

RARE DISEASE

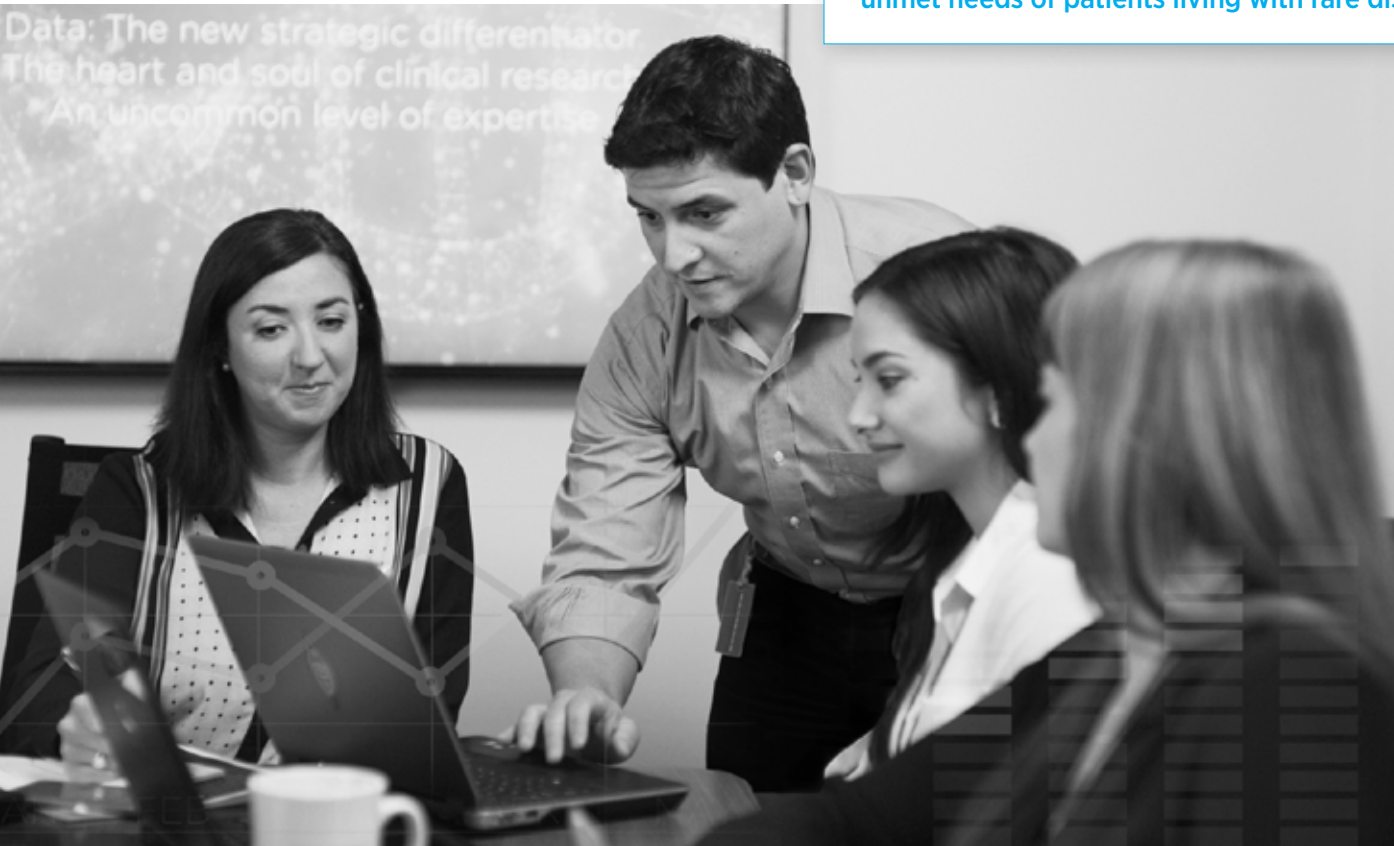
Mapping the New Landscape of Orphan Drug Development

ABSTRACT

The marketplace for orphan drugs is growing, and changes in the regulatory landscape are providing favorable conditions for collaboration in the area of drug development in rare diseases. Understanding the regulatory and operational nuances of orphan drug development can help sponsors position their promising compounds for clinical and commercial success.



