外資系企業における開発品目の傾向
～ PhRMA / EFPIA Japan 合同調査結果より ～

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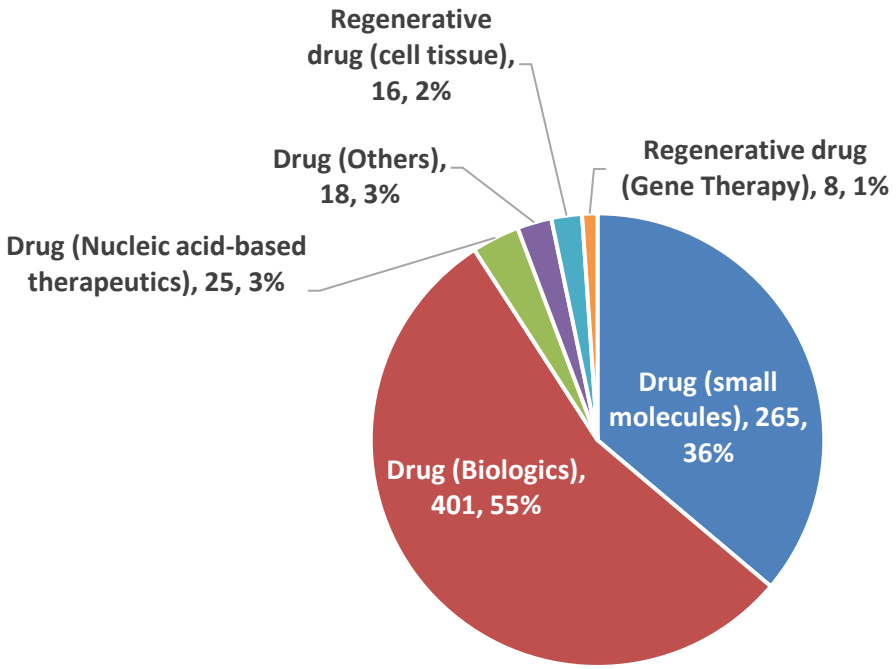
PhRMA/ EFPIA Japanで実施した2024年度の合同調査結果は以下のとおりであった。

- 2024年度は24社から733件のプロジェクトの回答が得られた。プロジェクトの申請区分で最も割合が高かったものは、医薬品では新有効性成分で48%（339件）、再生医療等製品では新効能再生医療等製品で50%（12件）であった。疾患領域では抗悪性腫瘍薬が最も多く、約半数を占めていた。
- 先駆的医薬品等指定制度、特定用途医薬品等、及び条件付き承認制度の利用割合は、検討中も含めてそれぞれ4.1%、0.8%及び1.8%と低かった。一方、希少疾病用医薬品等の指定制度の利用割合は、検討中も含めて31%と他の迅速審査制度よりも高い状況であった。抗悪性腫瘍薬の迅速審査制度の利用状況では、米国又は欧州で迅速審査制度を利用予定の149件（42%）のプロジェクトのうち、日本で迅速審査制度の利用予定があるものは74件と約半数にとどまっていた。
- 小児開発については、成人のみ、又は成人と青少年のみを対象としたプロジェクト633件のうち、95件（15%）は海外で小児開発を計画しており、そのうち45件は日本でも小児開発をする計画であった。
- 開発品目の申請データパッケージにリアルワールドデータの活用を検討しているプロジェクトは、1.1%（8件）と低い割合であった。
- 初回申請を予定している新有効成分のプロジェクト318件のうち、第II相又は第III相から国際共同治験に参加したプロジェクトは156件で、約半数を占めていた。このうち、海外第I相試験とは別で日本人での第I相試験を実施した割合は71%（111件）であり、その半数（56件）の実施時期は国際共同治験の参加前であった。一方、別途日本人第I相試験を実施しなかった29%（45件）のプロジェクトのうち、38%（17件）では参加した国際共同治験で日本人特有の安全性対策が講じられていた。なお、全プロジェクトの治験実施数は865件であり、そのうち国際共同治験が88%と大部分を占めるものの、一定数の国内試験も実施されている状況であった。
- 日本で2026年3月までに申請予定のプロジェクト（124件、17%）のうち、世界最初の申請から3ヵ月以内を予定しているものは61%であり、3ヵ月以内の申請が困難となる最も多い理由は日本特有の規制要件（治験相談による助言、追加の臨床試験等）によるものであった。
- ドラッグ・ロスの評価として、2024年度に米国又は欧州のいずれかで承認された新有効成分は40品目であった。そのうち11品目は日本での開発計画の情報が提供されなかったため、ドラッグ・ロスの可能性を完全に評価することはできなかった。回答が得られた29品目のうち、3品目は日本で開発予定がなく、ドラッグ・ロスとなる可能性が示唆された。

