



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Orphan Medicinal Product Designation

Overview 2000-2021





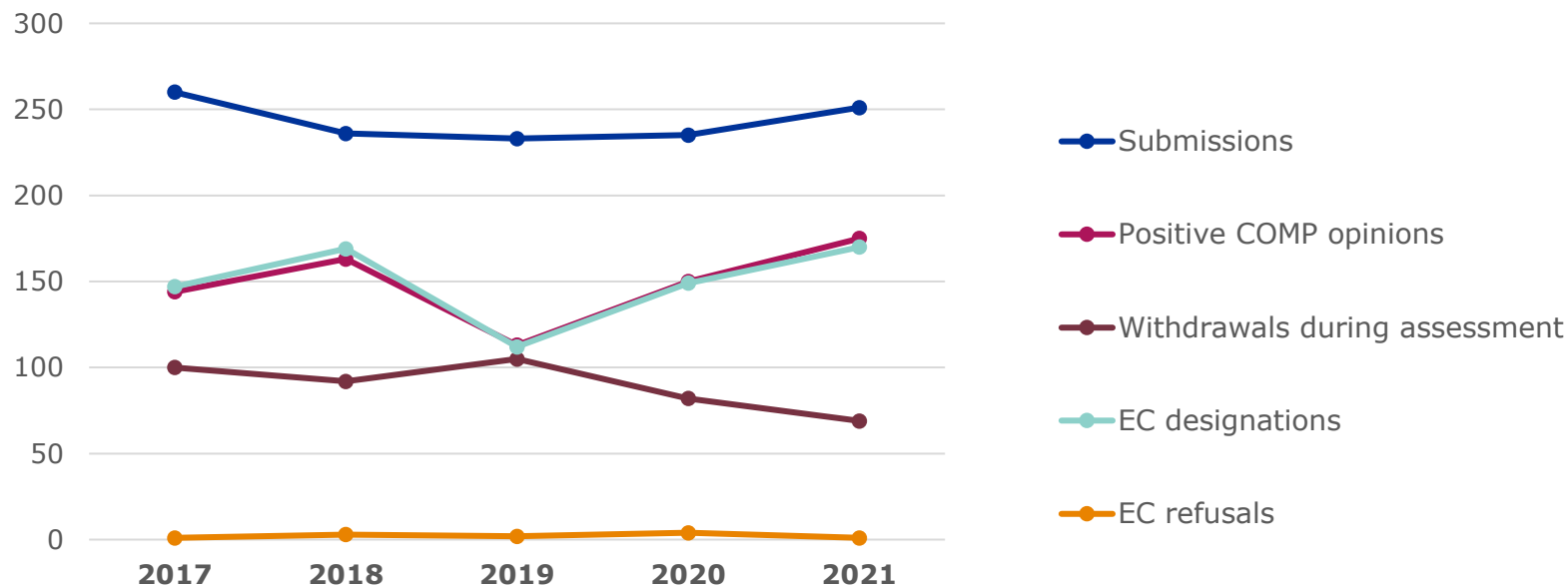
ORPHAN MEDICINAL PRODUCT DESIGNATION Overview

	2000-2005	2005-2010	2010-2015	2015-2020	2021	TOTAL
Submissions	548	686	1151	1293	251	3929
Positive COMP Opinions	348	500	759	790	175	2572
EC Designations	343	485	768	786	170	2552
EC Refusals	8	6	9	12	1	36
Withdrawals after submission	150	144	313	456	69	1132



