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# Regulatory Updates on Regenerative Medical Products in Japan

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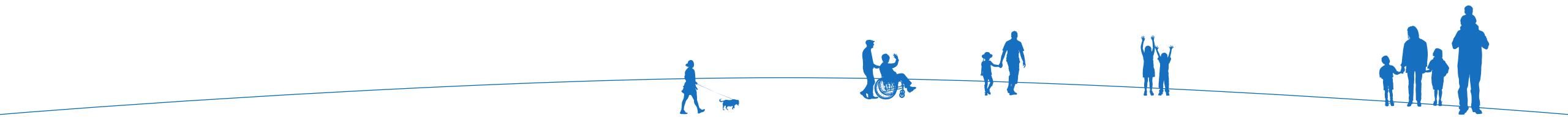
**The views and opinions expressed in this presentation are those of the presenter and should not necessarily represent the views and opinions of the PMDA.**



# Regenerative Medical Products approved in Japan

| Brand name | Category | Orphan | SAKIGAKE | Conditional & Time-limited Approval |
|---|---|---|---|---|
| Abecma | CAR-T | ✓ | | |
| Breyanzi | CAR-T | ✓ | | |
| CARVYKTI | CAR-T | ✓ | | |
| Delytact | Oncolytic virus | ✓ | ✓ | ✓ |
| Kymriah | CAR-T | ✓ | | |
| YESCARTA | CAR-T | ✓ | | |
| LUXTRNA | AAV | ✓ | | |
| Nepic | Somatic stem cell | ✓ | | |
| Ocural | Somatic stem cell | ✓ | | |
| Sakracy | Somatic stem cell | ✓ | | |
| Vyznova | Somatic cell | ✓ | | |
| Akuugo | Somatic stem cell | ✓ | ✓ | ✓ |
| ELEVIDYS | AAV | ✓ | | ✓ |
| STEMIRAC | Somatic stem cell | | ✓ | ✓ |
| ZOLGENSMA | AAV | ✓ | ✓ | |
| Collategene | Plasmid vector | | | ✓(expired (27 June 2024)) |
| HeartSheet | Somatic stem cell | | | ✓(withdrawn (25 July 2024)) |
| JACE | Somatic cell | ✓GCMN*, EB** | | |
| JACEMIN | Somatic cell | | | |
| Vyjuvek | HSV | ✓ | | |
| Alofice l | Somatic stem cell | ✓ | | |
| JACC | Somatic cell | | | |
| TEMCELL | Somatic stem cell | ✓ | | |

| Area of disease | No. |
|---|---|
| Oncology | 6 |
| Ophthalmology | 5 |
| Brain, Nerve | 4 |
| Circulation | (2) |
| Dermatology | 3 |
| Others | 3 |

**21(2)**

*GCMN: Giant congenital melanocytic nevi, **EB: Dystrophic epidermolysis bullosa

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# Orphan Designation

- Objective

  To promote R&D of the products for rare diseases to provide the patients with safe and effective therapies as early as possible

- Criteria for designation Notification in English: https://www.pmda.go.jp/files/000268408.pdf

  **1. Number of patients** (< 50,000 patients in Japan or one of the designated intractable diseases) (in Japanese)

  **2. Medical needs** (Serious diseases with high medical needs)
  **3. Possibility of development**

- Incentive (underlines; PMDA-related)
  - Grant-in-Aid for R&D of orphan designated drugs
  - Tax deduction for R&D expenses
  - Priority scientific consultation
  - Priority review (9 months; compared to 12 months for standard)
  - Premium drug pricing
  - Extension of re-examination period

# SAKIGAKE Designation

- Objective

  To put innovative products into medical practice faster in Japan

- Criteria for designation Notification in Japanese: https://www.pmda.go.jp/files/000240376.pdf

