EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Experience with the review of the orphan designation in the context of extension of indication

3rd Industry Stakeholder Platform on R&D support, 18 May 2018

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Key elements of the Notice

Availability: *"Significant benefit" should not be based on: possible increased supply/availability due to shortages of existing authorised products or to existing products being authorised in only one or a limited number of Member States. (Exceptions may be made if the sponsor has evidence of patient harm)"*

Hospital formulations: *"magistral formula" and "officinal formula" may be considered as satisfactory treatment if they are well known and safe and this is a general practice in the EU.*

Prevalence "0": *"or a prevalence of approximately zero in the EU"*

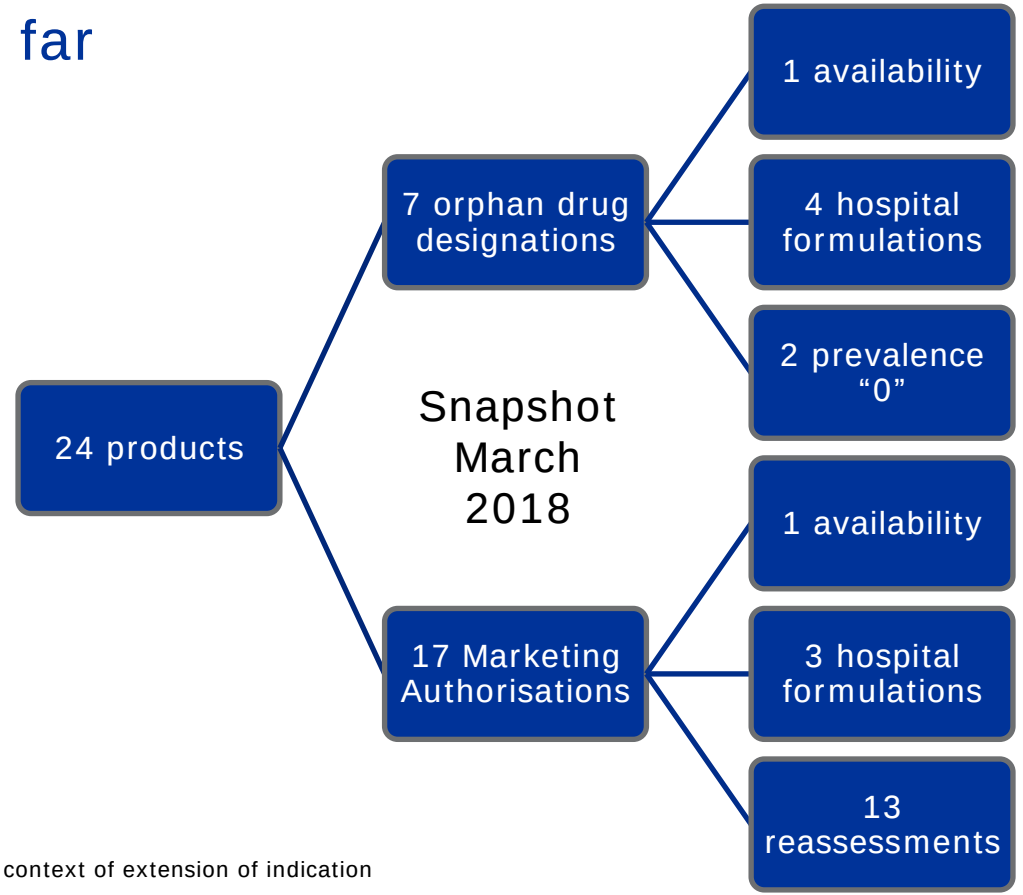
Reassessment: *"This verification should cover regulatory procedures relating to the addition of a new therapeutic indication or to the modification of an existing one (e.g. major type-II variations or extension of the marketing authorisation)."*

1 Experience with the review of the orphan designation in the context of extension of indication

Overview of experience so far

Since entry into force of Notice 18.11.2016:

- 24 products affected in total
- 7 at orphan drug designation
- 17 at time of marketing or extension of indication

