Provisional Translation (as of April 2026)*

PSB/PED Notification No. 0227-9
February 27, 2026

To: Prefectural Health Departments (Bureaus)

From: Director, Pharmaceutical Evaluation Division, Pharmaceutical Safety Bureau,
Ministry of Health, Labour and Welfare
(Official seal omitted)

Partial Revision of "Handling of Designation of Drugs for Specific Use"

Handling of designation of drugs for specific use has been shown in "Handling of Designation of Drugs for Specific Use" (PSEHB/PED Notification No. 0831-5 dated August 31, 2020, by Director of Pharmaceutical Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare), etc.

Based on the Ministerial Ordinance on the Development of Related Ministerial Ordinances in Accordance with Enforcement of the Act Partially Amending the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (MHLW Ordinance No. 117 of 2025), etc., the handling of designation of drugs for specific use has been changed as shown in the attached old-and-new comparison table, and the decision has been made to apply the changes from the date of enforcement of the ministerial ordinance (May 1, 2026). Please be aware of this and fully inform relevant businesses in your jurisdiction.

The revised Notification by Director is attached for reference.

Old-and-new comparison table

(The underlined parts are the revised.)

After amendment	Before amendment
1. Requirements for designation Either of the following (1) or (2) applies. (1) Those for diagnosis, treatment, or prevention of pediatric diseases which meet all of the following (A) and (B) (including development related to addition of dosage forms to be used for children): (deleted) A (omitted) B Particularly excellent value for the intended usage Both of the following [1] and [2] should be met: [1] (omitted) [2] Existing treatment methods, etc. are insufficient and if marketing approval is granted, they can be treatment options with respect to their intended use. In particular, drugs that require pharmaceutical development of the dosage forms or specifications suitable for children, or those that are established as a standard of care, etc. by international guidelines etc., or have good evidence from the results of randomized comparative studies etc. will be positively designated as drugs for specific use. (2) Used for diagnosis, treatment, or prevention of diseases caused by drug-resistant pathogens, and the requirements A and B apply in the case of 1) or 2) 1) In case of drugs for drug-resistant pathogens (deleted) A (omitted) B (omitted)	1. Requirements for designation Either of the following (1) or (2) applies. (1) Those for diagnosis, treatment, or prevention of pediatric diseases which meet all of the following (A) to (C): A Any of the following developments for the intended use [1] Change in the dosage and administration [2] Additional dosage forms B (omitted) C Particularly excellent value for the intended usage Both of the following [1] and [2] should be met: [1] (omitted) [2] Established as a standard of care by international guidelines etc. or have good evidence from the results of randomized comparative studies etc. (2) Used for diagnosis, treatment, or prevention of diseases caused by drug-resistant pathogens, and all of the requirements A to C apply in the case of 1) or 2) 1) In case of drugs for drug-resistant pathogens A Either of the following developments [1] Change in the indication [2] Change in the dosage and administration B (omitted) C (omitted)

2) In case of drugs to prevent the occurrence of drug-resistant pathogen (deleted) A and B (omitted)	2) In case of drugs to prevent the occurrence of drug-resistant pathogen A Either of the following developments [1] Change in the dosage and administration [2] Change in the indication B and C (omitted)
(deleted)	2. Procedures to application for designation (1) The Ministry of Health, Labour and Welfare collects requests or proposals for the development of drug candidates falling under the above 1. (2) The Ministry of Health, Labour and Welfare requests Study Group on Unapproved and Off-label Drugs of High Medical Need (hereinafter referred to as "the Study Group") to assess the eligibility of the collected requests for development to drugs for specific use (hereinafter referred to as "the Eligibility Assessment"). (3) The Study Group informs the Ministry of Health, Labour and Welfare of the results of Eligibility Assessment. (4) The Ministry of Health, Labour and Welfare notifies the results of Eligibility Assessment by the Study Group to the marketing authorization holder of the requested drug. (5) If the marketing authorization holder receiving the notification in the above (4) from the Ministry of Health, Labour and Welfare wishes to have the drug designated as a drug for specific use, the marketing authorization holder should submit the application for designation of drugs for specific use to the Ministry of Health, Labour and Welfare.
2. Procedures of designation (1) Application for designation Those who intend to apply for the designation of drugs for specific use should submit Form 107-3 specified in the revised Regulation for Enforcement of the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Order of the Ministry of Health and Welfare No. 1 of February 1, 1961, hereinafter referred to as "the Regulations") by the Ministerial Ordinance on the Development of Related Ministerial Ordinances in Accordance with Enforcement of the Act Partially	3. Procedures of designation (1) Application for designation Those who intend to apply for the designation of drugs for specific use should submit Form 107-3 specified in the revised Regulation for Enforcement of the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Order of the Ministry of Health, Labour and Welfare No. 1 of February 1, 1961, hereinafter referred to as "the Regulations") by the Ministerial Ordinance on the Development of Related Ministerial Ordinances in Accordance with Enforcement of the Act Partially

