IJBPAS, December, 2025, 14(12): 6743-6749

ISSN: 2277–4998

International Journal of Biology, Pharmacy and Allied Sciences (IJBPAS)

'A Bridge Between Laboratory and Reader'

www.ijbpas.com

REGULATORY REQUIREMENTS FOR APPROVAL OF RARE BLOOD DISEASE- FDA

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Received 24th Aug. 2024; Revised 15th Oct. 2024; Accepted 19th Dec. 2024; Available online 1st Dec. 2025

https://doi.org/10.31032/IJBPAS/2025/14.12.9685

ABSTRACT

FDA states that Blood Diseases are the conditions impair the functional ability of blood by affecting one or more blood components & the Blood Disorders are uncertainty that your platelets, red or white blood cells, or blood clotting proteins aren't working correctly, you might have a blood disorder. Centre for Drug Evaluation and Research's (CDER) Division of Non-Malignant Haematology. "This action demonstrates the FDA's dedication to assisting patients with rare diseases in accessing new treatments." The regulatory pathway guarantees a thorough evaluation of treatments safety and efficacy prior to patient administration. Gathering information from clinical trials about the treatment's effectiveness and safety is a crucial step in this process. Advisory groups and government oversight organizations offer a further degree of examination to guarantee that the benefits of the treatment. In terms of safety, the FDA emphasizes a thorough risk-benefit analysis, with a focus on minimizing potential risks associated with the treatment. Detailed pharmacovigilance plans and long-term follow-up studies are often required to monitor post-marketing safety. FDA's dedication to promoting innovation, enhancing patient safety, and developing cutting-edge therapies for these difficult diseases to provide patients with rare blood diseases life-changing treatments, developers and stakeholders must carefully meet these requirements.

Keywords: Blood Disease, Blood Disorders, CDER, FDA, Approval Process

INTRODUCTION:

Blood Diseases: These are the conditions impair the functional ability of blood by affecting one or more blood components. Numerous blood disorders and diseases have hereditary roots. Other factors include untreated illnesses, medication side effects, and inadequate dietary intake of specific nutrients. Anaemia and bleeding disorders like haemophilia are common blood disorders **[1]**.

Blood Disorders: If your platelets, red or white blood cells, or blood clotting proteins aren't working correctly, you might have a blood disorder **[2]**.

Different types of blood disorders as follows **[2]**.

Table 1: Shows The Different Types of Blood Disorders

S. No.	BLOOD DISORDERS
1.	ANAEMIA
2.	ANTIPHOSPHOLIPID SYNDROME (APS)
3.	HAEMOCHROMATOSIS
4.	HAEMOPHILIA
5.	HTLV-1 INFECTION
6.	LEUKAEMIA
7.	LYMPHOMA
8.	MULTIPLE MYELOMA
9.	SICKELCELL ANAEMIA
10.	THALSSAEMIA
11.	THROMBOCYTOPENIA
12.	THROMBOSIS
13.	VON WILLEBRAND DISEASE

Improvement in the pharmaceutical and biotechnology sciences has contributed to a growing number of new medications and biological products (drugs and biological products together, drugs unless otherwise noted) going through clinical development.

Over 7,000 rare ailments affect more than 30 million Americans. About 6,200 Americans are affected by polycythaemia vera annually, according to Ann Farrell, M.D., who oversees the FDA's **Center for Drug Evaluation and Research's** (CDER) Division of Non-Malignant Haematology. **"This action demonstrates the FDA's dedication to assisting patients with rare diseases in accessing new treatments."**

Besremi is the foremost FDA-licensed treatment for polycythaemia vera and the primary interferon therapy approved expressly aimed at the condition. Patients can utilize Besremi regardless of their prior medical background.

Obtaining designations of orphan status and preparing a medication for early clinical development under an IND are important aspects, though different, regulatory factors to take into account when developing drugs for rare diseases **[3]**.

An overview of these two issues is provided by representatives of the US Food and Drug Administration's (FDA)

- Office of Orphan Products Development (OOPD) and
- Office of New Drugs (OND) of the Center for Drug Evaluation and Research (CDER).

The process of developing a new drug is challenging and typically takes several investigational stages. Expertise in science and medicine, along with familiarity with pertinent laws and regulations, are crucial. This summary is intended only as an introduction to the field of drug development, along with some recommendations and resources for guidance and information during the initial stages of clinical development. FDA also emphasizes that individuals or groups interested in developing new drugs—called "sponsors" in Federal law (US FDA, 2010a)—who sometimes find it challenging to attend meetings with regulatory bodies are urged to overcome their reluctance.

