

Strategic Goal and Action Plan for Improving Drug Discovery Capabilities to Support Early Availability of Innovative Drug -Toward Implementation of the Interim Report-

July 30, 2024

National Healthcare Policy Secretariat

Three strategic goals and performance targets

The government, together with the relevant ministries, will pursue the **three strategic goals** set forth in the interim report by promoting and executing concrete measures and projects. **The aim is to achieve the following performance targets (outcomes):**

(1) **"Prompt Delivery of Novel Drugs to Patients"**

- Eliminate the current drug loss (start development by FY2026 for drugs that treat diseases for which no drugs exist in Japan)
* Furthermore, to prevent new drug loss in Japan, mid-term performance targets will be set based on the discussions and examinations in the public-private council, while considering the situation in the U.S. and Europe.
- Aim for 50 development plans for pediatric drugs and 150 approvals of orphan drugs for (cumulative from FY2024 to FY2028)

(2) **"Become one of the world's leading drug discovery sites"**

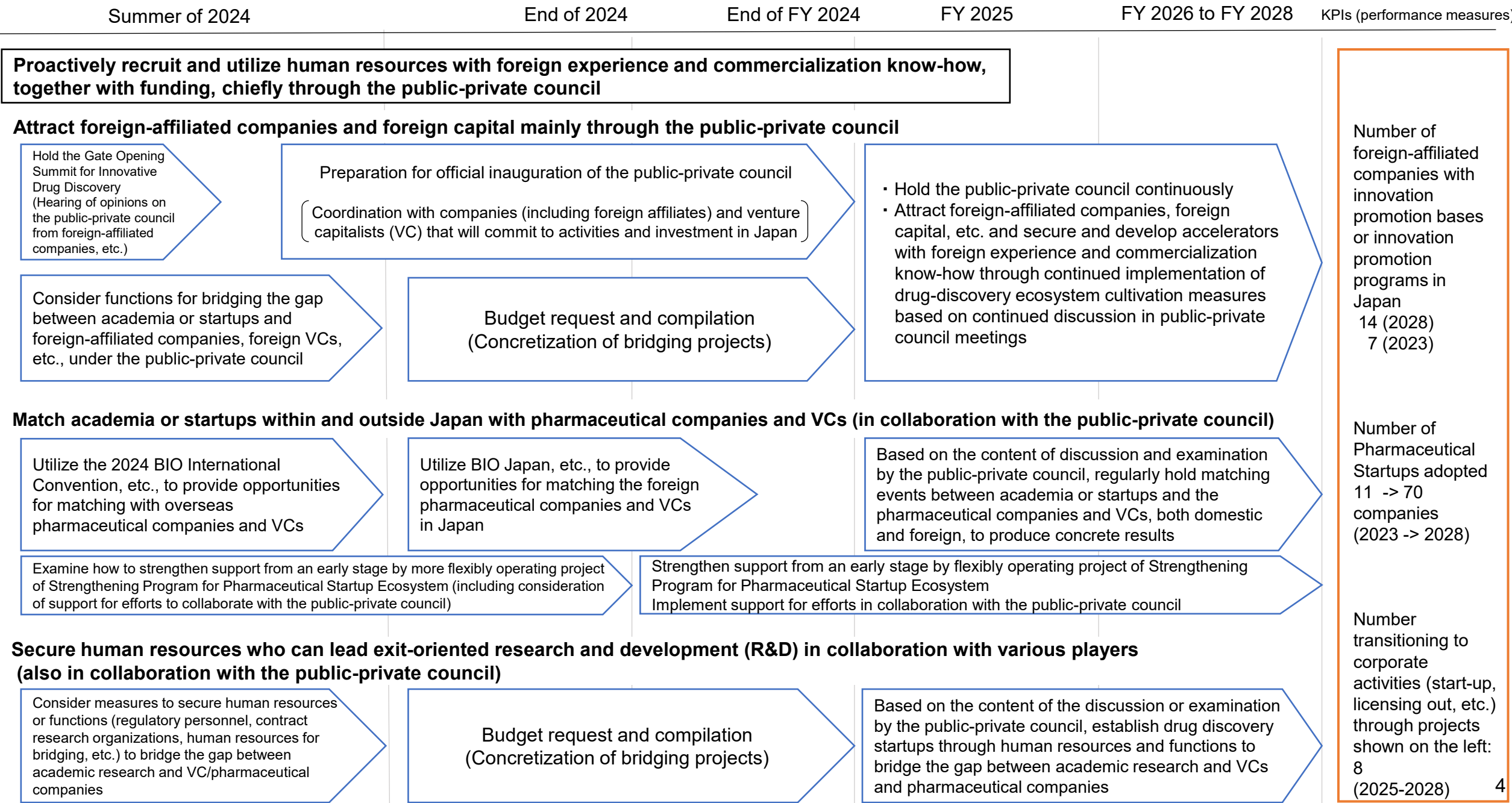
(3) **"Cyclically develop investment and innovation"**

