

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

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

Agenda

1. About FIRM

2. Characteristics of Regenerative Medicine Products

3. Current Trends in Japan & FIRM's Activities

The Forum for Innovative Regenerative Medicine (FIRM)

ONE ECOSYSTEM, FIVE COMPLEMENTARY CAPABILITY POOLS

AT A GLANCE

188

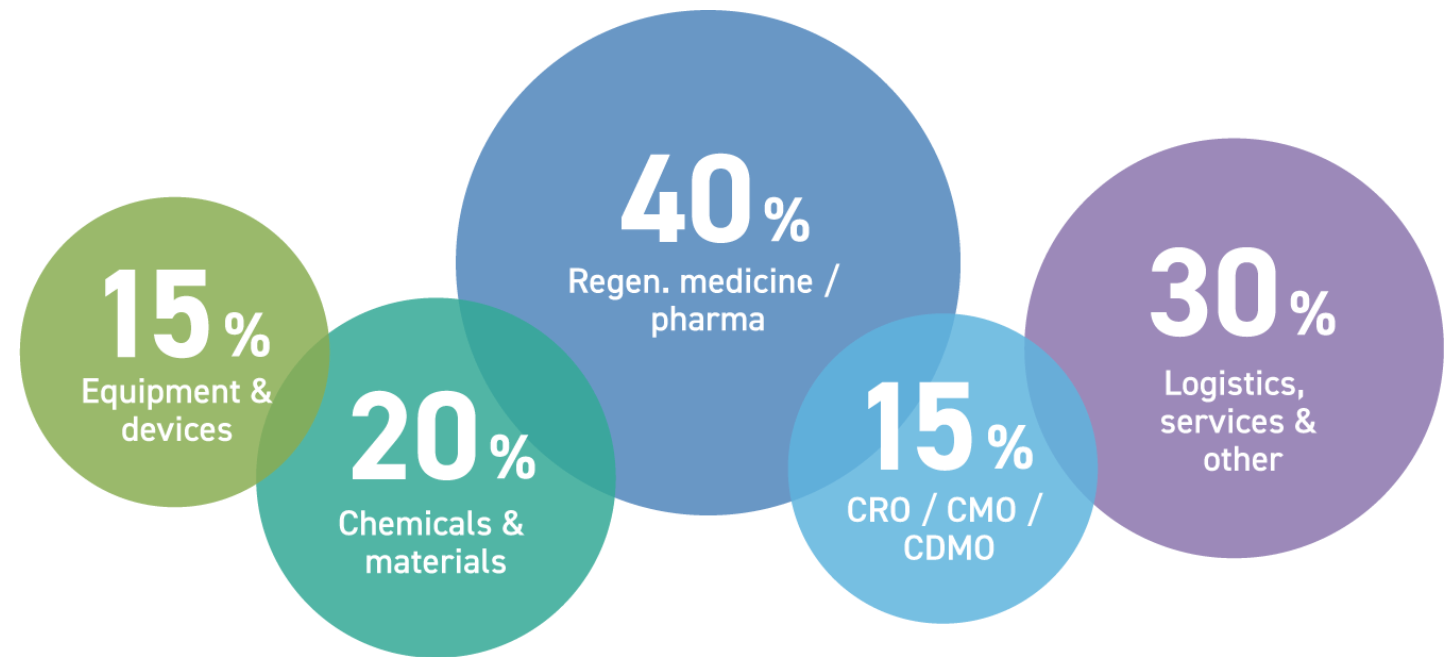
member organizations

Founded

June 17, 2011

Mission

**Accelerate the
industrialization of
regenerative medicine in
Japan**



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

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.

