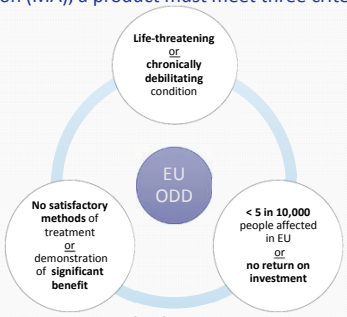
How patient's preference and interest are taken into account for the Orphan Drug Designation (ODD) and ODD maintenance in the EU?

Séverine Troubat, Regulatory Affairs Senior Specialist & Elsa Sirou Regulatory Affairs Specialist
Alexion Pharma GmbH, Giesshübelstrasse 30, 8045 Zurich, Switzerland

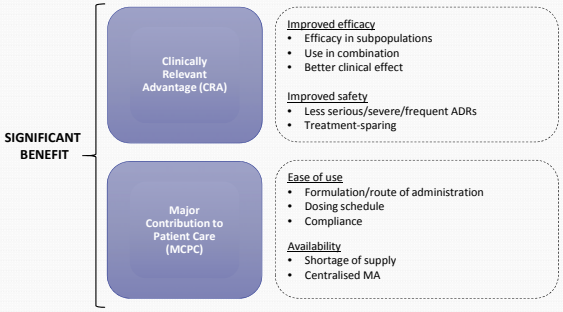
What is an ODD?

- European Commission (EC) Regulation No. 141/2000 and its provisions were created to promote the development of medicines for rare diseases
- To qualify for an ODD and maintain it at time of marketing authorization (MA), a product must meet three criteria



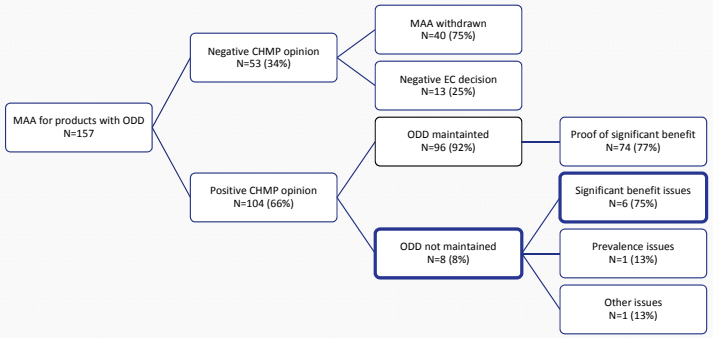
Significant Benefit (SB) and Major Contribution to Patient Care (MCPC)

- The term 'significant benefit' was first introduced in the regulatory environment with the EC Regulation No. 141/2000

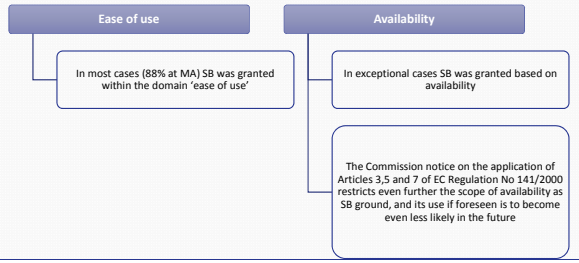


Real life experience in the EU between 2000-2013

- The success rate for orphan applications (66%) was significantly lower than for non-orphans (79%) at MAA

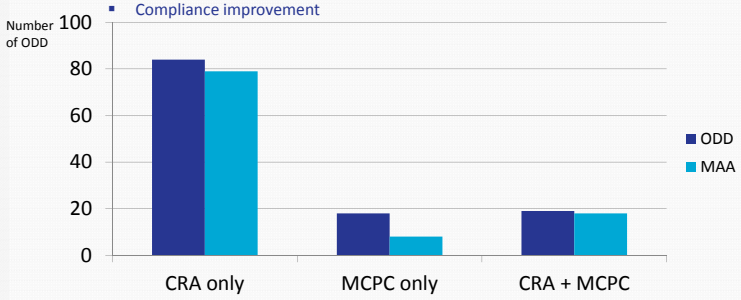


- Grounds of MCPC were granted on their own in only eight cases at MA, dropping from 18 cases that had acceptable assumptions at ODD
- > 50% of the medicinal products that withdrew ODDs at MA as a result of the COMP questioning SB had claimed grounds only in this area
  - Limited development of methodologies for measuring patient preferences and patient-reported outcomes
  - Their use is limited in clinical trials supporting MA



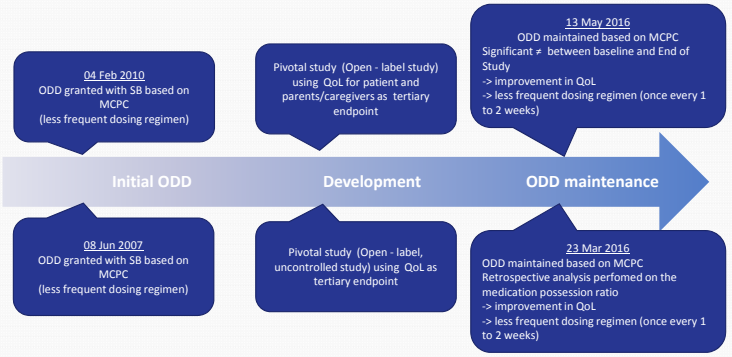
ODD maintenance in EU

- Maintaining orphan status over existing therapy can be challenging and relevant data should be included in the maintenance report
  - Data from pivotal study (side by side comparison),
  - Direct or indirect comparison,
  - Quality of Life (QoL) questionnaire, Patient Reported Outcome
  - Compliance improvement



Case Studies

Idelvion® (Treatment and prophylaxis of bleeding in patients with Haemophilia B)



Alprolix® (Treatment and prophylaxis of bleeding in patients with Haemophilia B)

Number of ODD maintained at time of MA based on patient preference are still low. Patient's preference is difficult to translate into quantifiable data.

References: European Commission (EC) Regulation No. 141/2000; EPAR Idelvion; EPAR, Alprolix; Hofer 2013, Marketing authorisation of orphan medicines in Europe from 2000 to 2013; Fregonese, 2018; Demonstrating significant benefit of orphan medicines: analysis of 15 years experience in Europe