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Regulatory Requirements for Paediatric Oncology Drugs As Per CDSCO In India Comparison with United States

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ABSTRACT

Paediatric oncology requires regulatory approaches that balance timely access with child-specific safety. This abstract equates the laws and rules for medicines used to treat childhood cancers in the India and United states. It reviews the regulations created by the Food and Drug Administration (FDA) in the US and the Central Drugs Standard Control Organization (CDSCO) in India focusing on trial design, approval pathways, incentives, ethics, and post-marketing safety.[9] Paediatric oncology is a medical field that deals with diagnosing and treating cancers in children, from babies to teenagers. Children's medicine is different from adult medicine in various ways, such as how medicines are administrated, how metabolism takes place in children body and the way the body functions. [11] Many medicines given to children are made for adults, so the doses need to be changed to be safe for them. The USA demonstrates structured paediatric mandates (PREA, BPCA, RACE Act) and robust post-marketing mechanisms, while India operates through NDCTR 2019 and ethics-based oversight with fewer formal incentives.[9] Differences exist in trial networks, orphan incentives, and pharmacovigilance capacity. The article looks at the current state of laws for children's medicines worldwide to show important efforts, difficulties, and progress in this area. These regulations require that medicines are drugged properly for a child's age, tested well in clinical trials, and labelled clearly to avoid misuse.[11] Harmonization, strengthened paediatric trial infrastructure, and targeted incentives in India could accelerate paediatric oncology drug availability while maintaining safety. Cross-border collaboration is recommended.

Keywords; Regulatory requirements, Paediatric Oncology, CDSCO, FDA, Pharmacovigilance

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INTRODUCTION

Oncology is the medical specialized field that deals with cancer, including its diagnosis, treatment, prevention, and research. The uncontrolled growth and spread of abnormal cells are known as cancer. The production of medications for children is not covered by any standards. India is developing on so many fronts today. Although India has achieved great strides in the treatment of paediatric cancer, Annually, India diagnoses approximately 76,800 new paediatric cancer cases among individuals aged 0 to 19. A notable difference is that childhood cancers in India (0-19 years) constitute 4.6% of all cancer cases reported at hospitals, a proportion significantly higher than the 1-2%. [10] Paediatric oncology uses a variety of drugs, depending on the type of cancer. The development of drugs for children is not governed by any regulations. Adult dosage results, safety and efficacy data published in other developed countries serve as the main sources of information for clinical practice in India.

In early 1980s there was hardly any paediatric oncology can see in India. The majority of children received treatment from either adult oncologist in a few cancer centres or practically self-taught pediatricians in medical school. The subpar quality of paediatric cancer units (PCUs) and interdisciplinary or protocol-based care were also a problem. Only a handful of paediatric oncologists were present, as they were all typically foreign-trained.[12]

The first paediatric cancer service was established at Tata Memorial Hospital in 1985. Only 10% of cancer centres had trained paediatric oncologists, 50% of cancer centres had adult oncologists treating children, and less than 15% had paediatric oncology services, according to a 1988 nationwide survey. had dedicated beds for paediatric patients.[12] A more optimistic but still inadequate picture was obtained from a recent evaluation of over 275 medical schools and cancer institutions. More than half lacked the means and expertise to treat youngsters with cancer.

In spite of these problems, for the past forty years, the nation's paediatric cancer outcomes have steadily improved. The results for solid tumours have also improved. However, the outcome is still considerably poor compared to western figures, especially in tumours like retinoblastoma, leukaemia, CNS tumours and germ cell tumours. The conclusions reached in the west are significantly different from those in the few regions that do obtain results equivalent to those in the west. The conclusions reached in the west are significantly different from those in the few regions that do obtain results equivalent to those in the west Nowadays, 75 to 80 percent of children with cancer are expected to live for five years.[4] India stagnates behind the West in three areas of paediatric oncology service, research, and education. This comprehensive and methodical

advancement in each of these fields is what will raise paediatric oncology in India to international norms.

OVERVIEW OF CDSCO IN INDIA

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. The main duty of CDSCO is to guarantee the quality, safety, and effectiveness of medications, medical equipment, and diagnostics that are sold in India. It is overseen by the DCGI, is the primary regulator. NDCTR 2019 modernized clinical trial oversight and included paediatric provisions, but a standalone paediatric statute is absent. Ethics committees follow ICMR guidelines, emphasizing informed consent and assent. India often relies on global clinical data and bridging studies due to limited local paediatric trials.[13]

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. Thirteen port offices, seven laboratories, six zonal offices, and four sub-zonal offices are all under its purview.[13]

