

**REGULATORY REQUIREMENTS FOR PAEDIATRIC ONCOLOGY DRUGS AS PER
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ABSTRACT

This study examines the regulatory requirements for paediatric oncology drugs in Japan and India are compared in this abstract. It draws attention to the frameworks set up by the Ministry of Health, Labour and Welfare (MHLW) and India's Central Drugs Standard Control Organization (CDSCO). The paediatric population differs from adults in several ways, including physiological factors, pharmacokinetics, pharmacodynamics, and dosage administration. However, many medications prescribed for children are formulated for adults, necessitating dose adjustments throughout childhood. This underscores the need for paediatric drug formulations and regulations worldwide. This research delves into the intricate landscape of international paediatric drug regulations to elucidate key initiatives, challenges, and advancements shaping this field. Commencing with a historical backdrop, the review outlines the evolution of regulatory frameworks across major regions, including the Japan, Europe, and other international regulatory frameworks, while underscoring the imperative for the implementation of paediatric drug regulations in India. Regulations mandate age-appropriate dosing, rigorous clinical trials, and thorough labelling to prevent misuse. While both the nations strive to enhance outcomes for children with cancer, their approaches, particularly concerning regulatory frameworks, incentives for pharmaceutical innovation and mechanisms for ensuring patient access, exhibit significant differences.

KEYWORDS: Paediatric Oncology Drugs; Regulatory Requirements; Japan; India; MHLW; CDSCO.**INTRODUCTION**

Oncology is the medical specialty that deals with cancer, including its diagnosis, treatment, prevention, and research. cancers are the group of diseases characterized by uncontrolled and spread of abnormal cells. If the spread of cancer cells this stage is known as metastasis is not controlled, it can result in death. In India, paediatric drugs are developed based on clinical trials and protocols for a healthy adult human. There are no specific drug development regulations for paediatrics. Indian clinical practice relies heavily upon safety and efficacy data published in other developed countries, or inference from adult dosing. India is shining on so many fronts today. It has evolved into a giant economy and will be number three in a short time. India have made massive in- its

development towards caring for children with cancer, the state of paediatric oncology in the country today is far from satisfactory. In paediatric oncology, treatment involves various drugs based on the type of cancer. Chemotherapy is commonly used, with drugs like vincristine, cyclophosphamide, doxorubicin, cisplatin, methotrexate, etoposide, and cytarabine being frequently administered. These are used in cancers such as leukaemia, lymphoma, neuroblastoma, Wilms tumour, and osteosarcoma.^[1]

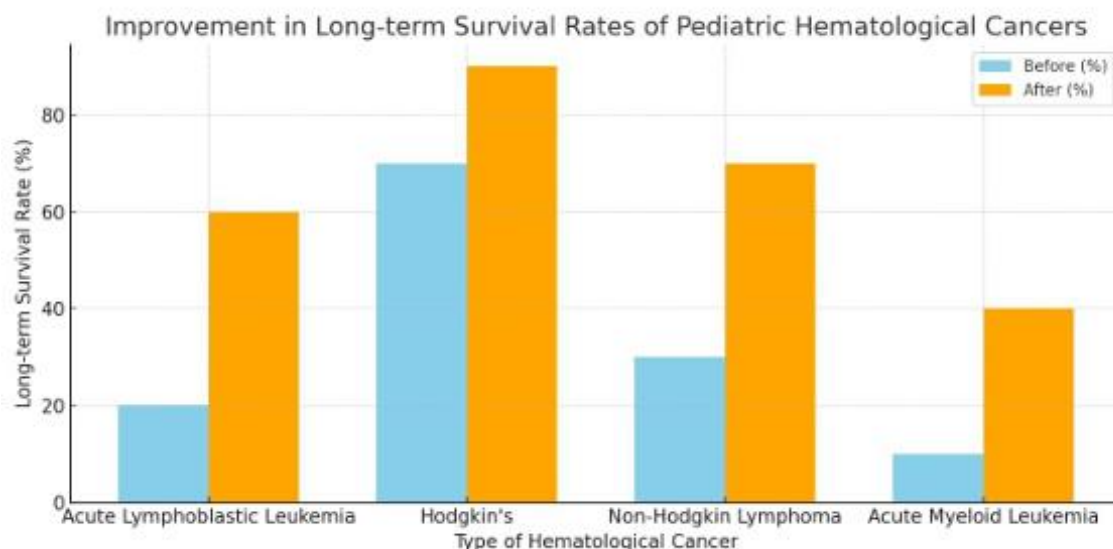


Fig no. 01: Improvement in longterm survival rates of paediatric haematological cancer.

