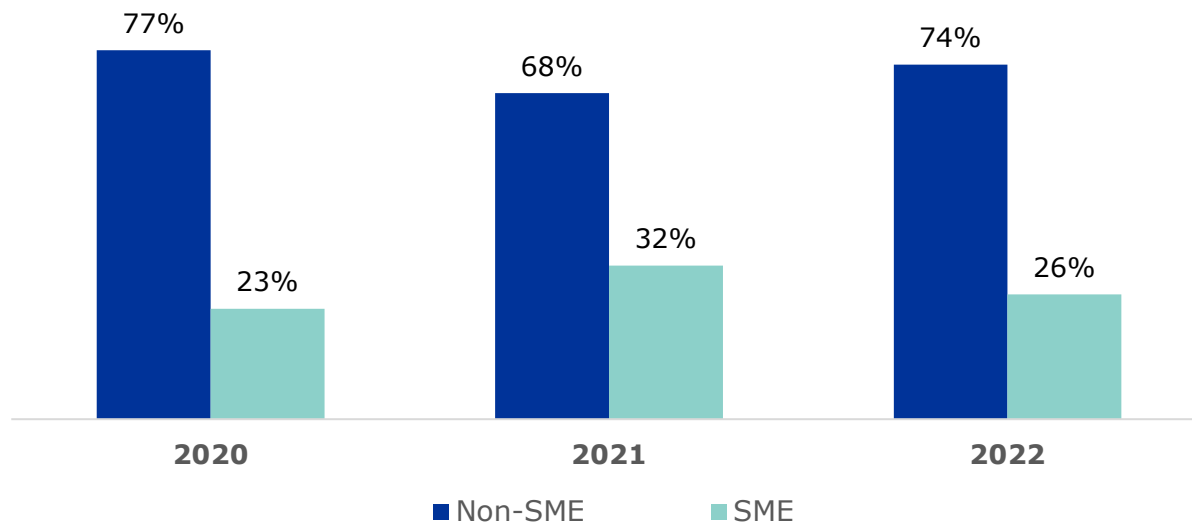
ORPHAN MEDICINAL PRODUCT DESIGNATION

Trend in recent years





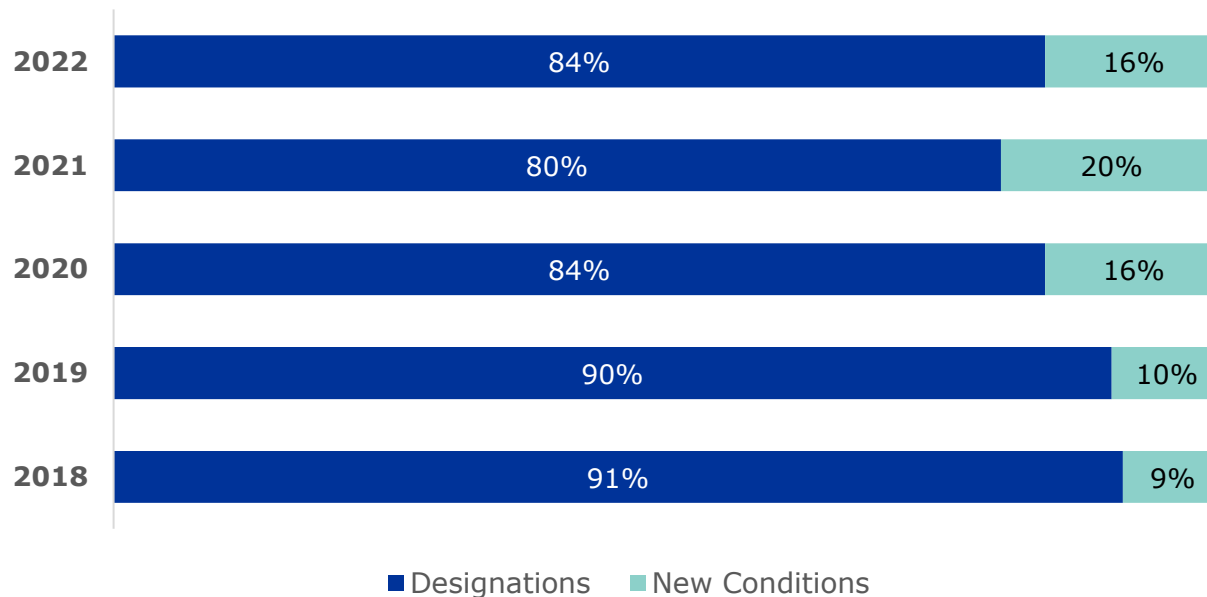
ORPHAN MEDICINAL PRODUCT DESIGNATION Submissions from SME sponsors