ORGANIZATION OF CDSCO

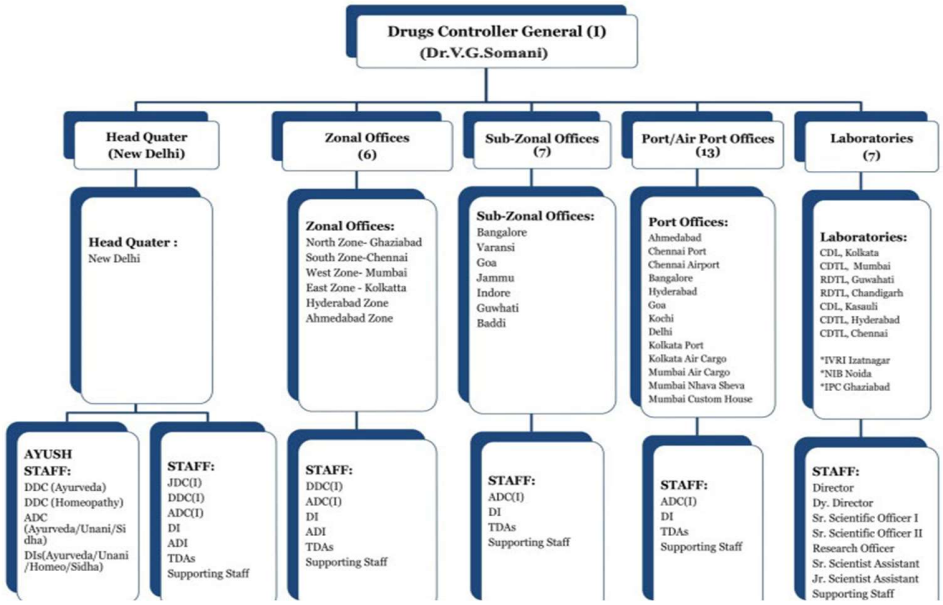

Figure 1: Organization of CDSCO

The Drugs Controller General of India (DCGI), a CDSCO officer, is the ultimate adjudicator for clinical trial approval in India. The Drug Consultative Committee (DCC) and the Drug Technical Advisory Board (DTAB) provide advice to the DCGI. Additionally, drugs like blood products and intravenous drugs must be approved by the DCGI. Under the Drug and Cosmetic Act, the regulation of manufacture, State authorities are primarily concerned with the sale and distribution

of drugs, conducting domestic clinical trials, setting drug standards, and ensuring quality control over imported products are under the purview in an effort to coordinate the actions of State Drug Control Organizations, provide knowledgeable guidance, and standardize the application of the Central Authorities' Drug and Cosmetic Act.

OVERVIEW OF FDA (USA)

Food and Drug Administration (FDA) is a regulatory agency of the United States federal government. It is part of the U.S. Department of Health and Human Services (HHS). The FDA is responsible for protecting public health by ensuring the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, the nation's food supply, cosmetics, and products that emit radiation. The FDA is led by the FDA Commissioner, who is appointed by the President of the United States and confirmed by the Senate. The Current FDA Commissioner (as of 2025): Martin A Makary (On March,2025).

The FDA governs paediatric drug development through PREA, BPCA, and the RACE for Children Act, which together mandate paediatric study plans, offer incentives, and expand the regulator's scope for oncology drugs with relevant targets. Cooperative groups like COG facilitate multi center paediatric trials.

ORGANIZATION OF FDA

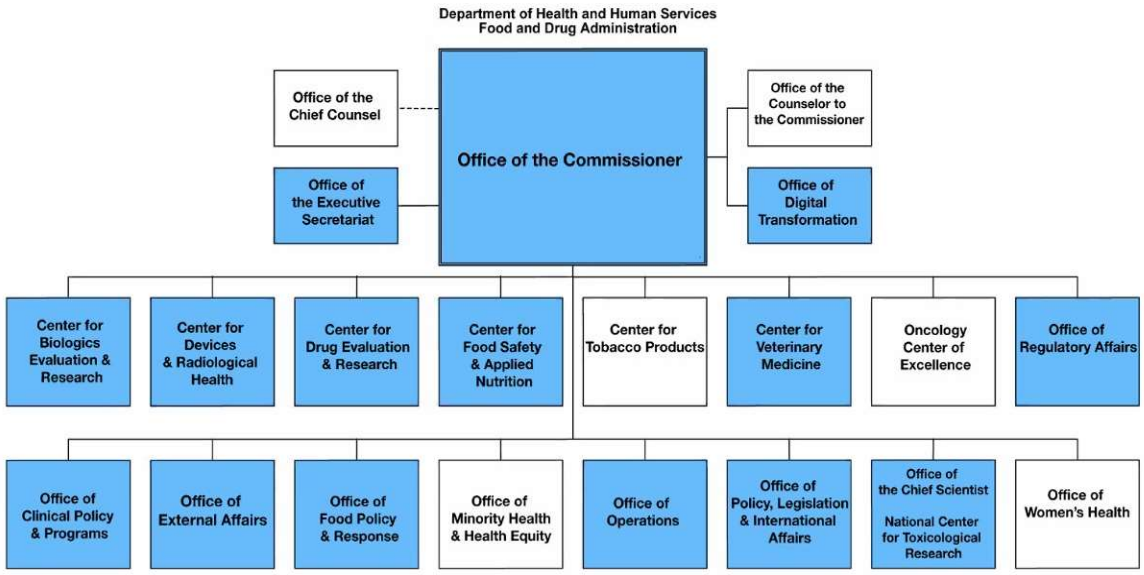

Figure 2: Organization of FDA

CURRENT SCENORIO INDIA

Over the past two decades, paediatric cancer in India has emerged as a growing public health issue. National Cancer Registry Programme (NCRP) data indicate that childhood cancers (ages 0–14) make up roughly 3–4% of all cancers.[1] Incidence has been rising: for example, one analysis found

