

EU Orphan Drug Launch

Understanding and delivering your EU Orphan Drug Launch



EU Orphan Drug Launch

eBook contents

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Section 1

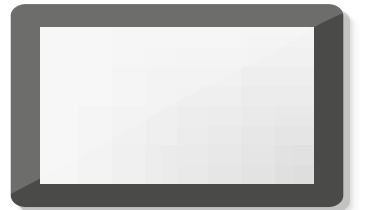
The EU rare disease marketplace

In Europe, a disease is defined as rare when it affects fewer than 5 in 10,000 people (Orphan Drug Regulation EC141/2000). It is estimated that between 5,000 and 8,000 distinct rare diseases exist, affecting between 6% and 8% of the population in the course of their lives. In other words, although rare diseases are characterised by low prevalence individually, the total number of people affected by rare diseases in the EU is estimated at between 27 and 36 million. (EUCERD Joint Action - 2014 Report on the State of the Art of Rare Disease Activities)

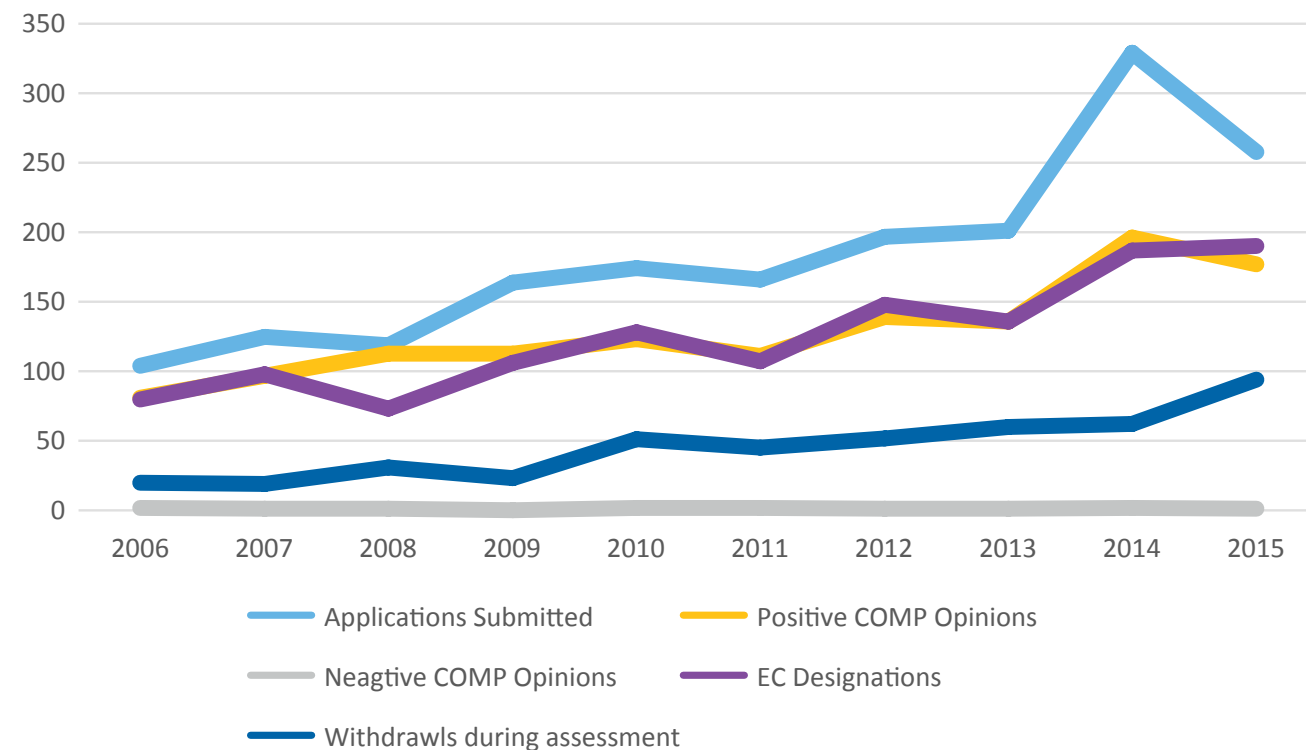
With a number of European regulations and policies in place in favour of rare disease research and providing incentives for the development and marketing of these innovative drug products, the number of applications has increased steadily each year.

Although the European marketplace presents a significant opportunity for innovative orphan drug companies to meet unmet patient needs, launching your drug product can present a real challenge if you are not familiar with country specific requirements. With its 28 member states, numerous official working languages and complex regulatory framework, this eBook aims to outline the key requirements for a successful EU product launch.

