CGTP Summit 2026, Bethesda North Marriot Hotel & Conference Center, 8 June 2026
CGTP Symposium 2026, Bethesda North Marriot Hotel & Conference Center, 9-11 June 2026

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Current Status of Regenerative Medical Products in Japan

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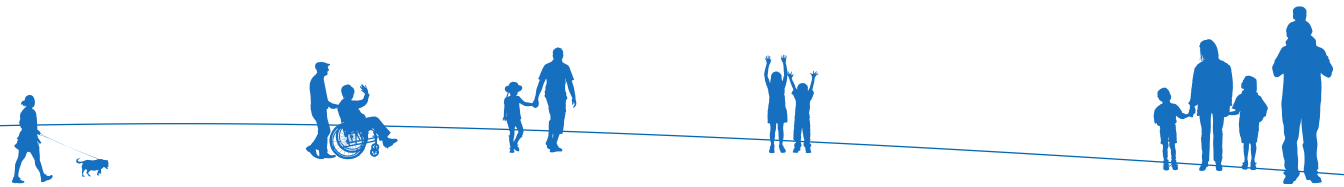
The views and opinions expressed in this presentation are those of the presenter and should not necessarily represent the views and opinions of the PMDA.



Regulatory History and Status of RMPs

"Regenerative Medical Products" (RMPs) has been defined under the Act on Pharmaceuticals and Medical Devices (PMD. Act)

As of 8 June, 2026

continuously published/updated relevant guidelines

25Nov.2014

2014 2015 2018 2019 2020 2021 2022 2023 2024 2025 2026

in vivo Gene therapy 7 (1)

- 26Mar.2019 Collategene → 27Jun.2024 Expired
- 11Jun.2021 Delytact
- 26Jun.2023 Luxturna
- 13May2025 Elevidys
- 8May2026 ADSTILADRIN
- 19Mar.2020 ZOLGENSMA
- 24Jul.2025 Vyjuvek
- 8Jun.2026 Telomelysin

ex vivo Gene therapy 5

- 26Mar.2019 Kymriah
- 22Jan.2021 YESCARTA
- 26Sep.2022 Carvykti
- 22Mar.2021 Breyanzi
- 20Jan.2022 Abecma

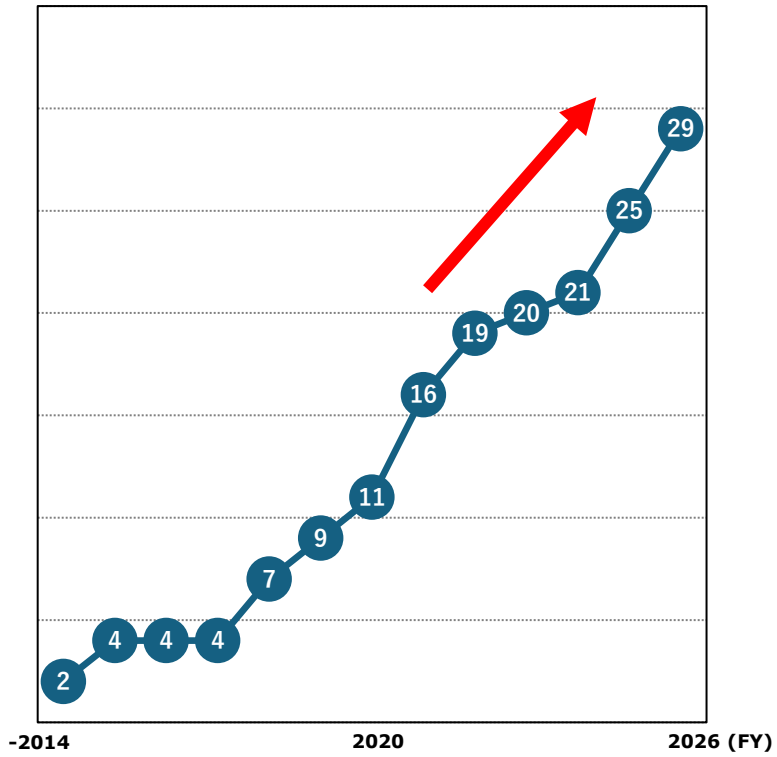
Cell/Tissue-based therapy 15 (1)

