Nederlandse Federatie van Kankerpatiëntenorganisaties

Patient representation at EMA committees

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What does the COMP do?

EMA Committee: 33 members + chairperson (+ 2)

- 1 member per Member State (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 Commission in liaising internationally and with patient support groups
- To assist Commission on guidelines

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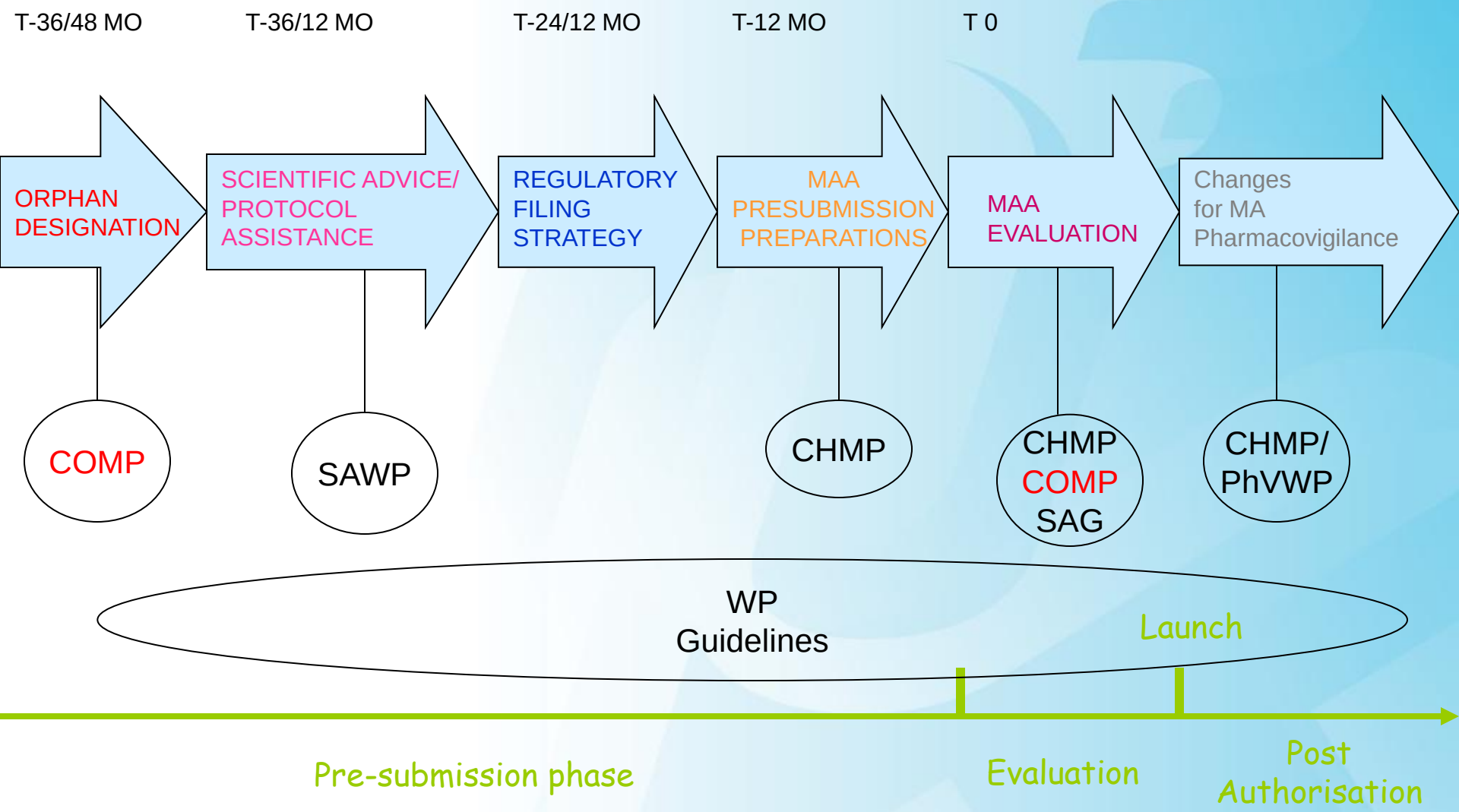
Phases of drug development



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Advantages of Orphan Drug Designation

- Economic / marketing
  - Fee reduction / exemption
  - Market exclusivity
- Product development
  - Protocol assistance
- Community marketing authorisation
- National incentives

- Spin off: attraction of investors money in development phase

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Orphan drug designation - criteria

- Rare disease, prevalence
- Life-threatening
- No other therapy available
- If other therapy available→ significant benefit
  - Major contribution to patient care
  - Improved efficacy as compared to what is authorised
  - Improved safety as compared to what is authorised

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Patient representatives in the COMP

- Present from the beginning in 2000
- Full voting members
- Coordinators at time of designation and at review when MA granted
- Liaising with patients with disease specific experience
- Advising the Commission on issues relating to orphan drugs and orphan diseases

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Patients role

Broadly we:

- Represent patients interests and provide a "patient perspective" view, on behalf of those directly affected by regulatory decisions
- Raise ethical issues during the discussion; identify ethical risk factors, propose risk prevention and minimisation measures
- Contribute, in a general capacity, to public health (raise awareness, where appropriate, of the public health impact of regulatory decisions)

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Patients role

Specifically we

- Grant Orphan Drug designation to products
- Contribute to the preparation of COMP Public Summaries of Opinion
- Contribute to Protocol Assistance procedures for Scientific Advice Working Party (SAWP)
- Upon request or own initiative, contribute to advising the Commission on the establishment and development of policies on orphan drugs for the EU
- Upon request or own initiative, contribute to assisting the Commission in liaising internationally on matters relating to orphan drugs and patients' support groups.

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Granting designation

- Product should have significant benefit:
  - Better efficacy
  - Better safety
  - New mechanism of action
  - Availability:
  - Major contribution to patient care
    - Ease of administration

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Bringing in expert patients

- Identify patients and brief them on their tasks in general
- Expert pats can play important role in granting designations

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Advising European Commission

e.g.

- COMP comments on EU documents like Commission Communication on rare diseases
- Involved in lobby for research grants for OD development in FrameworkPrograms (DG research)
- Input in legislative or guidance documents at EC level of EMA level

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Qualifications / Time investment

Qualifiations:

- To become a trained COMP member: attend the Eurordis summer school
- Training on the job by other COMP patient representatives
- COMP handbook
- Able to communicate in English

Time investment

- Meeting 2-3 days per month in London
- Preparation time 10-20 hours