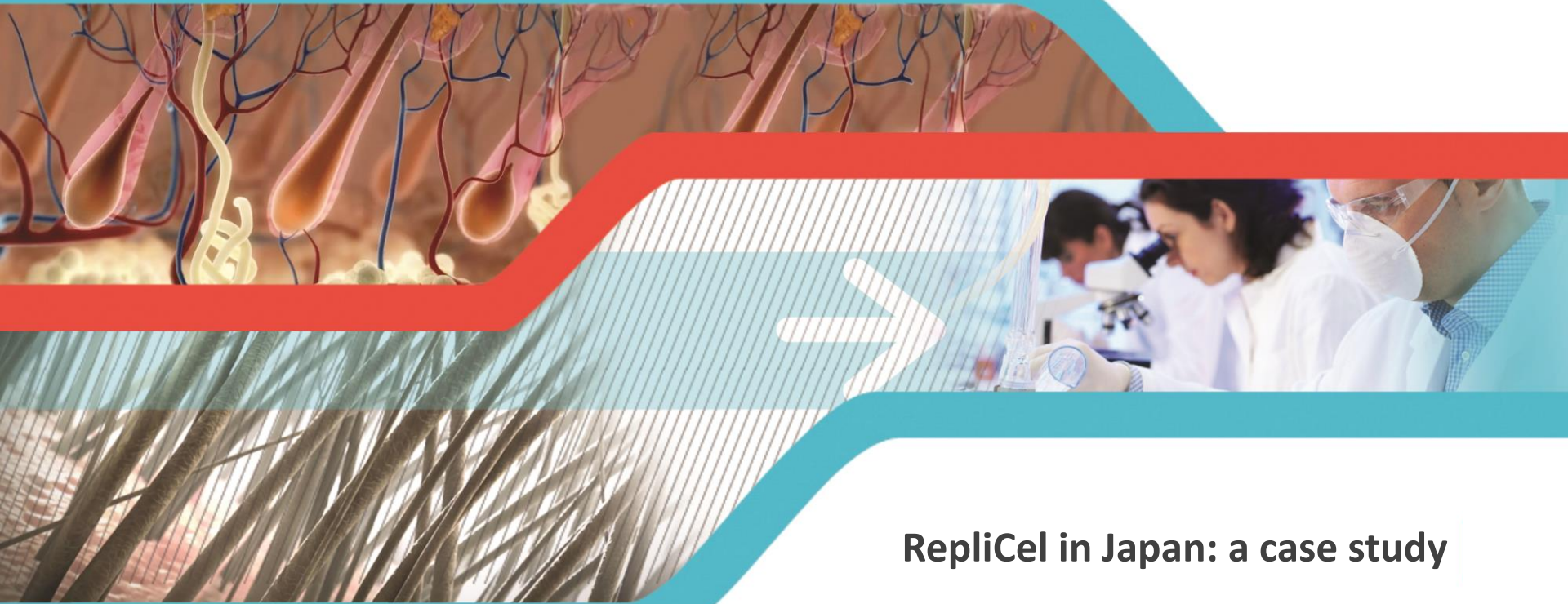




RepliCel

Using Cells for Healing



RepliCel in Japan: a case study

January 2016

Safe Harbor Statements

Statements included in this presentation that do not relate to present or historical conditions are “forward looking statements”. Forward-looking statements are projections in respect of future events or our future financial performance. In some cases, you can identify forward-looking statements by terminology such as “may”, “should”, “intend”, “expect”, “plan”, “anticipate”, “believe”, “estimate”, “predict”, “potential”, or “continue”, or the negative of these terms or other comparable terminology. Forward-looking information presented in such statements or disclosures may, among other things, include: the potential of our products, including its potential for success with women; forecasts of expenditures; the sources of financing; expectations regarding our ability to raise capital; our business outlook; plans and objectives of management for future operations; and anticipated financial performance.

Various assumptions or factors are typically applied in drawing conclusions or making the forecasts or projections set out in forward-looking information. Those assumptions and factors are based on information currently available to our Company, including information obtained from third-party industry analysts and other third party sources. In some instances, material assumptions and factors are presented or discussed elsewhere in this presentation in connection with the statements or disclosure containing the forward-looking information. You are cautioned that the following list of material factors and assumptions is not exhaustive. The factors and assumptions include, but are not limited to:

- no unforeseen changes in the legislative and operating framework for the business of our Company;
- a stable competitive environment; and
- no significant event occurring outside the ordinary course of business such as a natural disaster or other calamity.

These statements are only predictions and involve known and unknown risks, uncertainties and other factors, which may cause our or our industry’s actual results, levels of activity or performance to be materially different from any future results, levels of activity or performance expressed or implied by these forward-looking statements. These risks and uncertainties include:

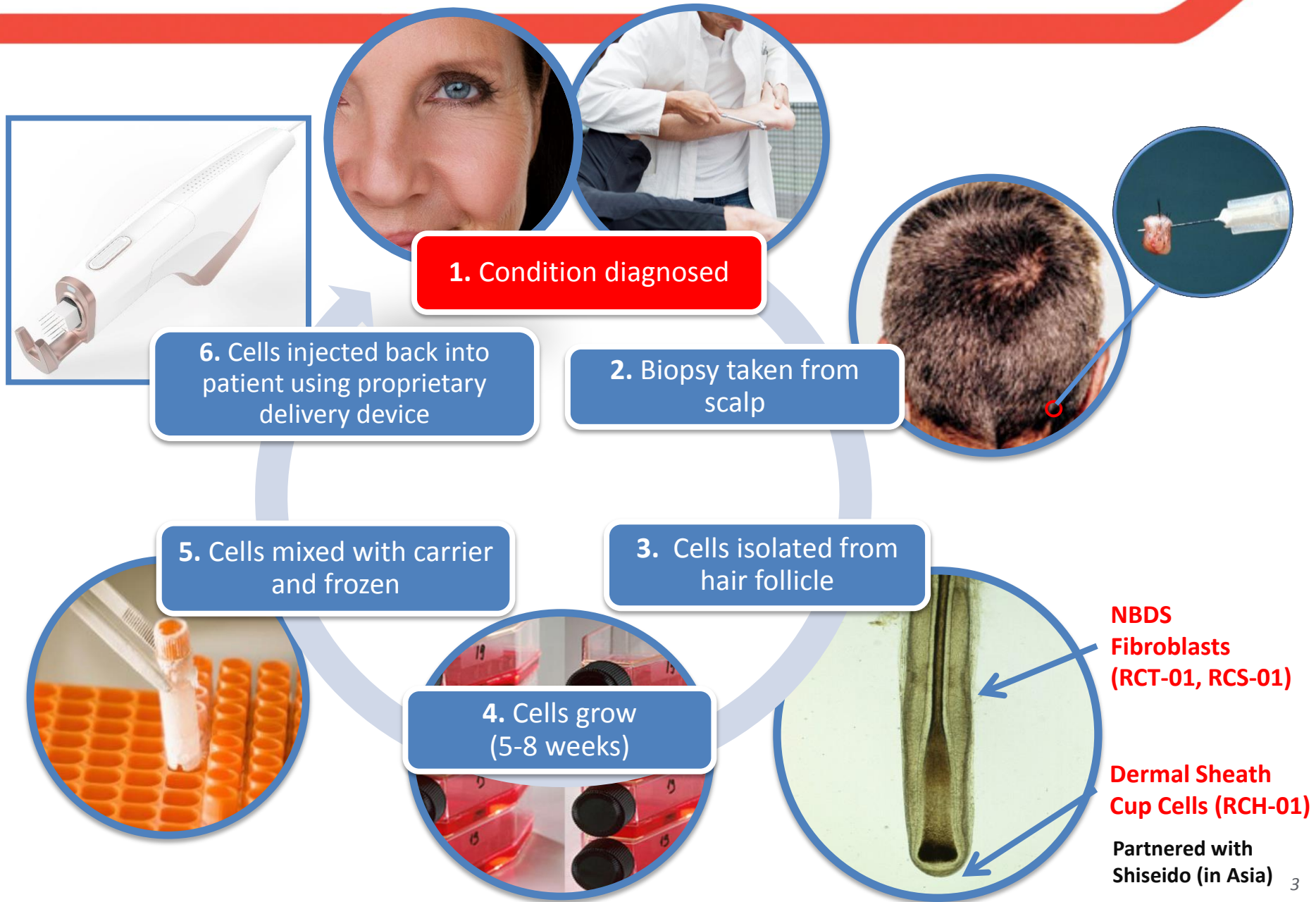
- negative results from any of our clinical trials;
- Failure to advance research and development programs into clinical trials;
- the effects of government regulation on our business;
- the viability and marketability of our cell replication technologies;
- our failure to successfully implement our marketing plan;
- the development of superior technology by our competitors;
- the failure of consumers and the medical community to accept our technology as safe and effective;
- the risk that publications on which certain data in this presentation are based are withdrawn or invalidated;
- risks associated with our ability to obtain and protect rights to our intellectual property;
- risks and uncertainties associated with our ability to raise additional capital; and
- other factors beyond our control.

Although we believe that the expectations reflected in the forward-looking statements are reasonable, we cannot guarantee future results, levels of activity or performance. Further, any forward-looking statement speaks only as of the date on which such statement is made, and, except as required by applicable law, we undertake no obligation to update any forward-looking statement to reflect events or circumstances after the date on which such statement is made or to reflect the occurrence of unanticipated events. New factors emerge from time to time, and it is not possible for management to predict all of such factors and to assess in advance the impact of such factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statement.

Readers should consult all of the information set forth herein and should also refer to the risk factor disclosure outlined in the Company’s annual report on Form 20-F for the fiscal year ended December 31, 2013 and other periodic reports filed from time-to-time with the Securities and Exchange Commission on Edgar at www.sec.gov and with the Canadian Securities Commissions on Sedar at www.sedar.com.

Not an Offer to Purchase or Sell Securities. This presentation is for informational purposes and is not an offer to sell or a solicitation of an offer to sell any securities in the Company, nor shall there be any sale of securities in any jurisdiction in which such offer, solicitation or sale would be unlawful prior to registration or qualification under the securities laws of such jurisdiction. This presentation may not be relied upon in connection with the purchase of any security. Securities of the Company, if offered, will only be available to parties who are “qualified investors”, pursuant to applicable securities laws.

RepliCel Platform



RepliCel Overview

- **Product portfolio – large commercial applications**
 - Two distinct cell therapy platforms built on hair follicle expertise
 - A unique dermal injector device

- **Near-term clinical pipeline**

NBDS	Chronic tendinosis	RCT-01	1/2	✓	Canada
NBDS	Aging and sun damaged skin	RCS-01	1	✓	Germany
DSC	Pattern Baldness	RCH-01	pivotal?	2015	Japan *

* funded by Shiseido

- **Near-term milestones**
 - 2016 clinical trial data (tendon repair and dermatology)
 - 2016 injector device CE mark

RepliCel Licensing Strategy

Maximum shareholder value creation is in discovery to mid-stage clinical development

- Develop early-to-mid-stage assets for license to partners for joint development and commercialization
- Partner assets prior to late-stage trials to leverage partner expertise, cash and infrastructure
- Only commercialize through experienced commercial partners
- Structure partnerships to allow for ongoing joint development
- Focus the first round of partnerships around geographic licenses for the Asian markets with initial commercialization in Japan

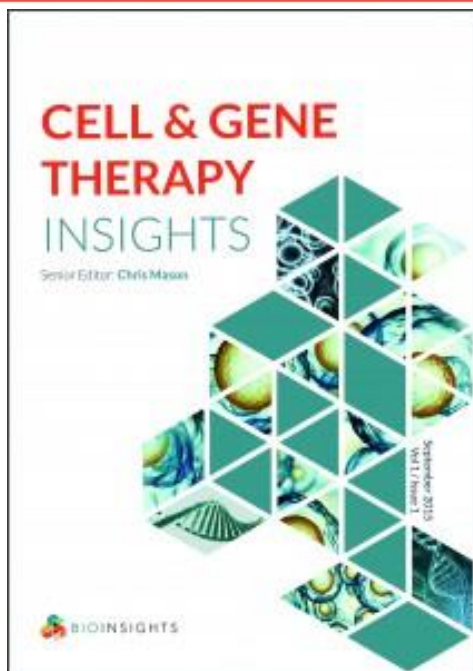




RepliCel™

and Japan

Recent Co-Authored Article on Japan



Japan's Regulatory Gamble and What it Means for the Industry

The Follower Takes the Lead:

How Japan's trifecta of economic policy, regulatory innovation, and an aging demographic may disrupt a global industry and the very future of medicine.