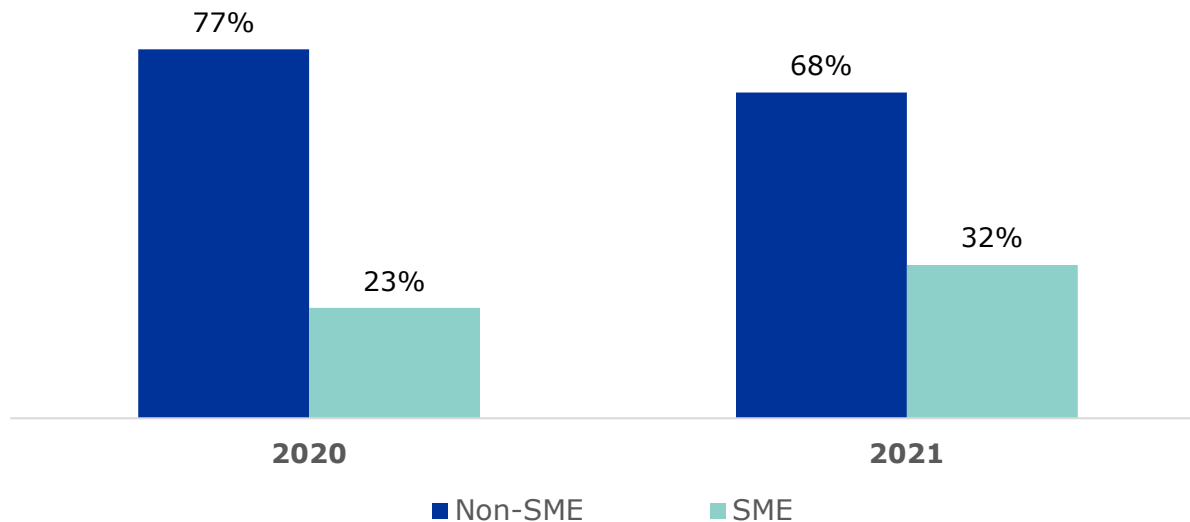
ORPHAN MEDICINAL PRODUCT DESIGNATION

Trend in recent years





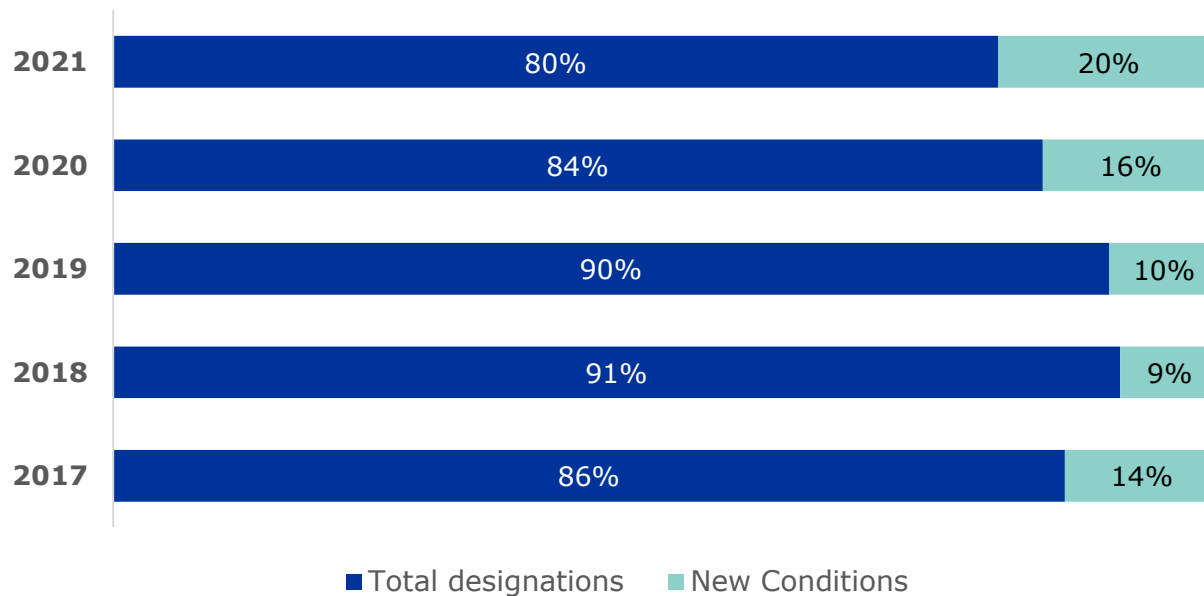
ORPHAN MEDICINAL PRODUCT DESIGNATION Submissions from SME sponsors





ORPHAN MEDICINAL PRODUCT DESIGNATION

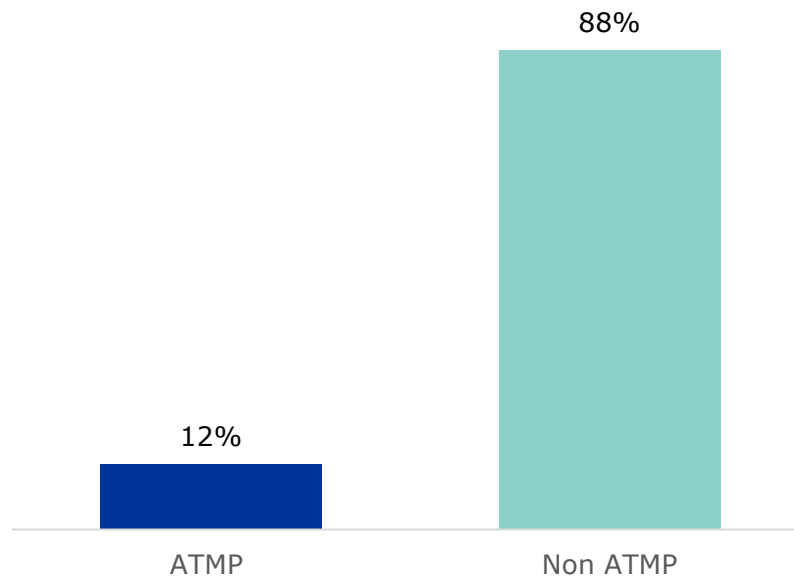
New designated conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