- 29Oct.2007 JACE
- 27Jul.2012 JACC
- 19Sep.2015 HeartSheet → 25Jul.2024 withdrawn
- TEMCELL
- 28Dec.2018 STEMIRAC
- 19Mar.2020 Nepic
- 11Jun.2021 Ocural
- 27Sep.2021 Alofisel
- 20Jan.2022 Sakracy
- 17Mar.2023 JACEMIN
- Vyznova
- 31Jul.2024 Akuugo
- 6Mar.2026 Amchepry
- RiHeart
- 3Apr.2025 Allostem Sheet
- 8May2026 SAVYSCUS

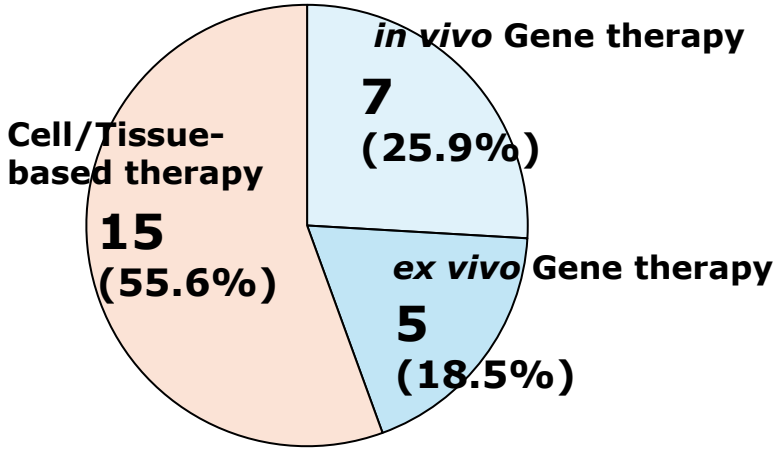
: Conditional and Time-limited (CTL) approval
: Orphan Designation
: SAKIGAKE Designation

Approved RMPs in Japan: Key Statistics and Trends

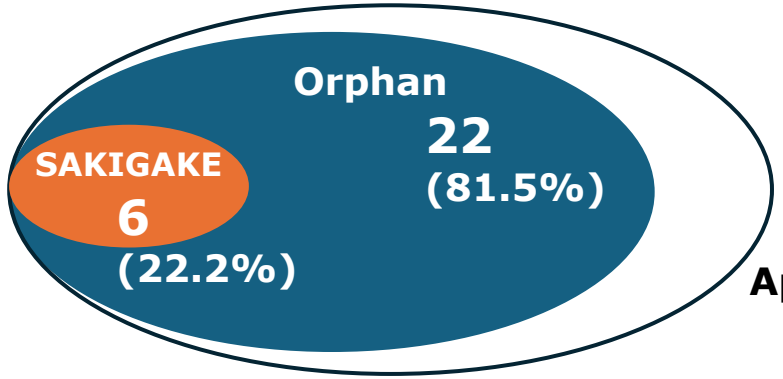
As of 8 June, 2026



No. of approved RMPs (Fig.1)

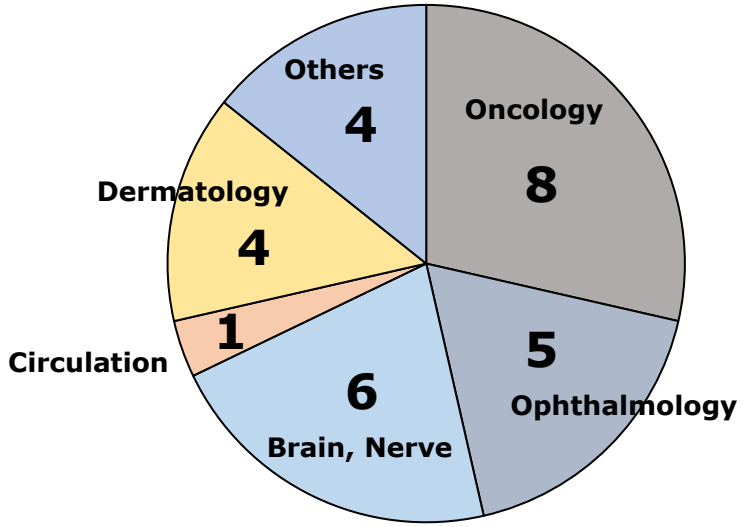


Type (Fig.2)



