Pioneering the Future of Regenerative Medicine: FIRM's Vision for Next-Generation Bioindustry

Forum for Innovative Regenerative Medicine (FIRM)
Chair, Steering Committee
Toshio Uemura

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Agenda

1. About FIRM
2. Characteristics of Regenerative Medicine Products
3. Current Trends in Japan & FIRM’s Activities

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The Forum for Innovative Regenerative Medicine (FIRM)

AT A GLANCE

188

member organizations

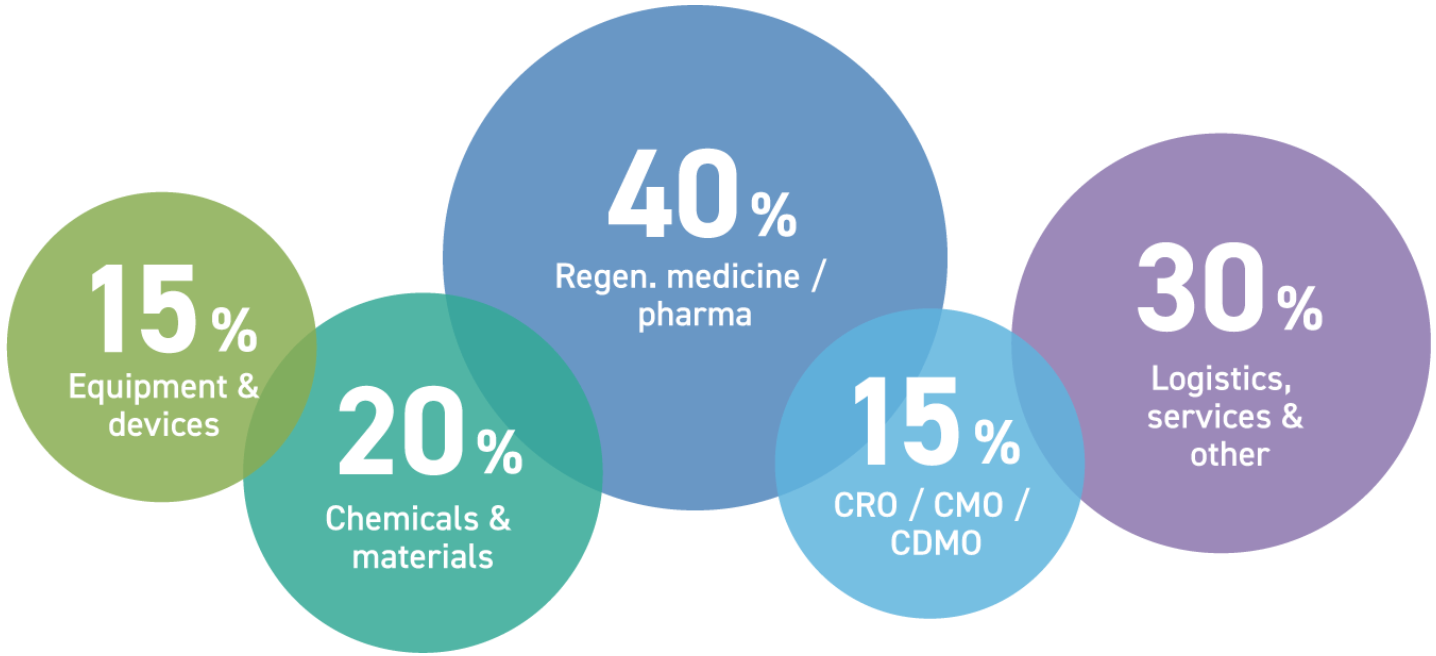
Founded

June 17, 2011

Mission

Accelerate the industrialization of regenerative medicine in Japan

ONE ECOSYSTEM, FIVE COMPLEMENTARY CAPABILITY POOLS



A single network to navigate Japan's R&D, manufacturing, supply and commercialization ecosystem for Regenerative Medicine

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FIRM Vision 2030

FIRM Vision 2030

Creating Meaningful Medical Value
by Unlocking the Full Potential
of Cell & Gene Therapy

Strategic Leadership & Innovation

Defining strategic priorities and strengthening policy advocacy to pioneer the future of cell and gene therapies.