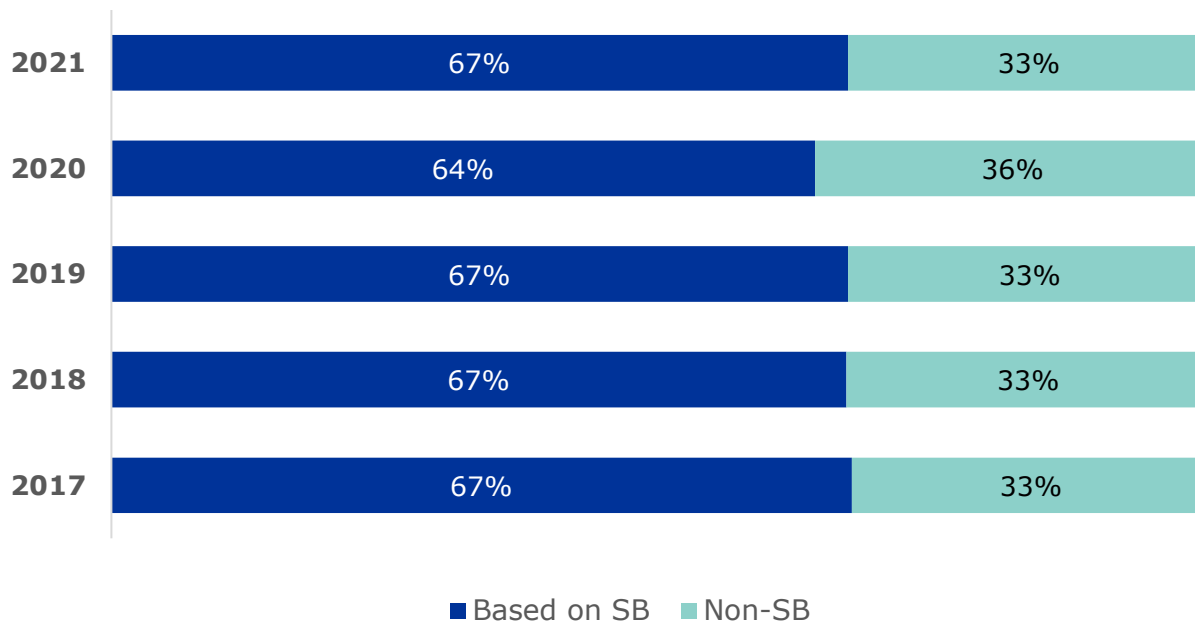
Submissions with and ATMP like substance (2021)





ORPHAN MEDICINAL PRODUCT DESIGNATION

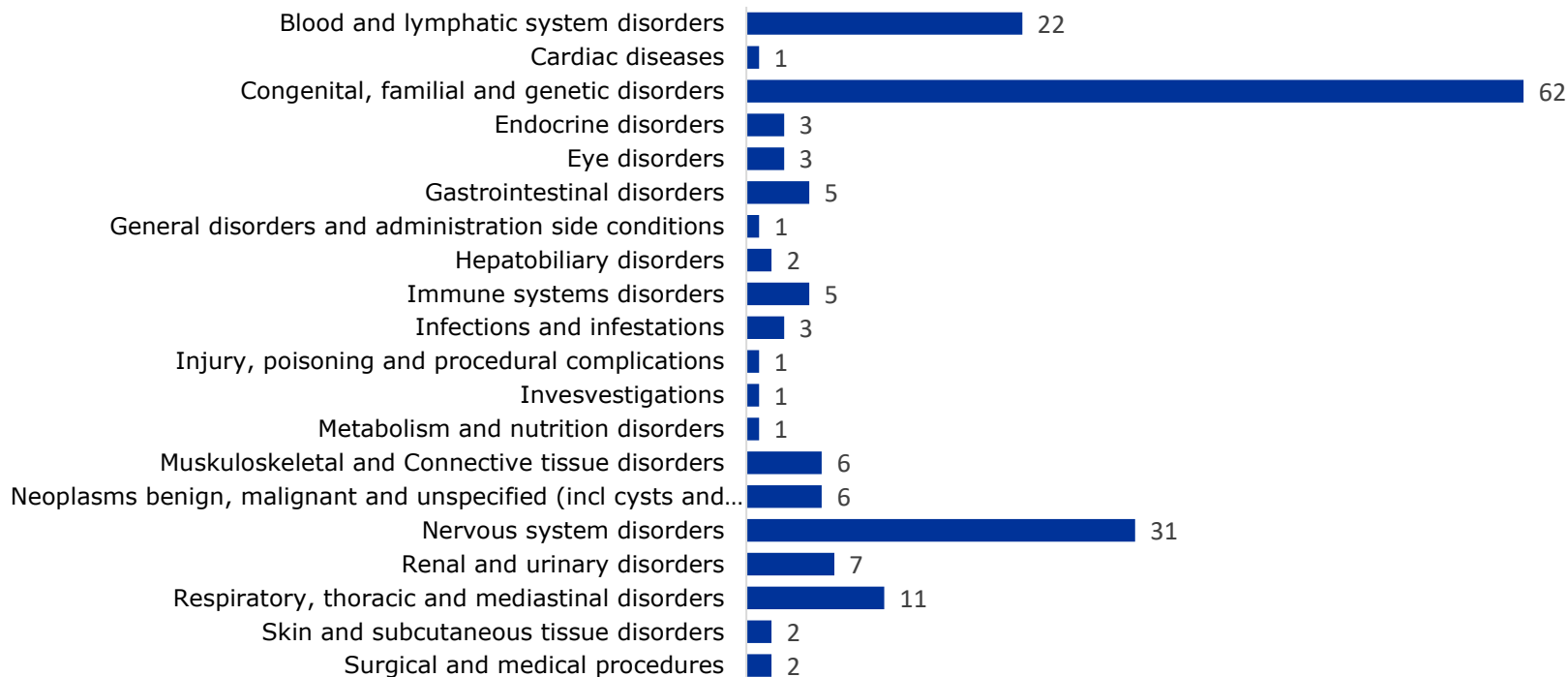
Designations based on significant benefit (SB)