- Increase the number of initial clinical trial plan notifications for global clinical trials in Japan from 100 to 150 (from 2021 to 2028)
- Ensure private investment in drug discovery startups (two-fold increase; 2023 -> 2028)
- Develop 10 or more new drug discovery startups with a corporate value of 10 billion yen or more (2028) *Develop drug discovery unicorns by 2033
- Ensure recognition of Japan's cities as among the world leader in drug discovery (within the top 10; 2028)

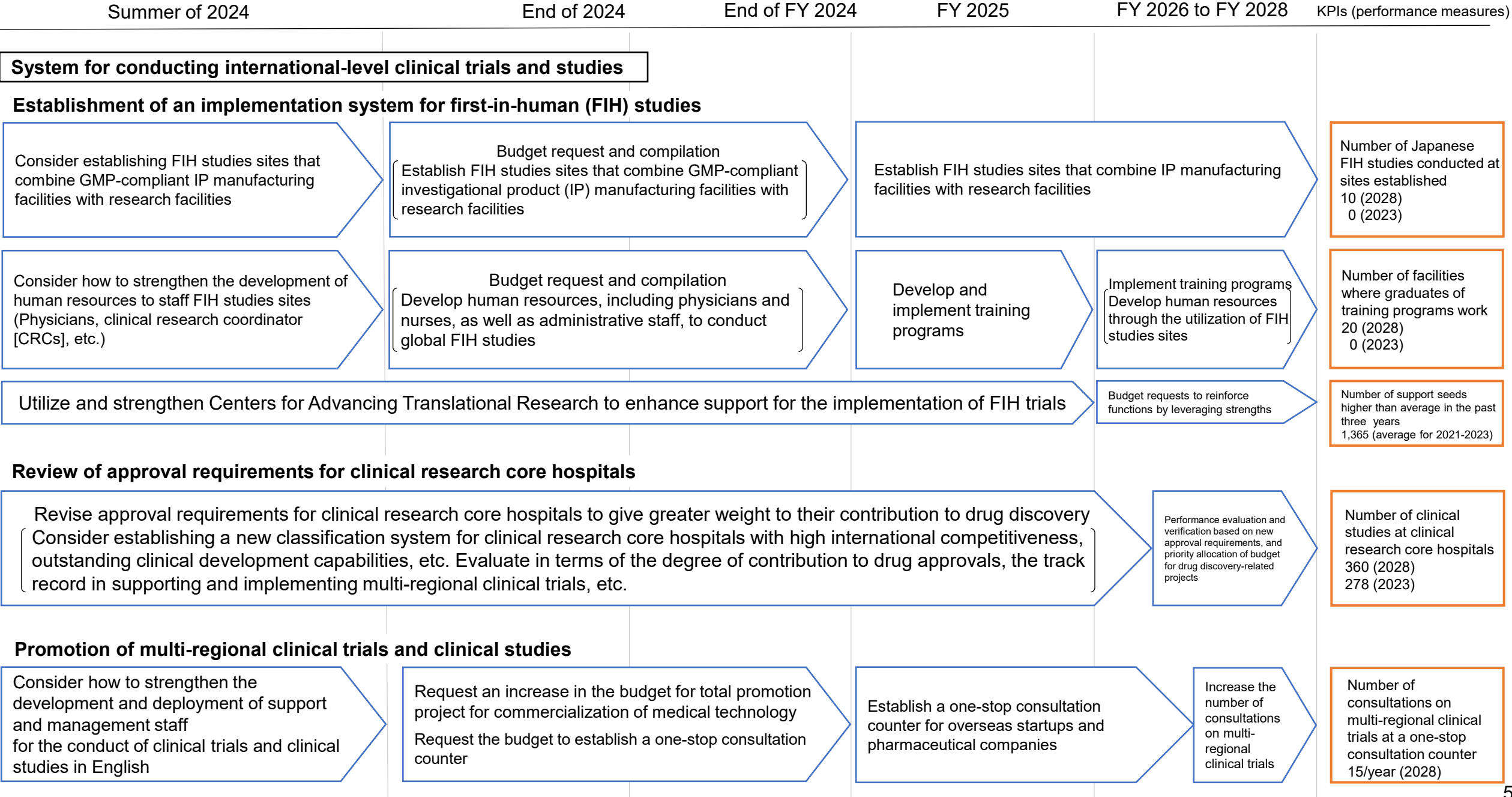
To achieve the above goals, create five-year flowcharts for each policy, establish **outcome measures (key performance indicators [KPIs])**, and provide **follow-ups to track progress**. In addition, **review** the flowcharts and KPIs in a **timely and appropriate manner based on the achievement status of performance targets (outcomes), the progress of policy measures, and any changes in the circumstances surrounding drug discovery**. Implement follow-up through **comprehensive evaluation by experts** in addition to evaluation of the above performance targets.