Resources



Considerations for a successful EU orphan drug launch.



Source: The European Medicines Agency, EMA/COMP/205240 Rev1, April 2015

Regulatory considerations

Similar to the US, all medicinal products need to obtain a license / Marketing Authorisation (MA) before they can be placed onto the European market.

The MA certifies that the product meets the required standards of safety, quality and efficacy. Unlike the US however, there are four main routes of gaining an MA in Europe (Centralised Procedure, Decentralised Procedure, Mutual Recognition Procedure and National Procedure), with varying critical time points throughout the process, including clock stop periods, making the process that bit more complicated.

Our simplified overview explains the process,
[Tap for more details](#)

[Introduction to Regulatory Framework.](#)

[Q&A - Launching Drug Product in Europe](#)

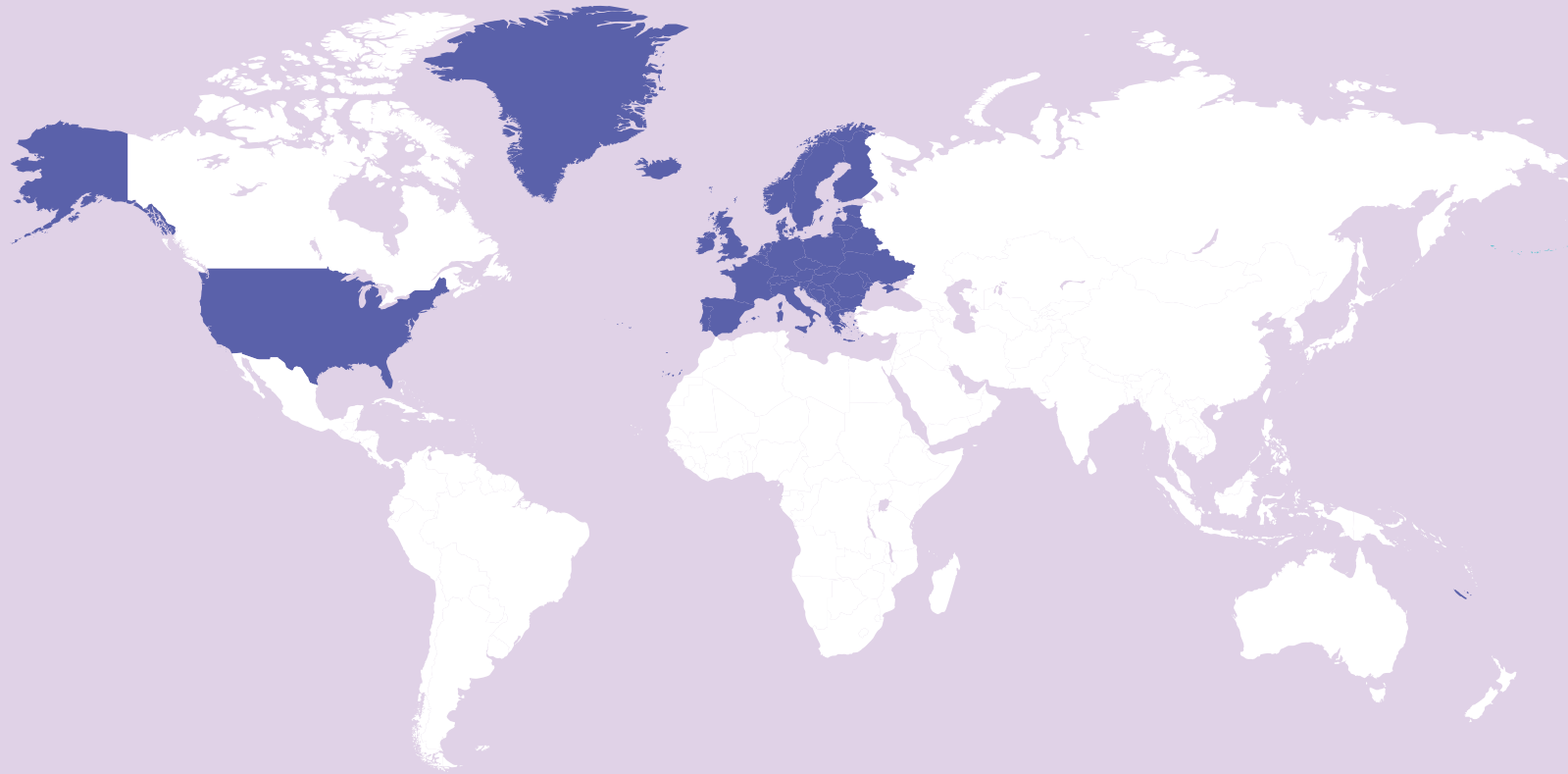
Regulatory considerations

Click/tap each section of the time line for details.