Approved products
27

Designation (Fig.3)



Area of disease (Fig.4)

Fig.1: Approval includes two products that were withdrawn or expired. Figs.2-4: Excludes two withdrawn or expired approvals
Figs.3 and 4: Based on the originally approved indication; indication extensions not considered

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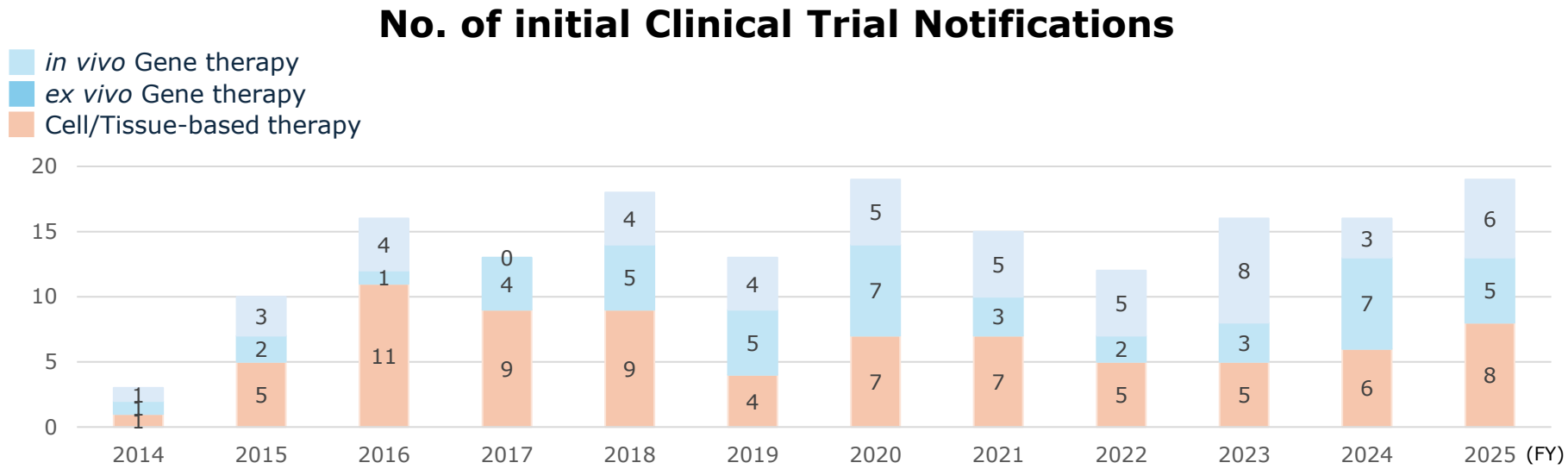
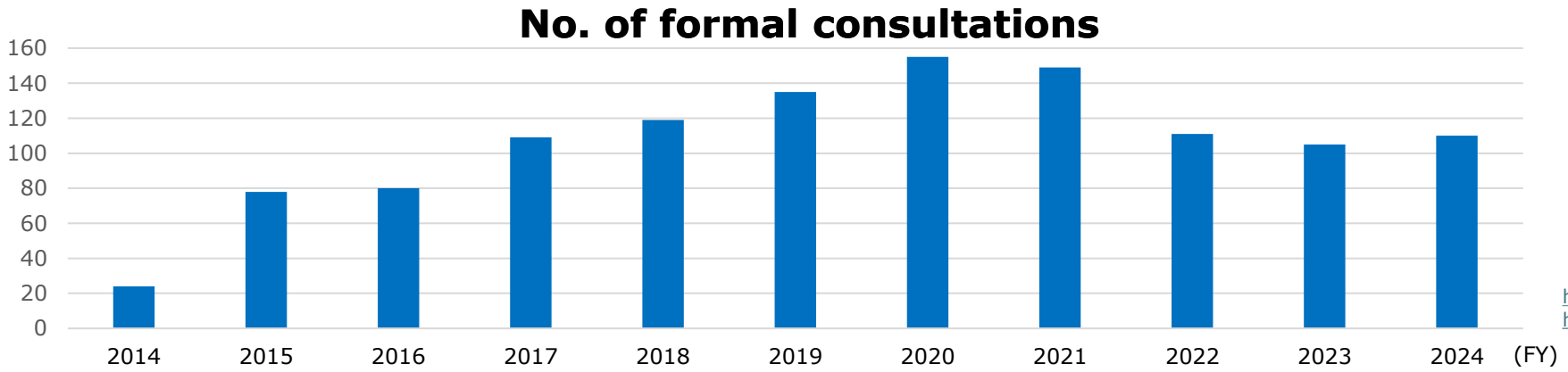
No. of Clinical Trial Notifications/consultations