ORPHAN MEDICINAL PRODUCT DESIGNATION

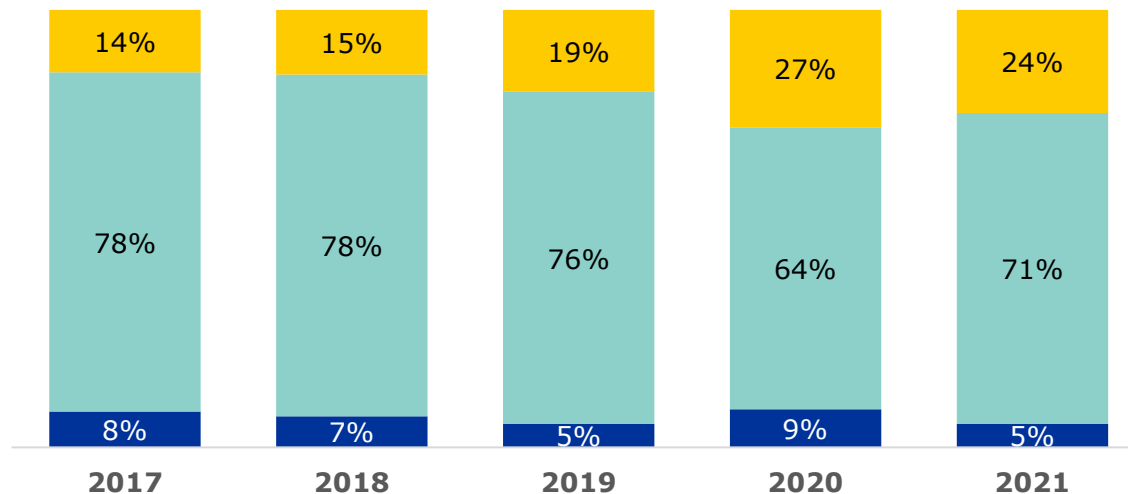
CHMP opinions 2021 – MedDRA classification





ORPHAN MEDICINAL PRODUCT DESIGNATION

Orphan designations adult/paediatric

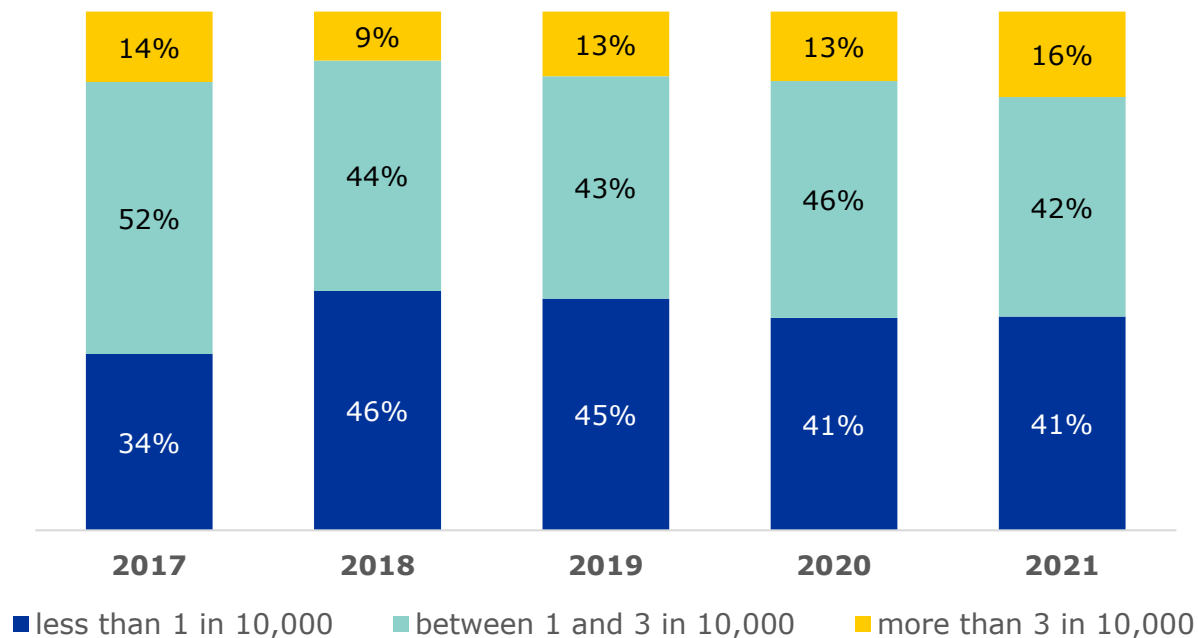


- Medical conditions affecting adults only
- Medical conditions affecting both children and adults
- Medical conditions affecting children only



