The 23rd Congress of the Japanese Society for Regenerative Medicine
2024. Mar. 23（Sat）13:50～15:50

SY-41-3

産業界から見た再生医療等製品の価値と適切な価格

Value and appropriate price of Cell and Gene Therapy from the industry perspective

Forum for Innovative Regenerative Medicine（FIRM）/Astellas Pharma Inc.

FIRM, Chair of Steering Committee
KANOH Hiroyuki

The 23rd Congress of the Japanese Society for Regenerative Medicine

Disclosure Statement of COI

Value and appropriate price of Cell and Gene Therapy from the industry perspective

The presenting author: KANOH Hiroyuki,

FIRM/ Astellas Pharma inc.

The presenting author has no financial conflicts of interest disclose concerning the presentation except the affiliated company in the past year (Jan to Dec).

Today's Talking point

1. **Future prediction** for the Regenerative Medicine (RM) market
2. **Characteristics and value** of RM Products
3. **Current issues** for RM Products
4. **New pricing system** for RM as an exit strategy
5. Countermeasures to **fill the gap with the future vision**

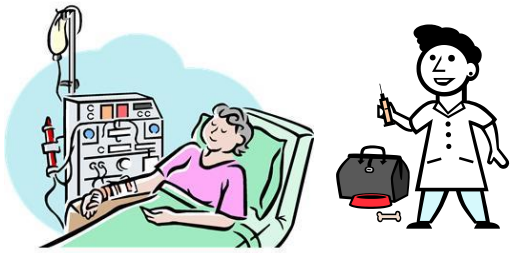
なお、本日の発表・発言の一部に私見を含んでおり、所属団体・企業を代表するものではない場合があります。

Regulatory Framework for RM in Japan

Enforced in 2014

Regenerative Medicine

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



The Act on the Safety of Regenerative Medicine

Medical Care or
Academic Research Purpose

Production and marketing of regenerative, cell and gene therapy **products** by firms

The Act on Pharmaceuticals and Medical Devices (PMD Act)

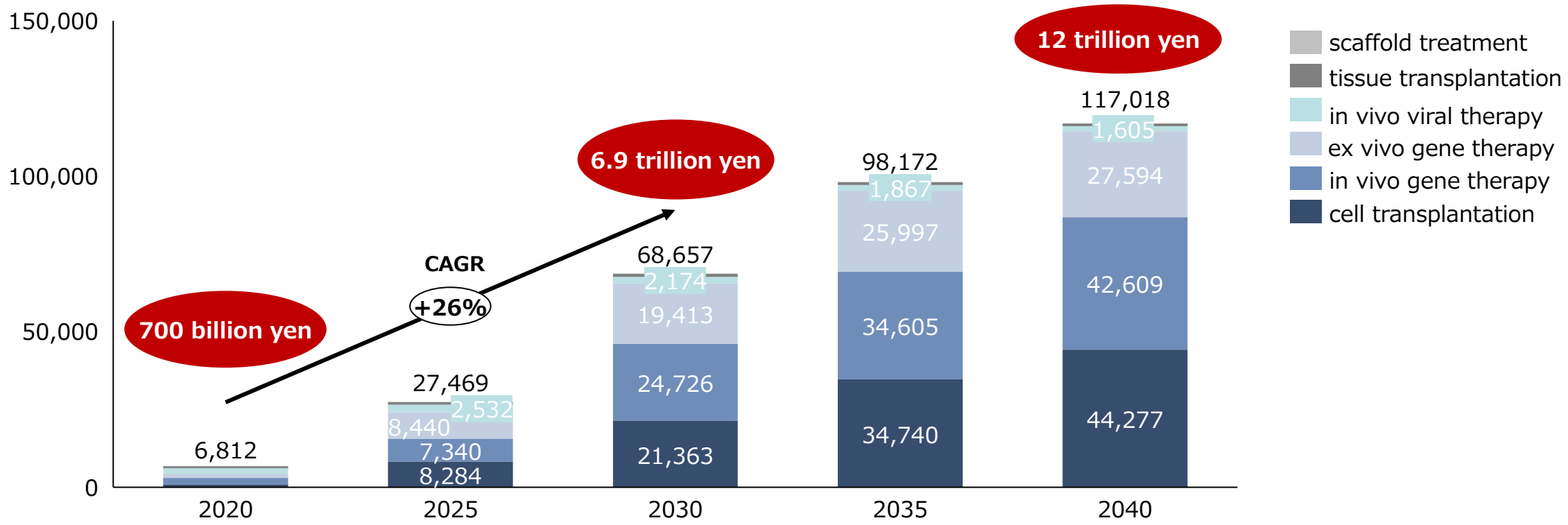
Commercial Product
Marketing Authorization Purpose

Reference: Pharmaceuticals and Medical Devices Agency (PMDA)

Market Size for RM Products (Global)

- Global market size of regenerative medicine products will grow to over 10 trillion yen in the next 20 years.

Global Market Size Estimates by Modality * based on pipelines as of October 2021



Source: Arthur D. Little Database

Pharmaceutical Market Size and Growth Potential by Modality (Global)

➢ Regenerative medicine products are expected to have the highest growth rate over the next 10 years.

Modality		Comparison of market trends for each modality (global)			
		Market-size *2 (2020)	Market-size *2 (2030)	Growth rate (20-30)	Examples of products
Regenerative medicine	Scaffold treatment *1	400 million yen	2.9 billion yen	21%	Orthocell (Europe)
	Tissue transplantation	60 billion yen	100 billion yen	4%	HeartSheet, JACC, and JACE
Cell therapy	Cell transplantation	90 billion yen	2.1 trillion yen	**37%**	TEMCELL, STEMIRAC
Gene therapy	Ex vivo gene therapy	140 billion yen	1.9 trillion yen	**30%**	KYMRIAH
	In vivo gene therapy	210 billion yen	2.5 trillion yen	**28%**	ZOLGENSMA
	In vivo viral therapy	180 billion yen	220 billion yen	2%	DELYTACT
Middle molecule drugs	Nucleic acids	450 billion yen	2.1 trillion yen	17%	SPINRAZA
	Peptides	3.2 trillion yen	4.7 trillion yen (2025)	8% *3	TERIPARATIDE, Non-standard cyclic peptides
Macromolecule drugs	Antibodys	16 trillion yen	23 trillion yen (2025)	8% *3	OPDIVO, ACTEMRA
	Proteins	6.4 trillion yen	10 trillion yen	4%	NESP, erythropoietin
Small molecule drugs	Small molecule compounds	47 trillion yen	74 trillion yen	5%	small molecule anticancer drugs (Docetaxel, etc.)

*1: Scaffolds developed as pharmaceuticals using synthetic substances, etc. (not including scaffolds using decellularized tissues) ; *2: predicted values ; *3: Growth rates (20-25)

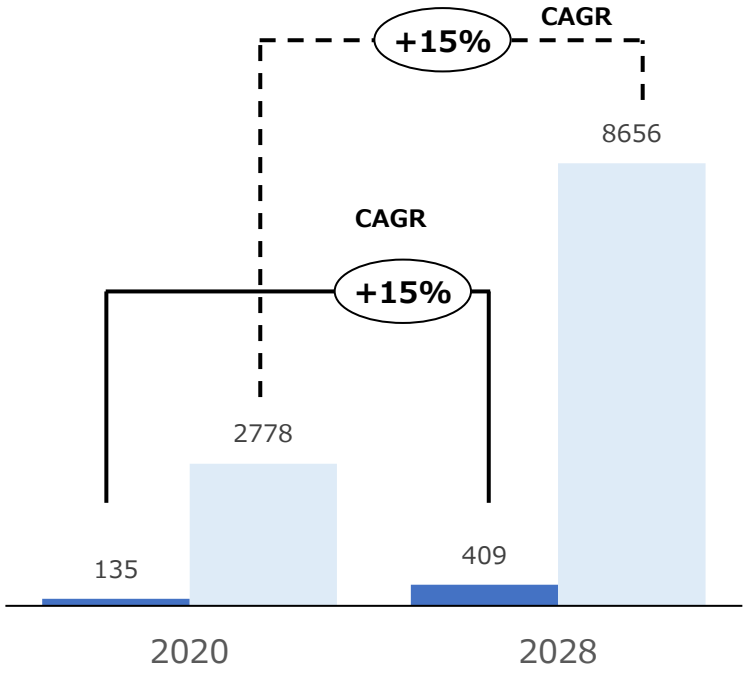
Source: Arthur D. Little Database

Growth Potential of Related Industries (global and domestic)

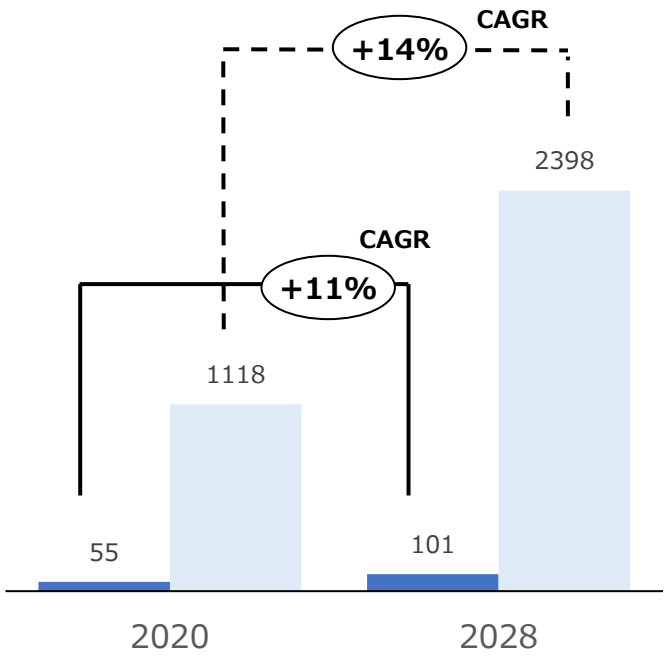
- Market sizes of related industries are expected to grow significantly, although the domestic market is about one-tenth of the global market.

Marketability of Related Industries of Regenerative Medicine Products (Unit: 100 million yen)

Materials market
Serum, media, reagents, containers, culture accessories, etc.