The orphan drug arena focuses on identifying and studying potential treatments to serve the significant unmet needs of patients living with rare diseases.



Introduction

Today more than ever, researchers are focused on providing care for the approximately 7,000 rare diseases that have been overlooked in the past due to the challenges of conducting clinical trials in small populations with limited commercialization potential. The orphan drug arena focuses on identifying and studying potential treatments to serve the significant unmet needs of patients living with rare diseases, and the current landscape offers a variety of incentives, government support, and regulatory agency assistance to encourage orphan drug development.

In this white paper, we will discuss key strategic considerations for both the U.S. and EU that may be employed to accelerate your orphan drug and rare disease development program. We will focus on the regulatory aspects of bringing therapies for rare diseases to market, with specific emphasis on the similarities and differences between the regulatory pathways in the U.S. and EU. We will also explore the operational nuances of orphan drug development that can help sponsors position their promising compounds for clinical and commercial success.

The market opportunity in rare diseases

Approximately 7,000 distinct rare diseases have been identified to date, affecting an estimated 350 million people worldwide. Since the Orphan Drug Act was passed in the U.S. in 1983, there have been over 2,900 orphan drug designations, and more than 40 orphan drugs have exceeded \$1 billion in global sales. With an estimated 250 new rare diseases identified each year, the worldwide orphan drug market is projected to grow to \$127 billion in 2018 and is expected to grow at an annual rate of 11 percent per year through 2024, compared with six percent for non-orphan drugs.¹

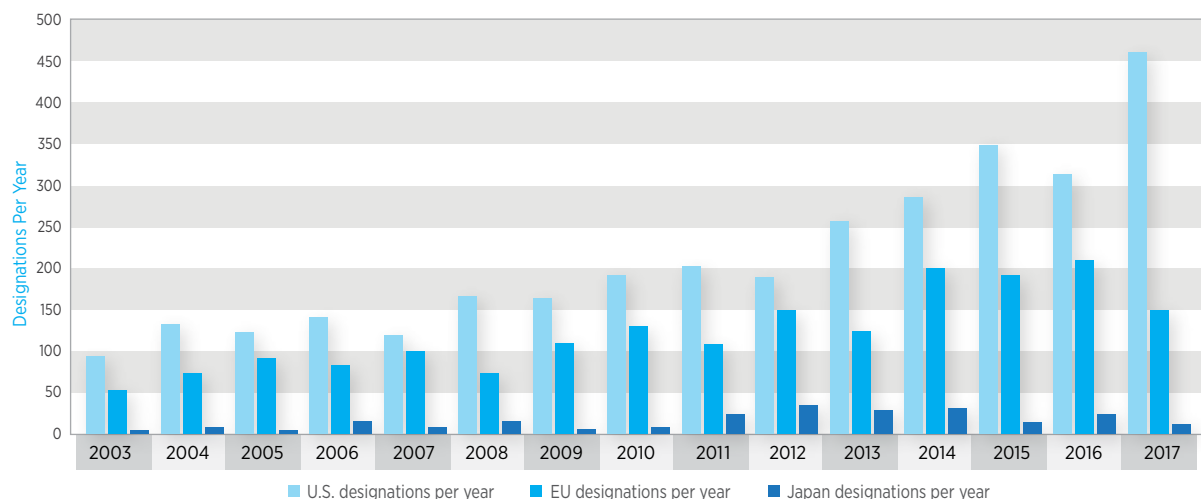
U.S. orphan drug and rare disease landscape

In the U.S., a rare disease is defined as a condition that affects fewer than 200,000 people, and an orphan drug is either:²

- A drug or biological product used for the prevention, diagnosis, or treatment of a rare disease in the U.S., or
- A drug or biological product that is not expected to recover the costs of development and marketing

Since 2003, there has been a dramatic increase in the number of orphan drug designations, particularly in the U.S., as sponsors better understand the incentives associated with orphan drug development and the strategies that can be leveraged to streamline development efforts. In 2017, the U.S. Food and Drug Administration (FDA) granted orphan designation requests at a record rate of more than two per work day.³ There has also been more financial commitment and a deliberate effort by both established firms and up-and-coming companies to invest more time and resources to developing compounds and products in the orphan drug arena.

