Provisional Translation

Document 1

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]

Summer of 2024 | End of 2024 | End of FY 2024 | FY 2025 | FY 2026 to FY 2028 | KPIs (performance measures)

Proactively recruit and utilize human resources with foreign experience and commercialization know-how, together with funding, chiefly through the public-private council

Attract foreign-affiliated companies and foreign capital mainly through the public-private council

- Hold the Gate Opening Summit for Innovative Drug Discovery (Hearing of opinions on the public-private council from foreign-affiliated companies, etc.)
- Preparation for official inauguration of the public-private council
  (Coordination with companies (including foreign affiliates) and venture capitalists (VC) that will commit to activities and investment in Japan)
- ・Hold the public-private council continuously
  ・Attract foreign-affiliated companies, foreign capital, etc. and secure and develop accelerators with foreign experience and commercialization know-how through continued implementation of drug-discovery ecosystem cultivation measures based on continued discussion in public-private council meetings
- Consider functions for bridging the gap between academia or startups and foreign-affiliated companies, foreign VCs, etc., under the public-private council
- Budget request and compilation (Concretization of bridging projects)

Match academia or startups within and outside Japan with pharmaceutical companies and VCs (in collaboration with the public-private council)

- Utilize the 2024 BIO International Convention, etc., to provide opportunities for matching with overseas pharmaceutical companies and VCs
- Utilize BIO Japan, etc., to provide opportunities for matching the foreign pharmaceutical companies and VCs in Japan
- Based on the content of discussion and examination by the public-private council, regularly hold matching events between academia or startups and the pharmaceutical companies and VCs, both domestic and foreign, to produce concrete results
- Examine how to strengthen support from an early stage by more flexibly operating project of Strengthening Program for Pharmaceutical Startup Ecosystem (including consideration of support for efforts to collaborate with the public-private council)
- Strengthen support from an early stage by flexibly operating project of Strengthening Program for Pharmaceutical Startup Ecosystem
  Implement support for efforts in collaboration with the public-private council

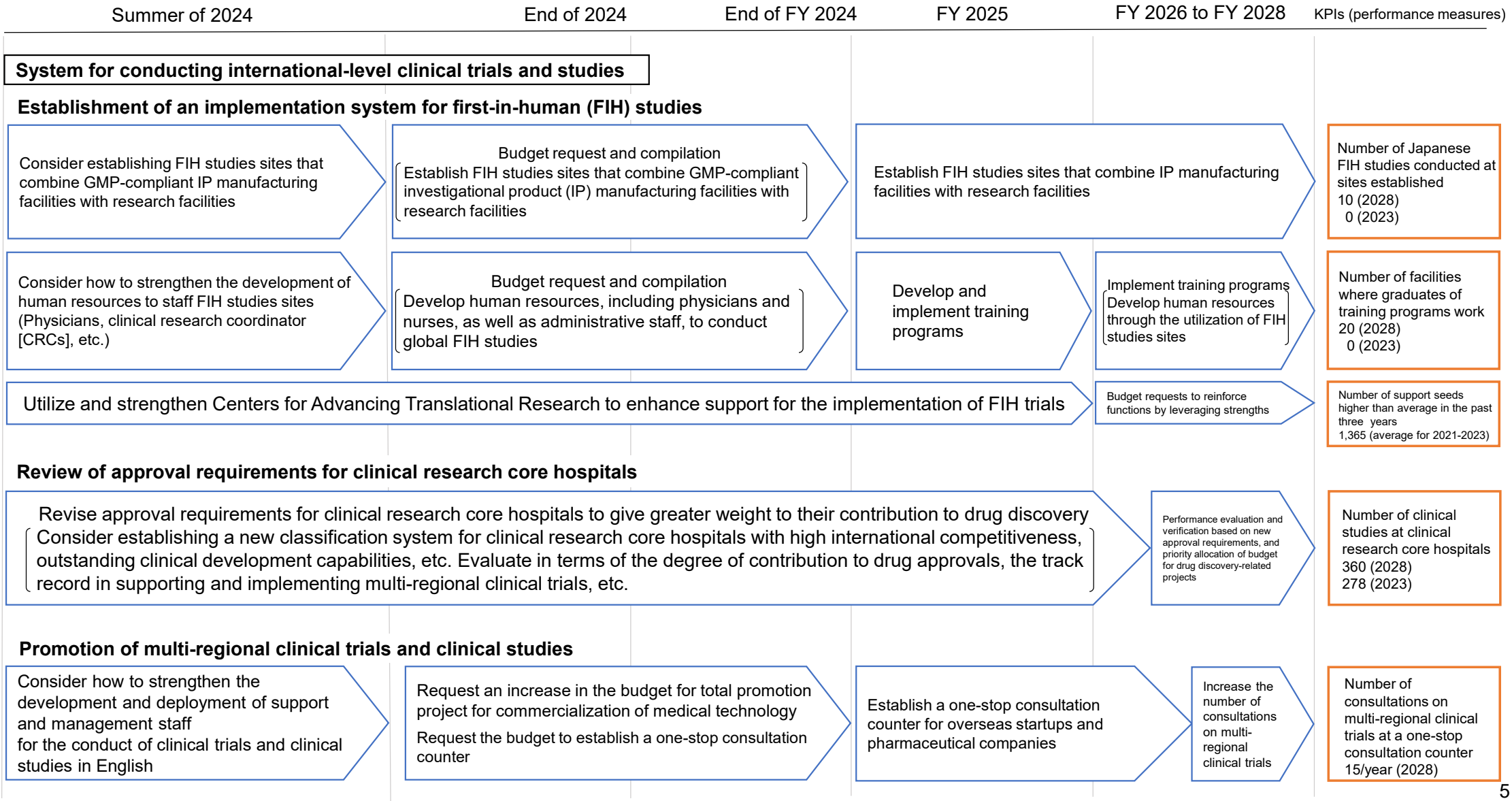
Secure human resources who can lead exit-oriented research and development (R&D) in collaboration with various players (also in collaboration with the public-private council)