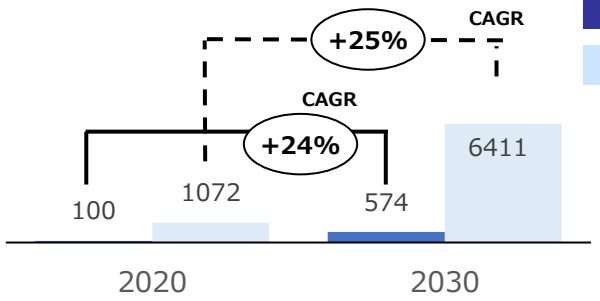


Equipment market
Filter device, incubator, cell counter, autoclave, centrifuge, microscope, cabinet, culture equipment, refrigerator, etc.

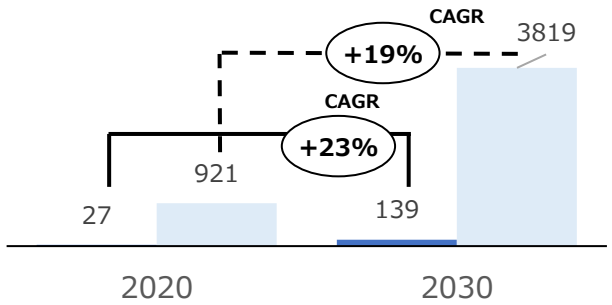


CDMO market

Cell CDMO market size *1



Viral Vector CDMO market size *2



domestic
global

Source: MarketsandMarkets "Cell Culture Market Global Forecast to 2028, estimated from ADL data base
*1 The global market is estimated based on the market size of the end market for cell/tissue transplantation and ex vivo gene therapy calculated from ADL database. Domestic sales in 2022 are estimated by ADL from current players' sales. The domestic market in 2030 is estimated from the global market based on the ratio of pipeline numbers. *3 Global market is estimated based on the market size of the end market for in vivo gene therapy and ex vivo gene therapy calculated from ADL database. The domestic market is estimated based on interviews with experts based on the global market.

Characteristics of RM Products

Characteristics

- Game changing Effects
- High diversity
- Depends on Dr.'s skill
- Long Duration
- Short shelf life
- Complex supply chain

Size

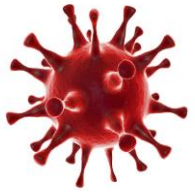
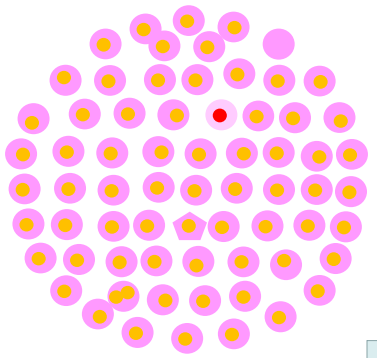
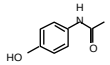
Cell Therapy	Gene Therapy	Antibody	Small molecule
 Actual size: >500 times			
dia. ca20μm	dia. ca22nm*	dia. ca a few nm	dia. <1nm

Image of Difficulty of quality control

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



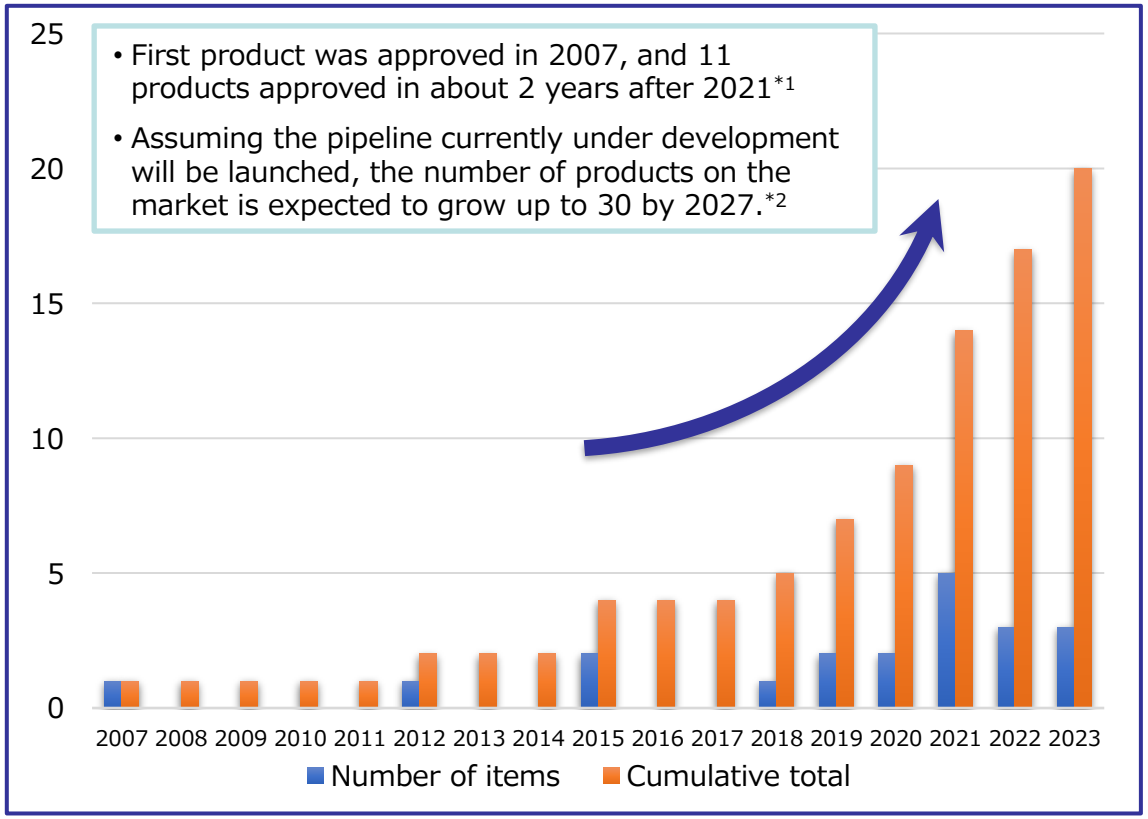


Concept and/or business model are different from existing pharmaceuticals

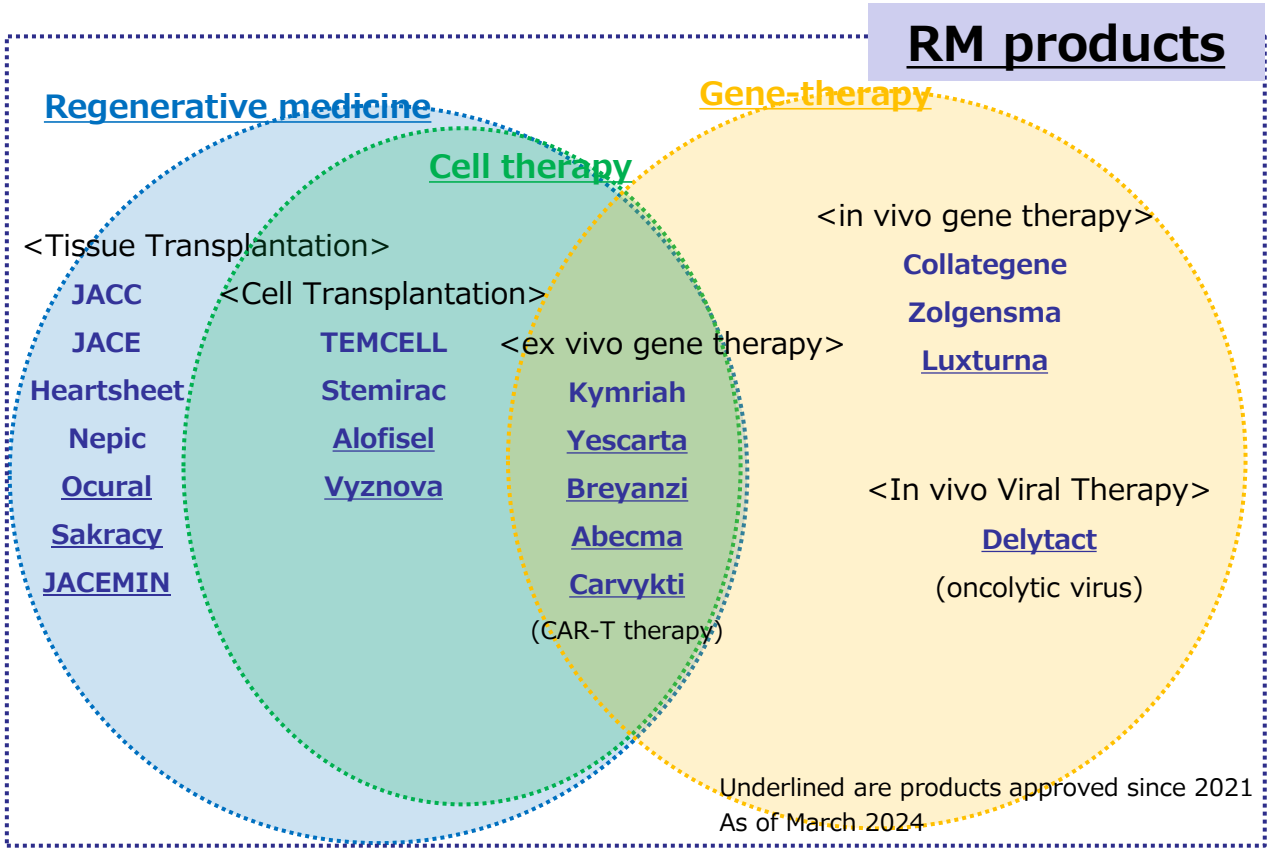
*adeno-associated virus (AAV): Picture is not AAV

Diverse modalities in Regenerative Medicine (RM) Products

➢ The number of approved products has reached to 20 (as of Mar, 2024), and it is expected to keep increasing in the future.



*1 Source: Graphs are prepared by FIRM based on published information.
*2 Source: Arthur D. Little Analysis



Sources: Reference Material 3 (January 27, 2021) of the Third Council for Regenerative Medicine, Cellular Medicine and Gene Therapy Development, Prepared by FIRM from the final report of AMED 2019 Year Regenerative Medicine/Gene Therapy

Products Originating in Japan have not been Developed Overseas

➢ 10 of the 20 approved products originated in Japan, but none of them have been approved overseas.

