

# Critical Steps for Guiding Your Orphan Drug to FDA Approval

## The Orphan Drug Act

was passed in 1983 to stimulate the development of treatments for rare diseases by providing incentives for drug developers, including seven-year marketing exclusivity and tax credits for clinical trial costs. While the program has been effective in driving increased clinical development in the rare disease space, many orphan drugs still struggle to achieve the ultimate goal: receiving regulatory approval and becoming commercially available to patients.

The challenges facing a rare disease drug on the road to regulatory approval are varied and many. Because rare diseases often affect pediatric patients and the populations are small, collecting the necessary clinical data can be difficult. Failure to define clinically meaningful endpoints in the early stages of development can result in costly delays for both drug developers and patients who are awaiting treatment. For sponsors developing orphan drugs, there are critical steps to facilitate a faster and more seamless regulatory process.

### 1 Start with a clear and comprehensive development plan

Beginning with the end in mind is key. A well-thought out development plan can help to guide all of your downstream clinical and regulatory decisions. It should clearly define your disease state, your target population, how the product will be used and your expected outcomes. This plan will serve as a foundation for your orphan drug designation application and Investigational New Drug (IND) application.

### 2 Conduct IND-enabling studies in a timely manner

Because rare disease drugs are required to meet the same safety standards as common disease drugs, but are limited by small patient populations, comprehensive clinical research planning is essential. Typically, your IND should contain a minimum of 15 animal safety, pharmacology and toxicology studies,

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which aligns with FDA standards. Inclusion of these studies will greatly reduce the probability of the IND being placed on clinical hold.

### 3 Communicate with the FDA early and often

Frequent communication with the FDA is a critical factor to success. Be sure to prepare thoroughly for all meetings with the agency to ensure you are taking full advantage. Identify your experts early and make certain they are intimately familiar with guidance documents and ready to answer questions. Setting the right tone and expectations with the FDA early can help prevent requests for rework later in the process.

### 4 Evaluate eligibility for an FDA expedited pathway

The FDA has four expedited programs to speed the development and review of new drug products that address unmet medical needs in the treatment of serious conditions — many of which are orphan drugs. These programs are designed to reduce the time necessary for drug development without compromising the standards for safety and effectiveness. Understanding if your product would be eligible for



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an expedited program and how to apply may significantly accelerate your time to market.

### 5 Be selective when choosing partners

Companies developing therapies for rare diseases often have limited resources, so it is important to get the development strategy right the first time and prevent any need for changes in course or redundant steps. Partnering with regulatory and clinical experts who have knowledge and experience guiding orphan products to approval can help save you significant time and resources. Be sure to ask about specific rare disease expertise when evaluating potential suppliers.

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