1. Strengthen Japan's Drug Discovery Capabilities

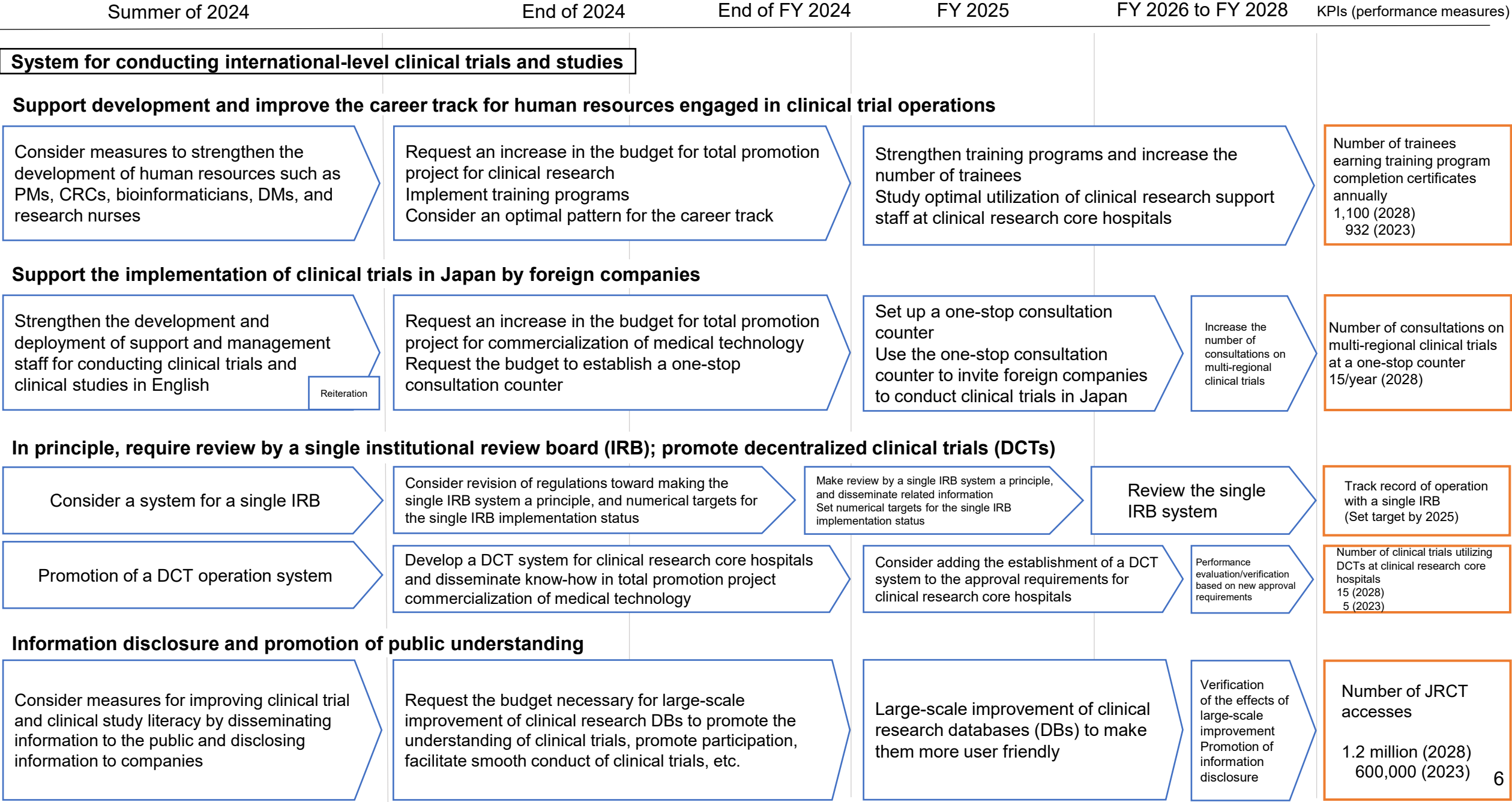
Schedules and flowcharts for each policy measure [1]



