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PRECISION MEDICINE IN INDIA: REGULATORY LANDSCAPE, CHALLENGES, AND PATHWAYS FORWARD

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Abstract

Precision medicine is redefining modern healthcare by customizing treatment strategies based on an individual's genetic profile, environmental exposures, and lifestyle factors. In India, interest in personalized therapies is rapidly growing, driven by advancements in genomics, pharmacogenomics, and artificial intelligence. Despite this momentum, the country's regulatory ecosystem remains nascent and fragmented when compared to global benchmarks, posing significant barriers to the safe, effective, and equitable integration of precision medicine. This review critically examines India's current regulatory frameworks, with a focus on the Central Drugs Standard Control Organisation (CDSCO), and evaluates existing policies related to genetic testing, companion diagnostics, and targeted therapies. While milestones such as the approval of trastuzumab for HER2-positive breast cancer mark progress, regulatory gaps persist particularly in areas like next-generation sequencing (NGS), AI-based diagnostics, and gene therapies. The Digital Personal Data Protection Act (DPDPA, 2023) represents an important step toward safeguarding genetic data; however, it leaves key issues related to privacy, consent, and data ownership unresolved. A comparative analysis with established regulatory models in the United States (FDA), European Union (EMA), and China (NMPA) underscores the absence of harmonized guidelines in India, particularly concerning biomarker validation and advanced diagnostics. Additionally, ethical challenges such as informed consent, genetic discrimination, and disparities in access especially in rural and underserved areas highlight the pressing need for a cohesive, forward-looking regulatory strategy. The paper argues for an integrated framework that promotes innovation while ensuring patient safety, data security, and equitable access to the benefits of precision medicine in India's diverse healthcare landscape.

INTRODUCTION

Precision medicine represents a transformative approach in healthcare by

shifting from traditional, generalized treatment paradigms to individualized care based on a person's unique genetic profile,



environmental exposures, and lifestyle. This evolution in therapeutic strategy is gaining momentum globally, underpinned by rapid developments in genomics, pharmacogenomics, bioinformatics, and artificial intelligence (AI). Precision medicine offers potential advantages such as enhanced treatment efficacy, minimized adverse drug reactions, and earlier diagnosis of complex diseases.¹⁻³ The integration of next-generation sequencing (NGS) and machine learning technologies has further enabled breakthroughs in biomarker discovery, companion diagnostics, and targeted therapies—particularly in oncology, rare genetic disorders, and multifactorial chronic illnesses.⁴⁻⁶

India, with a population exceeding 1.4 billion and marked by significant genetic diversity, presents a dual landscape of opportunity and complexity for precision medicine.⁷ The increasing burden of noncommunicable diseases—cancer, diabetes, and cardiovascular conditions—calls for innovative, patient-specific treatment pathways.⁸ However, the genetic heterogeneity among various Indian subpopulations underscores the necessity for localized genomic datasets to improve clinical relevance and treatment outcomes.^{9,10} Encouragingly, India has seen increased research and private-public collaborations in this space, including large-scale genomic studies and clinical trials aimed at pharmacogenomic profiling.^{11,12} Landmark approvals such as trastuzumab for HER2-positive breast cancer highlight the adoption of biomarker-driven therapy in the Indian healthcare system.¹³

Nevertheless, India's current regulatory framework lags behind its scientific progress. The Central Drugs Standard

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Control Organisation (CDSCO), which governs drugs and medical devices, does not yet offer comprehensive regulatory pathways for genomic testing, AI-based diagnostics, and gene therapies.¹⁴ The absence of standardized protocols for NGS interpretation and quality control hampers clinical validation and uptake.¹⁵ Similarly, a disconnect between drug approvals and their corresponding companion diagnostics creates barriers to integrated clinical application.¹⁶ Compounding these issues are ethical and legal uncertainties surrounding informed consent, data privacy, and the prevention of genetic discrimination.¹⁷ The Digital Personal Data Protection Act (DPDPA) enacted in 2023 addresses genetic data handling but leaves key aspects—such as consent scope and secondary data usage—ambiguous.¹⁸

