Human and veterinary pharmaceuticals regulation

Towards EU accession: Serbia's regulatory challenges, expectations and opportunities

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Republika Srbija
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Ministry of Health
Ministry of Agriculture, Forestry and Water Management,
Veterinary Directorate

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Agencija za lekove i medicinska sredstva Srbije
Medicines and Medical Devices Agency of Serbia

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Orphan medicinal products

Activities of the Committee for Orphan Medicinal Products (COMP)

Presented by: Paolo Tomasi, European Medicines Agency

Orphan designation

- Principles
  - Designation criteria
  - Incentives
  - Procedure
- Experience
  - Designations
  - Authorizations
  - Conclusions

Orphans
by Thomas Kennington

Why is there a EU legislation to stimulate development of drugs for rare diseases?

for

Ligneous conjunctivitis
~150 cases described

=

Why is there a EU legislation to stimulate development of drugs for rare diseases?

"Persons suffering from rare conditions should be entitled to the same quality of treatment as other patients"

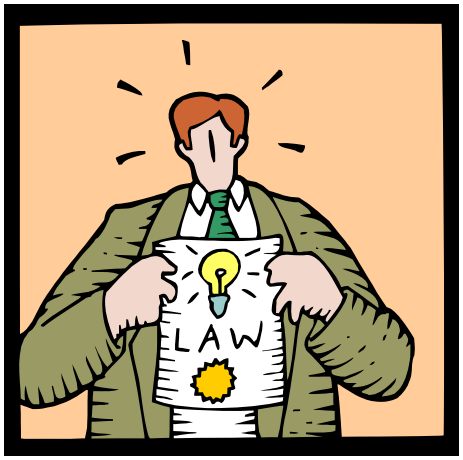
But…

" the pharmaceutical industry would be unwilling to develop the medicinal product under normal market conditions"

As…

"some conditions occur so infrequently that the cost of developing and bringing to the market a medicinal product (…) would not be recovered by the expected sales"

Orphan Regulations in the EU



Regulation (EC) No 141/2000 of the EP on Orphan Medicinal Products (16/12/1999)

- Criteria for designation
- Committee (COMP)
- Procedure
- Incentives

Commission Regulation (EC) 847/2000 of 27/04/2000

- Criteria for designation
- Similarity
- Clinical superiority

Commission Regulation (EC) 726/2004 of 20/11/2005

- Mandatory centralised MA application for orphans

COMP (Committee for Orphan Medicinal Products)

EMEA Committee: 33 (+2) members + chairperson

- 1 member per Member State (N=27)
- 6 members nominated by the European Commission
  - 3 patient representatives
  - 3 members proposed by EMA
- 2 non-voting members (Iceland and Norway)

COMP tasks:

- Opinions on designation
- To advise Commission on establishment and development of a policy on orphan medicinal products
- To assist EU Commission in liaising internationally and with patient support groups
- To assist EU Commission on guidelines

Orphan Medicines Section (EMA)