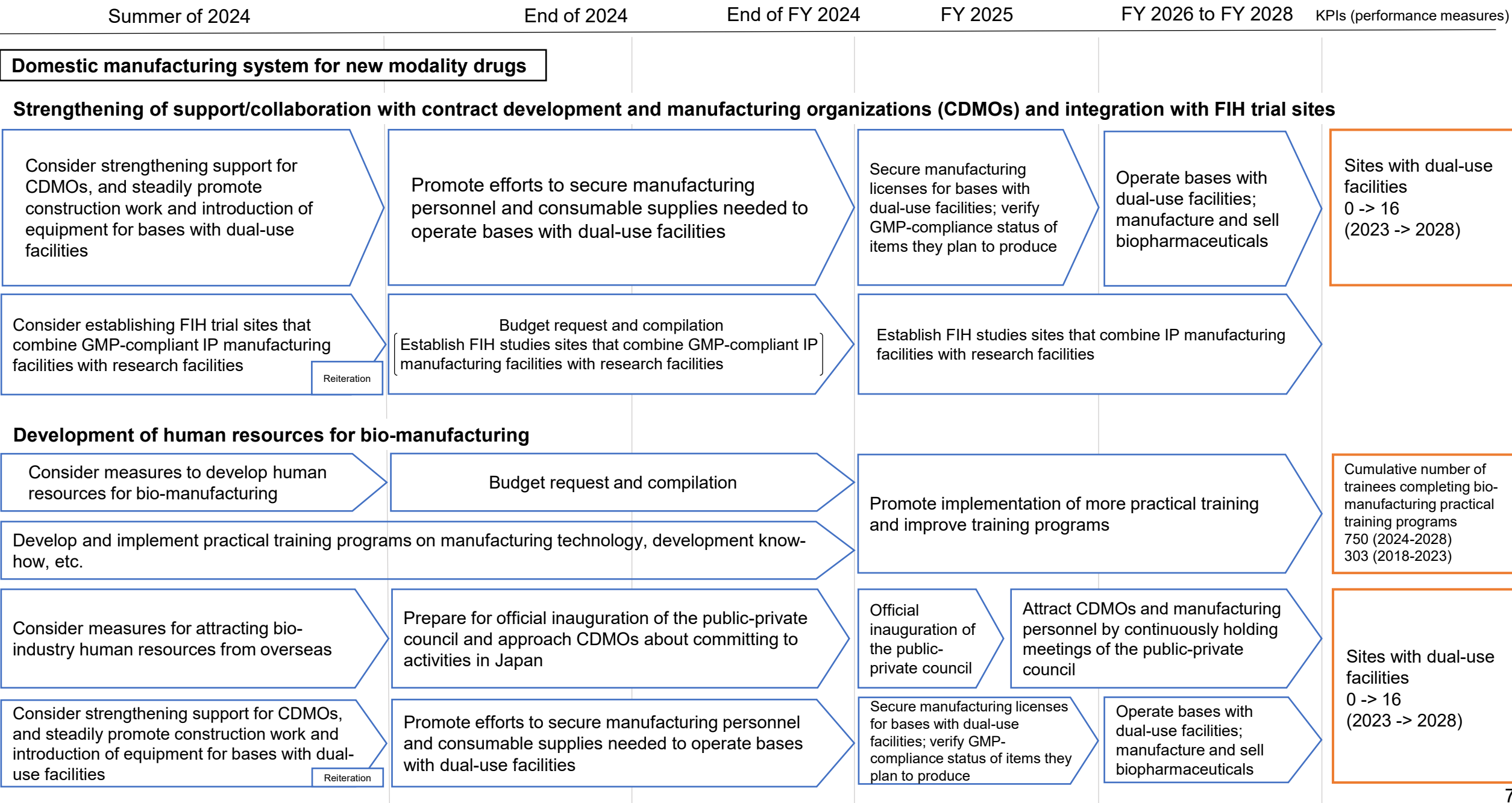
Schedules and flowcharts for each policy measure [2]



Schedules and flowcharts for each policy measure [3]



Schedules and flowcharts for each policy measure [4]