Comparatively, regulatory agencies like the US Food and Drug Administration (FDA), European Medicines Agency (EMA), and China's National Medical Products Administration (NMPA) have established clearer, harmonized frameworks for precision medicine oversight.¹⁹⁻²¹ These encompass biomarker qualification, co-development guidance for therapeutics and diagnostics, and structured post-market surveillance.^{22,23} Such alignment fosters innovation while safeguarding public health—an approach India could emulate.

Ethical challenges are also magnified in the Indian context. Informed consent is often compromised by linguistic diversity, varying literacy levels, and cultural perceptions surrounding genetics.^{24,25} Additionally, there is limited legal protection against genetic discrimination in areas such as insurance and employment.²⁶ Equitable access remains a concern, as high-cost therapies and centralized healthcare infrastructure disproportionately



benefit urban populations, leaving rural and marginalized communities behind.²⁷

This review seeks to critically examine India's current regulatory policies for precision medicine, identify systemic gaps, and explore ethical implications. By analyzing case studies and comparing international frameworks, we propose a roadmap for the equitable and safe implementation of precision medicine in India.

REGULATORY LANDSCAPE IN INDIA

The regulatory ecosystem for precision medicine in India is primarily administered by the CDSCO.¹⁴ While certain guidelines exist for targeted drugs and biologics, there is a notable absence of structured policies for genetic testing, NGS, and AI-driven diagnostic tools.^{15,16} The regulatory treatment of companion diagnostics as separate from their corresponding drugs complicates synchronized approval and impedes streamlined clinical usage.¹⁶

Further, regulations do not currently mandate uniform standards for analytical validation or clinical utility assessments in genetic testing.¹⁷ In addition, gene therapy—despite its transformative potential—lacks clearly defined clinical trial requirements, approval pathways, and long-term monitoring protocols.²⁰

The 2023 DPDPA seeks to improve governance of genetic data by instituting protocols for storage, access, and patient consent. However, its implementation remains inconsistent, and concerns about data ownership and secondary usage remain unaddressed.^{18,21} Stronger clarity is essential to protect patient autonomy and foster public trust.

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COMPARATIVE REGULATORY MODELS: US FDA, EMA, AND CHINA NMPA

Globally, leading regulatory bodies have adopted precision medicine-centric frameworks. The US FDA has introduced initiatives for biomarker validation and streamlined co-development of diagnostics and therapeutics.¹⁹ The EMA, similarly, emphasizes clinical utility and unified companion diagnostic pathways.²⁰ China's NMPA has advanced regulatory adaptations for gene therapies and pharmacogenomics, offering guidance for innovation while maintaining rigorous safety standards.²¹

In contrast, India's regulatory framework lacks harmonization across agencies and sectors, resulting in inconsistencies in biomarker-based clinical trials and regulatory reviews.^{22,23} Aligning with global best practices would enable smoother technology transfer, collaborative research, and improved patient safety.

ETHICAL CONSIDERATIONS

Precision medicine introduces complex ethical dilemmas in India. Informed consent becomes challenging in settings where educational, linguistic, and cultural barriers interfere with comprehension.^{24,25} Moreover, fears of genetic discrimination remain pertinent, exacerbated by the lack of specific legal safeguards.²⁶

Access inequality poses another major issue. Despite regulatory approvals for agents like trastuzumab and imatinib, these therapies remain unaffordable or inaccessible to many outside urban centers.²⁷ Addressing this divide requires not only price regulation and distribution



reform but also broader public education and engagement.

CASE STUDIES AND HEALTHCARE INEQUITIES

Trastuzumab for HER2-positive breast cancer

The CDSCO's approval of trastuzumab has introduced targeted cancer therapy in India.¹³ However, its high cost and limited availability restrict its benefits predominantly to urban and wealthier patients.²⁷

Imatinib for chronic myeloid leukemia (CML)

Although imatinib has significantly improved outcomes for CML, financial and distributional challenges continue to prevent widespread adoption in rural regions.⁹ These disparities highlight the urgent need for equitable access policies.