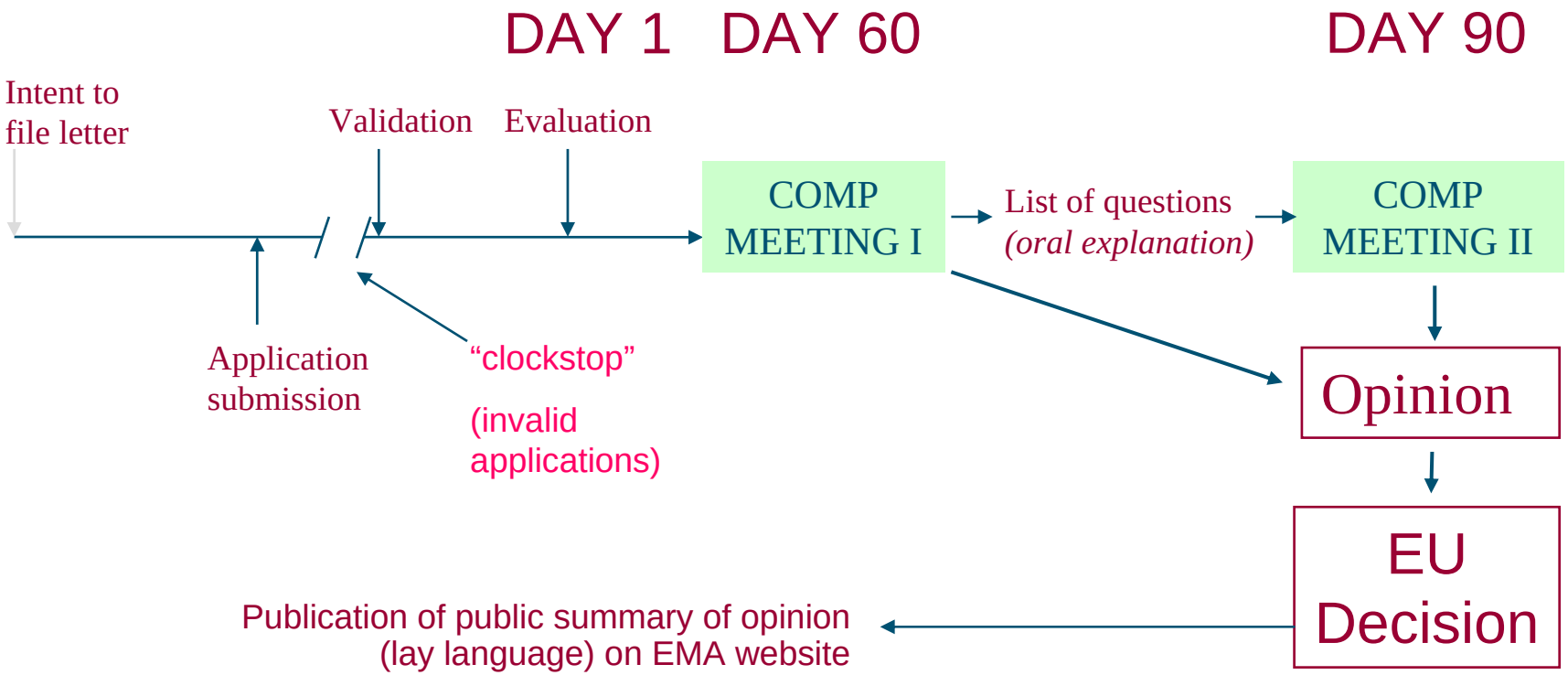
In the sector Human Medicines Special Areas

- Head of Section: Jordi Llinares Garcia
- 4 Scientific Administrators (physicians, pharmacists…)
- 3 Assistants

Tasks:

- coordination of procedures for the designation of orphan medicinal products:
  - Preparation of Summary Reports on applications
  - Scientific and Administrative secretariat of COMP
- Coordination of the review of orphan designation criteria at the time of granting/varying a marketing authorisation
- coordination of the review of market exclusivity of authorised orphan medicine products 5 years from the granting of the marketing authorisation

Overview of orphan designation process



Orphan designation

- For medicinal products for human use
- Procedure is free of charge 
- Can be requested at any stage of development, but before request for MA (even 1 day)
- Sponsor can be either company or individual
  - Established in the EEA (EU, Iceland, Lichtenstein, Norway)
- European Commission Decision gives access to incentives 
- Centralised procedure for MA compulsory

Orphan designation: 3 criteria

1: "Prevalence" criterion

Prevalence

(≤ 5 / 10,000)

or

Insufficient return on investment

(costs > expected revenues)