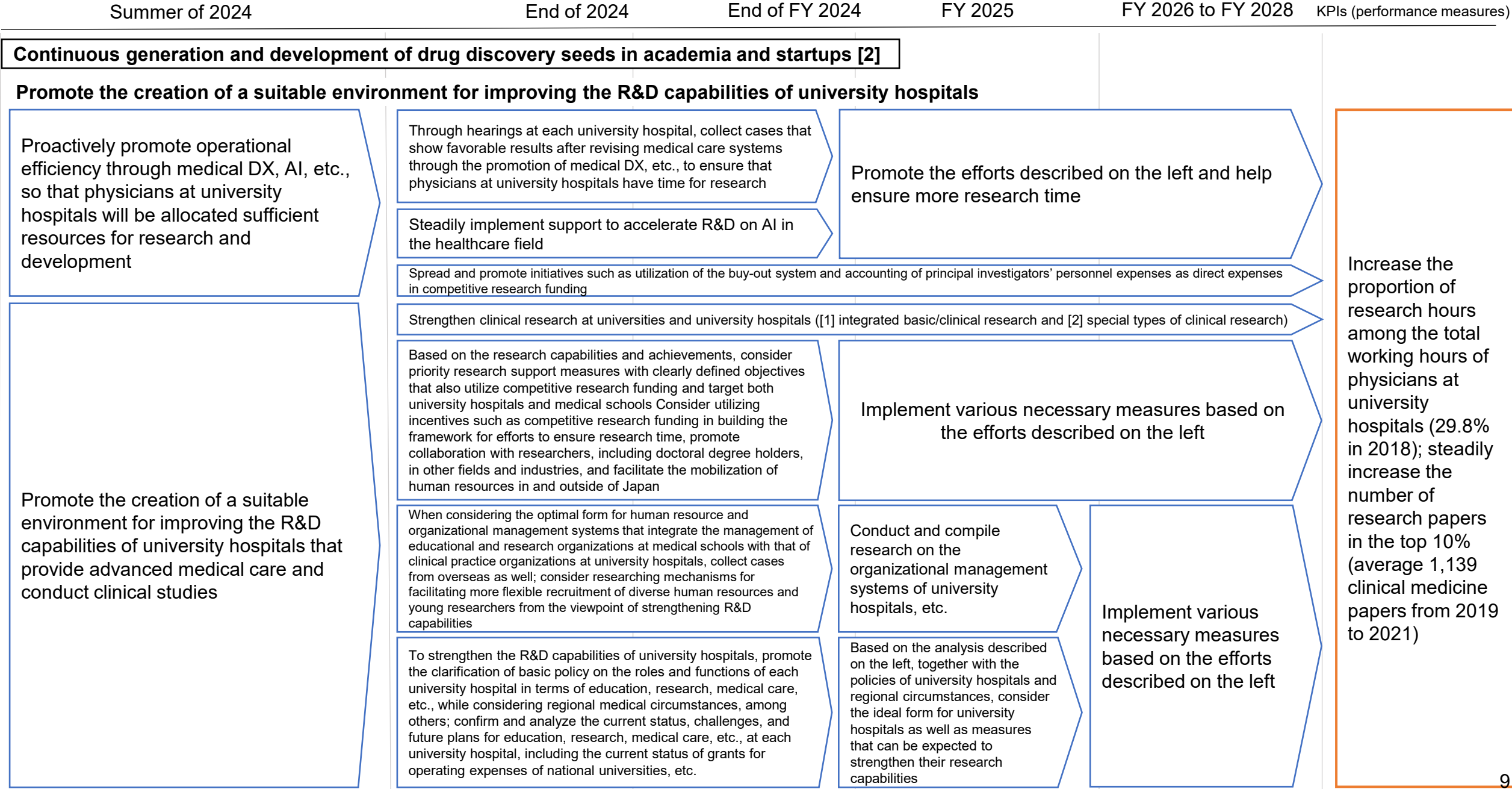
Schedules and flowcharts for each policy measure [5]

Summer of 2024	End of 2024	End of FY 2024	FY 2025	FY 2026 to FY 2028	KPIs (performance measures)
Continuous generation and development of drug discovery seeds in academia and startups [1]					
Promotion of cross-disciplinary research and basic research on new modalities					
Promotion of cross-disciplinary/basic research on new modalities, combining artificial intelligence (AI)/robotics with drug discovery, in addition to traditional research on medicine, pharmacology, physical sciences, etc.	At RIKEN, build multiple state-of-the-art research platforms that continuously generate drug discovery target candidates by combining cutting-edge research outcomes from academia, etc.				Creation of an average of three or more innovative drug discovery seeds per year (2028) <- 12 cases in six years from 2018 to 2023
	(Example) Establish sophisticated high throughput screening platform to explore novel drug discovery targets/compound seeds by utilizing patient-derived iPS cells and robotization/automation with AI analysis				
	(Example) Construct a robust technology platform to obtain candidate antibodies for diagnostic and therapeutic drugs by leveraging RIKEN's technologies such as structure analysis of antibody-antigen binding, robotization/automation for protein production with AI analysis				
	(Example) Develop RIKEN's unique cell-specific drug delivery system platform using glycans with other stakeholders' technologies to provide personalized medicine for unmet medical needs, such as cancers with genetic mutations or refractory brain diseases				
	Establish novel drug discovery R&D system and network that utilize the integrated scientific strength of RIKEN (biology X AI), including Fugaku supercomputer and robotization technology, AI technology, etc., to streamline and accelerate the creation of clinical development candidates from drug discovery seeds				
Development of academic human resources (including optimization of medical and pharmaceutical education)					Further increase the number of people engaged in drug discovery* (1,225 people in 2023) *Among persons completing undergraduate- or graduate-level pharmaceutical science programs, those engaged in drug discovery-related work, research, etc.
Accept global human resources (including data science workers) in academia and consider what direction to take in reviewing human resource development and educational content based on new modes of clinical research and drug discovery	Consider how to enhance university education programs (medicine, pharmaceutical science, etc.) to develop medical human resources who can contribute to drug discovery		Promote the efforts described on the left while promoting the development of academic human resources that can handle new modes of drug discovery		
	Examine educational content for pharmaceutical human resource development that leads to drug discovery, with an eye on revising the pharmaceutical education model core curriculum for the next term (plan to start examining it in 2026)			Discussion and work related to the revision of the model core curriculum for pharmaceutical education for the next term	