Centralised Procedure Overview



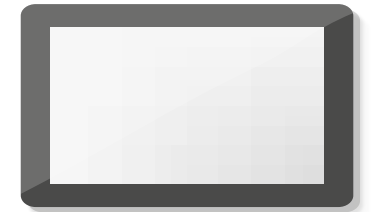
Quality requirements - EU Import testing and QP release



In contrast to the US, Europe has certain market specific quality requirements before drug products can enter the marketplace.

If your drug product is manufactured outside of the EU, there is a legal requirement outlined in Directive 2001/83/EC: Article 51, which stipulates that all pharmaceutical products manufactured outside the EU must be analysed in line with the specification detailed in the product's MA upon entry into the EU.

In addition, in the EU, all drug products need to be certified as suitable for release in line with the requirements of EU Good Manufacturing Practices (GMP) and your MA, by a Qualified Person (QP). Therefore, nominating a QP to release your drug product is essential when you are submitting your MA.



Quality Requirements
for Launch

The role of the QP

Packaging design and regionalisation considerations

When preparing for launch, commercial pack design can sometimes be under or over engineered, often resulting in unnecessarily complex and subsequently expensive pack prototypes.

Unlike the US market with typically a single language pack, in Europe, to minimise stock holding and maximise flexibility, grouping markets into multi-language / country packs and implementing regionalisation strategies is beneficial.

[Packaging Considerations](#)

[Pack design for EU Orphan Drug launch](#)

European supply chain considerations

The European market presents a complex supply chain, from varying import/export requirements, tax, VAT and custom duties, differing country legislation regarding physical distribution of drug product to temperature extremities impacting how the drug product is shipped.

As there is no “one size fits all” supply chain, pharmaceutical companies are developing tailored supply chains depending on product type (orphan drug / generic / patented / controlled drugs etc.) and/or by channel (wholesale, direct to pharmacy, direct to patient, etc).

With high value orphan drug products, Pharma companies seek a more agile and responsive distribution model, as the efficacy of the product can be impacted through a single cold chain break leading to costly delays in bringing new drugs to market or in the worst case having to write off the drug product which could be worth millions of dollars.

With this in mind regulators are now placing a much higher level of scrutiny on distribution quality systems and processes, with temperature management being one of the key elements.

Regulatory bodies require proof that all drugs and biological components have been distributed within the necessary temperature range – meaning all processes need to be validated to ensure that there has been no negative impact on the safety, efficacy or quality of the product.

EU supply chain requirements and considerations for temperature sensitive products.

Temperature control distribution

Serialisation

The World Health Organisation (WHO) estimates that counterfeit medicines represents 10% of all global medicine trade. The increase in the international trade of counterfeit medicines presents serious risks to public health.

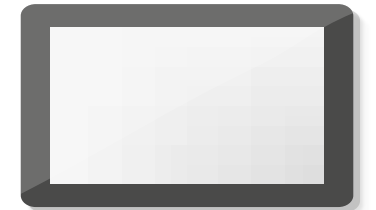
To ensure patient safety, biopharma companies are urgently seeking ways to protect the integrity of their valuable drug products and keep counterfeit medicines out of the legitimate supply chain.

While individual countries have started to build local legislative frameworks to address the problem, for example the US Drug Quality & Security Act (DQSA) and the European Falsified Medicines Directive (FMD), there is currently no standardised global approach to prevent trade in illegitimate drugs.

What is clear, however, is that serialisation – applying a unique identifier at the level of the individual product unit – is emerging as a core requirement for any supply chain security initiative.

Which one is the real drug?

Rollover or tap to find out.