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

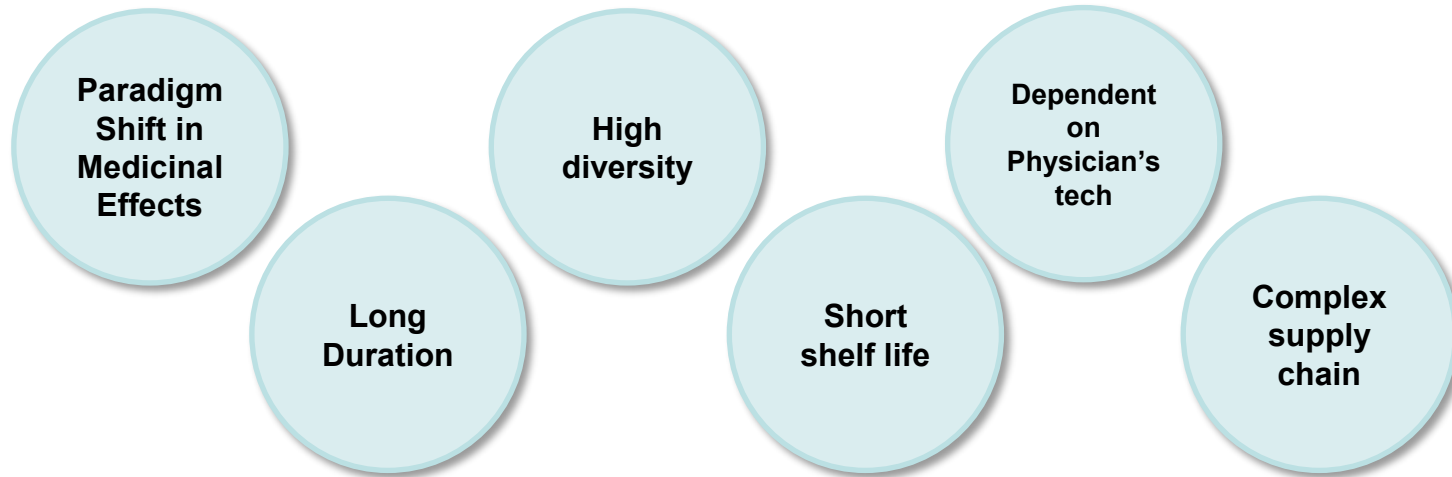


Image of Difficulty of quality control

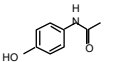
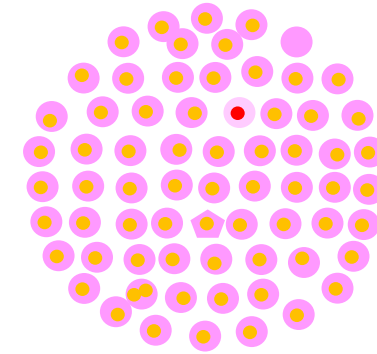
Regenerative Medicine Products

Whole cell homogeneity control

Small molecule

1 component control

VS



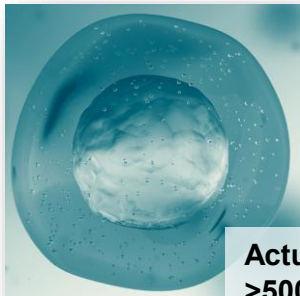
Size

Cell Therapy

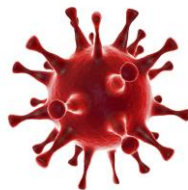
Gene Therapy

Antibody

Small molecule



Actual size:
>500 times



dia. ca20 μ m

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

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

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)