Sustainable Value & Growth

Delivering sustainable value to society by nurturing next-generation global leaders who advance medical excellence and drive industrial innovation.

Trust & Integrity

Fostering ethical and transparent culture to build a leading brand trusted by all stakeholders.

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FIRM Vision 2030 – Seven challenges -

Funding Value-based pricing and reimbursement, and sustainable funding

Regulation Flexible, modality-appropriate regulation, regulatory innovation for emerging technologies, and international convergence

Global reach Global market development, regulatory convergence, and global engagement across Asia, Europe, and North America

Public trust Evidence generation, transparency, patient and public involvement, health literacy

Manufacturing Resilient domestic manufacturing and CDMO capacity, automation, as economic security

Data Patient registries, digitalization and utilization of manufacturing and quality data, and infrastructure for personalized medicine

Talent Next-generation leaders, bioengineering, biomanufacturing, AI/DX, data science, and workforce mobility

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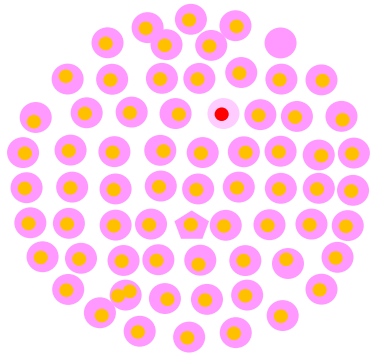
RMPs' different benefits and characteristics from existing pharmaceuticals

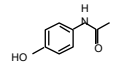
Characteristics of Regenerative Medicine Products

- Paradigm Shift in Medicinal Effects
- Long Duration
- High diversity
- Short shelf life
- Dependent on Physician's tech
- Complex supply chain

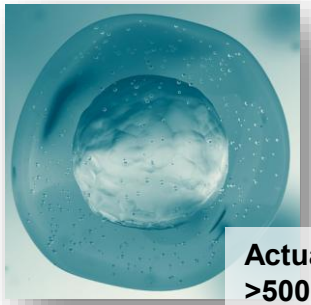
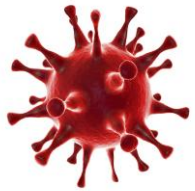
Image of Difficulty of quality control

Regenerative Medicine Products		Small molecule
Whole cell homogeneity control	VS	1 component control





Size

Cell Therapy	Gene Therapy	Antibody	Small molecule
 Actual size: >500 times			
dia. ca20μm	dia. ca22nm*	dia. ca a few nm	dia. ＜1nm

*adeno-associated virus (AAV): Picture is not AAV

Concept and/or business model are different from existing pharmaceuticals

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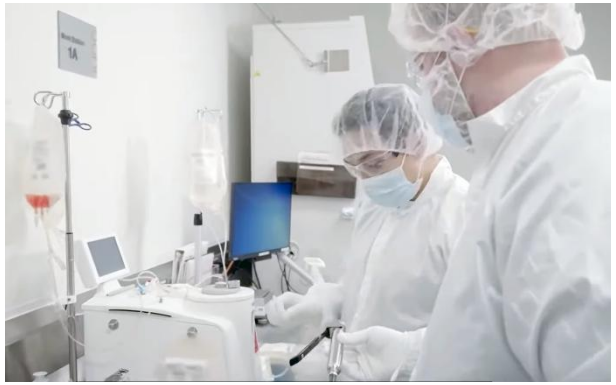
RMPs' different benefits and characteristics from existing pharmaceuticals

		Regenerative Medicine	Small molecule / Biologics
Research and Development	Regulations	• Japanese regulatory barriers (Standards for Biological Ingredients, Cartagena, etc.)	• Harmonized internationally by ICH
	Patents	• Needs for multiple patents to create and protect products (increased license fee)	• Protected by substance patents
	Human Resources	• Shortage of experienced operators	• Abundance
Manufacturing	Raw materials	• Dependent on donor availability • Inter-donor variability	• No sampling
	Manufacturing	• Dedicated equipments • Cooperation with numerous related industries • Difficulty in large-scale production	• Diverting equipment into the same modality • Limited specific industries (chemical and biotechnology) • Ease in large-scale production
	Quality	• Control of homogeneity • Heavier burden of quality inspection	• Control of uniformity
Distribution	Transport	• Dedicated transportation	• Normal transportation
Administration	Medical institution	• Limited to specialized medical institution • Dependent on the physician's procedure	• Widespread use
	Patients	• Relatively small number, including rare diseases • Autologous cells is the ultimate personalized medicine	• Relatively large number including lifestyle diseases