Figure 1. Trends in orphan drug designation¹





Despite the consistent growth in orphan drug development over the past 15 years, there is still significant work to be done. We have only begun to scratch the surface of the approximately 7,000 rare diseases, many of which are genetic, and the majority of patients with rare diseases remain underserved.

As compared with standard drugs, orphan drugs typically involve smaller Phase III trial sizes, spend fewer months in regulatory review, and are more likely to be approved by the FDA since these therapies often represent significant therapeutic advances.⁴ The expedited development pathways and higher likelihood of FDA approval highlight the impact sponsors can have in the area of orphan drugs and rare diseases, keeping in mind that orphan drugs are subject to the same regulatory requirements and marketing approval process as standard drugs. Productive partnerships and proper, proactive regulatory planning can potentially reduce development time and budget significantly while increasing development success.

Strategic considerations for U.S. development

Despite broad awareness of the existence of incentives for orphan drug development, many sponsors still remain uncertain about how to take advantage of these incentives. A top-line consideration when undertaking orphan drug development is to partner early and often with regulators to bring therapies for rare diseases forward.

The pathway to orphan drug development begins with a request to the Office of Orphan Products Development (OOPD) to grant orphan designation to a drug or biological product, and it ends

with submission of a new drug application (NDA) or a biologics license application (BLA), to the Center for Drug Evaluation and Research (CDER) or the Center for Biologics Evaluation and Research (CBER) to market an orphan drug or biological product.

The process typically involves frequent interactions with the FDA to discuss and agree upon the clinical development program requirements needed to support product registration. Of note, meetings with the OOPD are also available prior to submitting an application for orphan designation and the process of orphan drug development.

The FDA offers a variety of incentives, both during development and following approval, to support sponsors of orphan drugs. These incentives include:

FDA incentives for orphan drugs

- Grant program
- Tax credits
- Market exclusivity
- Rare pediatric disease priority review vouchers

Related strategic options for expedited development programs

- GAIN Act
- Fast track designation
- Breakthrough therapy designation
- Accelerated approval
- Priority review

FDA incentives for orphan drugs

Grant program

Approximately \$14 million is provided per year to sponsor companies through the orphan drug grant program, which is applicable to drugs, biologics, medical devices, and even medical foods. While this amount may seem small for traditional drugs, it has a significant impact in the orphan drug arena where trials are typically smaller and shorter in duration. The typical grant provides \$200,000-\$400,000 per year for three to four years followed by a re-compete process, which is more likely to be successful when sponsors are able to provide data showing product viability from a therapeutic and safety perspective. Any domestic or foreign, public or private, for-profit or nonprofit entities may apply for the grant program.

Tax credits

Tax credits can cover up to 50 percent of the clinical trial costs and are available for qualifying costs incurred between the date orphan drug designation is granted and the date of FDA approval. In 2014, more than \$2 million in user fees was waived for sponsors investigating potential rare disease therapies.

Market exclusivity

Orphan drug exclusivity offers seven years of exclusivity following FDA market approval and significant lead time against competitive threats to gain market traction.

Rare pediatric disease vouchers

The Rare Pediatric Disease Priority Review Voucher Program was created under the FDA Safety and Innovations Act (FDASIA) to encourage development of drugs and biologics for rare pediatric diseases. Products that undergo priority review are generally given an approval decision – positive or negative – within six months after the filing date, rather than the usual 10 months. The premise of the program is that sponsors who receive approval of a product application for a rare pediatric disease are eligible to receive a priority review voucher (PRV), which can be redeemed, transferred, or sold to another sponsor to obtain priority review of a subsequent application for a different product. The FDA has published the Rare Pediatric Disease Priority Review Vouchers Draft Guidance for Industry to assist sponsors in navigating this program, which requires a sponsor to notify the FDA of its intention to redeem a voucher at least 90 days prior to redemption.

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Related strategic options for expedited development

GAIN Act

The Generating Antibiotic Incentives Now (GAIN) Act created under FDASIA aims to encourage development of antibacterial and antifungal drugs for the treatment of serious or life-threatening infections. Eligible products are granted a Qualified Infectious Diseases Product (QIDP) designation. Products with a QIDP designation are eligible for priority review of marketing applications and fast track designation as well as an additional five years of exclusivity at the time of FDA approval.