2: "Seriousness" criterion

Life-threatening or chronically debilitating

Life-threatening, seriously debilitating or serious and chronic

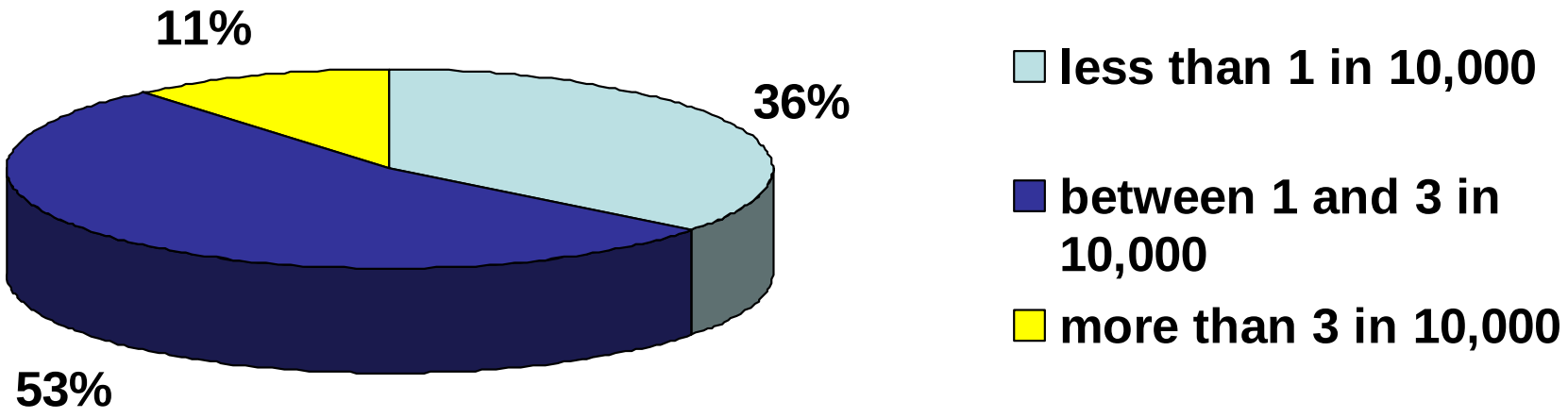
3: "Significant Benefit" criterion

Are there available "methods" for diagnosis / prevention / treatment?

NO → OK

YES → Significant benefit / current methods non satisfactory

Prevalence of designated orphan conditions (must be <5/10,000)



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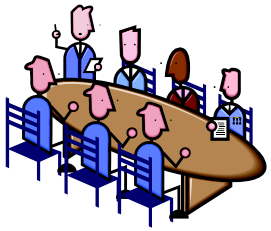
Incentives (1/3)

Economic / marketing:

- Fee reduction / exemption
  - Extended incentives for SMEs (post authorisation)
- Market exclusivity (10 years)

Product development:

- Free protocol assistance (Scientific Advice for Orphan Medicinal products)



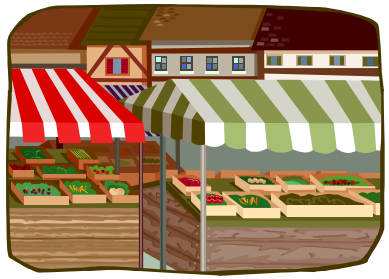
Community marketing authorisation

National incentives (EC inventory)

Incentives: economic/marketing (2/3)

- Fee reductions:
  - 50% market authorisation application
  - 100% protocol assistance
  - 100% post authorisation fees

- 10-year market exclusivity
  - protection against:
    - similar products (structure/mechanism of action
    - for the same indication
  - Four possible derogations:
    - Sponsor's consent
    - Lack of supply
    - Clinical superiority of another similar product
    - Review after 5 years at MS request