https://www.pmda.go.jp/files/000271121.pdf
https://www.pmda.go.jp/files/000265314.pdf

The number of initial CTNs and consultations were increased dramatically when the PMD. Act was enacted, and they have remained constantly high for the last decade.

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Regulatory Reform in 2014

Safety Act — The Act on the Safety of Regenerative Medicine

PMD Act — Revision of the Pharmaceutical Affaires Law:
The Act on Pharmaceuticals and Medical Devices

Enacted on 25 November 2014

2014

These two acts were promulgated in November 2013 by the Japanese Diet (Parliament) in line with the **Regenerative Medicine Promotion Act** in order to reform the pharmaceutical and medical regulation related to regenerative medicine.

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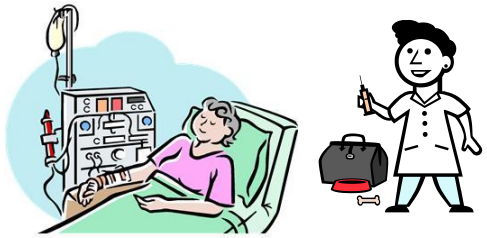
Two Acts Regulating Regenerative Medicine in Japan

Technology & **Product**

Regenerative Medicine

All medical **technologies** using processed cells which safety and efficacy have not yet been established



Safety Act

The Act on the Safety of Regenerative Medicine

Medical Care or Academic Research Purpose

Production and marketing of regenerative and cellular therapeutic **products** by firms

The Act on Pharmaceuticals and Medical Devices

PMD Act

Commercial Product Marketing Authorization Purpose

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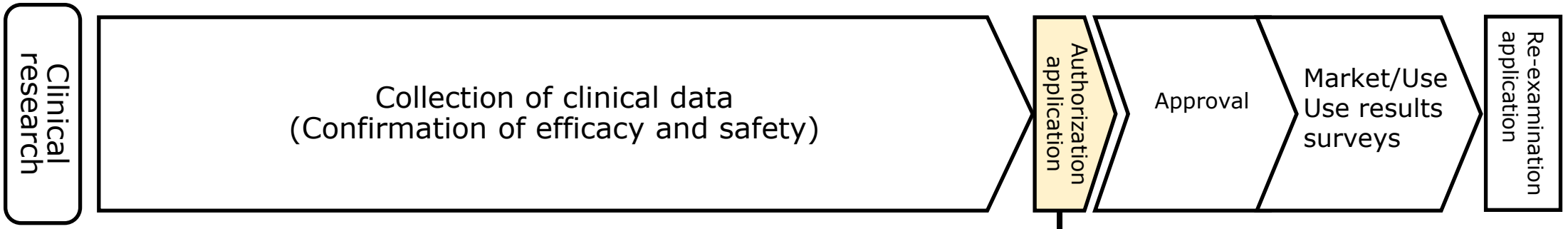
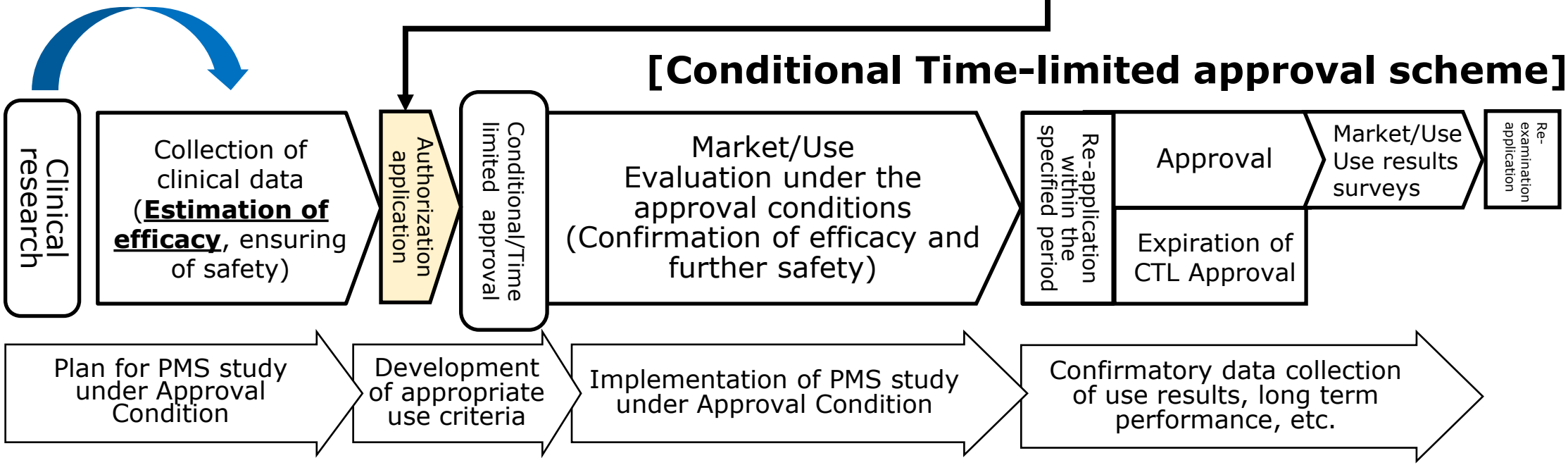
Conditional and Time-limited Approval Scheme