ORPHAN MEDICINAL PRODUCT DESIGNATION

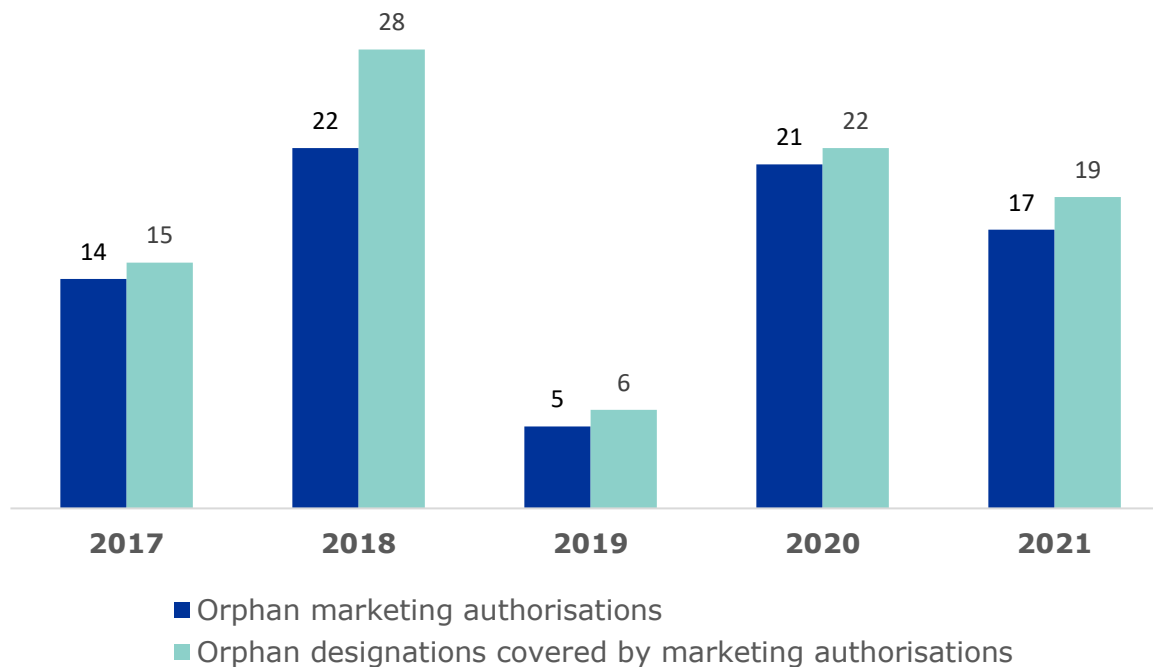
Prevalence for designated orphan conditions