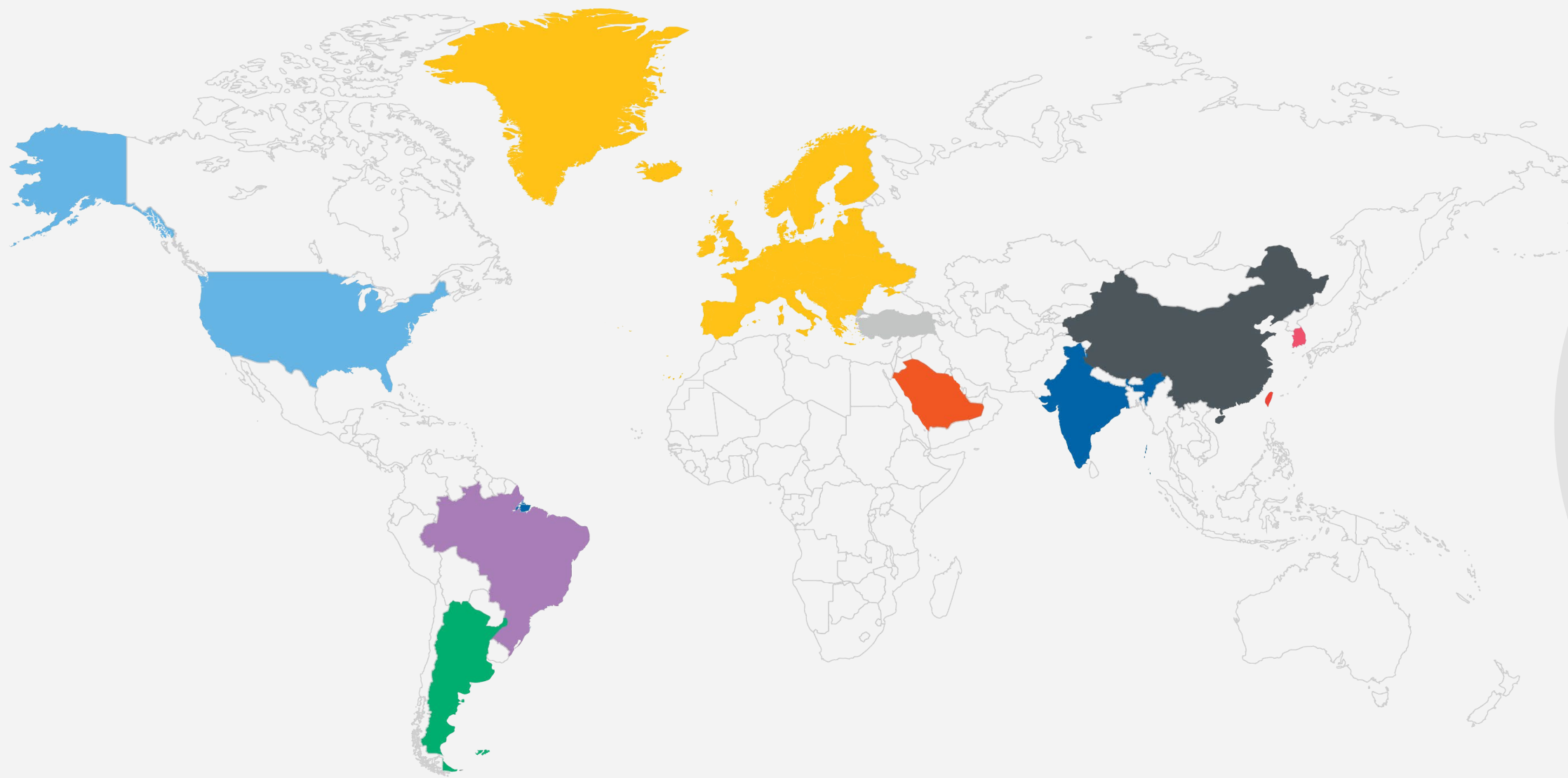


Serialisation solutions to meet global requirements

Serialisation solutions to meet client needs

Serialisation

Click/tap highlighted areas of the map for details.



Almac's Orphan Drug expertise

With over 40 years' experience, Almac is an FDA and EU approved outsourcing partner to the global pharmaceutical and biotechnology industries.

Having successfully partnered in the commercial launch of many of the industry's recent first in class, innovative orphan drug products in areas such as Cystic Fibrosis, Muscular Dystrophy, Hemolysis and Short Bowel Syndrome, Almac leads the CMO field in integrating its services to support orphan drugs.

Assisting client partners to achieve launch time lines and maximise product flexibility to serve global markets, Almac offer a full range of product launch support services including:

- Product launch consulting services
- Regulatory support for EU filings
- EU import testing and QP market release
- Packaging design and artwork generation
- Customised packaging solutions
- Order processing and Financial services
- Storage and end-user distribution (Ambient, Cold, Frozen and Controlled Drugs)
- Serialisation

Click or tap to discover Almac's ability

Benefiting from integrated pharmaceutical and distribution services from a single supply team, clients minimise effort and maximise return. It's never too early to start to think about your pharmaceutical product launch.

We take pride in our ability to support clients with varying time frames, from as far as 24 months before submission of their MA to those with shorter time frames post submission.

Our dedicated product supply team are experts in navigating marketing requirements throughout the launch process from pre-submission, submission, approval, launch and finally ongoing commercial supply.

To ask them a question, contact us at pharmaservices@almacgroup.com



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Tap to jump to:

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