

## Navigating Regulatory Landscapes

# PrimeVigilance's Strategic Approach to Establishing Post-Marketing Surveillance (PMS) in Japan for Advanced Therapies

Streamlining Compliance and Operational Excellence in the CGT/ATMP Sector

### Client Portfolio Overview



Phases I-III

#### 11 assets total

including *four approved products* (three biologics and one small molecule) and *seven products in various stages* of clinical trials (six gene therapy and one biologic)

#### Regions covered



USA/NA  
EU & UK  
LATAM  
APAC

## General Challenges in CGT/ATMP

| Rare Disease Focus                                                                                                                                                                                                                                                                                                                                                                                                             | Extended Safety Data Collection                                                                                                                                                                                                                                                                                                                                        | Challenges in SAE Collection                                                                                                                                                                                                                                                                                                                                                                      | High Regulatory Scrutiny                                                                                                                                                                                                                                                                                                                                                                                          | Long-Term Follow-Up Studies                                                                                                                                                                                                                                                                                                                                                                                  |
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| The <i>scarcity of patients</i> presents a significant commercial viability conundrum. The <i>potential market size</i> for these therapies becomes severely limited, raising concerns about their long-term sustainability. This limitation not only impacts the economic feasibility of developing and manufacturing such treatments but also complicates efforts to secure regulatory approval and negotiate reimbursement. | Due to small patient populations, the process of gathering sufficient safety data becomes prolonged. This <i>extended timeframe</i> poses a persistent challenge in <i>ensuring the adequacy of safety data</i> . This often necessitates prolonged observation periods to detect potential adverse effects, complicating the assessment of long-term safety profiles. | The variability in <i>treatment protocols</i> complicates the standardization of Serious Adverse Events (SAEs) collection methods across clinical trials and patient cohorts. Consequently, continuous updates and refinements to <i>evaluation methods are necessary until the 'final hour'</i> before regulatory submission to ensure comprehensive and accurate assessment of safety outcomes. | Due to the innovative nature and potential risks associated with these therapies, regulatory bodies subject ATMP/CGT trials to <i>intense scrutiny</i> throughout the development process. Because of this, there is a <i>greater risk of regulatory agencies imposing clinical holds on trials</i> , halting progress and requiring additional evidence or modifications to address safety or efficacy concerns. | Requirement for <i>mandatory long-term follow-up studies</i> , which may extend for periods exceeding 30 years post-treatment are necessary to comprehensively assess the long-term safety, efficacy, and durability of these innovative therapies. However, ensuring compliance and maintaining data integrity over such prolonged periods presents formidable <i>logistical and financial challenges</i> . |

## Case Study Challenge

Obtaining Marketing Authorization (MAH) approval in Japan for Advanced Therapy Medicinal Products (ATMPs) without a direct in-country presence and subsequently establishing PMS studies to comply with regulatory requirements.

## **PRIMEVIGILANCE** Input

### Onboarding of Japan Team

This involved recruiting individuals with expertise in Japanese pharmacovigilance regulations and practices, as well as providing comprehensive training on PrimeVigilance's processes and systems. By seamlessly integrating the Japan team within the global structure, PrimeVigilance ensured efficient communication, collaboration, and alignment of activities across regions.

### Development of Japanese-Specific Project Documents

PrimeVigilance spearheaded the development of Japanese-specific project documents in collaboration with EU, NA, and APAC teams. These documents were tailored to Japanese regulatory requirements in order to ensure consistency, compliance, and seamless execution of pharmacovigilance activities in Japan.

### Establishment of Argus-J Safety Database Module & Case Migration

This involved configuring the database to accommodate Japanese-specific data fields and regulatory reporting standards. PrimeVigilance also undertook the complex task of migrating existing case data from EU to APAC teams into the Argus-J module, ensuring seamless continuity of pharmacovigilance activities and adherence to regulatory timelines.

### Partnership with Japanese CRO for PMS Study Set-up

Understanding the significance of leveraging local expertise and resources, PrimeVigilance established a strategic partnership with a reputable Japanese CRO. This partnership facilitated the setup of PMS studies in Japan, leveraging the CRO's local expertise and resources. This partnership was managed by PrimeVigilance for the client.



## The Outcome

### Establishing PMS & Achieving Full Operational Capability for Japan Activities within 3 Months

Through PrimeVigilance's proactive approach and collaborative efforts, the client successfully established PMS activities and achieved full operational capability for Japan activities within a remarkably short timeframe of three months. This achievement not only ensured compliance with regulatory requirements but also underscored PrimeVigilance's dedication to providing efficient and customized pharmacovigilance solutions tailored to the CGT/ATMP sector's unique needs.