Tissue Transplantation

JACE ¥4.8 mil
Autologous Epidermal Cell Sheet for Severe burns etc. (2007)

JACC ¥2.3 mil
Autologous Cartilage-Derived Tissue for Traumatic cartilage defect (2012)

Heartsheet ★ ¥8.0 mil
Autologous Skeletal Myoblast Sheet for Severe heart failure (2015)

Nepic ¥9.7 mil
Autologous Corneal Epithelial Cell Sheet for Limbal stem cell deficit (2020)

Ocural ¥9.7 mil
Autologous Oral Mucosa Epithelial Cell Sheet for Limbal stem cell deficiency (2021)

Sakracy ¥13 mil
Autologous Oral Mucosa Epithelial Cell Sheet for Limbal stem cell deficiency (2022)

JACEMIN N/A
Autologous Skin epithelial cell with melanocyte for Vitiligo (2023)

Cell Transplantation

TEMCELL® HS Injection ¥0.9 mil
Acute GvHD(2015) after hematopoietic stem-cell transplantation.

Stemirac ★ ¥15 mil
Autologous Bone Marrow MSC for Spinal cord injury (2018).

Alofisel ¥5.6 mil
Allogeneic Adipose Tissue Stem Cells for Anal fistula in Crohn's disease (2021)

Vyznova N/A
Allogenic endothelial cell for bullous keratopathy (2023)

JP's Strength

CAR-T

KYMRIAH ¥33 mil
Relapsed or refractory B-cell acute lymphoblastic leukemia /diffuse large B-cell lymphoma (2019)
Relapsed or refractory follicular lymphoma (2022)

YESCARTA ¥33 mil
Large B-cell lymphoma (2021)

Breyanzi ¥33 mil
Relapsed or refractory with at least 2 prior regimens
Large B-cell lymphoma/follicular lymphoma (2021)

Abecma ¥33 mil
Relapsed or refractory multiple myeloma (2022)

CARVYKTI N/A
Relapsed or refractory multiple myeloma (2022)

In vivo gene

Collategene ★ ¥0.6 mil
Ulcers in chronic arterial occlusive disease (2019).

Zolgensma ¥167 mil
Spinal Muscular Atrophies (2020)

Luxturna ¥50 mil
Hereditary retinal dystrophy (2023)

Oncolytic virus

Delytact★ ¥1.4 mil
Malignant glioma (2021)

From Japan

Overseas approved,
Existing foreign products/similar products

★: Conditional and Time Limited Approval

Values in the upper right corner are prices

Value of Regenerative Medicine Products to the Public

- Providing new treatments to patients without effective treatments.
- Showing effectiveness with fewer doses and reducing burden on patients.
- Having various values which cannot adequately evaluate at the timing of approval (clinical, economic, ethical, industrial, etc.) .

3～7 years*1 | 1 year*1 | Approval | 7～8 years

Clinical Trial | review | Launch

*1 JPMA website

＜Cell Therapy＞

One injection

Leukemia

Difficult to treat with existing therapies*2

No recurrence 10 years after treatment*2

＜Gene Therapy＞

One injection

spinal muscular atrophy (SMA)

The main symptom is difficulty in swallowing, but patient is able to hold and eat ice cream on his own*3

Despite an average age of death of 6~9 months, the patient is able to walk using a walker*3

*2 Photo: Novartis Pharma
Source : Emily Whitehead Foundation

*3 Source : Novartis Zolgensma official website

Current Issues, Ideal Status, and Policies Required for RM Product in Japan

Current issues | Ideal status | Policies required

- Collaboration between policy makers, government, academia, and industry is necessary for realizing the ideal status of Regenerative Medicine

Future Products

- Many low-profit products
- Low attractiveness of the Japanese market

- Little progress in (re)investment for innovation
- Still-developing supporting industry and CDMO

Environment shaping for the business

- Soundness of regenerative medicine Business
- Attractive Japanese Market

- Increase (re) investment by companies
- Continuous innovation generation through Start-up support
- Development of supporting industry and CDMO
- Abundant human resources

Existing Products

Investment Recovery

R & D / Manufacturing

Establishment of venture ecosystem and human resources development

Pricing system that reflects diverse values

Evaluation of Innovation

Provision of New Treatment

- Difficulty for appropriate evaluation by current pricing system

- New pricing system that can reflect values appropriately

- Regulatory barriers
- Drug lag / loss

- Timely access to cutting-edge regenerative medicine

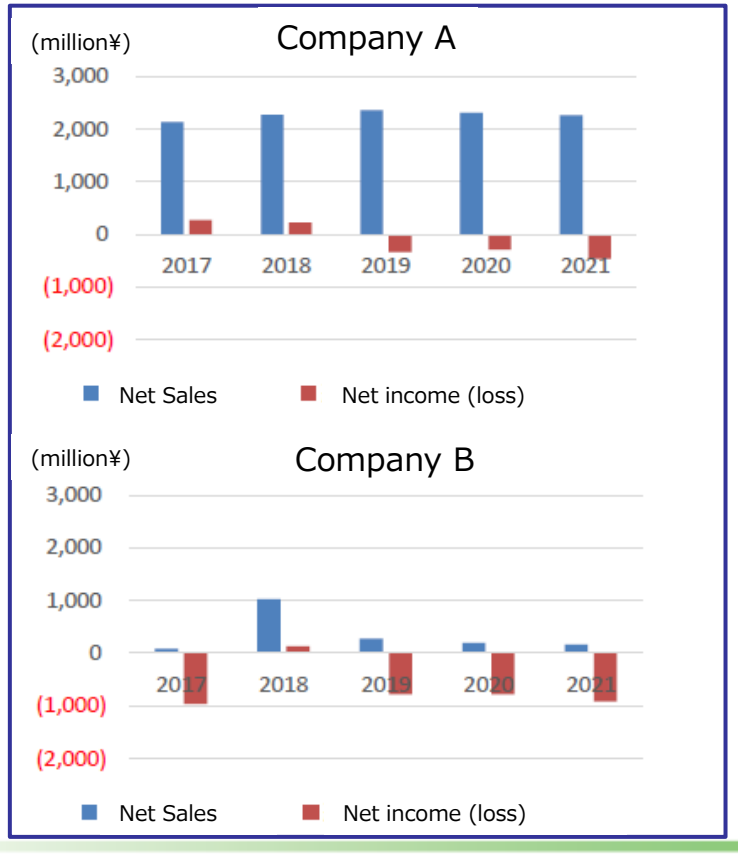
Regulation in line with product diversity

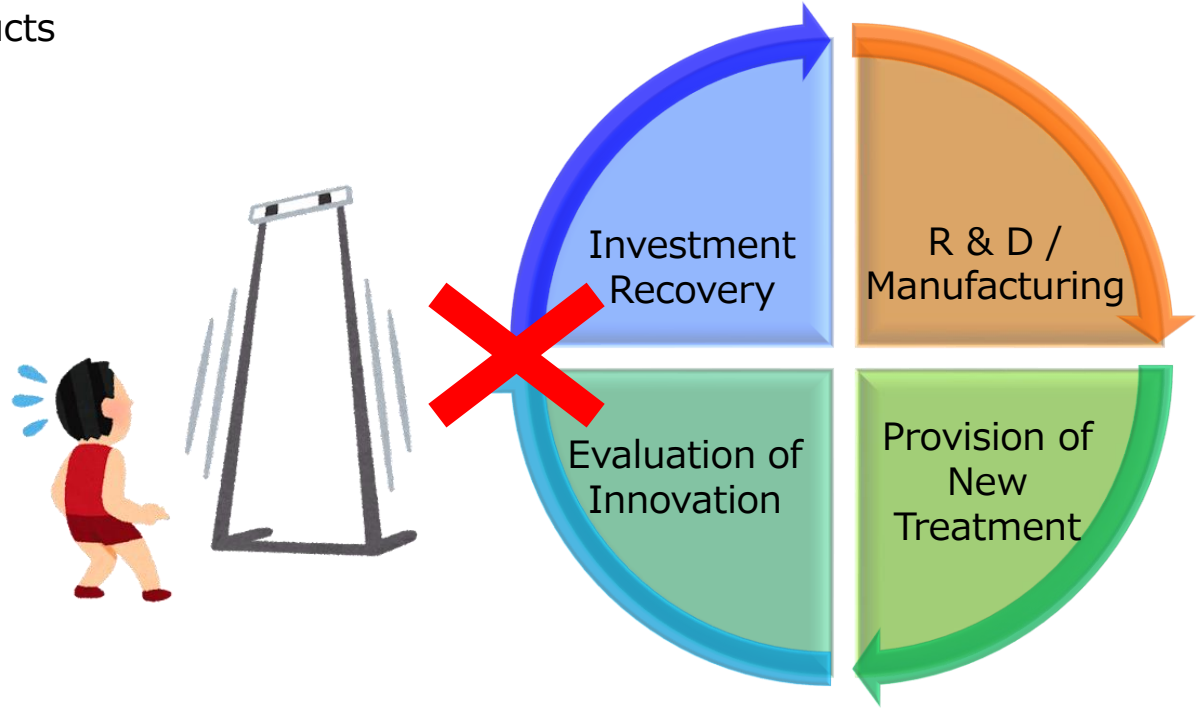
CDMO : Contract Development and Manufacturing Organization

Issues with the pricing system for RM products

- Companies can deliver innovative products to patients continuously with appropriate R&D investment cycle. However, domestic investment recovery is not working well.
- The issues , which is calculating the price of regenerative medicine products by accumulating costs as same as pharmaceuticals, has appeared.