that the age adjusted incidence rate (per million) in the boys climbed from ~157 in 2006 to ~235 in 2014.[2] NCRP reports that high rates in some urban areas – e.g. Delhi's rates reached ~203 per million in boys (2012–2016) and while overall rates remain lower than in high-income countries.[2] Recent ICMR projections predict roughly a 13% rise in all cancer cases by 2025, and global modelling suggests India's case load will exceed 1.5 million by mid-2020s.[1] Within this rising burden, leukaemia dominates: the NCRP estimates for 2022 show lymphoid leukaemia accounting for ~30% of childhood cancers, followed by central nervous system (CNS) tumours (~12–14%).[1] Paediatric cancer shows clearly that reported incidence is higher in the cities than villages, likely reflecting better diagnosis and reporting in urban registries. NCRP notes that rural areas appear to have lower childhood tumor incidence – largely attributed to under-ascertainment. For example, well-established metropolitan PBCRs (Bangalore, Chennai, Delhi, Mumbai) record rates near global averages, whereas rural registries (Ahmedabad district, Barshi) report far fewer cases.[4] Beyond urban/rural gaps, regional contrasts persist: northeastern India stands out with extraordinarily high cancer rates (Aizawl, Papum Pare and Mizoram among the top in NCRP data).[3] Alarmingly, northeast states also lack adequate paediatric oncology services – most children present with advanced, metastatic disease, and survival there is very poor. In contrast, better-facilated and equipped northern cities (Delhi NCR), western metros see more diagnosed cases and somewhat better outcomes, reflecting both referral bias and resource concentration.

Outcomes for India's common childhood cancers lag far behind high-income settings. Acute lymphoblastic leukaemia (ALL) is the single most frequent childhood malignancy. Published overview report Indian 5 year overall survival for ALL in the range about of 45–81% (often 50–60%), whereas high-income countries now achieved ~90%.[5]For example, multi center studies and single center series from the 2000s–2010s typically find 5-year event-free survival around 50–70% in Indian ALL cohorts.[5] Acute myeloid leukaemia (AML) fares worse: up to 50–80% of treated Indian children suffer relapse, refractory disease or treatment-related death, Risk-stratified national protocols (ICiCLe ALL) are now in use to improve these outcomes – historically, Indian centre's reports the <70% survival for ALL.[5]

Lymphomas and CNS tumours are also significant. Childhood Hodgkin lymphoma, if properly treated, can yield high cure rates, but Indian data are sparse. In general, limited-resource protocols (e.g. ABVD-based regimens) have produced 5-year survival often in the 70–90% range in tertiary centre's (some reports note ~90% for early-stage Hodgkin with modern therapy). Non-Hodgkin lymphomas (e.g. Burkitt, lymphoblastic) respond variably to chemotherapy; older series reported 3-year OS around 50–70%, with lower rates for Burkitt's lymphoma in undernourished patients.

Brain and other CNS tumours pose particular challenges: only a minority of Indian children with brain tumours survive long-term. A systematic review found extremely poor survival in low resource settings – e.g. 5-year OS for childhood astrocytoma was only ~39% in India (as of 1996 data).[8] More recent single institution reports from India still cite <30–40% 5-year survival for high-grade gliomas, medulloblastoma and other embryonal tumours, far below Western benchmarks. Retinoblastoma (eye tumours) is comparatively better – early diagnosis yields ~80% survival – but late-stage referrals remain common in rural areas.

Several systemic challenges underlie these outcomes. Treatment abandonment is a major cause of treatment failure. Studies over the 2000s–2010s have estimated that 10–63% of paediatrics cancer patients in India quit therapy prematurely. The true national rate is unknown, but hospital audits found ~20% drop-out by 2010, disproportionately affecting girls and rural patients.[6] These defaults are driven by poverty, travel distance, cultural beliefs (cancer as incurable) and the lack of support systems. Similarly, late diagnosis is rampant: most Indian children present at advanced stages. Lack of awareness in rural areas is key – parents and even local doctors may miss early cancer signs. Childhood cancers often have nonspecific symptoms, so initial work-up may treat fever/anaemia without detecting leukaemia, or misinterpret a brain tumour as infection. By the time families reach a specialist, disease is often disseminated, raising morbidity and reducing survival.