ORPHAN MEDICINAL PRODUCT DESIGNATION

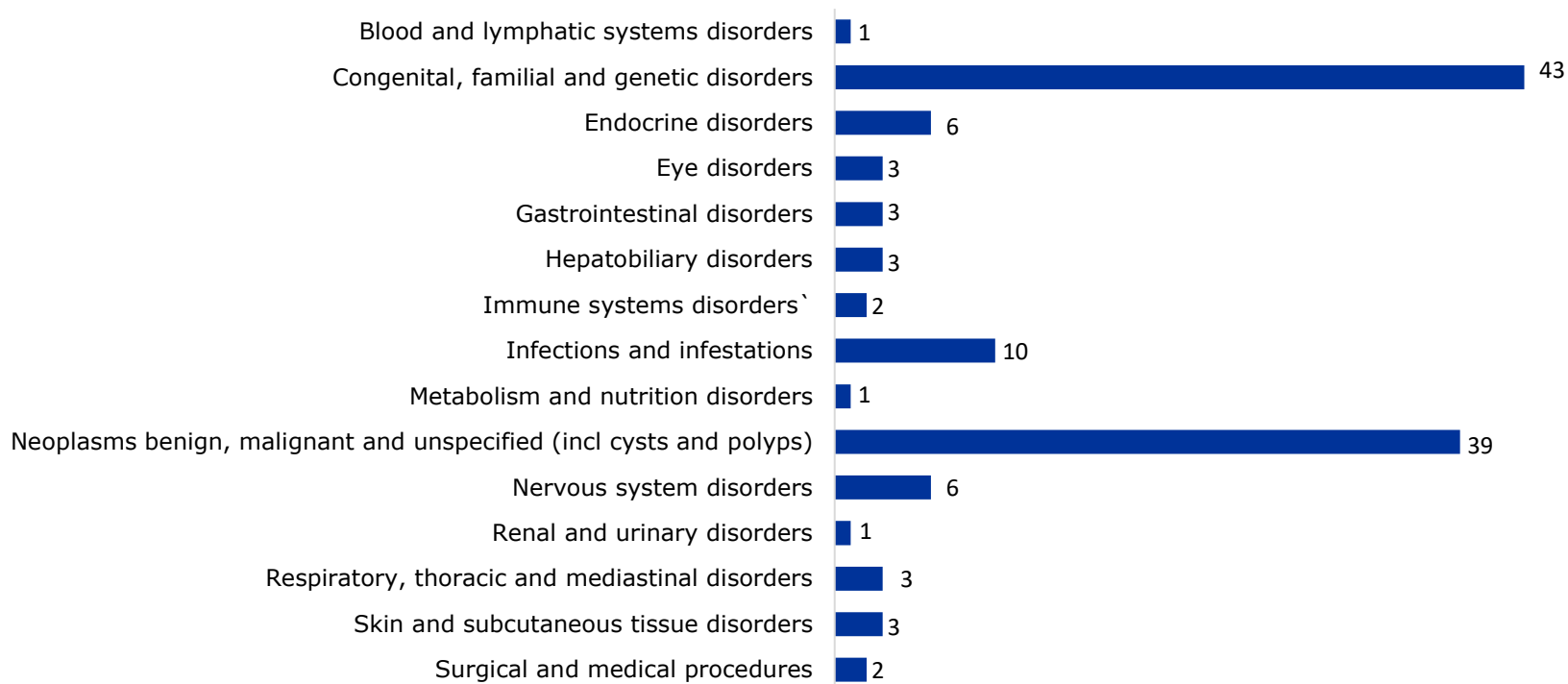
Authorised orphan medicinal products





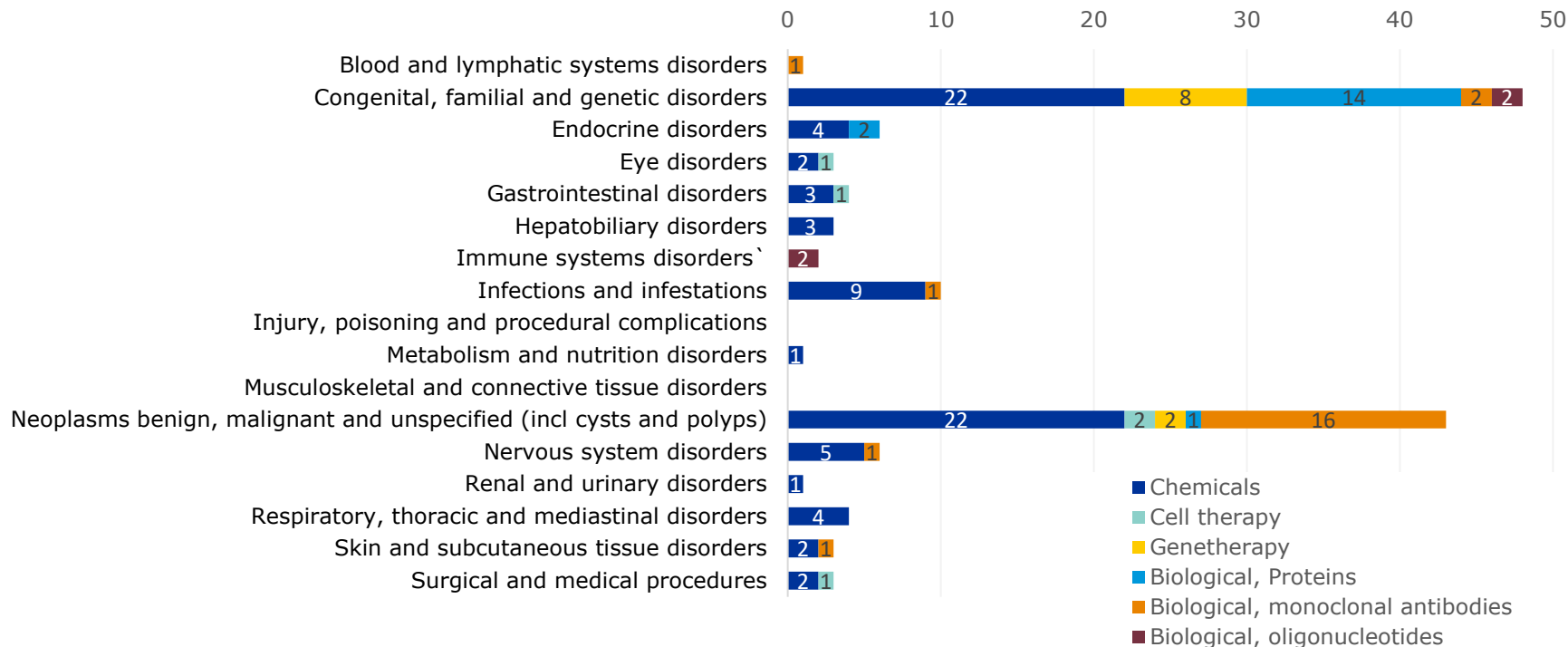
ORPHAN MEDICINAL PRODUCT DESIGNATION