[Conventional approval scheme]

(CTL Approval Scheme)



[Conditional Time-limited approval scheme]



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

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Orphan Designation

- Objective

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

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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** - New 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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Where to Find Information?

Reviews and Related Services

Regenerative Medical Products

2. Approved Regenerative Medical Prodcuts

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

3. Support for Venture Companies

- For Industy - for Development in Japan

4. Related Guidelines and Notifications for Regenerative Medical Products

Marketing authorization application (MAA)

- Applications for Marketing Approval of Regenerative Medical Products, PFSB Notification No. 0812-30,2014. Partial revision on 2026. [Japanese[263KB]][English[143KB]]
- Points to Consider for Applications for Marketing Approval of Regenerative Medical Products, PFSB/ELD/OMDE/CMS Notification No. 0812-5, 2014. Partial revision on 2026. [Japanese[439KB]][English[218KB]]
- Guideline for Preparation of Attached Data and Overviews for Applications for Approval of Regenerative Medical Products[Japanese[1.04MB]][English[1000KB]]

Conditional and time-limited approval

- Guidance for conditional and time-limited approval for regenerative medical products and the development of subsequent efficacy evaluation plan, PSB/MDED Notification No.0329-3, 2024 [Japanese [431KB]] [English [249KB]]
- Handling of the Conditional and Time-Limited Approval for Regenerative Medical Products, PSB/MDED Notification No.1006-1, 2025 [Japanese [259KB]] [English [151KB]]

Reviews and Related Services

Review Reports: Regenerative Medical Products

The following English translations of review reports are intended to be a reference material to provide convenience for users. In the event of inconsistency between the Japanese originals and the translations, the former shall prevail. PMDA shall not be responsible for any consequence resulting from use of the English versions.

The review reports were selected for translation among those of new regenerative medical products that recently received marketing approval, in consideration of relevant factors including the novelty and priority.

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Brand Name: N to Z	N	O	P	Q	R	S	T	U	V	W	X	Y	Z

A

Brand Name	Non-proprietary Name	Approved In	English	Japanese
Abecma Initial Approval	Idecabtagene vicleucel	January 2022	English [2.34 MB]	Japanese [2.32 MB]
Abecma Partial Change Approval	Idecabtagene vicleucel	December 2023	English [607.25 KB]	Japanese [847.24 KB]
Akuugo	Vandefitemcel	July 2024 (Conditional and time-limited approval)	English [1.31 MB]	Japanese [4.88 MB]
Alofisel	Darvadstrocel	September 2021	English [953.40 KB]	Japanese [1.45 MB]

Thank you for your attention!

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Acknowledgements

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