OBJECTIVES

- Understand the regulatory framework governing paediatric oncology medications in India, specifically under the CDSCO (Central Drugs Standard Control Organization)
- Investigate the regulatory guidelines and procedures for paediatric oncology medications in the Japan, mainly under the MHLA (Ministry of Health, Labour and Welfare)
- Contrast significant regulatory elements between India and the Japan, encompassing drug approval pathways, clinical trial prerequisites, ethical considerations, and post-marketing surveillance.
- Pinpoint the challenges and deficiencies within the Indian regulatory system concerning the development and approval of paediatric oncology drugs.
- Examine recent initiatives, policies, or reforms in both nations aimed at enhancing the availability and safety of paediatric oncology medications.
- Evaluate the effects of regulatory disparities on drug accessibility, innovation, and clinical research in paediatric oncology.
- Propose potential strategies for harmonizing or enhancing the Indian regulatory framework based on international best practices.^[2]

CAUSES OF PAEDIATRIC ONCOLOGY

The childhood cancers are relatively rare compared to adult cancers, their causes are often more complex and less understood. Unlike adult cancers, which are frequently linked to lifestyle or environmental exposures, paediatric cancers are largely influenced by genetic and developmental factors.

1.3 TYPES OF PAEDIATRIC ONCOLOGY

- Leukaemia
- Wilms Tumour
- Germ Cell Tumours
- Lymphomas
- Retinoblastoma
- Hepatic Tumours.^[3]

1. Genetic Factors

Genetics plays a crucial role in the development of childhood cancers. Some children are born with mutations in specific genes that increase their risk of developing cancer.

2. Prenatal and Developmental Factors

Some cancers begin developing in utero (before birth) due to abnormalities in fetal cell growth or DNA replication. • Prenatal gene mutations have been identified in some children with leukaemia. • Rapid cell growth during embryonic and early childhood stages can sometimes lead to errors that result in cancer.

3. Environmental Factors

Though less prominent in children than adults, certain environmental exposures may contribute to paediatric cancers.

4. Infectious Agents

Some viruses may play a role in the development of specific paediatric cancers:

- **Epstein-Barr Virus (EBV):** Linked to certain types of lymphomas.
- **HIV:** Associated with an increased risk of Kaposi sarcoma and lymphomas.
- **Hepatitis B and C:** May contribute to liver cancers in children, though rare.

5. Secondary Cancers (Treatment-Related)

In a large percentage of paediatric cancer cases, the exact cause remains unidentified. Unlike adult cancers, lifestyle factors such as diet, smoking, and alcohol play little to no role in paediatric oncology.^[3]

- Brain Tumours
- Bone Cancers
- Neuroblastoma
- Rhabdomyosarcoma

CLASSIFICATION

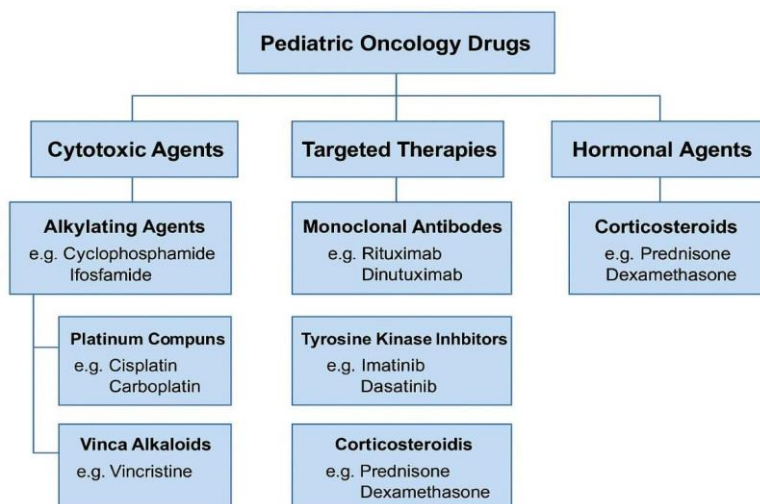


Fig. no. 02: Classification of Paediatric Oncology Drugs.

CENTRAL DRUG STANDARD CONTROL ORGANIZATION (CDSCO)



The Central Drugs Standard Control Organization (CDSCO) is India's national regulatory authority for drugs and medical devices. It operates under the Ministry of Health and Family Welfare government of India. CDSCO's primary responsibility is to ensure the safety, efficacy, and quality of drugs, medical devices, diagnostics available in the Indian market.