Access to care remains uneven. Paediatric oncology services are heavily urban-centric. A recent national survey found dedicated paediatrics oncology departments in only 41.6% of public-sector tertiary hospitals (and 48.6% of private tertiary centers). In practice, most rural hospitals have no paediatric oncologist; children must travel to cities for diagnosis and therapy. Even in cities, supportive resources (radiotherapy, paediatrics ICUs, blood products) are limited: <50% of public centers had full stocks of essential chemotherapy drugs or round-the-clock ICU support. Socioeconomic barriers compound these gaps. Treatment costs are catastrophic for most families, and government insurance schemes have only recently begun to help. Until 2018 most state schemes only covered specific cancers or adult oncology; the 2018 Ayushman Bharat (Pradhan Mantri Jan Arogya Yojana) was the first to broadly cover paediatrics cancer hospitalizations. NGOs and hospital social workers now play a key role in helping families navigate grants and crowdfunding.

Over the past decade, childhood cancer care in India has begun to change in visible ways. The Indian Council of Medical Research (ICMR), together with professional groups, has started building treatment protocols that work in local settings. One example is the ICiCLe-ALL trial,

launched in 2016, which brought together centers across the country to standardize therapy for acute lymphoblastic leukaemia (ALL).[6] Alongside this, ICMR has put out national guidelines for treating childhood cancers such as lymphomas and solid tumours. The Indian Paediatric Oncology Group (InPOG) now coordinates trials nationwide—something that was almost unthinkable a generation ago. These steps echo the "twinning" programs pioneered by hospitals like St. Jude's, showing how collaboration and standardization can improve survival.

Infrastructure has also grown, slowly but surely. Back in the 1980s, there were only a handful of paediatric oncology units. Today, about half of India's large hospitals—and many NGO-led centers—have dedicated childhood cancer teams. Major hubs in Delhi, Mumbai, Chennai, Kolkata, and Bengaluru are now joined by newer programs in Patna, Lucknow, and Guwahati. The training pipeline is also better than before. MD and DNB programs in paediatric oncology have expanded, and experts now suggest that India should aim for at least 50 fully trained specialists nationwide in the coming years.

International ties are playing a role too. India has joined the WHO's Global Initiative for Childhood Cancer (CureAll), which aims to raise survival to 60% worldwide by 2030. Partnerships with groups like ACT for Children have helped bring new drugs, diagnostic tools, and support to under-resourced hospitals. Pilot projects connected to CureAll stress the importance of "centers of excellence," telemedicine, and better data systems—all areas where India has started to make progress.

These efforts are not just on paper—they're showing results. At Tata Memorial Hospital, support from ImPaCCT Foundation helped cut treatment abandonment from 20% in 2010 to just 2% in 2022.[7] As a result, Tata's five-year survival rates got improved too, from roughly 41% in 2010 to almost 58% by 2018. Early report from the ICiCLe-ALL trial suggests the standardizing treatment has reduced deaths from toxicity. In some states, like Kerala and Punjab, survival rates for ALL and lymphomas are now approaching 75–80% in children who stay on protocol, though such numbers are still the exception rather than the rule.[7]

Of course, the challenges remain heavy. Childhood cancer cases are rising, and many children are still diagnosed late. Survival in India is far lower than in high-income countries, and families face barriers ranging from travel costs to social stigma. But the picture is changing. Awareness is stronger, more children are being diagnosed than before, and a new generation of doctors and researchers is shaping modern treatment in India. If current initiatives keep their momentum, the hope is that paediatric cancer will no longer be an "invisible" disease in India—but one where early diagnosis and effective treatment become the norm rather than the exception.