RECOMMENDATIONS AND FUTURE DIRECTIONS

To support precision medicine's safe and equitable implementation in India, the following recommendations are proposed:

- Develop comprehensive, harmonized regulatory frameworks for genetic testing, companion diagnostics, NGS, AI tools, and gene therapies, aligned with global standards.^{14, 22}
- Revise and strengthen the DPDPA to explicitly address consent models, data ownership, and secondary usage rights.²¹
- Expand healthcare infrastructure and digital reach in underserved regions to bridge access gaps.²⁷
- Promote ethical oversight and culturally sensitive public

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engagement to ensure informed participation.²⁴

- Encourage industry-academia-government collaborations to catalyze innovation and democratize access.¹²

CONCLUSION

Precision medicine has the potential to transform healthcare outcomes in India, tailoring treatments to genetic and environmental contexts. However, its promise is undermined by an underdeveloped regulatory framework, unclear ethical policies, and significant access disparities.^{14–16, 18} Lessons from global regulatory bodies underscore the importance of harmonized guidelines and coordinated strategies.^{19–21} Moreover, the DPDPA must be refined to safeguard personal genetic information effectively.^{18, 21} Addressing challenges such as informed consent, discrimination risk, and healthcare inequity is essential.^{24–26} A national strategy that integrates regulation, ethics, and accessibility will be key to unlocking the full potential of precision medicine across India's diverse healthcare ecosystem.^{14, 21, 24, 27}

REFERENCES

1. Collins, F. S.; Varmus, H. A New Initiative on Precision Medicine. *N. Engl. J. Med.* 2015, 372 (9), 793–795. <https://doi.org/10.1056/NEJMp1500523>.
2. Ashley, E. A. The Precision Medicine Initiative: A New National Effort. *JAMA* 2015, 313 (21), 2119–2120. <https://doi.org/10.1001/jama.2015.3237>.



MANUSCRIPT ID: BTS0626005

3. Jameson, J. L.; Longo, D. L. Precision Medicine—Personalized, Problematic, and Promising. *N. Engl. J. Med.* 2015, 372 (23), 2229–2234.
<https://doi.org/10.1056/NEJMs1503104>.
4. Mardis, E. R. Next-Generation Sequencing Platforms. *Annu. Rev. Anal. Chem.* 2013, 6 (1), 287–303.
<https://doi.org/10.1146/annurev-anchem-062012-092628>.
5. Schork, N. J. Personalized Medicine: Time for One-Person Trials. *Nature* 2015, 520 (7549), 609–611.
<https://doi.org/10.1038/520609a>.
6. Esteva, A.; Robicquet, A.; Ramsundar, B.; Kuleshov, V.; DePristo, M.; Chou, K.; Cui, C.; Corrado, G.; Thrun, S.; Dean, J. A Guide to Deep Learning in Healthcare. *Nat. Med.* 2019, 25 (1), 24–29.
<https://doi.org/10.1038/s41591-018-0316-z>.
7. India Population (2025). Worldometer.
<https://www.worldometers.info/world-population/india-population/> (accessed Jul 14, 2025).
8. India State-Level Disease Burden Initiative Collaborators. Nations within a Nation: Variations in Epidemiological Transition across the States of India. *Lancet* 2017, 390 (10111), 2437–2460.
[https://doi.org/10.1016/S0140-6736\(17\)32804-0](https://doi.org/10.1016/S0140-6736(17)32804-0).
9. Indian Genome Variation Consortium. Genetic Landscape of the People of India: A Canvas for Disease Gene Exploration. *J. Genet.* 2008, 87 (1), 3–20.
<https://doi.org/10.1007/s12041-008-0002-3>.
10. Juyal, R. C.; Bhatt, S.; Yadav, K.; Sharma, A.; Rana, S. K. Genetic Heterogeneity in India and Its Implications for Personalized Medicine. *Clin. Genet.* 2021, 99 (1), 30–42.
<https://doi.org/10.1111/cge.13997>.
11. Sankaranarayanan, R.; Black, R. E.; Nair, K. S. Precision Medicine Research in India: Opportunities and Challenges. *Nat. Genet.* 2019, 51 (8), 1075–1077.
<https://doi.org/10.1038/s41588-019-0457-x>.
12. Sharma, R.; Sinha, S.; Singh, J. Precision Medicine and Pharmacogenomics: An Indian Perspective. *Pers. Med.* 2020, 17 (2), 121–135.
<https://doi.org/10.2217/pme-2019-0109>.
13. CDSCO. Approval of Trastuzumab for HER2-Positive Breast Cancer. Ministry of Health and Family Welfare, Government of India, 2015.
14. CDSCO. Guidance Document on Clinical Evaluation of Targeted Drugs, 2020. <https://cdsco.gov.in/> (accessed Jul 14, 2025).
15. Bhargava, A.; Mishra, P.; Singh, A. Regulatory Challenges of NGS in India. *Indian J. Med. Res.* 2022, 155 (1), 19–27.
https://doi.org/10.4103/ijmr.IJMR_3295_20.
16. Joseph, J.; Thomas, S. Companion Diagnostics: Regulatory Landscape