FDA advise sponsors to get in touch with FDA as soon as they can to take advantage of available channels for communication. We list a few of these opportunities in this article. The FDA is aware of the difficulties involved in developing new medications, so it might be able to offer helpful development strategies **[4]**.

OBJECTIVES

- To provide in details about the regulations & guidance documents for Rare Blood Disease in US
- To understand the review process for Approval of Rare Blood Disease in US
- To comprehend the regulatory requirements for filing IND

RESULTS & DISCUSSIONS:

Overview, challenges & opportunities

Scientific advancements have resulted in significant improvements in patient longevity and quality of life for those with particular rare diseases.

Examples.

- The search for a cure for cystic fibrosis is still beyond reach, targeted treatments have greatly boosted children's average life expectancy, which went from less than 10 years in the 1960s to more than 40 years in the present (CFF, 2008).

Basic research on disease mechanisms has led to the development of treatments that have improved the lives of people with a range of illnesses, such as enzyme deficiency disorders, chronic myeloid leukaemia, and phenylketonuria. According to certain studies that have improved prevention, pregnant women may be able to lower their risk of congenital defects like

spina bifida by following basic dietary guidelines.

Despite these developments, there is still a lack of basic knowledge, let alone effective treatments, regarding the mechanisms underlying many rare conditions. Because rare diseases are so uncommon, most healthcare professionals—even specialists—have never seen a single patient with one. This makes clinical practice more difficult. Diagnosing someone can be difficult and take years. Many diagnoses are taken into consideration and finally ruled out. A patient who receives a delayed diagnosis may experience avoidable and irreversible harm if a successful treatment is available.

In recent years, new methods for conducting basic research have made it simpler, quicker, and less expensive to determine the genetic causes of uncommon diseases. However, extensive research may still be necessary to determine how a genetic defect interacts with other factors to cause the physical or mental manifestations of a illness. Concurrently, many common diseases are being broken down into smaller, sometimes even rare, molecularly defined subgroups with distinct beneficial features besides distinct needs for the development of new products as a result of analysis.

The 1983 Orphan Drug Act provides pharmaceutical companies with financial incentives to develop products for rare diseases, as will be discussed in more detail below. The Food and Drug Administration (FDA) has approved over 350 applications for orphan drugs since 1983. More than thirty percent of novel medications were approved by the FDA between 2004 and 2008, many of them were treatments for rare diseases (Coté, 2009).

For a variety of rare diseases, NIH has launched the Rare Diseases Clinical Research Network in addition to extra focused research projects. A growing quantity of small businesses are now concentrating on creating medications to treat uncommon illnesses, and some major pharmaceutical corporations are growing more intrigued by the benefits provided by the Orphan Drug Act **[5]**.

In fact, there are still a lot of obstacles in the way of developing new products and studying uncommon diseases. Obtaining backing from civic agencies for fundamental and translational research as well as safeguarding business reserves to produce goods for tiny sales outlets. Even with subsidy, researchers often struggle to find enough biological samples for crucial preclinical studies or to find and employee adequate research participants for clinical safety trials of a product and practicality. When expenses and challenges rise to the level where a product under.

The effects of learning can be either delicate or gradual. Recognizing and winning over

biological markers and proxy measurements for disease and treatment effects provide a difficulty for researchers studying typical

Notwithstanding these challenges, hundreds, if not thousands, of rare diseases—some of which are incredibly occasional—are being investigated by researchers with funding from the FDA, the NIH, and a variety of commercial and nonprofit organizations **[5]**.

Medical Products, Drugs, Biologics

In this report, "medical product" refers to pharmaceuticals, biologics, and medical devices. Products "intended to affect the structure or any function of the body of man or other animals" (apart from foods) and those "intended for use in the diagnosis, cure, mitigation, treatment, or prevention of disease".

Medical Devices Any device envisioned for therapeutic, precautionary, or diagnostic use that derives its prime action from substances or processes found naturally in the body is considered a medical device.

Orphan Products Orphan drugs are generally biological products and other medications meant for patients with rare diseases, meaning fewer than 200,000 people in the US are affected by them.