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Image of the manufacturing of RMPs

Example of *Ex Vivo* Gene Therapy Product Manufacturing




Source: The Cell Therapy Process (BMS Cell Therapy Manufacturing Tour)
（https://www.youtube.com/watch?v=lmXQvyV-V0k）

Examples of Biopharmaceutical Manufacturing





Source: Chugai Pharmaceutical, Manufacturing Process for Active Pharmaceutical Ingredients in Biopharmaceuticals
(https://www.chugai-pharm.co.jp/ptn/medicine/factory/factory002_002.html)

Source: Monthly IHEP; 364: 1-8, (2026)

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Regenerative Medicine Journey in Japan

1997 — **Organ Transplant Act**

Shortage of organs for transplantation :
A key driver for Japan's focus on regenerative medicine

2007 — **Japan's 1st commercialization of RM product: JACE®**

Autologous Cultured Skin Sheets

2011 — **FIRM Established**

2012 — **Nobel Prize for iPSC**



Shinya Yamanaka

2014 — **Regulatory Reforms**

PMD Act revised:
・Regenerative Medicine Category
・Conditional & Time-limited Approval

The Act on the Safety of Regenerative Medicine

2026

Conditional & Time-limited approval of iPSC-derived products:
Amchepry® & ReHeart®

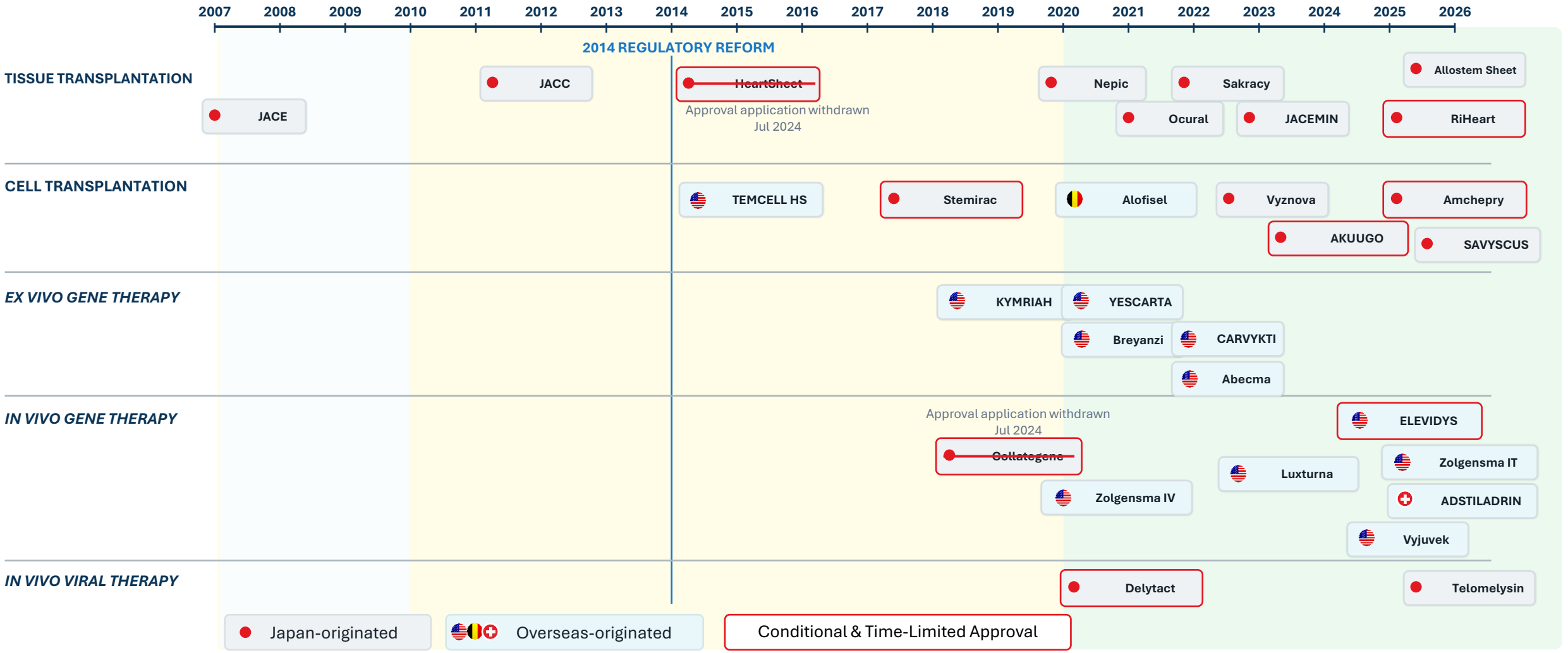
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https://www.japan-acad.go.jp/japanese/members/7/yamanaka_shinya.html

