



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

# Orphan medicines figures

2000- 2018

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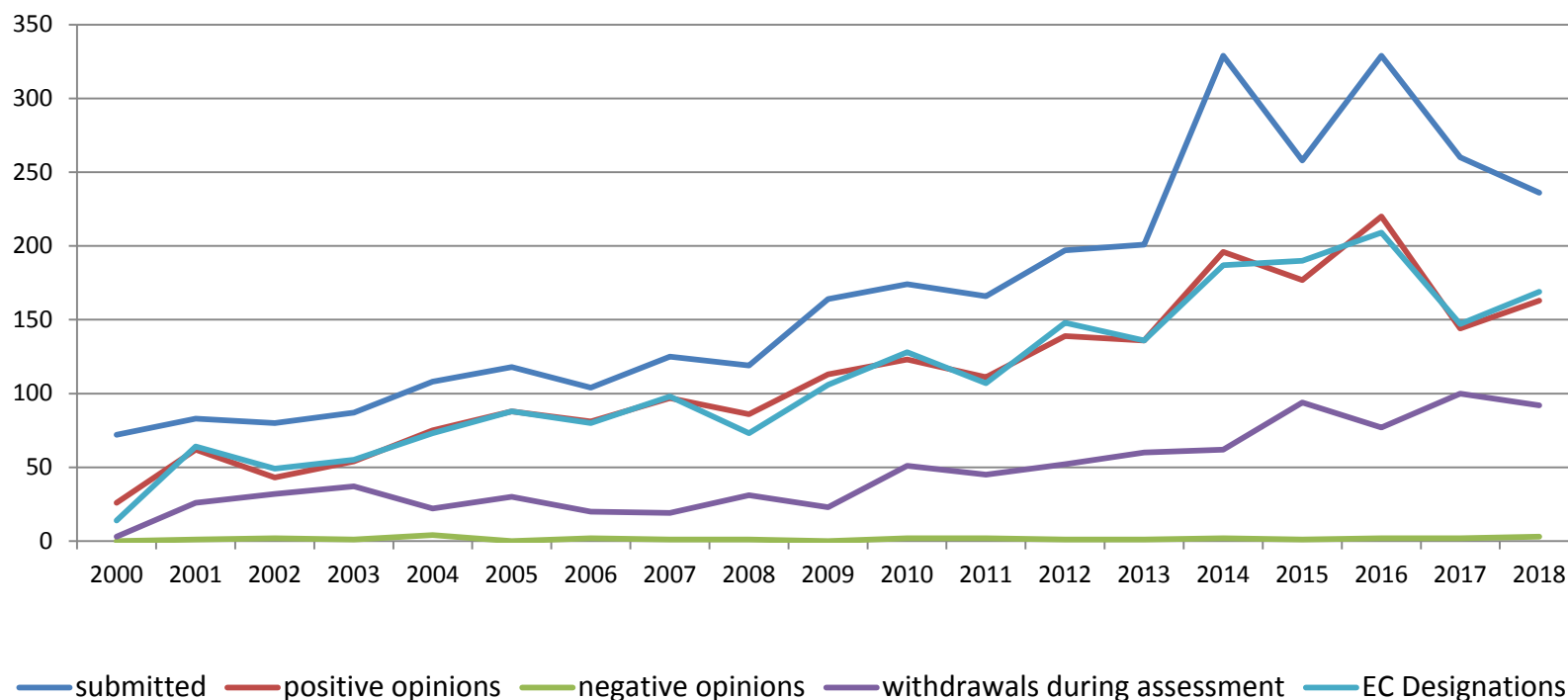


# Applications for orphan medicinal product designation

	2000	2006	2011	2016	2017	2018	<b>Total</b>
	2005	2010	2015				
Applications submitted	548	686	1151	329	260	236	3210
Positive COMP Opinions	348	500	759	220	144	163	2134
Negative COMP Opinions	8	6	7	2	2	3	28
EC Designations	343	485	768	209	147	169	2121
Withdrawals after submission	150	144	313	77	100	92	876