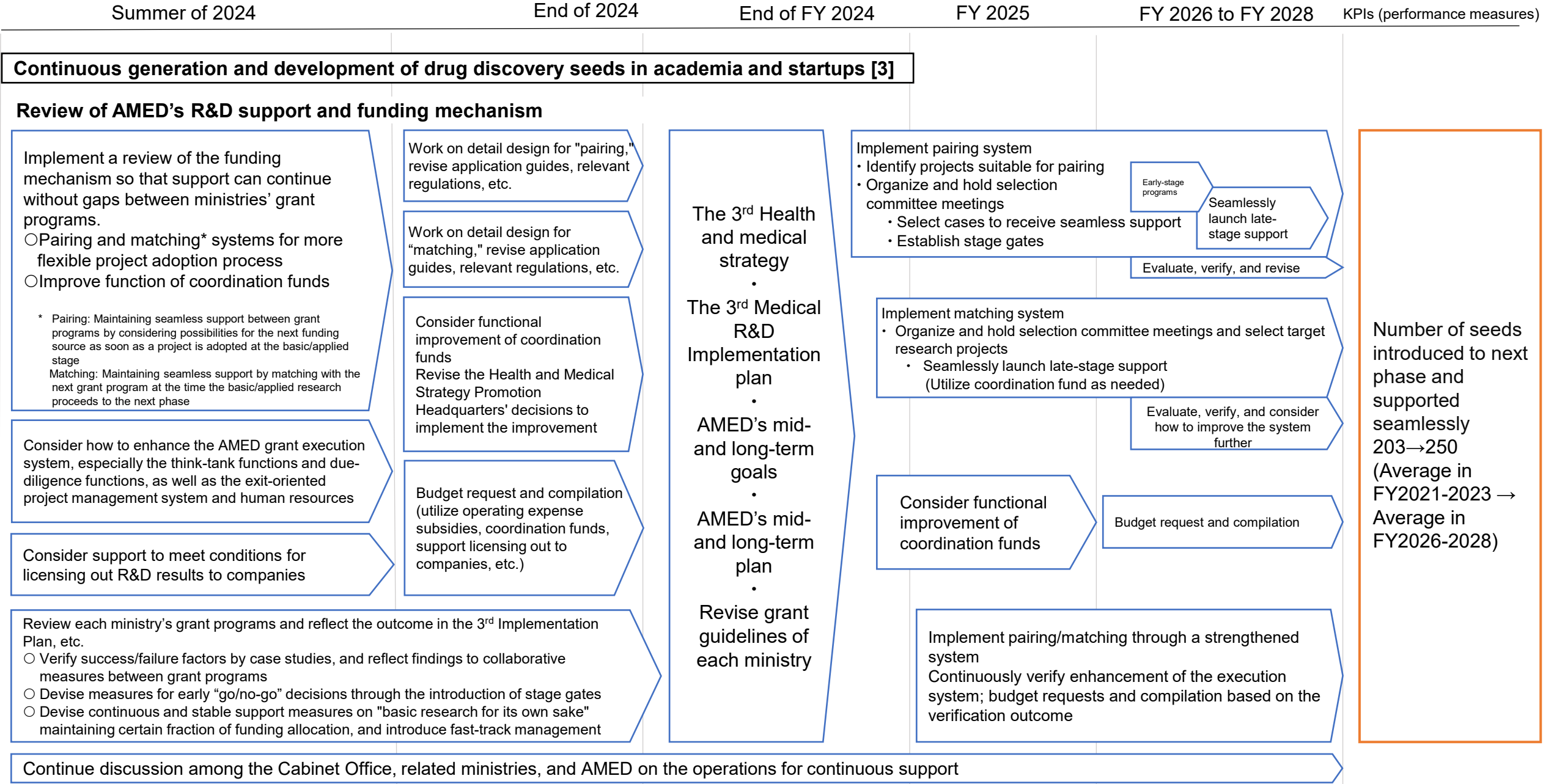
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*Among persons completing undergraduate- or graduate-level pharmaceutical science programs, those engaged in drug discovery-related work, research, etc.