The Central Drugs Standard Control Organization (CDSCO) is the Central Drug Authority for discharging functions assigned to the Central Government under the Drugs and Cosmetics Act. CDSCO has six zonal offices, four sub-zonal offices, thirteen port offices and seven laboratories under its control.^[4]

ORGANIZATION OF CDSCO

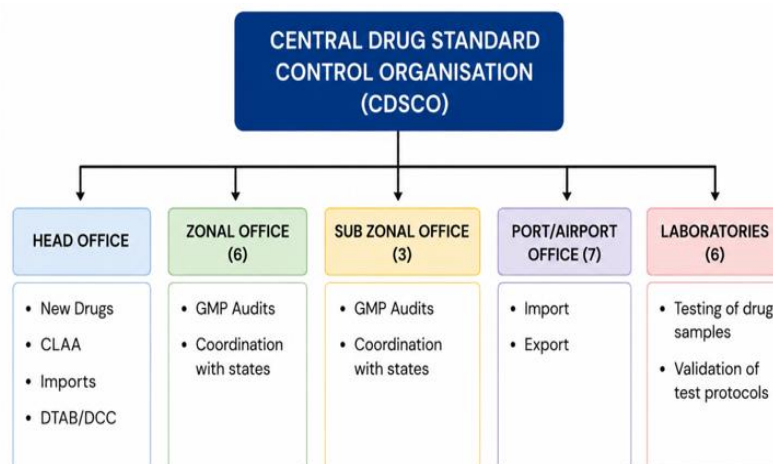


Fig. no. 03: Organization of CDSCO.

FUNCTIONS OF CDSCO

The Central Drugs Standard Control Organization (CDSCO) performs a wide range of functions to regulate drugs, medical devices, and cosmetics in India. These functions are designed to ensure the safety, efficacy, and quality of products available to the public. The key functions of CDSCO include:

1. Regulation of Drug Approvals

- **New Drug Approvals:** Evaluates and approves new drugs before they can be marketed in India, ensuring they meet safety and efficacy standards.
- **Clinical Trials:** Reviews and grants approval for conducting clinical trials, monitoring their progress, and ensuring compliance with ethical standards.

2. Regulation of Manufacturing, Import, and Export

- **Manufacturing Licenses:** Grants licenses to drug manufacturers, ensuring they comply with Good Manufacturing Practices (GMP).
- **Import and Export Control:** Regulates the import and export of drugs and medical devices, ensuring that only products meeting Indian standards are allowed.

3. Quality Control and Assurance

- **Inspection and Audits:** Conducts regular inspections and audits of manufacturing facilities, distribution networks, and retail outlets to ensure compliance with regulatory standards.
- **Surveillance and Monitoring:** Monitors the quality of drugs and medical devices in the market, conducting post-market surveillance to detect and address any safety or quality issues.

4. Pharmacovigilance

- **Adverse Drug Reaction Monitoring:** Establishes and monitors pharmacovigilance programs to collect, analyse, and respond to adverse drug reactions (ADRs), ensuring patient safety.
- **Risk Management:** Implements risk management strategies based on pharmacovigilance data to minimize risks associated with the use of drugs and medical devices.^[4]

REGULATORY AUTHORITIES OF JAPAN



The Ministry of Health, Labour and Welfare (MHLW) and the Pharmaceuticals and Medical Devices Agency (PMDA) are the principal regulatory authorities responsible for the regulation of pharmaceuticals, medical devices, and paediatric oncology drugs in Japan. These organizations ensure the safety, efficacy, and

quality of healthcare products available to the Japanese population.

The MHLW is a cabinet-level ministry of the Government of Japan responsible for formulating healthcare policies, granting final marketing approvals for drugs and medical devices, and overseeing public health programs. The PMDA functions as the scientific and technical arm of the MHLW and is responsible for product reviews, safety monitoring, and post-marketing surveillance. The Japanese regulatory system is recognized globally for its stringent standards and adherence to international guidelines established by the International Council for Harmonisation (ICH).

Parent Department: Government of Japan – Ministry of Health, Labour and Welfare (MHLW).

Supporting Regulatory Agency: Pharmaceuticals and Medical Devices Agency (PMDA).

Leadership: The MHLW is headed by the Minister of Health, Labour and Welfare, appointed by the Prime Minister of Japan. The PMDA is managed by a Chief Executive who oversees regulatory review and safety activities.^[6]

ORGANIZATION OF MHLW AND PMDA

The Japanese pharmaceutical regulatory system operates through close collaboration between the MHLW and PMDA. Together, these organizations ensure that pharmaceutical products meet the required standards of safety, efficacy, and quality before reaching patients.

