



RADIOPHARMACEUTICALS

Helping a Biotech Secure US FDA Breakthrough Therapy Designation

A European biotech developing a novel radiopharmaceutical therapy was preparing a Breakthrough Therapy Designation (BTD) submission to the US FDA and required specialist regulatory writing support to ensure success first time.

Securing BTD is a critical milestone for programs with early clinical evidence of substantial improvement over existing therapies. For this company, the designation represented an opportunity to accelerate development timelines and strengthen engagement with the FDA at a pivotal stage of their program.

OVERVIEW

The biotech had already received EMA PRIME (PRiority MEdicines) designation and was preparing to leverage this momentum through a US Breakthrough Therapy Designation submission. Their internal team had strong regulatory and clinical expertise, supported by robust source documentation including a PRIME dossier, pre-IND briefing book, and Investigator's Brochure.

However, the organization recognized that BTD submissions require more than technical accuracy. With no FDA-provided template and highly nuanced qualifying criteria, the company sought an experienced regulatory writing partner who could provide strategic leadership, deep familiarity with FDA/EMA designations, and specific expertise in radiopharmaceutical programs.

CHALLENGE

Navigating a High-Impact FDA Designation Without a Defined Framework

Although the biotech was well prepared scientifically, several challenges shaped their decision to outsource specialist support:

- No standard FDA template for BTD submissions, requiring expert judgement on structure, emphasis, and narrative flow.
- Complex regulatory positioning, aligning EMA PRIME documentation with FDA expectations while meeting BTD-specific criteria.
- Limited internal capacity for strategic medical writing, with senior regulatory leaders balancing multiple priorities.
- High stakes for first-time submission, where clarity, structure, and persuasive justification are essential to avoid delays or rework.



Key Takeaway for Biotechs

For companies pursuing accelerated FDA pathways, Breakthrough Therapy Designation submissions demand strategic narrative development, regulatory insight, and designation-specific expertise.

Partnering with experienced regulatory medical writers can significantly reduce risk, preserve internal capacity, and maximize the likelihood of success at critical milestones.

Get in touch to discuss how our experienced team and Medical Writers can support you.

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WHY BIOTECHS CHOOSE MORULA HEALTH

- ✓ Deep, designation-specific regulatory expertise
- ✓ Strategic leadership that reduces risk and internal burden
- ✓ Efficient, high-quality delivery with built-in quality control

SOLUTION

Early interactions with regulators had confirmed alignment with BTM eligibility criteria. The remaining challenge was translating this validation into a compelling, submission-ready document that clearly demonstrated the therapy's potential clinical advantage.

Following initial discussions, Morula Health assigned a lead medical writer with recent, hands-on experience in FDA Breakthrough Therapy Designations, radiopharmaceutical development, and cross-agency regulatory strategy. This ensured immediate strategic direction without onboarding delays.

1. Structured, Criteria-Led Submission Planning

The project began with a comprehensive review and gap analysis of all source materials, followed by a focused kick-off meeting to:

- Define a clear, FDA-appropriate BTM structure, mapped directly to qualifying criteria
- Establish a detailed content plan for each section based on available source documents
- Align expectations for subject-matter expert input across nonclinical, clinical, and CMC data

In the absence of an FDA template, a customized BTM format informed by TransCelerate principles was developed to ensure consistency, clarity, and regulatory suitability.

2. Expert-Led Writing and Quality Oversight

The lead writer brought extensive experience across multiple FDA pathways, including BTM, Orphan Drug Designation, Fast Track Designation, and Priority Review, as well as prior experience adapting EMA PRIME materials for FDA use.

An independent QC medical writer, already familiar with the Biotech through prior DSUR work, provided an additional layer of review, strengthening document quality and ensuring seamless integration with the Biotech's broader regulatory portfolio.

3. Efficient Delivery Through Proactive Planning

Strong source documentation and early alignment enabled efficient drafting and review cycles. Proactive planning ensured the project remained on schedule and within budget, despite the inherent complexity of designation-level submissions.

OUTCOME

A Submission-Ready Breakthrough Therapy Designation

The finalized BTM document was delivered **on time** and **under budget** and is scheduled for inclusion in Module 1 of the Biotech's upcoming Investigational New Drug (IND) submission.

Beyond the immediate designation milestone, the clarity, structure, and strategic value of the submission laid the groundwork for an ongoing partnership. Morula Health has since supported the Biotech across a broad range of regulatory documents, including: Clinical CTD Modules 2.4 and 2.6, Nonclinical CTD Modules 2.5 and 2.7, Investigator's Brochure, Clinical Trial Protocols, DSURs.