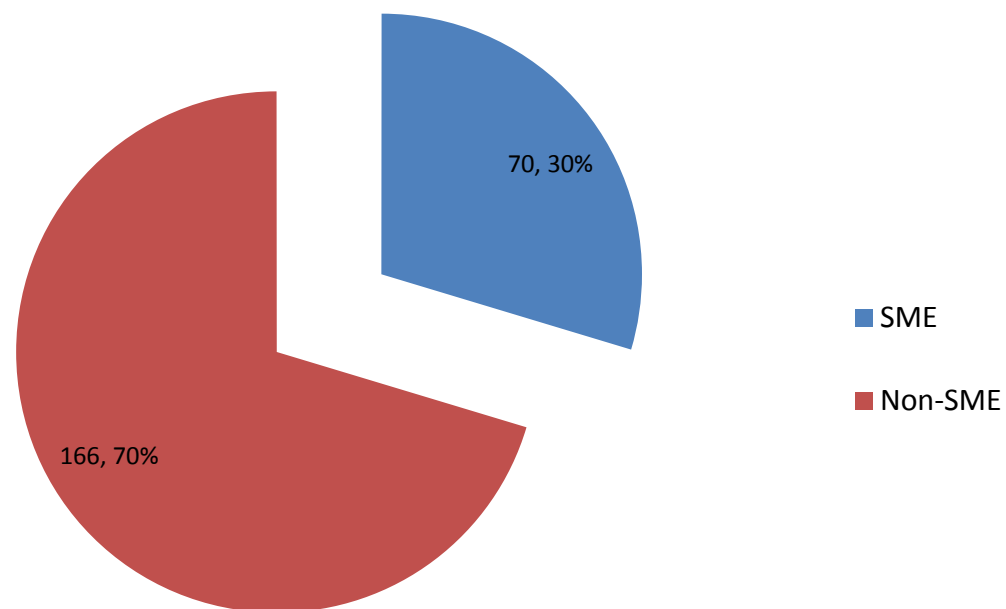


# Applications for orphan medicinal product designation



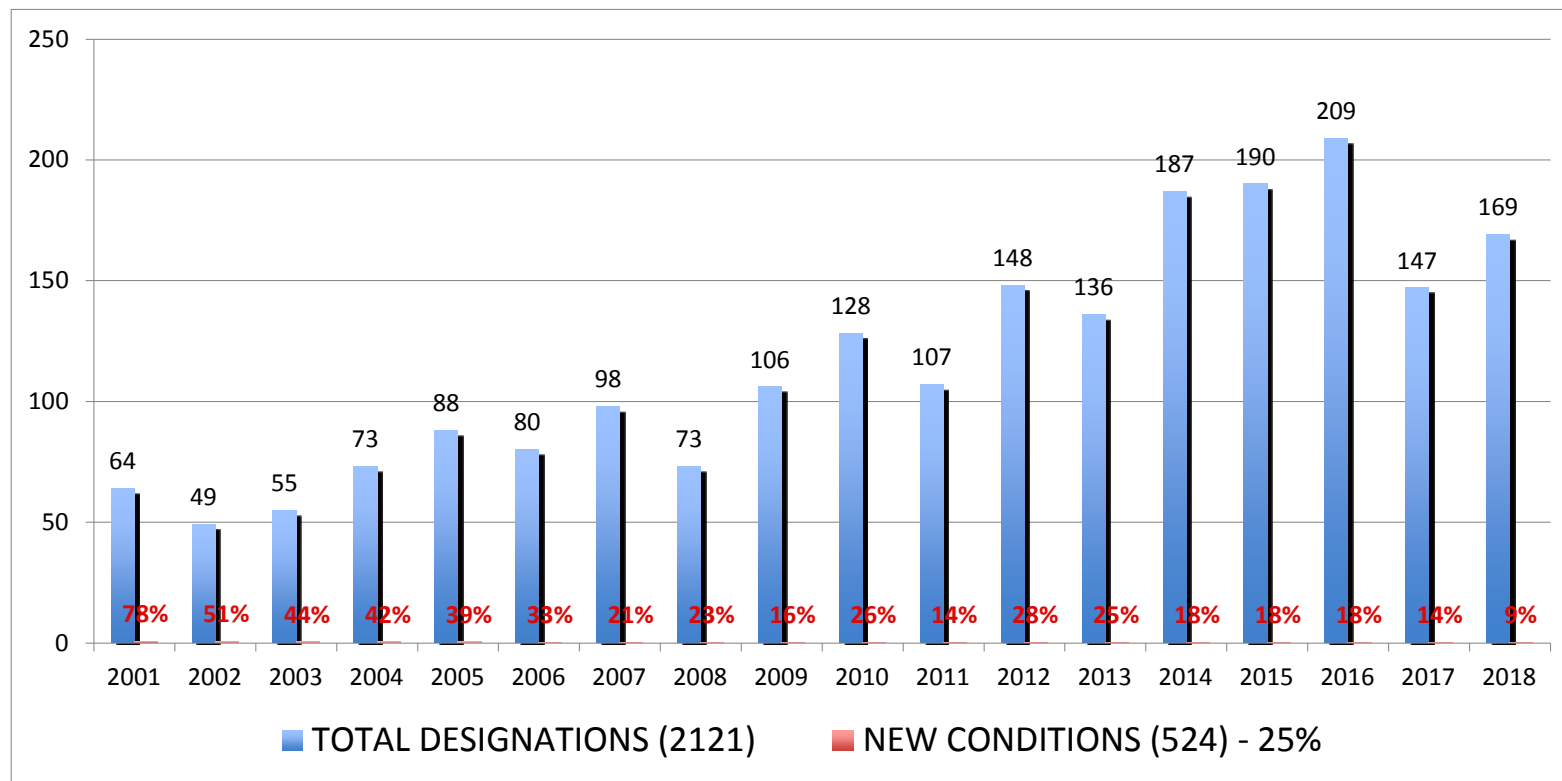


## 236 applications submitted for orphan designation