• MHLW

- Policy formulation
- Final approval of drugs and medical devices
- Public health administration
- Regulatory oversight

• PMDA

- Scientific review of drug applications
- Clinical trial consultation
- Good Clinical Practice (GCP) inspections
- Pharmacovigilance activities
- Post-marketing safety monitoring.^[5,6]

REGULATORY FRAMEWORK IN INDIA FOR PAEDIATRIC ONCOLOGY DRUGS

In India, the regulation of paediatric oncology drugs falls under the influence of the Central Drugs Standard Control Organization (CDSCO), which operates under the Ministry of Health and Family Welfare. The primary legal foundation is the Drugs and Cosmetics Act, 1940, and its associated Rules, 1945, along with the more recent New Drugs and Clinical Trials (NDCT) Rules, 2019. While India does not yet have a separate regulatory framework exclusively for paediatric drugs, the NDCT Rules provide predominant guidelines that apply to all age groups, including children. For paediatric

oncology, special ethical and scientific considerations are required due to the vulnerability of the population and the complexity of cancer treatment. Clinical trials involving children must receive prior approval from the Subject Expert Committee (SEC) and Ethics Committees, with special attention to informed consent and safety monitoring. Additionally, sponsors must justify the inclusion of paediatric populations, provide age-appropriate formulations, and ensure that trial protocols minimize risk. India does not currently have a dedicated paediatric drug legislation comparable to mandatory paediatric regulatory frameworks in some other countries efforts are being made to align with global standards through ICH-GCP guidelines and WHO recommendations. However, challenges remain, including limited paediatric oncology research infrastructure, inadequate incentives for sponsors, and lack of mandatory paediatric study requirements.^[7]

2.4 REGULATORY FRAMEWORK IN JAPAN FOR PAEDIATRIC ONCOLOGY DRUGS

Japan's regulatory framework for pediatric oncology drugs operates primarily through the **Pharmaceuticals and Medical Devices Agency (PMDA)** and the

Ministry of Health, Labour and Nelfare (MHLW). Japan has traditionally relied on voluntary measures, industry incentives, international clinical trial alignment, and regulatory guidance for paediatric drug development.

Mandatory Best Efforts: Under the PMD Act, new drug applicants are legally require to put forth their best efforts to establish and submit pediatric development plans during initial drug evaluation.

ICH E11 Guidelines: Japan follows the tripartite harmonized ICH E11 guideline, which provides global standards for the clinical investigation of medicinal products in pediatric populations.

CCPODD: The Consultation Center for Pediatric and Orphan Drug Development (CCPODD) offers subsidized consultations and regulatory advice help small biotech and pharmaceutical companies format pediatric development data packages, navigate regulatory requirements, design appropriate clinical development strategies, and facilitate the efficient development and approval of pediatric and orphan drugs in Japan.^[8]

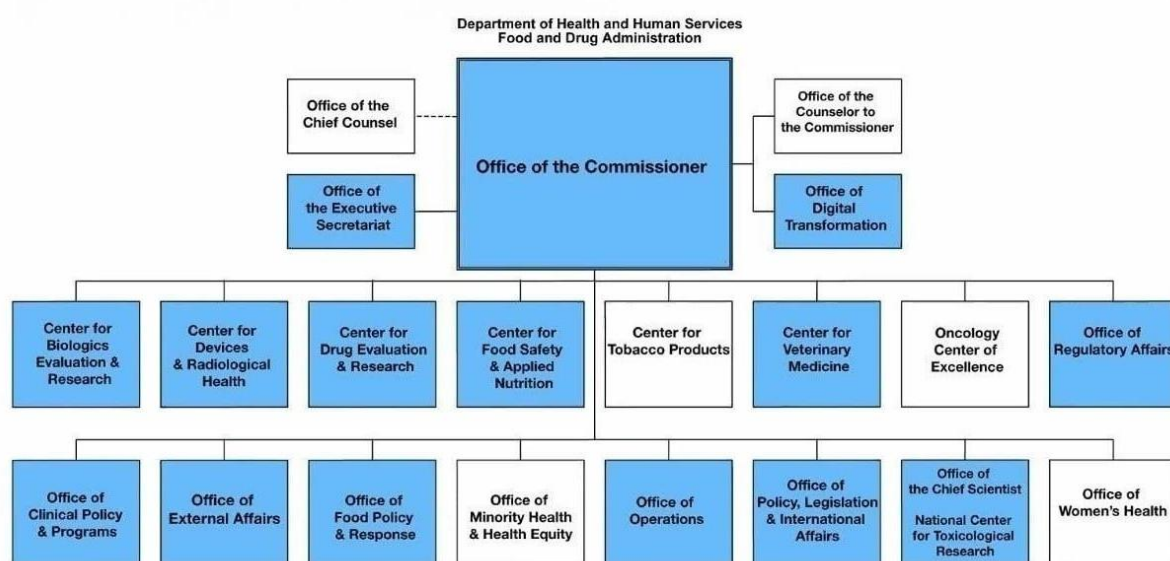


Fig. no. 04: Organization chart of MHLW and PMDA.

