

CRACKING THE **EMA** CODE

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**Article**

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## **The Global Landscape of Rare Disease Treatments**

### **Legal and Regulatory Considerations for Pharma and Biotech Companies**

Rare diseases, affecting a small percentage of the population, often face a lack of research funding and treatment options. However, the global pharmaceutical and biotechnology industry is increasingly focusing on developing therapies for these conditions. To incentivize this crucial research, governments around the world have implemented various legal and regulatory frameworks. This article explores the legal landscape for orphan drugs in key markets, highlighting the opportunities and challenges for companies developing treatments for rare diseases.

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## The United States

Market access involves the intricate process of bringing drugs from development to the hands of patients who need them. It includes regulatory approval by government agencies such as the FDA in the United States or the EMA in Europe, as well as navigating reimbursement policies set by payers, such as insurance companies or national health services. Additionally, market access considers factors such as pricing strategies, distribution channels, and the integration of drugs into clinical practice within healthcare systems.



## The European Union

The European Union (EU) also has a well-established framework for orphan medicinal products. The Orphan Medicinal Products Regulation (Regulation (EC) No 1495/2007) offers similar incentives to those in the US, including protocol assistance, protocol assessment fees waiver, and ten years of market exclusivity. This regulation has fostered a collaborative environment for orphan drug development across the EU member states.

## The Allure of Europe for Rare Disease Treatments

Europe presents a compelling opportunity for companies developing treatments for rare diseases. Here's why:

### Centralized Approval Process

The European Medicines Agency (EMA) offers a centralized marketing authorization process for drugs, potentially streamlining approval across the entire EU. This eliminates the need for separate national approvals in each member state.

### Orphan Drug Designation

The EMA's Orphan Drug Designation program incentivizes the development of treatments for rare diseases. Benefits include protocol assistance, fee reductions, and extended market exclusivity, providing valuable support for companies navigating the development process.

### Large Patient Pool

While individually rare, the combined patient population across Europe for any given rare disease can be significant. This translates to a larger potential market for your life-saving treatment.

### Robust Healthcare Systems

Most European countries boast well-funded healthcare systems with established mechanisms for reimbursing the cost of effective treatments. This can lead to faster market access and wider patient coverage for your drug.

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## Germany

# A Strategic Launchpad for Europe

Shifting gears to Europe's powerhouse, Germany boasts a robust healthcare system and a thriving life sciences sector. This makes it a key player in the fight against rare diseases. Let's delve into the legal landscape for orphan drugs and research incentives in Germany, exploring how they attract pharma and biotech companies developing treatments for rare patient populations.

## Centralized Location



Situated in the heart of Europe, Germany provides easy access to other major European markets. This simplifies logistics and distribution, potentially streamlining future pan-European marketing efforts.

## Strong Pharmaceutical Market



Germany boasts a large and well-funded healthcare system, translating to a reliable patient pool. Additionally, Germany's healthcare system is known for its efficiency in evaluating and approving new drugs with a high unmet medical need, such as those targeting rare diseases. This can potentially lead to faster reimbursement approvals for your treatment.

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## Reputation for Quality



Germany's stringent pharmaceutical regulations are recognized worldwide. Having a German legal entity can enhance trust and credibility for your drug throughout Europe, potentially expediting market acceptance from both regulators and healthcare providers.

## Government Funding Opportunities



The German government offers various grants and funding programs specifically for research and development of treatments for rare diseases. Establishing a legal entity in Germany can make your company eligible for such programs, providing crucial financial support during the development and clinical trial phases.

## Skilled Workforce



Germany is a hub for scientific and medical talent. The country boasts a robust pool of scientists, researchers, and clinical trial professionals who can contribute significantly to the efficient development and clinical testing of your rare disease drug.

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## Setting up in Germany Considerations for Success

While Germany offers a wealth of advantages, establishing a legal entity there requires careful planning and attention to detail. Here are some key considerations:

### Choosing the Right Legal Entity



Germany offers various legal structures for companies, such as the GmbH (limited liability company) or the AG (stock corporation). The most suitable structure depends on factors like your company's ownership structure, liability preferences, and fundraising goals. Consulting with a legal professional can help you navigate these complexities and choose the optimal structure for your specific needs.