Amending the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Ordinance of Ministry of Health, Labour and Welfare No. 155 of 2020, hereinafter referred to as "the Amendment Ordinance") together with the attachment in (2) to Pharmaceutical Evaluation Division, Pharmaceutical Safety Bureau, Ministry of Health, Labour and Welfare (hereinafter referred to as "Pharmaceutical Evaluation Division").	Amending the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Ordinance of Ministry of Health, Labour and Welfare No. 155 of 2020, hereinafter referred to as "the Amendment Ordinance") together with the attachment in (2) to Pharmaceutical Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare (hereinafter referred to as "Pharmaceutical Evaluation Division"). The applicant should mail or bring the data in paper form (one original, two copies) and in electronic form. Mail to: 1-2-2 Kasumigaseki, Chiyoda-ku, Tokyo 100-8916 Pharmaceutical Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare Attention to the person in charge of the designation system for drugs for specific use
(2) Attachment to the application form Article 251-2-2 of the Regulations revised by the Amendment Ordinance specifies the data to be attached to the application form. The below explains the specific details to be contained in the data: Submission of other data may be required as necessary. A Data on the satisfiability of needs for the intended use Data showing the eligibility for the above 1 and summary of the request for development and eligibility assessment in the above 2, if any B to D (omitted)	(2) Attachment to the application form Article 251-2-2 of the Regulations revised by the Amendment Ordinance specifies the data to be attached to the application form. The below explains the specific details to be contained in the data: Submission of other data may be required as necessary. A Data on the satisfiability of needs for the intended use Summary of the request for development and eligibility assessment in the above 2, B to D (omitted)
3. Method of designation Designation of the drug for specific use is made after hearing the opinions of Pharmaceutical Affairs Council. For the designated drug, the date of designation, name of drugs, target intended use and the name and addresses of the applicant are published on the website of the Ministry of Health, Labour and Welfare.	4. Method of designation Designation of the drug for specific use is made after hearing the opinions of Pharmaceutical Affairs and Food Sanitation Council. For the designated drug, the date of designation, name of drugs, target intended use and the name and addresses of the applicant are published on the website of the Ministry of Health, Labour and Welfare.
4. Consultation on designation When applying for the designation as a drug for specific use, consult the person in charge of	(new)

designation operations at the Pharmaceutical Evaluation Division, the Pharmaceutical Safety Bureau, the Ministry of Health, Labour and Welfare in advance.	
5. Application for designation in special cases In addition to the examination of eligibility for the consultations provided to applicants specified in "4. Consultation on designation," the Study Group on Unapproved and Off-label Drugs of High Medical Need (hereinafter referred to as the "Study Group") may assess the eligibility depending on the request for development as shown in (1) to (3) below. (1) The Ministry of Health, Labour and Welfare collects requests for the development of drug candidates falling under the above 1. (2) For a collected request for development related to drugs for specific use, if the company does not intend to conduct the development, the Ministry of Health, Labour and Welfare shall request the Study Group to assess the eligibility of the drug as a drug for specific use (hereinafter referred to as the "Eligibility Assessment"). (3) If the Study Group assesses the drug as a drug for specific use, the Ministry of Health, Labour and Welfare shall notify the company developing the requested drug of the result, and the company shall consider applying for designation based on the assessment.	(new)
6. Priority on designated drugs (1) (omitted) (2) Priority review Priority review is available when a drug is designated based on Article 14-7 of the Act.	5. Priority on designated drugs (1) (omitted) (2) Priority review Priority review is available when a drug is designated based on Article 14-8 of the Act.
7. (omitted)	6. (omitted)
8. Rescission of designation The Minister of Health, Labour and Welfare, when receiving a notification discontinuation under Article 77-5 of the Act, rescinds designation based on Article 77-6 (1) of the Act. Based on Article 77-6 (2) of the Act, the Minister of Health, Labour and Welfare may rescind the designation in cases that fall under any of the following items. A public announcement of the rescission of designation is made in accordance with the above 3.	7. Rescission of designation The Minister of Health, Labour and Welfare, when receiving a notification discontinuation under Article 77-5 of the Act, rescinds designation based on Article 77-6 (1) of the Act. Based on Article 77-6 (2) of the Act, the Minister of Health, Labour and Welfare may rescind the designation in cases that fall under any of the following items. A public announcement of the rescission of designation is made in accordance with the above 4.