Financial status of companies developing RM Products





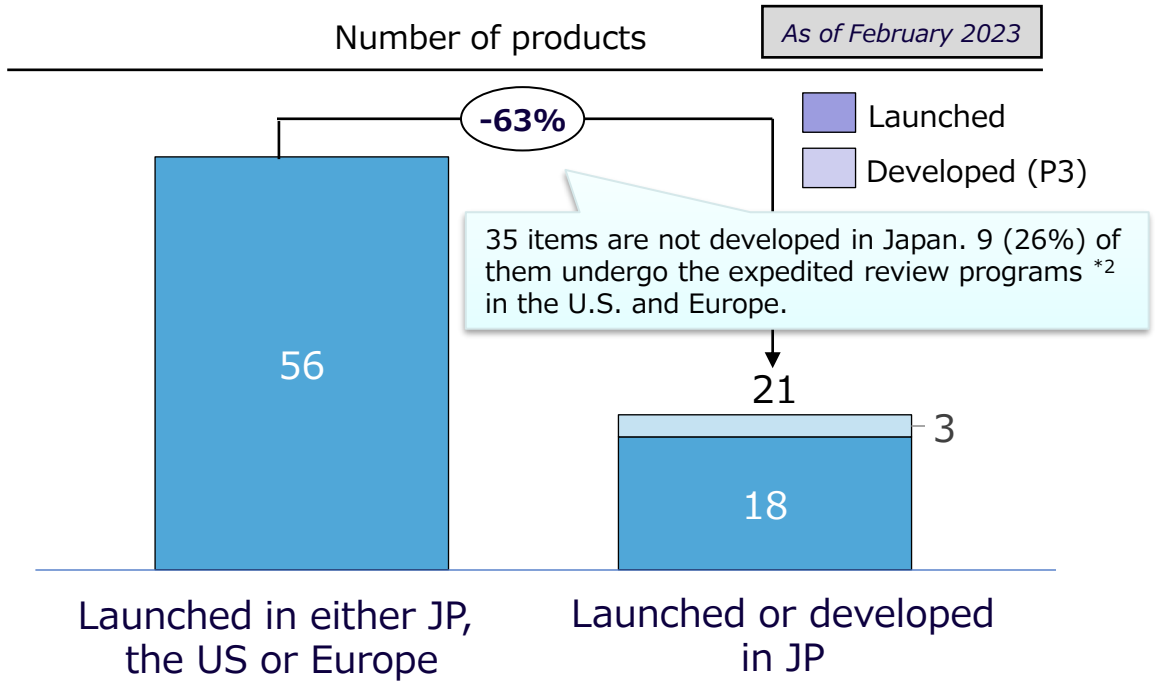
Need for a pricing system that reflects diverse values based on the features of products and modalities.

Development and launch status of RM products in JP, US and Europe

➢ **63% (35/56) of products are approved in the US and/or Europe*1, but not developed in Japan.**

*1 Europe: EU and UK and Switzerland

Development and launch in Japan of products approved in Japan, the U.S., and Europe



*2 (US): Priority review, Fast Track, Breakthrough Therapy, Regenerative Medicine Advanced Therapy, Accelerated Approval
(EU): Accelerated assessment, PRIME

Classification of 35 products not yet developed in JP

Class	Numbers
No existing medicine for the disease*3	5 (14%)
No existing medicine with same MoA	17 (49%)
Pediatric use	8 (23%)
Orphan designation	14 (40%)
With expedited review programs in US and Europe*2	9 (26%)

(Some products are duplicated in the numbers)

*3 : Including those with limited existing treatment (e.g. hematopoietic cell transplantation, symptomatic treatment only)

Sources: Prepared by FIRM Health Economics Sub-Committee using Pharmaprojects

RM products in Japan (1/2)

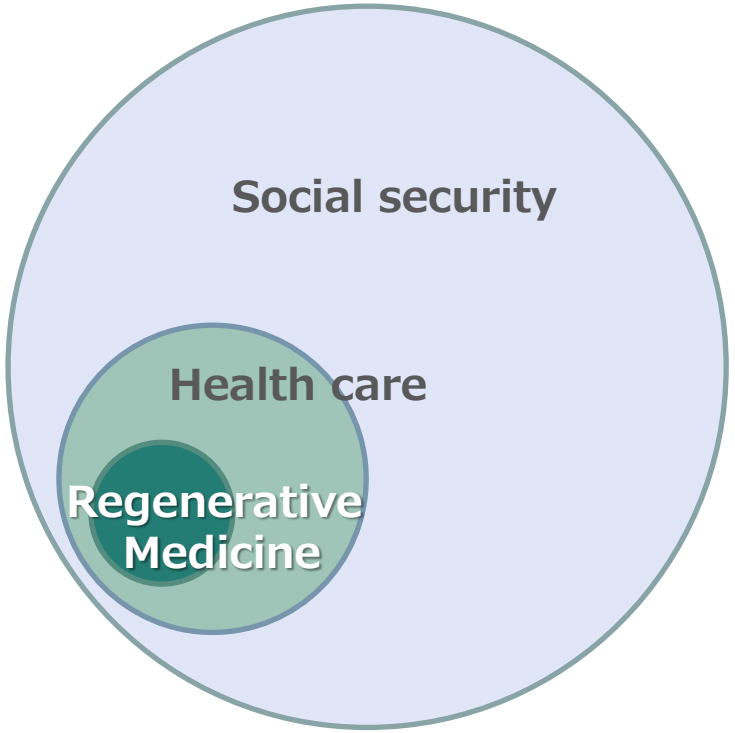
	Product	Company	Indication	Technology	Approval	Reimbursement Price
1	**JACE**	**J-TEC**	Severe burns, Giant congenital melanocytic nervi (2016), Epidermolysis bullosa (2019)	Autologous Epidermal-Derived Cell Sheet	2007	**Culture kit ¥4,460,000 Tx kit ¥154,000 /sheet**
2	**JACC**	**J-TEC**	Traumatic cartilage defect and osteochondritis dissecans of the knee	Autologous Cartilage-Derived Tissue	2012	**Culture kit ¥1,000,000 Tx kit ¥1,890,000**
3	**HeartSheet**	**TERUMO**	Severe heart failure due to ischemic heart disease	Autologous Skeletal Myoblast-Derived Sheets	2015, conditional/ time-limited	**A kit ¥6,480,000 B kit ¥1,710,000**
4	**TEMCELL HS Inj.**	**JCR Pharma**	Acute Graft-Versus-Host Disease	Allogenic Bone Marrow-Derived MSC	2015	**¥884,767 /10.8mL, 1 bag**
5	**Stemirac**	**NIPRO**	Spinal cord injury	Autologous Bone Marrow-Derived MSC	2018, conditional/ time-limited	**¥15,234,750 /shot**
6	**Collategene**	**AnGes**	CLI, Obstructive Arteriosclerosis and Buerger's Disease	A Plasmid DNA Encoding Human HGF Gene	2019, conditional/ time-limited	**¥611,478 /4mg 1.6mL vial**
7	**KYMRIAH**	**Novartis Pharma**	B-cell ALL in children and Relapsed/Refractory Adult DLBCL Relapsed or Refractory Follicular Lymphoma (2022)	CAR-T, CD19-Directed, Autologous	2019	**¥32,647,761/patient**
8	**Nepic**	**J-TEC/NIDEK**	Limbal stem cell deficiency	Autologous Corneal Limbus-Derived Corneal Epithelial Cell Sheet	2020	**Delivery set ¥4,280,000 Culture package ¥5,470,000**
9	**Zolgensma**	**Novartis Pharma**	Spinal Muscular Atrophy (SMA)	Recombinant AAV9 Expressing Human SMN Protein	2020	**¥167,077,222 /patient**
10	**YESCARTA**	**Gilead Sciences**	Relapsed/Refractory Large B-cell lymphoma	CAR-T, CD19-Directed, Autologous	2020	**¥32,647,761/patient**

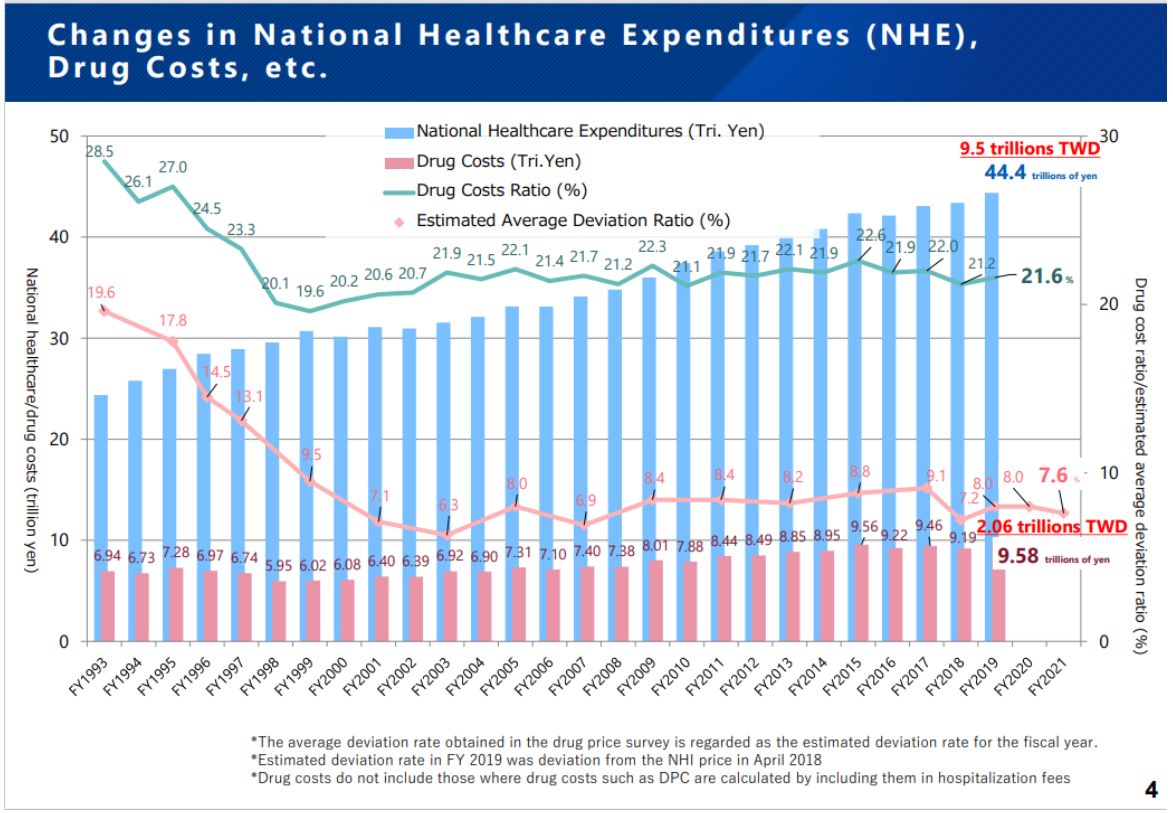
RM products in Japan (2/2)

