



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

COMP Feedback

PCWP/HCPWP joint meeting – November 14-15

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Update COMP Draft 2024 Working Plan



KEY OBJECTIVES



Optimisation of quality, consistency and transparency of designations



Explore Real World Evidence value in orphan designation process



Explore principles for piloting RWE analytics in support of COMP

Update COMP Draft 2024 Working Plan



Activities – Major Contribution to Patient Care



Defining requirements for major contribution to patient care at orphan designation & marketing authorisation stages



Webcast communication



Investigating patient preferences for MCPC
Collab. with the Patient Experience Data (PED) Project

Update COMP Draft 2024 Working Plan



Activities - Orphan Condition Definition



Modifier genes & defining conditions



Identifying applicable disease areas

Update COMP Draft 2024 Working Plan



Activities – Principal Decision DB & RWE



Continue collecting critical designation decisions



Continue RWE pilot & identification of applicable cases

Update COMP Draft 2024 Working Plan



Activities – Med. Pl. Adv/Innovative Therapies at initial OD



Publish the first results in a peer reviewed journal*



Continue research

CAR-T products & innovative therapies (i.e. siRNA, CRISPR cas, etc)

* Palomo, G. M. et al. The European landscape for gene therapies in orphan diseases: 6-year experience with the EMA Committee for Orphan Medicinal Products. *Mol Ther* S1525-0016(23)00540 (2023).

