Pharmaceuticals and Medical Devices Agency

Guidance on Patient Participation

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Pharmaceuticals and Medical Devices Agency
Patient Centricity Working Group

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Introduction

Pharmaceuticals, etc. aim to treat, diagnose, and prevent a patient's disease or complications, or to affect the structure or function of the body. The patient is a subject of treatment, diagnosis, and prevention with pharmaceuticals, etc., as well as is a central presence in drug development, etc., and has experience of various physical, mental, social, or economic burdens and symptoms. It is essential for the Pharmaceuticals and Medical Devices Agency (PMDA), the regulatory authority for pharmaceutical affairs in Japan, to collect, understand, and utilize voices of patients' etc. in its operations to realize the PMDA's "Patient First" approach. Furthermore, understanding the needs of patients could support the development of pharmaceuticals etc. based on the assumption of how they may be used after approval and for the promotion of post-marketing safety measures. In addition, by deepening the understanding of pharmaceutical administration, patients, etc. are expected to have merits, such as leading to the development of therapeutic agents, etc. that become the best option for them, benefitting both PMDA and patients.

However, most information that PMDA requires for its operations in conducting scientific consultations, approval reviews, and safety measures, etc. is collected through the Marketing Authorization Holder and pharmaceutical personnel (medical professionals). Hence, PMDA has limited opportunities to collect information about patient care and voices of patients, etc. directly. Even though, efforts have been initiated to receive and evaluate development requests at the study group on Unapproved and Off-label Drugs with High Medical Needs, as well as patient reports on adverse drug reactions, opportunities for PMDA to collect information directly from patients remain limited.

In recent years, efforts like patient-focused drug development and approval review have become internationally significant, and regulatory authorities in Europe and the United States are promoting the participation of patients in various meetings and formulation of related guidelines. In Japan, the Japan Pharmaceutical Manufacturers Association has developed a "Patient Centricity Guidebook," and the Japan Agency for Medical Research and Development (AMED) has produced the "Patient and Public Involvement (PPI) Guidebook," which has led to full-scale efforts by companies and academia in product development and clinical trials. Consequently, the movement toward patient participation is becoming active.

In order for PMDA to smoothly implement and promote patient participation in drug development, approval review, and safety measures, as well as contribute to patient

participation activities in Japan, this guidance has been developed as a guideline for PMDA and its staff to refer to. The specific measures described in this guidance are examples and not limited to them. This guidance will be revised as necessary depending on the status of patient participation activities.

Ⅰ. Overview

（1） Definition and philosophy of patient participation at PMDA

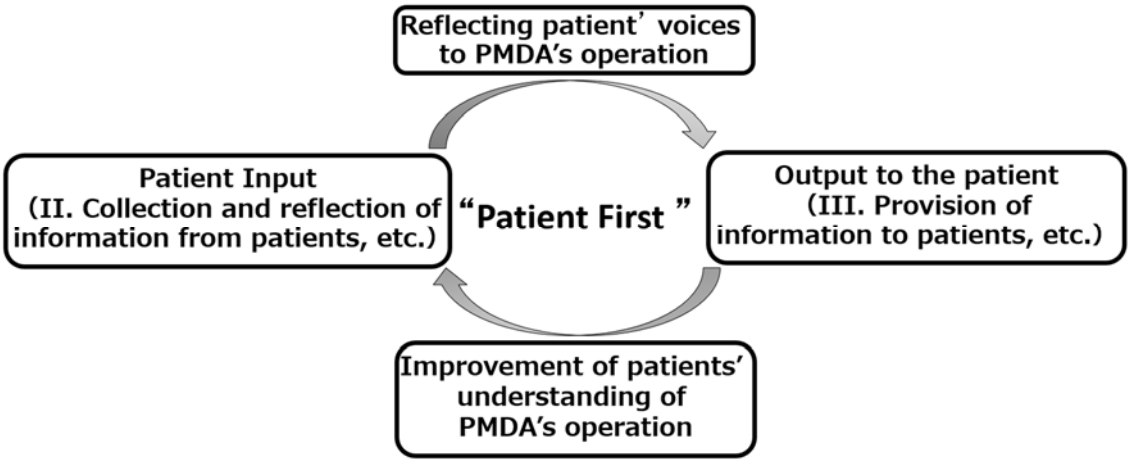
This guidance defines “patient participation” as identifying, understanding, and utilizing the voices of patients, etc. in business operations and aims to realize PMDA’s approach of “Patient First” through patient participation, as well as improve patients’ understanding and satisfaction with pharmaceutical administration.