- Consider measures to secure human resources or functions (regulatory personnel, contract research organizations, human resources for bridging, etc.) to bridge the gap between academic research and VC/pharmaceutical companies
- Budget request and compilation (Concretization of bridging projects)
- Based on the content of the discussion or examination by the public-private council, establish drug discovery startups through human resources and functions to bridge the gap between academic research and VCs and pharmaceutical companies

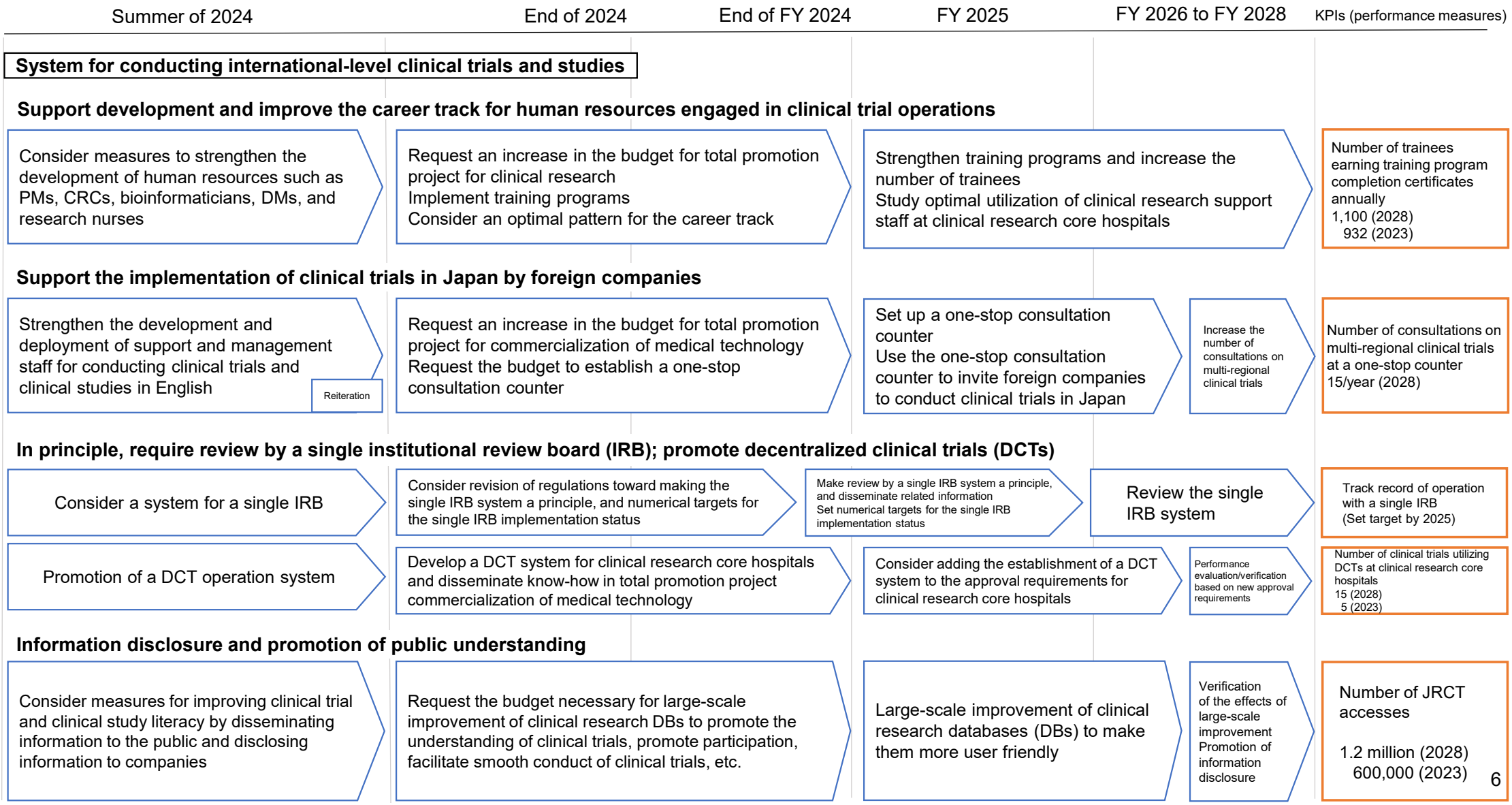
KPIs (performance measures)

