



Medicines & Healthcare products  
Regulatory Agency

# PCWP - HCPWP Joint Meeting: COMP update March 2017

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On behalf of the COMP



# Committee for Orphan Medicinal Products

- Sponsors who obtain orphan designation benefit from protocol assistance, market exclusivity and fee reductions
- 'Protocol assistance' provides sponsors with the scientific advice regarding the requirements to demonstrate quality, safety and efficacy
  - a process that increases the chances of successful approval and aims at speeding up patient access to an authorised product
- The EMA policy on the level of fee reductions reflects the priority given to 'protocol assistance' and the support to small and medium-sized enterprises (SMEs)

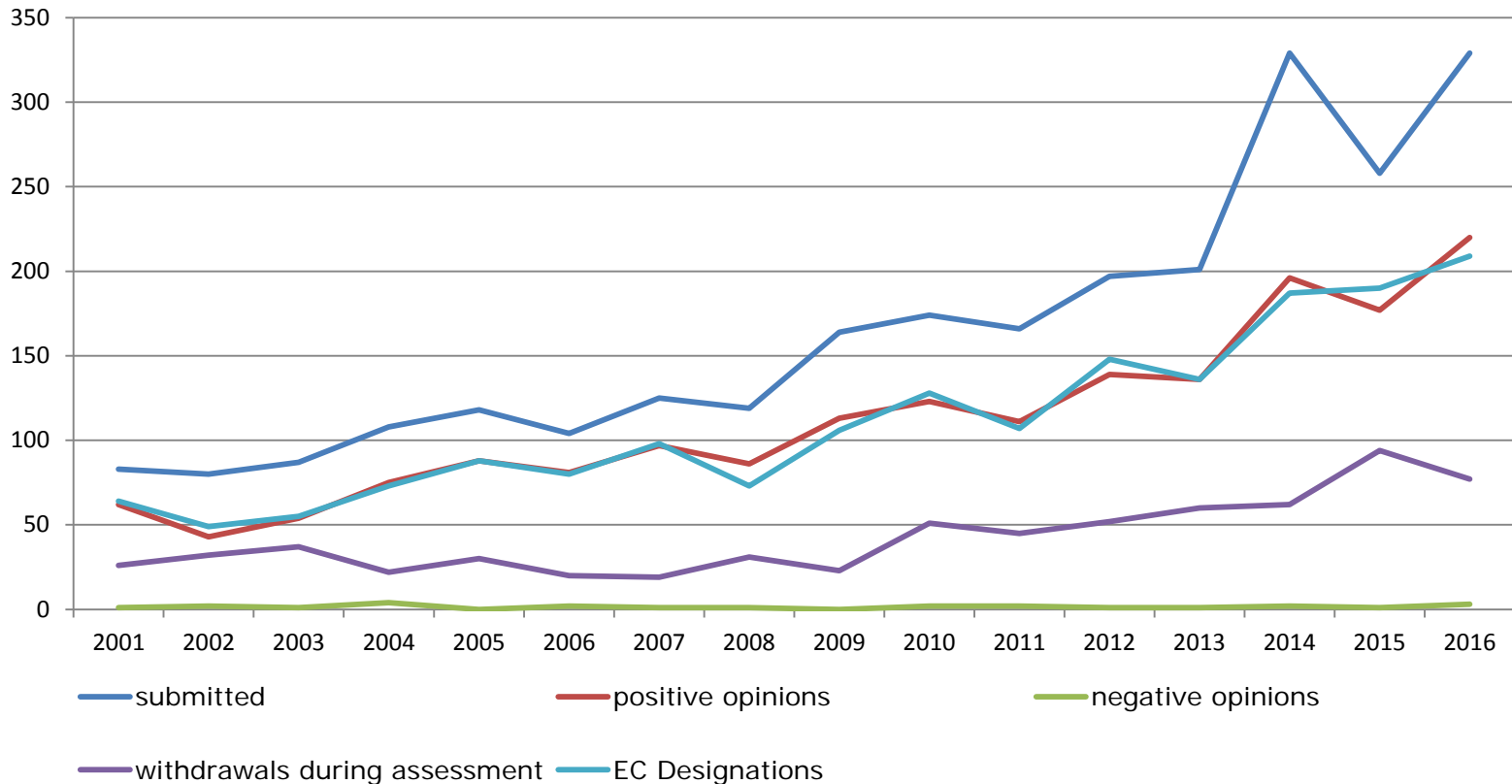
**Table 1.** Policy on orphan fee reductions 2016

Type of application	% reduction to the total applicable fee	
	Non-SME applicant	SME applicant
Protocol assistance (non-paediatric-related <sup>2</sup> )	75%	100%
Protocol assistance (paediatric-related <sup>3</sup> )	100%	100%
Inspection (pre-authorisation)	100%	100%
Application for a marketing authorisation	10%	100%
Post-authorisation activities, including annual fees, during the first year after marketing authorisation	0%	100%

# Committee for Orphan Medicinal Products

## Designated orphan medicines

- Status of orphan designations at end of 2016:
  - Individual conditions 487
  - Total designations 1805 (36% for oncology, 12% musculoskeletal/ NS)
  - Marketing authorisations 128



# Committee for Orphan Medicinal Products

- Patient registries workshop (28/10/2016)
  - Meeting report published February 2017 including recommendations and conclusions and next steps
  - [http://www.ema.europa.eu/docs/en\\_GB/document\\_library/Report/2017/02/WC500221618.pdf](http://www.ema.europa.eu/docs/en_GB/document_library/Report/2017/02/WC500221618.pdf)
- Defining orphan conditions – COMP Workshop (December 2016)
  - Aim of workshop to discuss challenges in delineating orphan conditions and to provoke reflections
  - The COMP condition: impact and interaction with other committee's mandates – CHMP, SAWP, PDCO
  - Evolving classification systems
  - Challenges of defining a condition – case presentations
  - The patient's view
  - COMP 2017 work plan - Development of strategies to implement recommendations stemming from the workshop on Conditions when assessing orphan designation applications

Thank You

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