Total Projects in FY2024

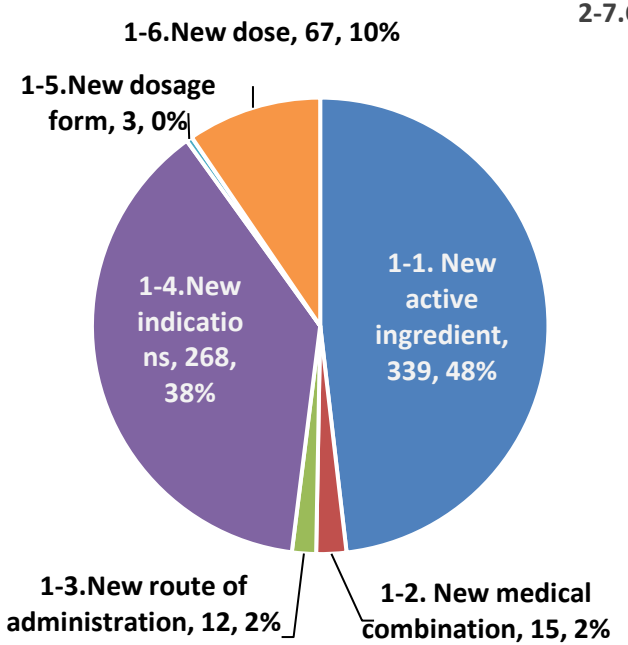
Survey Respondents 733 projects

Projects modality category

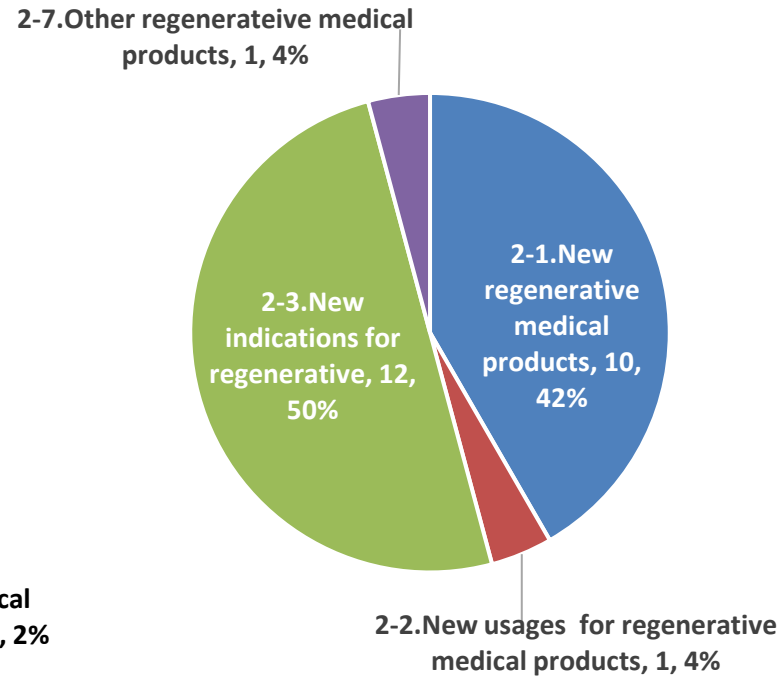


Filing category

Drug



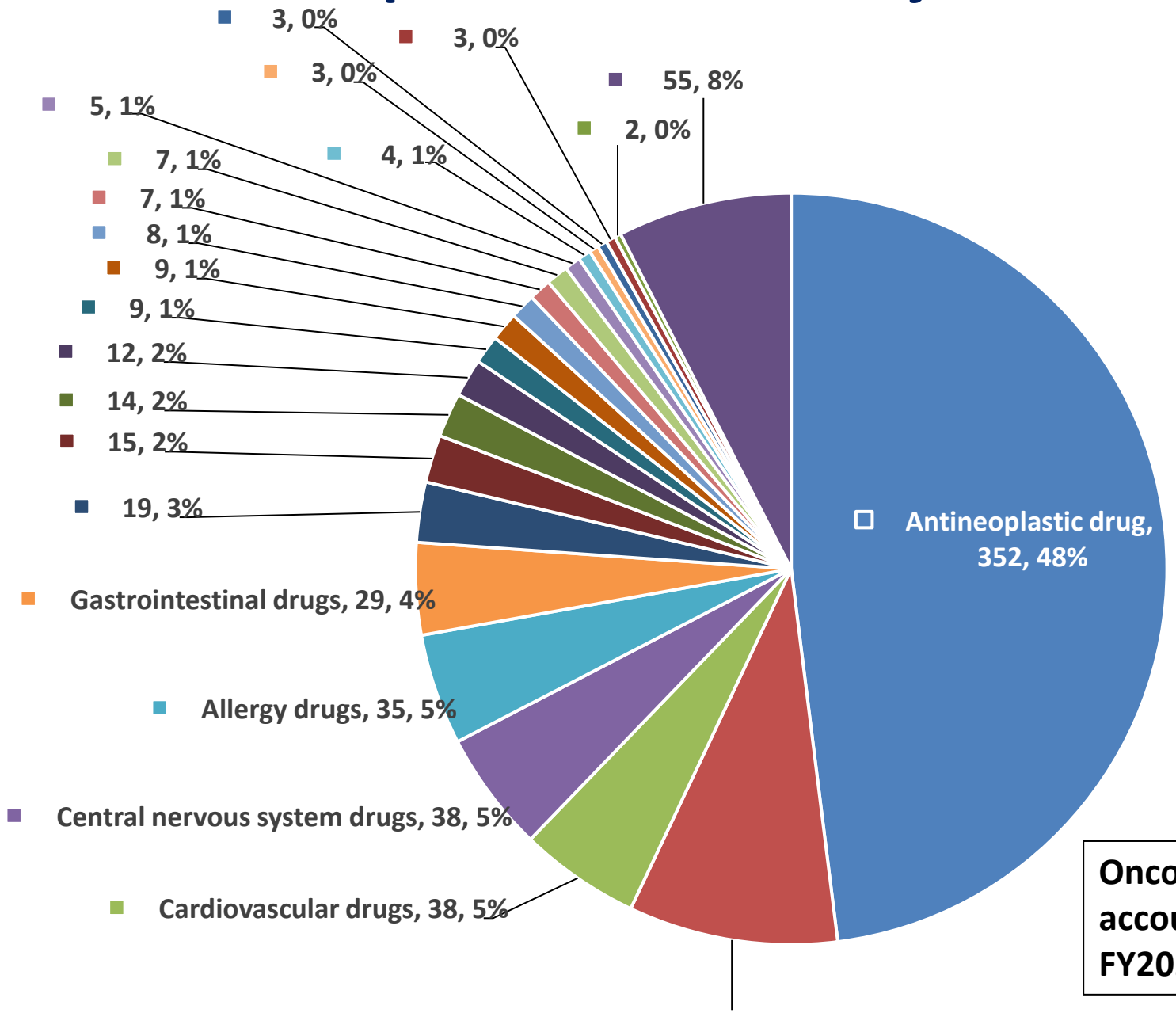
Regenerative medicine



- **In FY2024, the rate of drugs and regenerative medical products were 97% (n=709) and 3% (n=24), respectively. The majority were small molecules and biologics of drugs, but there are a certain number of new modality development (nucleic acid drugs and regenerative medical products); 6% (n=49).**
- **Filling category for both drug and regenerative medicine are mostly new active ingredient/products and new indications.**

Therapeutic Area for Projects in FY2024

Survey Respondents 733 projects



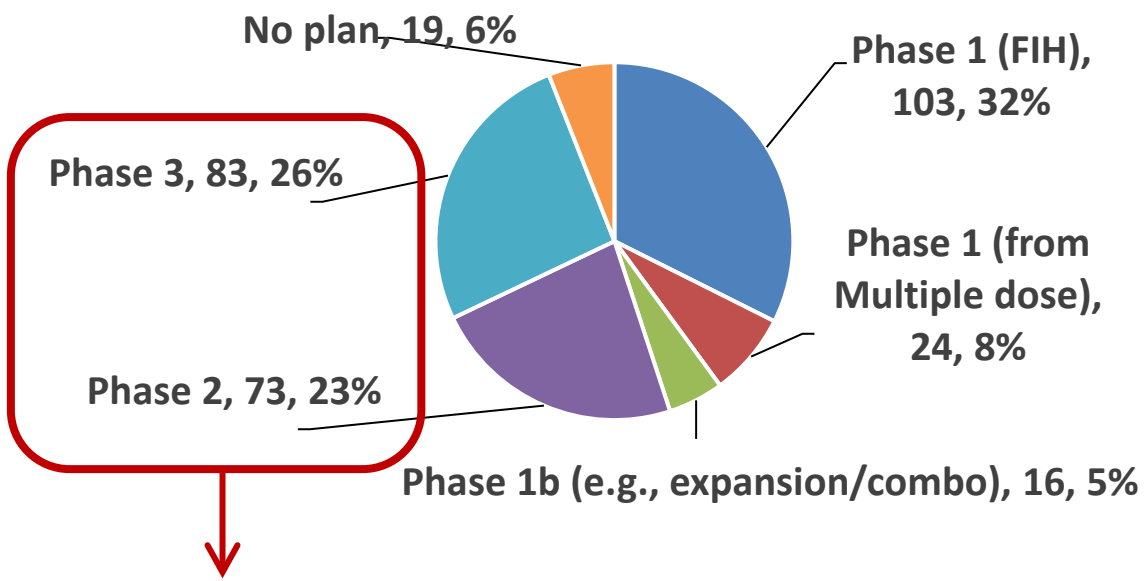
- Respiratory organ drugs **19, 3%**
- Alzheimer's disease drugs **15, 2%**
- Sensory organ drugs (related to inflammatory diseases) **14, 2%**
- Vaccines **12, 2%**
- Immunosuppressants **9, 1%**
- Sensory organ drugs (Excluding those related to inflammatory diseases) **9, 1%**
- Blood products **8, 1%**
- Peripheral nerve drugs **7, 1%**
- Antiviral agents **7, 1%**
- Medicines for urogenital and anal organs **5, 1%**
- Radiopharmaceuticals **4, 1%**
- Anti-Parkinson drugs **3**
- Antibacterial agents **3**
- HIV infection treatment drugs **3**
- Hormone drugs **2**
- Others **55, 8%**

Oncology (Antineoplastic drug) is a major focus area accounting for 48%(n=352) of the total projects in FY2024.

Implementation of Japanese Phase 1 study (New active ingredients for first application)

N = 318 projects

Timing of Japanese participating in Global study



Japanese Phase 1 separate from Global Phase 1

	Yes	No
Phase 2 (n=73)	52	21
Phase 3 (n=83)	59	24
Total (n=156)	**111 (71%)**	**45 (29%)**

Timing of Japan-specific Phase 1 (n=111)

	PMDA request	Company decision	Other	Total
Before Global study	5	51		56 (50%)
Parallel with Global study	1	52		53 (48%)
No Global study			2	2 (2%)
Total	6	103	2	111

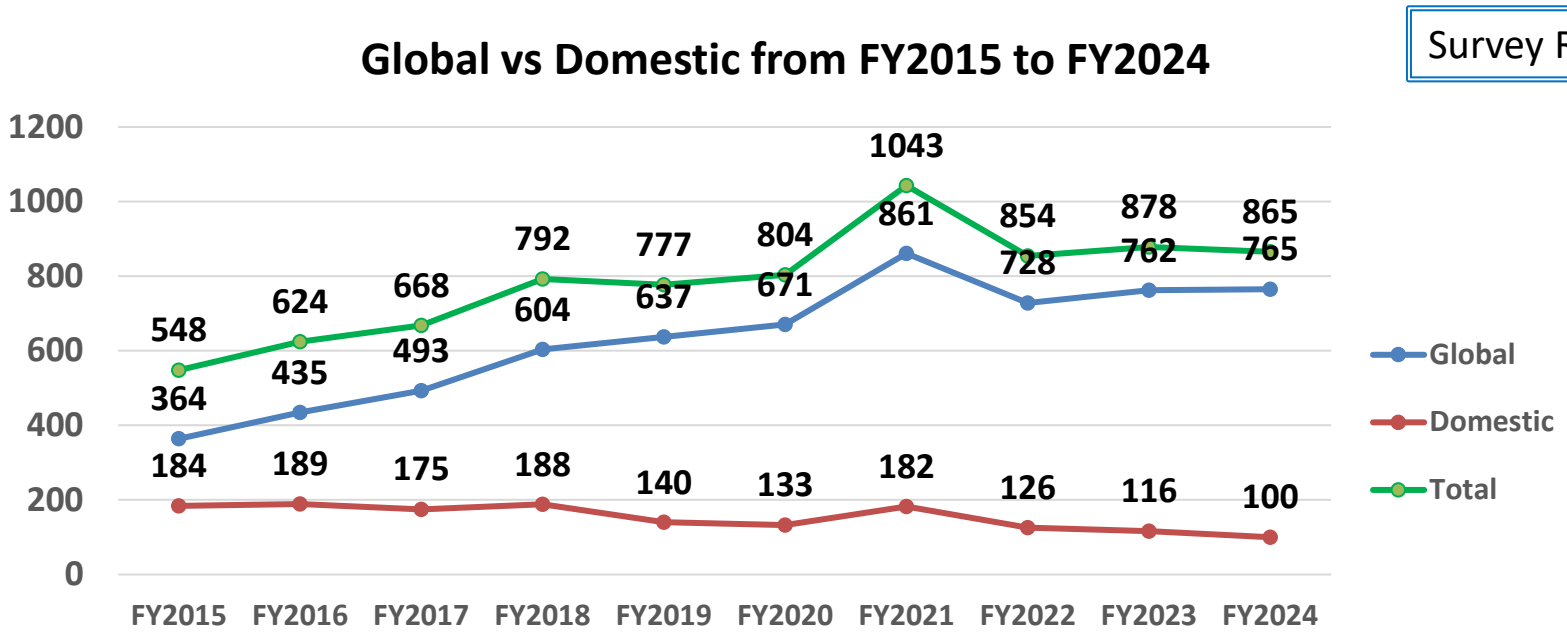
Japan-specific Safety Measures in Global study (n=45)