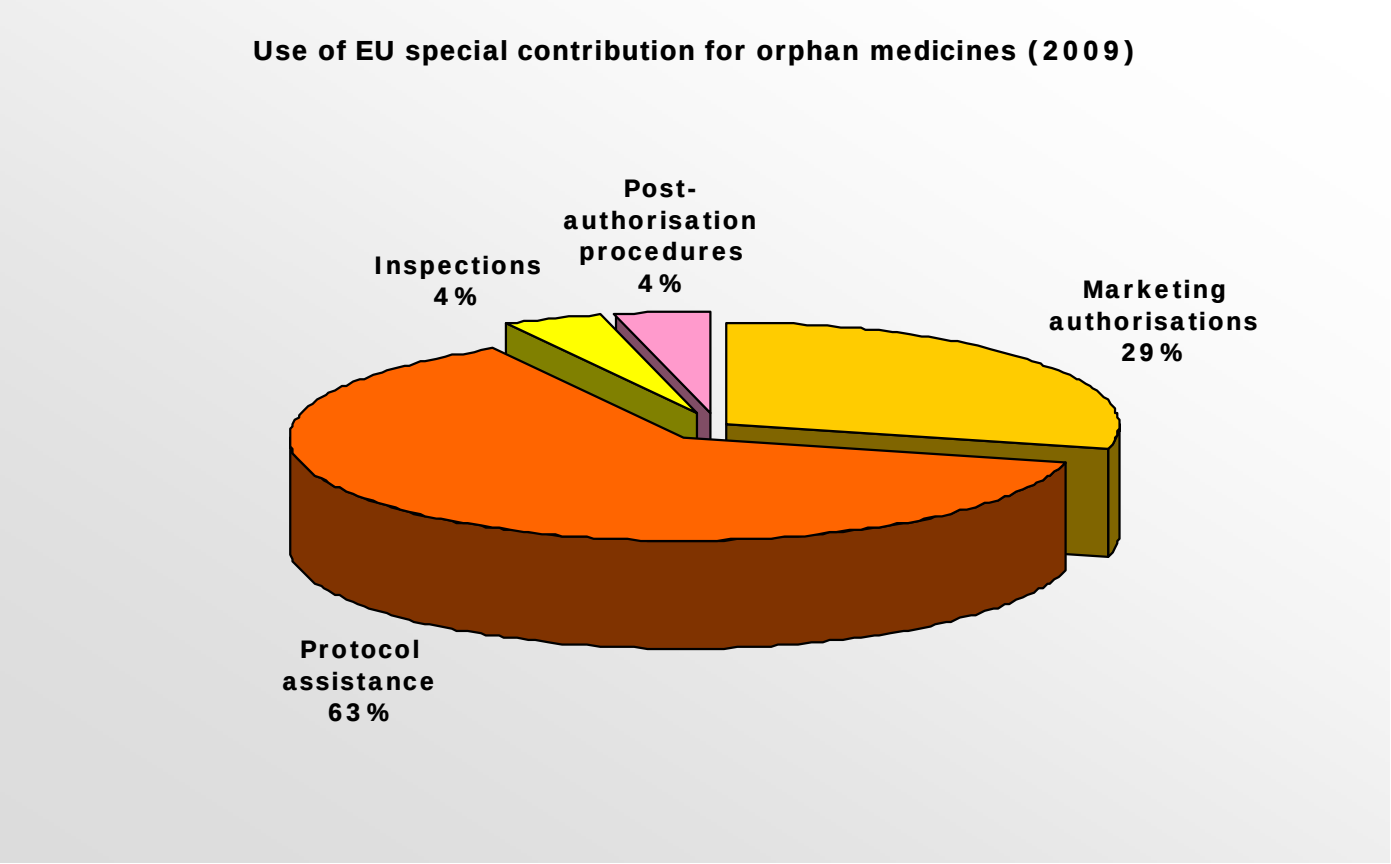
Incentives: product development 3/3

Protocol assistance

- Protocol assistance ≅ scientific advice for orphans
  - Questions on quality-efficacy-safety
  - Questions on significant benefit
  - Company position required
  - SAWP provides answers
  - CHMP adopts answers
  - COMP involved if issues on benefit

(small) differences PA / SA

Use of incentives fund



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Orphan designation is “competitive”

Designation can be granted for the same orphan indication, to two or more sponsors, even for similar or identical products

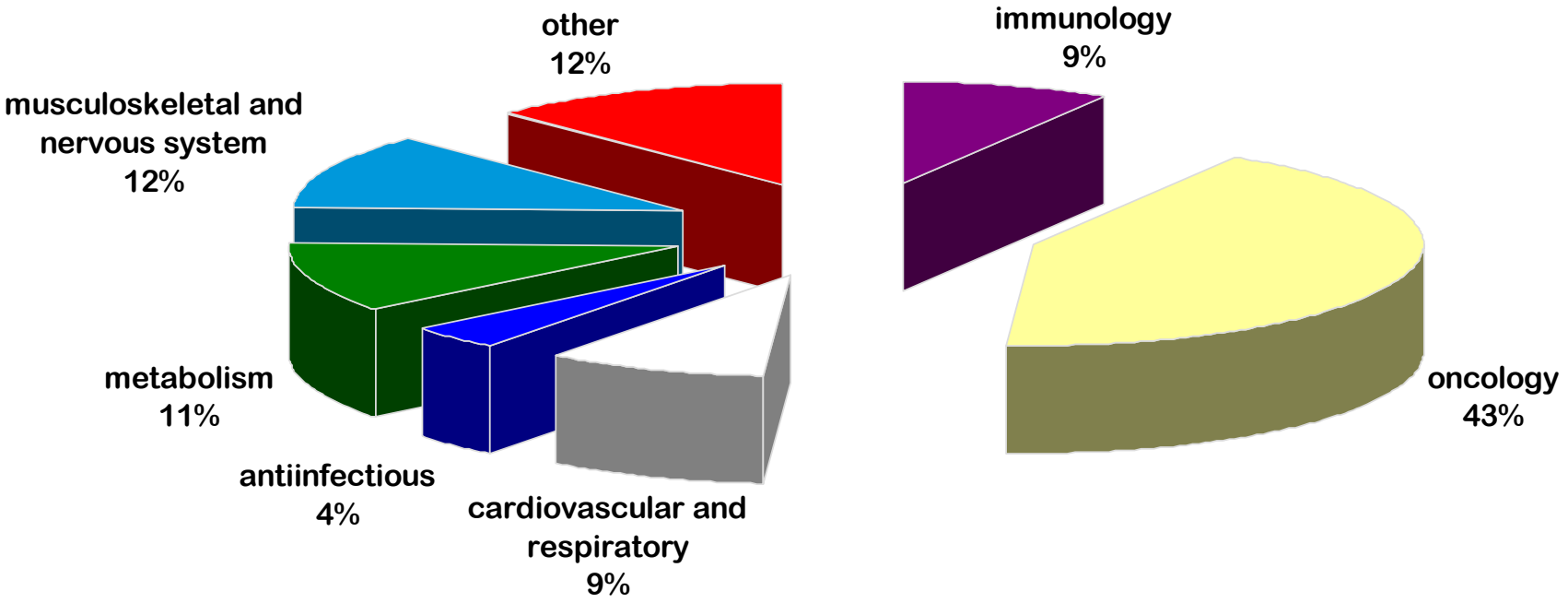
First sponsor with MA for an orphan indication obtains exclusivity (for the therapeutic indication)