Submitted: July 30 2015 **Published:** September 15 2015; **DOI:** 10.18609.cgti.2015.009

<http://insights.bio/cell-and-gene-therapy-insights/journal/>



Shiseido Partnership – RCH-01 – Asia



4th largest cosmetic company WW 2015

Licensing partnership with Shiseido (July 2013)

- Based on data from a 16-patient ph 1/2a open-label trial done in Eastern Europe
- Geographic license for pattern baldness only for Japan, China, Korea and ASEAN nations
- \$35 Million (\$5M upfront, \$31M in sales milestones and royalties)

Joint product and clinical development – shared data

Shiseido's manufacturing and clinical trial in Japan

- May 2014 Shiseido opened new cell-processing facility; PMDA certified 2015
 - Tech transfer & comparability/validation runs completed 2015
- Clinical protocol under ASRM (clinical research pathway) currently in review by MHLW
- Trial expected to readout late 2017 with potential to predicate a market approval application

RepliCel + Shiseido RCH-01 Partnership

- July 2013: exclusive geographic license for Japan, China, Korea and ASEAN nations
- May 2014: Shiseido opened **SPEC** (**S**hiseido cell-**P**rocessing & **E**xpansion **C**entre) facility in Located at Kobe Biomedical Innovation Cluster (KBIC)
 - Japanese centre for regenerative medicine
 - Capable of handling all aspects of the RCH-01 technology
 - Tissue-sample processing, manufacturing and product packaging
- 2015 SPEC was PMDA certified



Business Support Centre for Biomedical Research Activities



Kobe Biomedical Innovation Cluster

Impact of Shiseido Licensing Partnership

RepliCel:

- Only 1 of 2 foreign cell therapy companies with a cell manufacturing facility in Japan (through its partner)
- One of only a handful of foreign cell therapy companies with a Japanese partnership already in place
- One of the few foreign cell therapy companies with a clinical trial poised to commence in Japan
- A credible profile in the Japanese market amongst investors, strategic partners, and government
- Frequently in Japan to participate in industry affairs
- Strong relationship with METI, JETRO and PMDA
- Actively engaged in Japanese partnership discussions around other assets

RepliCel Next Steps in Japan

- Actively seeking licensing partners for other products
 - NBDS platform products
 - RCT-01 tendon repair
 - RCS-01 dermal rejuvenation
 - RCI-02 dermal injector
- Engaging in PMDA review of NBDS platform
 - Completed multiple pre-consultation meetings
 - Preparing for Manufacturing Quality/Safety consultation
- Actively engaging with investors re: funding domestic collaboration
- Working with Japanese agencies to support growth of the regenerative medicine industry



RepliCel™

Foreign Cell Therapy
Companies in Japan

Corporate Developments in JPN Regenerative Medicine

As Japan looks to further its standing as a global leader in the field of regenerative medicine, both domestic and foreign companies are looking to take advantage of this truly unique turn of events.

Foreign Companies Looking Towards Japan

Company Name	Japan Developments
Athersys (ATHX:NASDAQ)	Announced partnership and license agreement with Healios K.K. for novel cell therapy treatments including MultiStem on 08 JAN 2016
Avita Medical (AVH:ASX)	Announced signing of a distribution agreement with INDEE Medical for their ReCell® device on 14 JAN 2016
Cynata (CYP:ASX)	Japanese firm Regience K.K. announced that they had signed an LOI regarding a future strategic alliance with Cynata on 03 DEC 2015
Cytori (CYTX:NASDAQ)	Announced first patient enrollment/treatment in their Japanese physician-initiated ADRESU trial for Cell Therapy™ on 03 SEPT 2015
Mesoblast (MESO:NASDAQ)	TEMCELL® HS Inj. (Prochymal) approved in Japan on 18 SEPT 2015 and NHI price commencement date was on 26 NOV 2015
Pluristem (PSTI:NASDAQ)	Announced on 21 DEC 2015 that it had reached an agreement with the PMDA on the design for a 75-patient trial for PLX-PAD
RepliCel (RP:TSX.V)	Announced a Collaboration and Technology Transfer Framework Agreement with Shiseido for their RCH-01 therapy on 29 MAY 2013
Regeneus (RGS:ASX)	Announced that they were targeting Japan for their allogeneic off-the-shelf cell therapy for OA, Progenza, on 26 NOV 2014

Japanese Pharma on the Move

Company Name	Regenerative Medicine Developments
Astellas (4503:TSE)	Announced the establishment of a Regenerative Medicine unit on 01 APR 2014, and to announce acquisition of Ocata Therapeutics on 10 NOV 2015
Fujifilm (4901:TSE)	In addition to the continued efforts of subsidiary J-TEC, Fujifilm also announced the acquisition of Cellular Dynamics International on 30 MAR 2015.
Kaneka (4118:TSE)	Announced the establishment of an R&D facility in Kobe on 27 NOV 2015; they aim to develop amniotic stem cell therapies
Kyowa Kirin (4151:TSE)	On 03 DEC 2015, multiple news outlets reported that Kyowa Kirin was teaming up with CiRA investigate cancer immunotherapies using iPSCs
ReproCELL (4978:TSE)	Announced the acquisition of Biopta on 24 NOV 2015
Rohto (4527:TSE)	On 08 JAN 2016, news sources reported that Nuo Therapeutics and Rohto had signed an exclusive licensing/Distribution Agmt for Aurix in Japan
Sumitomo Dainippon (4506:TSE)	Looking to invest 2.2b JPY (~19m USD) to create a facility dedicated to culturing iPSCs, to help develop Japan's first iPSC drug
Takeda (4502:TSE)	Takeda announces its intent to fund collaborative research in iPSC applications to the tune of 20b JPY (~170m USD); with a new facility built DEC 2015

Regenerative Medicine R&D in Japan

In a testament to Japan's seeming greater focus on cellular-based technologies, there are many more early stage R&D products for "Cell Therapies" than there are "Gene Therapies" in Japan. However, both types of therapies are considered to be regenerative medical products within the PMD. Act.

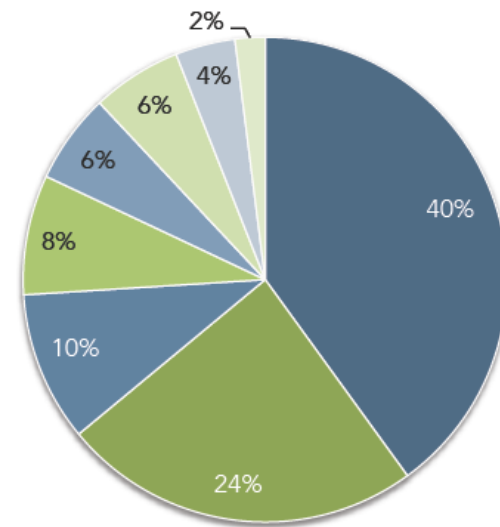
R&D Status of Regenerative Medicine aimed for Japan