in 2018



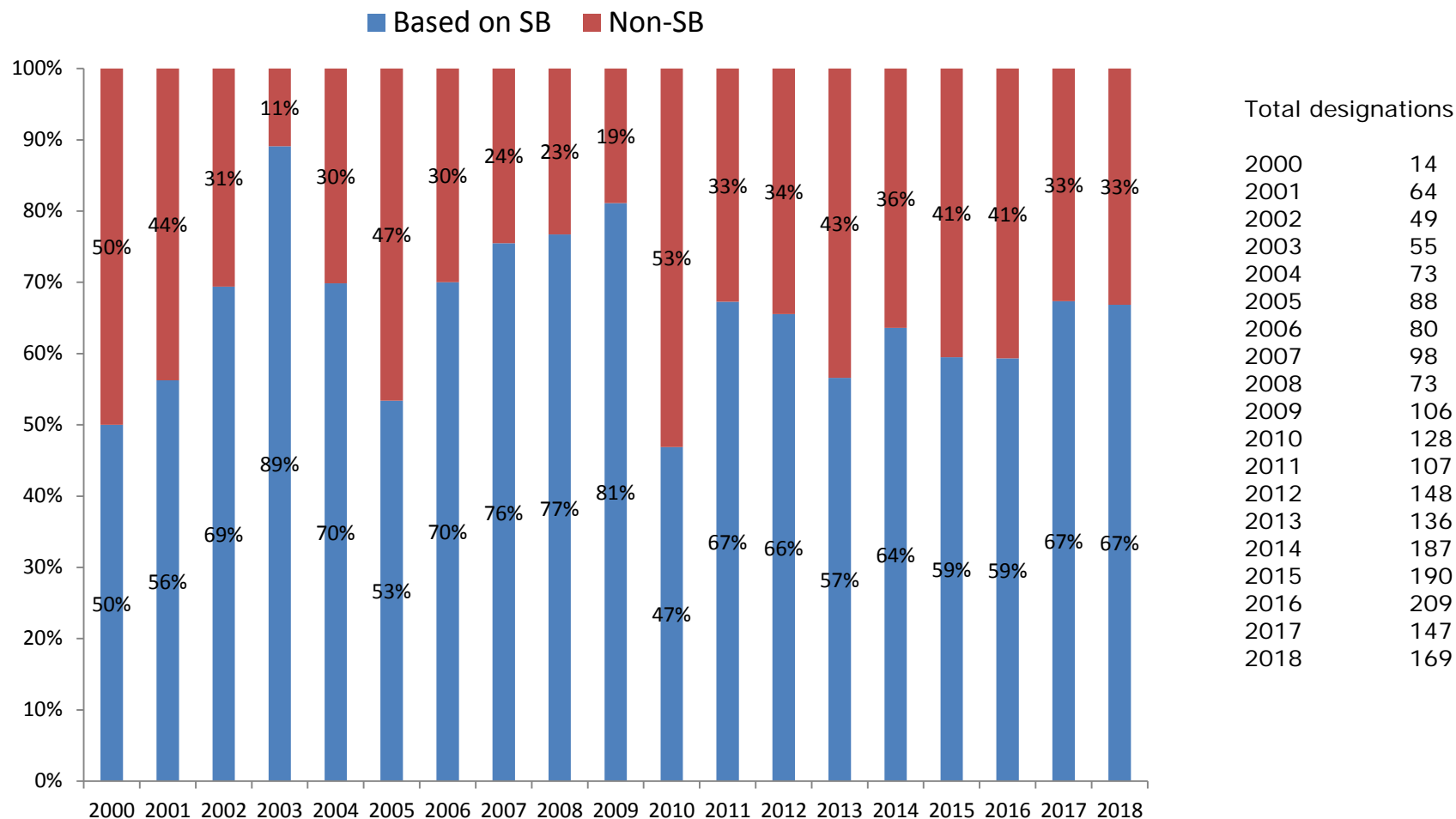


# New orphan designated conditions





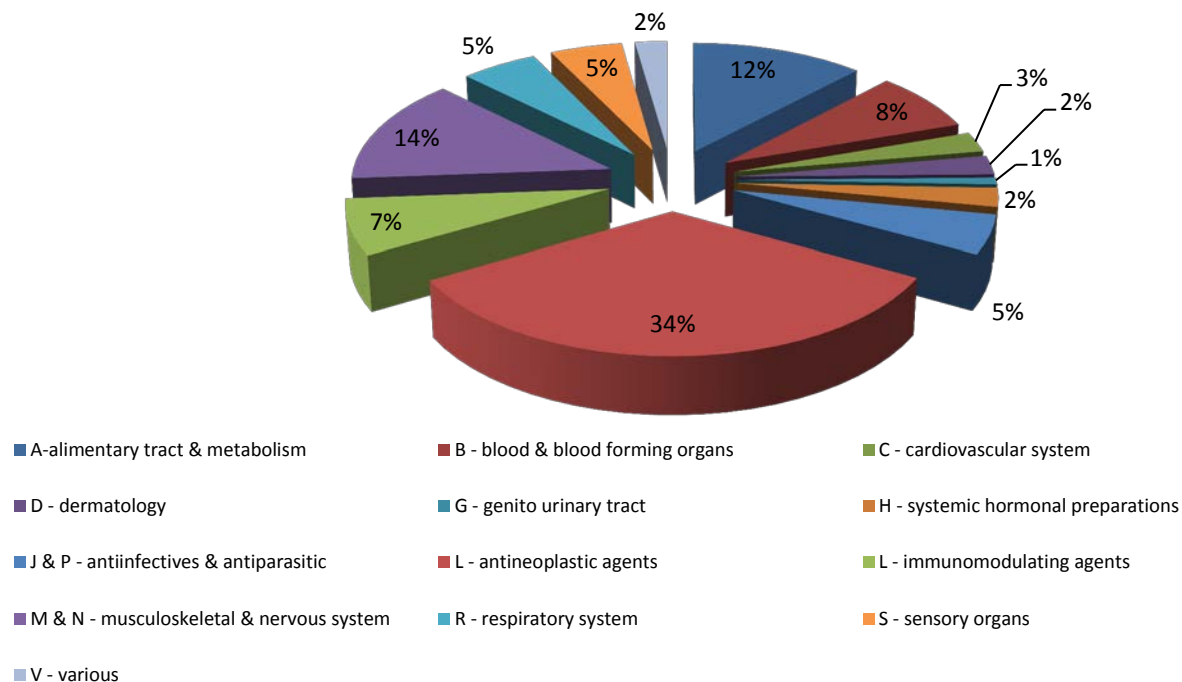
# Orphans designations based on significant benefit