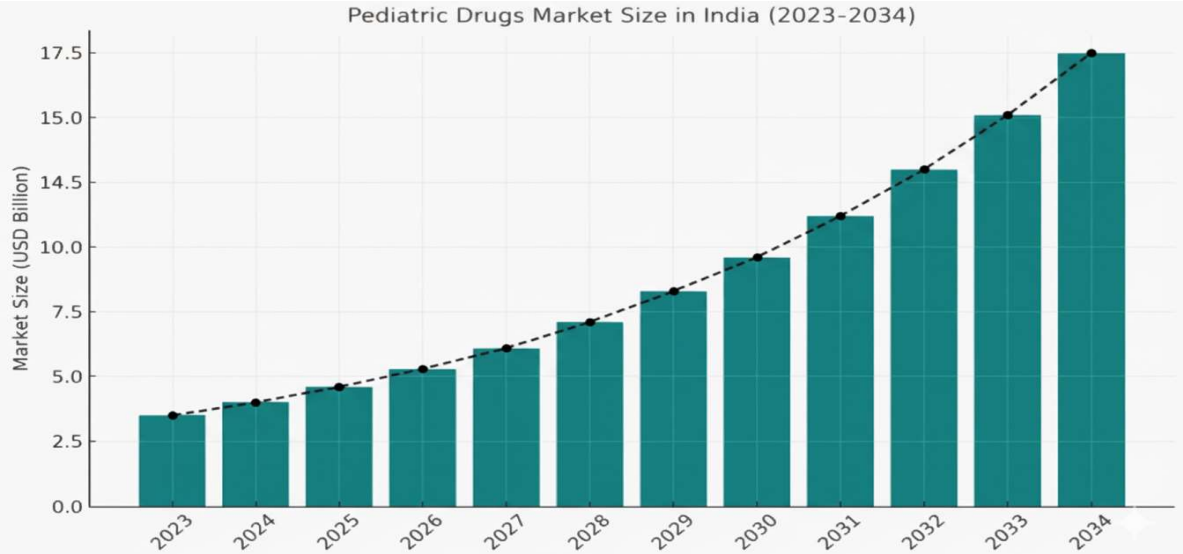

Figure 3. Paediatric drugs market size in India

The graph shows a clear and consistent upward trend, indicating that the market size is expected to grow steadily from 2023 to 2034. The data, measured in billion U.S. dollars ($ billion), shows a significant increase from an initial value of around $4 billion in 2023 to a projected value of over $15 billion by 2034. A dashed line connecting the top of each bar highlights this consistent growth trajectory.

USA

In the United States, paediatric cancer continues to be a significant public health focus, although outcomes have improved remarkably over the years. Between 2016 and 2018, the incidence rate of cancer among children and adolescents aged 0 to 19 years was 188.6 cases per million. For 2024, projections suggest that approximately 14,910 young individuals in this age group will be diagnosed with cancer, with an estimated 1,590 losing their lives to the disease. Incidence rates vary notably by age: the highest rates are observed in children under five and adolescents aged 15 to 19, with approximately 231 and 241 cases per million, respectively.

The survival outlook for paediatric cancer patients in the U.S. has improved dramatically over the past several decades. The overall 5-year survival rate now stands at 85%, a significant rise from around 58% in the mid-1970s. Certain cancers have seen particularly encouraging trends — for instance, the 5-year survival rate for leukaemia rose from 48.2% to 85.1% between 2010 and 2019. Similarly, for lymphomas, survival has increased from 72.9% to 94.2%. However, not all cancers have seen such progress. Survival rates for central nervous system (CNS) tumours, bone cancers, and sarcomas remain lower, averaging around 60%. Nonetheless, the overall success reflects the high standard of care in high-income countries, where survival rates generally exceed 80%.

In 2024, the pharmaceutical market in the United States is anticipated to generate US$636.90 billion in revenue. Oncology Drugs is expected to be the biggest market among them all, with a predicted market volume of US$114.[16] 60 billion in the same year. Forecasts indicate that the market will increase at a consistent annual rate of 5.96% between 2024 and 2028, reaching a market volume of US$802.80 billion by that year. [16] It is important to remember that, when looking at pharmaceutical markets globally, the United States is predicted to bring in the most money, with US$636.90 billion in 2024.[16] Personalized medicine and tailored medicines are seeing a boom in demand in the US pharmaceutical sector. Because of its high standard of research and development, the USA is the largest maker of pharmaceuticals. It holds a 42.6% global market share as 2022. America generates $171,300,816,183 worth of pharmaceuticals. Many of the top pharmaceutical companies in the world are based there.[16] It boasts the largest import and consumption pharmaceutical market. It is the world third-largest exporter of pharmaceutical. The most well-known pharmaceutical corporation in the United States is Eli Lilly and corporation, which markets the well-known drug Prozac. The US pharmaceutical industry was valued at USD 0.52 billion in 2023 and is projected to increase at a CAGR of 5.48% from 2024 to 2030.[16] Rising chronic illness prevalence, the growing senior population, increased healthcare spending by governments worldwide, and efforts to make medications more affordable and accessible are driving this trend. In May 2022, an article stated that U.S. policymakers are concentrating on prescription drug affordability due to unsustainable high prices, which can impact consumer purchases and raise health issues.