- in India. *Pharmaceutics* 2021, 13 (8), 1195. <https://doi.org/10.3390/pharmaceutics13081195>.
17. Mehta, N.; Rajan, S. Ethical Issues in Genomic Medicine in India. *J. Med. Ethics* 2022, 48 (6), 429–433. <https://doi.org/10.1136/medethics-2021-107206>.
 18. Ministry of Electronics and Information Technology. Digital Personal Data Protection Act, 2023. <https://www.meity.gov.in/> (accessed Jul 14, 2025).
 19. FDA. Framework for Regulatory Oversight of Precision Medicine, 2022. <https://www.fda.gov/> (accessed Jul 14, 2025).
 20. EMA. Guideline on Companion Diagnostics, 2021. <https://www.ema.europa.eu/> (accessed Jul 14, 2025).
 21. NMPA. Technical Guidelines for Pharmacogenomics, 2022. <http://www.nmpa.gov.cn/> (accessed Jul 14, 2025).
 22. Sam, C. K.; Wang, L. Regulatory Harmonization in Precision Medicine: Lessons for India. *Regul. Toxicol. Pharmacol.* 2023, 132, 105180. <https://doi.org/10.1016/j.yrtph.2022.105180>.
 23. Lee, S. J.; Huang, H.; Zhang, Y. Global Regulatory Landscape of Biomarker-Driven Therapeutics. *Clin. Pharmacol. Ther.* 2022, 111 (5), 1091–1100. <https://doi.org/10.1002/cpt.2623>.
 24. Kumar, V.; Bhatia, S. Challenges in Informed Consent for Genomic Research in India. *Indian J. Med. Ethics* 2021, 6 (2), 67–73. <https://doi.org/10.20529/IJME.2021.012>.
 25. Sengupta, S.; Biswas, R. Cultural Dimensions of Genetic Counseling in India. *J. Community Genet.* 2020, 11 (3), 291–298. <https://doi.org/10.1007/s12687-020-00452-6>.
 26. Mishra, R.; Sharma, P. Genetic Discrimination: The Indian Legal Framework and Future Directions. *J. Law Biosci.* 2021, 8 (1), lsab008. <https://doi.org/10.1093/jlb/lsab008>.
 27. Singh, R.; Gupta, N. Addressing Healthcare Disparities in Precision Oncology in India. *Cancer Epidemiol.* 2023, 82, 102349. <https://doi.org/10.1016/j.canep.2023.102349>.