（2） Purpose of this guidance

This guidance is prepared as a course of basic action for PMDA and its staff to promote patient participation in PMDA’s operations to achieve the above mentioned principles.

（3） Basic policy for promoting patient participation at PMDA

Through active collection of information on the voices of patients, etc., the quality of operations is enhanced. We will strive to enhance information provision for patients, etc., and improve their understanding of PMDA’s operations and pharmaceutical administration. Furthermore, we will promote patient participation by promoting the exchange of information shown in the figure below.



Ⅱ. Collection and reflection of information from patients, etc.

（1） Collection and reflection of information from patients, etc.

Overseas, the European Medicines Agency (EMA) established the Patients’ and

Consumers' Working Party (PCWP) to promote public health by providing information to patients about EMA's various activities and collecting and reflecting patients' voices in their operation. The U.S. Food and Drug Administration (FDA) has established the Clinical Trial Transformation Initiative (CTTI) and the Patient Engagement Collaborative (PEC) to engage patient representatives in discussions to achieve more meaningful patient participation in the development and regulation of pharmaceuticals, etc. at the FDA. In addition, the FDA has developed the Patient-Focused Drug Development (PFDD) guidance with patients and has published specific policies on collecting information from patients. Besides, the FDA is actively disclosing information by participating in meetings organized by academic societies.

The PMDA's work on approval review and safety measures, etc. is based on scientific discussions, and it is necessary to be aware of scientific validity when collecting and reflecting the voices of patients etc.

Regarding patient participation, the PMDA Information Security Policy and other related regulations shall be followed. Hence, PMDA will not provide sensitive information or trade secrets to patients, etc. or other external parties. On the other hand, when a patient, etc., is a member of the expert committee, the guidelines and procedures for commissioning stipulate that the patient, etc., shall not divulge secrets obtained through his/her duties or use them for his/her own or others' benefit, and this shall be observed.

（２） A framework for collecting the voices of patients, etc.

① Exchange of opinions and study sessions with patient groups

Exchanging opinions and holding study sessions with patient groups are useful opportunities to directly listen to the voices of patients, etc., and are actively implemented. Therefore, it is desirable to not only collect information but also provide information to patients, etc. described in Section III, as well as to conduct interactive information exchange.

When exchanging opinions and holding study sessions, it is necessary to consider a certain level of fairness and transparency, and appropriately control conflicts of interest, as it is required to have a broad range of participation based on the size of the patient group and the disease area.

② Consideration of participation of patients, etc. in meetings held by PMDA

If the participation of patients, etc. is deemed beneficial for discussion at the meeting, their participation is considered.

③ Information gathering through existing systems

The current framework for accepting information from patients, such as the study group on Unapproved and Off-label Drugs with High Medical Needs, the Study Meeting on early Introduction of Medical Devices of High Medical Need, and patient reports on adverse drug reactions, continues to be smoothly operated and is reviewed as appropriate to efficiently collect the voices of patients, etc.

（３） Responding to information obtained from patients at PMDA operations

① Responding to patient participation activities at the development stage

In the development of pharmaceuticals, etc., it is necessary to understand the needs and concerns of patients who are targets of pharmaceuticals, etc., and efforts to utilize the voices of patients, etc. are becoming active in the implementation of clinical trials from the planning stage. In the future, the number of applications for approval that includes the results of clinical trials in which pharmaceuticals, etc. developed based on the voices of patients, etc., and Patient-Reported Outcomes (PROs) as part of the evaluation items is expected to increase.

When patients, etc. participate in the consultation, on the part of consulter, the purpose is to obtain information that contributes to the scientific evaluation of the subject product.

The PROs is a useful tool for assessing patient benefits in the review process, and its use is contributing to the efficient development of pharmaceuticals, etc. whose clinical significance is supported by patients. When using PROs scales, it is important to ensure that they have been developed and validated per the scientific methods described in the guidelines. When interpreting the results of PROs scales, it is necessary to consider the clinical significance of the assessment using the scale. Therefore, when such data are submitted as part of the application material, their handling is examined, considering the status of implementation of language validation and the results of use in Japan and overseas, including the description in clinical evaluation guidelines.

