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CRACKING THE EMA CODE

RARE DISEASE CLINICAL TRIALS

Article 3

A Collaborative Approach to Design and Execution

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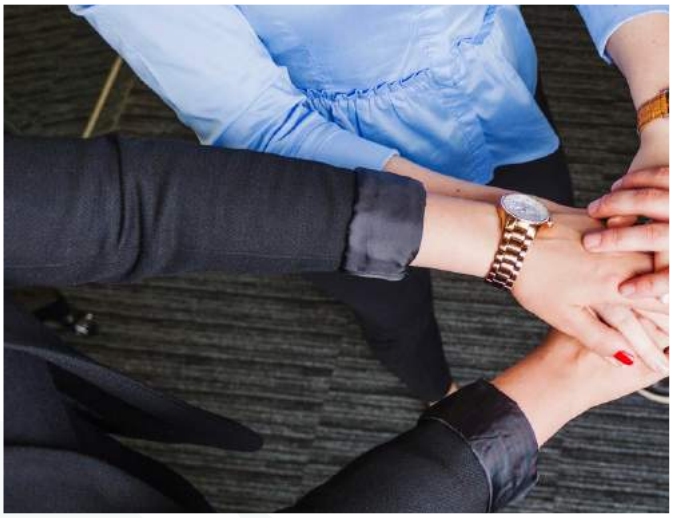
Patients who suffer from rare diseases need access to effective and safe medications. Given the low incidence of the diseases, the collaboration between regulatory authorities, producers, insurances, and market access teams is essential for pharmaceutical companies seeking to bring new medications to patients and the national health care systems.

Early Collaboration is Essential



Market access considerations should be integrated into regulatory strategy development from the outset. Understanding the access landscape and potential reimbursement challenges can inform decisions about clinical trial design, endpoints, and regulatory pathways.

To achieve these insights, patients and public involvement (PPI) specialists together with regulatory and market access teams should collaborate to ensure that the data generated during clinical development meet both regulatory and payer requirements.

This may involve conducting studies that generate evidence relevant to both stakeholders, such as real-world evidence (RWE) studies or health economic analyses.

Partner Rare provides comprehensive expertise in clinical trial design and execution. Our collaborative approach brings together seasoned regulatory and market access specialists to align strategies and expedite access to both US and European healthcare systems, ensuring your rare disease therapy reaches patients efficiently.

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Selecting Meaningful Endpoints

Crucial factors to consider may include clinical effectiveness, safety, cost-effectiveness, and impact on patient outcomes and quality of life.

The choice of the Primary Endpoints must be meaningful to all patients, regulatory authorities, and payers. These endpoints should reflect clinically relevant outcomes that demonstrate the medication's efficacy and align with payer expectations for coverage and reimbursement. While meeting payer requirements is essential, ensure that the selected endpoints also align with regulatory requirements for drug approval.

This ensures that the trial results can support both regulatory submission and market access efforts.

Secondary endpoints need to provide additional insights into the medication's clinical benefit, safety profile, quality of life, and economic value. These endpoints can help support broader claims about the medication's effectiveness and differentiate it from potential existing treatments. Each payor may mandate different health economic endpoints which should be considered in your trial design. This may involve measuring outcomes such as quality-adjusted life years (QALYs), healthcare resource utilization, and direct and indirect costs. The input of patients and public involvement (PPI) specialists can be key in tackling the appropriate items.

Real-World Evidence (RWE) may be already incorporated in endpoints to supplement the findings from clinical trials. RWE studies can provide insights into how the medication performs in routine clinical practice and its impact on patient outcomes, which may be valuable to payers.

Patient-Reported Outcomes (PROs) should be included to capture the impact of the medication on patients' symptoms, functional status, and quality of life whenever possible. PRO data can provide important insights into the patient experience and may be influential in payer decision-making. The selected endpoints must be statistically robust and adequately powered to detect meaningful differences between treatment groups.

Consideration should also be given to handling missing data and controlling potential confounding factors. Partner Rare offers consultation with experts to seek input from experts in clinical trial design, health economics, and payer engagement to ensure that your endpoint selection strategy is comprehensive and well-informed. By carefully planning primary and secondary endpoints that address payer requirements, pharmaceutical companies can generate the evidence needed to support reimbursement and access to their medications, ultimately benefiting patients and healthcare systems.

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EMA vs. FDA Considerations for Rare Disease Trials

When planning clinical trials for drug development, there are several key differences to consider between the European Medicines Agency (EMA) and the U.S. Food and Drug Administration (FDA). These variances encompass regulatory requirements, submission processes, and cultural nuances. EMA generally emphasizes the demonstration of clinical efficacy, safety, and quality of the product. Clinical trials often incorporate endpoints relevant to European healthcare systems and patient populations.