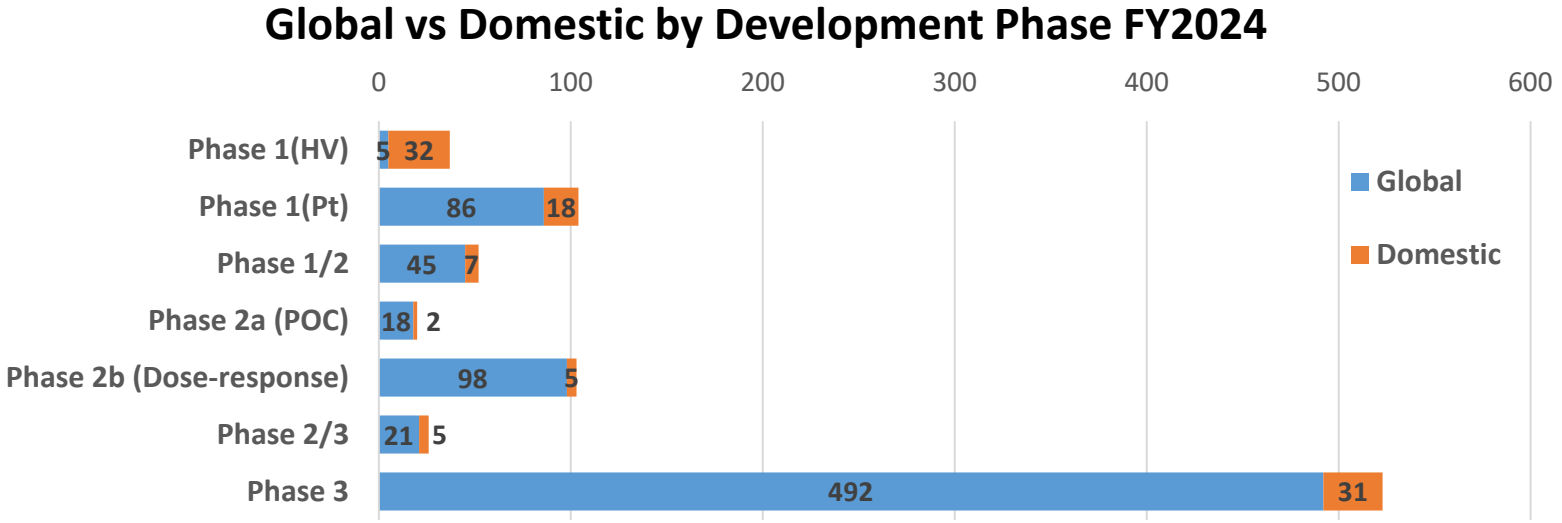
		Consulted with PMDA	Not consulted PMDA	Others	Total
No	Company decision	14	1		15 (33%)
Yes	Company decision	5	3		17 (38%)
	PMDA request	8	1		
Others	Others		4	9	13 (29%)
Total		27	9	9	45

156 projects (49%) joined Global study from Phase 2 or 3. Of these, 111 (71%) ran Japan-specific Phase 1.
Of the 45 (29%) that didn't, 17 (38%) had Japan-specific safety measures in Global study.

Number of Clinical Studies (Global / Domestic)

Survey Respondents 865 studies

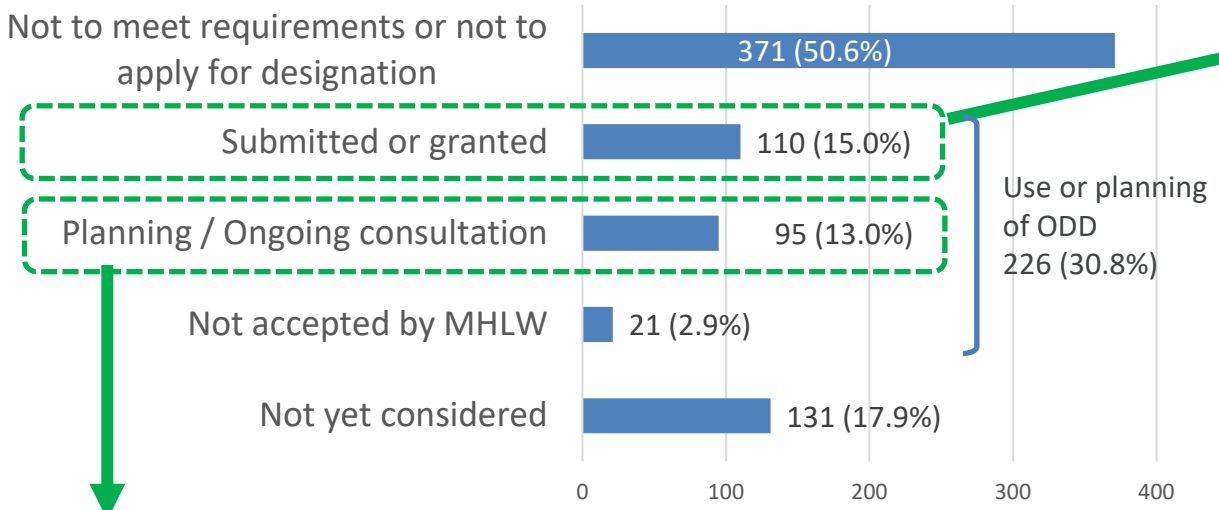




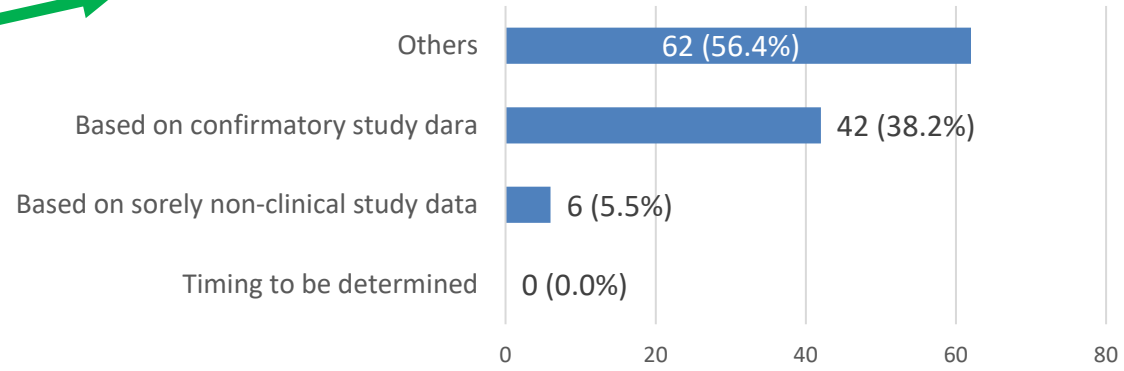
- **The total number of ongoing clinical studies was 865 and the ratio of Global studies was 88% in FY2024.**
- **The most common clinical study was in Phase 3 study, although there are some domestic studies in Phase 1 and 3.**

Plan for Orphan Drug Designation (ODD)

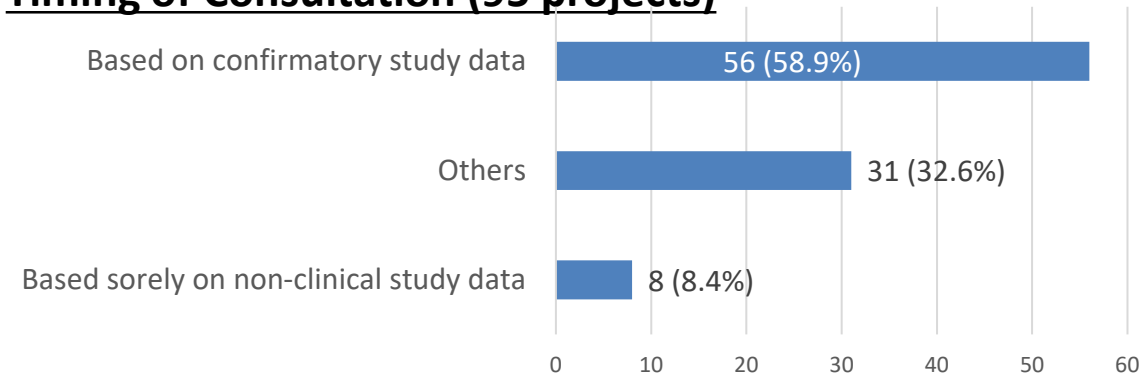
Survey Respondents 733 projects



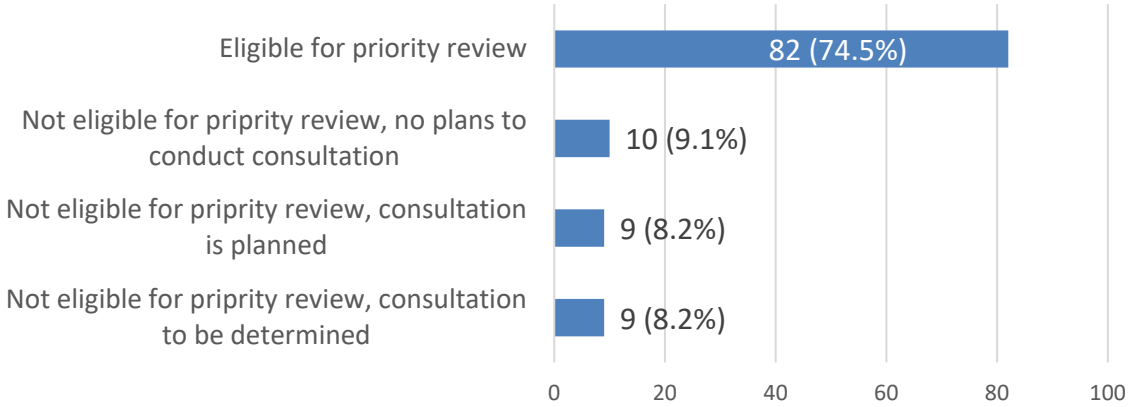
Timing of Application (110 projects)



Timing of Consultation (95 projects)



Priority Review Eligibility Status (110 projects)

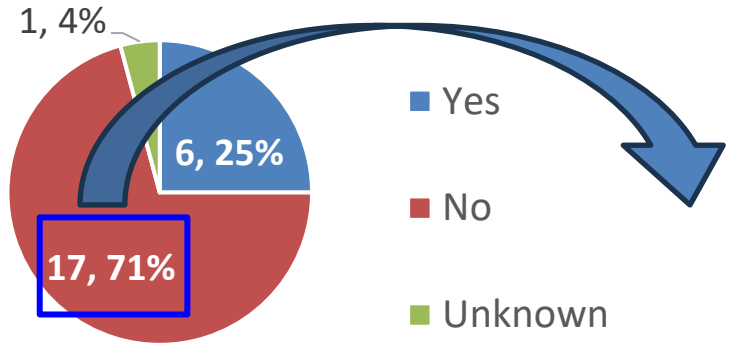


- The utilization status of the orphan designation, including those under consideration, was 30.8%.
- More than half were designated consultations based on confirmatory study data. Of the 31 projects categorized as 'other', 7 were Phase 1 trial and 14 were Phase 2 trial, including Proof-of-Concept (POC) trial. These consultations were sought without waiting for the results of confirmatory studies.
- The most common entries were designated applications based on confirmatory study data. Of the 62 projects categorized as 'other', 20 were Phase 1 trial and 24 were Phase 2 trial. There was a tendency to seek designated applications without waiting for confirmatory study results.
- More than 70% of the projects were granted ODD with priority review. 9 projects were planned to utilize the priority review designation consultation after granted ODD without priority review.

