Promote the strategy package facilitating all the process from R&D, clinical research/trials, pre- and post- marketing safety, insurance coverage, through globalization of innovative products which are to be put into practical use. Specifically, this package is targeting innovative pharmaceuticals/medical devices/regenerative medicine which can cure serious illnesses (such as rare diseases/cancer etc.) unless established therapy is available.

<table>
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<th>Prioritized Policy I</th>
<th>Prioritized Policy II</th>
<th>Scheme to rapid authorization of unapproved drug</th>
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<td><strong>SAKIGAKE</strong></td>
<td><em><em>NHI</em> Price Listing</em>*</td>
<td><strong>facilitate the environment for industry activities</strong></td>
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**Accelerate R&D through supporting each stage**

- **Pre-Clinical Research**
  - Coalition between “Network for Drug Discovery” and “Pharmaceutical Affairs Consultation on Research and Development (R&D) Strategy”
  - Support of Drug- Repositioning (DR) and development of off-label use
  - Development of safety assessment technique for using iPS derived cells followed by international standardization
  - R&D through public-private joint project

- **Clinical Research /Trial**
  - High-quality clinical trials by Clinical Trial Core Hospital·NC and coalition with research group for rare diseases
  - Support for orphan drug R&D
  - Support for ultra-orphan through the R&D to Early designation
  - Support for Drug Development through Medical Information and Communication Technology (MICT)
    - DB of Medical Information
    - Rapid and effective Clinical Trials
    - Incorporation into review for approval

- **Approval**
  - Analysis by Modeling and Simulation (M&S) conducted by PMDA
  - Improve the predictability of NHI drug price
  - Discussion on Premium to promote the development of new drugs and to eliminate off-label use
  - Utilizing “Pre-application Consultation”

- **NHI* Price Listing**
  - Strengthening industry competitiveness
    - tax incentive
    - HR Development
  - Support for SME and venture
    - Discussion on funding system for review user fee to be implemented
  - Utilization of the data from clinical research of rare disease / cancer for post-marketing surveillance

- **International Deployment**
  - Mutual understanding of the process from R&D to approval with the trading partner, to promote export
  - Strengthening measures on post-marketing safety
  - Development of system of patient registry
  - Research on biomarker
  - Utilization of “Pre-application Consultation”

**Strengthen the structure of PMDA** (consultation, review, safety measures in terms of quality and quantity)

**Promotion of Regulatory Science** (Developing guidelines/assessment for the state-of-the-art technology)
SAKIGAKE Designation System

**Designation Criteria**

Medical products for diseases in urgent need of innovative therapy which may satisfy the following two conditions:

1. Having firstly developed in Japan and planned an application for approvals (desired to have PMDA consultation from the beginning of R&D)
2. Prominent effectiveness (i.e. radical improvement compared to existing therapy), can be expected based on the data of mechanism of action, non-clinical study and early phase of clinical trials (phase I to II)

**Designation Advantage**

1. **Prioritized Consultation**
   - *Waiting time: 2 months → 1 month*
   - Shortening a waiting time for a clinical trial consultation from the submission of materials.

2. **Substantial Pre-application Consultation**
   - *[de facto review before application]*
   - Encouraging Consultation
   - Accepting materials in English

3. **Prioritized Review**
   - *[12 months → 6 months]*
   - Targeting total reviewing time: 6 months
   - Accept the result of phase III study after the application on a case-by-case basis to shorten the time from R&D to approval

4. **Review Partner**
   - *[PMDA manager as a concierge]*
   - Assign a manager as a concierge to take on overall management for the whole process toward approval including conformity assurance, quality management, safety measures, and reviewing application

5. **Substantial Post-Marketing Safety Measures**
   - *[Extension of re-examination period]*
   - Strengthening post-marketing safety measures such as extension of re-examination period after approvals as well as facilitating coalition with scientific societies, and global information dissemination.

**Designation Procedure**

1. **Option 1**: Application is to be submitted to Evaluation and Licensing Division (ELD) and to be reviewed by PMDA. The result of designation is to be notified within 60 days.

2. **Option 2**: ELD is to approach a potential applicant. The result of designation is to be notified within 30 days after the submission, if agreed by the applicant.

SAKIGAKE is a system to put into practice innovative medicines/medical devices/regenerative medicines initially developed by Japan.
General Timeframe of SAKIGAKE

【Ordinal Review】

1. Priority Consultation
   - Consultation on Clinical Trial
   - 2 months

2. Prior Review
   - Consultation on Clinical Trial
   - 6 months

3. Priority Review
   - Consultation on Clinical Trial
   - 12 months

4. Review Partner
   - Consultation on Clinical Trial
   - 1 month

5. Practical application of innovative medical products

【Review under SAKIGAKE Designation System】

1. Priority Consultation
   - Consultation on Clinical Trial
   - 2 months

2. Prior Review
   - Consultation on Clinical Trial
   - 6 months

3. Priority Review
   - Consultation on Clinical Trial
   - 12 months

4. Review Partner
   - Consultation on Clinical Trial
   - 1 month

5. Strengthening post-marketing safety measures (re-evaluation period)
Scheme for Rapid Authorization of Unapproved Drug

Expand the scope of the Council on Unapproved Drug / Off-label Use to the products unapproved in EU/US, when satisfying certain conditions. Through the cooperation with industry on R&D for the products, lead the world in the practical use of innovative pharmaceuticals for life threatening rare/serious diseases.

Facilitate the environment for industries and support its R&D through proactive conduct of clinical trials or Advanced Medical Care at Clinical Trials Core Hospitals, and National Center for Advanced Medical Technology for products which have difficulty to make matching the data with company developing the product.

**Unapproved drug / Off-label Use**

(currently limited only to products approved in EU or US)

Accept and evaluate the as needed

Expand the current scope to products unapproved in EU/US if they satisfy one of the following conditions

1. Conducting/finalizing phase III study in Japan
2. Promising clinical data shown in public domain such as a paper in scientific journals
3. Achievement in Advanced Medical Care B

Evaluation committee on unapproved or off-labeled drugs with high medical needs

**Basic Scheme** (Almost all products fall into the scheme)

Request on a company / Public recruiting of company for R&D

Clinical Trial to be conducted by company

Submission of Application for Approval

**Where it takes time for matching due to R&D carried out overseas, etc.**

Clinical trials / Advanced Medical Care to be conducted at Clinical Trials Core Hospitals / National Center for Advanced Medical Technology to accumulate data enough for application

※Support the company for its R&D
※Utilize PMDA’s Pharmaceutical Affairs Consultation on Research and Development (R&D) Strategy

Company conducting R&D