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DEMYSTIFYING THE MAZE

Article 2

Centralized vs. National Procedures for Rare Disease Drug Registration in the EU

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Introduction

The European market presents a compelling opportunity for US pharma and biotech companies developing life-changing therapies for rare diseases. Engaged patient communities and sophisticated healthcare systems create a fertile ground for innovation. However, navigating the intricacies of the European Medicines Agency's (EMA) regulatory landscape can be a complex undertaking. A critical first step involves selecting the most appropriate registration pathway: the centralized procedure or national procedures.

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Understanding the Options

The EMA offers two primary routes for drug registration

Centralized Procedure

This streamlined approach grants a Single Marketing Authorization (MA), providing access to the entire European Economic Area (EEA) with a single application. This translates to faster market entry and consistent evaluation criteria across member states.

The centralized procedure is mandatory for certain categories of drugs, including those derived from biotechnology and those targeting unmet medical needs in areas like HIV/AIDS and rare diseases. Additionally, orphan medicinal products designated for rare diseases must be registered centrally to qualify for extended market exclusivity (10 years).

National Procedures

This route allows for individual application in one. EU member state. This approach can be advantageous for drugs that don't qualify for the centralized procedure or when the initial commercial focus is on one specific European market.

Companies with established European operations may also find national procedures appealing due to existing relationships with local regulatory agencies. However, national procedures come with their own challenges. Varying timelines, application requirements, and potential for inconsistent decisions across member states can significantly increase complexity and resource demands.

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Additional Options for Streamlined Registration

Mutual Recognition Procedure (MRP)

Authorization granted by one reputable EU member state to be recognized by other designated member states with simplified procedures. It's ideal for established medicines with existing authorization in the EU and a target market in specific countries.

Decentralized Procedure (DCP)

This enables simultaneous applications for marketing authorization in several, but not all, EU member states. It offers a faster path to market compared to the centralized process but requires agreement and collaboration between participating member states. It's suitable for medicines seeking authorization in a select group of EU countries.

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Choosing the Right Path

Selecting the optimal registration strategy requires careful consideration of several factors, including the drug's characteristics, commercial goals, and eligibility for the centralized procedure. Partner Rare's team of experienced regulatory consultants can guide you through this process with:

In-depth eligibility assessment to determine the most suitable pathway.

We leverage our extensive experience navigating both centralized and national procedures to ensure your application is placed on the most efficient track.

Market access and pricing strategies to optimize your success in the European market.

Our team's deep understanding of the European healthcare landscape allows us to develop tailored strategies to maximize patient access to your therapy.

Strategic dossier preparation ensuring a comprehensive and compliant application.

Our Rare Disease specialists have a proven track record of crafting successful dossiers that meet the EMA's rigorous standards.

Expert liaison with the EMA or national agencies throughout the process, facilitating smooth communication and efficient resolution of any queries.

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Partnering for Your Success

At Partner Rare, we understand that navigating the European regulatory landscape can be a complex undertaking. That's why we go beyond simply providing consultancy services. We become your trusted partner, offering comprehensive support and guidance throughout the registration process. Our team of experienced professionals will work collaboratively with you to ensure a smooth and efficient journey to market.

Taking Your Therapy to European Patients

Our primary goal is to help you bring your life-changing rare disease therapy to the patients in Europe who need it most. We achieve this by leveraging our deep understanding of the EMA's regulatory framework and by providing strategic guidance tailored to your specific needs. Our team's proven experience in rare disease drug registration ensures a comprehensive understanding of the unique challenges and opportunities associated with bringing these essential therapies to market.

Contact Partner Rare Today

We invite you to contact Partner Rare today to discuss your unique requirements and explore how we can help you navigate the European market.

About the Author

Agnes Kohl is a passionate advocate for patients with rare diseases. As a C-level executive with 20+ years in the field, she has spearheaded the development and commercialization of numerous therapies worldwide. With over 300 regulatory approvals to her name, Agnes possesses unmatched expertise in navigating the complexities of the European rare disorders market. A seasoned expert in the in/out licensing of rare disease assets, with a particular interest in ALS; Agnes is passionate about expanding access to treatments for rare diseases and leverages her deep industry knowledge and strategic insight to drive growth and innovation in the European market.


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