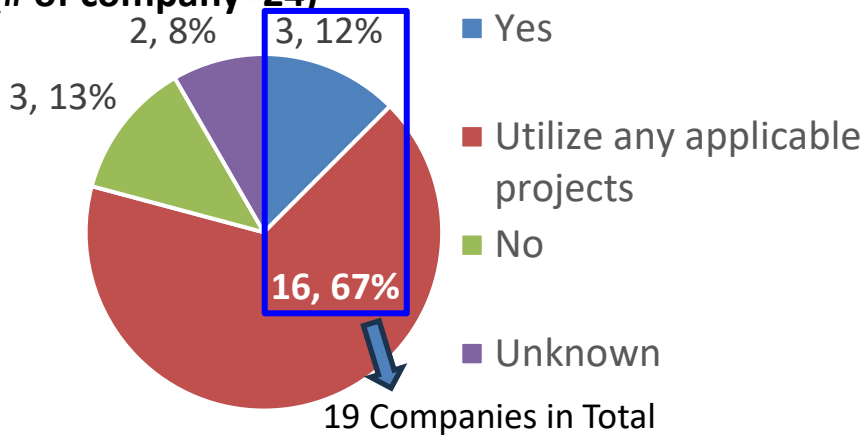
Consultation on eligibility for priority review of Orphan Drug Designation (ODD)

(1 answer from each company)

Experience in the consultation on eligibility for priority review of orphan drug (# of company=24)



Future plan to utilize the consultation (# of company=24)



The reasons for No Experience

From the 17 applicable companies, # is companies's number, multiple selection is acceptable

No applicable project (13), No applicable clinical data is available so far (3) Company gave up applying priority review (1), Company concluded regular review is enough(1), Additional time is required (1), Additional cost required (1)

Issues/problems with the consultation, request for improvement (Free text)

Issues/Problems

- Risk of delay in application schedule due to consultation and risk of attenuation of merit of priority review in association with that
- Criteria for priority review are not clear
- Cost-effectiveness unclear

Request for improvement

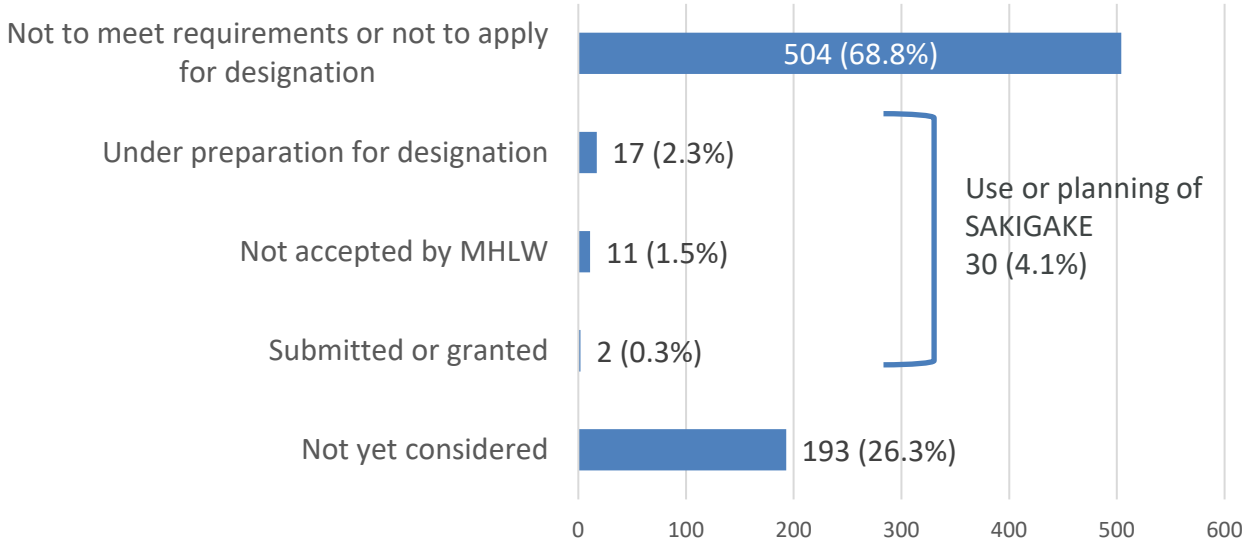
- Should be clarify what needs to be demonstrated
- Need to prepare a Q & A especially for the cases that cannot be identified from the notification(e.g. oncology drugs, etc.)
- Should be ensured all ODD are subject to priority review as soon as possible

Of the 24 companies, 6 had experience in consultation on eligibility for priority review. The most common reason for lack of experience was no applicable project (13 out of 17 companies). 19 out of 24 companies answered that they would utilize the consultation if there is any applicable project in the future. With regard to issues/problems, respondents noted concerns that consultations could delay the application schedule and unclear criteria for eligibility for priority review. Proposed improvements for MHLW/PMDA included preparing a Q&A and granting priority review to all ODD were mentioned.

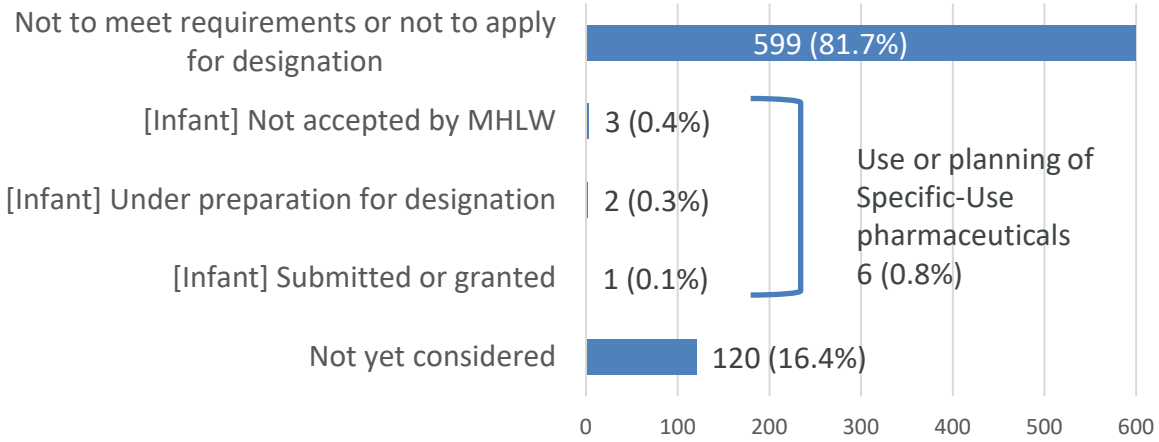
Plan for SAKIGAKE and Specific-Use pharmaceuticals