Regulatory Framework for Drugs for Rare Diseases

The Food and Drug Administration (FDA) must make difficult decisions about how to carry out a number of intricate tasks. Determining what sign is adequate to backing the agency's sanction of drugs for patients with rare sicknesses is one area of complexity. More generally, when balancing the potential for a policy to promote public health by encouraging innovation and access to new therapies against the possible for that policy to expose the community to unsafe or unproductive products, both the FDA and Congress are faced with difficult decisions and frequently lack conclusive information **[6]**.

The Orphan Drug Act, offers sponsors encouragements such as grant support, tax acclaims for specific clinical development costs, and exclusive marketing rights to inspire the progress of medications for patients with rare diseases. State agencies and nonprofit research groups have supported applications to designate and approve drugs for use as orphans on occasion, but the majority of sponsors are for-profit biotechnology or pharmaceutical companies **[5]**.

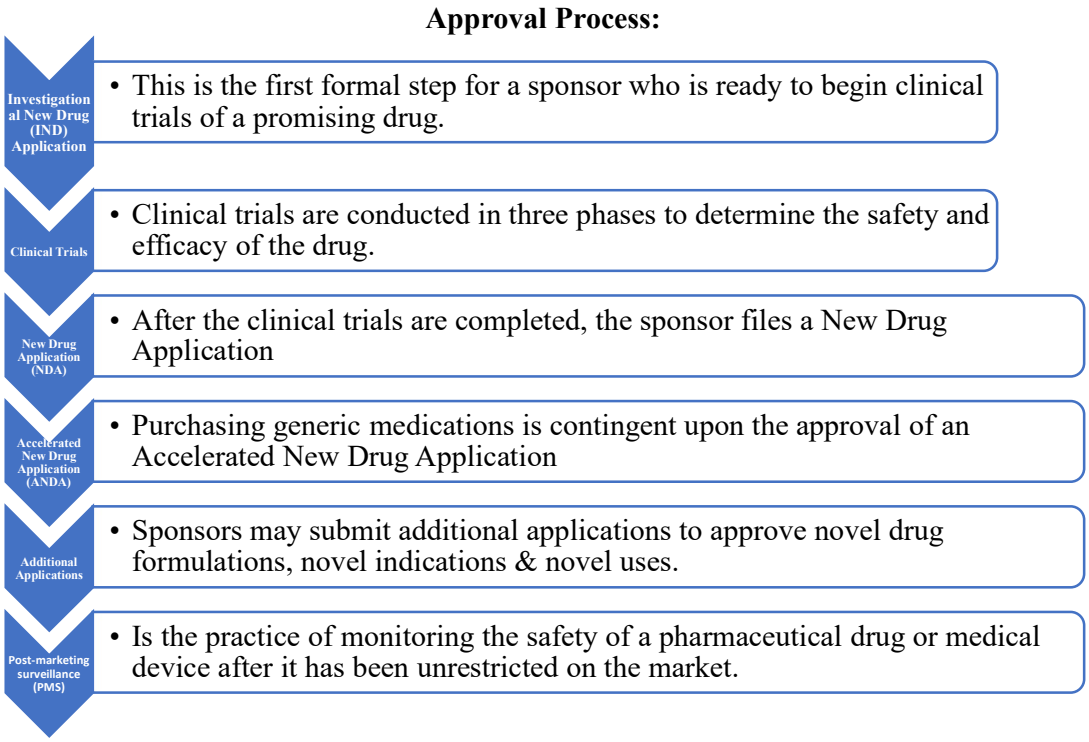

Figure 1: Shows the Approval Process of Rare Blood Diseases

SUMMARY & CONCLUSION

The above study provides a summary of the legal requirements for approval of treatment for rare blood diseases. A number of significant process steps are covered, including advisory committee meetings, post-marketing surveillance, clinical trials, IND applications, regulatory reviews, NDA/BLA submissions, and preclinical research. The study also emphasizes the significance of classifying medications as orphan drugs for uncommon diseases. The regulatory pathway guarantees a thorough evaluation of treatments' safety and efficacy prior to patient administration. Gathering information from clinical trials about the treatment's effectiveness and safety is a crucial step in this process. Advisory groups and government oversight organizations offer a further degree of examination to guarantee that the benefits of the treatment.

ACKNOWLEDGEMENT

I would like to thank Dr. Abhishek B V, Assistant Professor, Department of Pharmaceutics & Regulatory Affairs, SACCP, ACU, B G Nagar, Karnataka, India. And Nishant P Manasani for useful discussion during the studies and support in Review.

CONFLICT OF INTEREST

The authors declare no Conflict of interest

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