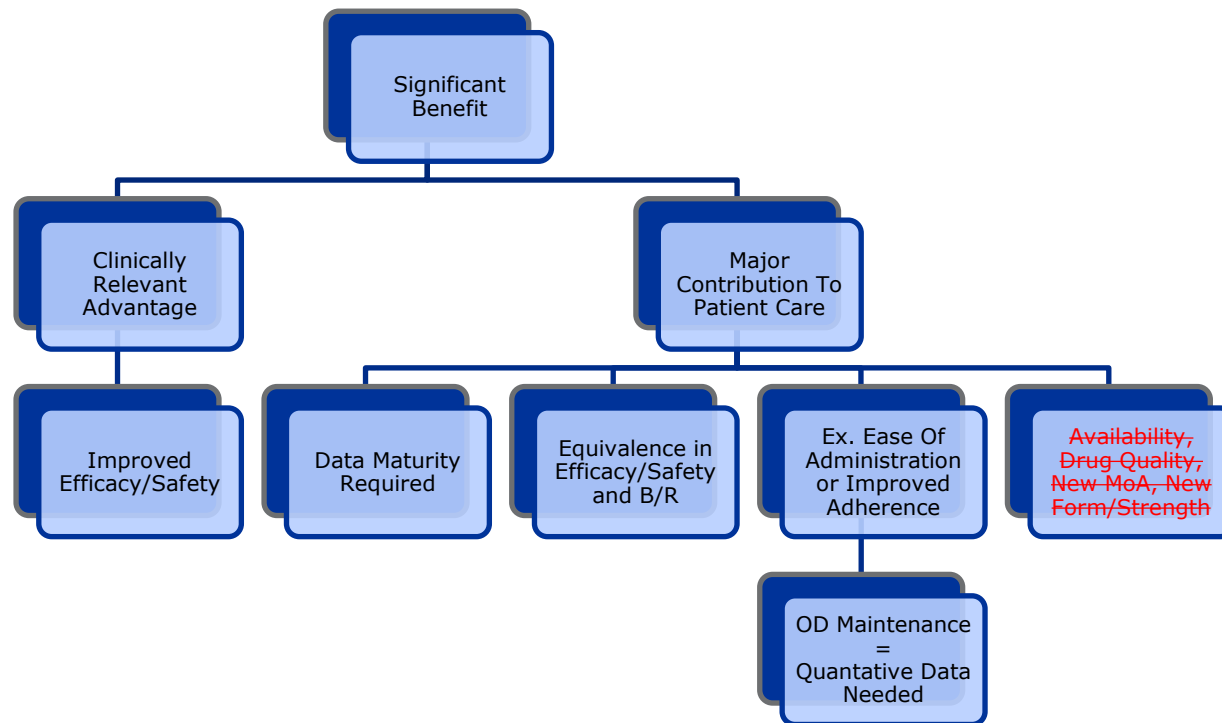


Project Major Contribution to Patient

(Very) Brief Introduction

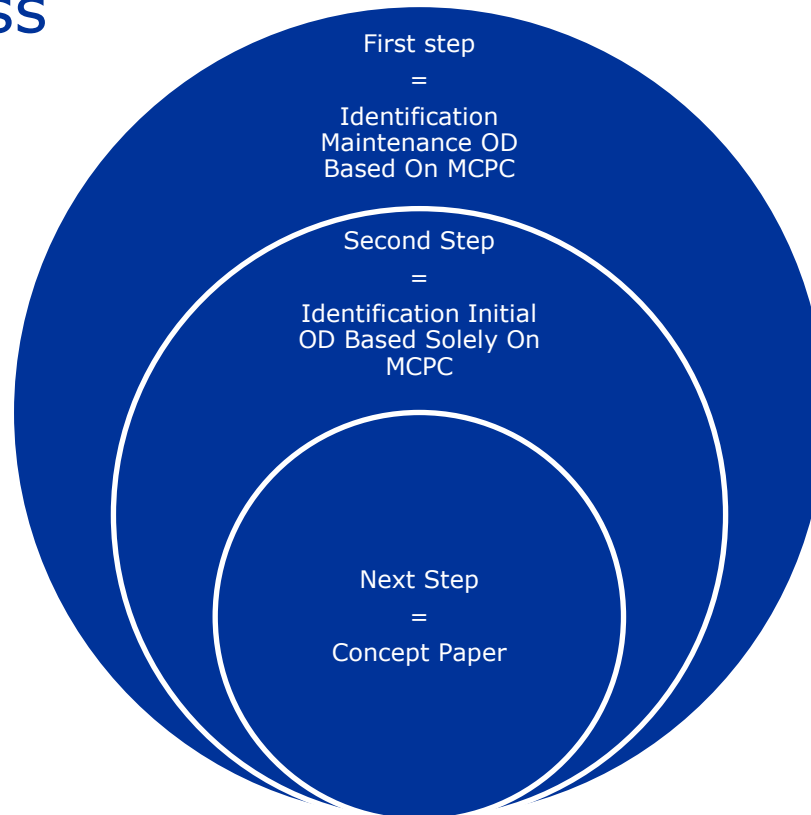


Notice 2016 *Section 5*: Significant benefit



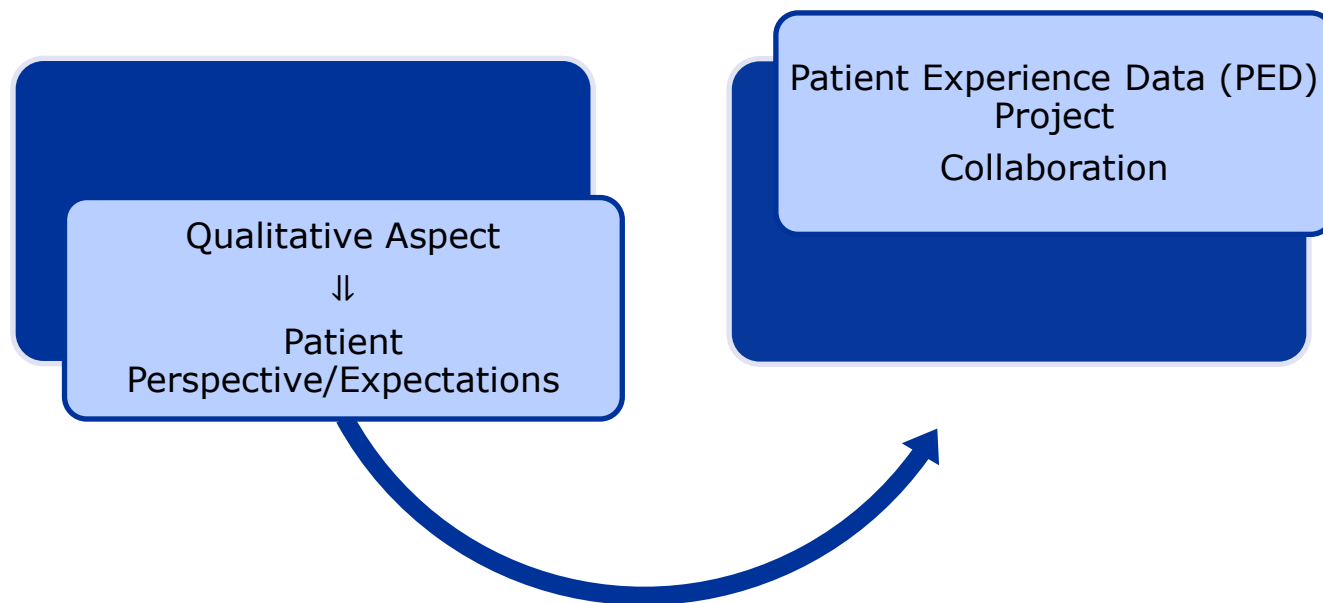


Project progress





Currently Amiss





Thank you for your attention