ORPHAN MEDICINAL PRODUCT DESIGNATION

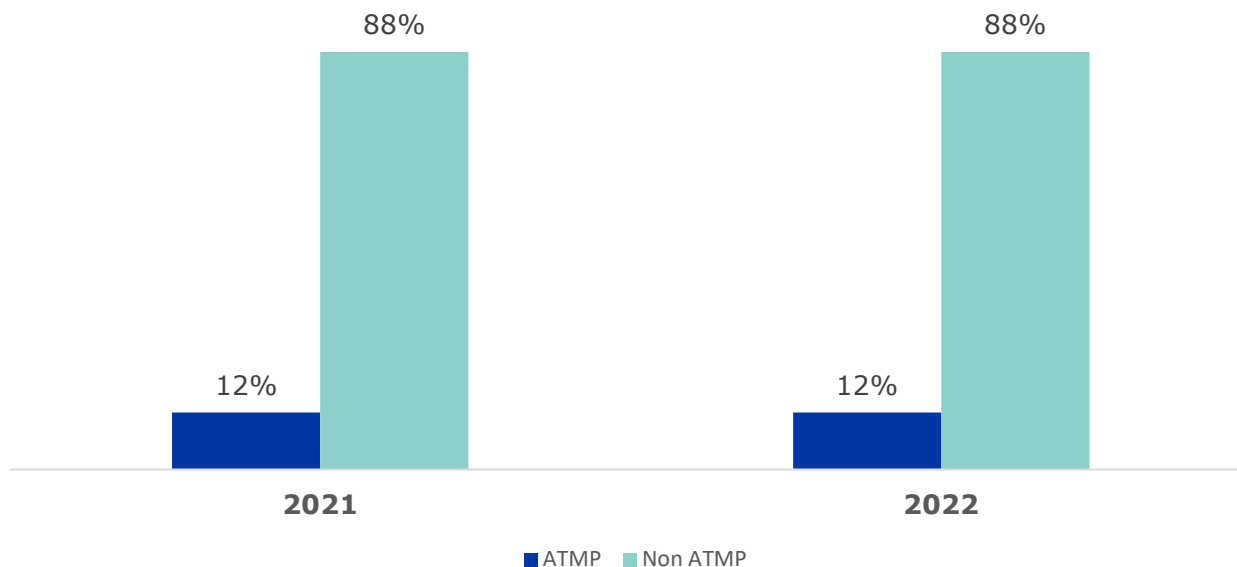
New designated conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

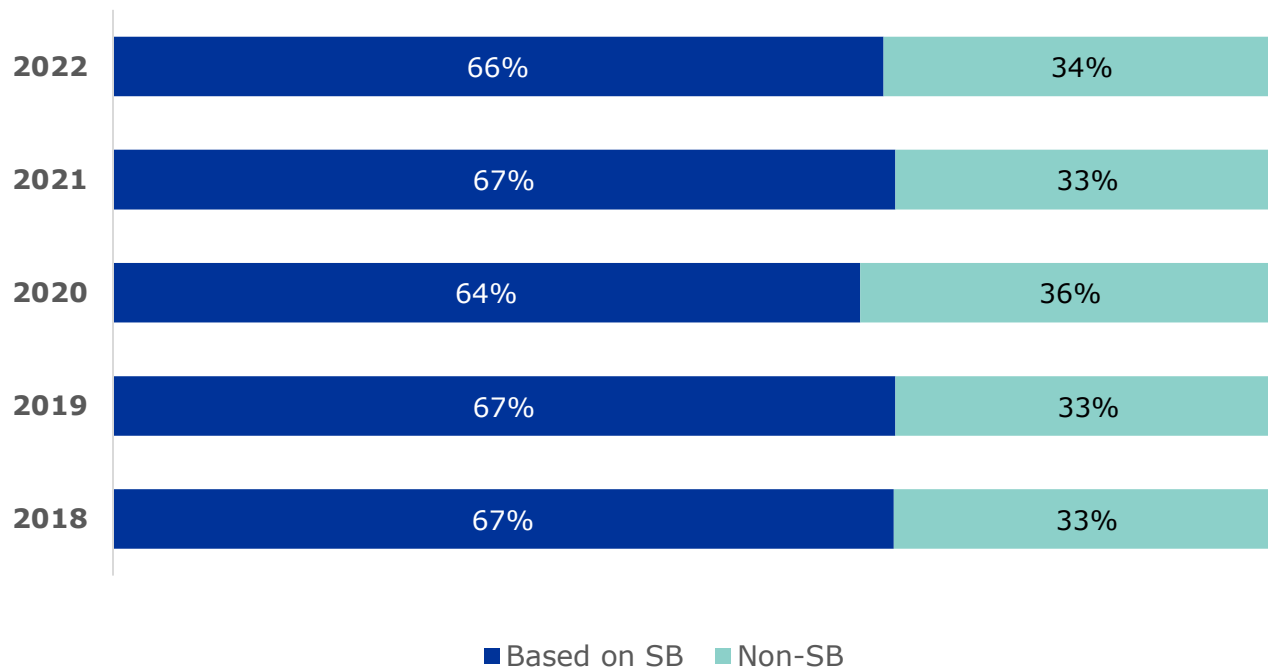
Submissions with and ATMP like substance