Approval Status of Regenerative Medicine Products in Japan

A 19-year journey: High agility of Japan's regulatory framework & rapid expansion of RM ecosystem

28 products approved including **2** World-first iPSC-derived products

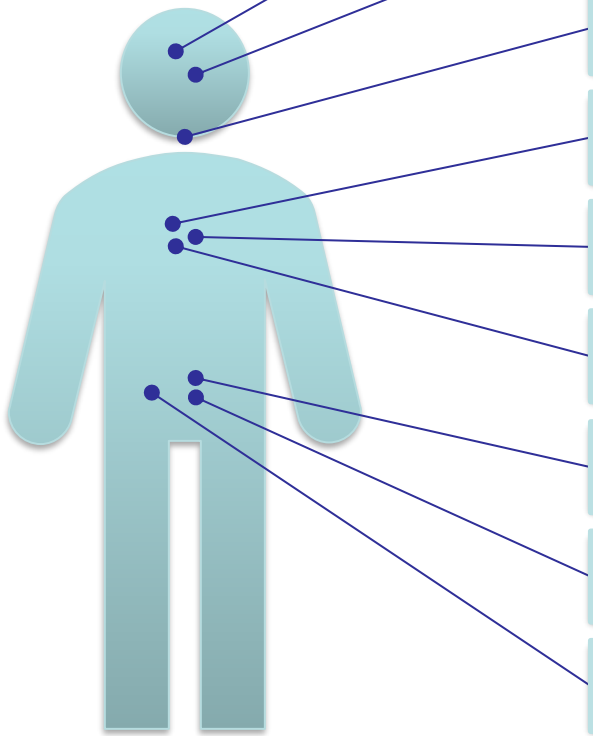


© Forum for Innovative Regenerative Medicine

Source: NIHS; developer websites; ClinicalTrials.gov; jRCT. As of Sep 2026.

Next Wave of iPSC-Derived Regenerative Medicine

A diversified clinical pipeline spanning neurological, cardiovascular, ophthalmic, oncology and metabolic diseases



Developers	Transplanted Cells	Disease	Status
StatusSumitomo Pharma / Kyoto University	Dopaminergic neural progenitor cells	Parkinson's disease	**Approved**
Sumitomo Pharma / Helios	Retinal pigment epithelial cells	Retinal pigment epithelial tear	Phase 1/2
BrightPath Biopharma / Chiba University / RIKEN	NKT Cells	Recurrent/advanced head and neck cancer	Phase 1
Heartseed / Keio University	Cardiomyocytes	Severe heart failure due to ischemic heart disease	Preparing for approval application
Cuorips / Osaka University	Cardiomyocytes	Severe heart failure due to ischemic cardiomyopathy	**Approved**
iHeart Japan	Multilayered cardiovascular cells	Dilated cardiomyopathy	Phase 1/2
Juntendo University	Cytotoxic T cells	HPV16-positive cervical cancer	Phase 1
National Cancer Center / Kyoto University	NK cells	Ovarian cancer	Phase 1
Orizuru Therapeutics / Kyoto University	Islet cells	Severe type 1 diabetes	Phase 1/1b

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Source: National Institute of Health Sciences, each developer's website, clinical trial sites (ClinicalTrials.gov, jRCT) as of September 2026

Conditional and Time-Limited Approval Process under PMD Act

WHY IT MATTERS TO INNOVATORS — **Earlier patient access • Evidence generation in market • Clear re-application milestone**