Fast track designation

Fast track designation applies to drugs with the potential to address unmet needs. This pathway accelerates new drug development and review by increasing FDA communication and enabling rolling review. The key distinguishing factor of this expedited development program is that fast track designation may be based on results from non-clinical studies indicating that the product may represent a significant therapeutic advantage. Sponsors should request fast track designation no later than the pre-submission meeting, if possible. Of note, the designation may be rescinded if the drug no longer meets qualifying criteria.⁵

Breakthrough therapy designation

Also created under FDASIA, the breakthrough therapy designation is designed to expedite development and review of breakthrough therapies. To qualify as a breakthrough therapy, a product must

be intended to treat a serious or life-threatening condition, with preliminary clinical evidence that the product demonstrates substantial improvement on one or more clinically significant endpoints compared with available therapies. Of note, approval may be based on an effect on a surrogate or intermediate clinical endpoint that is reasonably likely to predict a drug's clinical benefit, and the designation may be withdrawn if the product no longer meets qualifying criteria.

Sponsors should be aware that breakthrough therapy designation carries with it rigorous guidance on efficient drug development during the Investigational New Drug (IND) phase, beginning as early as Phase I, and so involves more intensive regulatory scrutiny on planning.

Accelerated approval

This pathway allows early approval of a drug that offers a benefit over current treatments based on a surrogate endpoint or other clinical measure that is reasonably likely to predict a clinical benefit.⁶ After accelerated approval is granted, the drug must undergo additional confirmatory studies to establish benefit.

Priority review

Priority review calls for review within six months instead of the standard 10 months. This pathway is reserved for situations in which the CDER has determined that the investigational drug could potentially provide a significant advance in medical care. Priority review is typically requested with the original BLA, NDA, or efficacy supplement.⁶

EU orphan landscape

Orphan drug legislation was introduced in the EU in 2000. These regulations stipulate that patients with rare diseases deserve the same quality, safety, and efficacy standards for medicinal products as other patients and that, in order to make these products available to all patients in the EU, orphan drugs are required to obtain a Community Authorisation through the centralized marketing authorization procedure.

To qualify for orphan designation in the EU, a product must:⁶

- Be intended for the diagnosis, prevention, or treatment of a life-threatening or chronically debilitating condition that affects no more than five in 10,000 people in the EU
- Be unlikely to be marketed without incentives

In addition, there should exist no satisfactory method of diagnosis, prevention, or treatment of the condition in question that has been authorized in the EU or, if such method exists, the product will be of significant benefit to those affected by that condition. The Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) is responsible for determining whether all orphan designation criteria have been met, and the Committee for Medicinal Products for Human Use (CHMP) is responsible for providing a scientific opinion on the application merits for marketing authorization. The success of orphan incentives in driving drug development is reflected in the steady growth of orphan designations in the EU (see Figure 2). While the actual percentages vary from year to year, more than half of the medicines that receive a positive opinion for orphan designation from the EMA concern medical conditions affecting children (see Figure 3).⁷

