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## Key considerations of orphan products designation and registration regulation

**Mona Mohammed**  
Medac GmbH, Germany

The orphan designation is a legal procedure that allows for the designation of a medicinal substance with therapeutic potential for a rare disease, before its first administration in humans or during its clinical development. The exact therapeutic indication is then defined at the time of marketing authorization. Developing a new drug or device can be expensive. Because rare diseases affect a relatively small number of people, drug companies generally demonstrate little interest in performing research or development of new products to treat such diseases. Developers face a further difficulty in testing potential treatments because it is difficult to recruit a sufficient number of people to study safety and effectiveness. The main objective is to examine an application for orphan medicinal product designation and to check the designation criteria are met, i.e.:

- The life-threatening or debilitating nature of the condition
- The medical plausibility of the proposed orphan indication
- No satisfactory method of diagnosis prevention or treatment exists, or if such a method exists, that the medicinal product will be of significant benefit to those affected by the condition

The manufacturer needs to inform health authority about the submission of orphan medical product designation 2 months prior to the actual submission date. The submission should be done via secure system and authority officers will validate the application. Once the validation will be completed successfully, the evaluation procedure will start and the sponsor will be informed for the same. The final opinion will be taken after evaluation through many committees. After receipt of the final approval for designation, the application for marketing authorization will be submitted. A successful regulatory strategy for any drug product require a strong understanding of the regulations, guidelines and effective communications with the health authorities of different countries & take advantage of the opportunities to expand their products in this dynamic part of the world.

### Biography

Mona Mohammed is Regulatory Affairs Senior officer at Medac GmbH, Germany which specialized in therapeutic agents for the treatment of oncological, urological and autoimmune diseases and their related symptoms covering Middle East, Turkey and some Asian countries, her pervious experiences as Quality and regulatory expert in one of the biggest pharmaceutical local industry in Middle East and she has experience in managing registration requirements in 12 different countries in Middle East, She has directed Post-Market Surveillance/ Vigilance served as compliance officer, throughout her career.

[mona.elgandour@gmail.com](mailto:mona.elgandour@gmail.com)