- Number of foreign-affiliated companies with innovation promotion bases or innovation promotion programs in Japan
  14 (2028)
  7 (2023)
- Number of Pharmaceutical Startups adopted
  11 -> 70 companies
  (2023 -> 2028)
- Number transitioning to corporate activities (start-up, licensing out, etc.) through projects shown on the left:
  8
  (2025-2028)

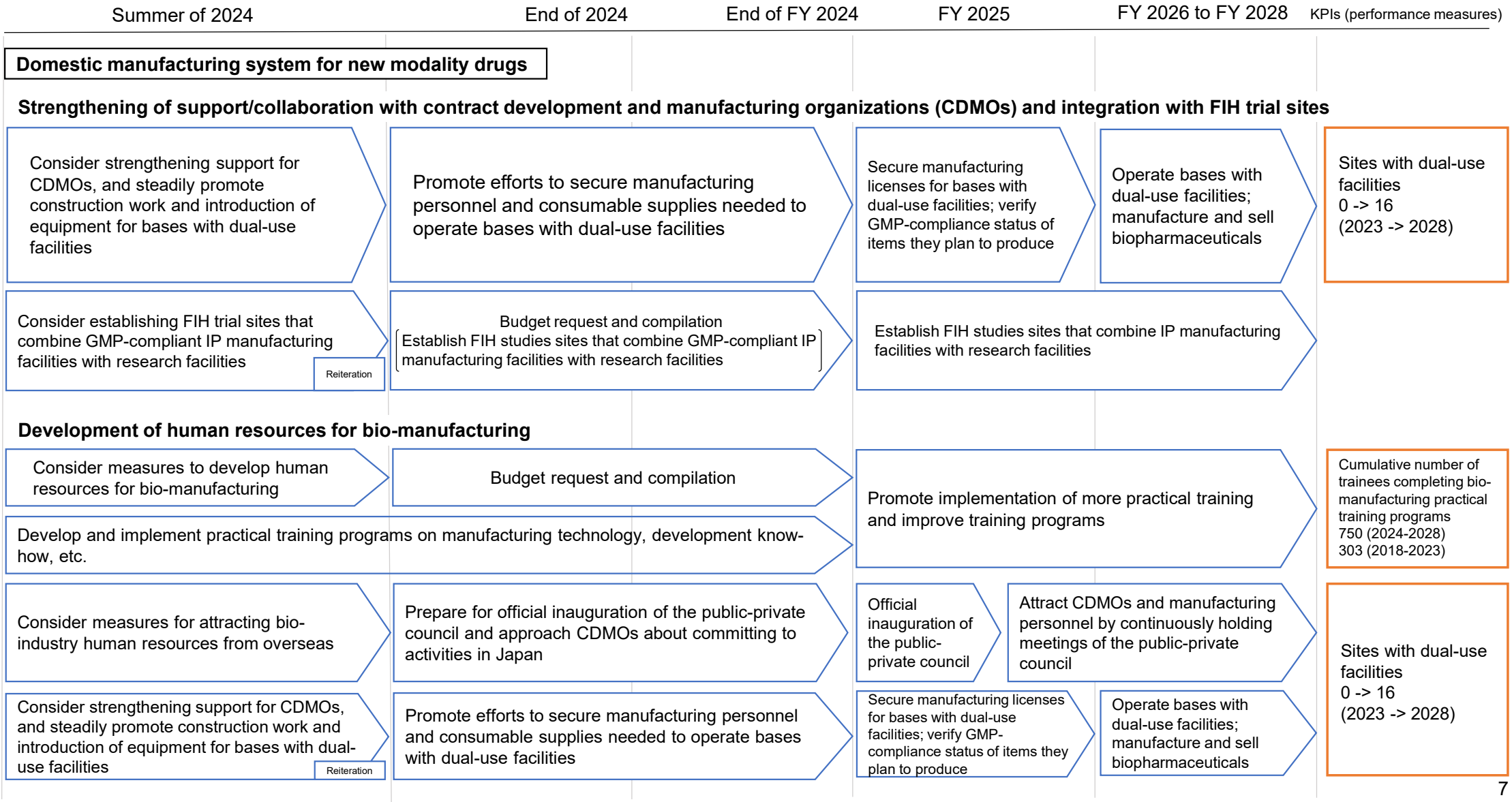
Schedules and flowcharts for each policy measure [2]



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.

(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)

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

Creation of an average of three or more innovative drug discovery seeds per year (2028) <- 12 cases in six years from 2018 to 2023

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.

Schedules and flowcharts for each policy measure [6]

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 [2]

Promote the creation of a suitable environment for improving the R&D capabilities of university hospitals

Proactively promote operational efficiency through medical DX, AI, etc., so that physicians at university hospitals will be allocated sufficient resources for research and development

Through hearings at each university hospital, collect cases that show favorable results after revising medical care systems through the promotion of medical DX, etc., to ensure that physicians at university hospitals have time for research

Steadily implement support to accelerate R&D on AI in the healthcare field

Promote the efforts described on the left and help ensure more research time

Spread and promote initiatives such as utilization of the buy-out system and accounting of principal investigators' personnel expenses as direct expenses in competitive research funding

Strengthen clinical research at universities and university hospitals ([1] integrated basic/clinical research and [2] special types of clinical research)

Promote the creation of a suitable environment for improving the R&D capabilities of university hospitals that provide advanced medical care and conduct clinical studies

Based on the research capabilities and achievements, consider priority research support measures with clearly defined objectives that also utilize competitive research funding and target both university hospitals and medical schools Consider utilizing incentives such as competitive research funding in building the framework for efforts to ensure research time, promote collaboration with researchers, including doctoral degree holders, in other fields and industries, and facilitate the mobilization of human resources in and outside of Japan

Implement various necessary measures based on the efforts described on the left

When considering the optimal form for human resource and organizational management systems that integrate the management of educational and research organizations at medical schools with that of clinical practice organizations at university hospitals, collect cases from overseas as well; consider researching mechanisms for facilitating more flexible recruitment of diverse human resources and young researchers from the viewpoint of strengthening R&D capabilities

Conduct and compile research on the organizational management systems of university hospitals, etc.