5 Comparison between India and Japan: Regulatory Requirements for Paediatric Oncology Drugs

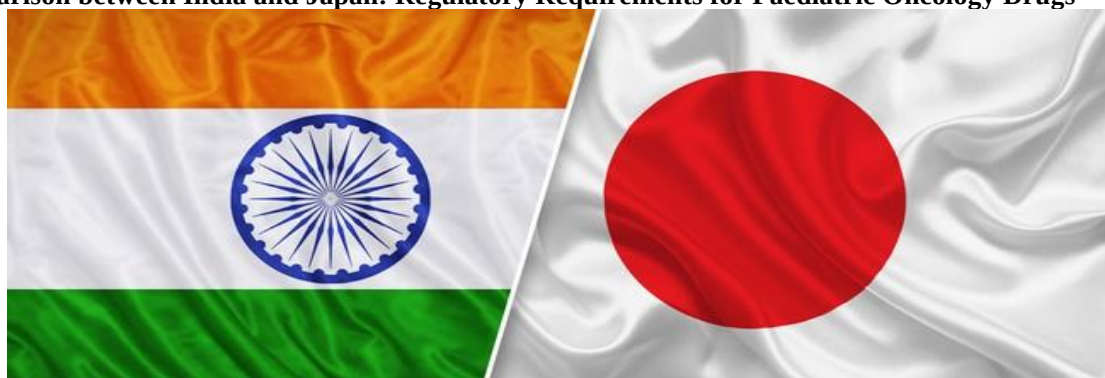


Table no 01: Regulatory Requirements for Paediatric Oncology Drugs as per CDSCO in India: Comparison with Japan.

Aspect	India	Japan
Regulatory Authority	Central Drugs Standard Control Organization (CDSCO)	Ministry of Health, Labour and Welfare (MHLW) and Pharmaceuticals and Medical Devices Agency (PMDA)
Key Laws/Guidelines	Drugs and Cosmetics Act, 1940; NDCT Rules, 2019	Pharmaceuticals and Medical Devices Act (PMD Act); MHLW and PMDA Guidelines
Paediatric-Specific Legislation	No specific law for paediatrics; general rules apply	No dedicated paediatric legislation, but specific guidance and support programs for paediatric drug development exist
Mandatory Paediatric Studies	Not mandatory	Generally not mandatory, but strongly encouraged when paediatric use is anticipated
Incentives for Sponsors	Limited or no specific incentives	Orphan drug designation, priority review, consultation support, and extended re-examination periods
Paediatric Formulations	Encouraged but not mandated	Encouraged and often requested when necessary for paediatric use
Ethical Review	Required via Institutional Ethics Committees	Required via Institutional Review Boards (IRBs) or Ethics Committees
Data Requirements	Based on adult data; paediatric extrapolation with justification allowed	Paediatric data, bridging studies, and extrapolation approaches may be accepted depending on clinical need
Clinical Trial Approval Time	Moderate to long (varies case-by-case)	Generally streamlined through PMDA consultations and review procedures
ization with Global Standards	Partially aligned with ICH-GCP; ongoing development	Strongly aligned with ICH guidelines and international regulatory standards

REGULATORY REQUIREMENTS FOR APPROVAL OF PAEDIATRIC ONCOLOGY DRUGS IN INDIA

The approval of paediatric oncology drugs in India is primarily regulated by the Central Drugs Standard Control Organization (CDSCO) under the Drugs and Cosmetics Act, 1940, and the New Drugs and Clinical Trials Rules, 2019 (NDCT Rules, 2019). Although India does not have a separate regulatory framework exclusively for paediatric oncology drugs, specific provisions and considerations apply to the development and approval of medicines for children.

Clinical trial requirements: Applications for permission to conduct clinical trials of new drugs or investigational new drugs are submitted to CDSCO in Form CT-04. Clinical development generally involves Phase I, II and III studies to establish pharmacokinetic, pharmacodynamic, safety and efficacy data. Clinical trials must receive approval from a registered Ethics Committee and be registered with the Clinical Trials Registry–India (CTRI). For serious or life-threatening conditions such as cancer, provisions for accelerated approval and expedited review may facilitate development of therapies addressing unmet medical needs.

Paediatric-specific considerations: Children constitute a vulnerable population and therefore require additional ethical safeguards. Informed consent from parents or legal guardians, along with age-appropriate assent where applicable, is required. Clinical trial protocols should

consider paediatric-specific requirements, including suitable age-appropriate formulations, dosing, pharmacokinetic (PK) and pharmacodynamic (PD) evaluation, and appropriate efficacy and safety endpoints. Where scientifically justified, extrapolation of efficacy data from adults to children may be considered, supported by appropriate paediatric PK/PD and safety data.