ORPHAN MEDICINAL PRODUCT DESIGNATION

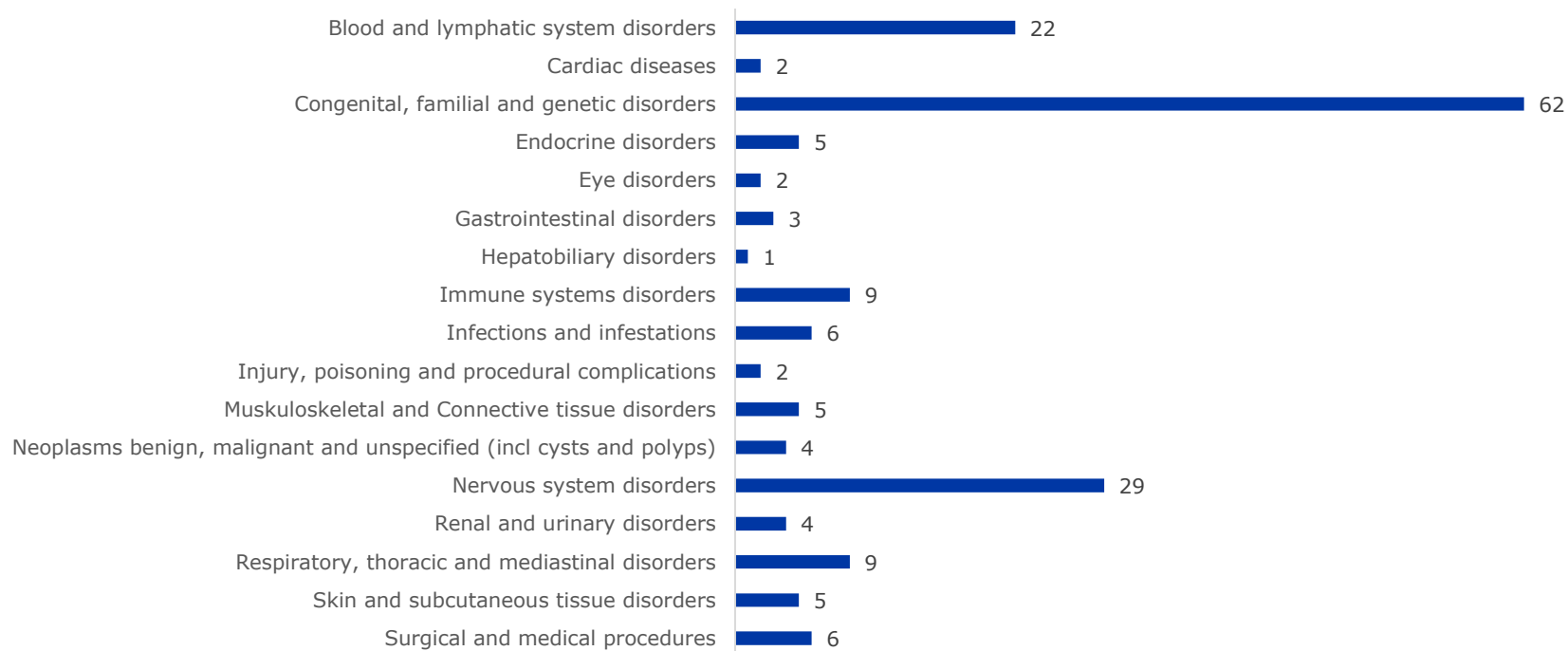
Designations based on significant benefit (SB)