FDA emphasizes substantial evidence of effectiveness and safety for regulatory approval. Trials must align with FDA guidance documents and may require endpoints that are particularly meaningful to U.S. healthcare providers and patients. The clinical trial phases for EMA follow a phased approach like the FDA including Phase I, II, and III trials, but with variations in requirements and emphasis on endpoints. FDA requires Phase I, II, and III trials for drug approval, with specific emphasis on safety and efficacy endpoints tailored to the intended indication.

In the context of the European Medicines Agency (EMA), "Payor data requirements" typically refer to the information needed by health authorities or payors (such as insurance companies or government healthcare agencies) to make decisions about the reimbursement or coverage of a particular medication.
These requirements can vary depending on the country or region, but they generally include data on the clinical effectiveness, safety, and cost-effectiveness of the medication.
Manufacturers seeking reimbursement or coverage for their products must often submit comprehensive dossiers containing this information to regulatory agencies like the EMA and payors in individual European countries.
Therefore, the knowledge of payors' requirements in the clinical trial design can save time and money and accelerate the patient's access to treatment.

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Partner Rare: Accelerating Orphan Drug Development Through Expertise

Building Strong Partnerships

Partner Rare offers well-established connections with key organizations which are crucial for successful enrolment in clinical trials. Patient advocacy groups, healthcare institutions, and specialist centers often have direct access to patient populations affected by specific medical conditions. Collaborating with these organizations facilitates access to potential trial participants, especially for rare diseases or niche populations. Trust and credibility within the patient community will promote participating in a clinical trial if they feel confident in the legitimacy and reputation of the sponsoring organization and collaborating institutions. Referral networks will streamline the recruitment process and increases the pool of eligible candidates for enrolment. Partnering with key organizations allows sponsors to raise awareness about the clinical trial among patients, caregivers, and healthcare providers.

These organizations can disseminate information about the trial, educate stakeholders about its purpose and benefits, and address any concerns or misconceptions.

Engaging with key organizations demonstrates a commitment to the community and fosters collaboration between researchers, healthcare providers, patients, and advocacy groups. This collaborative approach promotes transparency, inclusivity, and patient-centeredness throughout the clinical trial process. Input from key organizations during the protocol design phase can help ensure that the trial is feasible, acceptable, and aligned with the needs and preferences of the target patient population. Collaborative efforts can also facilitate the implementation of the protocol by addressing logistical challenges and operational considerations.

Collaboration is Key

Utilizing Existing Healthcare Infrastructure for Efficient Rare Disease Trials

Key organizations often provide support services and resources to trial participants, such as counseling, transportation assistance, and financial aid. Partnering with these organizations allows sponsors to leverage existing support networks and enhance the overall participant experience. Collaborating with key organizations can amplify advocacy efforts and influence policy decisions related to clinical research and healthcare access. By working together, stakeholders can advocate for policies that promote patient-centered research, improve access to clinical trials, and advance medical innovation.

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Optimizing Your Approach

Key Considerations for Successful Rare Disease Trials

In summary, bringing new treatments to patients in the EU requires a thorough approach, with the goal of successful development and market access. This "reversed engineering" necessitates collaboration between clinical trial specialists, regulatory experts, marketing teams, and patient public involvement (PPI) groups. By working together, these stakeholders can design and collect the data needed to demonstrate a medication's value for patients and healthcare systems.

Partner Rare offers extensive experience in navigating this complex landscape. We can assist you in planning a coordinated approach to both US and European markets, ensuring your rare disease treatment reaches the patients who need it most.

If your organization is embarking on the journey of bringing a life-changing therapy to the European market, let Partner Rare be your guide. Our expertise and experience can help you navigate the challenges, optimize your strategy, and ultimately, deliver hope to patients in need.

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About the Author

Professor Boris Kramer is a globally recognized expert in neonatology and translational medicine with over 25 years of experience. He has made significant contributions to the development of innovative therapies for children and adults, particularly in the field of cell therapies. His expertise spans preclinical research, clinical trial design, and regulatory affairs, leading to two European Orphan Designations for rare diseases and extensive experience in leading and sponsoring clinical trials.

As a Professor of Experimental Perinatology/Paediatrics, a prolific researcher (with over 302 peer-reviewed publications), and a dedicated mentor, Professor Kramer is passionate about advancing the field of medicine and improving patient outcomes.

To discuss your specific requirements or explore potential collaborations with Professor Kramer, contact Partner Rare for a complimentary consultation to discuss your specific needs.


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