Safety and efficacy: Approval requires adequate evidence demonstrating the quality, safety and efficacy of the drug in the intended paediatric population. Due to physiological differences between children and adults, paediatric dosing and formulation should be appropriately evaluated rather than relying solely on adult data.

Post-marketing surveillance: Following approval, continued monitoring of safety is required through a pharmacovigilance system, including the submission of Periodic Safety Update Reports (PSURs) to CDSCO. Particular attention to adverse drug reactions in children is important because of their age-specific susceptibility and limited long-term safety data. Overall, the Indian regulatory framework aims to ensure that paediatric oncology drugs meet appropriate standards of quality, safety and efficacy, while incorporating ethical safeguards and age-specific scientific requirements for the protection of children.^[9]

REGULATORY REQUIREMENTS FOR APPROVAL OF PAEDIATRIC ONCOLOGY DRUGS IN JAPAN

Japan has a well-established regulatory framework for the development and approval of paediatric oncology drugs, primarily overseen by the Ministry of Health, Labour and Welfare (MHLW) and the Pharmaceuticals and Medical Devices Agency (PMDA). The framework focuses on ensuring the quality, safety and efficacy of medicines while addressing the specific needs of paediatric patients.

1. Regulatory Framework: The Pharmaceuticals and Medical Devices Act (PMD Act) provides the principal legal framework for pharmaceutical approval in Japan. Japan also follows relevant ICH guidelines, particularly ICH E11, for clinical investigation of medicinal products in paediatric populations. The Orphan Drug Designation System provides regulatory incentives and support for medicines intended for rare diseases, including rare paediatric cancers.

2. Paediatric Development: MHLW and PMDA provide guidance on paediatric clinical trial design, dose selection, pharmacokinetic (PK) and pharmacodynamic (PD) evaluation, safety assessment and age-appropriate formulations. Sponsors are encouraged to consider paediatric development early and consult PMDA regarding appropriate age groups, dosing strategies and clinical endpoints. Paediatric studies may be deferred, modified or waived when scientifically justified.

3. Clinical & Ethical Requirements: Paediatric oncology trials should consider physiological differences across age groups and generate appropriate safety, efficacy and PK/PD data. Age-appropriate formulations, including liquids, granules, powders and dispersible tablets, may be required. As children are a vulnerable population, parental/legal guardian consent and child assent where appropriate are required, with unnecessary risks and discomfort minimized.

4. PMDA Consultation: Early consultation with PMDA helps establish appropriate development strategies. Due to the rarity and heterogeneity of paediatric cancers, multicentre, international, adaptive, basket and umbrella trials may be used to improve recruitment and efficiently evaluate targeted therapies.

5. Approval & Post-Marketing: After adequate quality, non-clinical and clinical data are generated, a New Drug Application (NDA) is submitted. PMDA conducts the scientific review and provides its assessment to MHLW for the final regulatory decision. Following approval, post-marketing surveillance monitors safety and effectiveness, with additional studies conducted when necessary to evaluate paediatric dosing, long-term safety and clinical outcomes.^[10]

REGULATORY REQUIREMENTS FOR MANUFACTURE OF PAEDIATRIC ONCOLOGY DRUGS

INDIA (Central Drugs Standard Control Organization – CDSCO)

In India, the manufacture of paediatric oncology drugs is regulated by the Drugs and Cosmetics Act, 1940 and Rules, 1945, under CDSCO. Manufacturers must follow the Good Manufacturing Practices (GMP) specified in Schedule M, which ensure product quality, safety and consistency.

- **General GMP Compliance:** Paediatric oncology medicines must comply with Schedule M requirements, including quality control, validated manufacturing processes and appropriate facility controls.
- **Age-Appropriate Formulations:** The New Drugs and Clinical Trials Rules, 2019 (NDCT Rules) require consideration of suitable formulations for paediatric populations during drug development and approval. Formulations such as liquids, dispersible tablets and appropriate strengths should support accurate dosing, palatability and ease of administration.
- **Paediatric-Specific Manufacturing Mandates:** India does not have broad legislation specifically requiring paediatric investigations or formulations for every new oncology drug at the manufacturing stage. Requirements are mainly addressed through the drug-development and approval process.
- **Quality and Safety:** Due to the high potency and toxicity of oncology medicines, strict quality control, sterility requirements for injectables, and suitable containment measures are essential to prevent contamination and protect personnel.^[11]

JAPAN (Pharmaceuticals and Medical Devices Agency – PMDA)

In Japan, pharmaceutical manufacturing is regulated under the Pharmaceuticals and Medical Devices Act (PMD Act). GMP requirements are established by MHLW Ministerial Ordinance No. 179, while PMDA conducts regulatory reviews and GMP inspections and supports the Ministry of Health, Labour and Welfare (MHLW) in ensuring medicine quality, safety and efficacy.