# Distribution of opinions on orphan designation by therapeutic area

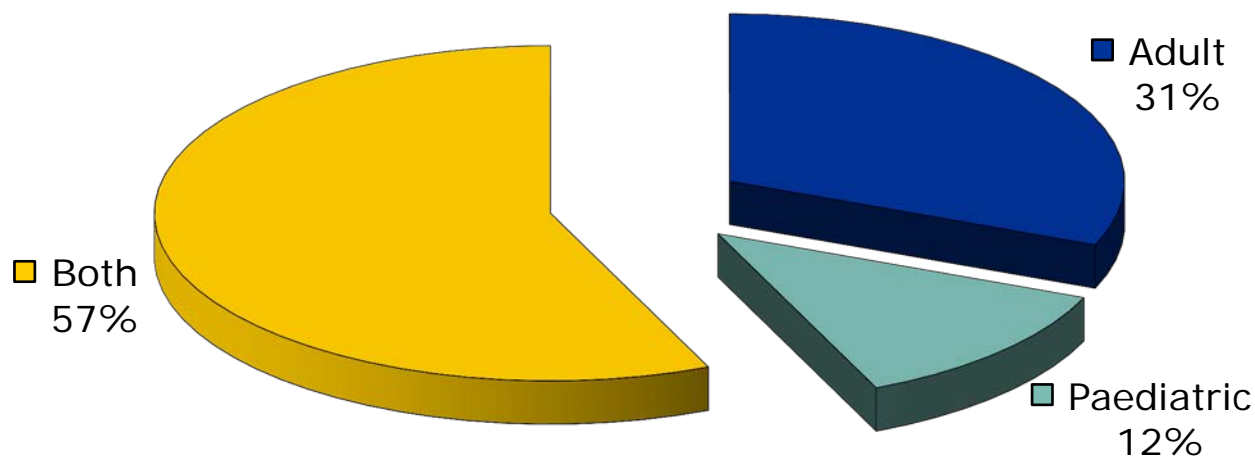
Period 2000 – 2018 / Total opinions 2134





# Distribution of orphan designations adult/paediatriac use

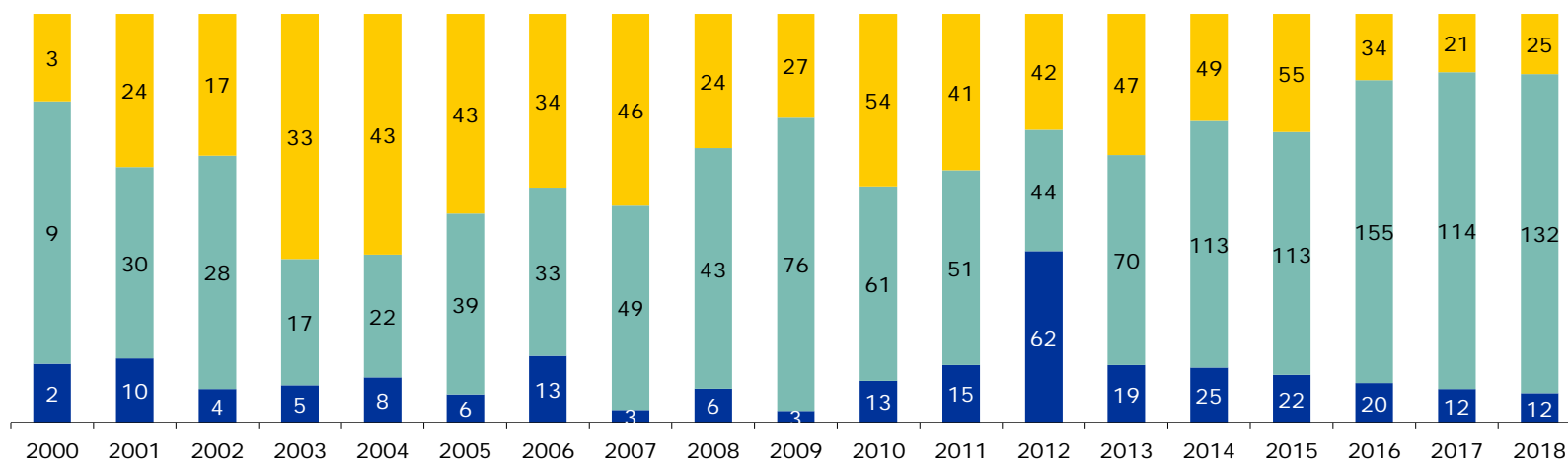
Period 2000 – 2018/ Total designations 2121