Figure 2. Orphan designations: EU and U.S.⁷

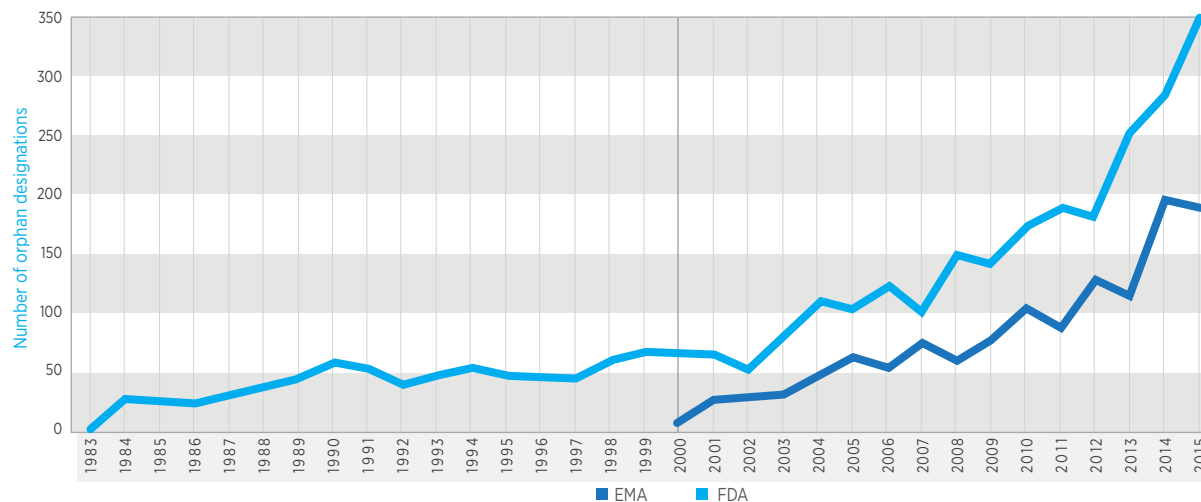
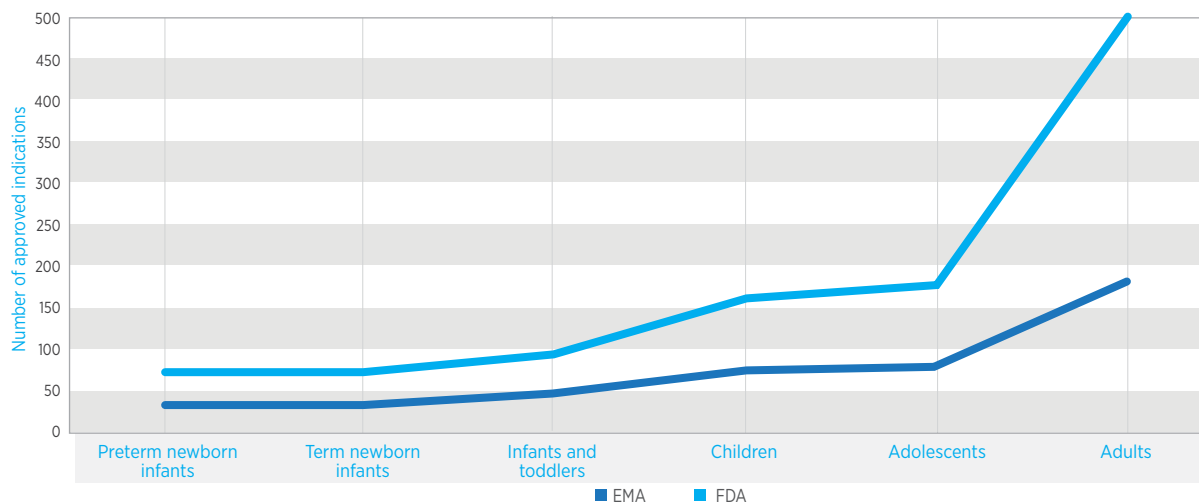


Figure 3. Orphan designations by age group: EU and U.S.⁷



Strategic considerations for EU development

An application for orphan designation in the EU can be made at any stage of development prior to submission of the application for marketing authorization. It is recommended to apply as early as possible during the development lifecycle in order to maximize the benefits from available incentives, but it is worth noting that all products receiving orphan designation are published on the EMA website, making orphan designation status public knowledge. Pre-submission meetings with COMP are also encouraged.

Incentives

In addition to the competitive, development, and pricing advantages that may be associated with orphan drugs, incentives for development of orphan medicinal products in the EU include:

- Protocol assistance (scientific advice) which is provided free of charge for orphan drugs for pediatric studies and at a 75 percent discount for other studies
- 10 years of market exclusivity
- Fee reductions for marketing authorization applications and maintenance
- EU incentives in the form of grants
- National incentives in the form of grants and tax reductions

Related strategic options to accelerate development

Expedited review programs offered by the EMA include:

- Accelerated assessment, which applies when a medicinal product is expected to be of major health interest. This pathway can reduce review time from 210 days to 150 days exclusive of clock stops.⁸ Accelerated assessment should be requested at least two to three months before submitting the marketing authorization application. In addition, the EMA strongly recommends that sponsors request a pre-submission meeting six to seven months before submission.
- Conditional marketing approval, applied to medicinal products that address seriously debilitating or life-threatening diseases, emergency threats, or orphan indications. This pathway can be used when the benefit of immediate availability to public health outweighs the risk that additional data is still required.⁹ Conditional marketing approval is only valid for a year at a time and must be renewed annually until full approval is granted.