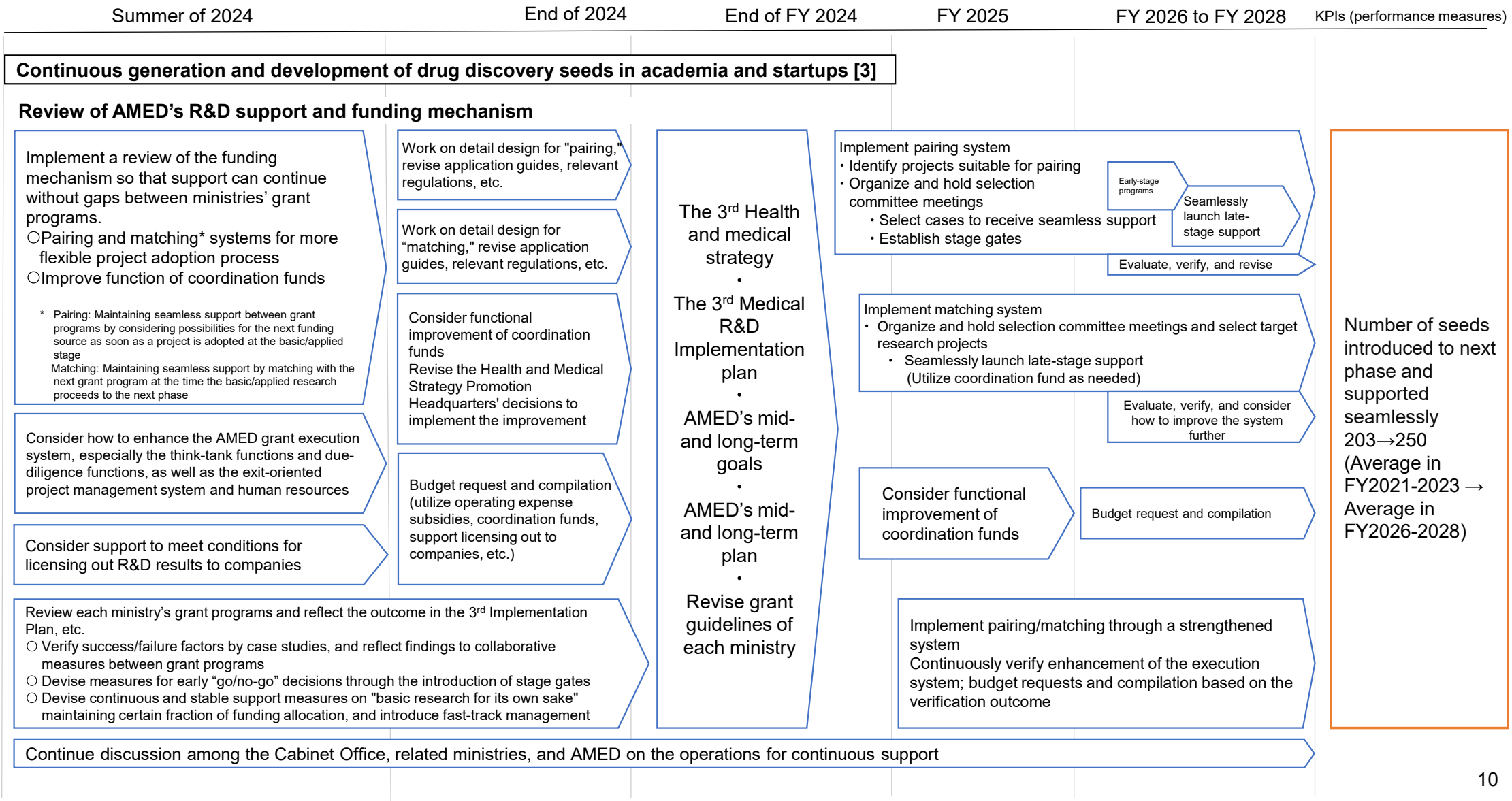
To strengthen the R&D capabilities of university hospitals, promote the clarification of basic policy on the roles and functions of each university hospital in terms of education, research, medical care, etc., while considering regional medical circumstances, among others; confirm and analyze the current status, challenges, and future plans for education, research, medical care, etc., at each university hospital, including the current status of grants for operating expenses of national universities, etc.