# Distribution of orphan designations adult/paediatic use

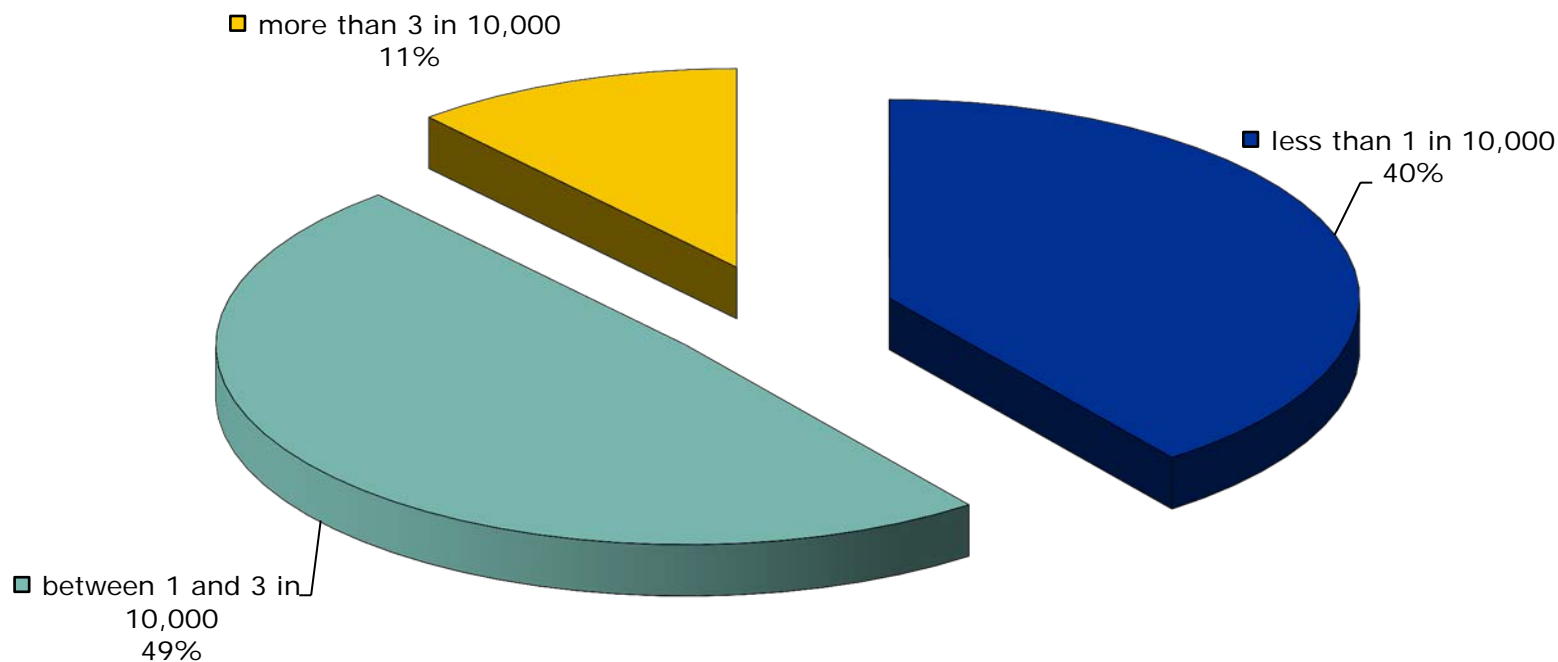


- Medical conditions affecting adults only
- Medical conditions affecting both children and adults
- Medical conditions affecting children only