### Taxation



Germany's corporate tax rate is moderate compared to some European countries. Additionally, research and development costs associated with your rare disease treatment may be tax-deductible, offering some financial advantages. A thorough understanding of the German tax system and its implications for the business model is therefore of great importance.



## Bank Account



The banking system and its supervision are also increasingly subject to international regulations. It is therefore advisable to exercise caution when following the tempting advice of “consultants” to open an account in a tax haven which, in case of doubt, prohibits any bank from opening a (much needed) local account for the share capital. Consequently, it would therefore be impossible to open a local branch without a bank account.

## Regulatory Compliance



Partnering with a local legal expert can ensure smooth compliance with German regulations regarding licensing, manufacturing, and marketing of pharmaceuticals.

## Language and Cultural Considerations



While English is increasingly used in business, fluency in German can be highly beneficial. Consider offering German language courses or translation services for your employees.

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## Partnering for Success in Europe

The ever-changing legal and regulatory landscape for rare disease treatments demands expert navigation.

Partner Rare, with deep expertise in international regulations and proven success in complex markets like the EU and Germany, provides comprehensive legal support services to streamline your path to success.

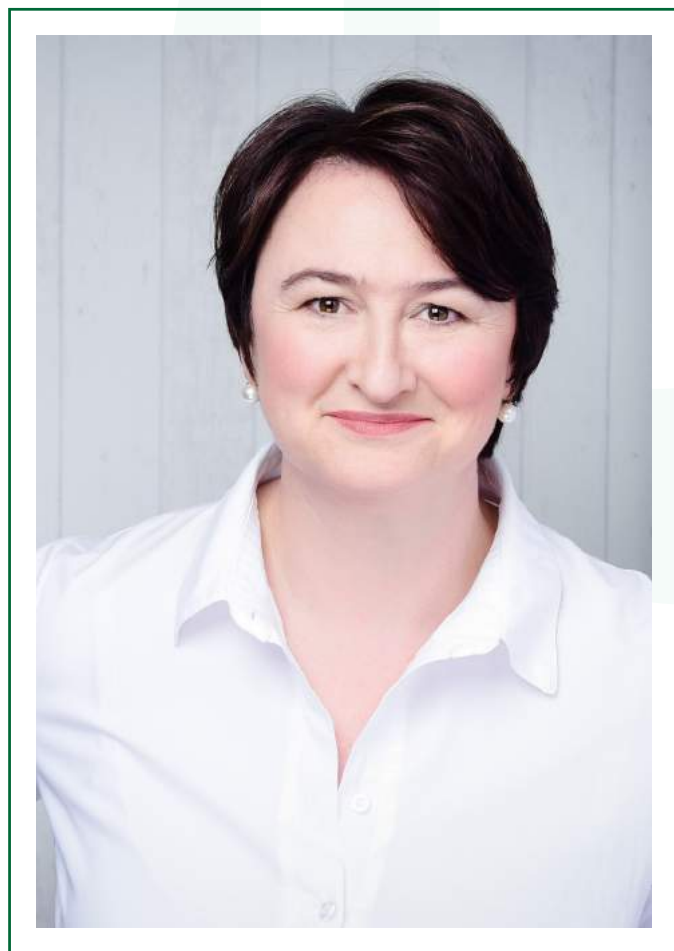
We guide companies through the complexities of orphan drug regulations, secure market access, ensure end-to-end compliance, and expertly establish subsidiaries and secure drug licenses.

To learn more about how Partner Rare can help your company navigate the European market and accelerate the development of life-changing treatments for rare diseases, contact us today!

## About the Author

Sita Schubert is a seasoned legal and business executive with extensive experience in the healthcare and pharmaceutical industries. She has served on the board of the pharmaceutical industry and the Pharma Code Association and is an active investor in promising start-ups. Sita is a recognized expert in medicinal cannabis, regulatory affairs, orphan drugs, in/out licensing deals, and mergers and acquisitions, offering comprehensive consultancy and operational advice to companies in the life science sector.

As a founding member and Secretary General of the European Medical Cannabis Association, she is a passionate advocate for patient-focused, evidence-based solutions. Sita also specializes in helping companies establish affiliate companies in Germany, leveraging her deep understanding of German law and business practices.



Don't miss the opportunity to gain a competitive edge with expert guidance. Contact us today to schedule a complimentary discussion with Sita Schubert and explore how her proven track record can elevate your business strategies.