Conventional Review Process

Clinical trial [Efficacy + Safety] → Approval → Marketed

Conditional and Time-Limited Approval Process

Accelerated patient access

Clinical trial [Safety confirmed Efficacy estimated] → Conditional & time-limited approval → Post-market verification [Efficacy + Safety] → Full approval → Continued marketing

Re-application within the time limit

PROOF OF USE - Approval with accountability

8 products have used the pathway

2 exits demonstrate active post-market accountability

In 2026 Two world's first approvals of iPSC-derived products

	2015	2018	2019	2021	2024	2025	2026	2026
Drug	HeartSheet	STEMIRAC	Collategene	DELYTACT	AKUGO	ELEVIDYS	Amshepli	RiHeart
Indication	Cell sheet for HF	MSC for SCI	GT for CLI	Oncolytic virus	Modified MSC for TBI	GT for DMD	CT for Parkinson	Cell sheet for HF
Granted								
Duration	Exited 2024	7y	Exited 2024	7y	7y	3y	7y	7y

iPSC-derived

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From Policy to Practice: A Clearer Path to Full Approval

CORE REQUIREMENT

Efficacy must be verified through post-marketing evaluation within the approval period.

Conditional approval starts patient access. It does not end evidence generation.

01 GUIDANCE

MHLW Notification No. 0329-3

March 29, 2024

Defines the regulatory framework

- Improves predictability for use of the conditional and time-limited approval pathway.
- Clarifies statistical achievement criteria for estimating efficacy.
- Sets considerations for feasible post-marketing evaluation plans leading to regular approval.

02 HANDLING

MHLW Notification No. 1006-1

October 6, 2025

Clarifies the application process

- Use PMDA pre-application consultation to discuss whether the available clinical data support an application.
- Demonstrate efficacy through the post-marketing approval-condition evaluation.
- Review outcomes are disclosed to support transparency and learning.

03 PRACTICAL GUIDE

FIRM industry guide

Planned for October 2026

Translates policy into execution

- For companies developing or seeking marketing authorization for regenerative medical products.
- Integrates the system, notifications, guidance, case examples and practical considerations.
- Draws on experience to date and discussions held when the system was established.

MHLW: Ministry of Health, Labour and Welfare PMDA: Pharmaceuticals and Medical Devices Agency

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Japan Has Put Regenerative Medicine on Its “Growth Strategy”

Japan’s Growth Strategy (July 2026): Injecting public-private partnership strategic investments that enhance resilience against potential crises and investments that promote growth, centered on the 17 strategic fields.

Regenerative medicine was subsequently recognized as a strategic investment area for Japan.



Industry voice at the policy table

FIRM contributed the sector’s perspective as Japan shaped its future industrial and innovation policies.

FIRM leadership was invited to contribute to Japan’s Growth Strategy discussions.

¥20.8T

$ 140B

indicative cumulative public-private investment through FY2040

for regenerative medicine and advanced therapies as one of Japan's strategic growth sectors

TAKEAWAY FOR INNOVATORS

Japan is signaling long-term commitment for regenerative medicine

Note: ¥20.8T is an estimate, not an appropriated government budget or legally committed investment.

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Holistic Support System for Entering the Japan Market

Strengthening Program for Pharmaceutical Startup Ecosystem Funding

Japan Agency for Medical Research and Development (AMED)

- Strategic Non-Dilutive Capital:
  Massive subsidies for startups with a Japan Corporation
- **2x Subsidy Match**:
  AMED provides the double investment amount made by Registered Venture Capitals
- Japan Footprint Required:
  Focuses on trials and innovation that directly benefit the Japan ecosystem

Japan Entry Acceleration Program (JEAP)

Japan External Trade Organization (JETRO)

- Japan Market Entry Support Program:
  Mentoring and networking with PMDA, KOLs, local firms, and investors.
- **Proven Results (2025):**
  Approx. 70 global applications;
  10 selected for 100+ strategic meetings
- 2026 Focus:
  Seamless Japan entry for APAC biotechs via integrated expert networks

Bio-CDMO Infrastructure

Ministry of Economy, Trade and Industry (METI)
Forum for Innovative RM (FIRM)