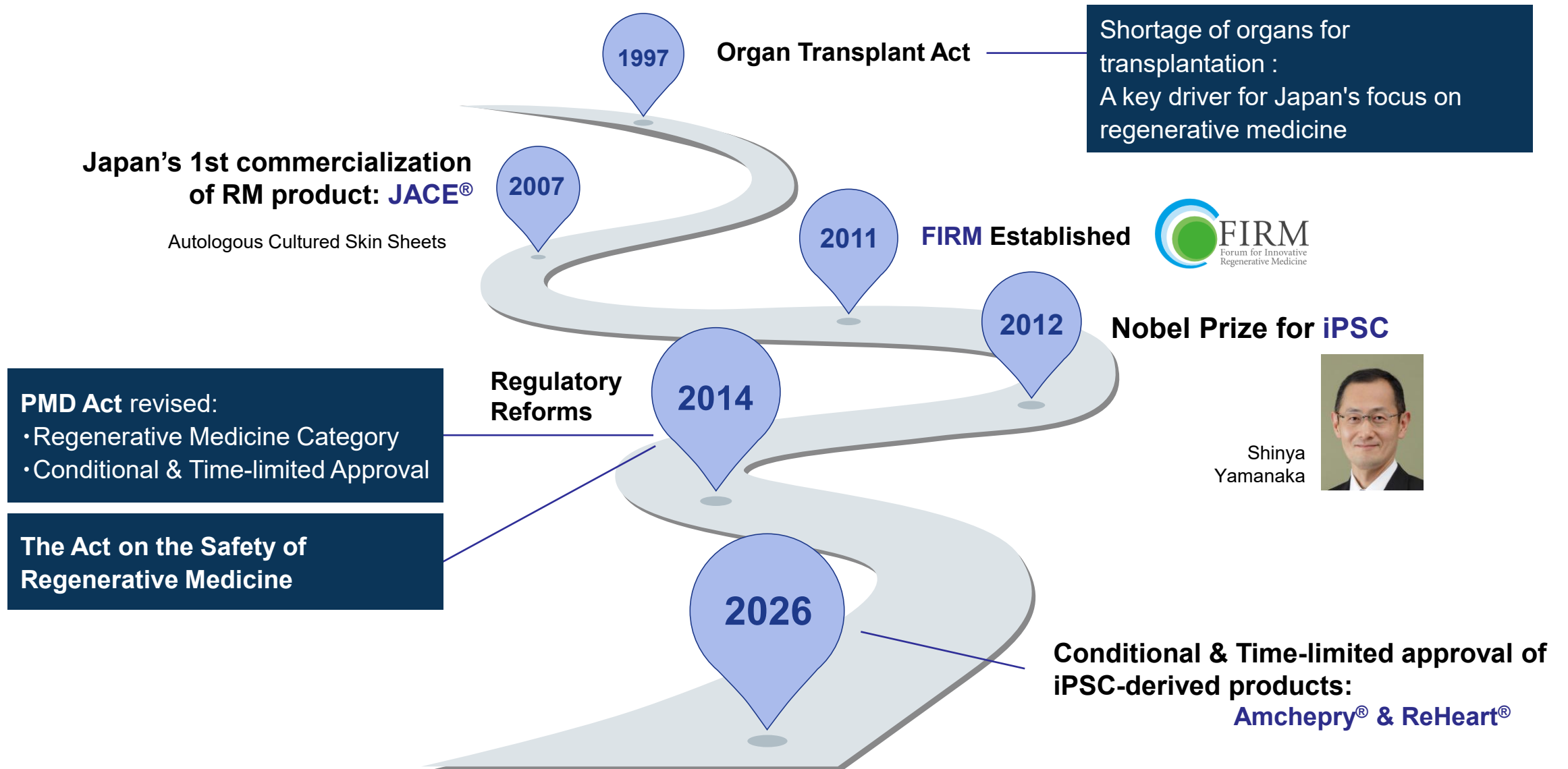
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Regenerative Medicine Journey in Japan



Japan's Dual-Track Regulatory Framework for Regenerative Medicine

Two complementary pathways have governed regenerative medicine since November 2014

1

Act on the Safety of Regenerative Medicine

GOVERNS

Physician-led clinical research and medical practice

FOCUS

Safety, bioethics and oversight of regenerative medicine technologies using processed cells

2

The Act on Pharmaceuticals and Medical Devices (PMD Act)

GOVERNS

Company-led development, manufacturing and marketing

COVERS

Regenerative medical products, including processed cell products and gene therapy products

WHY IT MATTERS TO INNOVATORS

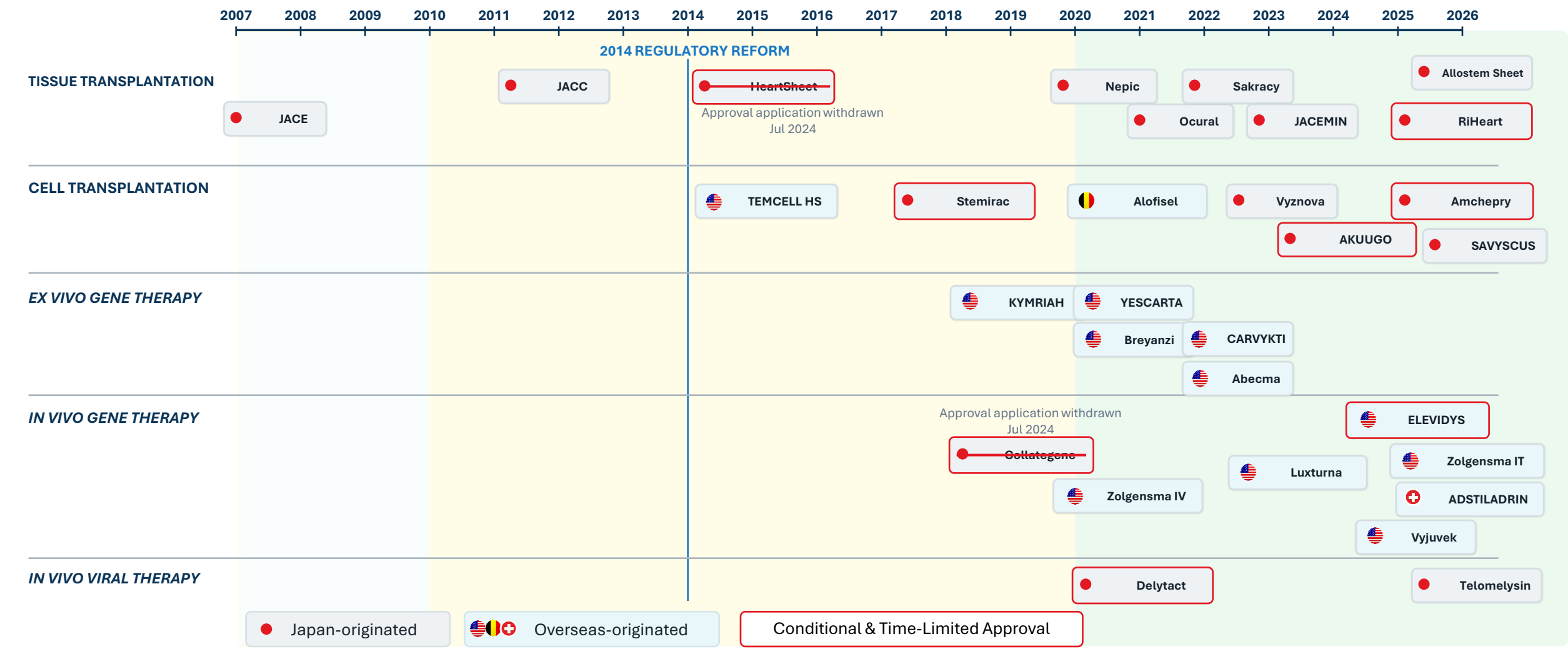
Japan separates physician-led use from commercial product development, with distinct requirements and oversight under each pathway.