  **1. Innovativeness** - Novel mode of action (in principle)
  **2. Severity of target disease** - Life-threatening or no curative therapies available
  **3. Prominent efficacy** - No existing therapies, or probable significant improvement in efficacy or safety compared to existing therapies
  **4. Plan / System** - Submit the Marketing authorization application (MAA) in Japan first or at the same timing (within 3 months) as the first MAA submission in other regions

- Incentive (underlines; PMDA-related)
  - Concierge service offered by senior review partner
  - Priority scientific consultation
  - Pre-assessment in consultation
  - Priority review (6 months; compared to 12 months for standard)
  - Premium drug pricing
  - Extension of re-examination period

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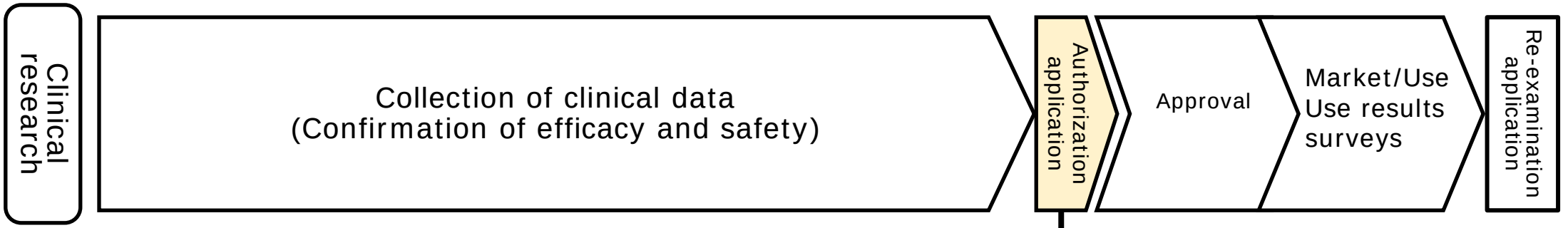
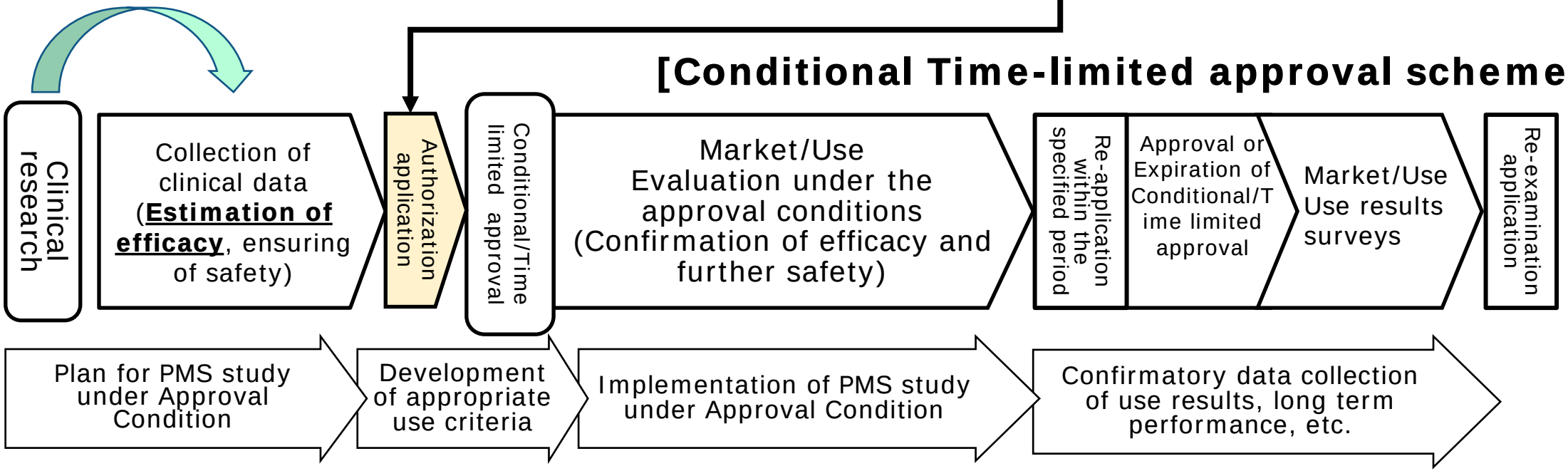
# Conditional and Time-limited Approval Scheme