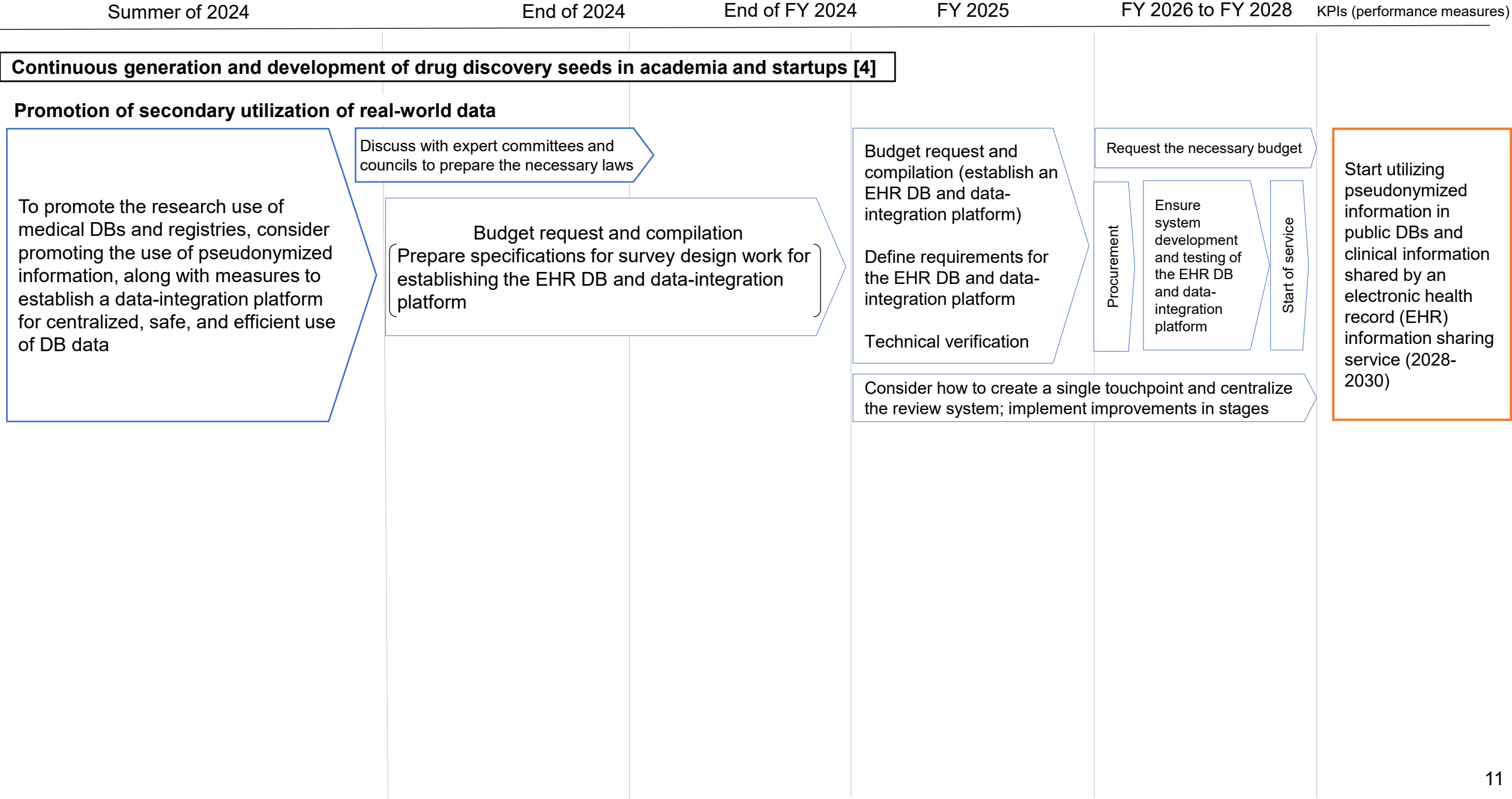
Schedules and flowcharts for each policy measure [6]



Schedules and flowcharts for each policy measure [7]



Schedules and flowcharts for each policy measure [8]