ORPHAN MEDICINAL PRODUCT DESIGNATION

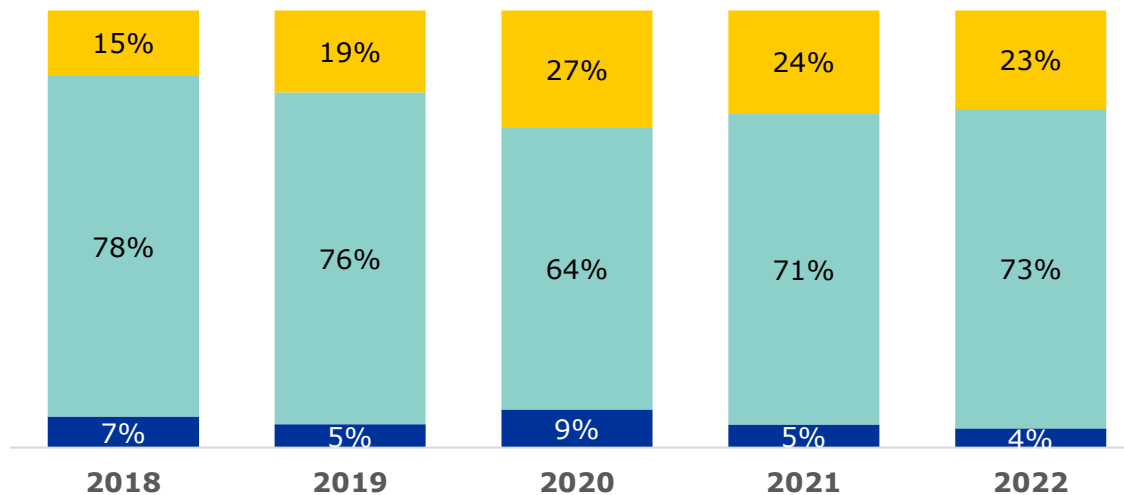
COMP opinions 2022 – MedDRA classification





ORPHAN MEDICINAL PRODUCT DESIGNATION

Orphan designations adult/paediatric

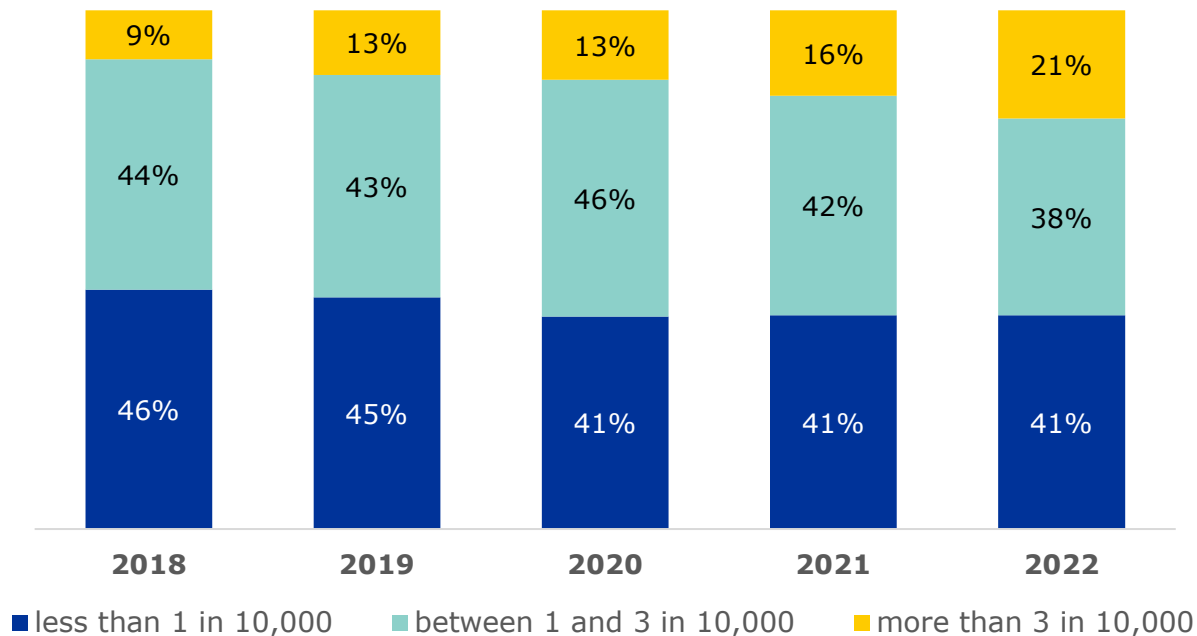


- Medical conditions affecting adults only
- Medical conditions affecting both children and adults
- Medical conditions affecting children only