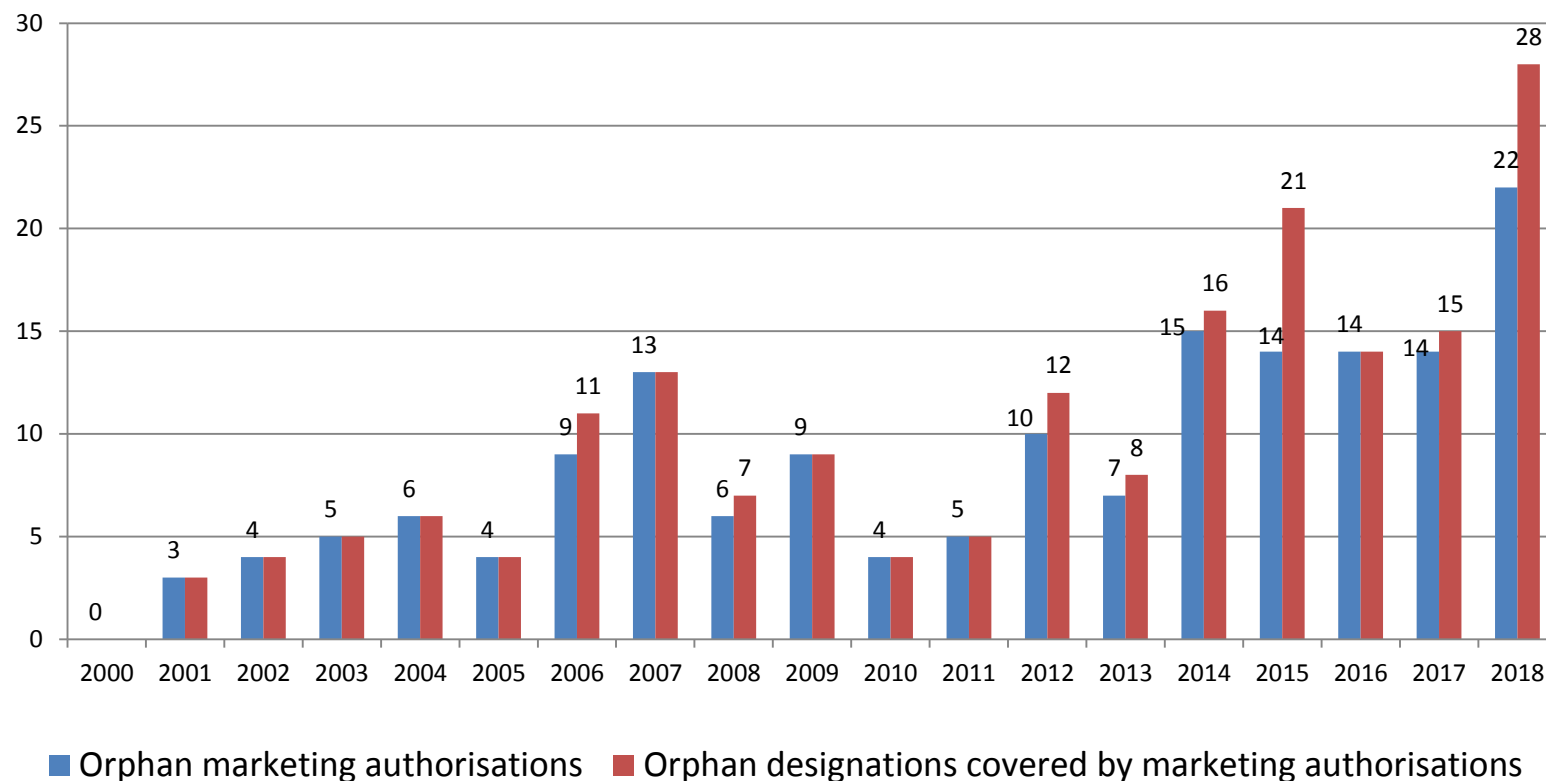
# Prevalence for designated orphan conditions