Figure 4. EMA scientific advice fees

Scientific Advice	Fee
Small- and medium-sized enterprise (SME)	90% reduction
SME + orphan	100% reduction
Orphan	75% reduction
Advanced Therapy MP (ATMP)	65% reduction
ATMP + SME	90% reduction
ATMP + orphan	bd
Normal product	€83,600 - 41,700

- Exceptional circumstances, applied when applicants are unable to provide comprehensive clinical data due to the rarity of the disease, the present state of scientific knowledge, and/or ethical constraints.¹⁰ Of note, orphan drugs must still meet the additional criteria for orphan designation to be granted this status.

As of March 2016, the EMA has introduced the PRiority MEdicines (PRIME) program, which applies to medicinal products that either offer a significant therapeutic advantage over existing therapies or benefit patients without treatment options.¹¹ To be eligible for PRIME, a medicine must show potential to benefit patients with unmet medical needs based on early clinical data. Like accelerated assessment, PRIME can reduce review time.

Notably, in December 2015, the European Commission convened a workshop to review the orphan medicinal product guideline with regard to the definition of “significant benefit” and the provisions for market exclusivity.¹²

EMA/FDA harmonization

For sponsors who are investigating products for use in both the EU and the U.S., there are points of commonality between the development and approval processes for the EMA and FDA.

Many companies will submit a joint EMA/FDA application when seeking orphan drug designation and, following designation, a joint annual report summarizing the status of product development.

Key differences between orphan drug development in the EU and the U.S. include:

- Prevalence criteria to qualify as a rare disease – five in 10,000 in the EU vs. fewer than 200,000 in the U.S.
- Period of market exclusivity – 10 years in the EU vs. seven years in the U.S.
- Review of exclusivity – After five years if prevalence changes in the EU and no review in the U.S.

Of note, in the EU sponsors may apply for orphan designation for an already-approved medicinal product provided the orphan designation concerns a not-yet-approved therapeutic indication.

This requires a separate marketing authorization that only covers the orphan indication.

Figure 5. Orphan drug development in EU vs. U.S.

	EU	U.S.
Legal basis	Reg EC/141/200	Orphan drug act 1983
Prevalence	5 in 10,000	Fewer than 200,000
Incentives	10 years market exclusivity	7 years market exclusivity
Exclusivity review	After 5 years if prevalence changes	No change
Timeline	90d (COMP) + 30d (EC)	No timelines (1-3 months)
Procedural	Centralized procedure and EU wide approval	n/a

Improving regulatory and operational performance in rare disease trials

The smaller Phase III trial sizes required for rare diseases help lower the cost of development for orphan drugs compared with non-orphan drugs but do not necessarily save time, as there is no significant difference in the median length of a Phase III trial for an orphan or non-orphan drug. Moreover, sponsors might be surprised to learn that, despite the existence of accelerated approval pathways, the median FDA approval time for orphan drugs is not much shorter than it is for non-orphan drugs.¹³ These statistics highlight the need for improved regulatory and operational performance, creating an opportunity for rare disease sponsors to shift the paradigm in orphan drug development.

Optimizing the regulatory pathway

After obtaining orphan drug designation for a rare disease compound, there are a variety of factors sponsors should consider for optimizing the regulatory pathway (see Figure 7). It is important to note that every stage of the development cycle is an opportunity for investor engagement.

Optimization of the regulatory pathways has the potential to increase revenue through sales expansion or protection, as seen in the real-life examples illustrated in Figure 8:

1. **Early access** – starting with an orphan disease as the primary indication, followed by a subsequent non-orphan disease indication
2. **Multiple orphan drug designations** – pursuing orphan disease designations in multiple indications
3. **Sub indications** – starting with a non-orphan disease as the primary indication, followed by a subsequent orphan disease indication

Historically, the greatest monetary gains have come from the transition of an orphan drug into non-orphan indications.