ORPHAN MEDICINAL PRODUCT DESIGNATION

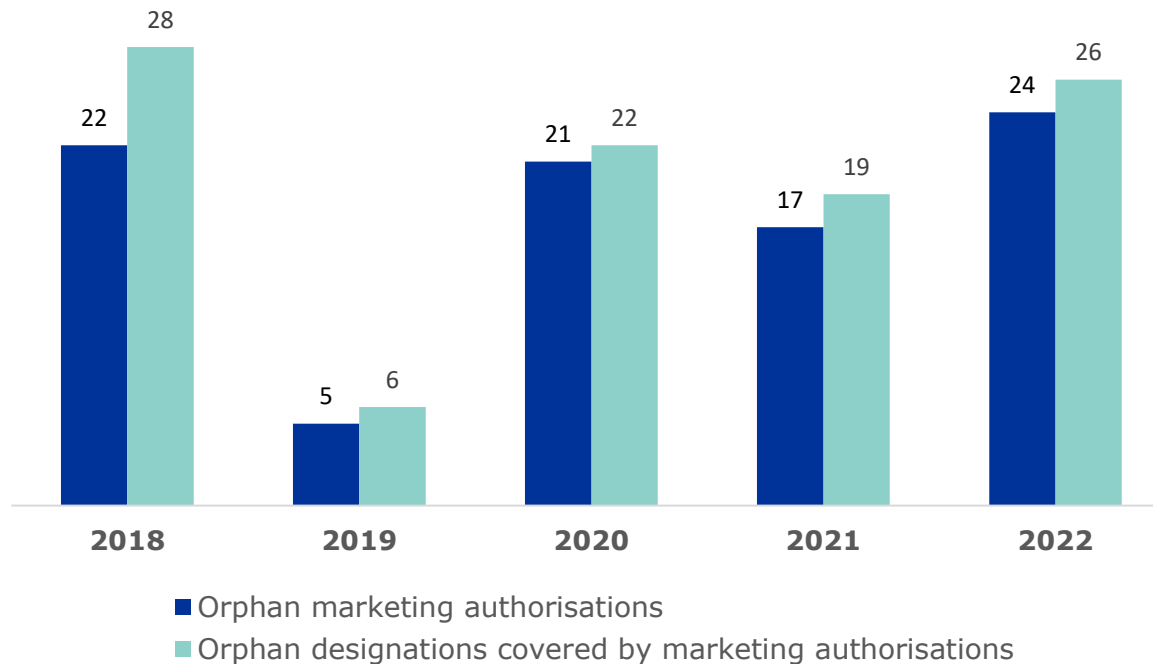
Prevalence for designated orphan conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