	Product	Company	Indication	Technology	Approval	Reimbursement Price
11	**Breyanzi**	**Celgene/BMS**	Relapsed or refractory large-cell B-cell lymphoma	CAR-T, CD19-directed, autologous	2021	**¥32,647,761/patient**
12	**Delytact**	**Daiichi-Sankyo**	Malignant glioma, Subjects with Relapsed/Recurrent Disease	HSV-1 Oncolytic virus recombinant	2021, conditional/ time-limited	**¥1,431,918/vial**
13	**Ocural**	**J-TEC**	Limbal stem cell deficiency	Autologous Oral Mucosa-Derived Epithelial Cell Sheet	2021	**Culture kit ¥4,280,000 Tx kit ¥5,470,000**
14	**Alofisel**	**Takeda**	Complex anal fistula in patients with inactive or mildly active Crohn's disease	Allogeneic Adipose Tissue-Derived Stem Cells	2021	**¥5,620,004/4vial**
15	**Abecma**	**BMS**	Recurrent and Refractory Multiple myeloma	CAR-T, BCMA-Directed, Autologous	2022	**¥32,647,761/patient**
16	**Sakracy**	**Hirosaki LI**	Sympleparon in limbal stem cell deficiency	Autologous Oral Mucosa-Derived Epithelial Cell Sheet using Human Amniotic Membrane Matrix	2022	**Cell Sheet ¥7,940,000 Logistic Set ¥5,470,000**
17	**CARVYKTI**	**Janssen Pharma**	Recurrent and refractory multiple myeloma	CAR-T, BCMA-Directed, Autologous	2022	**Before reimbursement**
18	**JACEMIN**	**J-TEC**	Vitiligo for which nonsurgical treatment is ineffective or not indicated	Melanocyte containing Human (Autologous) Epidermis-derived Cell Sheet	2023	**Before reimbursement**
19	**Vyznova**	**Aurion Biotech**	bullous keratopathy	Allogenic endothelial cell	2023	**Before reimbursement**
20	**LUXTURNA**	**Novartis Pharma**	Hereditary retinal dystrophy due to biallelic RPE65 gene mutation	Gene expressing human RPE65 protein Recombinant adeno-associated virus	2023	**¥49,600,226/vial**

Sales of RM products as a percentage of social security expenditures

- National healthcare expenditure in Japan is 43.4 trillion yen, with pharmaceutical costs at 9.19 trillion yen (2018). Top 100 ranked domestic product were 16 billion yen (2020).
- The highest-selling regenerative medicine product reached 3.5 billion yen in sales (2021), a smaller proportion.





*based on IR disclosure of each company

Source: Japan's NHI Drug Price System, Deputy Director for Pharmaceutical Industry Promotion and Medical Information Management, Health Policy Bureau, MHLW Yukio Abe, Presentation Material

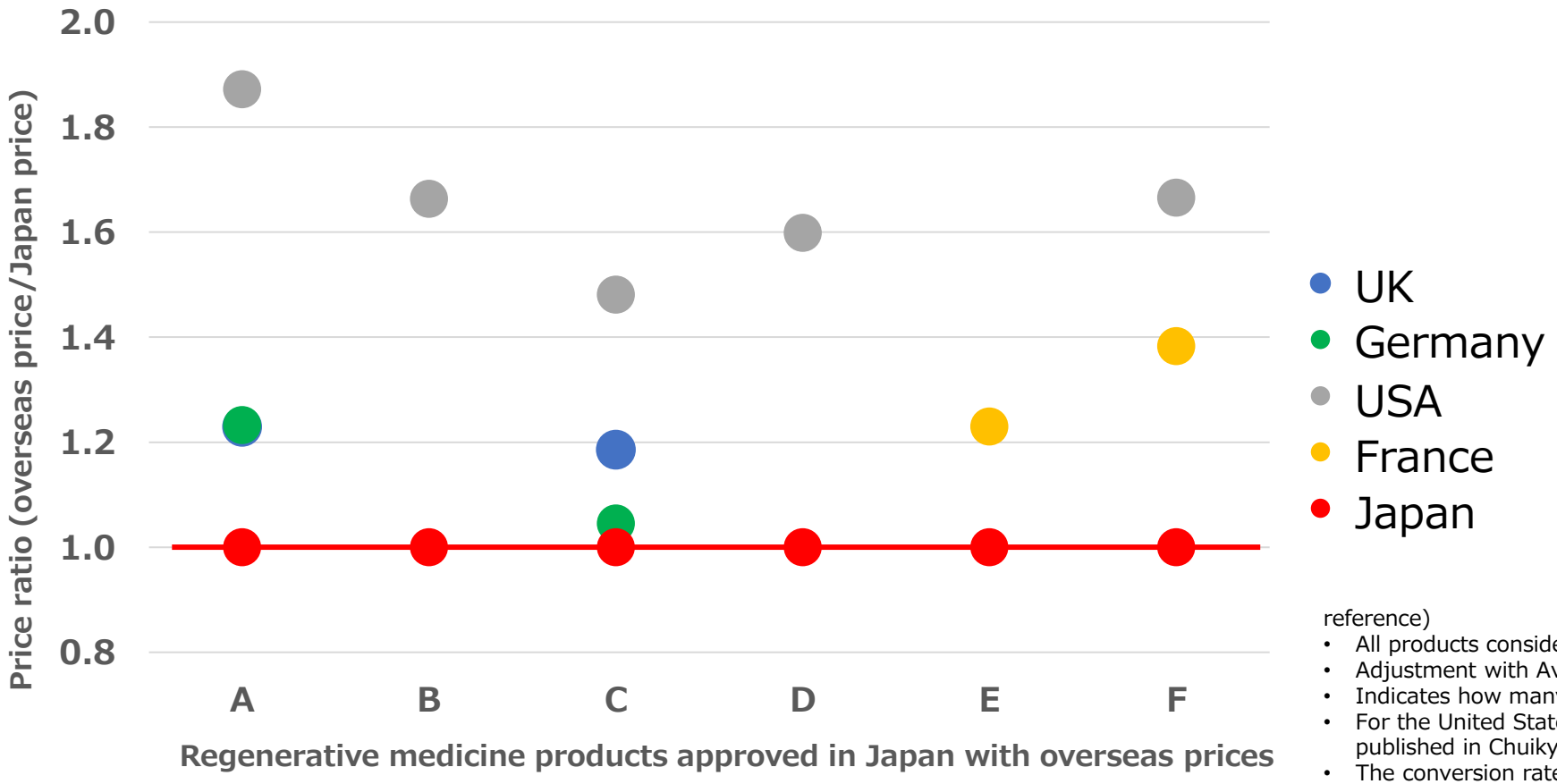
In order to continuously provide innovative therapies to patients in Japan, an immediate introduction of a new pricing system that appropriately evaluates innovation is needed.

Comparison with overseas prices of RM products

Compared to Europe and USA, prices in Japan are calculated to be the lowest.

Japanese market may become less attractive and prioritized

Innovative regenerative medicine products will be harder to reach patients (drug lag / loss)



reference)
- All products considered as pharmaceuticals
- Adjustment with Average Foreign Price has not been implemented
- Indicates how many times the overseas price is multiplied with Japan as 1.
- For the United States, AWP numbers are used. For other countries, figures published in Chuikyo materials were used.
- The conversion rate uses the figures published in Chuikyo materials.

CSIMC: Central Social Insurance Medical Council

Source: Created by FIRM Medical Economics Subcommittee from Chuikyo materials

Necessity of new pricing system for RM products

- **Current pricing methods cannot reflect the diverse values of RM products.**

Current system

R6 pricing system reform

Consideration by CHUIKYO and/or other research society

R8 pricing system reform

System improvement (Temporary measures)