- **GMP Compliance:** Manufacturers must comply with Japanese GMP requirements. PMDA conducts on-site and document-based GMP inspections, and compliance is an important requirement for marketing approval.
- **Paediatric Drug Development:** Japan does not have a direct equivalent to the US PREA/BPCA system. Instead, it promotes paediatric development through national guidance and internationally harmonized ICH guidelines, particularly ICH E11(R1) and ICH E11A.
- **Paediatric Oncology:** Development of paediatric cancer medicines considers age-specific clinical and scientific requirements, including scientifically

justified extrapolation. PMDA's Pediatric Drugs Working Group supports paediatric medicine development, including cancer medicines.

- **Formulation and Manufacturing Considerations:** Paediatric formulations should provide accurate dosing, acceptable taste, suitable dosage forms, adequate stability and safe, user-friendly container-closure systems. ICH quality principles, including ICH Q8 (Pharmaceutical Development), support a science- and risk-based approach.
- **Facility and Equipment:** Manufacturing facilities must prevent contamination and cross-contamination. For highly potent or cytotoxic oncology drugs, appropriate containment, environmental controls and specialized handling are particularly important.
- **Foreign Manufacturers:** Overseas manufacturing sites supplying medicines to Japan are also subject to Japanese GMP requirements and may be inspected by PMDA.^[12]

REGULATORY REQUIREMENTS FOR IMPORT AND SALE OF PAEDIATRIC ONCOLOGY DRUGS

The import and sale of paediatric oncology drugs in India are regulated to ensure their safety, efficacy, quality and proper distribution. The major requirements are governed by CDSCO under the Drugs and Cosmetics Act, 1940 and Rules, 1945, along with the New Drugs and Clinical Trials Rules, 2019.

INDIA (Central Drugs Standard Control Organization – CDSCO)

I. Import Requirements

- **Registration:** Foreign manufacturers and the specific drug product must be registered with CDSCO. Applications are generally submitted through an authorized Indian agent, with applicable fees.
- **New Drug Approval:** If the paediatric oncology drug is classified as a *New Drug*, approval under the NDCT Rules, 2019 is required before import. Relevant clinical and safety data must be submitted to CDSCO.
- **Import Licence:** After required registration and approval, an import licence in **Form 10** must be obtained as applicable.
- **Quality Control:** Imported drugs may be inspected and tested at ports to verify their identity, quality, specifications and compliance with the import licence.
- **Personal Importation:** In exceptional cases, lifesaving or unavailable medicines may be imported for personal use with appropriate CDSCO permission/NOC, prescription and medical justification. Such provisions do not permit commercial sale.
- **Shelf Life:** Imported medicines are generally expected to have adequate remaining shelf life, subject to applicable regulatory conditions.

II. Sale Requirements

- **Wholesale and Retail Licences:** Wholesalers and pharmacies must possess valid drug licences issued by the appropriate State Drug Control Authority.
- **Prescription Control:** Paediatric oncology medicines are generally prescription medicines and may fall under **Schedules H/H1**, depending on the drug.
- **Controlled Substances:** Drugs containing substances regulated under the **NDPS Act, 1985** require additional controls for storage, sale and dispensing.
- **Schedule H2 – Track and Trace:** Anti-cancer medicines covered under Schedule H2 require prescribed **barcode/QR-code labelling** to support authentication and traceability. Information may include the generic name, product identification, brand name, batch number, manufacturing/expiry dates and manufacturer details.
- **Good Distribution Practices (GDP):** Importers, distributors and sellers must maintain appropriate storage, handling and transportation conditions to preserve the quality and stability of oncology medicines.

Thus, India's regulatory framework controls paediatric oncology drugs throughout registration, importation, distribution and sale, with particular emphasis on product quality, prescription control, traceability and safe handling.^[13]

JAPAN (Ministry of Health, Labour and Welfare – MHLW / PMDA)

I. Import Requirements

- **Marketing Approval:** Commercial import and marketing of paediatric oncology drugs require appropriate approval under Japan's Pharmaceuticals and Medical Devices Act (PMD Act). Applications are reviewed by PMDA and approved by MHLW.
- **Foreign Manufacturer Accreditation:** Foreign manufacturers exporting medicines to Japan must obtain the required Foreign Manufacturer Accreditation under the PMD Act. PMDA may inspect the manufacturing facility.
- **Marketing Authorization Holder (MAH):** Foreign manufacturers generally operate through a Japanese MAH, which manages marketing authorization and regulatory responsibilities.
- **GMP Compliance:** Imported medicines must comply with Japanese GMP requirements. PMDA may conduct on-site or document-based GMP inspections of foreign manufacturing sites.
- **Quality and Documentation:** Imported products must meet Japanese requirements for quality, safety and efficacy, supported by appropriate manufacturing and quality-control documentation.
- **Labelling and Package Inserts:** Medicines must comply with Japanese labelling requirements and provide appropriate information on dosage, administration, precautions and paediatric use.