## [Conventional approval scheme] (CTL Approval Scheme)




## [Conditional Time-limited approval scheme]




Maruyama et al., Adv Exp Med Biol, 1430, 155-179 (2023)

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# For development in Japan

PMDA 独立行政法人 医薬品医療機器総合機構
Pharmaceuticals and Medical Devices Agency

https://www.pmda.go.jp/english/int-activities/industry/0001.html

Safety Alert & Recalls/ Review Reports/ Package Inserts etc. | Formats DL

International Activities

## For Industry - for development in Japan

Add this page


1.Overview

2.Regulations and Services of PMDA

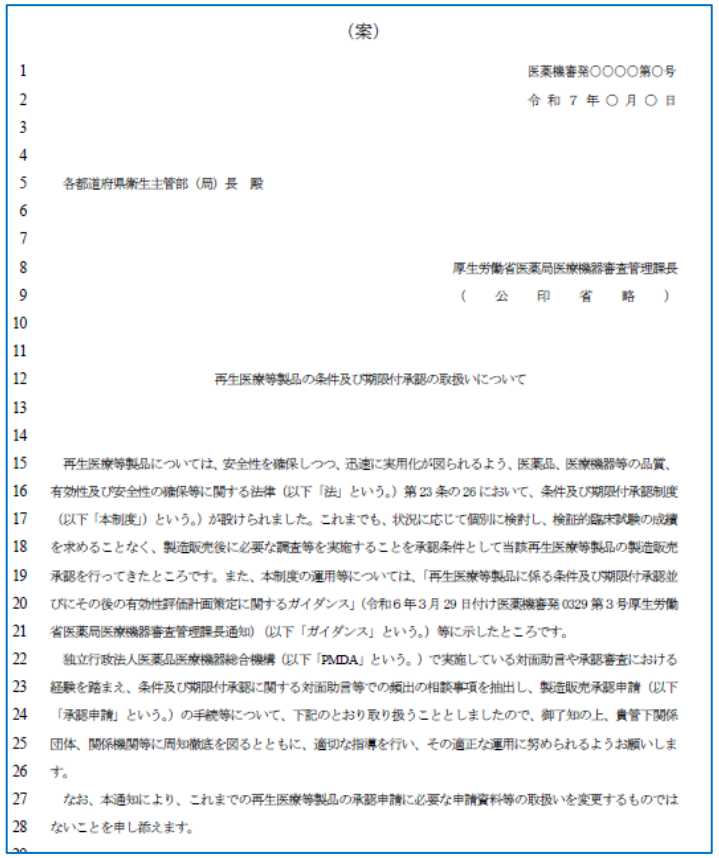
3.Information for Approved Products in Japan

4.Procedures in Japan

5.Advanced Efforts

6.Links

# Draft Notification: Handling of Conditional and Time-limited Approval Scheme



（案）

医薬機審発○○○○第○号
令 和 ７ 年 ○ 月 ○ 日

各都道府県衛生主管部（局）長 殿

厚生労働省医薬局医療機器審査管理課長
（ 公 印 省 略 ）

再生医療等製品の条件及び期限付承認の取扱いについて

再生医療等製品については、安全性を確保しつつ、迅速に実用化が図られるよう、医薬品、医療機器等の品質、有効性及び安全性の確保等に関する法律（以下「法」という。）第 23 条の 26 において、条件及び期限付承認制度（以下「本制度」という。）が設けられました。これまでも、状況に応じて個別に検討し、検証的臨床試験の成績を求めることなく、製造販売後に必要な調査等を実施することを承認条件として当該再生医療等製品の製造販売承認を行ってきたところです。また、本制度の運用等については、「再生医療等製品に係る条件及び期限付承認並びにその後の有効性評価計画策定に関するガイダンス」（令和６年３月 29 日付け医薬機審発 0329 第３号厚生労働省医薬局医療機器審査管理課長通知）（以下「ガイダンス」という。）等に示したところです。