How can we implement the mechanism to reflect

- **multiple value to pricing?**
- **added value after launch?**

New pricing methods that can reflect the diverse values of RM products

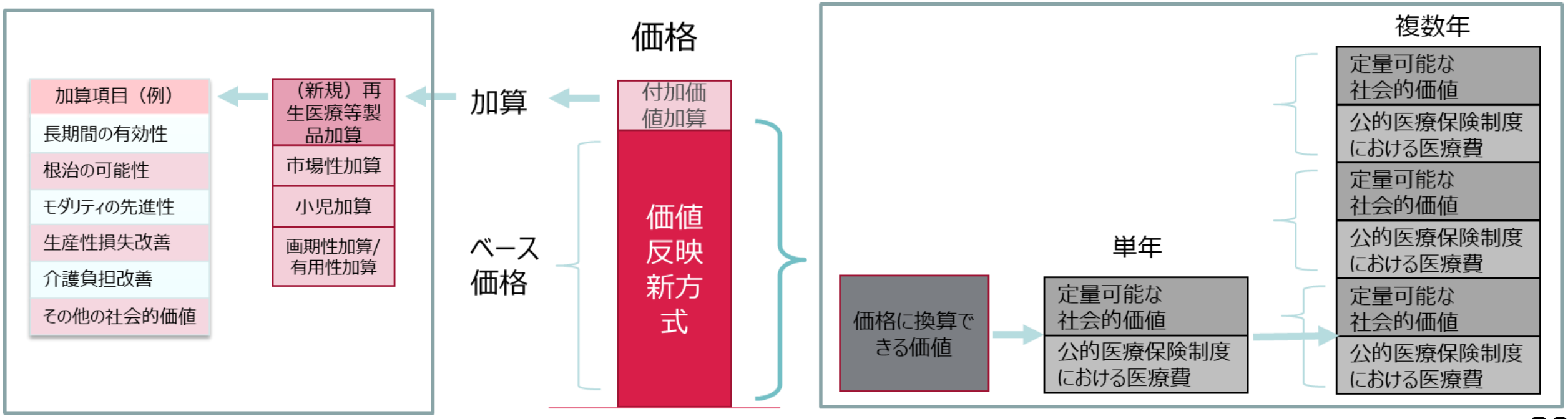
Not reflect

Reflect

Diverse values of RM

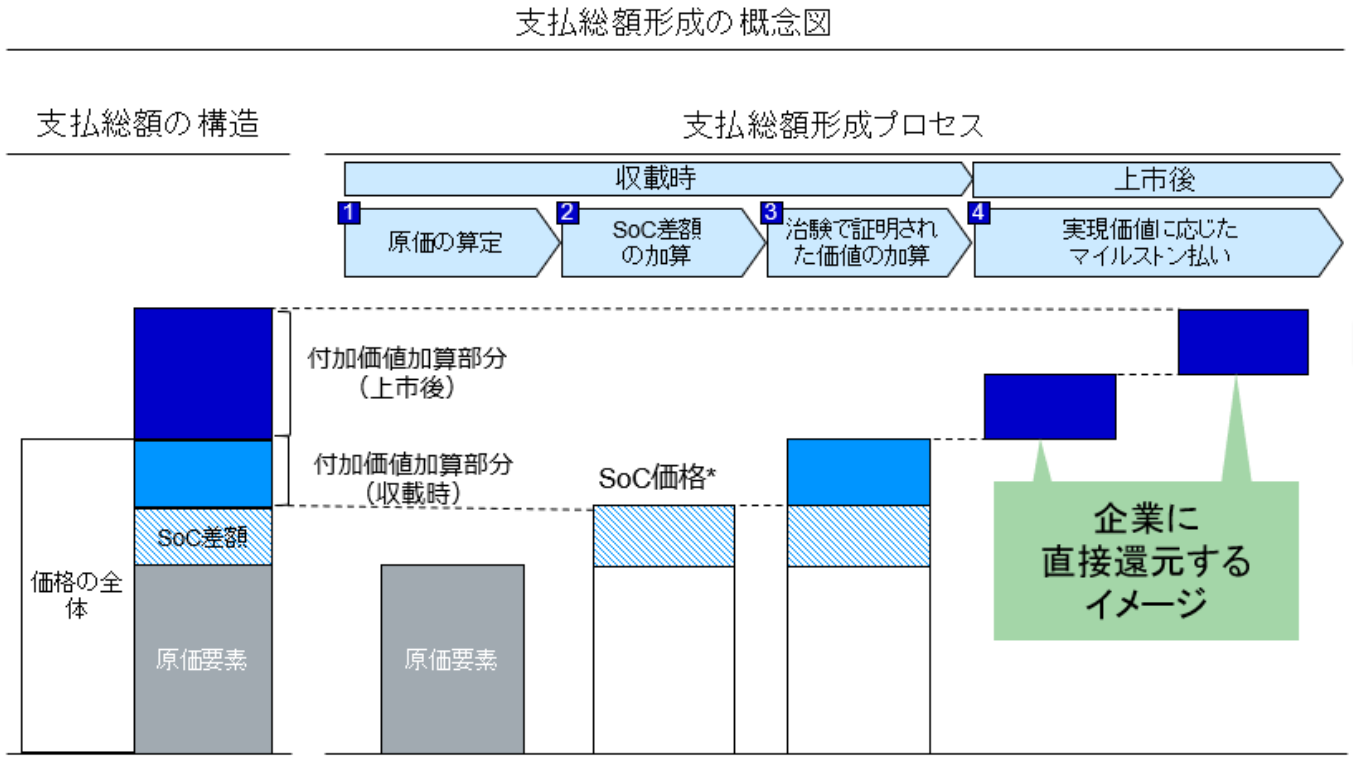
FIRM's Proposal 1: Pricing based on social value

- ◆ Method that reflects the value of regenerative medicine and other products at an initial price
  - ✓ Medical costs of standard care
  - ✓ Quantifiable social value (e.g., productivity loss and care costs)
- ◆ Considering the long-term effectiveness of regenerative medicine
  - ✓ Multiple years cost and value are reflected in the price
- ◆ Non-quantified value is evaluated by "FUKAKACHI-KASAN"



FIRM's Proposal 2: Reflect the value after launched

- As a pricing method for stepwise return, the value of regenerative medicine is evaluated separately at the time of approval and after launch, after that return the value to companies each period.



The return model is considered to be

1) Return to the target product directly

2) Return to the future products*.

*: Improve profitability of target products (reduce product costs, strengthen exclusivity), R&D incentives (reduce R&D costs, provide multifaceted support), improve future earnings (strengthen market competitiveness), etc.

Medical fee issues related to RM products

製品名(収載年)	原材料			投与・移植		
	採取点数	当初準用点数	点数に対する課題	投与・処置等点数	当初準用点数	点数に対する課題
ジェイス(2007)	• K000創傷処理又はK000-2小児創傷処理(6歳未満) 450~7,170点 *移植に至らなかった場合のみ算定可能	(N/A)	✓当初は点数がなかったが、キットに分かれたことにより移植に至らなかった時のみ付くようになった	• K014 皮膚移植術(生体/ 培養) 6,110点	• K014 皮膚移植術(生体) 6,110点	(N/A) *他の手技で点数が付いているため
ジャック(2012)	• K126-2自家培養軟骨組織採取術4,510点	• K126脊椎, 骨盤骨(軟骨)組織再手術2その他のもの 4,510点	(N/A)	• K059 骨移植術(軟骨移植術を含む)4自家培養軟骨移植術 14,030点	• K059 骨移植術(軟骨移植術を含む)1自家骨移植術 14,030点	✓技術が適切に点数に反映されていない
ハートシート(2015)	• K000創傷処理又は K000-2小児創傷処理(6歳未満)筋肉に達するもの 1,680点 *現状小児適用はなし	• D417組織試験採取、切採法1皮膚, 筋肉(皮下, 筋膜, 腱及び腱鞘を含み, 心筋を除く) 500点 *提案は*K126-2 4,510*点	✓骨格筋採取の点数が低すぎる ✓血清採取の点数の設定なし	• K605-5骨格筋由来細胞シート心表面移植術 9,420点	• K539心膜切開術 9,420点 *提案は*K059 4 14,030*点	✓シート調製の点数設定がない ✓投与の点数が低すぎる
テムセルHS注(2015)	(N/A)	(N/A)	(N/A)	• 点滴注射(G004) 97点	• 点滴注射(G004) 97点 *1日分の注射量が500mL以上の場合	✓保管に関する点数がない ✓細胞調製に関する点数がない
ステミラック注(2018)	• 末梢血採取: K920-3自己血貯血イ(200mLごとに)(1)液状保存の場合 250点 • 骨髄採取: K921-2間葉系幹細胞採取(一連につき) 17,440点	• 末梢血採取: K920-3自己血貯血イ(200mLごとに)(1)液状保存の場合 250点 • 骨髄採取: K921造血幹細胞採取(一連につき)の1(骨髄採取)のロ(自家移植) 17,440点	✓末梢血採取に対する点数が低く、採取に必要な器具や、体制、衛生管理などの費用が現状ではまかなえず、医療施設が赤字になる	• K922-3 自己骨髄由来間葉系幹細胞投与（一連につき）22,280点	• 点滴注射(G004) 97点	✓「K922-2造血幹細胞移植 2末梢血幹細胞移植 ロ自家移植の場合 30,850点に該当すると考えているが「点滴注射(G004)」としての手技料97点しかなく、投与に必要な器具や衛生管理などの費用がまかなえず医療施設は赤字になる
キムリア(2019)	• K921-3 採取、細胞調製及び凍結保存を行う場合 19,410点	• K921 造血幹細胞採取(一連につき)-2 末梢血幹細胞採取のロ自家移植の場合 17,440点	✓原則として1回のみのため複数回採取の際に不足 細胞調製(凍結)のための点数がない	• K922-2CAR発現生Ｔ細胞投与(一連につき) 30,850点	• K922造血幹細胞移植の2末梢血細胞移植のロ自家移植の場合 30,850点	(N/A)
コラテジェン(2019)	(N/A)	(N/A)	(N/A)	• 皮内, 皮下及び筋肉内注射(G000) 20点 • 超音波下肢血管(D215) 450点	• 皮内, 皮下及び筋肉内注射(G000) 20点 • 超音波下肢血管 (D215) 450点	✓注射箇所の同定と注射箇所数に対して点数が低い
ゾルゲンスマ(2020)	(N/A)	(N/A)	(N/A)	• 点滴注射(G004) 97点	• 点滴注射(G004) 97点	✓カルタヘナ対応が必要

2022年の診療報酬改定で対応

2022年の診療報酬改定では対応されず

Treatment of patients with RM products causes hospitals to run into the red, hindering the spread of regenerative medicine.

Recommendations for introduction of a new pricing system

- FIRM published an opinion paper regarding an introduction of a new pricing system for RM products in April 2023.
- The opinion paper was handed over to Dr. IMAEDA, who is a Secretary General of “SAISEI IROU-GIREN”, on 11th April 2023.

Reflected on:

Final report of Expert meeting “Comprehensive measures to realize rapid and stable supply of Pharmaceuticals” held by **MHLW**

Opinion paper of “Strengthen drug discovery capabilities Project Team” **(Policy Research Council of Liberal Democratic Party)**

Opinion paper of “**SAISEI IROU-GIREN**”

Status of consideration for prices of RM products in JAPAN

➢ Some of FIRM's opinions were reflected in the Basic Policy 2023 (Hone-buto 2023).

経済財政運営と改革の基本方針（骨太の方針）2023　（該当箇所抜粋）

２．持続可能な社会保障制度の構築　（社会保障分野における経済・財政一体改革の強化・推進）

創薬力強化に向けて、革新的な医薬品、医療機器、再生医療等製品の開発強化、研究開発型のビジネスモデルへの転換促進等を行うため、保険収載時を始めとするイノベーションの適切な評価などの更なる薬価上の措置、（中略）等を推進する。

出典：医薬品の迅速・安定供給実現に向けた総合対策に関する有識者検討会 報告書（令和5年6月9日）
経済財政運営と改革の基本方針2023　加速する新しい資本主義～未来への投資の拡大と構造的賃上げの実現～（令和５年６月16日閣議決定）

令和6年度薬価制度改革の骨子（令和5年12月20日 中央社会保険医療協議会 了解）　（該当箇所抜粋）

第2　具体的内容（6）②新規モダリティのイノベーション評価

〇医薬品の例により対応する再生医療等製品も含め、新規モダリティ等の類似薬がない革新的新薬における薬価上の適切なイノベーション評価の在り方等について、次期薬価改定に向けて検討を進めることとする。

出典：中央社会保険医療協議会 総会（第579回）　総-2参考1（令和6年1月17日）

Innovation evaluation of new modalities, including RM products, will continue to be considered in preparation for the 2026 price system reform, so we will continue to reach out to stakeholders.