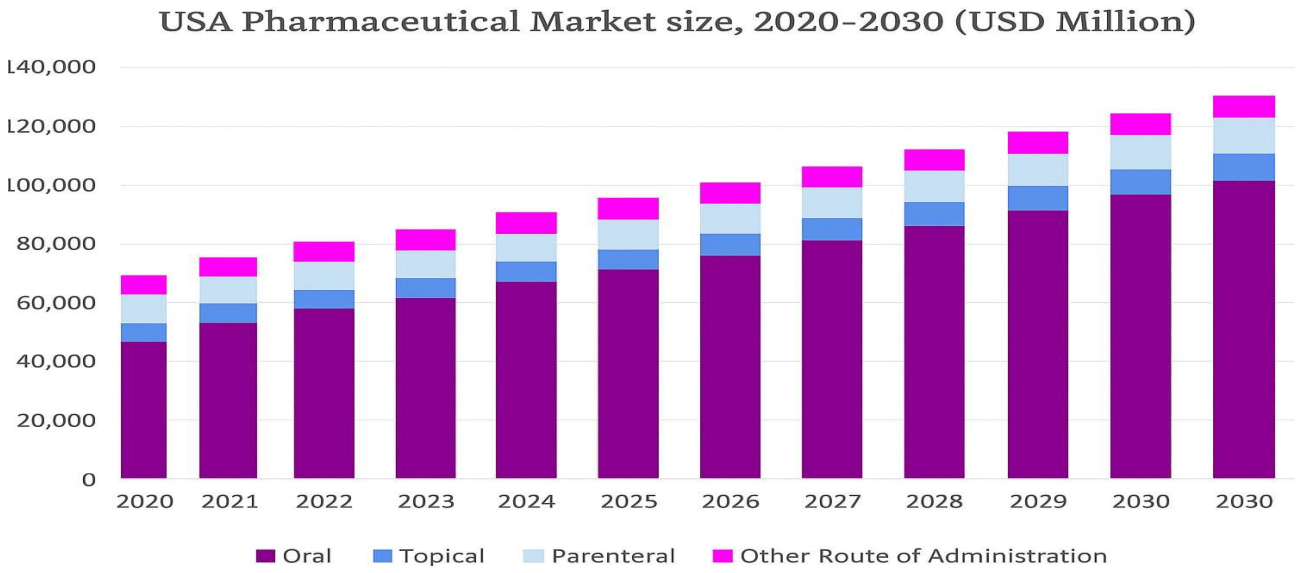

Figure 4. USA Pharmaceutical market size

APPROVAL OF PAEDIATRIC ONCOLOGY DRUGS

India

In India, paediatric oncology drug approval is carefully regulated to protect children while enabling access to potentially life-saving therapies. The process is guided by the Central Drugs Standard Control Organization (CDSCO) under the Drug Controller General of India (DCGI), with ethical oversight provided by Institutional Ethics Committees (IECs) according to NDCTR 2019 rules.

Preclinical Assessment

Before trials in children, drugs undergo preclinical evaluation to identify toxicity, safe dose ranges, and potential organ-specific effects in growing bodies. Juvenile animal studies may require for certain high-risk therapies.

Clinical Trial Approval

Sponsors submit clinical trial protocols to the DCGI and local IECs.

Trials are typically Phases I–III, with Phase I focused on safety, Phase II on early efficacy, and Phase III on confirming effectiveness compared to existing standards.

Given the rarity of paediatric cancers, multi-center and adaptive trial designs are increasingly used to gather meaningful data from small patient populations.

Bridging Studies

India often leverages foreign paediatric clinical data or adult studies with bridging trials to determine local relevance. This helps accelerate approvals but may result in limited country-specific paediatric data.

Ethical and Safety Considerations

Children are considered a vulnerable population. Parental consent is necessary, and child assent is obtained wherever needed. IECs carefully review study protocols to ensure that potential benefits outweigh risks. Continuous safety monitoring and adverse event reporting are mandatory.

USA

The United States has a structured regulatory system for paediatric oncology drugs that balances safety, efficacy, and early access. The U.S. Food and Drug Administration (FDA) oversees all stages of drug development and approval, guided by key legislations such as the Paediatric Research Equity Act (PREA, 2003), the Best Pharmaceuticals for Children Act (BPCA, 2002), and the RACE for Children Act (2017).

Preclinical Evaluation

Before paediatric trials, drugs undergo extensive laboratory and juvenile animal studies to determine safety, dosing, and potential long-term effects. Preclinical work ensures that initial paediatric exposure is as safe as possible.

Paediatric Study Planning (PSP)

Sponsors must submit a Paediatric Study Plan (PSP) early in development, outlining proposed paediatric studies, design, and timelines. PREA mandates paediatric assessments for most new drugs, unless waived or deferred, while BPCA offers incentives such as extended market exclusivity for voluntary paediatric studies. The RACE for Children Act ensures that targeted oncology drugs with mechanisms relevant to paediatric cancers undergo paediatric evaluation, even if originally developed for adults.

Clinical Trial Design

Paediatric oncology trials in the USA are often multi-center and adaptive, employing innovative designs such as basket or umbrella trials to maximize efficiency with small patient populations. Trials typically progress through Phase I (safety), Phase II (efficacy), and Phase III (confirmatory) stages. Modelling and simulation approaches are frequently used to optimize dose selection and extrapolate adult data when scientifically justified.

Ethical Oversight

Children are a vulnerable population, so trials require parental consent and child assent when appropriate. Institutional Review Boards (IRBs) evaluate the risk–benefit ratio, monitoring safety, stopping rules, and adherence to federal regulations (45 CFR 46 Subpart D).

Regulatory Review and Approval

The FDA reviews all trial data, including safety, efficacy, and pharmacokinetic information. Paediatric labelling is often updated promptly to reflect study outcomes. The US system encourages early interaction between sponsors and regulators, helping to streamline development while ensuring rigorous oversight.

Access and Incentives

While approval does not guarantee immediate access, financial incentives, grants, and exclusivity programs encourage sponsors to conduct paediatric studies. Networks such as the Children's Oncology Group (COG) support multi center trials, helping overcome recruitment challenges in rare paediatric cancers.