A The requirement of the above 1 is no longer met due to the approval of other drugs etc. B to D (omitted)	A The requirement B or C of the above 1 is no longer met due to the approval of other drugs etc. B to D (omitted)
9. Succession If the designee transfers the right of development in Japan to others (hereinafter referred to as "successor"), the designee should notify discontinuation of study/research in accordance with the above 7, and the successor should submit the data in the above 2 (1) the application form and (2). However, if the contents of the documents have been changed since the time when the designee receives the designation, data that show that the changes satisfy the requirements at the time of succession should also be submitted. Succession is approved with the letter of designation that is separately issued. Designees who consider succession should consult with Pharmaceutical Evaluation Division in advance. In so doing, the designee should also submit a copy of the contract for succession and data that explain the background of succession.	8. Succession If the designee transfers the right of development in Japan to others (hereinafter referred to as "successor"), the designee should notify discontinuation of study/research in accordance with the above 6, and the successor should submit the data in the above 3 (1) the application form and (2) D. However, if the contents of the documents have been changed since the time when the designee receives the designation, data that show that the changes satisfy the requirements at the time of succession should also be submitted. Succession is approved with the letter of designation that is separately issued. Designees who consider succession should consult with Pharmaceutical Evaluation Division in advance. In so doing, the designee should also submit a copy of the contract for succession and data that explain the background of succession.
10. (omitted)	9. (omitted)
(deleted)	10. Other Categories subject to the designation of drugs for specific use shall be reviewed as necessary.

Reference
(full text after revision)

PSEHB/PED Notification No. 0831-5
August 31, 2020
[Partially revised] February 27, 2026

To: Prefectural Health Departments (Bureaus)

From: Director, Pharmaceutical Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare
(Official seal omitted)

Handling of Designation of Drugs for Specific Use

In association with the issuance of the Act Partially Amending the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Act No. 63 of 2019, hereinafter referred to as the "Amendment Act") on December 4, 2019, the designation system for drugs for specific use has been established for the purpose of contributing to the promotion of research and development of drugs with significant unmet medical needs, for instance, dosage and administration for pediatric patients have not been defined, etc. Handling of designation of drugs for specific use specified in Article 77-2 (3) of the revised Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Act No. 145 of 1960, hereinafter referred to as "the Act") by the Amendment Act has been specified as shown below. We ask that you understand and inform related parties and organizations under your administration.

Note

1. Requirements for designation

Either of the following (1) or (2) applies.

(1) Those for diagnosis, treatment, or prevention of pediatric diseases which meet all of the following (A) and (B) (including development related to addition of dosage forms to be used for children):

A Significantly unmet needs of the intended use

Any of the following applies:

[1] No existing therapy, preventive or diagnosis methods (hereinafter referred to as "therapy etc.") are available (including cases where there is no standard of care except medication and there is no drug with pediatric dosage and administration)

[2] Therapy, prevention or diagnosis methods with higher medical usefulness compared to existing therapy etc. are required from a point of view of efficacy and safety on pediatric patients and physical/mental burdens on patients or caregivers

B Particularly excellent value for the intended usage

Both of the following [1] and [2] should be met:

[1] Indicated for serious diseases or used for serious diseases in a supportive manner

[2] The drugs are insufficient as existing treatment methods, etc., and if marketing approval is granted, they can be treatment options with respect to their intended use. In particular, drugs that require pharmaceutical development of the dosage forms or specifications suitable for children, or those that are established as a standard of care, etc. by international guidelines etc., or have good evidence from the results of randomized comparative studies etc. will be positively designated as drugs for specific use.