BX Strategy and Opinion Paper Summary by KEIDANREN

経団連 バイオトランスフォーメーション（BX）戦略

レッドバイオ（医療・健康）の必要な施策

1. **再生医療等製品等における法規制の国際調和**
2. **創薬ベンチャー支援事業の機動性強化**
3. **バイオ医療を促進するための支援技術の強化と産業活動への包括的支援**
4. **再生医療等製品の価値や特徴を評価する新たな価格算定方式の導入**
5. 全ゲノム解析等実行計画の加速推進
6. 治験実施環境の整備

出典： 経団連 バイオトランスフォーメーション（BX）戦略
～BX for Sustainable Future～（2023年3月14日）

バイオ医薬品の産業強化に向けて －再生医療等製品の普及と産業化－

“ドラッグラグ・ロス ”の問題の解消や再生医療等製品の普及と産業化を図るため、
産業界や政府が検討すべき課題

1. 法規制等の環境整備

(1)カルタヘナ法、生物由来原料基準およびその他関連法規制
▶規制改革やより柔軟な運用、関連規制の順次見直し

(2)先端技術開発の加速とその利用の促進
▶臓器チップなどの先端技術を製品開発時の評価等へ利用
・ガイダンス作成
・開発支援

(3)施設認定制度の導入
▶原材料の品質確保のため国際的に通用する第三者評価による医療機関の施設認定制度導入

(4)規格外品提供の仕組みづくり
▶代替療法がない唯一の治療選択肢の場合に提供できる持続可能な枠組みを整備

(5)輸出入時の手続き短縮
▶事前申請により追加書類検査や貨物検査を対象外に

(6)国民への理解深化と適切な情報発信

2. イノベーションの評価

▶新たな価格算定方式の導入

3. 製造設備・人材育成の強化

▶国際競争力のある国内CDMO育成
▶サポーティングインダストリー強化
▶大学・高専・企業での人材育成

4. スタートアップ支援の強化

▶企業によるメンタリング等の伴走支援等
▶生産施設の共有等によるコスト負担軽減策の導入

Keidanren Policy & Action
一般社団法人日本経済団体連合会
バイオエコノミー委員会
企画部会

RM product specific recommendation

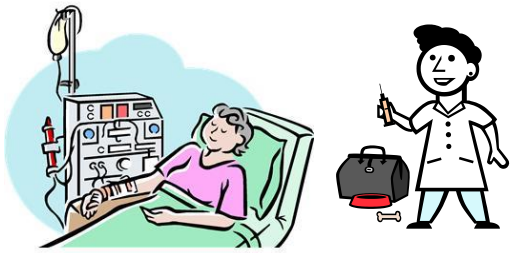
出典：経団連 バイオ医薬品の産業強化に向けて
－再生医療等製品の普及と産業化－（2023年11月7日）

Regulatory Framework for RM in Japan

Enforced in 2014

Regenerative Medicine

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



The Act on the Safety of Regenerative Medicine

Medical Care or
Academic Research Purpose

Production and marketing of regenerative, cell and gene therapy **products** by firms

The Act on Pharmaceuticals and Medical Devices (PMD Act)

Commercial Product
Marketing Authorization Purpose

Reference: Pharmaceuticals and Medical Devices Agency (PMDA)

Activity of "Envisioning Japan's Future through RM" Research Conference

「再生医療で描く日本の未来」研究会

回	テーマ	演者
第1回 7月25日	Kick-off	参議院議員 古川俊治 先生 内閣府 科学技術･ｲﾉﾍﾞｰｼｮﾝ推進事務局 松尾泰樹 事務局長 厚生労働省 医政局 研究開発政策課 佐野圭吾 室長
第2回 9月7日	再生医療の産業化	日本再生医療学会 岡野栄之 理事長 Heartseed 安井季久央 COO 経済産業省 商務･ｻｰﾋﾞｽG生物化学産業課　下田裕和 課長
第3回 10月5日	再生医療の普及	日本再生医療学会 八代嘉美 理事 医薬品医療機器総合機構(PMDA)　藤原康弘 理事長 再生医療ｲﾉﾍﾞｰｼｮﾝﾌｫｰﾗﾑ　志鷹義嗣 会長 /畠賢一郎 副会長
第4回 11月24日	国民負担のあり方・ 再生医療の価値	日本総合研究所　翁百合 理事長 日本再生医療学会 高橋政代 理事 慶應義塾大学 後藤励 教授 厚生労働省 保険局 医療課 木下栄作 室長
第5回 12月22日	国民の理解向上 国民の参画	日本再生医療学会 森尾友宏 顧問 川崎市議会議員 青木功雄 先生 日本放送協会 籔内潤也 解説委員

■ 座長：事業構想大学院大学　学長 **田中里沙**

■ 委員：

1. 参議院議員 **古川俊治 先生**
2. 日本再生医療学会 **岡野栄之 理事長**
3. 日本総合研究所 **翁百合 理事長**
4. 再生医療ｲﾉﾍﾞｰｼｮﾝﾌｫｰﾗﾑ **志鷹義嗣 会長**
5. 慶應義塾大学 **後藤励 教授**

■ オブザーバー：

1. 内閣府：　科学技術・ｲﾉﾍﾞｰｼｮﾝ推進事務局
   健康・医療戦略推進事務局
2. 厚生労働省：　医政局 研究開発政策課
   保険局 医療課
   医薬局　医療機器審査管理課
3. 経済産業省：　商務ｻｰﾋﾞｽｸﾞﾙｰﾌﾟ生物化学産業課
4. 文部科学省：　研究振興局 ﾗｲﾌｻｲｴﾝｽ課
5. PMDA：　再生医療製品等審査部

Discussion summary of the Research Conference

Talent Development and Reliable Communication

Talent Development

Communication to the World

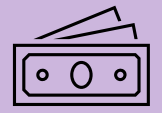
Diverse Communication

Highly Reliable Information

Support for Industrialization

Enrichment of Academia Research

Industrialization Driven by Ventures



Support by Venture Capitals

Manufacturing and Quality Assurance

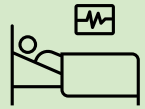
Manufacturing Technology

Quality Assurance

New Rules and Guidelines

Improvement of Medical Access

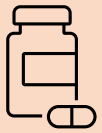
Evidence of Efficacy



Expansion of Treatment Options

Expansion to the Global Market

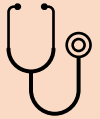
Sustainable Health Insurance System



Value Assessment

Pricing System

Health Insurance System



Uninsured Medical Treatment

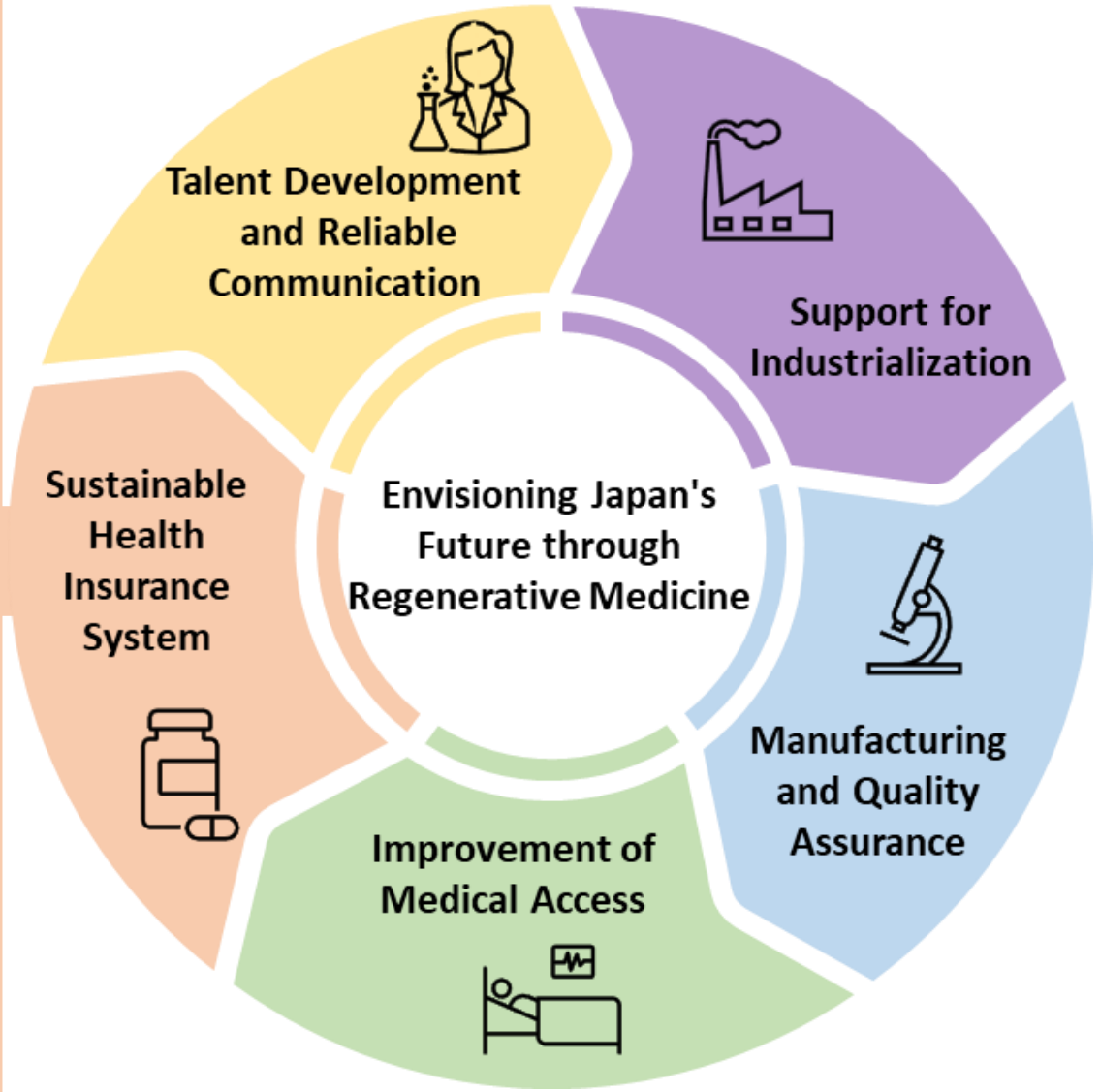
Recommendation summary of the Research Conference