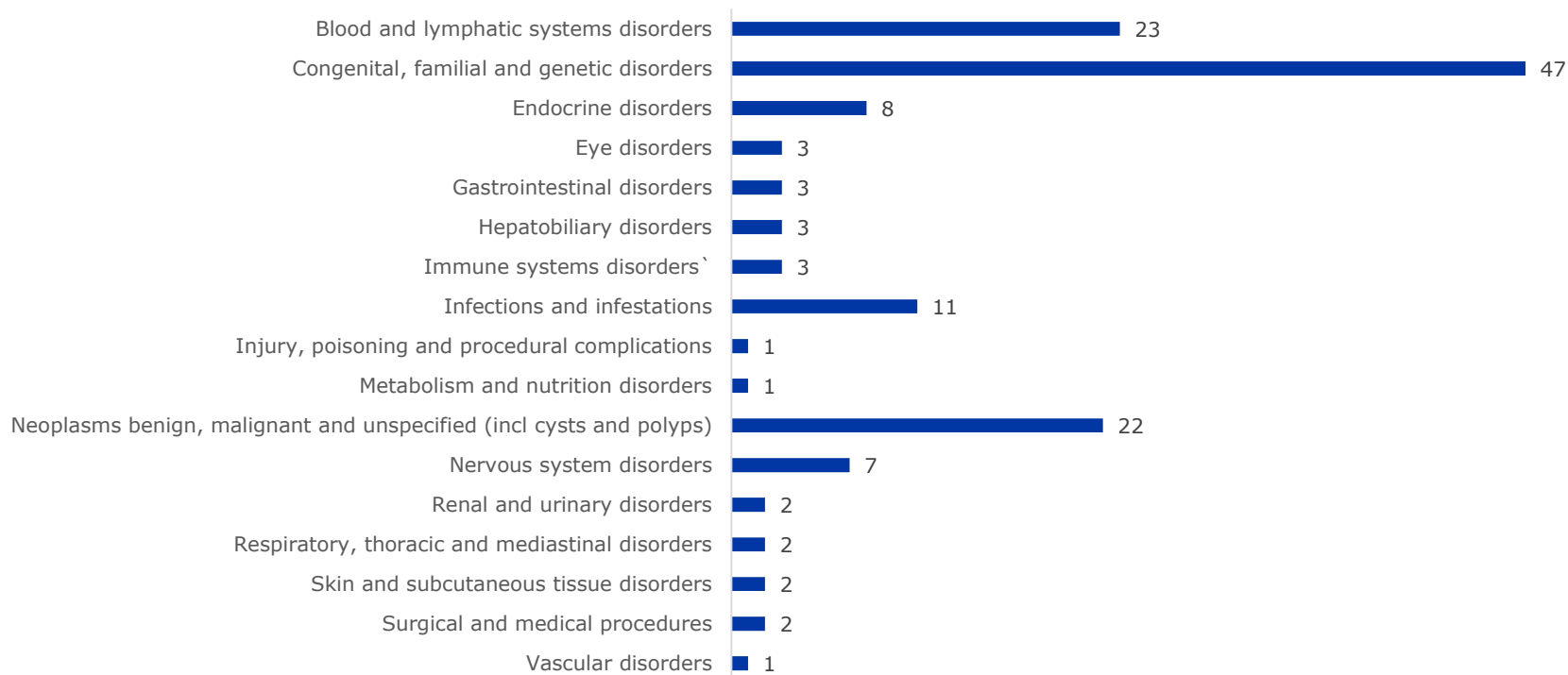
Authorised orphan medicinal products





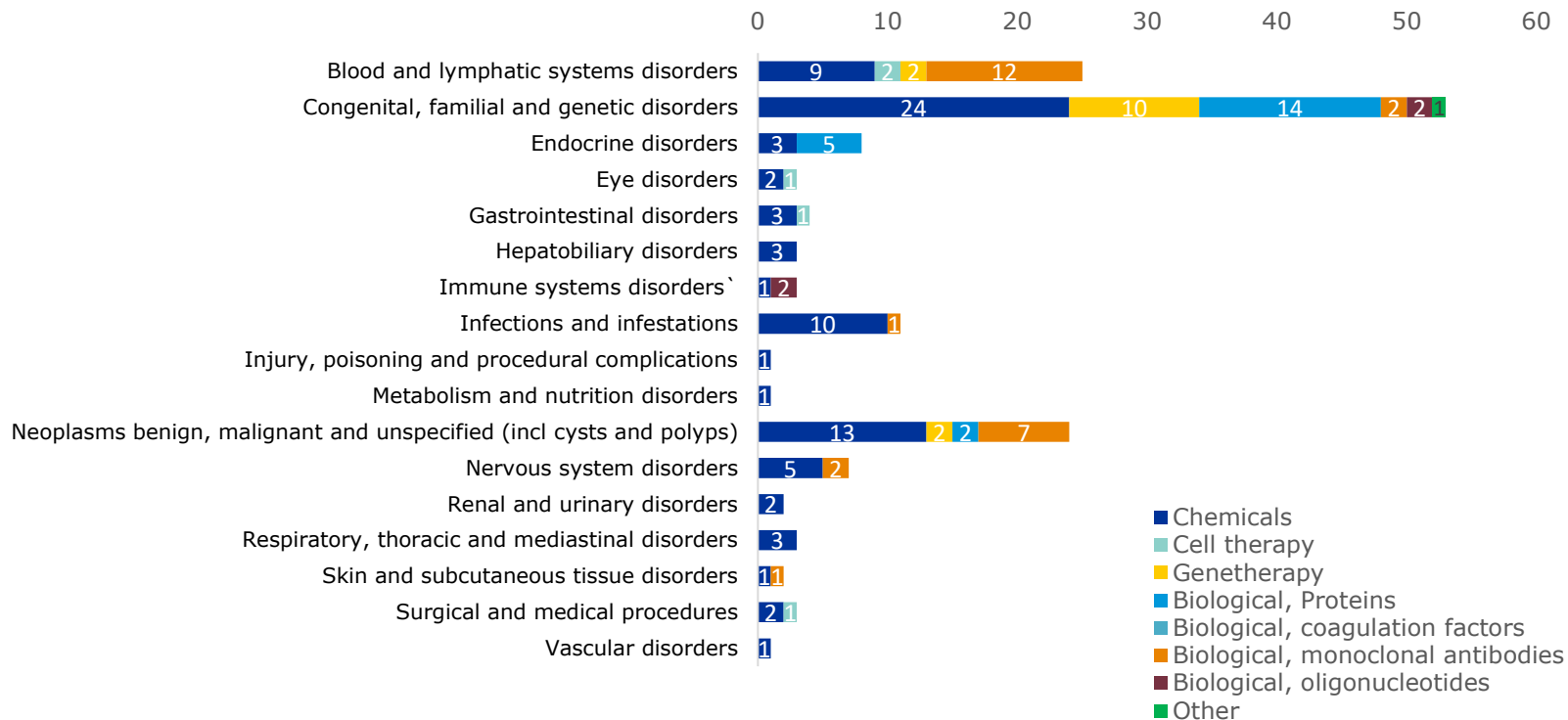
ORPHAN MEDICINAL PRODUCT DESIGNATION

