Clinical Development of Cell and Gene Therapy (CAGT) Products in Japan

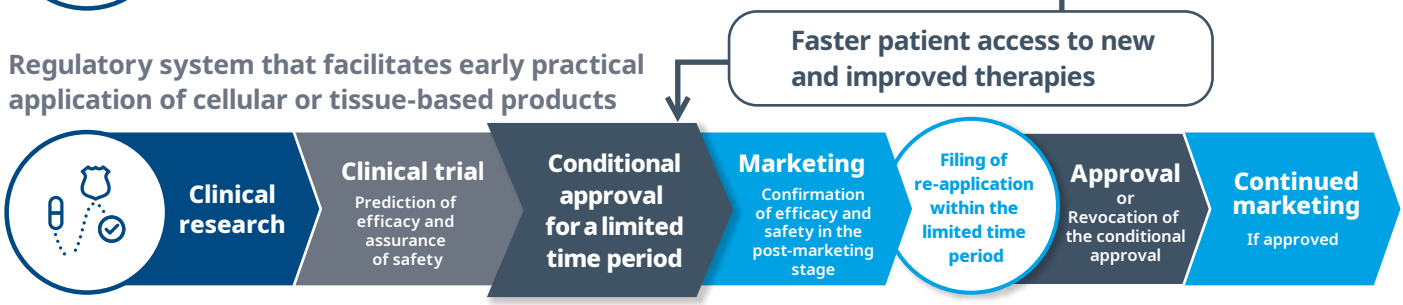
The 2nd largest market for innovation

Accelerated approval system for regenerative medical products

- The MHLW* introduced an updated approval system that rewards developers with **accelerated approvals to encourage clinical development of CAGT products in Japan.**
  * MHLW : Ministry of Health, Labor and Welfare
- Previously, clinical trials that evaluated the efficacy and safety were required for approval but **under the new system sponsors can obtain approval if efficacy is predicted and safety is demonstrated.**

Conventional regulatory approval process

Regulatory system that facilitates early practical application of cellular or tissue-based products



Cell, gene and tissue therapies which treat severe diseases are typically eligible to this system.

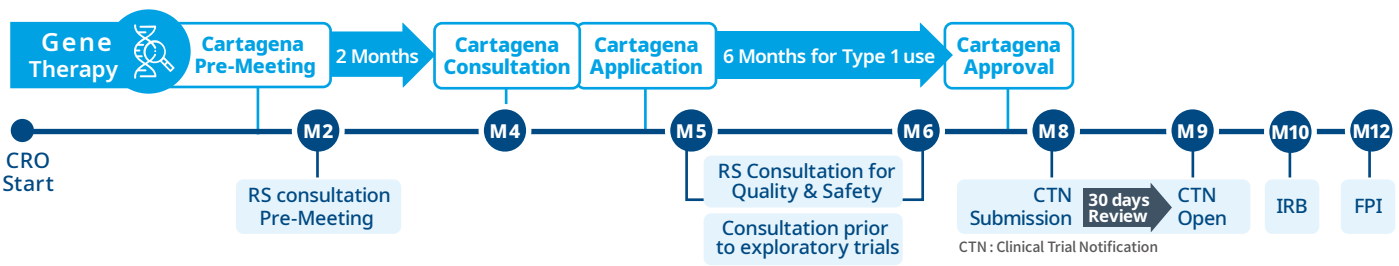
Path to FPI

Required Meetings and Rough Timelines Leading to Clinical Development

Important points to consider for CAGT development in Japan

The PMDA requires sponsors to conduct the following consultations:

1. Regulatory Science (RS) Consultation for Quality (e.g. Process, Specification, Testing method, Biological materials standard)
2. RS Consultation for Safety (e.g. Tumorigenicity test)
3. Cartagena Consultation (e.g. Satisfaction and appropriateness of submission documents for gene therapy)
4. Consultation prior to starting exploratory clinical trials (e.g. Protocol design, Clinical study package)



CTN : Clinical Trial Notification

IQVIA, the leading CRO in Japan

Supporting CAGT products from pre-clinical to post marketing

Regulatory Affairs team with the **capability** and experience to support **specific Japanese regulatory requirements** for **CAGT** products.

