Provisional Translation (as of October 2014) *

Utilization of Electronic Study Data: Summary Report of Pilot Project in the First Half of FY 2014

October 15, 2014

● Purpose

Through analyzing submitted clinical study data of new drugs for regulatory review using selected software with specific procedures, to confirm that the persons in charge in the Pharmaceuticals and Medical Devices Agency (PMDA) can obtain the analysis results necessary for regulatory review, and to consider the ways in which to utilize electronic data in the review process. In addition, to confirm that the submitted population pharmacokinetics (PPK) datasets of new drugs for regulatory review can be stored and managed appropriately in the PMDA system and that the persons in charge are able to conduct PPK analysis.

● Submission of clinical study data

- Based on the “Re: Request for Electronic Clinical Study Data for Pilot Project" (PMDA/CPE Notification No. 0327003, March 27, 2014), companies were requested to cooperate in the middle of April 2014 in the provision of available data. CDISC-compliant clinical study datasets for four drug products, one from each of four companies, and PPK datasets for three drug products, one from each of three companies, were submitted by June 2014.
- Before submission of data, interviews were conducted with the companies based on the information that was provided regarding the confirmation items about which PMDA had inquired in advance (target drug products considered for data submission, details of clinical studies and data, conformance to standards, etc.).
- Clinical study data, which was provided for the pilot project in FY 2013, was continuously utilized during this pilot project period if permission was obtained from the company providing the data.

● Details

The pilot project was conducted from June to August 2014 by about 180 reviewers, most of whom were persons in charge in the areas of clinical medicine, clinical pharmacology, and biostatistics, and select reviewers from each Office of New Drug,

including review directors; and about 20 staff from the Advanced Review with Electronic Data Promotion Group, including persons in charge of IT.

● Results and Future Action

More than half of the reviewers could reproduce the results described in CTD using the provided data. As the pilot confirmed the ability of a part of the reviewers to conduct their own data analyses to obtain information necessary for review, it is thought that there is merit in utilizing submitted clinical study data in regulatory review. In addition, no issues arose in the storage and management of PPK datasets, the confirmation of which was one of the purposes of this pilot project.

In future consideration of the review process, we plan to take into account the need to consider, at an early stage of regulatory review of each drug product, the role of data analysis in that particular review, due to the difference in significance of the various analysis results on regulatory review and necessity and timing of analysis according to the content of the each application, and due to the possibility of situations in which the reviews of multiple applications must be conducted in approximately the same timeline by one review team.

We plan to solve issues likely caused by commercial software specifications, such as through inquiring with vendors.

We will continue aiming to enrich various training options available to reviewers, such as through providing training regarding CDISC data standards and use of software, and in the upcoming pilot project planned for the latter half of FY 2014 plan to continue examining the process of regulatory review after the start of electronic study data submission.