Active orphan MAA – MedDRA classification





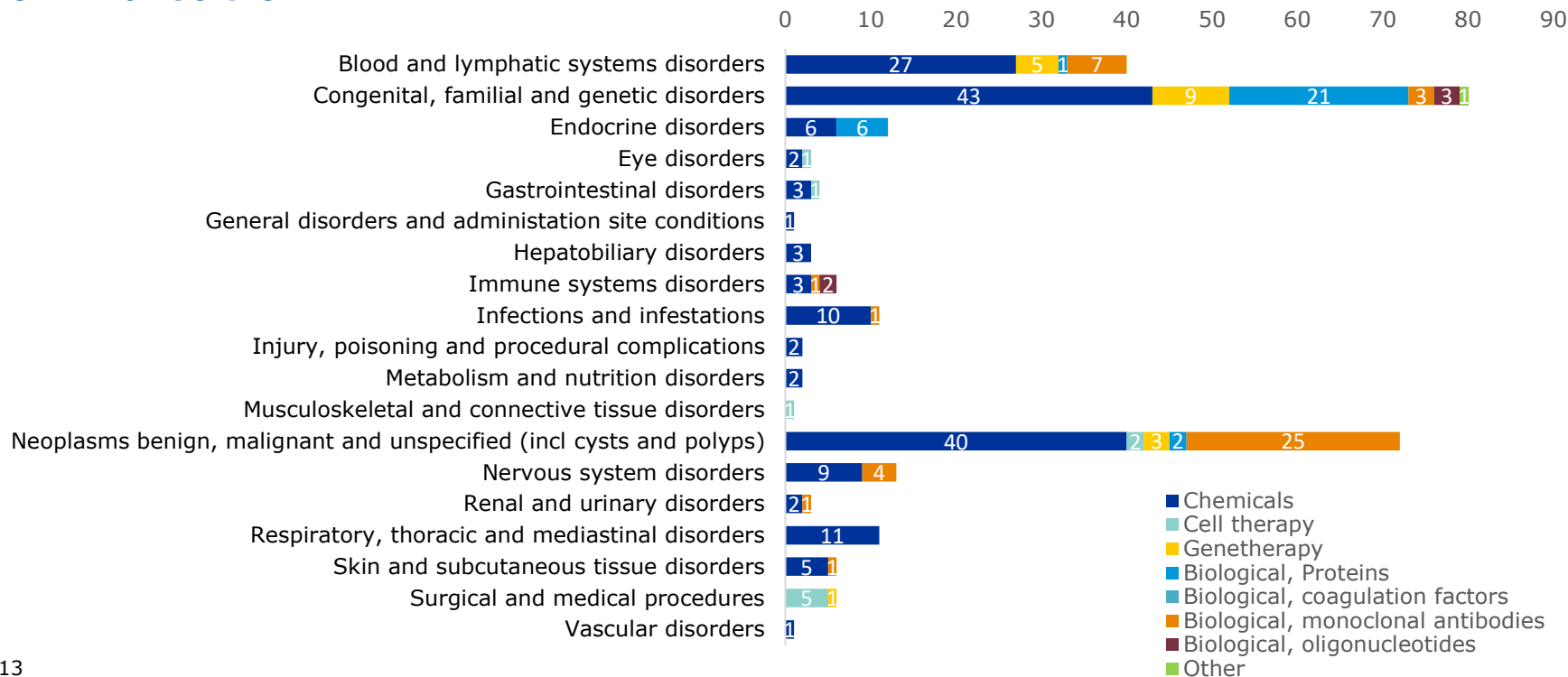
ORPHAN MEDICINAL PRODUCT DESIGNATION Authorisations by type of product