Stage	Cell Therapies	Gene Therapies	Companies
Research Project*1	6	1	【Sumitomo Dainippon/Healios/SighRegen (1)】, 【Cardio (1)】, 【SBI Biotech (1)】, 【Regience/Osaka University (1)】, etc.
Pre-Clinical	13	1	【CellSeed (2)】, 【J-TEC (1)】, 【Caladrius Biosciences (1)】, 【Pluristem (1)】, 【Nagoya University (7)】, etc.
Phase I	10	3	【Regeneus (1)】 【MEDINET (1)】, 【Athersys (1)】, 【RepliCel (2)】, 【MolMed (1)】, 【ID Pharma (1)】, etc.
Phase II	4	5	【MEDINET (1)】, 【Caladrius Biosciences (1)】, 【National Cerebral and Cardio Center (1)】, 【Shiseido/RepliCel (1)】, 【AnGes MG (1)】, etc.
Phase III	2	1	【Rohto/Nuo (1)】, 【Cytori (1)】, 【AnGes MG/Sosei/Mitsubishi Tanabe/Osaka University (1)】
Approved/Marketed	4	0	【JCR Pharma/Mesoblast (1)】, 【Terumo (1)】, 【J-TEC (2)】

red font = cell therapies
orange font = gene therapies

Therapeutic Categories

[n=50]



- Oncology & Immunomodulators
- Cardiovascular
- Dermatology
- Gastro-Intestinal
- Musculoskeletal
- Genito-Urinary
- Sensory Organ
- CNS

The "Oncology & Immunomodulators" and "Cardiovascular" categories combine to account for 64% of the regenerative medical products currently being developed in Japan.

The "Musculoskeletal" category occupies a lower position than has been predicted for the industry.

Foreign Cell Rx Company Partnerships in Japan

- **Mesoblast & JCR Pharmaceuticals**

- MBTLY came to JCR through acquisition of Prochymal assets from Osiris
- Prochymal (allogeneic, bone marrow-derived MSC): approved in CDA & NZ for refractory pediatric graft-versus-host disease (GvHD)
- Mesoblast is currently conducting a 60-patient P3 trial in children with steroid-refractory acute Graft Versus Host Disease in the US.
- JCR conducted a 25-patient single-cohort trial in Japan; JCR has manufacturing in place in Japan
- December 2014: JCR files for marketing approval for JCR-031 (Prochymal) based on JCR study; granted orphan drug priority review
- September 2015: TEMCELL® HS Inj. (aka JCR-031) was recommended for approval by Committee on Regenerative Medicine Products and Biological Technology of Pharmaceutical Affairs and Food Sanitation Council of the Japan Ministry of Health, Labour and Welfare and subsequently received approval, making it the first allogeneic therapy approved in Japan.
- November 2015: NHI announced reimbursement coverage ¥13,898,800 (~ \$117,530 USD)

Foreign Cell Rx Company Partnerships in Japan

- **Terumo**
 - 2007: Began developing cell sheets for cardiac repair
 - 2012: Commenced clinical trial in Japan
 - October 2014: Applied for approval to MHLW
 - September 2015: Received 5-year conditional approval from PMDA for HeartSheet (aka TCD-51073) - autologous skeletal myoblast sheets for severe heart failure caused by chronic ischemic heart disease.

Foreign Cell Rx Company Partnerships in Japan

- **Living Cell Technologies & Otsuka Pharmaceutical Factory**
 - 2011 JV company established called Diatranz Otsuka Limited (DOL) to bring DIABECELL (encapsulated porcine-derived islets in development for treatment of T1 diabetes) to market
 - 2014 Otsuka Pharmaceutical Factory licensed DIABECELL from DOL for Japanese and US markets
- **Cytori Therapeutics**
 - Tokyo-based medical subsidiary Cytori Therapeutics KK was established largely to support the sale of the Cellution System for autologous, point-of-care, adipose (fat)-derived regenerative cells into the Japanese market for treatment of various conditions
 - Sept 2015: announced 1st pt enrollment in phase 3 (pivotal clinical research) PI-initiated ADRESU trial (multi-center, 45 patients, open label) for urinary incontinence related to prostate cancer

Other Foreign Cell Rx Company Activity in Japan

- **Collectis** – 2014 sold Collectis AB (company focused on applications of human embryonic stem cell products and technologies) to Takara Bio
- **Stemgent, Inc.** – 2014 ReproCELL acquired the iPS Cell Business Unit of Stemgent
- **PluriStem** – 2014 announced they are evaluating co-development and commercialization opportunities for its PLacental eXpanded cell therapies in Japan
- **Athersys**
 - 2015 announced an exclusive license to and partnership with Chugai Pharma (a member of the Roche Group) for MultiStem (an allogeneic bone marrow-derived stem cell product) for the treatment of ischemic stroke in Japan.
 - 2015 clinical trial failed to meet endpoints; Chugai terminated agreement
 - Jan 2016 Athersys announced partnership and license agreement with Healios K.K.
- **SanBio** – 2014 announced joint development and license agreement with Sumitomo Dainippon Pharma for exclusive marketing rights for SB623 in US and Canada



RepliCel™

Impact on
Industry

FAQs Re: Future of Cell Therapies in Japan

- **Can therapeutic candidates developed and manufactured outside of Japan now qualify to utilize the new regulatory framework?**
 - While data from trials outside Japan can be used to support applications for conditional approval, a bridging study involving Japanese patients will be required.
- **Will a Japanese partner be needed to bring a product to the PMDA for conditional approval?**
 - A Japanese entity will likely be required to be a sponsor. It could be a Japanese subsidiary rather than partner. A national representative is required even to set up a meeting with the PMDA.
- **Are Japanese companies showing an appetite to partner with foreign companies to bring cell therapies to Japan?**
 - Yes and not just Japan-based biopharma or healthcare companies. Japan based Life Science investors are also positioning to play a role in these partnerships.
- **Will the dearth of venture capital in Japan dictate that the commercialization of cell therapies be different than in other countries?**
 - We expect yes. Investment arms of big companies, new divisions of big companies, newco's set up by investors, etc. will all likely play an active role. But Prime Minister Abe is also trying to incentivize more venture capital.

Advice for Foreign Cell Therapy Co's

- Develop a clear strategy for what you are looking to do in Japan
 - At least determine what is off the table
- You will need to spend frequent time in Japan to communicate commitment to doing business there. Personal interaction is a must.
- Participate in bio-partnering conferences in Japan
- Identify a national representative – point-of-contact
- Have a Japanese-speaker on your team
- Prepare in advance for intense scientific and technical due diligence with as much as possible in writing

Advice for Foreign Cell Therapy Co's Cont'd...

- Set up a direct dialogue with the PMDA
- Leverage your embassy in Japan
- Leverage Japan Bioindustry Association, Foundation for Innovative Regenerative Medicine (FIRM), Japanese Regenerative Medicine Society, Asian Society for Cellular Therapy, Ministry of Economy, Trade and Investment (METI), Japanese External Trade Organization (JETRO), etc.
- Seek out Japanese nationals at conferences in North America
- Consider establishing a Japanese manufacturing footprint to serve both the Japanese and broader Asian market
- Strongly consider Japanese partners for a pan-Asian license

RepliCel Life Sciences Inc.