Based on the analysis described on the left, together with the policies of university hospitals and regional circumstances, consider the ideal form for university hospitals as well as measures that can be expected to strengthen their research capabilities

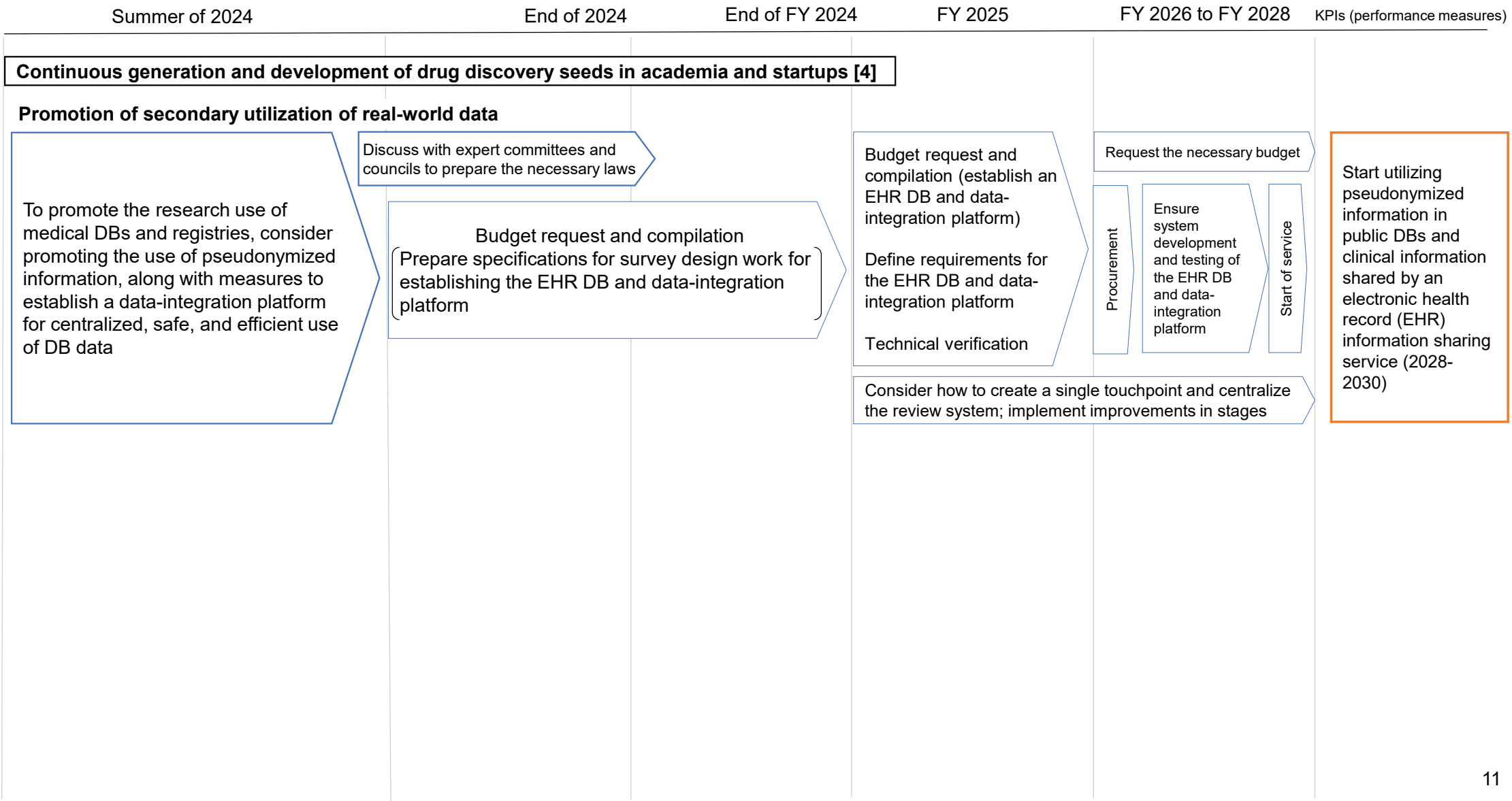
Implement various necessary measures based on the efforts described on the left

Increase the proportion of research hours among the total working hours of physicians at university hospitals (29.8% in 2018); steadily increase the number of research papers in the top 10% (average 1,139 clinical medicine papers from 2019 to 2021)

Schedules and flowcharts for each policy measure [7]

