Orphan Drug Development Guidebook

Milestone: MAA – NDA/BLA (registration)

If a drug developer has evidence from preclinical and clinical (phases I to III) research that a drug is safe and effective for its intended use, the applicant can file an application to market the drug to the relevant Regulatory Authority (i.e., FDA, EMA, MHLW/PMDA, etc). The relevant Regulatory Authority review team thoroughly examines all submitted data on the drug, decides if it is complete and makes a decision to approve or not to approve it.

In cases where the Regulatory Authority determines that a drug has been shown to be safe and effective for its intended use, it is then necessary to work with the applicant to develop and refine prescribing information. This is referred to as “labeling.” Labeling accurately and objectively describes the basis for approval and how best to use the drug.