OTCQB: REPCF

TSXV: RP



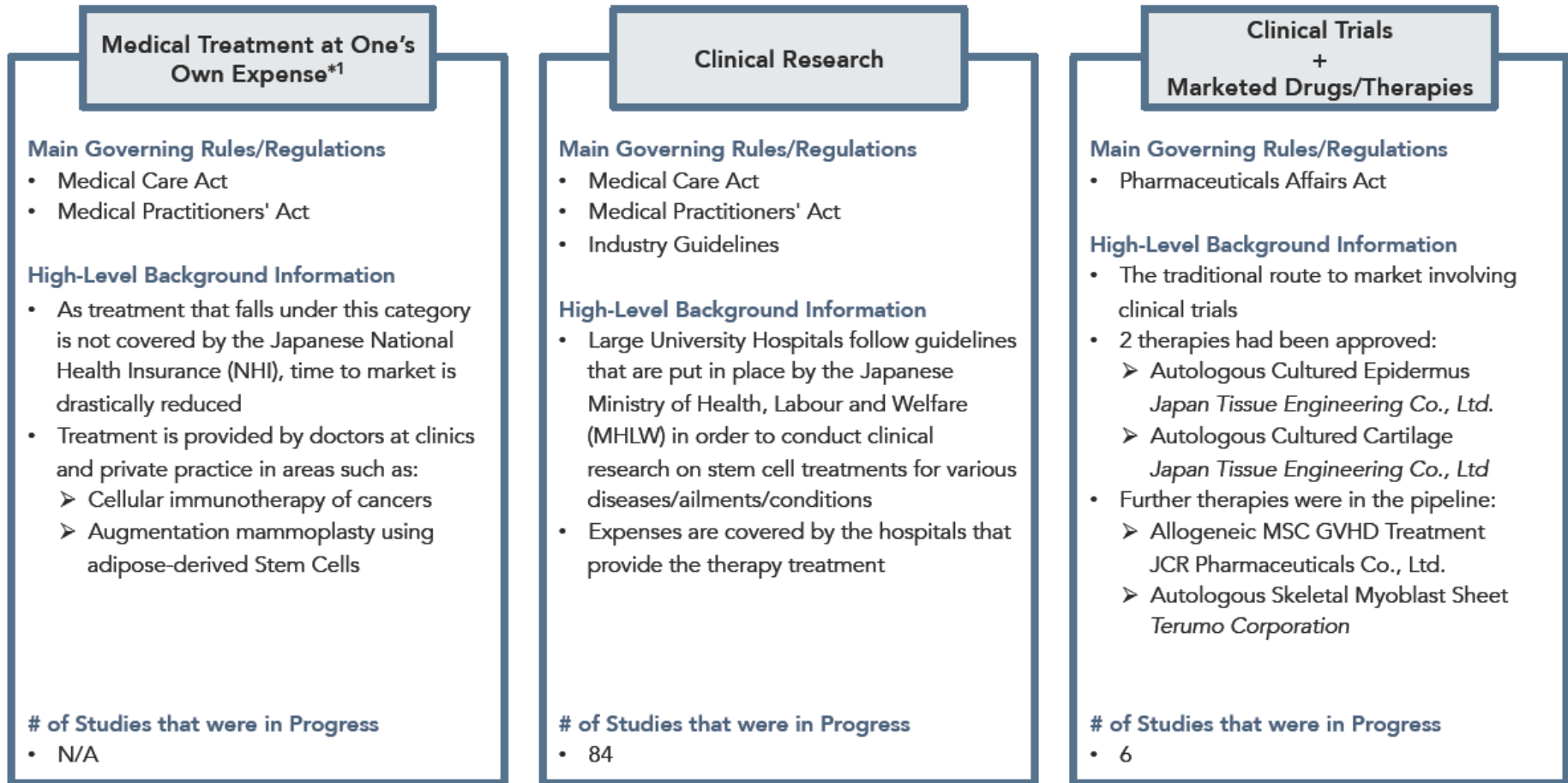
RepliCel™

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Prior Regenerative Medicine Regulatory Landscape

The “Act on the Safety of Regenerative Medicine” and a revision to the “Pharmaceutical Affairs Act” were promulgated by the Japanese government on Nov. 27, 2013 with the aim of providing a route to market that was more in sync with current industry/patient needs. Both laws came into force the following year on Nov. 25, 2014.

Japanese Regenerative Medicine Landscape Prior to Nov. 25, 2014



High-Level Introduction to Japan's Regulatory Environment

Japan has two laws regulating its regenerative medicine market: the Act on the Safety of Regenerative Medicine (ASRM) and the revised Pharmaceutical Affairs Act (PMD. Act).

ASRM

Category	Comment
Official Name	Act on the Safety of Regenerative Medicine (ASRM)
Effective Date	November 25, 2014*1
Purpose	<ul style="list-style-type: none"> Establish steps for the practice of regenerative medicine in order to ensure the safe and ethical administration of regenerative medical technologies Ensure the safe yet accelerated adoption of specific processed cellular products by establishing a manufacturing permit system
Key Definitions	<ul style="list-style-type: none"> Regenerative Medicine: Medical care that involves the use of regenerative medical technologies. Regenerative Medical Technologies: Medical care that involves the use of processed cellular products to reconstruct/restore/repair the human body (or its functions) or to cure/prevent a disease. Depending on their risk-level, these technologies are sub-divided into 3 classes. Specific Processed Cellular Products: Processed cellular products produced under the guidance of a medical institution, for the purposes of "clinical research" or "medical treatment at one's own expense", and meant for the treatment of a specific patient.

PMD. Act

Category	Comment
Official Name	Pharmaceuticals and Medical Devices Act (PMD. Act)
Effective Date	August 10, 1960
Purpose	<ul style="list-style-type: none"> Revise the previous Act so as to provide a route to market for regenerative medicine that is more in line with the current industry/patient needs Establish regulations for regenerative medicine that are independent from regular ethical drugs, medical devices, and non-medical/cosmetic products
Key Definitions	<ul style="list-style-type: none"> Regenerative Medical Products: Medicinal products, produced by corporate entities for an unspecified large number of people, that involve human/animal cell culturing in order to: <ul style="list-style-type: none"> ➢ Reconstruct/restore/repair the human/animal body ➢ Cure/prevent a human/animal disease ➢ Obtain a gene expression Conditional Approval: A system of approval put in place for those regenerative medical products that have all of the following conditions, and allow for the sale of said products for up to 7 years: <ul style="list-style-type: none"> ➢ They do not have any major safety concerns ➢ They have "probable" efficacy ➢ They are not uniform in nature