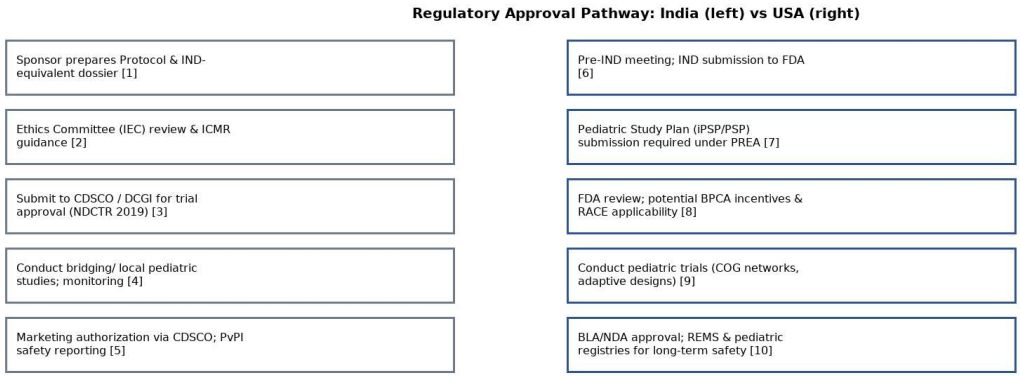

Figure 5: Regulatory approval pathway for paediatric oncology drugs in India vs. USA.

MANUFACTURE OF PAEDIATRIC ONCOLOGY DRUGS

The manufacturing of paediatric oncology drug like all pharmaceutical products is subject to stringent Good Manufacturing Practices (GMP) regulations in both India and the USA. However, there are shades and specific considerations, particularly in the USA. which are designed to encourage and ensure the availability of age-appropriate formulations for children with cancer. Here's a comparison of regulatory requirements for paediatric oncology drug *manufacture* in India and USA.

India (CDSCO) – Paediatric Oncology Drug Manufacturing

- **Governing Body**: CDSCO under Drugs & Cosmetics Act, 1940 and Rules, 1945.
- **GMP Compliance**: All drug manufacturers must follow Schedule M GMP guidelines—no separate GMP for paediatrics.
- **Paediatric Formulations**: Required only during approval (NDCT Rules, 2019). Must show suitability (e.g., liquid, dispersible, lower strength).
- **No Mandatory Paediatric Manufacturing**: Unlike the US, India does not mandate separate paediatric oncology development at manufacturing stage.
- **Quality Control Focus**: High emphasis on sterility, containment, and safety, especially for potent oncology drugs.

USA(FDA)

In the United States, the FDA regulates drug manufacturing under the Federal Food, Drug and Cosmetic Act (FD&C Act). While all pharmaceutical manufacturing must comply with Current Good Manufacturing Practices (cGMP) outlined in 21 CFR Parts 210 & 211, paediatric oncology drugs require additional considerations driven by child-focused regulatory initiatives.

- **PREA (Paediatric Research Equity Act):**
  Mandates paediatric studies for certain new drugs/biologics. The Initial Paediatric Study Plan (iPSP) may require development of age-appropriate formulations if the drug benefits or is likely to be used in children.
- **BPCA (Best Pharmaceuticals for Children Act):**
  Offers incentives for voluntary paediatric studies, encouraging development of paediatric-friendly dosage forms.
- **RACE for Children Act:**
  Requires paediatric studies for adult cancer drugs if their molecular target is relevant to paediatric cancers — even overriding orphan drug status.

Ethics, Consent and Assent

Both countries treat children as a vulnerable population requiring additional protection. India's ICMR ethical guidelines emphasize parental informed consent and child assent where necessary; IECs review benefit–risk and consent documentation. The USA follow federal protections (45 CFR 46 Subpart D) under IRB oversight and clearer guidance on the assent by age and maturity.