独立行政法人医薬品医療機器総合機構（以下「PMDA」という。）で実施している対面助言や承認審査における経験を踏まえ、条件及び期限付承認に関する対面助言等での頻出の相談事項を抽出し、製造販売承認申請（以下「承認申請」という。）の手続等について、下記のとおり取り扱うこととしましたので、御了知の上、貴管下関係団体、関係機関等に周知徹底を図るとともに、適切な指導を行い、その適正な運用に努められるようお願いします。

なお、本通知により、これまでの再生医療等製品の承認申請に必要な申請資料等の取扱いを変更するものではないことを申し添えます。

https://public-comment.e-gov.go.jp/pcm/detail?CLASSNAME=PCMMSTDETAIL&id=495250120&Mode=0
(in Japanese) (Published for public consultation on 25 July 2025)

- Public consultation ended on 25 August 2025
- To clarify the procedures of CTL Approval Scheme based on accumulated experience through scientific consultation and assessment

- Content
  1. Procedures for CTL Approval
     (1) Scientific consultation for pre-application
     (2) Marketing authorization application and assessment
  2. Procedures for post-approval
  3. Procedures for re-application within the specified period
  4. Utilization of the CTL Approval Scheme for already-approved products overseas for rare diseases, and basic perspective of Japanese clinical data in such cases
  5. Perspective of Quality Control Strategy considering Product Lifecycle

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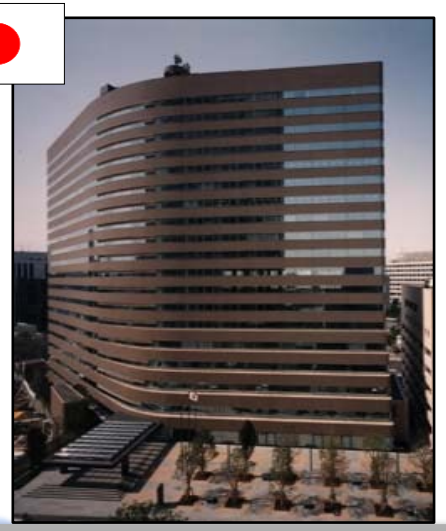
# PMDA's International Hubs

Asia Office, Bangkok



PMDA Central Office, Tokyo

Washington D.C Office

**Establishment of PMDA's international hubs**
**to enhance international contribution/capability for regulatory proposal under PMDA's 5th mid-term plan**

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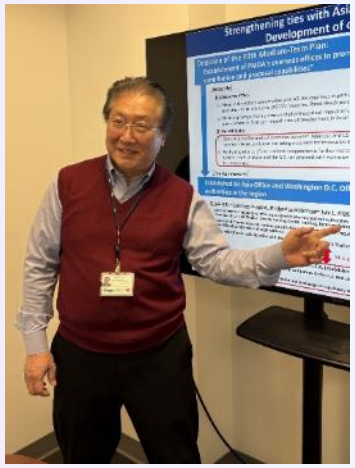
# PMDA Washington D.C. Office Staff Members