Period 2000 – 2018 / Total designations 2121



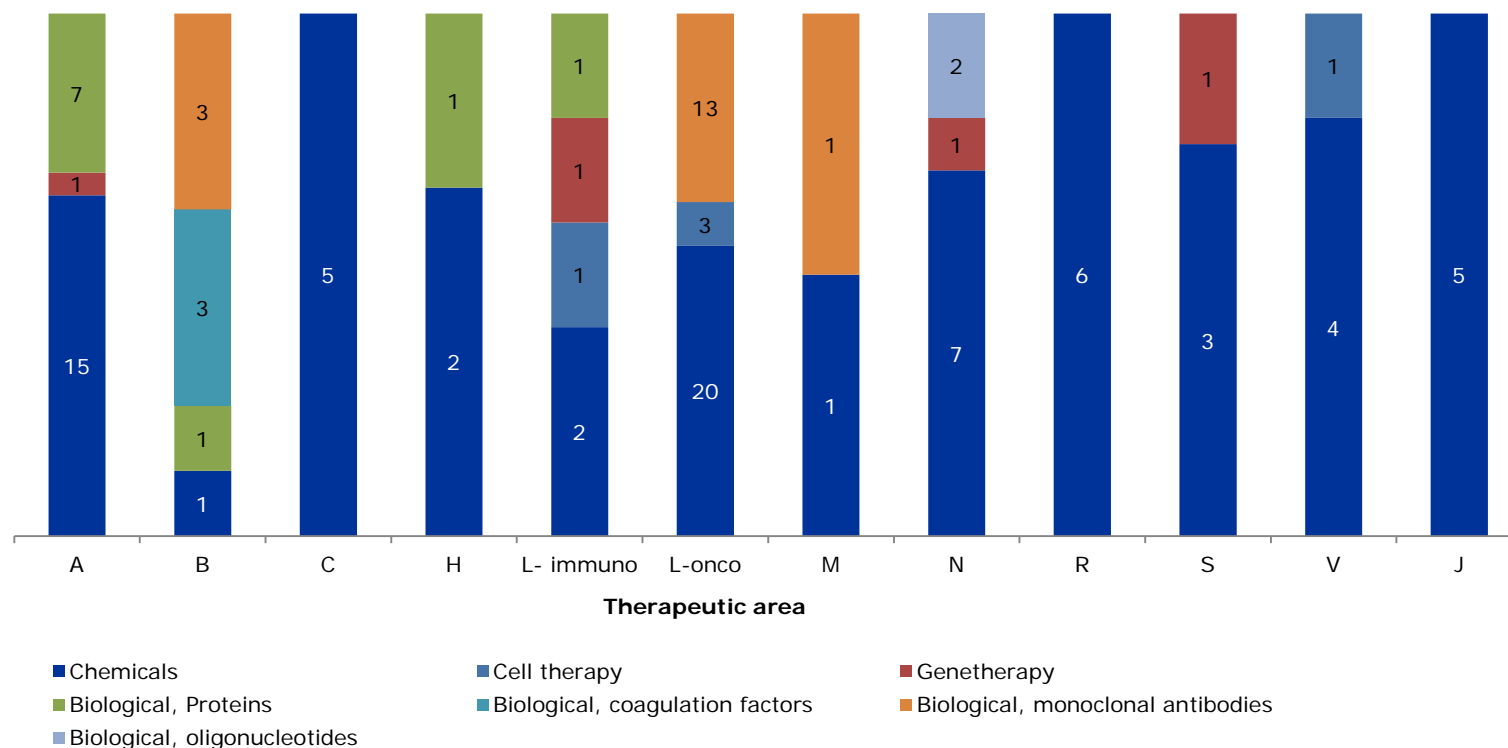


# Authorised orphan medicinal products





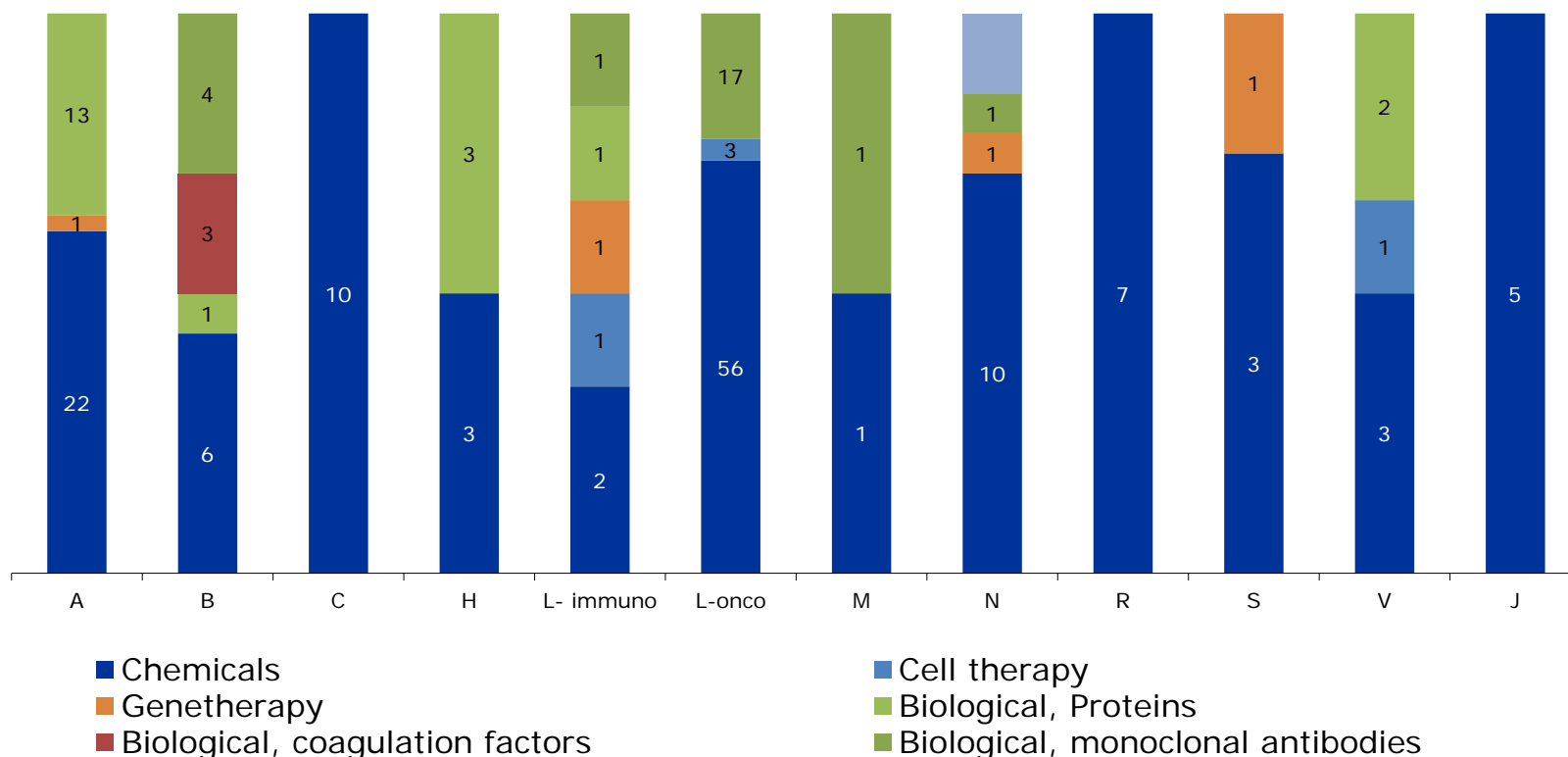
# Authorisations by type of product



112 authorisations including 5 withdrawals from the register of medicinal products human use



# Authorisations by type of product including extensions of indications



186 products including withdrawals from register of orphan medicinal products, register medicinal products human use and expired orphan status