Figure 6. Phase III trials: standard vs. orphan drug development¹⁴

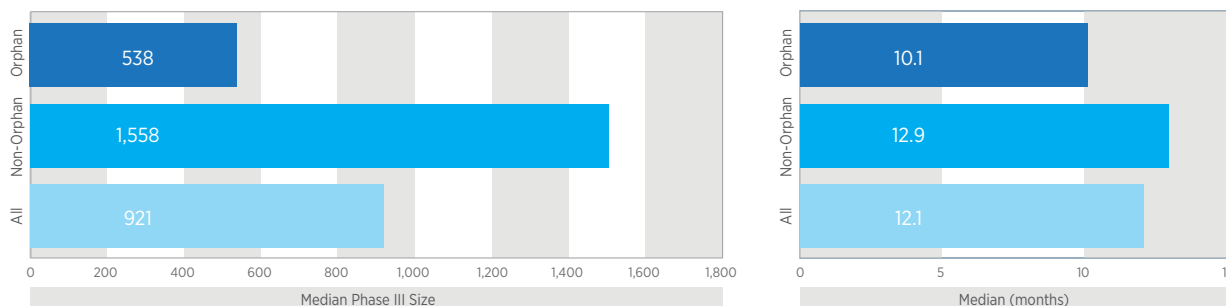


Figure 7. Regulatory optimization pathway

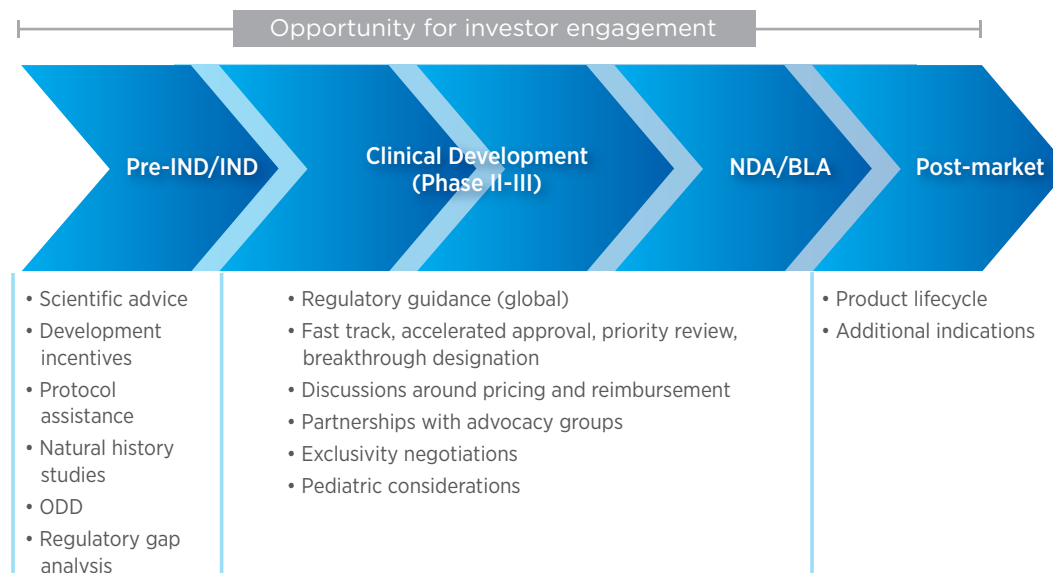


Figure 8. Strategies for increasing revenue potential¹⁵

Pathways	Primary Indication	Subsequent indication	Examples
1. Early access	Orphan Disease A	Non-Orphan Disease B	Avastin bevacizumab
2. Multiple OD	Orphan Disease A1	Orphan Disease A2	Glivec imatinib
3. Sub indications	Non-Orphan Disease A	Orphan Disease B	Erbix cetuximab

In the area of rare diseases, one size does not fit all and every development pathway is unique. Thus, it is critically important to initiate regulatory engagement early when developing and operationalizing plans for orphan drug development.

Strategies for successfully navigating the regulatory landscape include:

1. **Engaging early** and often with regulators
2. **Involving stakeholders** in the regulatory process
3. **Leading with science** to educate regulators and set new precedents
4. **Developing a global strategy**, even if budgets don't permit
5. **Developing a reimbursement strategy upfront** and demonstrating the value to regulators

6. **Creating a unique development algorithm** for each rare disease compound
7. **Innovating**, as only five percent of rare diseases currently have a treatment

Optimizing operational performance in rare disease clinical trials

There are five key areas to consider when operationalizing clinical trials within rare disease to support orphan drug designation: patient identification, patient engagement, patient retention, investigative site selection, and site team engagement.