Survey Respondents 733 projects

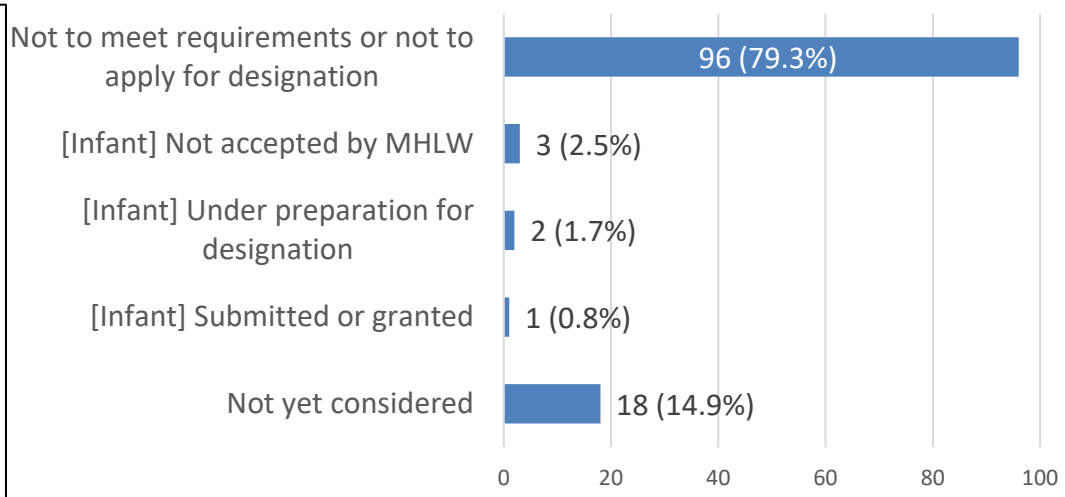
SAKIGAKE



Specific-Use pharmaceuticals



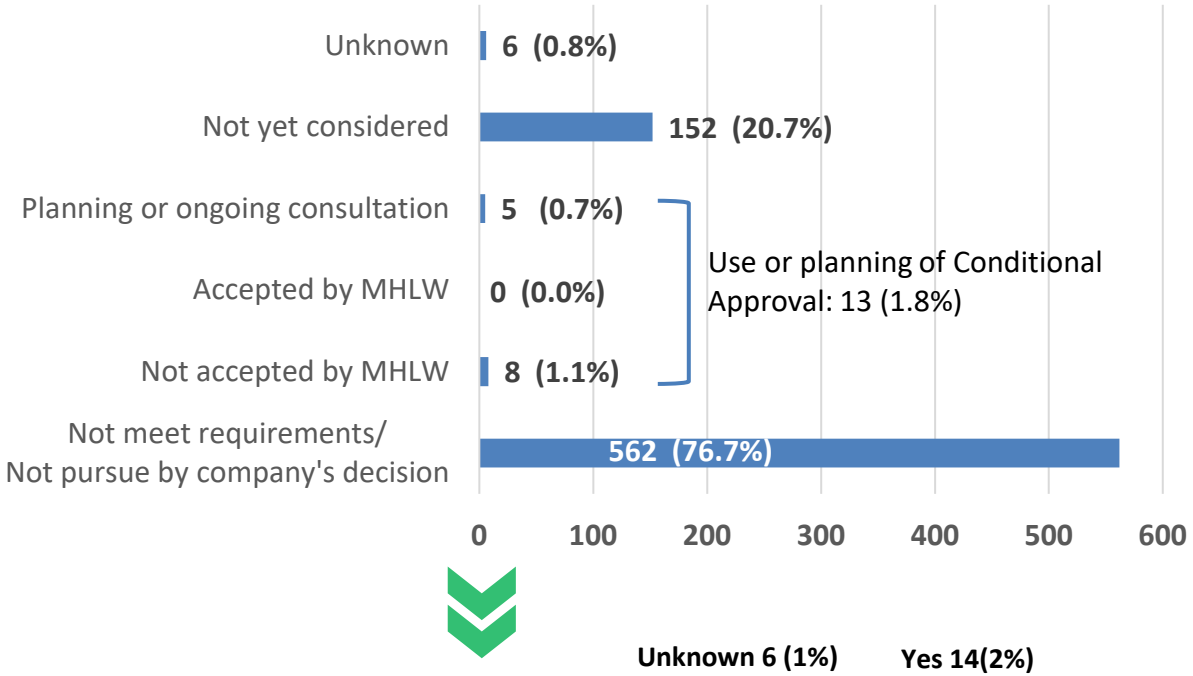
Within the 121 pediatric projects



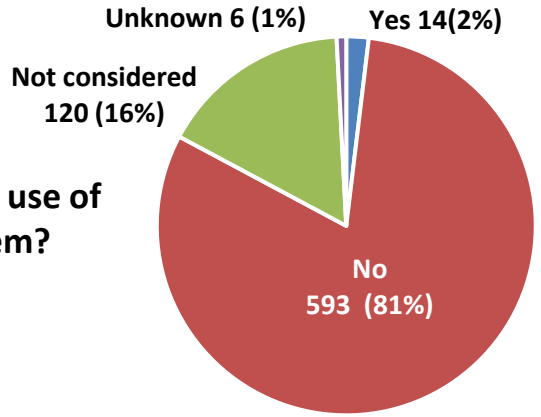
The utilization rates of the SAKIGAKE and Specific-Use pharmaceuticals, including those under consideration, were 4.1% and 0.8%, respectively.
In the context of innovative and Specific-Use pharmaceutical designation systems, many projects appear reluctant to utilize these systems, as seen in the results. A large portion of companies judge internally that they do not meet the necessary requirements for designation, likely due to the stringent criteria and uncertainties about future prospects.
Among the Specific-Use pharmaceuticals, there were zero cases used for the diagnosis, treatment, or prevention of diseases caused by drug-resistant pathogens.

Plan for Conditional Approval and Priority Review

Conditional Approval

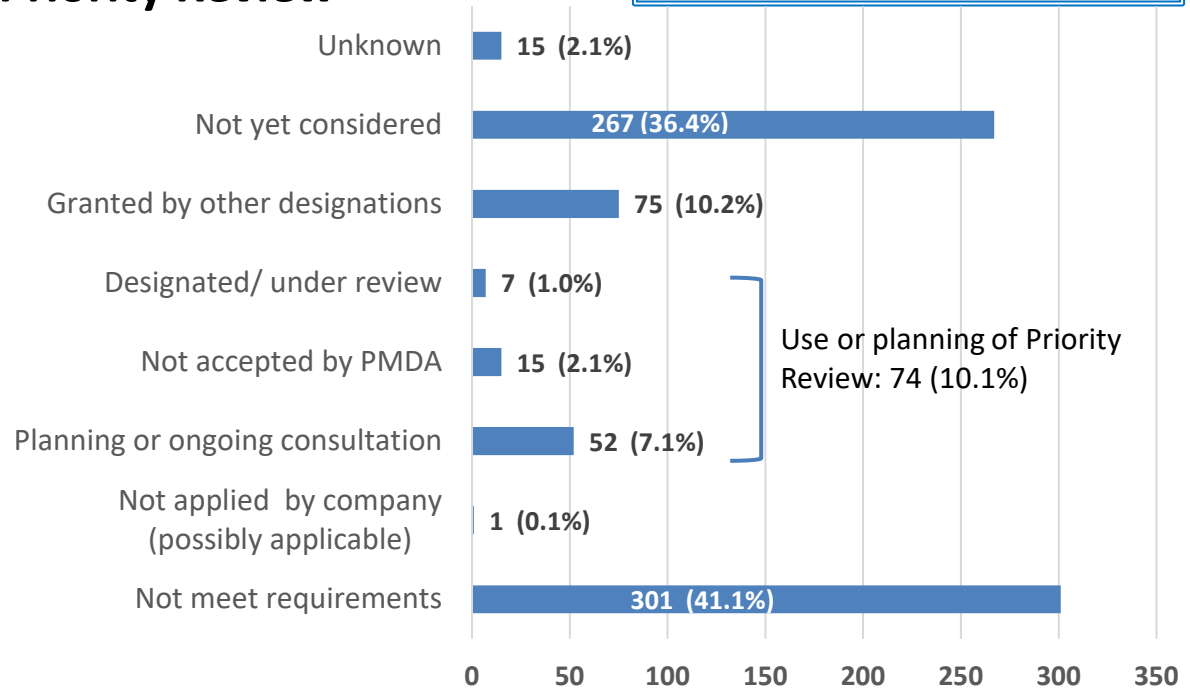


Did new regulation impact the use of the Conditional Approval system?



Priority Review

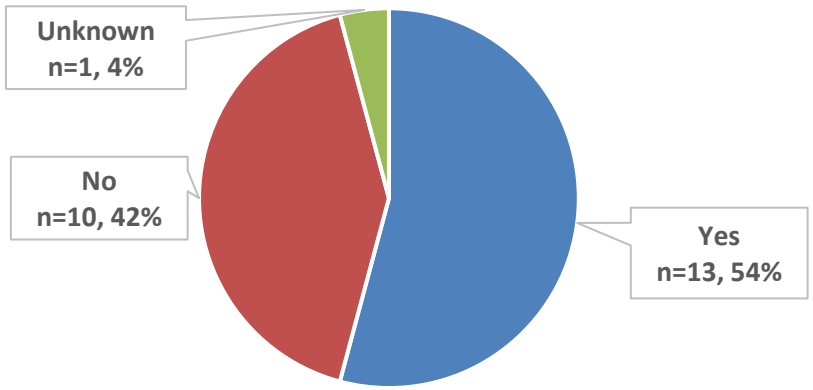
Survey Respondents 733 projects



Number of projects planning or using conditional approval system was 13 (1.8%), slightly increased from it in last year (6, 0.8%). However, impacts from revised regulation issued in Oct 2024 were limited.
Number of projects planning or using Priority Review was 74 (10.1%), comparable with those already granted by other designation system (i.e., ODD, SAKIGAKE, Specific-use)

Basic Principles on Japanese Clinical Trial Data for Rare Diseases (Notification; PMSB/ELD No.1023-3, Oct 23, 2024)

Are there any projects you plan to consider developing/ applying for using this notification? (# of company = 24)



- 13 out of 24 companies (54%) plan to utilize the notification for development or application in the future.

- One of the 24 companies had decided not to develop in Japan because Japanese data were requested at PMDA consultation before notification issue and there is no plan to reconsider development based on this notification due to missing the development timing.
- Following concerns/requests on notifications are raised;
  - Flexibility of target diseases
  - Not requiring final agreement with PMDA
  - Publication of cases and analysis results based on accumulated cases
  - Acceptance of CTD M2 in English and clarification of applicants' preventing action to avoid review delay
  - Promotion of risk-benefit assessment utilizing efficacy and safety information including RWD and M&S than risk-benefit assessment obtained from clinical trials
  - Mention of the necessity of Japanese data on rare diseases in pediatric patients.

Handling of Conditional Approval of Drugs (Revised Notification; PMSB/ELD No.1023-2, Oct 23,2024)

- Following concerns/requests on notifications are raised;
  - Cases that have been approved based on P2 results w/o any conditions should be approved as in the past (3)
  - Inform the applicability of conditional approval and the details of the conditions at the consultation prior to NDA
  - Acceptance of consultation on eligibility for conditional approval as part of pre-NDA consultation
  - Q & A publication
  - Flexible use of notifications

Utilization of Expedited Programs in Oncology NCEs*

*New Chemical Entities/New biologics/New regenerative medical products

N=65 out of 352 oncology projects, each row indicates a project

US								EU					Japan			
BT	AA	FT	PR	Orphan	RTOR	AAid	Orbis	PRIME	AA	CMA	EC	Orphan	Orphan	SAKIGAKE	CA	PR
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		●		●								●	●			N/A
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Note : No projects were planned, consulted or applied for Specific Use system in Japan.