Source: Pharmaceuticals and Medical Devices Agency (PMDA). Simplified overview for presentation purposes.

Approval Status of Regenerative Medicine Products in Japan

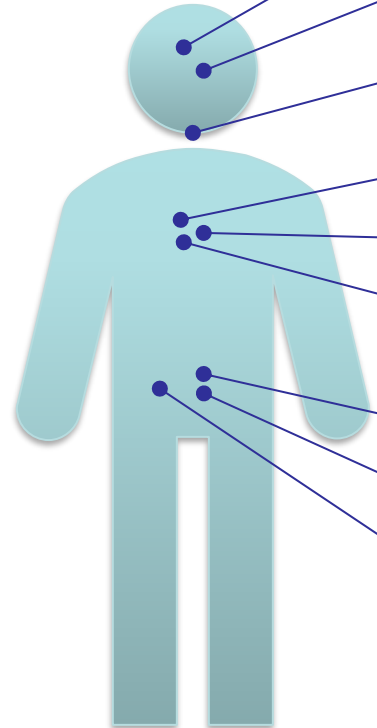
A 19-year journey: High agility of Japan's regulatory framework & rapid expansion of RM ecosystem

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



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

Conditional and Time-Limited Approval Process under PMD Act

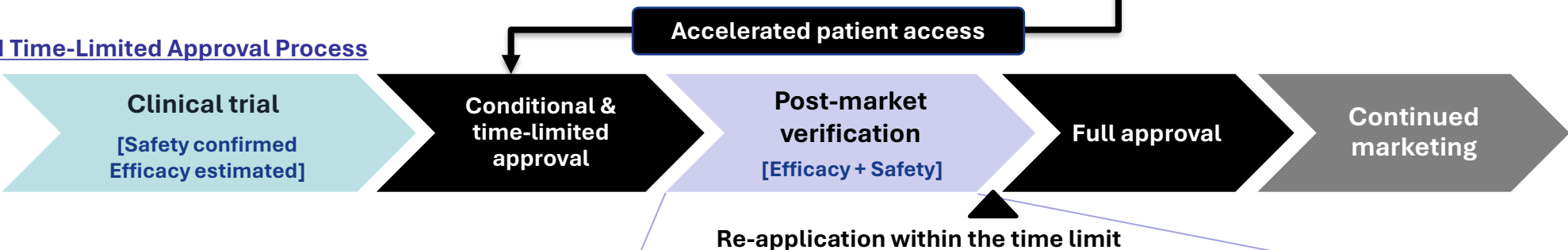
WHY IT MATTERS TO INNOVATORS

Earlier patient access • Evidence generation in market • Clear re-application milestone