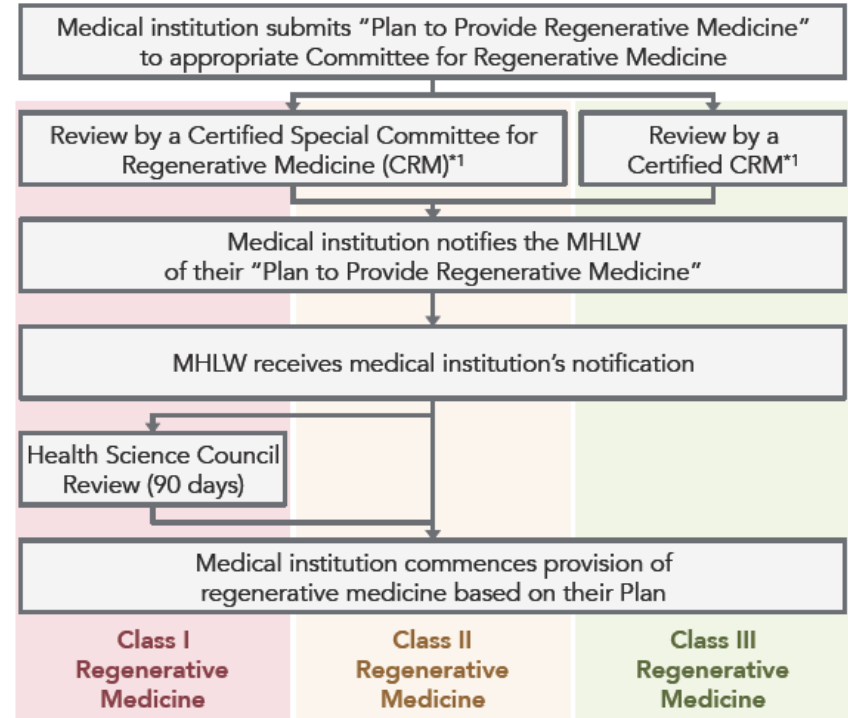
Act on the Safety of Regenerative Medicine (1/2)

The wording of the ASRM necessitates that doctors be the gate keepers of treatment. Pharmaceutical companies that would like to provide their drug/therapy to patients under this law would need to operate in non-traditional sectors of the market, such as operating as a Cell Processing Center (CPC), or receiving royalty payments.

3 Risk Categories of Regenerative Medical Technologies

Category	Legal Definition	Comments
Class I Regenerative Medicine (High Risk)	The effects of the regenerative medical technology on the patient's well-being are either: <ul style="list-style-type: none"> • not readily apparent; or • the effects of the regenerative medical technology on a patient's wellbeing are potentially harmful 	Regenerative medical technologies that fall under this category are generally those that utilize iPS or ES cells
Class II Regenerative Medicine (Medium Risk)	The effects of the regenerative medical technology on the patient's well-being have the potential to have negative repercussions despite providing due care	Regenerative medical technologies that fall under this category are generally those that utilize somatic (adult) stem cells
Class III Regenerative Medicine (Low Risk)	Regenerative medical technology that does not fall under the other 2 risk categories	Regenerative medical technologies that fall under this category are generally those that utilized processed somatic (adult) cells

Route to Market under the ASRM

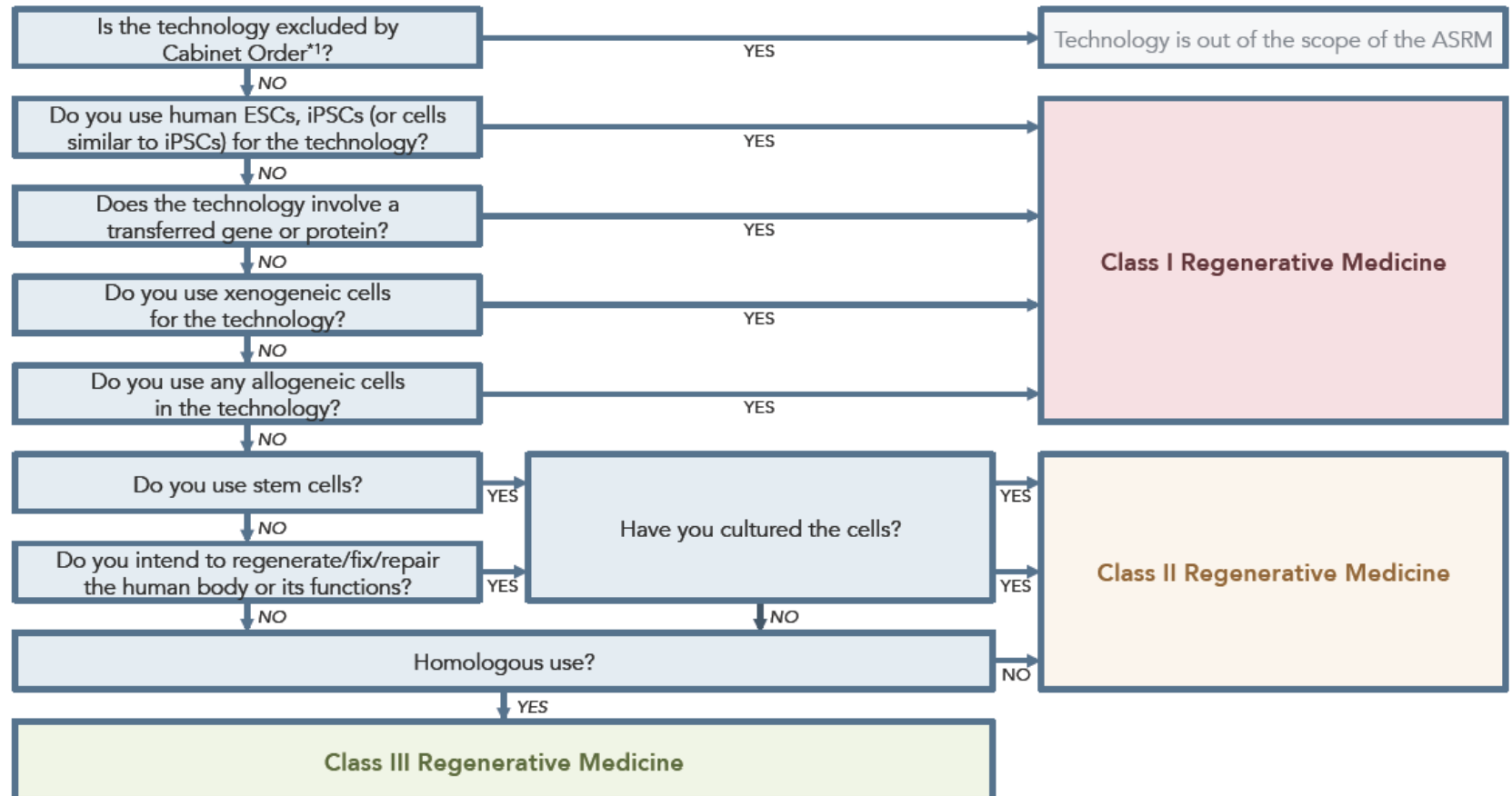


- As treatments under the ASRM must be provided by a medical institution for the purposes of "medical research" or as a "medical treatment at one's own expense", therapies provided under this framework are not covered by Japan's NHI.