US- BT: Breakthrough, AA: Accelerated Approval, FT: Fast Track, PR: Priority Review, RTOR: Real-Time Oncology Review, AAid: Assessment Aid

EU- AA: Accelerated Assessment, CMA: Conditional Marketing Authorization, EC: Exceptional Circumstances

Japan- CA: Conditional Approval, PR: Priority Review, N/A: granted through other designations

US								EU					Japan			
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Utilization of Expedited Programs in Oncology LCMs*

*Lifecycle Management(LCM) includes new indication/ dosage/ combination/ route of administration/ formulation

N=84 out of 352 oncology projects, each row indicates a project

US								EU					Japan			
BT	AA	FT	PR	Orphan	RTOR	AAid	Orbis	PRIME	AA	CMA	EC	Orphan	Orphan	SAKIGAKE	CA	PR
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US								EU					Japan			
BT	AA	FT	PR	Orphan	RTOR	AAid	Orbis	PRIME	AA	CMA	EC	Orphan	Orphan	SAKIGAKE	CA	PR
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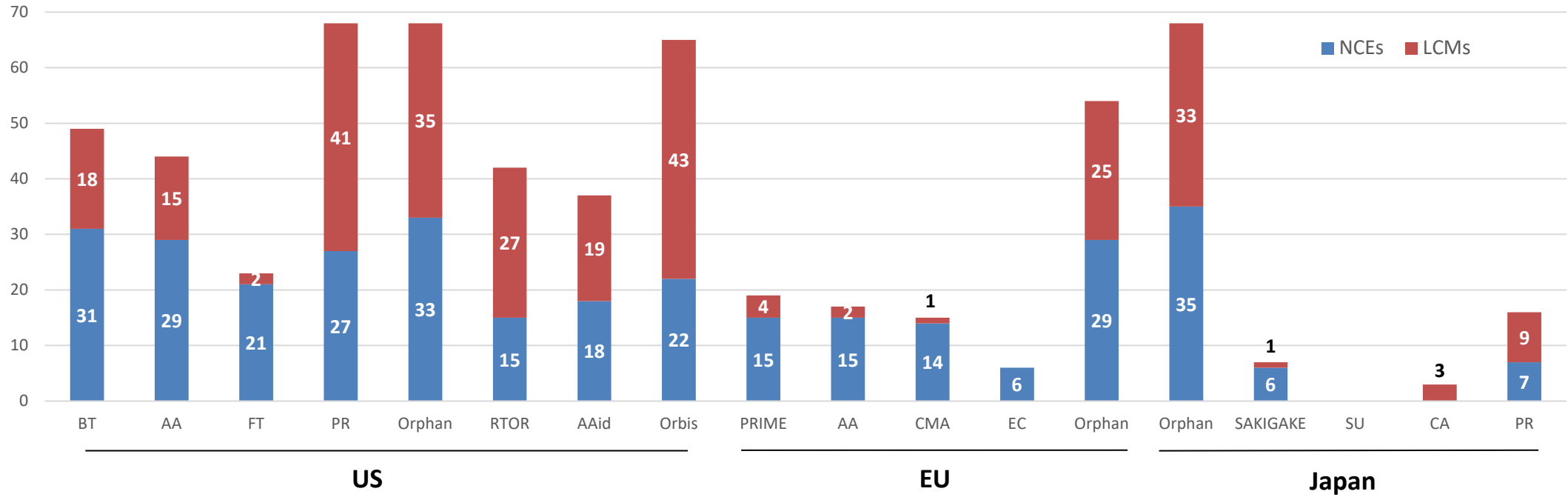
Note : No projects were planned, consulted or applied for Specific Use system in Japan.

US- BT: Breakthrough, AA: Accelerated Approval, FT: Fast Track, PR: Priority Review, RTOR: Real-Time Oncology Review, AAid: Assessment Aid

EU- AA: Accelerated Assessment, CMA: Conditional Marketing Authorization, EC: Exceptional Circumstances

Japan- CA: Conditional Approval, PR: Priority Review, N/A: granted through other designations

Utilization of Expedited Programs in Oncology - Summary

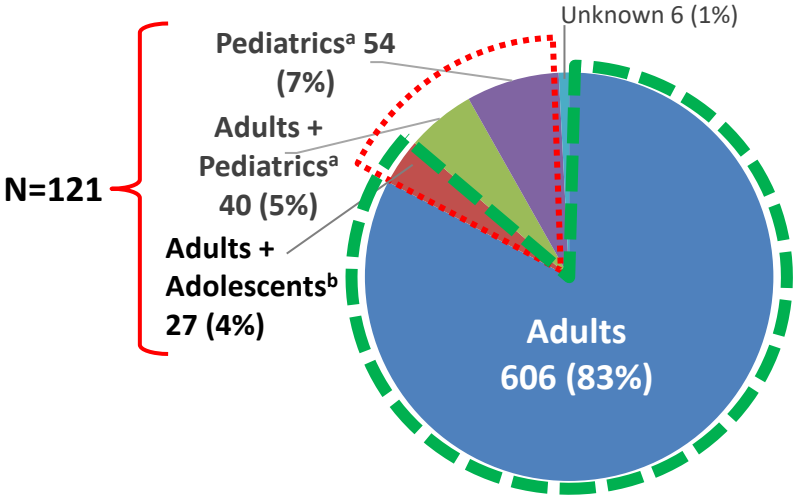


US- BT: Breakthrough, AA: Accelerated Approval, FT: Fast Track, PR: Priority Review, RTOR: Real-Time Oncology Review, AAid: Assessment Aid
EU- AA: Accelerated Assessment, CMA: Conditional Marketing Authorization, EC: Exceptional Circumstances
Japan- CA: Conditional Approval, SU: Special Use, PR: Priority Review

- Number of projects using expedited programs in US or EU were 149 over 352 Oncology projects (42%), more than 5% increase from last year. Of these, 65 were NCEs/New biologics/New regenerative medical products and 84 were new indication/ dosage/ combination/ route of administration/ formulation.
- Most common expedited program was Orphan across region. Priority Review and Project Orbis were also common in the US.
- In the US, all 149 projects used any of 8 expedited programs. In Japan, usage of expedited programs were limited to 74 of 149 projects. No project used Drugs for Specific Use. In EU, it is also limited to 68 of 149 projects.

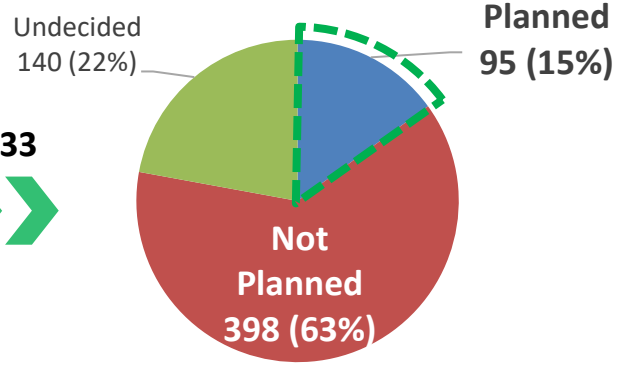
Pediatric Development

Target patients of projects (N=733)



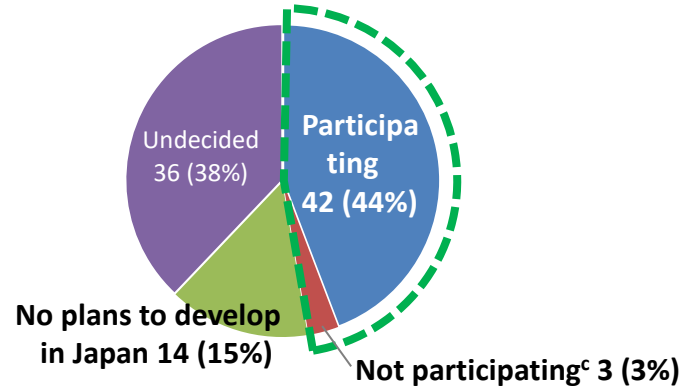
Plan for pediatric development globally?

N=633



Japan joins or will join Global study in PIP/PSP?

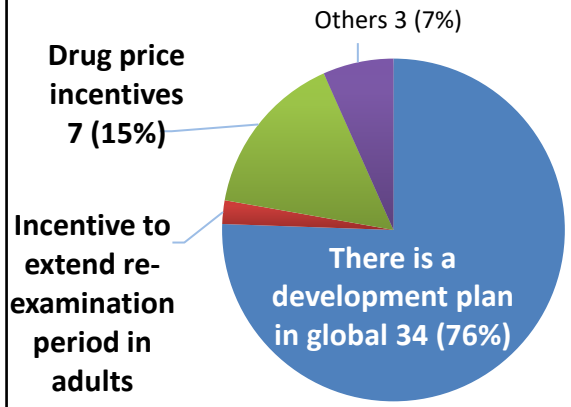
N=95



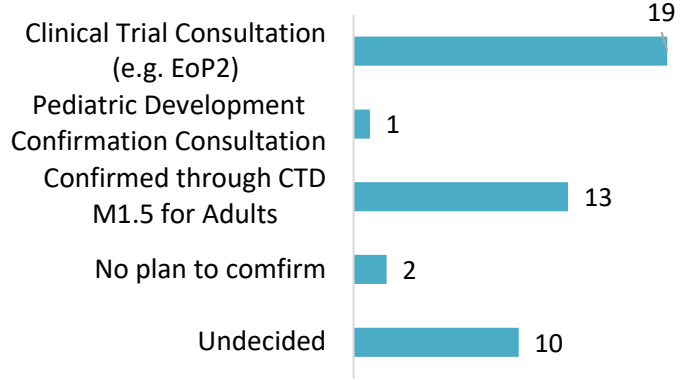
- 121 of 733 (17%) projects include pediatrics (and adolescents). (Same result as in 2024 (17%)).
- 95 of 633 (15%) adults (and adults + adolescents) projects had pediatric development plan. About half of projects (44%) join global studies in PIP/PSP.
- Primary reasons for pediatric development plan in Japan was a global plan, followed by pricing incentives. Ratio of “Drug price incentives” became higher than 2024. On the other hand, “re-examination period extension in adults” decreased.
- The new consultation process for pediatric development plan confirmation has been used in a project.
- The notification on “The development plan for pediatric drugs to be performed during the development period of a drug intended for adults” on 12 Jan 2024 may change decision to pursue pediatric development. However, there is limited or no impact yet on increasing number of pediatric development at this time. Further enhancement and update of system for pediatric development is expected for further improvement.