(2) Used for diagnosis, treatment, or prevention of diseases caused by drug-resistant pathogens, and the requirements A and B apply in the case of 1) or 2)

1) In case of drugs for drug-resistant pathogens

A Significantly unmet needs of the intended use

Both of the following [1] and [2] should be met:

[1] Used for pathogens resistant (or may become resistant) to drugs primarily used at present

[2] Apart from the drugs primarily used at present, there are no drugs approved for diseases caused by the target pathogen

B Particularly excellent value for the intended usage

Both of the following [1] and [2] should be met:

[1] High needs for drugs from a comprehensive point of view such as the infectivity of the target drug-resistant pathogen and seriousness of diseases by the pathogen

[2] Established as a standard of care by international guidelines etc. or have good evidence from the results of randomized comparative studies etc.

2) In case of drugs to prevent the occurrence of drug-resistant pathogen

A Significantly unmet needs of the intended use

Either of the following [1] or [2] applies:

[1] Likely to induce drug resistance in the pathogen of the target disease if used at the approved dosage and administration

[2] Although it has been established as the standard of care in international guidelines etc., it is not indicated for the target disease.

B Particularly excellent value for the intended usage

Both of the following [1] and [2] should be met:

[1] High needs for drugs from a comprehensive point of view such as the expected infectivity of a pathogen and seriousness of diseases by the pathogen, if the drug-resistant pathogen occurs

[2] Established as a standard of care by international guidelines etc. or have good evidence from the results of randomized comparative studies etc.

2. Procedures of designation

(1) Application for designation

Those who intend to apply for the designation of drugs for specific use should submit Form 107-3 specified in the revised Regulation for Enforcement of the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Order of the Ministry of Health, Labour and Welfare No. 1 of February 1, 1961, hereinafter referred to as "the Regulations") by the Ministerial Ordinance on the Development of Related Ministerial Ordinances in Accordance with Enforcement of the Act Partially Amending the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Ordinance of Ministry of Health, Labour and Welfare No. 155 of 2020, hereinafter referred to as "the Amendment Ordinance") together with the attachment in (2) to Pharmaceutical Evaluation Division, Pharmaceutical Safety Bureau, Ministry of Health, Labour and Welfare (hereinafter referred to as "Pharmaceutical Evaluation Division").

(2) Attachment to the application form

Article 251-2-2 of the Regulations revised by the Amendment Ordinance

specifies the data to be attached to the application form. The below explains the specific details to be contained in the data: Submission of other data may be required as necessary.

A Data on the satisfiability of needs for the intended use

Data showing the eligibility for the above 1 and summary of the request for development and eligibility assessment in the above 2, if any

B Data serving as the rationale for the use of the drug concerned

Outline of data that are listed in Article 40-1 (i) of the Regulations revised by the Amendment Ordinance and available at the time of application filing

C Development plan

Document that explains the outline of the development plan such as planned investigation items and study period etc.

D Summary of the drug for specific use

Summary prepared in accordance with the Attachment Form 1 or Attachment Form 2 as explanation documents for the committee and documents for publication (the name of applicant and the product name as well as the target indication [or proposed indication] should accompany their English name and translation)

3. Method of designation

Designation of the drug for specific use is made after hearing the opinions of Pharmaceutical Affairs Council.

For the designated drug, the date of designation, name of drugs, target intended use and the name and addresses of the applicant are published on the website of the Ministry of Health, Labour and Welfare.

4. Consultation on designation

When applying for the designation as a drug for specific use, consult the person in charge of designation operations at the Pharmaceutical Evaluation Division, the Pharmaceutical Safety Bureau, the Ministry of Health, Labour and Welfare in advance.

5. Application for designation in special cases

In addition to the examination of eligibility for the consultations provided to applicants specified in "4. Consultation on designation," the Study Group on Unapproved and Off-label Drugs of High Medical Need (hereinafter referred to as the "Study Group") may assess the eligibility depending on the request for development as shown in (1) to (3) below.

(1) The Ministry of Health, Labour and Welfare collects requests for the development of drug candidates falling under the above 1.

