Experience from the Committee for Orphan Medicinal Products and the Paediatric Development Committee regarding medical condition(s) targeted by medicinal products in the context of Orphan Designation and Paediatric Investigational Plan.

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Background:

The Orphan and Paediatric legislations require applicants to identify the condition(s) intended for a medicinal product. Differences of view with regard the intended condition(s) may occur between both committees. This may have implications in terms of regulatory strategy, in particular regarding the incentives foreseen by the orphan and paediatric legislations.

Aim:

The aim was to perform a comparative analysis of PIP conditions with OD conditions and to identify the extent of differences if any between the Paediatric Committee (PDCO) and the Committee for Orphan Medicinal Products (COMP) regarding determining the appropriate condition.

Methods:

For the analysis all PIPs were selected where the applicant indicated in the application that they have agreed or are planning to apply for an OD. This was done for a period covering 2007 to 2013. The analysis does not include medicinal products for which a waiver of paediatric development was agreed. And those where the OD was obtained after an agreed PIP. The search was performed in January 2014.

Results:

The search yielded 133 PIPs where a comparison was made between the OD condition(s) with the PIP condition(s). Overall in 41.4% the conditions were worded identically. In 16.5% there was a difference in the wording but without any true scientific difference. In 24.8% the wording was in the same therapeutic class but with some significant differences. In only 4.5% there was a significant difference. In 9% there were PIPs which had multiple ODs. It has also been noted that there has been an increase in the number of PIPs between 2008 and 2014.

Conclusions:

The survey has shown that in the majority of cases there is no divergence regarding the condition between the COMP and PDCO for a given medicinal product. This has important implications regarding the applicability of the Market Exclusivity rewards under the Orphan and Paediatric Regulations.

Figure 1 Number of Paediatric Investigational Plans for Orphan Designated Products

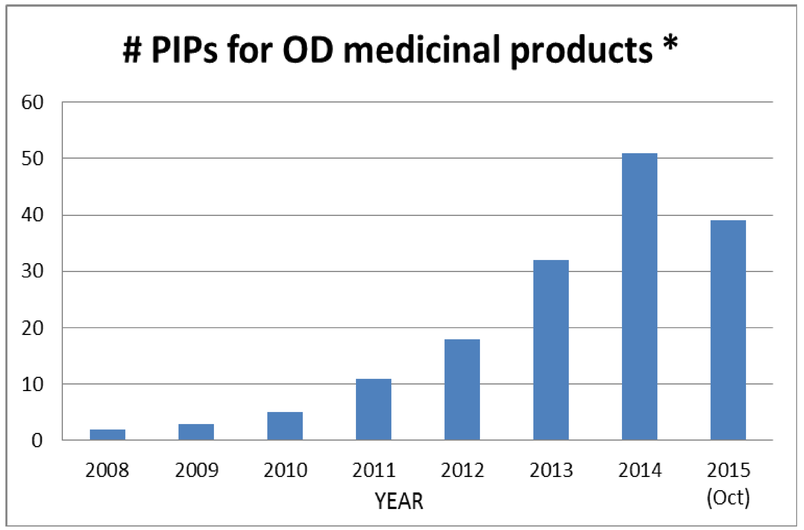

Figure 2: Orphan Designation vs. Paediatric Investigational Plan medical condition wording from 2007-2014