Conventional Review Process



Conditional and Time-Limited Approval Process



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

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

iPSC-derived

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

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

Japan's Expanding CGT CDMO Network

Japan-based CDMO	Cell	Viral vector	Plasmid DNA	mRNA
ID Pharma	●	○		
iPS PORTAL	●			
ARCALIS				○
Interstem	●			
AGC	○		●	○
H.U. Cells	●			
S-RACMO	●			
Kaneka	●			
Cuorips	○			
Kohjin Bio	○			
Cyto-Facto	○	○		
JCR Pharmaceuticals	○	○		
Japan Tissue Engineering	●			
Synplogen		●	●	●
Cellafa Bioscience	●			
Cellab Healthcare Service	○			
CellSeed	●			
CellSource				
CELL RESOURCES	○			
Taiyo Pharma Tech	●	●		
TaKaRa Bio		○	●	○
TFBS Bioscience		●		
Teijin Regenet	○	○		
Nikon CeLL innovation	○			
MicroBiopharm Japan		○	○	
PharmaBio	●			
FUJIFILM Toyama Chemical				●
Minaris Advanced Therapies	○			
MEDINET	●			
MediRidge	●	●	○	●
U-Medico		●		
Ricoh Biosciences				●
REPROCELL	●			
Rohto Pharmaceutical	○			
Rohto Cell Factory Tokyo	●			

● Available ○ Capacity/facility under construction

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

26

Cell / ex vivo

11

Viral vector

5

Plasmid DNA

7

mRNA

WHY IT MATTERS TO GLOBAL INNOVATORS

- 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/>



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

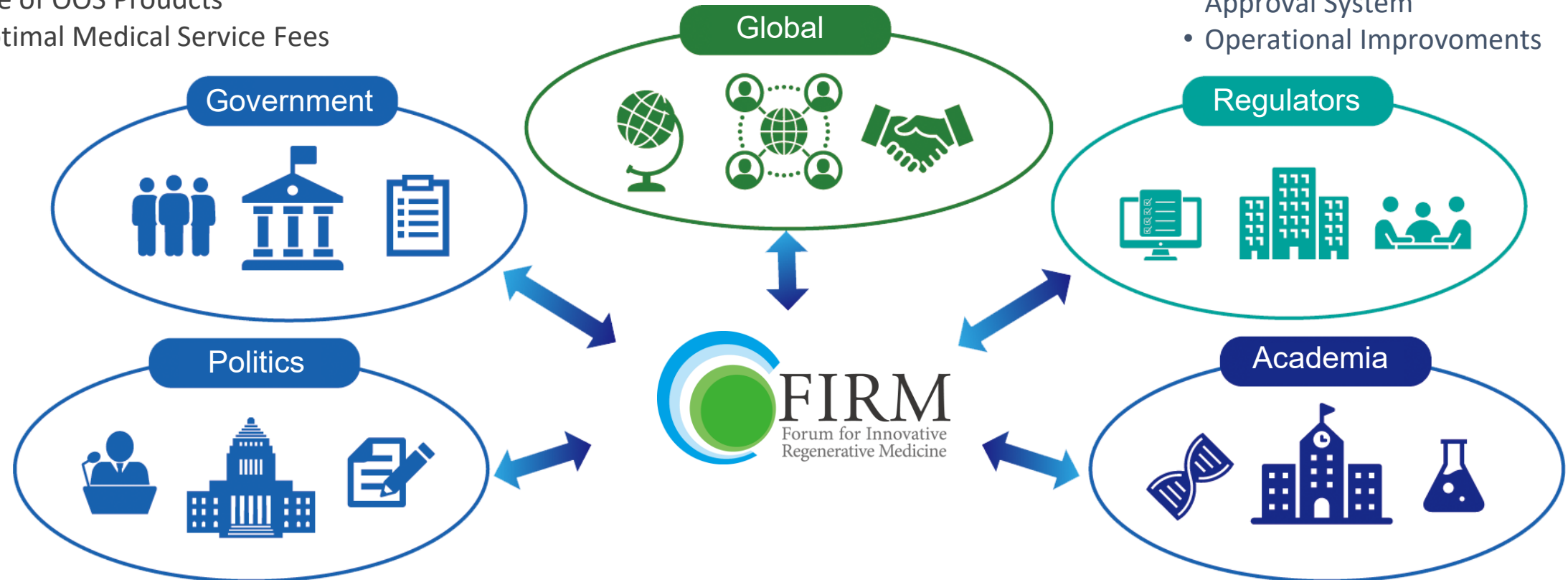
Note: ¥20.8T is an estimate, not an appropriated government budget or legally committed investment.

Collaboration Toward Industrialization

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

- APACRM international platform

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



- Japan's Growth 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

FIRM入会のメリット

- 1 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、講演会等を定期的を開催しています
会員はこれらのイベントを無料で受講できます
- 2 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
- 3 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
- 4 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

	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			
12:30~13:00	12:40-13:40 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
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		G303, G304	
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
14:30~15:00			G303, G304
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)	

Thank you very much for your attention.



<https://firm.or.jp/en>