Active orphan MAA – MedDRA classification





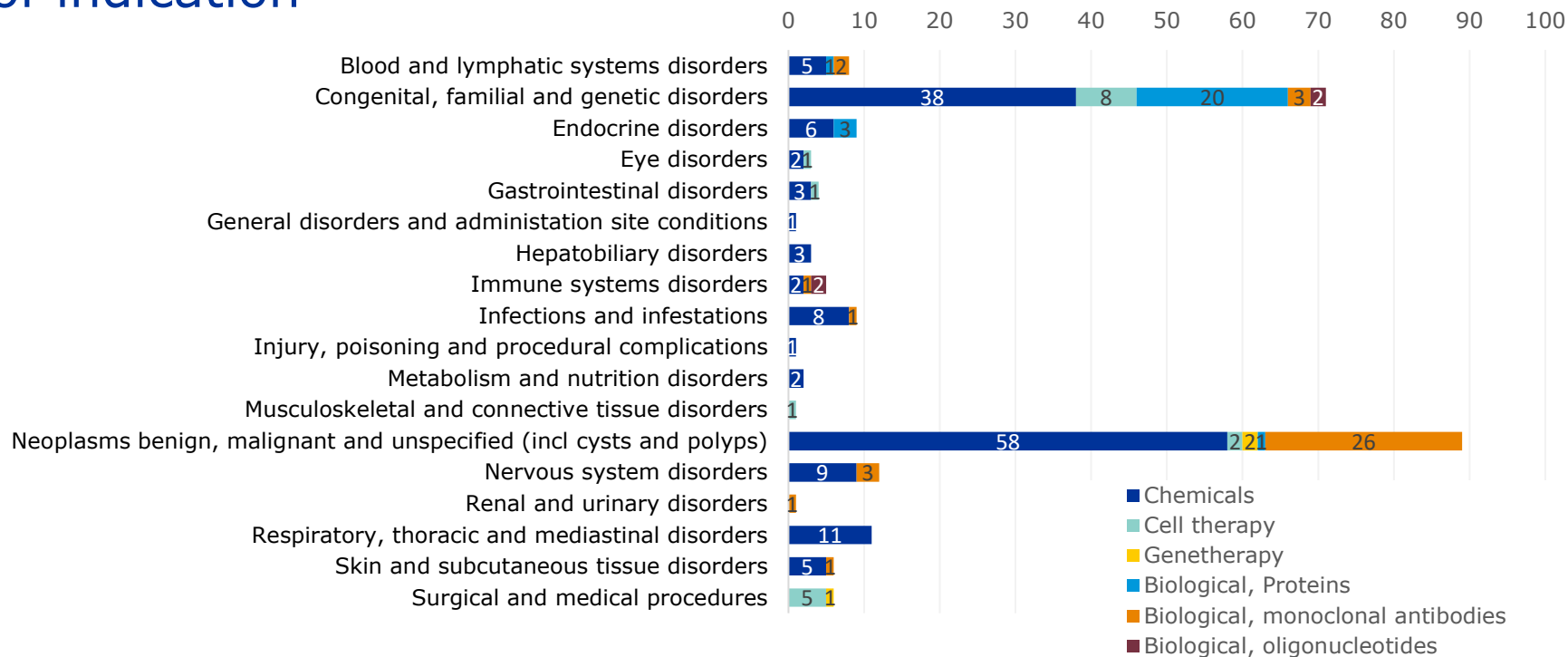
ORPHAN MEDICINAL PRODUCT DESIGNATION Authorisations by type of product