N=45

Primary reason for pediatric development

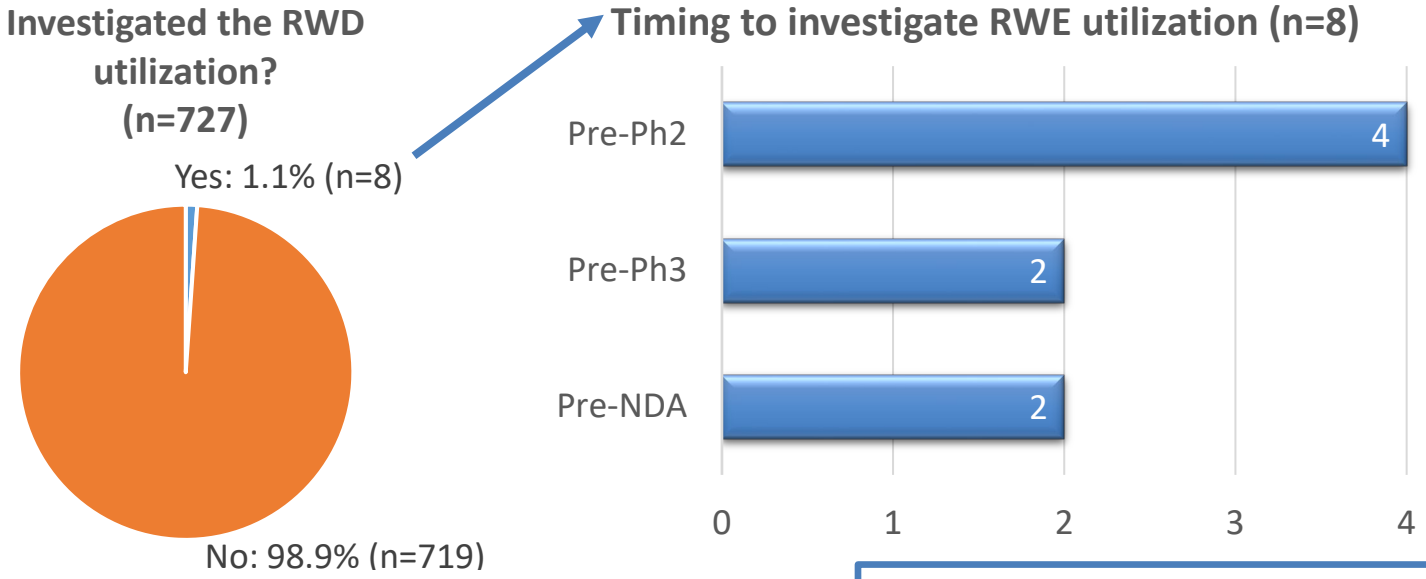


Plan to confirm pediatric development plan with PMDA



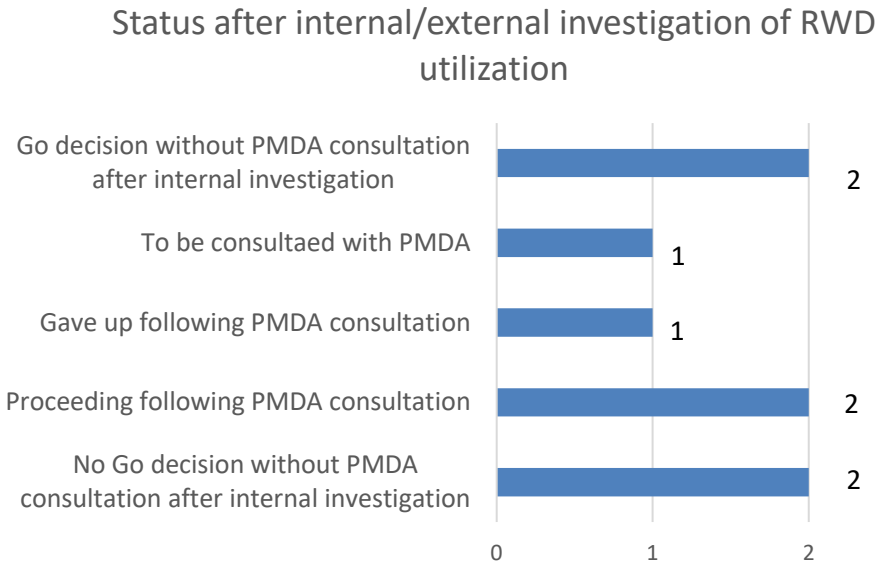
a: < 18 years old (including patients less than 12 years old)
b: >= 12 years old, < 18 years old
c: Japan local or MRCT with countries other than the US/EU

Utilization of Real-World Data in Clinical Data Package



Objective for the RWD utilization (multiple responses allowed)

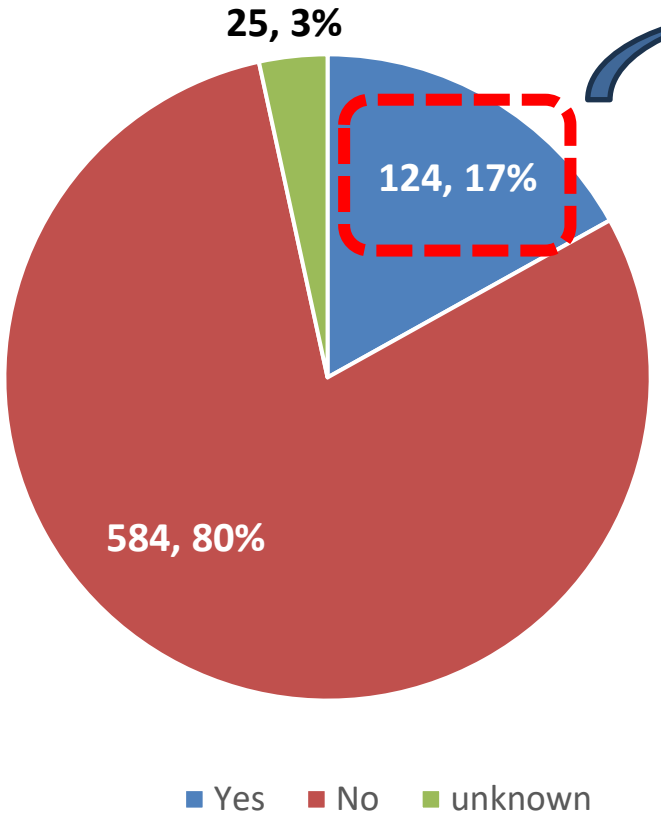
	Yes	No
Efficacy evaluation data	0	8
Safety evaluation data	0	8
Efficacy reference data	5	3
Safety reference data	2	6



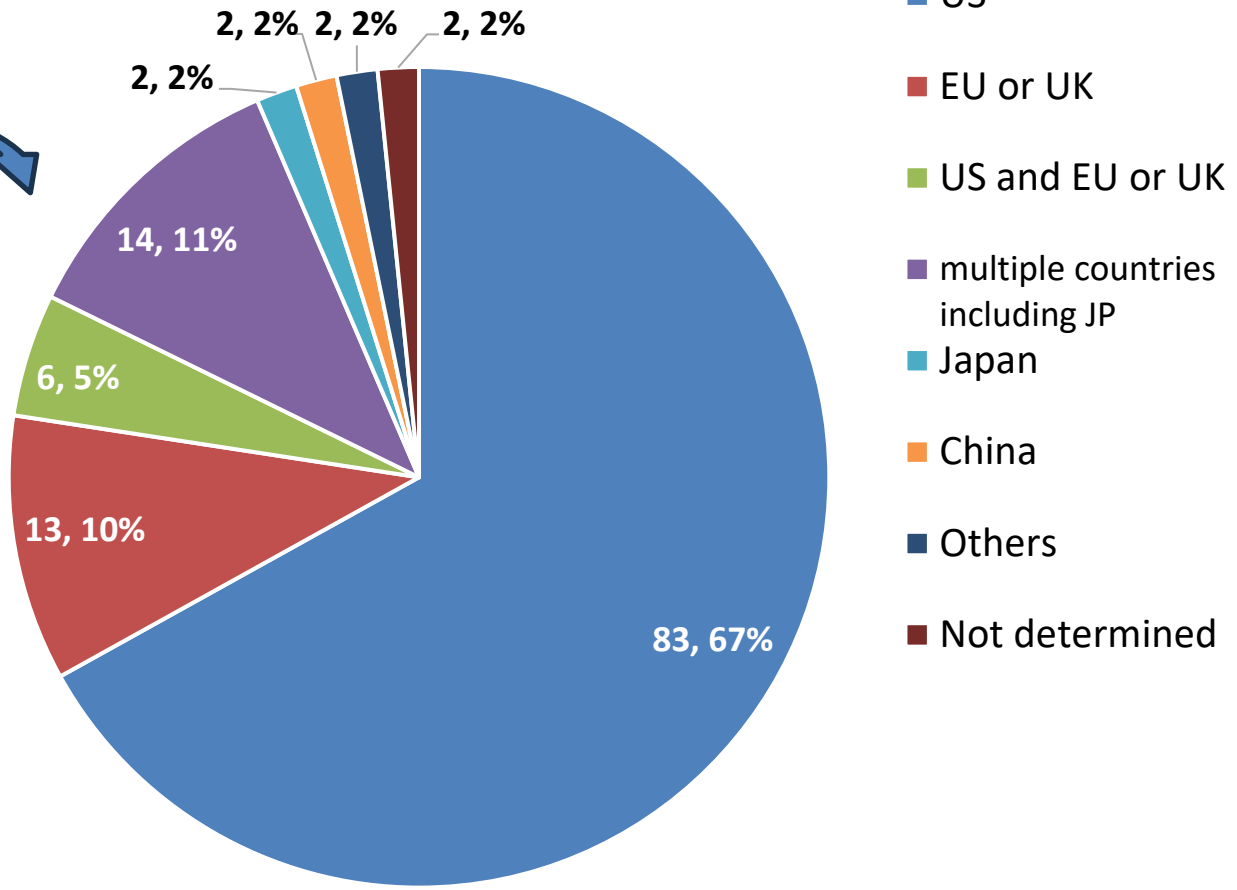
- The utilization of RWD data was investigated in 8 (1.1%) of 727 projects, all of which considered orphan designation. Among these, 4 were categorized as New Active Ingredient and 4 were categorized as New Indication and/or New Dose in filing category.
- Timing of investigating RWD utilization varied across 8 projects: 2 projects at pre-NDA, 2 projects at pre-Ph3, and 4 projects at pre-Ph2. Most common purpose for using RWD was efficacy reference data (5 projects).
- 4 projects have held or are planning PMDA consultations for RWD use. None of these used registry-related consultation category. As a result of consultations: 2 projects are proceeding with RWD and 1 project gave up. Another 1 project is planning PMDA consultation. The remaining four projects made decisions without PMDA consultation.
- The scope of RWD's use in Japan is expanding, but cases were still limited to external controls, public knowledge-based applications, and reference studies in CTD. Companies want to use RWD more easily by allowing flexibility in regulations regarding data sources and reliability.