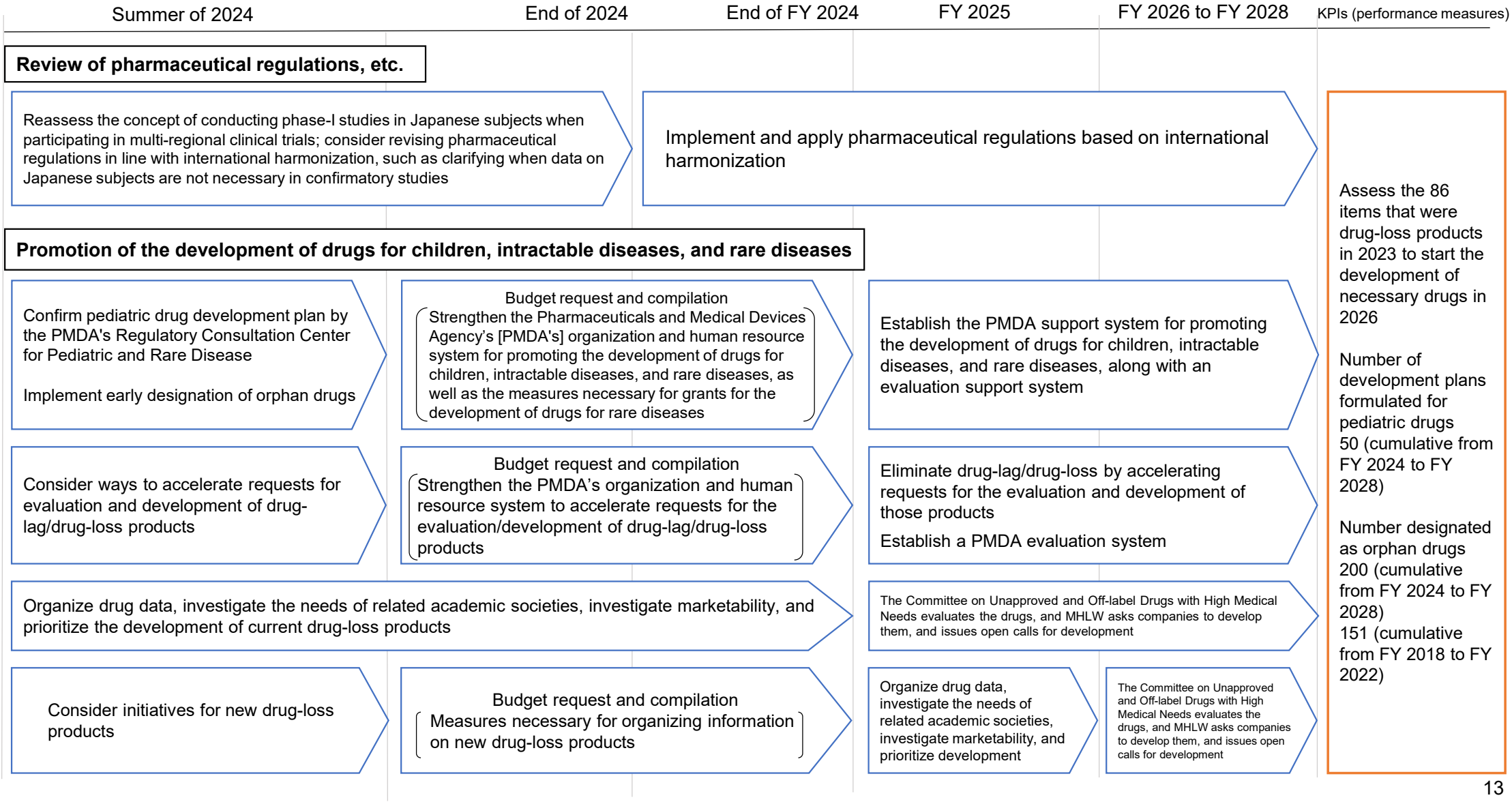


Schedules and flowcharts for each policy measure [8]



2. Prompt Delivery of Novel Drugs to Patients

Schedules and flowcharts for each policy measure [1]



Schedules and flowcharts for each policy measure [2]

Summer of 2024	End of 2024	End of FY 2024	FY 2025	FY 2026 to FY 2028	KPIs (performance measures)

PMDA's consultation/review system

Consultation and support to promote the clinical application of new modality drugs