Patient identification

Patients can be identified through:

- **Medical informatics** – these mechanisms may include site databases, feasibility assessments, in-depth review of the disease population during the site qualification process, standard of care for the target population, and other touchpoints to raise trial awareness
- **Key opinion leaders**
- **Patient registries**
- **Patient advocacy groups** – leveraging relationships with appropriate advocacy organizations and their social media platforms can provide valuable insight into the challenges patients and their families face, which can assist with the development of operational considerations when conducting a clinical trial

Patient engagement

Patient engagement is important when developing a rare disease clinical trial. Hearing the voice of the patient allows sponsors to understand what the patient is going through, and sponsors should take advantage of the opportunity to gain that perspective. Tailoring communications so that patients and their families truly understand what it means to be involved in a clinical trial – not only the potential benefits, but also the requirements – is essential. As many rare diseases affect children, parents are critical to the success of the recruitment process. As such, all clinical trial-related materials, including patient outreach materials, branding, and advertising, should be created with both parents and children in mind.

Patient retention

Putting patients first is the cornerstone of a successful rare disease clinical trial. One of the first steps for increasing retention is clarifying the commitment to participate. Developing participation packets or manuals can help patients and families understand the level of commitment required and how it might impact work or school. Sponsors should also consider travel-related expenses and accommodations for patients and families when site visits are required. Providing a patient payment system is one option for addressing this issue.

Another key consideration is to acknowledge the contribution patients and families are making through periodic updates of study results or thank you notes, providing a personal touch. In some cases, it may be appropriate to solicit feedback from patients and families. When it comes to patient centricity, sponsors are encouraged to think creatively, taking into account patients' situations and considering all the little things that could improve the clinical trial experience.

Investigative site selection

Due to the inherent complexity of rare disease trials, investigative site selection is very important. When selecting investigative sites for a rare disease trial, a good rule of thumb is to go where the patients are. This will require sponsors to identify and understand country regulatory submission requirements and Good Clinical Practice (GCP) standards for investigators and monitors in every country under consideration. Sponsors will also need to identify travel restrictions, import/export license requirements, and even the geopolitical landscape in each country. Regarding site selection, sponsors should perform due diligence on the rare disease experience and technical capabilities of prospective sites to ensure they are able to execute all study requirements.

Site team engagement

In rare disease trials, every patient counts, so site team engagement and support are critical. In addition to regular communication and quality monitoring, thorough training should be provided at site qualification visits, site initiation visits, and monitoring visits. Where required, additional GCP training may be needed. Sponsors should also ensure that there is a succession plan for staff transit.

Conclusion

Wide-ranging incentives that include grants, tax credits, user fee waivers, and longer periods of market exclusivity are fueling the growth in orphan designations and orphan drug approvals. These incentives help to lower development costs, shorten review times, and facilitate earlier and more frequent communication with regulatory agencies, all of which accelerate the time to market for drugs and biologics that have the potential to transform the lives of people suffering with rare diseases.

For sponsors seeking to bring orphan drugs to market and into the hands of patients, early engagement with regulators is a critical first step. Frequent interaction with operational teams is also essential for ensuring successful study execution. Finally, involving key stakeholders throughout the entire regulatory and clinical development process, and thinking both creatively and globally around strategy, can help in optimizing the likelihood of clinical – and commercial – success.



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Angi Robinson has been studying rare diseases and pediatrics at Premier Research for over 15 years. She has provided oversight and management support for more than 30 rare disease studies in the U.S. and globally and has supported FDA pre-IND meetings, IND submissions, and NDA/BLA project directorship. Angi has worked on industry sponsored studies to support the approvals of four drugs with orphan drug designations. Her experience includes multiple study designs including natural history studies with retrospective and prospective designs as well as PK/PD, adaptive design, and FDA fast track and Breakthrough Therapy designations.

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Nach Davé oversees Premier Research's regulatory affairs service offerings across its broad range of therapeutic focus areas, bringing to his position more than 20 years of experience in the pharmaceutical and contract research industries. He previously served the company as Director of Regulatory Affairs and rejoined Premier Research after two years as Senior Director of Regulatory Affairs at PRA Health Sciences. He also led clinical and regulatory affairs at Maxx Orthopedics, a developer of orthopedic medical devices, and has held roles in clinical operations, business development, strategic consulting, and medical affairs at companies such as Merck, Bristol-Myers Squibb, Aventis Pharmaceuticals, and Mitsubishi Pharma America.

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