- **CDMO Directory:**
  Comprehensive list of Japanese CDMOs for CGT
- Optimal Partnering:
  Identifies providers by modality and manufacturing experience.
- METI-Backed Capacity:
  State-subsidized, world-class Bio-CDMO infrastructure

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Japan’s Expanding CGT CDMO Network

CDMO Companies with Regenerative Cell Therapy & Gene Therapy Manufacturing Facilities in Japan	CDMO Business Information (Max. 2 links per company)	ATMPs Manufacturable at Domestic Contract Manufacturing Facilities				ATMP Manufacturing License under Japan's Pharmaceuticals and Medical Devices Act (PMD Act)	Manufacturing License for Specific Cell-Processed Products under Japan's Act on the Safety of Regenerative Medicine	Biopharmaceutical Manufacturing Operations for Vaccine Production Capacity Enhancement	Partnership Status
		Cell Therapeutics (including ex vivo gene therapy)	Viral Vectors (AAV, LV, etc.)	Plasmid DNA	mRNA				
AGC Inc./AGC Biologics	WEB SITE	Under construction		✓	Under construction			✓	MEDINET Co., Ltd.
ARCALIS, Inc.	WEB SITE CDMO Business Information				✓ / Facilities under construction			✓	
Cellab Healthcare Service CO.,LTD.	WEB SITE CDMO Business Information	✓ / Facilities under construction				✓			DAI-DAN CO.,LTD.
Cellafa Bioscience Inc.	WEB SITE CDMO Business Information	✓							By integrating Astellas Pharma's expertise in drug discovery and quality with Yaskawa Electric's robotics capabilities, Cellafa provides end-to-end PROMO (Partnership, Research, Development and Manufacturing) support to partners across research, development, and manufacturing.
Cell Resources Corporation	CDMO Business Information	✓ / Facilities under construction					✓		
CellSeed Inc.	WEB SITE CDMO Business Information	✓				✓	✓		
CellSource Co., Ltd.	WEB SITE CDMO Business Information						✓		
Cyto-Facto Inc.	WEB SITE CDMO Business Information	✓ / Facilities under construction	Under construction			✓			
FUJIFILM Toyama Chemical Co., Ltd.	WEB SITE CDMO Business Information				✓			✓	
H.U.Cells, Inc.	WEB SITE CDMO Business Information	✓					✓		H.U. Group Holdings, Inc.
ID Pharma Co., Ltd	WEB SITE WEB SITE	✓ / Facilities under construction	✓ / Facilities under construction			✓	✓		
Interstem Co., Ltd.	WEB SITE	✓					✓		We are processing adipose-derived mesenchymal stromal cells in collaboration with Rohto Pharmaceutical.
I Peace, Inc.	WEB SITE	✓					✓		Successfully supplied GMP-grade iPS cells to several pharmaceutical companies both in Japan and overseas.
Japan Tissue Engineering Co., Ltd. (J-TEC)	WEB SITE CDMO Business Information	✓				✓	✓		Teijin Limited, J-TEC, Mitsui Fudosan Co., Ltd. and the National Cancer Center (NCC) have established a regenerative medicine platform in Japan. Overseas, Teijin and J-TEC have entered into a Letter of Intent for a global strategic collaboration with Resilience US, Inc. (Resilience)
JCR Pharmaceuticals Co., Ltd.	WEB SITE	Under construction	Under construction			✓		✓	