Subsequent sponsor(s) for the same therapeutic indication - to break exclusivity:

Orphan applications 2000-2009

	2000	2001	2002	2003	2004	2005	2006	2007	2008	2009	Total
No. of applications submitted	72	83	80	87	108	118	104	126	119	164	1061
Positive Opinions	26	64	43	54	75	88	81	97	86	113	727
Commission Decisions	14	64	49	55	72	88	80	98	73	106	699
Negative Opinions	0	1	3	1	4	0	2	1	1	2	15
Withdrawals	6	27	30	41	22	30	20	19	31	23	249

Designations by therapeutic area



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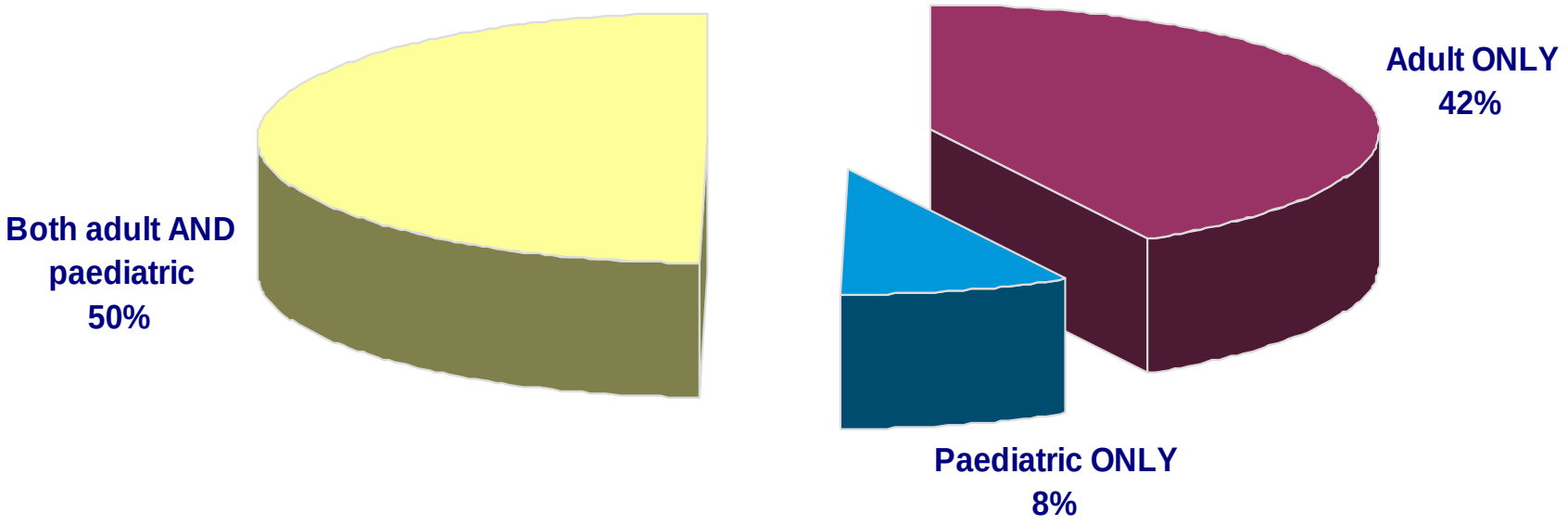
Orphan conditions affecting children