Act on the Safety of Regenerative Medicine (2/2)

The risk categorizations under the ASRM are determined as follows. Almost all pertinent uses will fall either under the Class I or Class II Regenerative Medicine categories.

Determining Risk Categorization of Regenerative Medical Technologies



Pharmaceuticals and Medical Devices Act (1/4)

The Pharmaceuticals and Medical Devices Act (PMD. Act) instituted a conditional approval system for Regenerative Medical Products in Japan. The minute details of this new path are still being defined, but Terumo Corporation became the first company to receive conditional approval with its HeartSheet product on Sept. 18, 2015.

Conditional Approval for Regenerative Medical Products

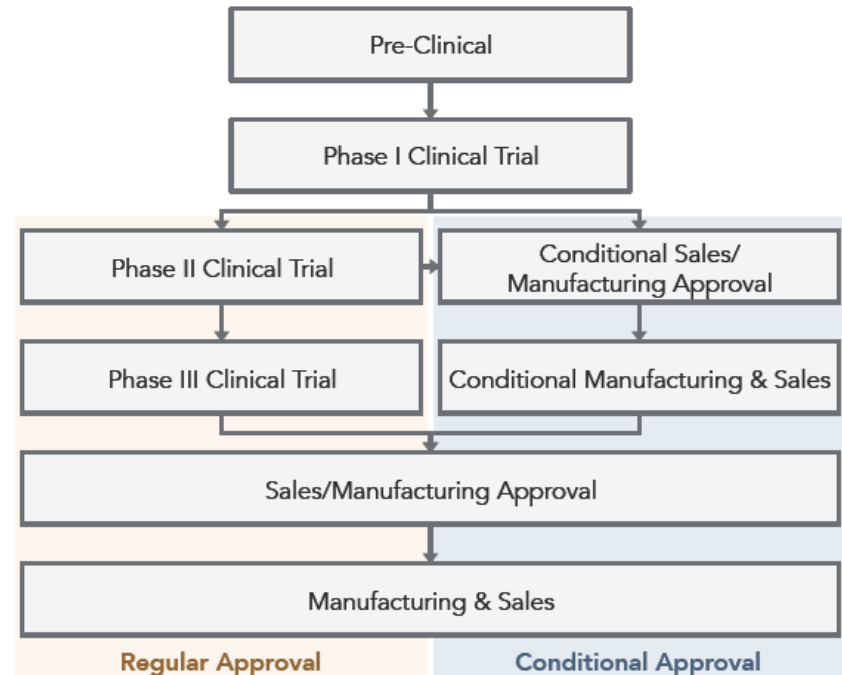
The PMD. Act differs from the law that it revised (i.e. the PAA: Pharmaceutical Affairs Act) by the inclusion of Regenerative Medical Products as a stand-alone medical category with a novel “conditional approval” system. This system is summarized below:

1. If the Regenerative Medical Product, that a corporate entity is looking to obtain sales/manufacturing approval for, satisfies all of the following conditions, then said entity can obtain input from a sub-committee of the Pharmaceutical Affairs and Food Sanitation Council and receive conditional approval for said Regenerative Medical Product’s release:
 - It does not have any major safety concerns
 - It has “probable” efficacy
 - It is not uniform in nature
2. Entities that receive conditional approval for a specific Regenerative Medical Product must re-apply for a full release within the timeframe provided to them under said approval (no longer than 7 years)

Regenerative medical products are oftentimes produced by processing cells. This “processing” can introduce certain risks including “the manifestation of additional properties that differ from the cells that were originally processed” and “an inconsistency of quality.” To help adequately deal with these inherent risks, regenerative medical products that are provided conditional approval must stay within the following boundaries:

- They must not be carcinogenic
- Conditional approval must not last longer than 7 years, and during this period measures must be taken to ascertain the proper use of the regenerative medical products
- Upon re-application they must demonstrate adequate efficacy and safety

Route to Market under the PMD. Act



- Conditional approval is not guaranteed for Regenerative Medical Products that meet the requirements delineated at left. Rather the PMDA reserves the right to decide on which Regenerative Medical Products will be allowed the shortened path to market.

Pharmaceuticals and Medical Devices Act (2/4)

Japan's NHI is only supposed to cover those items that have demonstrated clinical efficacy and allowing conditionally approved therapies to be covered by NHI was counterintuitive. However, by treating them in a similar manner to orphan drugs, the MHLW was able to extend insurance coverage to conditionally approved regenerative medical products.