Submission lag (1)

Currently filed or scheduled to be filed to PMDA by the end of March 2026 based on the results of MRCT (N=733)



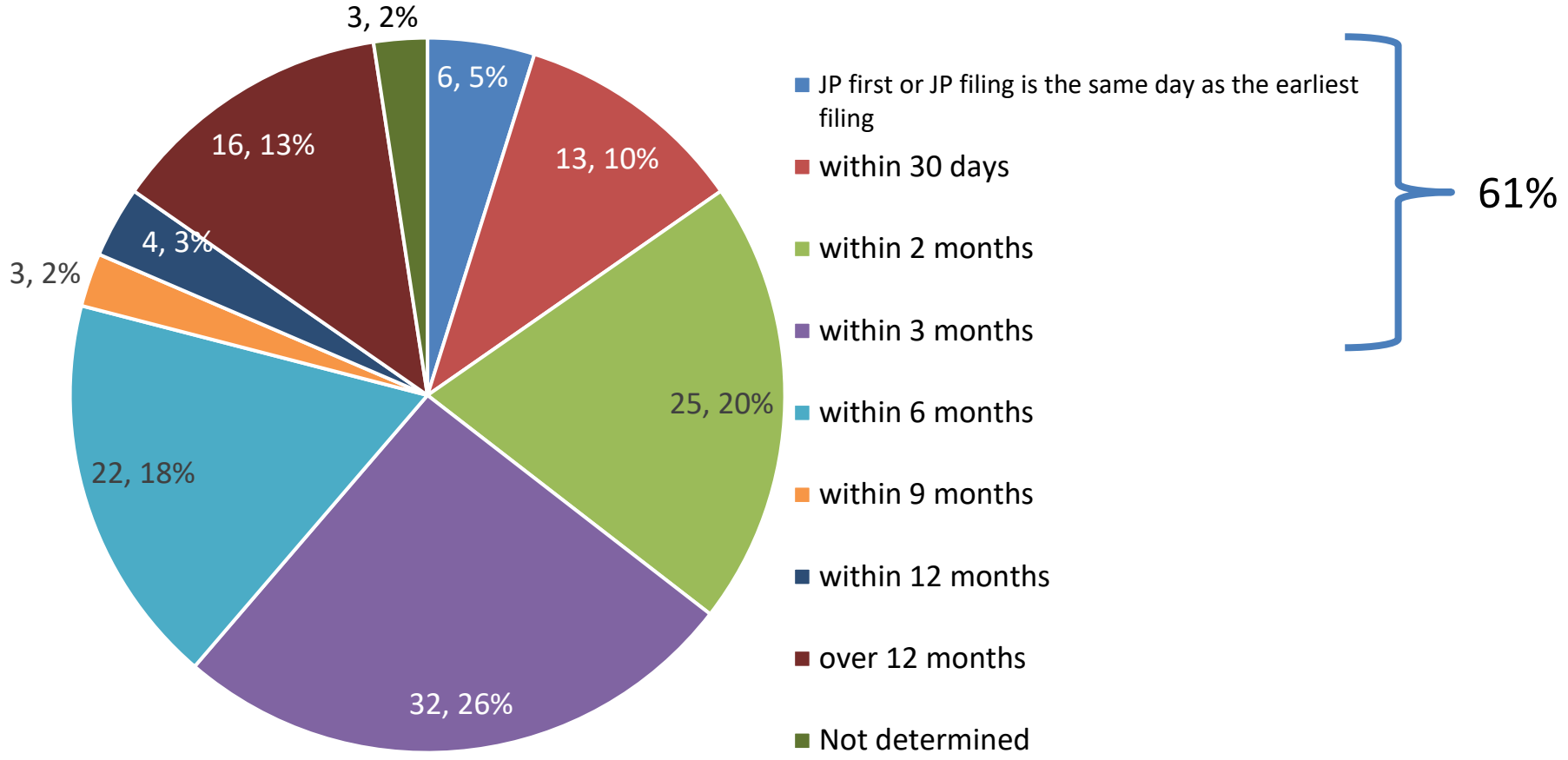
First submission region(N=124)



Almost all the first submission regions were the US and EU･UK . The first submission in Japan alone was 2 %.

Submission lag (2)

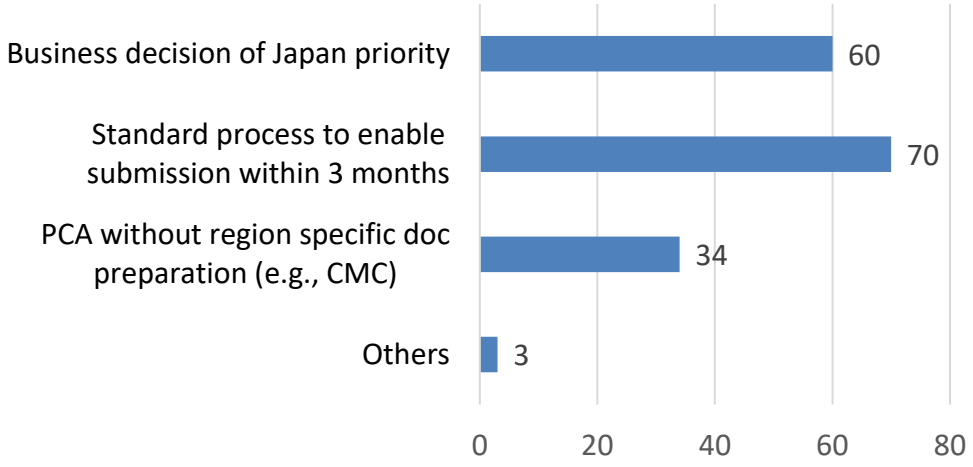
Time lag between the 1st Submission in the World and the JP filing (N=124)



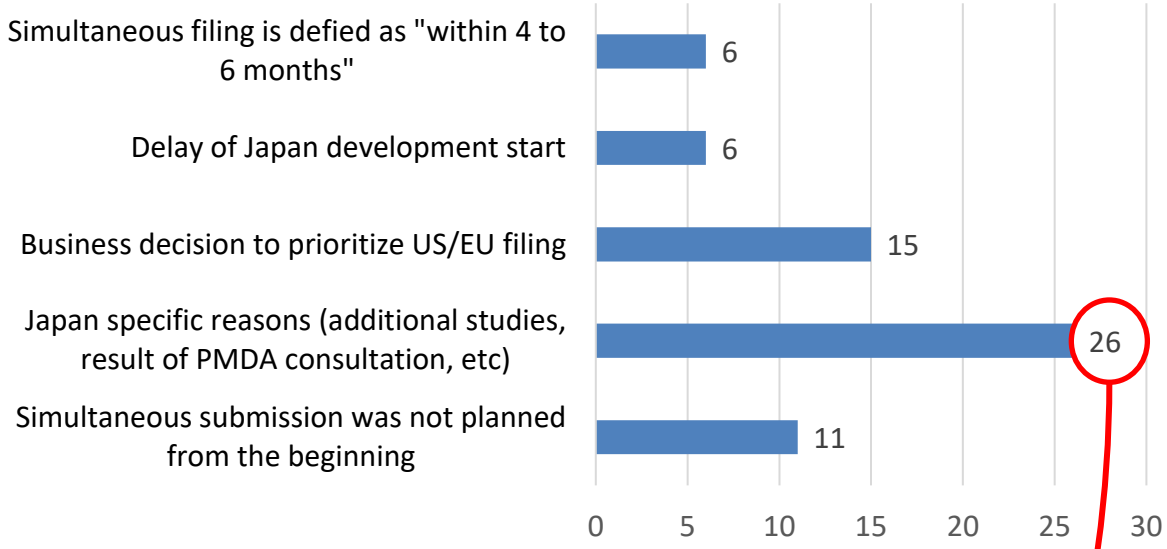
The first submission in Japan or same-day submission with the earliest filing is 5%, but submission in Japan within 3 months is planned in about 61% of projects; this number increased from 2024 (52% in 2024).

Submission lag (3)

Reasons why submission in Japan within 3 months from 1st submission can be done.（n=76, multiple answers）



Reasons why submission in Japan within 3 months from 1st submission cannot be done（n=45, multiple answers）



- **Japan's first or within 3 months from the earliest submission was achieved by business decision and standard processes that enable submission within 3 months from the earliest submission.**
- **The main reasons for not filing JP first or not within 3 months from the earliest submission were Japan-specific regulatory requirements and a business decision.**
- **Major Japan specific reasons which caused delay in Japan submission were:**
  - **PMDA opinion affected submission timing (13/26)**
  - **Others (10/26)**
  - **Preparation of M2.3 or applicant form for Japan (1/26)**
  - **Additional study or additional analysis conducted by company decision (1/26)**

- PMDA required an additional clinical study (4 cases).
- Clinical data package deemed insufficient for J-NDA (5 cases).
- PMDA pre-NDA consultation was required (5 cases).

Drug Loss

NCEs approved in either the U.S. or Europe at the global headquarters during the year
(from April 2024 to the end of March 2025)

40 products (24 Companies)

Respondents did not provide development plans for 11 products

Under development or approved in Japan
25 products
Approved : **15 products**
Under Ph3 study or Under Review：**9 products**
Prior to CTN of Ph3 study : **1 product**

No plan for development in Japan branch
4 products
Oncology area: **1 product**
Non-Oncology area: **3 products**

License out
1 product

Possibility of drug loss
3 products

No license out
3 products

Last year, 40 NCEs were approved in either the US or Europe. However, since development plans in Japan were not provided for 11 of these products, it is not possible to fully assess potential drug loss. Among the 29 products for which responses were received, 3 were reported to have no development plans in Japan, suggesting that they may lead to drug loss.