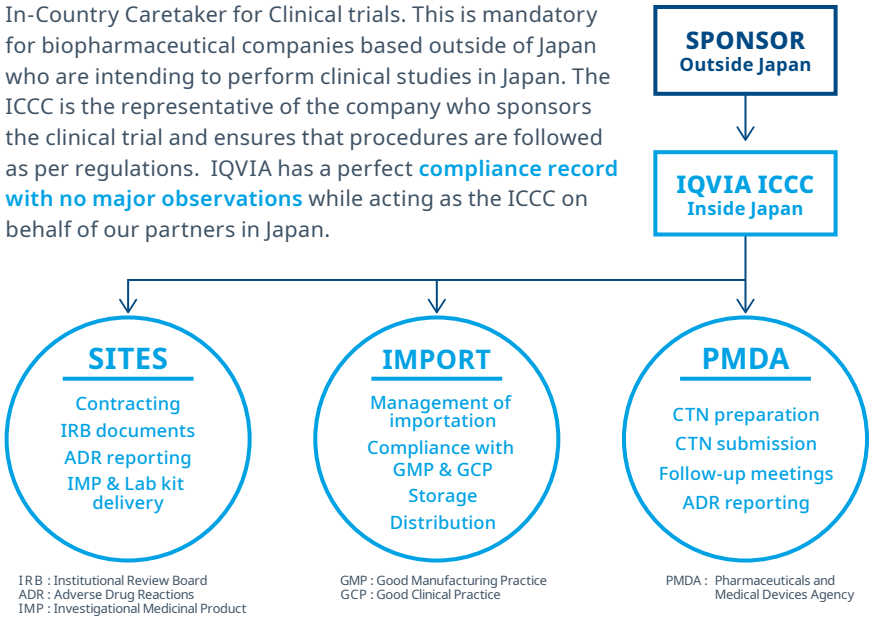
IQVIA's **Core Data coverage** supports identifying **limited patient populations** for any CAGT products targeting rare diseases.

Global footprint provides sponsors the option to **include Japan** as part of a **Multi-Regional Development Plan for CAGT** investigational products.

IQVIA's end-to-end support for CAGT products in Japan

CONSULTING	CLINICAL		NDA (New Drug Application)	APPROVAL	POST- MARKETING
GCTP (Good Gene, Cellular, and Tissue-based Products Manufacturing Practic	Regulatory	Data management	CTD (Common Technical Document)		Post-Marketing Surveillance
Gap analysis	Monitoring	Biostatistics			
Development strategy	Investigational product management	Audit	Post-Marketing Surveillance		Medical Science Liaison
Regulatory strategy	Medical monitoring	Medical writing			
Pre-IND (Pre- Investigation New Drug	Safety	ICCC (In-Country Clinical Caretaker	Conformity audit		Sales (MR)

ICCC:
In-Country Caretaker for Clinical trials. This is mandatory for biopharmaceutical companies based outside of Japan who are intending to perform clinical studies in Japan. The ICCC is the representative of the company who sponsors the clinical trial and ensures that procedures are followed as per regulations. IQVIA has a perfect **compliance record with no major observations** while acting as the ICCC on behalf of our partners in Japan.



IRB : Institutional Review Board
ADR : Adverse Drug Reactions
IMP : Investigational Medicinal Product

GMP : Good Manufacturing Practice
GCP : Good Clinical Practice

PMDA : Pharmaceuticals and Medical Devices Agency

IQVIA Clinical Development Office for Regenerative Medicine located in the Kobe Biomedical Innovation Cluster (KBIC).
Provides fully integrated services based upon extensive experience of global clinical development and monitoring, ICCC, Regulatory consulting.

Collaborates with IQVIA's Global Center of Excellence of Cell and Gene Therapy located in California, US.

Provides the data, expertise, and services helping you transcend the challenges faced in the development and commercialization of cell and gene therapies.

CONTACT US

IQVIA Japan Group
Keikyu Dai-1 Bldg., 4-10-18 Takanawa, Minato-ku, Tokyo
E-mail : Japan@iqvia.com URL: www.iqvia.co.jp