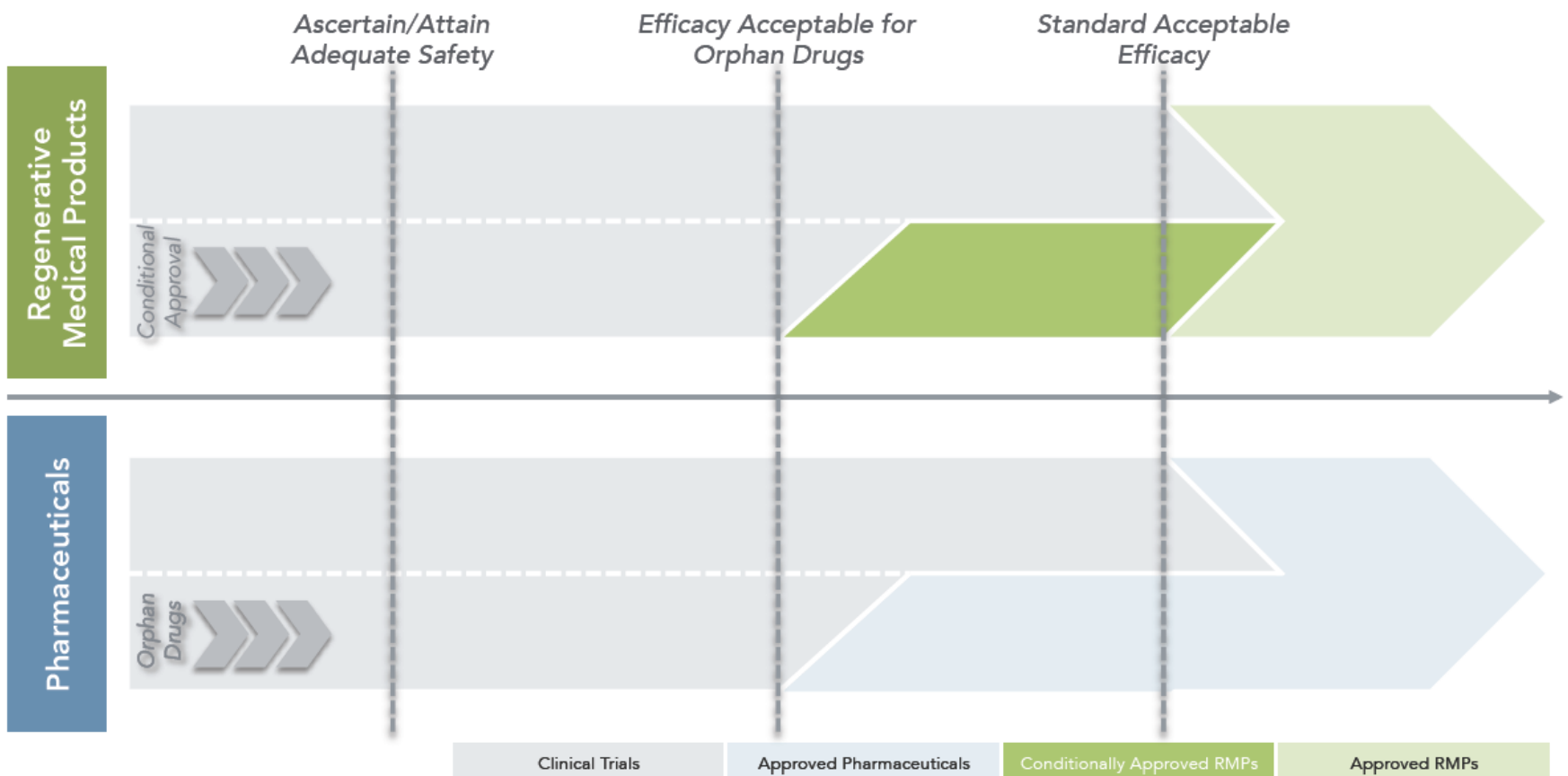
Conditional Approval for Regenerative Medical Products vs. Regular Approval for Pharmaceuticals

Drug Category	Clinical Trials	Efficacy Evidence	Post Approval
Pharmaceuticals	<ul style="list-style-type: none"> Evaluation is conducted on suitably sized clinical trials which are determined based on disease characteristics 	<ul style="list-style-type: none"> Controlled clinical trial that exhibits a statistically significant difference 	<ul style="list-style-type: none"> Conduct post-marketing observation studies (PMOS) as necessary
Orphan Drugs	<ul style="list-style-type: none"> Evaluation oftentimes needs to be done based on a small number of study participants due to patient scarcity Controlled clinical trials are oftentimes difficult to perform 	<ul style="list-style-type: none"> There are cases when it is difficult to conduct rigorous statistical analysis 	<ul style="list-style-type: none"> Follow-up investigations conducted on all patients (and/or follow-on clinical trials) so as to accumulate adequate data <i>-stipulated condition of approval-</i> Limit the number of medical institutions that are allowed to administer the drug so as to ensure proper use <i>-stipulated condition of approval-</i>
Regenerative Medical Products (Conditional Approval)	<ul style="list-style-type: none"> Evaluation oftentimes needs to be done based on a small number of study participants due to patient scarcity Controlled clinical trials are oftentimes difficult to perform Cellular heterogeneity make it difficult to evaluate based on a fixed/limited number study participants 	<ul style="list-style-type: none"> There are oftentimes cases when it is difficult to conducts rigorous statistical analysis 	<ul style="list-style-type: none"> Follow-up investigations conducted on all patients (and/or follow-on clinical trials) so as to accumulate adequate data <i>-stipulated condition of approval-</i> Limit the number of medical institutions that are allowed to administer the drug so as to ensure proper use <i>-stipulated condition of approval-</i> Limit the number of years of approval to a term of no more than 7 years <i>-stipulated condition of approval-</i>

Pharmaceuticals and Medical Devices Act (3/4)

Treating conditionally approved regenerative medical products along the same lines as orphan drugs extends beyond allowing them to be covered by Japan's NHI. The level of efficacy that one needs to obtain for conditional approval is also analogous.

Conditionally Approved Regenerative Medical Products ≈ Orphan Drugs

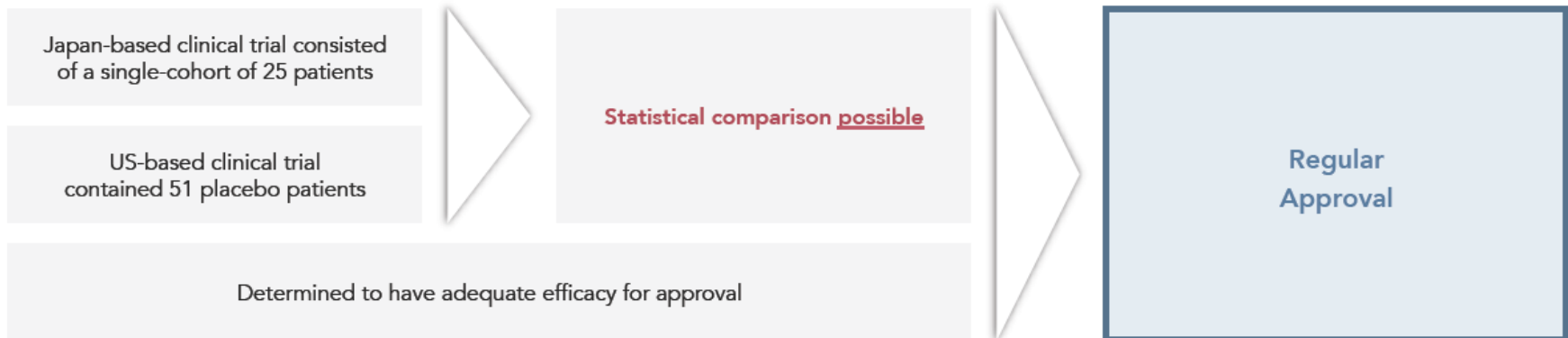


Pharmaceuticals and Medical Devices Act (4/4)

Whether one is able to obtain conditional approval is at the discretion of the regulatory authorities and guidelines are not yet available. However, the recent approvals of TEMCELL® HS Inj. and HeartSheet provide indications of what the regulators look for when they provide conditional approval (i.e. instances where statistical comparison is difficult).

TEMCELL® HS Inj.

[JCR Pharmaceuticals]



HeartSheet

[Terumo Corporation]