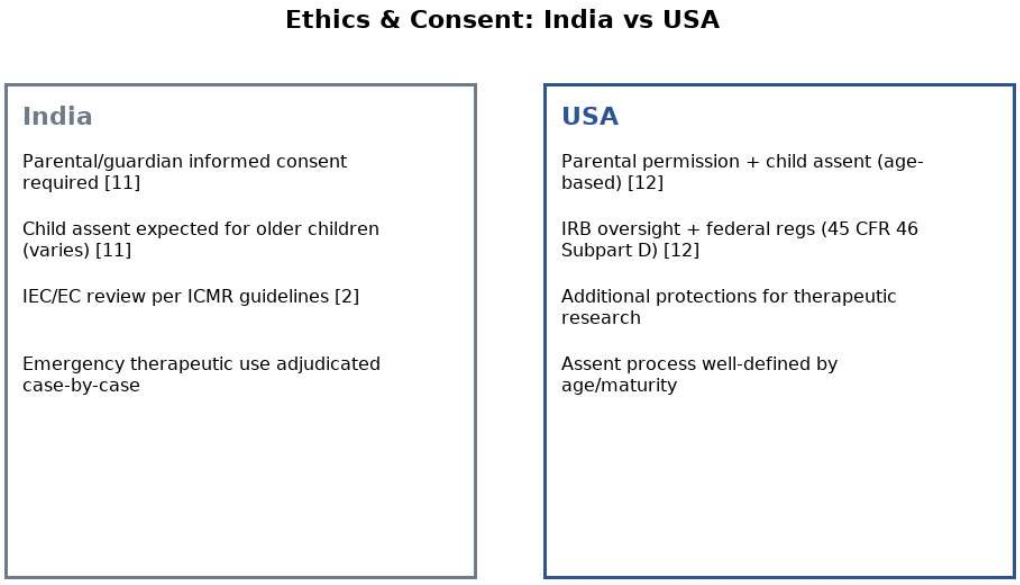

Figure 6: Ethics and consent—comparison between India and USA.

Practical differences include variability in assent age thresholds and differences in informed consent comprehension across socio-economic groups. India's IECs may vary in capacity and experience, affecting review timelines. For life-threatening conditions, therapeutic trials often permit higher acceptable risk thresholds when direct benefit is anticipated, but robust monitoring (DSMBs, stopping rules) is mandatory in both contexts.

Case Study 1: Blinatumomab (Blincyto) for Pediatric Acute Lymphoblastic Leukemia (ALL

Disease Overview: Condition: B-cell precursor Acute Lymphoblastic Leukemia

Age Group: Most common in children aged 2–5

Issue: High relapse rates despite chemotherapy.

Drug Profile:

Type: Bispecific T-cell engager (BiTE)

Mechanism: Links CD19+ B-cells to CD3+ T-cells, enabling immune cell destruction of leukemia cells

Development Path:

FDA designated as Breakthrough Therapy and Orphan Drug

Approved for pediatric use in 2016 based on data from Children's Oncology Group

Approved under Accelerated Approval Pathway in USA

Outcome: Improved survival in relapsed/refractory pediatric ALL

Reduced toxicities compared to standard chemotherapy

COMPARISON BETWEEN INDIA AND USA: REGULATORY REQUIREMENTS FOR PAEDIATRIC ONCOLOGY DRUGS

Aspect	India	USA
Regulatory Authority	Central Drugs Standard Control Organization (CDSCO)	U.S. Food and Drug Administration (FDA)
Key Laws/Guidelines	Drugs and Cosmetics Act, 1940; NDCT Rules, 2019	Paediatric Research Equity Act (PREA), Best Pharmaceuticals for Children Act

		(BPCA)
Paediatric-Specific Legislation	No specific law for paediatrics; general rules apply	Yes – PREA (mandatory paediatric studies) and BPCA (incentives for voluntary studies)
Mandatory Paediatric Studies	Not mandatory	Mandatory under PREA for certain drug applications
Incentives for Sponsors	Limited or no specific incentives	Yes – e.g., 6-month patent extension under BPCA
Paediatric Formulations	Encouraged but not mandated	Encouraged and often required when applicable
Ethical Review	Required via Institutional Ethics Committees	Required via Institutional Review Boards (IRBs)
Data Requirements	Based on adult data; paediatric extrapolation with justification allowed	Paediatric study plans required with detailed data
Clinical Trial Approval Time	Moderate to long (varies case-by-case)	Defined timelines with expedited pathways for paediatric oncology (e.g., priority review)
Harmonization with Global Standards	Partially aligned with ICH-GCP; ongoing development	Fully aligned with ICH guidelines and global practices
Infrastructure for Paediatric Trials	Limited paediatric oncology trial sites and expertise	Well-established paediatric clinical trial networks (e.g., COG, NCI-funded sites)

CONCLUSION

A comparative analysis of regulatory frameworks governing paediatric oncology drugs reveals a marked contrast between India and the USA. The United States has a well-developed and proactive regulatory system, with specific regulatory laws like the Paediatric Research Equity Act (PREA) and Best Pharmaceuticals for Children Act (BPCA) mandating and incentivizing paediatric studies. The FDA requires paediatric data submissions and provides pathways such as Orphan Drug Designation and Priority Review to accelerate approval for life-saving treatments in children.

In contrast, India's regulatory environment, governed primarily by the Central Drugs Standard Control Organization (CDSCO), basically lacks paediatric-specific mandates and incentives. While recent revisions to the New Drugs and Clinical Trials Rules (2019) had brought some progress, there is still no equivalent framework to encourage paediatric-specific research. Regulatory approval processes in India tend to be slower and less streamlined, particularly for innovative or targeted oncology therapies.

To equate this gap, India needs to concentrate on paediatric-specific regulatory guidelines, align more closely with international standards, and create incentives for pharmaceutical companies to invest in paediatric oncology drug development. Harmonization of practices and stronger

government support may improve the availability and with time approval of paediatric oncology drugs in India.

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