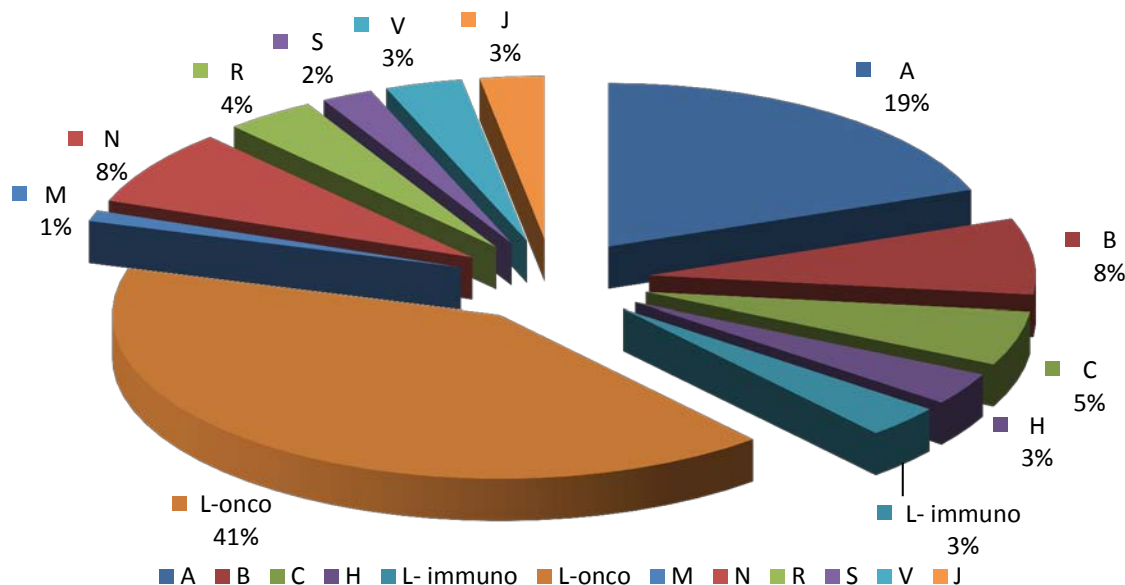
## Status of Orphan MAA

- **164** initial marketing authorisations granted and **22** extensions of indication
- Adopted opinions by CHMP awaiting EC decision: **1 ext of indication**
- On-going applications in review process: **19**
- Extensions of indication in review process: **3**
- Expired – end of market exclusivity: **40** initial MAA & **1** extension of indication
- Withdrawals
  - **68** during evaluation, including 2 extensions
  - **5** from register human medicinal products; of these 2 from orphan register
  - **18** from register orphan medicinal products after authorisation, including 6 extensions
  - **36** from register orphan medicinal products before authorisation, including 7 extensions
- Negative outcomes: **19** refusals, including 1 extension



# 164 initial orphan marketing authorisations and 22 extension of indication granted to date

- A** Alimentary tract and metabolism
- B** Haematology
- C** Cardiovascular system
- H** Systemic hormonal
- J** Anti-infectives for systemic use
- L** Immunology
- L** Antineoplastic
- M** Musculo-skeletal system
- N** Nervous system
- R** Respiratory system
- S** Sensory organs
- V** Various



Number of conditions: 124

Active orphan MA: 107

Active extension of indication: 15

Chart includes:

15 authorised extensions of indication

18 withdrawals from the register of orphan medicinal products (including 6 ext. of indication)

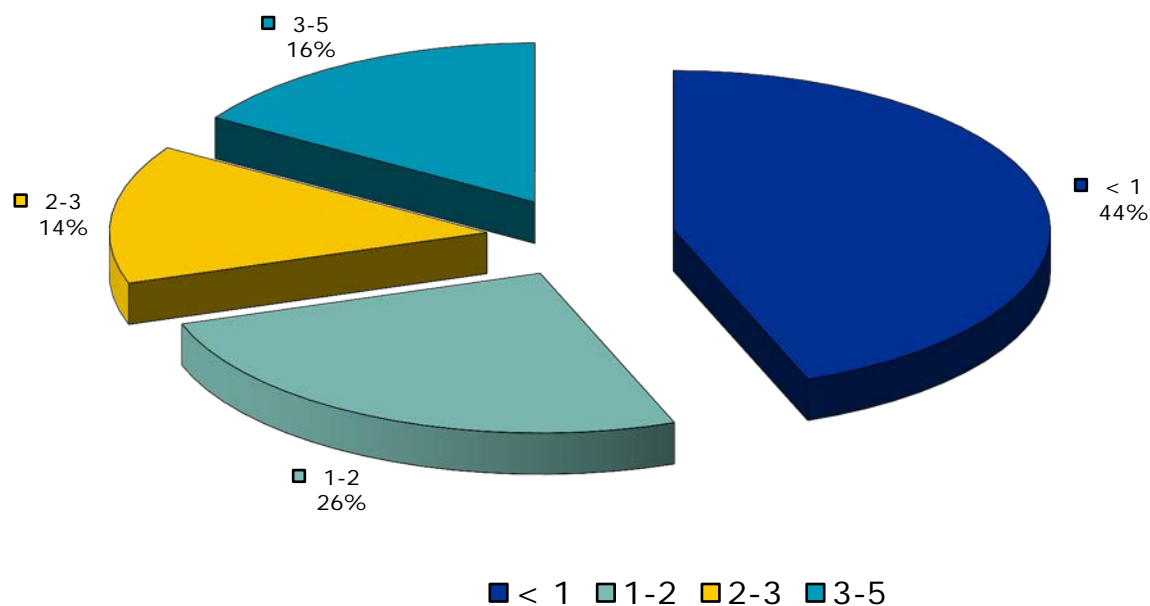
5 withdrawals from register medicinal products human use

40 removals of initial MAA from register after expire of the market exclusivity period & 1 ext of indication



# Prevalence for orphan marketing authorisations period 2000-2018

**Prevalence per 10,000 All orphan products**  
Including extensions of indication, withdrawals and products which market exclusivity has expired







# Thank you for your attention

## Further information

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