**Akihiro Ishiguro**

**Head**



**Koki Fukuhara**

**Senior Technical Officer**

**Hiroyuki Okayama**

**Administrative Manager**


**1730 Rhode Island Avenue, NW, Suite 403,**
**Washington, D.C. 20036 USA**

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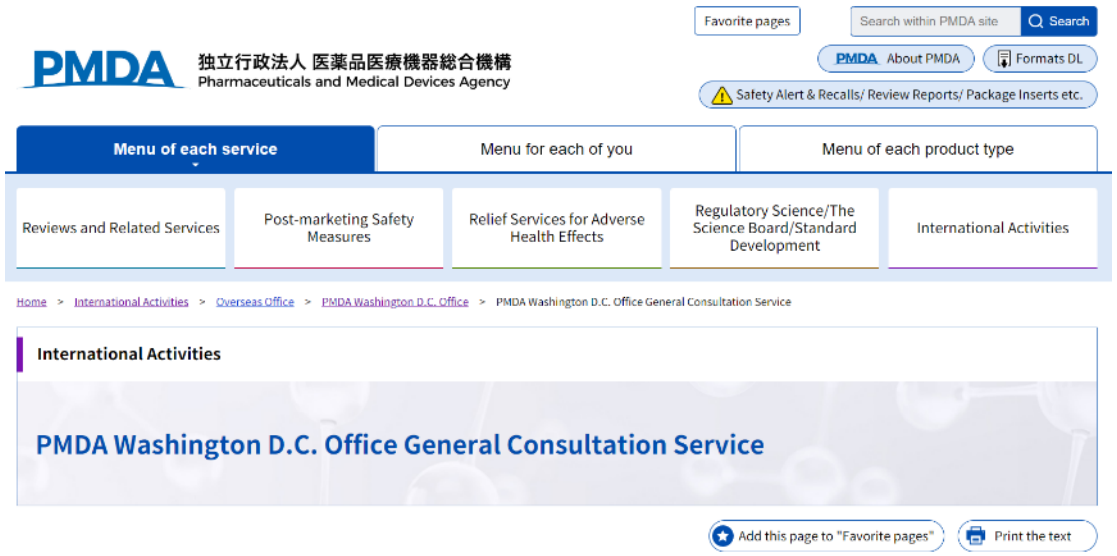
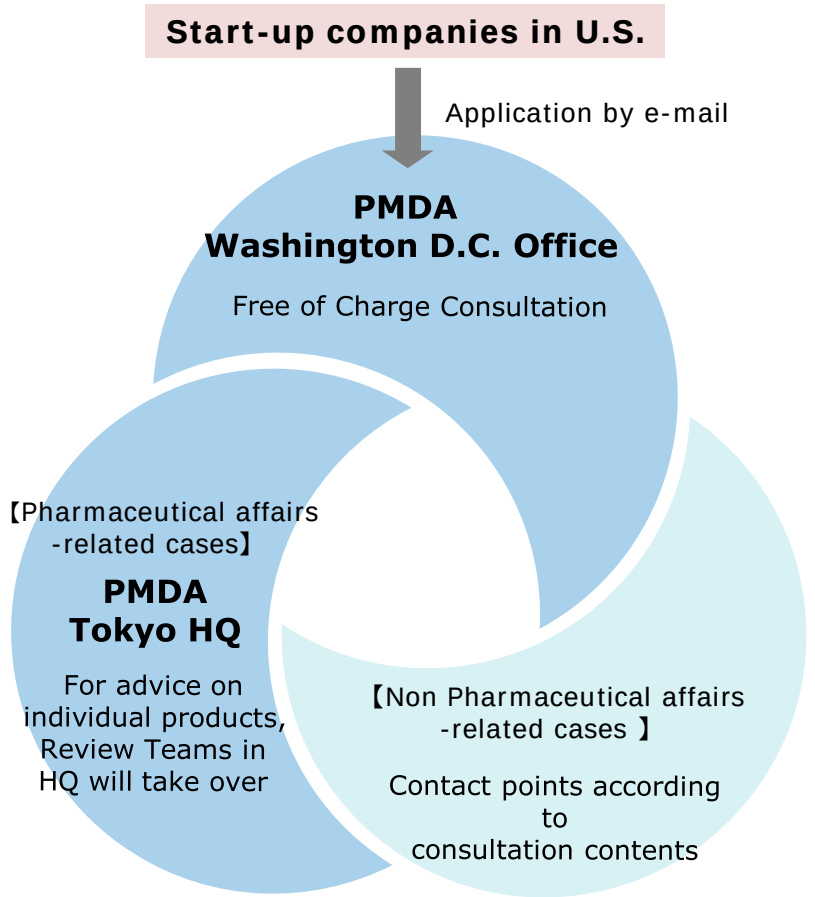
# General Consultation Service at Washington D.C. Office