Kohjin Bio Co., Ltd.	CDMO Business Information	✓ / Facilities under construction					✓		
MEDINET Co., Ltd.	WEB SITE CDMO Business Information	✓				✓	✓		AGC Inc.
MediRidge Co.,Ltd.	WEB SITE	✓	✓	✓ / Facilities under construction	✓				MediRidge has agreement with Teijin Regenet Co., Ltd. to supply plasmid DNA.
MicroBiopharm Japan Co., Ltd.	WEB SITE WEB SITE		✓ / Facilities under construction	✓ / Facilities under construction					
Minaris Advanced Therapies Co., Ltd.	WEB SITE CDMO Business Information	✓ / Facilities under construction				✓			Athnea Holdings Corporation, Cell Resources Corporation, VectorBuilder Inc., TFBS Bioscience Inc., Myoridge Co. Ltd., Cryoport Systems, LLC., Locus Cell Co., Ltd., and Cell Tech Innovation Venture Studio Co., Ltd.
Nikon CeLL innovation Co., Ltd.	WEB SITE CDMO Business Information	✓ / Facilities under construction				✓			Lonza
PharmaBio Corporation	WEB SITE CDMO Business Information	✓					✓		A2 Healthcare Corporation
REPROCELL Inc.	CDMO Business Information	✓					✓		BioBridge Global, Histocell, Toxcell
Ricoh Biosciences, K.K.	WEB SITE WEB SITE				✓			✓	
Rohto Cell Factory Tokyo Co., Ltd.	CDMO Business Information	✓					✓		
ROHTO Pharmaceutical Co., Ltd.	CDMO Business Information	✓ / Facilities under construction				✓	✓		
S-RACMO Co.,Ltd.	WEB SITE CDMO Business Information	✓				✓			
Synplogen Co., Ltd.	WEB SITE CDMO Business Information		✓	✓	✓				Viral Vectors : Takara Bio Inc., FUJIFILM Corporation, Merck Group Plasmid DNA : MicroBiopharm Japan Co., Ltd. mRNA : FUJIFILM Toyama Chemical Co., Ltd., ARCALIS, Inc.
TAIYO Pharma Tech Co., Ltd.	WEB SITE CDMO Business Information	✓	✓						
Takara Bio Inc.	WEB SITE CDMO Business Information		✓ / Facilities under construction	✓	✓ / Facilities under construction	✓	✓	✓	SGS Vitrology Limited U-Medico Inc. Toshiba Corporation
TEIJIN REGENET CO., LTD.	WEB SITE CDMO Business Information	✓ / Facilities under construction	✓ / Facilities under construction	✓ / Facilities under construction		✓	✓		We are building the "Kashiwanoha Regenerative Medicine Platform" in collaboration with the National Cancer Center, J-TEC and Mitsui Fudosan, supporting seeds technology holders in addressing challenges including clinical trial preparation. Through business partnerships with TFBS Bioscience Inc. and Medridge Co., Ltd, both team support the supply of GMP-grade viral vectors and plasmids. By forming strategic alliances with overseas CDMOs such as National RESILIENCE, Inc.(USA), Hilleman Laboratories (Singapore), and others, we are establishing a global collaborative framework.
TFBS Bioscience, LLC	WEB SITE		✓						●GMP manufacturing is available up to a commercial scale at the facility of a group company (TFBS Bioscience, Inc.) in Taiwan. ●Integrated viral vector and plasmid supply is available through the alliance with Teijin Regenet and MediRidge.
U-Medico Inc.	WEB SITE WEB SITE		✓						TAIYO Pharma Tech Co., Ltd. Takara Bio Inc.