Consider how to improve early-stage access to PMDA's consultation and support system on pharmaceutical regulations for startups and academia

Budget request and compilation
(Measures necessary to strengthen PMDA's organization and human resource system for handling consultations at an early stage)

Establish the PMDA organization/human resource system
Implement consultations on startups, etc.
Publicize and spread early consideration of new modality drugs

Anglicization of processes and documents and participation in the framework of international joint review

Promote anglicization of processes and documents and consider active participation in the cooperative framework for international review

Budget request and compilation
(Measures necessary for strengthening the PMDA's organization and human resource system and handling anglicization)

Accept English materials on a trial basis
Participate actively in a cooperative framework for international review
Coordinate with foreign authorities to promote regulatory harmonization
Establish the PMDA system for handling anglicization

Disseminating the message that pharmaceutical regulations in Japan are open internationally

Consider a plan on how to inform overseas startups of regulatory initiatives being taken in Japan

Establish a PMDA office in Washington, D.C.
Budget request and compilation
(Measures regulatory staff need to take to disseminate information overseas)

Promote publication of anglicized guidelines, etc.
Disseminate information on the Japanese system at overseas academic conferences, etc.
Operate PMDA's overseas offices and handle consultations

Assess the 86 items that were drug-loss products in 2023 to start the development of necessary drugs in 2026 (reiteration)

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

Schedules and flowcharts for each policy measure

Summer of 2024	End of 2024	End of FY 2024	FY 2025	FY 2026 to FY 2028	KPIs (performance measures)

Appropriate evaluation of the value of innovative drugs, departure from dependence on long-listed products, etc.

Verify the FY 2024 drug pricing system reform → Discuss reform of the FY 2026 drug pricing system → Discuss individual drug pricing system reforms

Assess the 86 items that were drug-loss products in 2023 to start the development of necessary drugs in 2026

Based on the report of the Council on Industrial Structure for the Realization of Stable Supply of Generic Drugs, while maintaining a stable supply of pharmaceuticals, promote structural reforms with an eye on the ideal form of the generic drug industry, with a view to industry restructuring, and develop a legal framework for stable supply

Consider support measures such as financial/fiscal measures (Request the budget as needed)

Consider the legal framework

Starting with those first realized, sequentially implement financial/fiscal measures, legal framework, etc.

Share of generic drugs by quantity: 80% or more in all prefectures (2028)
Unachieved in 11 prefectures (2023)
Share of generic drugs by monetary value: 65% or more (2029)
56.7%(2023)

Sort out legal issues related to the Antimonopoly Act to promote collaboration and cooperation between companies; Consider the establishment of a consultation desk, etc.

Promotion of self-care and self-medication by supporting the switch to over-the-counter (OTC) drugs, etc.

Consider programs to conduct highly versatile and effective educational activities that redirect behavior of users, along with verifying the effectiveness of the self-medication tax system

Receive requests for the OTC switch from academic societies, etc.; evaluate the validity of switching based on the Evaluation and Review Committee on Switching from Ethical Drugs to BTC/OTC Drugs; and promote the development and launch of switch OTC drugs

Spread health-related knowledge among the public through the Smart Life Project, e-health net, etc.
*Update the disseminated information as necessary and consider public awareness themes, based on the latest information and scientific knowledge

In principle, switch to OTC drugs by the end of 2026 for drugs that have already been switched to OTC in at least two other countries

Promote the use of biosimilars and utilize private insurance in addition to public insurance for new technologies

Formulate a roadmap for promoting the use of generics based on confirmation of the status of achievement in promoting the use of biosimilars, and discussion in council of experts, etc.

Implement and promote measures based on the roadmap to be created in FY 2024
Verify the effect of measures to promote the spread of biosimilars, and consider further efforts

Number of original biopharmaceuticals with 80%+ replacement by biosimilars
60%(2029)
25%(2023)

Expand the scope of the system of uninsured concomitant medical care expenses to enable quickly access to cutting-edge medical care the efficacy of which has not been sufficiently evaluated. Consider utilizing private insurance from the viewpoint of smooth access and reducing the burden on patients. While maintaining the universal health insurance system, consider making certain drugs available to patients upon request, such as drugs that have biosimilar or other alternatives and can be selected in treatment covered by health insurance.