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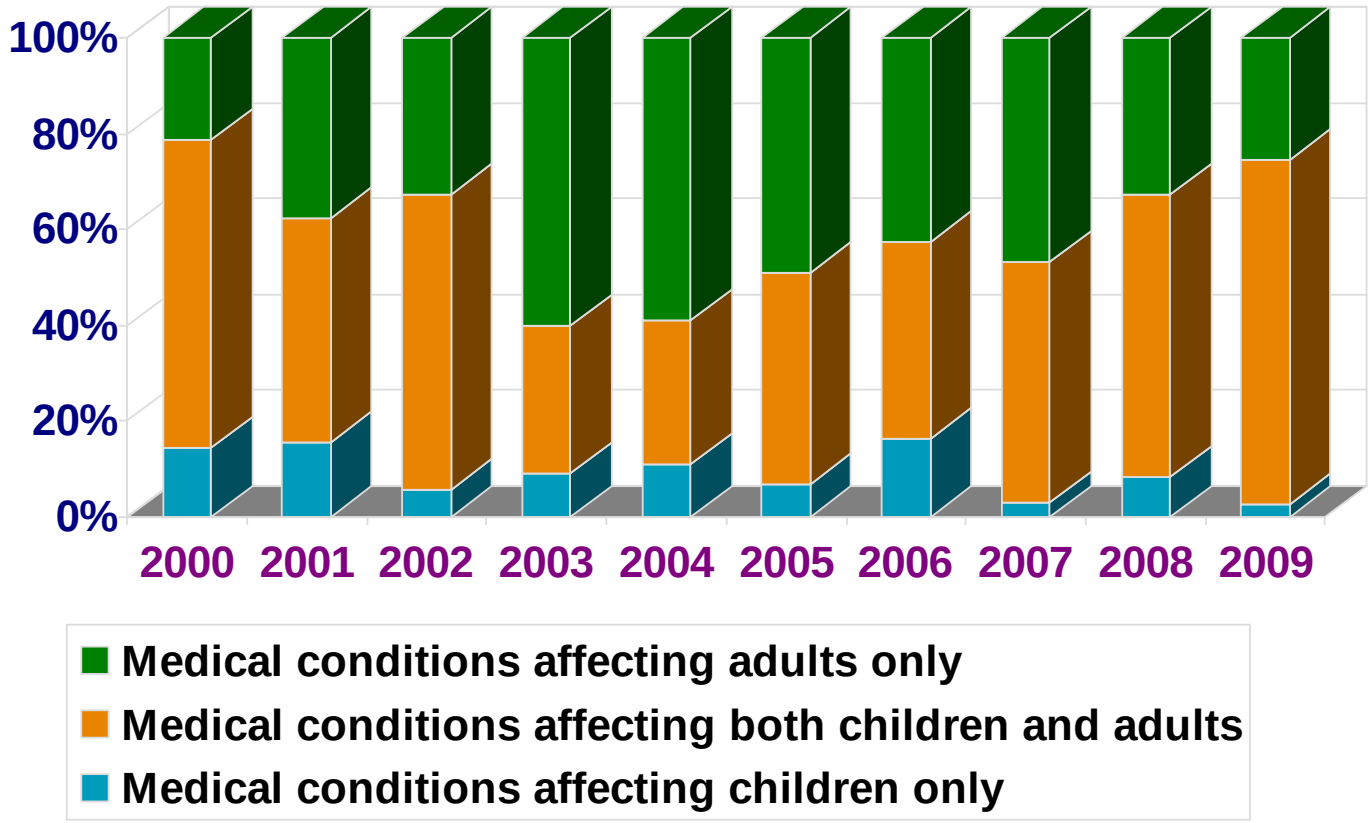
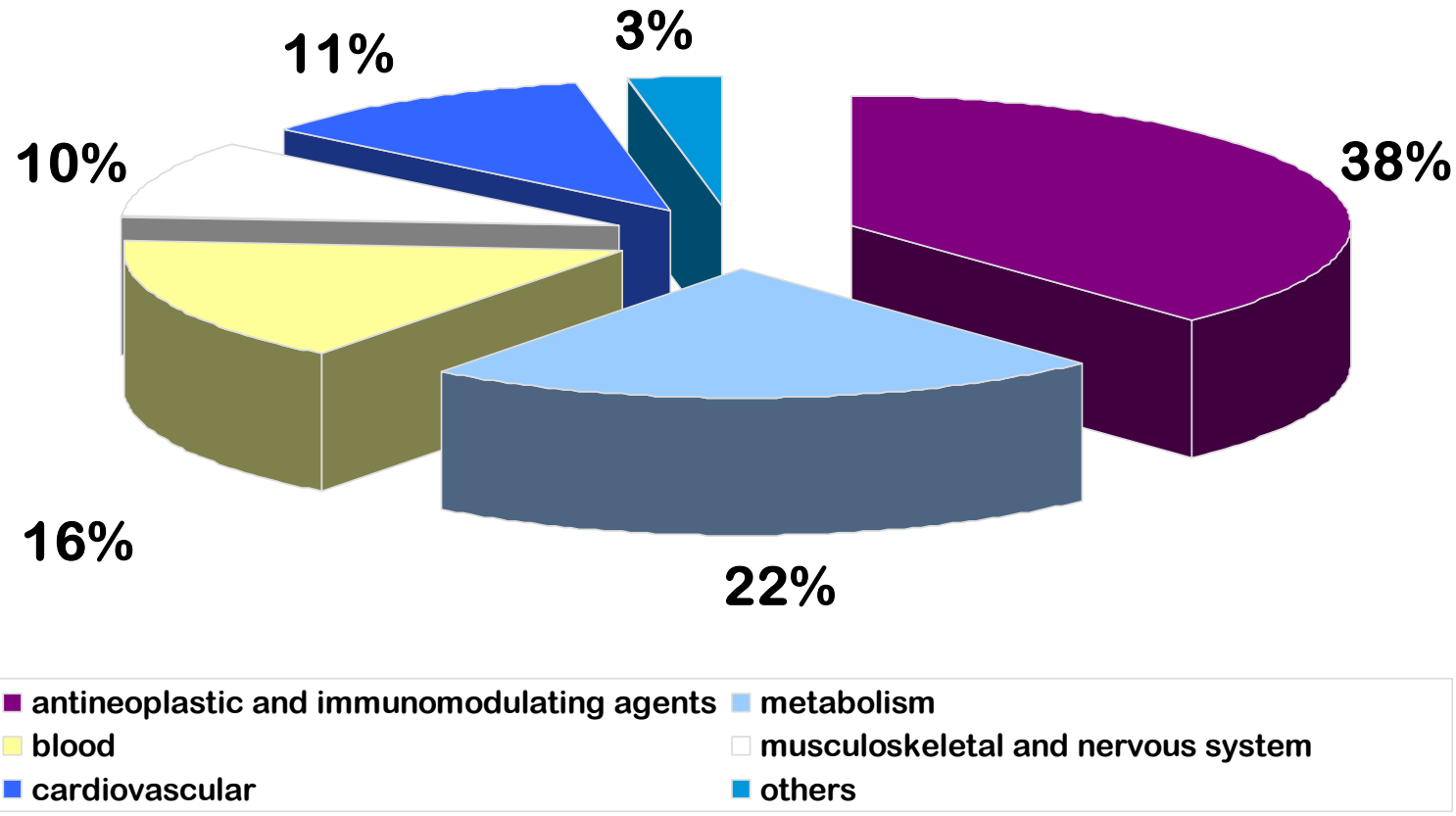


Therapeutic areas of the 63 authorised orphan medicinal products



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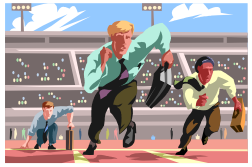
Conclusions

- 90-day procedure
- 3 legislation requirements (rarity/return, prevalence, no other treatments/unsatisfactory/benefit)



- Designation is competitive
- Orphan indication (at designation) is not necessarily the same as therapeutic indication at MA
- Significant benefit to be:
  - Claimed at OD application stage, **if** authorised drugs exist (or satisfactory non-medicinal treatments);
  - Confirmed at MA with relevant data

Thank you for your attention

Questions?