Japan offers more than market access: it offers an increasingly complete CGT manufacturing ecosystem

26	11	5	7
Cell / ex vivo	Viral vector	Plasmid DNA	mRNA

- **Coverage from cell products to enabling materials**
- **Multiple providers building additional capacity**
- **A practical partner base for Japan and broader Asia entry**

Visit FIRM Home page https://firm.or.jp/en/cdmo/

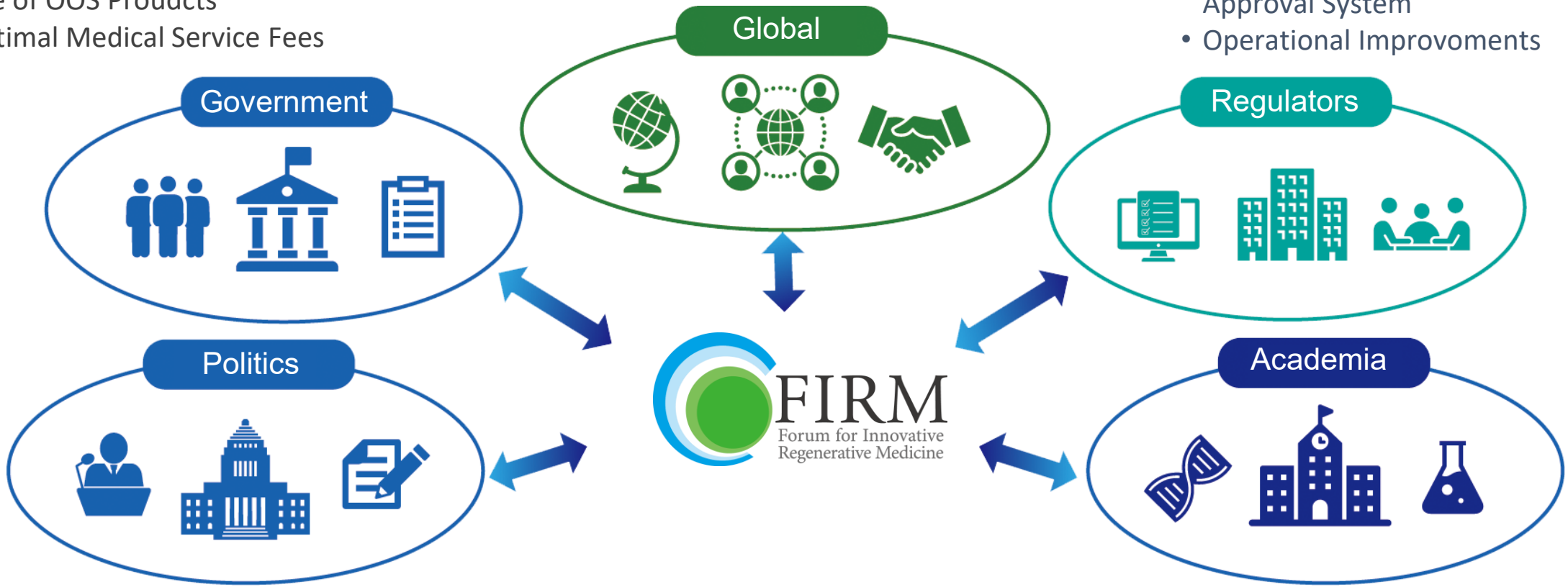
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Collaboration Toward Industrialization

- Promotion of Appropriate Use
- Use of OOS Proudcts
- Optimal Medical Service Fees

- APACRM international platform

- Exceptional Release of OOS Products
- Condtional and Time-limited Approval System
- Operational Improvements



- Japan’s Gowth Strategy
- Revision of the PMD Act
- Revision of the Act on the Safety of Regenerative Medicine

- Post-marketing Evidence Generation
- Raw Material handling with QMS

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FIRM入会のメリット

❶ 勉強会・セミナーが無料で受講できます

FIRMでは再生医療に関する勉強会やセミナー、講演会等を定期的に開催しています
会員はこれらのイベントを無料で受講できます

❷ 委員会活動を通じて様々な関連情報が得られます

FIRMの各種委員会へ参加することにより、委員会資料（調査報告書等）を閲覧できます
FIRM経由で配信される行政通知等も企業に有益な情報源となります

❸ FIRM活動を通じて様々な議論ができ、行政等への意見の発信が可能です

FIRMでは再生医療の普及・産業化に向けて、業界団体として意見発信を行っています
関連団体や関係省庁との連携も実施しております

❹ 会員企業間のビジネスチャンスも期待できます

FIRM会員交流会等の各種イベントで会員企業間の交流を行っています
新たなビジネスチャンスが生じることも期待できます

FIRM入会はこちら

FIRM入会

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再生医療JAPAN FIRM関連 プレゼンテーション

：主催セミナー　：再生医療ステージ

再生医療イノベーションフォーラム FIRM

	10月7日	10月8日	10月9日
11:00～11:30	FIRM		CDMO
11:30～12:00	サポーティングインダストリー Supporting Industries		
12:00～12:30		12:05-13:45 FIRM会員	安確法下で提供される再生医療・遺伝子治療等の改善に向けて G303, G304
12:30～13:00	12:40-13:40 FIRM会員		12:35-13:45 FIRM会員
13:00～13:30		条件及び期限付承認された製品及び制度の活用について G303, G304	
13:30～14:00			
14:00～14:30			日本の再生・細胞医療・遺伝子治療の産業化を加速するCDMOエコシステムの現在地 G303, G304
14:30～15:00			
15:00～15:30		標準化 Standardization	
15:30～16:00		起業・事業化 Venture Establishment	
16:00～16:30		JETRO: Japan Entry Acceleration Program（JEAP）	

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Thank you very much for your attention.

https://firm.or.jp/en

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