Application for General Consultation




- Guide for better understanding PMD Act
- Provide initial general comment on Japanese regulatory information to U.S. start-up companies
- Support procedures for formal consultation at PMDA Tokyo Office

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# PMDA Asian Training Center

PMDA-ATC

- Established in 2016
- contribute to universal health coverage in Asia through regulatory harmonization and capacity building

**2016–2024**

Participated from **76** countries/regions

All Participants **3,559** / Participants from Asia **3,067**

**PMDA-ATC in numbers**

Total number of seminars held by PMDA-ATC (FY2016-2024)

**120** times

Total number of Pmda Channel (YouTube) videos released by PMDA-ATC

**48** videos

Total number of e-learning course videos for overseas regulatory authorities released by PMDA-ATC

**58** videos

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# PMDA-ATC Review of CGTPs Seminar 2025 for South-East Asian countries (15-17, July 2025)


**Comments from the participants:**

*"I find the case studies interesting and so much I have learned from there."*

*"This seminar helped me deepen my understanding on CGTPs and learned key points for consideration in reviewing CGTPs in my home country."*

*"I will apply the knowledge from the seminar to develop CGTP guidelines on registration."*

*"As we have on-going review of a CGTP application, this seminar helped me to see the bigger picture of the evaluation of CGTPs."*

https://www.pmda.go.jp/english/symposia/0338.html

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# Where to Find Information?

## Regenerative Medical Products

### 2. Approved Regenerative Medical Prodcuts

- List of Approved Products
- Review Reports
- Revisions of PRECAUTIONS

### 3. Support to Venture Companies

- PMDA's Support to Venture Companies [2.1MB]
- Doing business in Japan: What is attracting? [1.4 MB]
- Supports from Japanese government (MHLW)

### 4. Related Guidelines and Notifications for Regenerative Medical Products

#### Marketing authorization application (MAA)

- Questions and answers (Q&A) on release testing of the imported regenerative medical products, PSB/MDED, PSB/CLD Administrative Notice, 2024. [Japanese [144KB]] [English [143KB]]
- Guideline for comparability of human cell-processed products subject to changes in their manufacturing process, PSB/MDED Notification No. 0329-1, 2024. [Japanese [431KB]] [English [245KB]]

#### Good tissue practice (GTP)

- Standards for biological raw materials, MHLW Notification No. 210, 2003. [Japanese [315KB]] [English [73KB]]

## Review Reports: Regenerative Medical Products

A

| Brand Name | Non-proprietary Name | Approved In | English | Japanese |
|---|---|---|---|---|
| Abecma<br>Initial Approval | Idecabtagene vicleucel | January 2022 | | |
| Abecma<br>Partial Change Approval | Idecabtagene vicleucel | December 2023 | English [607.25 KB] | Japanese [847.24 KB] |
| Alofisel | Darvadstrocel | September 2021 | | |

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B

| Brand Name | Non-proprietary Name | Approved In | English | Japanese |
|---|---|---|---|---|
| Breyanzi<br>Initial Approval | Lisocabtagene maraleucel | March 2021 | | |
| Breyanzi<br>Partial Change Approval | Lisocabtagene maraleucel | December 2022 | | |

# Thank you for your attention!

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## Acknowledgements

Colleagues in the Office of Cellular and Tissue-based Products, PMDA