(2) For a collected request for development related to drugs for specific use, if the company does not intend to conduct the development, the Ministry of Health, Labour and Welfare shall request the Study Group to assess the eligibility of the drug as a drug for specific use (hereinafter referred to as the "Eligibility Assessment").

(3) If the Study Group assesses the drug as a drug for specific use, the Ministry of Health, Labour and Welfare shall notify the company developing the requested drug of the result, and the company shall consider applying for designation based on the assessment.

6. Priority on designated drugs

(1) Priority consultation

This allows a drug to be prioritized over other drugs in consultations by the Pharmaceuticals and Medical Devices Agency.

(2) Priority review

Priority review is available when a drug is designated based on Article 14-7 of the Act.

7. Discontinuation of study/research

When a person designated (hereinafter referred to as "designee") pursuant to the provisions of Article 77-2 (3) of the Act intends to discontinue test and research or manufacturing of the drug for specific use that pertains to the designation, they must promptly notify the Minister of Health, Labour and Welfare thereof based on Article 77-5 of the Act.

As a notification of discontinuation, Form No. 108 of the Enforcement Regulation should be submitted.

8. Rescission of designation

The Minister of Health, Labour and Welfare, when receiving a notification discontinuation under Article 77-5 of the Act, rescinds designation based on Article 77-6 (1) of the Act. Based on Article 77-6 (2) of the Act, the Minister of Health, Labour and Welfare may rescind the designation in cases that fall under any of the following items.

A public announcement of the rescission of designation is made in accordance with

the above 3.

A The requirement of the above 1 is no longer met due to the approval of other drugs etc.

B When unlawfulness such as false descriptions in the application for designation is found.

C When no test and research or marketing is provided for the drug for specific use without any legitimate grounds.

D When the designee violates the Act or other pharmaceutical laws and regulations specified by Cabinet Order, or any of the dispositions thereupon.

9. Succession

If the designee transfers the right of development in Japan to others (hereinafter referred to as "successor"), the designee should notify discontinuation of study/research in accordance with the above 7, and the successor should submit the data in the above 2 (1) the application form and (2). However, if the contents of the documents have been changed since the time when the designee receives the designation, data that show that the changes satisfy the requirements at the time of succession should also be submitted. Succession is approved with the letter of designation that is separately issued.

Designees who consider succession should consult with Pharmaceutical Evaluation Division in advance. In so doing, the designee should also submit a copy of the contract for succession and data that explain the background of succession.

10. Date of Enforcement

This notification shall be put into force on September 1, 2020.

Provisional Translation (as of April 2026)*
Attachment Form 1

Summary of Drug for Specific Use
(Those for diagnosis, treatment, or prevention of pediatric diseases)

Name of applicant		This should be accompanied with the English translation.
Name	Japanese accepted names	This should be accompanied with its English name such as INN.
	Brand name	This should be accompanied with the English translation.
Target indication		This should be accompanied with the English translation.
Proposed dosage and administration		
Target disease		Outline of the target disease and status of needs for the intended use should be explained.
Particularly excellent value for usage		Summary of particularly excellent value for usage should be explained.

(Note) 1 The forms should be in A4 size
2 If a further explanation is required for the details, it may be described in an attachment
3 This should be prepared with the understanding that this will be used for publication

Attachment Form 2

Summary of Drug for Specific Use

(Those used for diagnosis, treatment, or prevention of diseases caused by drug-resistant pathogens)

Name of applicant		This should be accompanied with the English translation.
Name	Japanese accepted names	This should be accompanied with its English name such as INN.
	Brand name	This should be accompanied with the English translation.
Target of development		□ Drugs for drug-resistant pathogens □ Drugs to prevent the occurrence of drug-resistant pathogen
Indication		□ With no change in the indication (Target indication: [to be accompanied with the English translation]) □ With changes in the indication (Proposed indication: [to be accompanied with the English translation])
Proposed dosage and administration		
Target disease		Outline of the target disease and status of needs for the intended use should be explained.
Particularly excellent value for usage		Summary of particularly excellent value for usage should be explained.

(Note) 1 The forms should be in A4 size
2 If a further explanation is required for the details, it may be described in an attachment
3 This should be prepared with the understanding that this will be used for publication