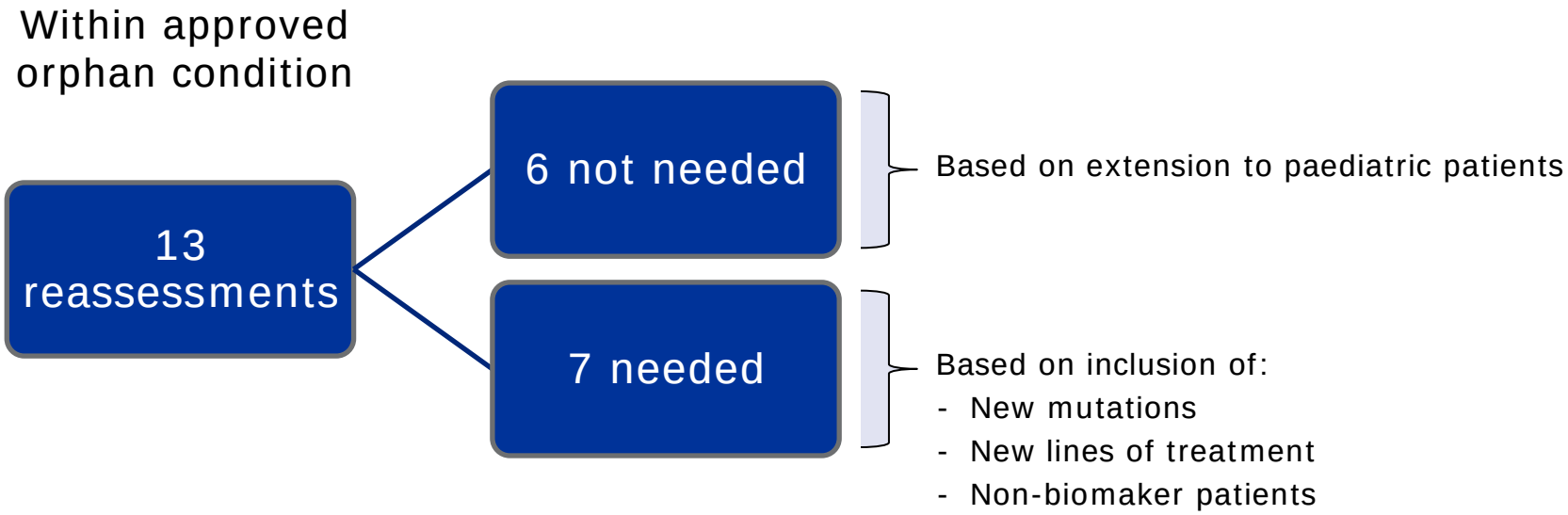


Impact at Orphan Drug Designation

Impact on COMP work resources:

- 7 products out of >250 designated in 2017 is about 3%.
- Limited impact on COMP resources.

Market Authorisation - reassessment at extension of indication



Clarification on reassessment

> *"to further therapeutic indications within the same orphan condition or to vary the indication as a first-line treatment"*

- No reassessment of
  - Extensions to different age groups. The COMP considers that there are no changes to the clinical characteristics of the condition by changing the age of the target population and as such there is no "*justified and serious doubts*".
  - If nothing is approved for that "new" target population (within the same condition).
  - Already authorised indication for which the COMP has already done an assessment.
- Reassessment needed if target patient population is broadened (within the orphan condition) e.g.:
  - New line of treatment
  - New subgroup (severity, biomarker, mutations etc.)

Marketing Authorisation

Impact on COMP work resources:

- 9 reassessments out of 29 procedures in 2017 is about 30% of all COMP work on marketing authorisations.
  (or an ~ 45% increase in work compared to 2016.)
- Large impact on COMP resources.

What is needed from the applicant?

Same procedure as for as for initial maintenance:

- Same type of maintenance report from applicant
- Similar timelines (same as for accelerated assessment)
- Same level of assessment by COMP
  - Consistency and reliability for applicant as well as COMP
  - Same challenges as for initial MA (establish a significant benefit over all authorised products)
- Protocol Assistance can be requested

Process for reassessment, extension of indication