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

231 initial marketing authorisations granted

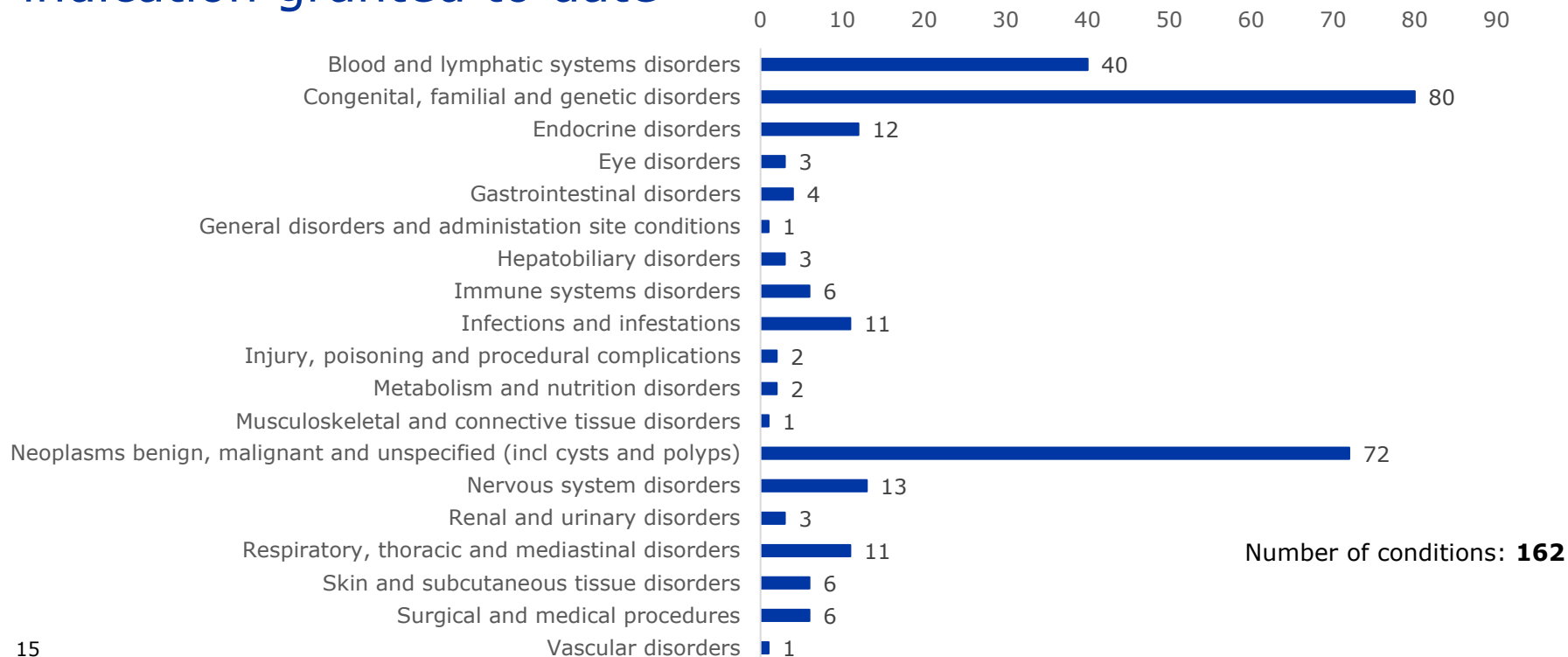
46 extensions of indication

- **141 active initial authorisations**
- **27 active extensions of indication**
- **27 withdrawals** from the register of orphan medicinal products (including 11 ext. of indication)
- **12 withdrawals** from register medicinal products human use/ orphan status expired
- **1 revoked** from register medicinal products human use/ orphan status expired
- **61 removals of initial MAA** from register after expire of the market exclusivity period plus **8 removals of extensions of indication**



ORPHAN MEDICINAL PRODUCT DESIGNATION

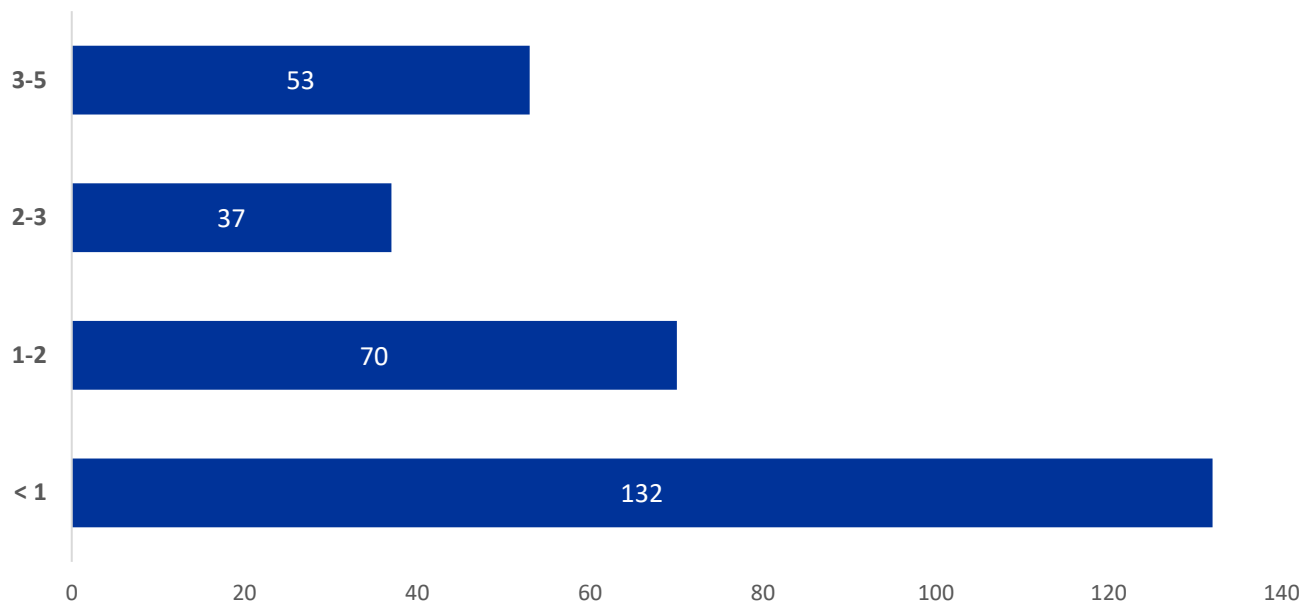
Initial orphan marketing authorisations and extension of indication granted to date





ORPHAN MEDICINAL PRODUCT DESIGNATION

Overview of prevalence for orphan marketing authorisations to date





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Further information

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