II. Sale Requirements

- **Marketing Approval:** Only medicines with the required PMD Act approval can be commercially marketed in Japan.
- **Prescription Control:** Most paediatric oncology medicines are prescription drugs and are supplied through medical institutions and pharmacies under healthcare-professional supervision.
- **MAH Responsibility:** The authorized MAH is responsible for maintaining regulatory compliance, product quality and post-marketing safety.
- **Distribution Control:** Medicines must be distributed through authorized pharmaceutical businesses under appropriate storage, handling and supply-chain conditions.
- **Labelling and Package Inserts:** Approved Japanese package inserts must include indications, dosage, administration, warnings, contraindications, precautions and adverse reactions.
- **Paediatric Information:** Where approved for children, product information should provide

relevant age/weight-based dosing, administration instructions and paediatric safety information.

- **Post-Marketing Safety:** The MAH must monitor product safety and take appropriate action when new safety information emerges. Risk Management Plans (RMPs) are used where applicable.
- **Serious Diseases:** Japan has mechanisms such as the Conditional Early Approval System for certain medicines addressing serious diseases with limited treatment options, with possible post-marketing requirements.

Overall, Japan's system emphasizes regulatory approval, foreign-manufacturer GMP compliance, authorized marketing and distribution, paediatric-specific product information, and continuous post-marketing safety monitoring.^[14]

CHALLENGES IN PAEDIATRIC ONCOLOGY DEVELOPMENT: INDIA vs JAPAN

Table no. 02: Challenges in Paediatric oncology Drug Development.

Challenge Area	India	Japan
Paediatric-Specific Legislation	No comprehensive paediatric drug law; studies are not routinely mandatory.	Paediatric studies are encouraged through guidelines and incentives; no PREA-equivalent law.
Infrastructure and Expertise	Limited specialized paediatric oncology centres and trained professionals, especially outside major cities.	Well-developed infrastructure and specialized centres; regional access may vary.
Ethical and Logistical Hurdles	Complex consent procedures, limited standardized protocols, and difficulty recruiting patients.	Strict ethical requirements and difficulty recruiting due to the small patient population.
Financial Incentives	Limited dedicated incentives for paediatric oncology trials.	Regulatory incentives and programmes support paediatric drug development.
Regulatory Clarity and Timelines	Regulatory pathways are improving, but timelines may vary and cause delays.	Established PMDA/MHLW framework with defined paediatric development procedures.
Public and Institutional Support	Limited awareness, advocacy and research funding for paediatric oncology.	Stronger institutional and research networks, but small patient populations remain a challenge.

CONCLUSION

Paediatric oncology drug development shows important differences between India and Japan. Japan has established a more structured regulatory framework through MHLW and PMDA, with increasing emphasis on early paediatric development planning, regulatory consultation, international clinical trials, and incentives for paediatric medicines. These measures have supported the development and availability of medicines for children with cancer and rare diseases.

In India, paediatric oncology drug development is still evolving. Although CDSCO has established a regulatory framework for new drugs and clinical trials, challenges such as limited paediatric-specific policies, inadequate local clinical trials, high treatment costs, and insufficient specialised infrastructure continue to affect access to advanced therapies. Institutions such as AIIMS and Tata Memorial Centre play an important role in paediatric cancer research, treatment, and clinical studies.

To strengthen paediatric oncology drug development in India, greater emphasis should be placed on paediatric-specific regulatory guidelines, multicentre clinical trials, improved pharmacovigilance, development of age-appropriate formulations, and stronger collaboration between regulatory authorities, researchers, hospitals, and pharmaceutical companies. Financial incentives and support for research could also encourage pharmaceutical companies to invest in paediatric oncology medicines.

Both India and Japan can benefit from greater international collaboration and harmonisation of regulatory standards. Sharing clinical data, research expertise, and regulatory experience may help reduce duplication in drug development and accelerate access to safe and effective treatments. Overall, strengthening regulatory systems, research infrastructure, affordability, and patient access will be essential for improving outcomes for children with cancer.^[15,16]

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