新たな医療保険制度

- 再生医療を提供する**医師の手技料等を診療報酬として反映**する
  - Reflect the procedural fees for providing regenerative medicine as reimbursement

- 再生医療等製品の**特長・価値を踏まえた価格制度に向けて、政産官学での議論**を継続する
  - Continue cross-sectorial discussions toward the realization of a pricing system that reflects the characteristics and value of regenerative medicine products

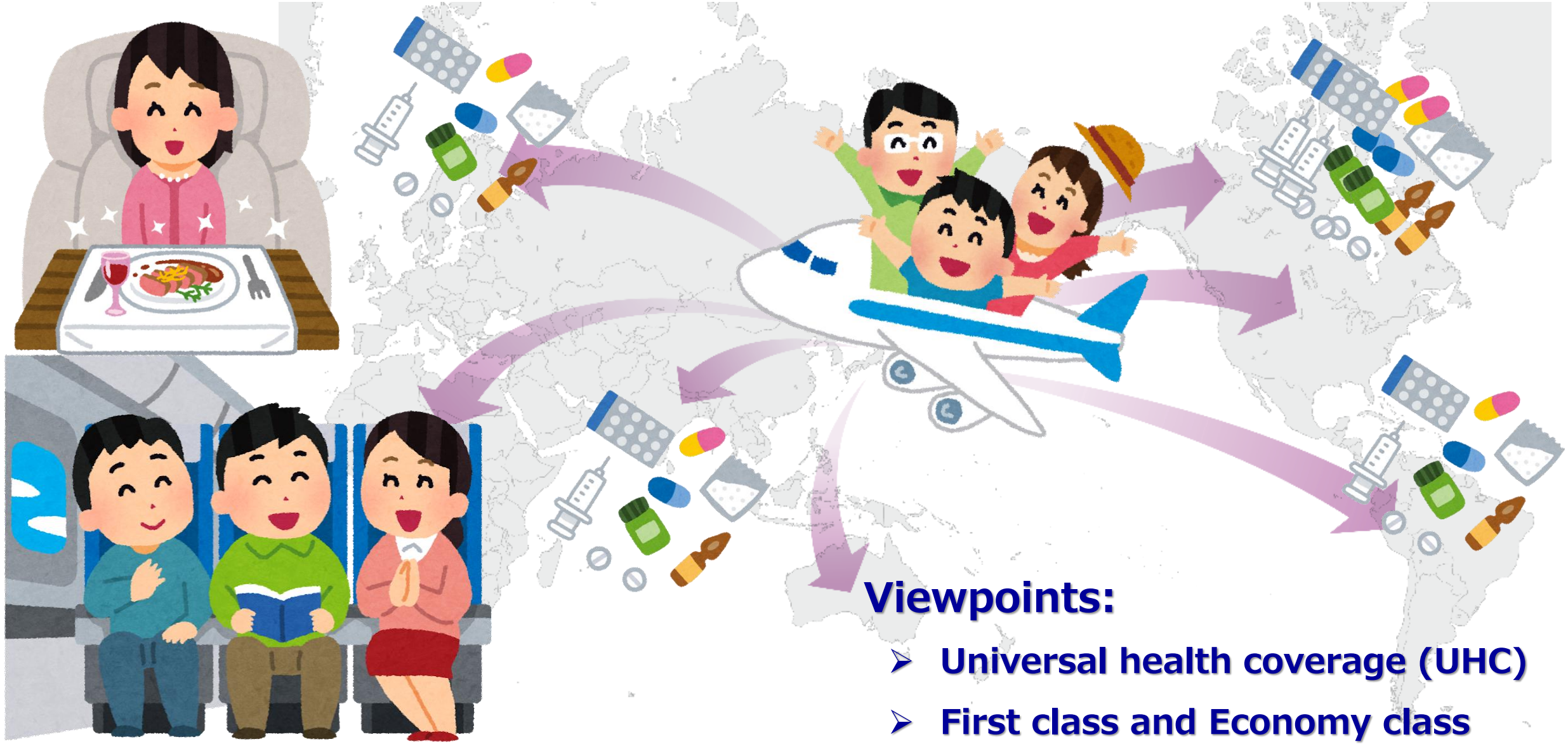
- 再生医療に関する**公的医療保険の適応範囲を整理**し、**民間保険の活用や新たな保険外併用療養費制度を検討**する
  - Define the scope of reimbursement for regenerative medicine, and consider the use of private insurance and a new type of uninsured combined health care payment system



What is the philosophy / mission of social security

Self-help, mutual assistance, public assistance

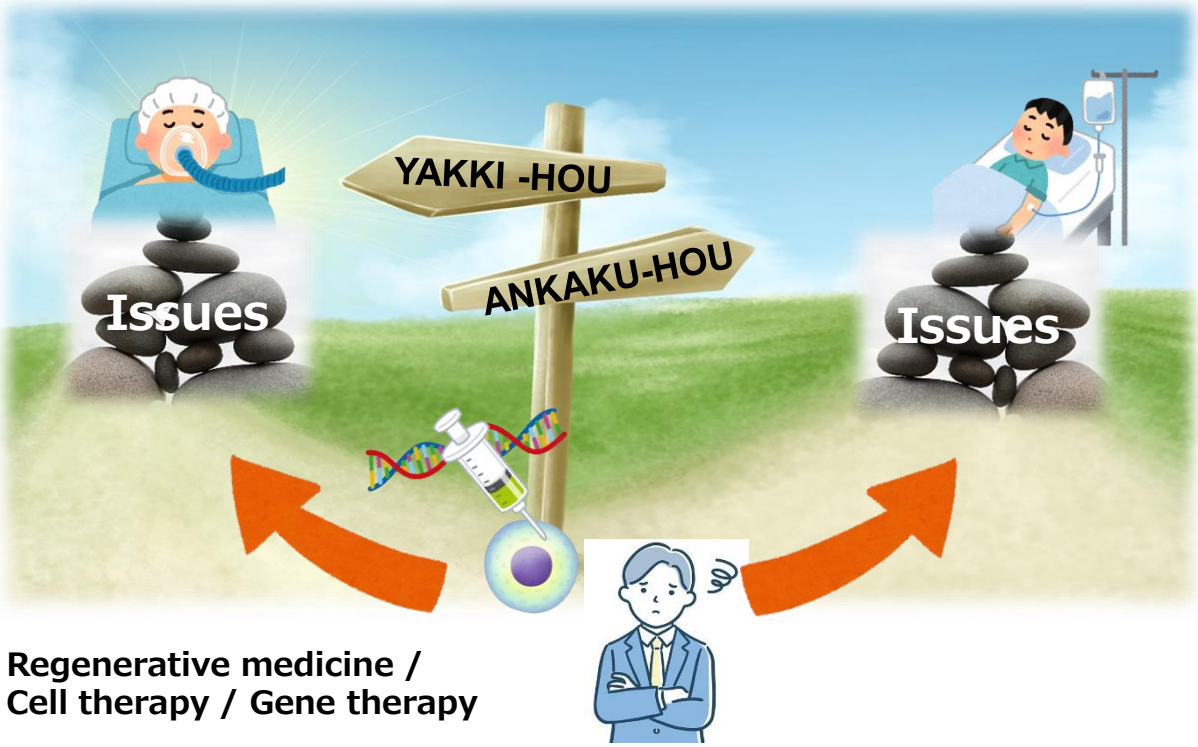
How should we consider appropriate medical prices?



Viewpoints:

- Universal health coverage (UHC)
- First class and Economy class

Current issues of Regenerative Medicine (Summary)



Regenerative medicine /
Cell therapy / Gene therapy

R&D Environmental improvement
(Pharmaceutical regulations, etc.)

Business Environmental improvement
(product price)

- **The development of R&D and business environments are fundamental for RM promotion.**
- **Concerned about the negative impact on product access in JP unless a business environment is improved that allows companies involved in RM to secure profits and invest in R&D.**

Source: FIRM press conference material (July 8, 2022)

Thank you very much for your attention.

FIRM YouTube チャンネル

* At the time of video release

PIVOT 公式 YouTube チャンネル

APPENDIX

Differences between RM products and Existing Pharmaceuticals

NOTE :Including some problems arising from biopharmaceuticals

		Regenerative Medicine	Small molecule / Biologics
Research and Development	Regulations	• JP unique local rules (SEIGENKI, Cartagena)	• International harmonization by ICH
	Patents	• Needs for multiple patents (license fee)	• Protected by substance patents
	HR	• Lack of specialized human resources	• Abundance
Manufacturing	Raw materials	• Dependent on donor availability • Inter-donor variability	• No sampling
	Manufacturing	• Difficulty in large-scale production	• Easy 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 hospital • Depend on the physician's procedure	• Widespread use
	No. of Patients	• Relatively small, incl. rare diseases • Autologous CT is personalized therapy	• Relatively large (incl. lifestyle diseases)

Source: Partially revised from Regenerative Medicine Innovation Forum, Opinion Report on Pricing of Regenerative Medicine Products (released on April 26, 2023)

Conditional and Time-Limited Approval

PMD Act*1 **Approval process for regenerative medicine products**

Current Approval Process

Clinical Research → Clinical trial (Confirmation of efficacy and safety) → Approval → Launch

Conditional and Time-Limited Approval Process

Clinical Research → Clinical trial (Confirmation of safety, **Estimation of Efficacy**) → **Conditional and Time-Limited Approval** → Additional validation of safety and efficacy (Clinical trial, PMS) → Approval or lapse → Launch

Faster access for patients!

Future use of RWD

① **Evaluation as a medical practice**

② **Objective third-party evaluation after early approval**

Use of the registry

*1 Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices