



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Optimising the guidance on significant benefit demonstration in the context of protocol assistance

Recent developments stimulated through the work of both COMP and SAWP

Industry stakeholder platform on research and development support

Presented by Matthias Hofer on 25 April 2017
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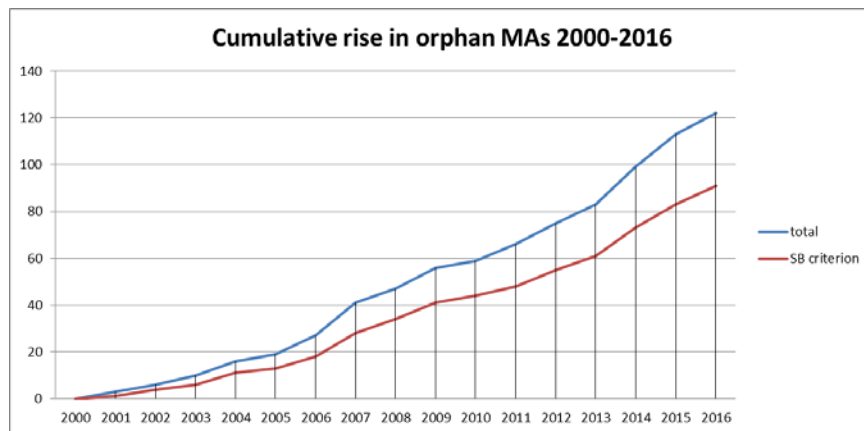
Glossary:

- SB...significant benefit
- PA...EMA protocol assistance
- SA... EMA scientific advice
- PA+SB... EMA protocol assistance procedure with COMP SB question
- MA... marketing authorisation
- MAA... marketing authorisation application





Orphan environment after 16 years of EU orphan legislation

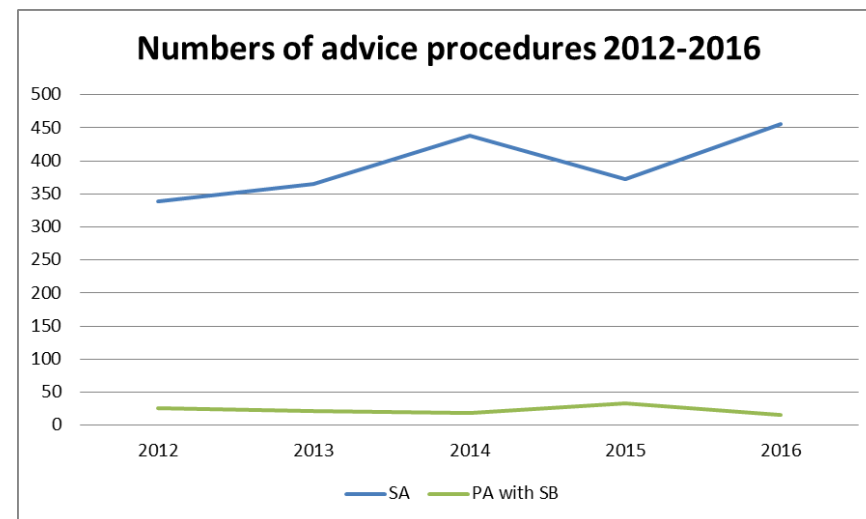


- Success of EU orphan legislation
- 128 orphan MAs until 2016 and rising
- ~ 75% require demonstration of SB
- Crowded areas, e.g. oncology, haematology, pulmonology
- Importance of adequate data to support SB



Orphan environment after 16 years of EU orphan legislation

- Observations on orphan MAAs in 2016
 - Products that received previous PA+SB kept orphan status
 - Rise in appeal procedures on COMP SB assessment
 - More important role for PA+SB
- The number of PA+ SB procedures not rising over the last years





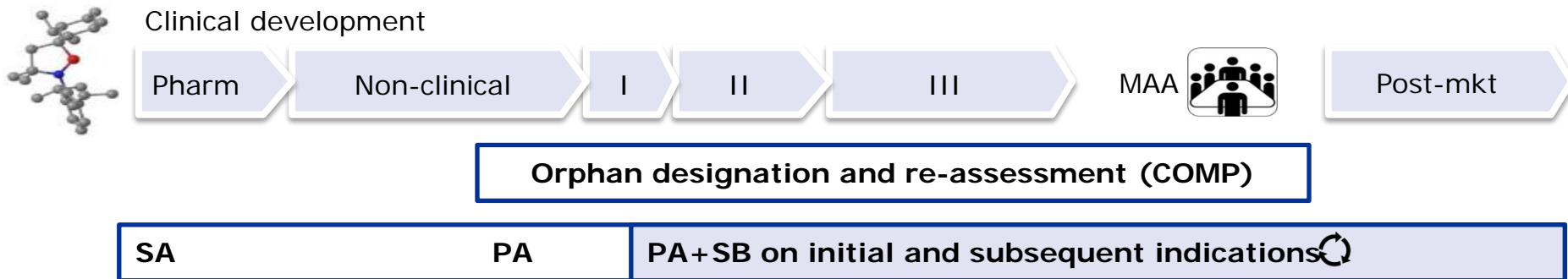
COMP priority is to foster early dialogue on SB via EMA PA procedure

Kind invitation for sponsors to use the tool of PA+SB



COMP raises their hands for Rare Disease Day 2017

When should SB question be asked?





Iterative PA



- Cumulative body of evidence over time
- Updates to clinical development
- Changes in regulatory environment, e.g. EC notice 2016/C 424/03
- Changes in authorised competitors



- PA has reduced fee structure to allow for iterative PA process
- PA fee incentives can be combined with SME fee reductions
- SB question has to be part of PA procedure with other questions
- Combination with other initiatives, e.g. PRIME, HTA parallel advice




SB question and COMP answer



- Provide clear methodology for demonstration of SB, e.g. indirect/direct comparisons, details on quality of life tools (validation, disease specific, reduction of treatment burden)



- Efforts of the COMP to provide better guidance
 - Improved SB answer template with a clear conclusive statement 
 - Proactive discussions on hot topics with network support, i.e. indirect comparisons, major contribution to patient care
- If uncertainties remain, or updates are necessary: clarification request or follow up PA

Who provides COMP SB answer?



COMP-SAWP
coordinator
as link to EU network



COMP
peer review



COMP PA
working group



COMP

more expertise, better peer-review and more time for critical discussion



EMA support



- EMA SA officer: one single point of contact for PA
- EMA orphan office
 - Support for PA presubmission meeting/ PA validation process
 - General requests



- Scientific publication on the scientific assessment and grounds of significant benefit (due to be published in 2017)
- Scientific publication on orphan marketing authorisation and protocol assistance (due to be published on 2017)



Thank you for your attention

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