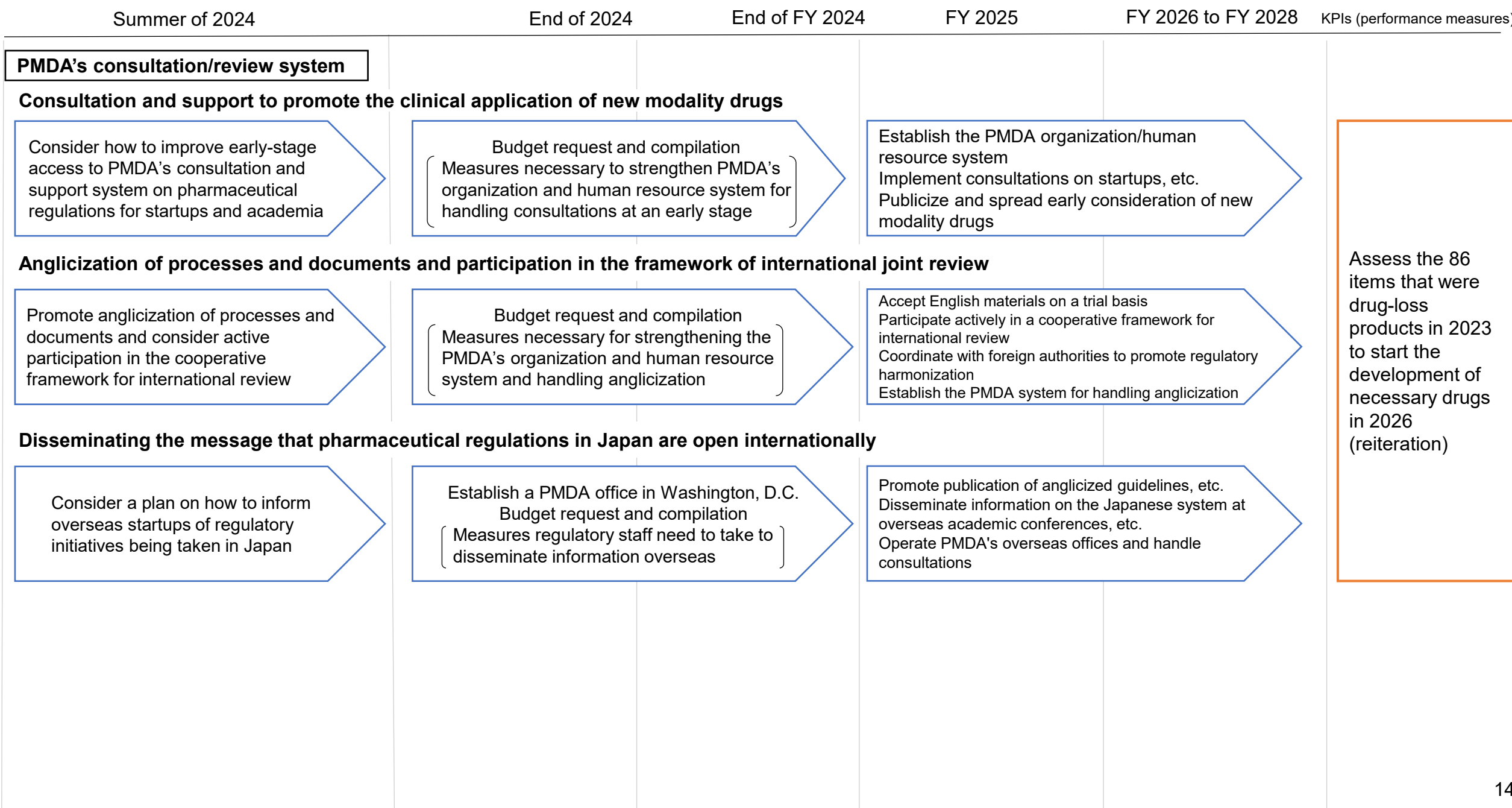
2. Prompt Delivery of Novel Drugs to Patients

Schedules and flowcharts for each policy measure [1]

Summer of 2024	End of 2024	End of FY 2024	FY 2025	FY 2026 to FY 2028	KPIs (performance measures)
Review of pharmaceutical regulations, etc.					<div>Assess the 86 items that were drug-loss products in 2023 to start the development of necessary drugs in 2026</div> <div>Number of development plans formulated for pediatric drugs 50 (cumulative from FY 2024 to FY 2028)</div> <div>Number designated as orphan drugs 200 (cumulative from FY 2024 to FY 2028)</div> <div>151 (cumulative from FY 2018 to FY 2022)</div>
Reassess the concept of conducting phase-I studies in Japanese subjects when participating in multi-regional clinical trials; consider revising pharmaceutical regulations in line with international harmonization, such as clarifying when data on Japanese subjects are not necessary in confirmatory studies		Implement and apply pharmaceutical regulations based on international harmonization			
Promotion of the development of drugs for children, intractable diseases, and rare diseases					
Confirm pediatric drug development plan by the PMDA's Regulatory Consultation Center for Pediatric and Rare Disease Implement early designation of orphan drugs	Budget request and compilation Strengthen the Pharmaceuticals and Medical Devices Agency's [PMDA's] organization and human resource system for promoting the development of drugs for children, intractable diseases, and rare diseases, as well as the measures necessary for grants for the development of drugs for rare diseases		Establish the PMDA support system for promoting the development of drugs for children, intractable diseases, and rare diseases, along with an evaluation support system		
Consider ways to accelerate requests for evaluation and development of drug-lag/drug-loss products	Budget request and compilation Strengthen the PMDA's organization and human resource system to accelerate requests for the evaluation/development of drug-lag/drug-loss products		Eliminate drug-lag/drug-loss by accelerating requests for the evaluation and development of those products Establish a PMDA evaluation system		
Organize drug data, investigate the needs of related academic societies, investigate marketability, and prioritize the development of current drug-loss products			The Committee on Unapproved and Off-label Drugs with High Medical Needs evaluates the drugs, and MHLW asks companies to develop them, and issues open calls for development		
Consider initiatives for new drug-loss products	Budget request and compilation Measures necessary for organizing information on new drug-loss products		Organize drug data, investigate the needs of related academic societies, investigate marketability, and prioritize development	The Committee on Unapproved and Off-label Drugs with High Medical Needs evaluates the drugs, and MHLW asks companies to develop them, and issues open calls for development	

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Schedules and flowcharts for each policy measure [2]



3. Construction of a Social System That Allows Continued Cyclical Development of Investment and Innovation

Schedules and flowcharts for each policy measure

