Questions and Answers on Clinical Trial Consultations for Pediatric Drug Development

PMDA Pediatric Drug Working Group
October 23, 2026

Q1: We would like to share information and exchange views with the Pharmaceuticals and Medical Devices Agency (PMDA) on pediatric development strategies that use approaches such as modeling and simulation (M&S) or pediatric extrapolation. We understand that the acceptability and appropriateness of these strategies need to be discussed in a formal scientific consultation. What meeting options are available for an initial exchange of information and views?

A1: Information can be shared and views exchanged at a pre-consultation meeting for a product-specific development strategy, or at a drug development pipeline meeting for discussions that are not specific to an individual product.

If, following such a meeting, you wish to discuss the strategy on the basis of detailed data, a formal scientific consultation is required. Please use a pre-consultation meeting to discuss which formal scientific consultation category would be appropriate.

Q2: We are preparing an M&S plan for pediatric drug development and intend to seek a formal scientific consultation. If we reach agreement with PMDA during that consultation on a pediatric development plan that does not involve pediatric clinical trials either in Japan or overseas, can we still obtain PMDA confirmation under the notification entitled "Planning of the Pediatric Drug Development Program during Development of Drugs for Adults" (PSB/PED Notification No. 0112-3, dated January 12, 2024, last amended on February 27, 2026; hereinafter, the "2024 Notification")?

A2: As stated in the 2024 Notification and its accompanying Q&A, if agreement is reached during the formal scientific consultation on a clinical data package based on the M&S plan, with no pediatric clinical trials to be conducted either in Japan or overseas, PMDA may state in the consultation record that the agreed content has been confirmed under the 2024 Notification. This can be done at the applicant's request.

Q3: Q&A 14 of "Revision of the Q&A for 'Planning of the Pediatric Drug Development Program during Development of Drugs for Adults'" (Administrative Notice, dated February 27, 2026) explains how to handle substantial changes to a pediatric drug development plan after PMDA scientific confirmation. It states that obtaining confirmation again is not mandatory, however, sponsors may still seek confirmation from PMDA. Which consultation category should we use to seek technical and strategic advice on a revised plan?

A3: Technical and strategic discussions on changes to a development plan should take place in an appropriate formal scientific consultation based on the stage of development, rather than in a "Consultation on Confirmation of the Pediatric Drug Development Program." However, you may first share information at a pre-consultation meeting. You may also use a pre-consultation meeting if you are unsure which consultation category is appropriate.

* This English document has been prepared for reference purposes only. In the event of any inconsistency and discrepancy between the Japanese original and the English translation, the Japanese text shall prevail.

If a formal scientific consultation has already taken place, such as a "Consultation after completion of phase II study for drugs," the "Additional scientific consultation for drugs" category may be available. Please also refer to Q6.

Q4: How should we seek scientific advice on the overall pediatric drug development strategy at an early stage of development, or inquire about eligibility for regulatory support schemes such as priority review?

A4: To obtain scientific advice on the overall pediatric drug development strategy, please clearly define the issues you wish to discuss and request a formal scientific consultation with PMDA. If you are unsure which consultation category is appropriate, please discuss this at a pre-consultation meeting.

Various designation schemes, notably those for orphan drugs and drugs for specific use, provide access to priority consultations, priority review, and other support for pediatric drug development. Please review the eligibility criteria for the relevant scheme. For inquiries about eligibility for orphan drug designation or designation as a drug for specific use, please contact the Pharmaceutical Evaluation Division, Pharmaceutical Safety Bureau, Ministry of Health, Labour and Welfare (MHLW), which serves as the contact point for these schemes.

For other designation schemes for which PMDA has established dedicated consultation categories, please use a pre-consultation meeting to discuss how to access the relevant consultation and how best to proceed.

Q5: We plan to conduct separate clinical trials in adults and children for a disease or condition that is the same or similar in both populations, and to request formal scientific consultations at the same time. Do we need to submit a separate consultation application for each population, or can both development plans be discussed together in a single formal scientific consultation?

A5: Depending on the proposed consultation topics, it may be possible to discuss the adult and pediatric development plans together in a single formal scientific consultation. For example, this may be possible when the clinical trial designs and key issues are similar in both populations.

However, if the trial designs or key issues differ substantially between adults and children, the range of topics may be too broad to discuss adequately within the available time. In such cases, a single formal scientific consultation may not be feasible.

You must first request a pre-consultation meeting, provide information on both the adult and pediatric development plans, and discuss whether the proposed questions can be addressed together in a single formal scientific consultation.

Q6: When pediatric clinical trials are conducted sequentially in different age groups (for example, children aged 12 years and older, followed by those aged 6 to less than 12 years), the trial plans are developed at different times. If the overall pediatric drug development plan has already been confirmed under the 2024 Notification, can we use the "Additional scientific consultation for drugs" category for a subsequent formal scientific consultation, treating development in different age groups as a single, ongoing pediatric development program?

A6: The "Additional scientific consultation for drugs" category may be available for a subsequent formal scientific consultation on a clinical trial plan for another pediatric age group if all of the following conditions are met:

- A formal scientific consultation has already taken place on pediatric development in a particular age group, as in the example above.
- During that consultation, the applicant indicated an intention to pursue pediatric development in another age group, although the detailed plan for that age group had not yet been developed.
- PMDA has already confirmed the overall pediatric development plan under the 2024 Notification, as documented in a consultation record, review report, or other relevant document.

In addition, as a time-limited pilot initiative, the "Additional scientific consultation for drugs" category may be available for a formal scientific consultation on a pediatric clinical trial plan within the pediatric development plan already confirmed under the 2024 Notification, if all of the following conditions are met:

- A formal scientific consultation has already taken place regarding a confirmatory trial in adults, during which the applicant indicated an intention to pursue pediatric development for the same indication being developed in adults.
- The pediatric drug development plan has already been confirmed under the 2024 Notification.

However, the "Additional scientific consultation for drugs" category may not be available if the clinical trial designs (such as development strategies using M&S), disease characteristics, or issues to be discussed differ substantially between age groups. You must therefore use a pre-consultation meeting to discuss whether this category can be used.

When submitting the request form for scheduling the formal scientific consultation under this category, enter the reference number and category of the original consultation in the "Consultation category" field. In addition, enter in the "Remarks" field the date of the pre-consultation meeting at which use of the "Additional scientific consultation for drugs" category was discussed.