

Orphan Drug Development Guidebook

Building Block J303

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

| ITEM | DESCRIPTION |
|---------------------------|--|
| Building Block (BB) Title | PMDA consultations |
| References | <ul style="list-style-type: none"> • PMDA Profile of Services 2018-2019 <ul style="list-style-type: none"> – https://www.pmda.go.jp/files/000221139.pdf • PMDA Website: Consultations <ul style="list-style-type: none"> – https://www.pmda.go.jp/english/review-services/consultations/0002.html • PMDA Website: User Fees <ul style="list-style-type: none"> – https://www.pmda.go.jp/review-services/drug-reviews/user-fees/0001.html (Information of consultation fees, Japanese) |
| Description | <p>PMDA offers consultations to give guidance and advice on clinical trials of drugs, medical devices, and regenerative medical products as well as on data for regulatory submissions. For designated orphan drugs, PMDA offers Priority consultation.</p> <p>Fees of some main consultations for development of drugs are shown below. Consultations for medical devices and regenerative medical products have different fee tables.</p> |
| Category | Regulatory Building Block |

| ITEM | DESCRIPTION |
|------------------------------------|---|
| Geographical scope | Japan |
| Availability | Developers within Japan regardless of the applicant's nature (i.e., public/no-profit/academia, SME, etc). Priority consultation is offered to designated orphan drugs. |
| Scope of use | In clinical trial consultations for drugs, medical devices, and regenerative medical products, PMDA checks whether a proposed clinical trial complies with the requirements for regulatory submission, taking into consideration the ethical and scientific aspects and reliability of the clinical trial as well as the safety of trial subjects, and also gives advice that leads to an improvement in the quality of the clinical trial. |
| Stakeholders | <ul style="list-style-type: none"> • Drug/Medical device/Regenerative Medical Product developers • PMDA |
| Enablers/ Requirements | N/A |
| Output | Scientific advice with meeting minutes from PMDA |
| Best time to apply and time window | <p>Anytime during medical product development. Various types of consultations are available.</p> <p>Summary of applicant's procedure (It takes approximately three months from application to receiving minutes)</p> <ol style="list-style-type: none"> 1. Application of consultation 2. Submission of consultation materials 3. Receiving inquiry from PMDA and submission of response 4. Receiving preliminary opinion from PMDA and submission of response 5. Face-to-Face meeting with PMDA 6. Receiving the minutes of consultation from PMDA |

| ITEM | DESCRIPTION |
|-------------|--|
| Expert tips | <p>PROs: Provision from PMDA for appropriate CMC, preclinical and clinical development (i.e., tests and studies) required in the development of a drug/medical device/regenerative medical product is obtained. If the advices are followed or justified, this should minimize objections at the time of MAA evaluation which could delay or compromise the registration.</p> <p>CONs: N/A</p> |