② Responding to requests, etc. directly sent to PMDA

Efforts are made to refer to the contents of written requests received from patient groups, etc., if they contain information that contributes to scientific evaluation.

Consideration is given to utilizing the voices of patients, etc. received in the course of PMDA's regular operations for improvement within the framework of those operations.

Ⅲ. Provision of information to patients, etc.

（１） How to provide information to patients, etc.

To promote information collection activities from patients, etc. described in Section II, it is necessary to provide opportunities for patients to further their understanding of

PMDA's operations and pharmaceutical administration, as their basic understanding is a prerequisite. Therefore, consideration is given to providing appropriate feedback on the information collected through the activities described in Section II.

Considering the current situation that PMDA's direct experience with patient groups is limited, it is necessary to consider how to provide information that can be understood by the general public, besides the information provision activities that PMDA conducts as a regular operation.

As opportunities to provide information to patients, etc. are limited for PMDA, it is necessary to utilize various opportunities to promote understanding. For example, when exchanging opinions and conducting study sessions with patient groups, as described in Section II, it is useful to provide information on PMDA's operations and pharmaceutical administration.

（2） Information to be provided for patient participation activities

① Transmission of basic information related to pharmaceutical administration

The review process of pharmaceuticals, etc., including providing information on the PMDA website, ensures transparency and discloses information appropriately, as well as provide easy-to-understand information on adverse drug reactions and malfunction.

In addition, appropriate information is provided on the collection of information on adverse drug reactions and malfunction in post-marketing, epidemiological studies conducted by PMDA, and the dissemination of information related to safety and the basis for warnings.

Furthermore, information useful to patients, such as the Relief System from Adverse Drug Reactions, is widely provided using various forms of media.

② Active transmission of safety information, etc.

The content and dissemination methods of materials for patients, etc., such as the Patient Drug Guide provided on the PMDA website, are continually enhanced and improved to promote their use.

For information that needs to be delivered to patients, as well as medical professionals, timely, including safety issues, the method and content of dissemination (e.g., what actions should be taken when patients receive the information and who should be consulted) are considered.

Accordingly, cooperation from patient groups is obtained as deemed necessary, and information is disseminated in an easy-to-understand manner.

③ Other

Besides, the provision of various information is to accurately identify patient needs based on the information collected in Section II (2) and (3), and expand. When providing

such information to patients, etc., the effectiveness of the information should be evaluated through questionnaires whenever possible, which leads to subsequent improvements.

If there are cases of patient participation in each department, the details, specific and meaningful points, and issues, etc. are appropriately shared within PMDA and utilized for subsequent patient participation.

（３） Media used for information provision, etc.

① Enhancement of the PMDA website

The website is one of the principal media in PMDA's operations in that it can provide users with the information they need quickly, regardless of time or location. From time to time, the usability of the PMDA website is enhanced from the patient's perspective so that not only developers and medical professionals but also patients, etc. can easily obtain the information they need.

Other than the PMDA website, methods easily accessible to patients (e.g., social media [SNS, etc.], e-mail, video sites, and distribution via publications) are considered.

② Participation and holding of various events

PMDA attempts to publicize PMDA and its operations by actively participating in gatherings comprising patients and other participants, such as activities of patient groups and lectures for citizens at academic conferences.

PMDA continues to consider holding public symposiums and a way to disseminate information better.

③ Enhancement of various materials, etc.

An environment that facilitates the above-mentioned information provision activities is created by preparing easy-to-understand explanatory materials for patients, etc. and sharing them widely within PMDA.

④ Other

For information on specific diseases or information that needs to be delivered to patients timely, cooperate with patient groups, etc. in advance and consider distribution routes.

Ⅳ. Definition of terms

The definition of each term in this guidance is as follows:

Term	Definition
Pharmaceuticals, etc.	Pharmaceuticals, medical devices, regenerative medicine.
Patients, etc.	Patients subject to treatment, diagnosis, and prevention

	using pharmaceuticals, etc., their caregivers, and guardians. The subjects of vaccination and over-the-general use drugs are also expected.
Voices of patients, etc.	Information obtained from patients etc., such as voices, opinions, and requests.