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

207 initial marketing authorisations granted

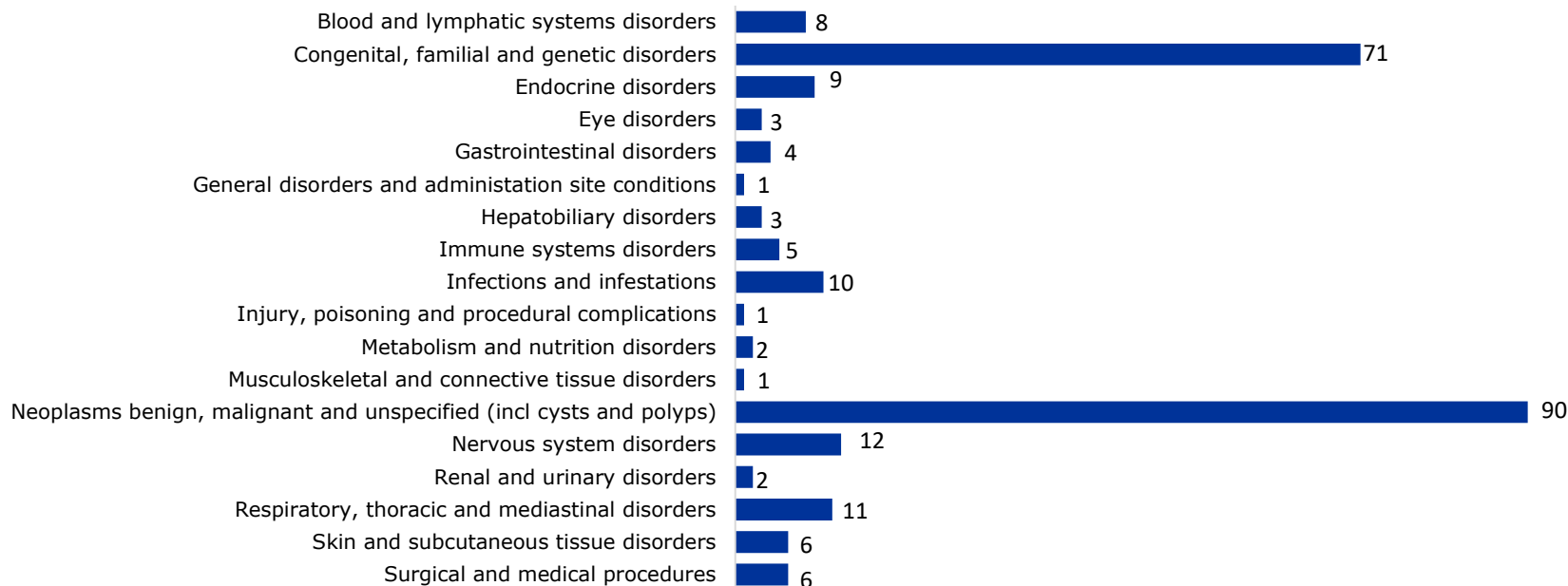
38 extensions of indication

- **126 active initial authorisations**
- **24 active extensions of indication**
- **23 withdrawals** from the register of orphan medicinal products (including 9 ext. of indication)
- **11 withdrawals** from register medicinal products human use/ orphan status expired
- **1 revoked** from register medicinal products human use/ orphan status expired
- **55 removals of initial MAA** from register after expire of the market exclusivity period plus **5 removals of extensions of indication**



ORPHAN MEDICINAL PRODUCT DESIGNATION

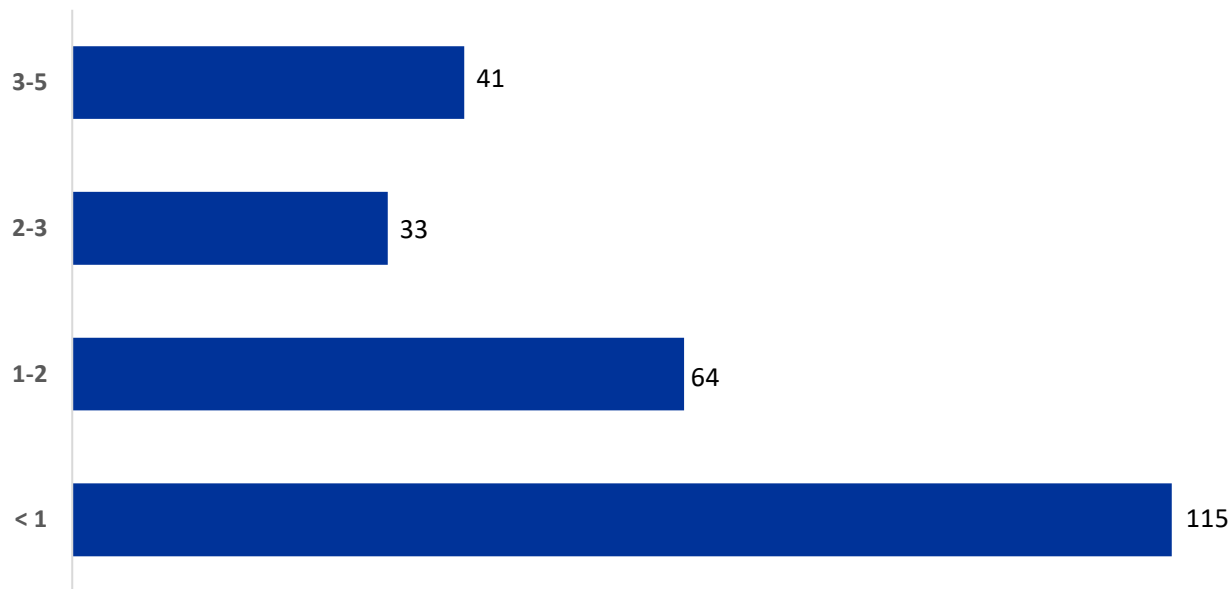
Initial orphan marketing authorisations and extension of indication granted to date

Number of conditions: **148**



ORPHAN MEDICINAL PRODUCT DESIGNATION

Overview of prevalence for orphan marketing authorisations to date





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Further information

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