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Regulation of Human Cellular and Gene Therapeutic Products Incorporating Genome Editing Technologies in India

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ABSTRACT

New and advanced cellular and gene therapeutic (CGT) products using genome editing technologies have emerged in recent years as a curative treatment option for rare and genetic diseases. CGT products that edit the genome of human somatic cells are making it possible to treat the root cause of many rare and genetic diseases previously regarded as untreatable, and clinical trials of such products are under way worldwide. At present the only approved CGT product using genome editing is Casgevy, which is approved in the United States, the European Union and several other jurisdictions for the treatment of sickle cell disease and beta thalassaemia. In India a closely comparable therapy, BIRSA 101, has been developed indigenously for sickle cell disease and is expected to secure approval in the coming years. The Government of India has notified the New Drugs and Clinical Trials Rules, 2019 under the Drugs and Cosmetics Act, 1940 to accelerate the approval of CGT products. Under the 2019 Rules the Central Drugs Standard Control Organisation grants commercial approval of stem cell derived products and gene therapeutic products as new drugs. Alongside their curative potential, CGT products that edit the genome of human somatic cells carry additional risks, because they make permanent changes to the human genome and may produce off-target effects and other adverse events. The legal framework and the guidelines issued by the regulatory bodies must secure the safe and ethical application of these products, while timely approval is equally necessary if the unmet need for these lifesaving therapies is to be met. This article critically examines the legal framework and offers suggestions for addressing the challenges now facing India.

Keywords: *Cellular and gene therapeutic products, Human genome editing, Indian legal framework, New drug, Rare and genetic diseases*

I. INTRODUCTION

Genome editing is a transformative tool for making targeted additions, alterations or deletions to the genome or DNA of any organism.[1] Genome editing technologies such as CRISPR-Cas9

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[1] NAT'L ACADS. OF SCIS., ENG'G & MED., HUMAN GENOME EDITING: SCIENCE, ETHICS, AND GOVERNANCE 1 (2017).

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have become pivotal to the development of new and advanced cellular and gene therapeutic (CGT) products.[2] CRISPR-based therapies are beginning to offer curative options for rare and genetic diseases such as beta thalassaemia and sickle cell anaemia.[3] Alongside those therapeutic benefits, human genome editing remains the subject of serious debate, principally over germline editing, because an altered genome can be transmitted to future generations.[4] All applications of germline genome editing are at present prohibited in India on safety and ethical grounds. Genome editing also raises concerns about eugenics and enhancement, which are equally to be resisted.[5] India is now witnessing a marked increase in cell and gene therapy research, and a balanced legal framework is needed to secure the safe and ethical application of these advanced therapies.[6]

CRISPR-based advanced therapeutic products are rapidly transforming regenerative and personalised medicine. Their potential benefits are reasonably well understood, but the risks associated with gene editing are not yet fully understood. Those risks include off-target effects, tumorigenicity and long-term adverse events arising from errors in editing.[7]

Research and trials on CGT products must address off-target editing and any undesired consequence of on-target editing that is not known at the time the therapy is administered to the patient. Clinical trials must establish safety and efficacy, and trial participants must be monitored over the long term after administration.[8] The development of stable and safe gene-edited CGT products remains a key challenge, and safer delivery pathways will determine whether these therapies can be applied clinically to treat rare and genetic diseases.[9] CGT products developed by altering somatic cell genes are currently the subject of numerous clinical trials. Changes made to human somatic cells are not transmitted to the next generation and are confined to the treated individual.[10]

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[2] Hongyi Li et al., *Applications of Genome Editing Technology in the Targeted Therapy of Human Diseases: Mechanisms, Advances and Prospects*, 5 SIGNAL TRANSDUCTION & TARGETED THERAPY 1, 2 (2020).
[3] Qingyang Li et al., *CRISPR-Based Tools for Fighting Rare Diseases*, 12 LIFE 1, 7-13 (2022).
[4] WORLD HEALTH ORG., HUMAN GENOME EDITING: A FRAMEWORK FOR GOVERNANCE 22 (2021), https://iris.who.int/server/api/core/bitstreams/cdcfed77-66d5-46c3-9073-4018a3d2c48d/content.
[5] Fatma Betül Ayanoglu et al., *Bioethical Issues in Genome Editing by CRISPR-Cas9 Technology*, 44 TURK. J. BIOLOGY 110, 110-116 (2020).
[6] Kiruthika Sivagourounadin et al., *National Guidelines for Gene Therapy Product (2019): A Road-Map to Gene Therapy Products Development and Clinical Trials*, 12 PERSP. CLIN. RES. 118, 124 (2021).
[7] U.S. FOOD & DRUG ADMIN., HUMAN GENE THERAPY PRODUCTS INCORPORATING HUMAN GENOME EDITING: GUIDANCE FOR INDUSTRY 1-2 (2024), https://www.fda.gov/media/156894/download.
[8] *Id.* at 12-13.
[9] Tianxiang Li et al., *CRISPR/Cas9 Therapeutics: Progress and Prospects*, 8 SIGNAL TRANSDUCTION & TARGETED THERAPY 1, 17 (2023).
[10] NAT'L ACADS. OF SCIS., ENG'G & MED., *supra* note 1, at 63.

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The 2017 report on human genome editing recommends that somatic cell gene therapies be evaluated and reviewed under the existing national regulatory framework for CGT products, and that the authorities charged with oversight assess the safety and efficacy of gene-edited CGT products under clinical trial.[11] Germline editing, by contrast, is not to proceed to clinical trial, given the social and ethical concerns it raises, and much further research is required before any such trial could be authorised.[12] As a precautionary measure, all health research and clinical trials of CGT products must evaluate the risk of unintended germline modification.[13]

II. RESEARCH OBJECTIVES

The paper pursues four objectives.

First, to examine the ethical, legal and social concerns raised by CGT products incorporating genome editing technologies in the Indian context.

Second, to examine the legal framework and the guidelines governing CGT products that use genome editing technologies in India.

Third, to assess the readiness of that framework and those guidelines to meet the emerging challenges posed by such products.

Fourth, to suggest how a balanced legal framework might be built for the governance and oversight of CGT products incorporating genome editing technologies in India.

III. METHODOLOGY

The study integrates doctrinal and analytical methods to examine and evaluate the existing legal framework on CGT products using genome editing technologies. It adopts the Bluebook citation system for all primary and secondary sources. The work draws on primary and secondary material, including the Constitution, statutes, rules, guidelines, regulations, case law, policy documents, government reports, journal articles, books and internet resources concerning the regulation of cell and gene therapy using genome editing technologies in India. For the purpose of framing suggestions, documents published by foreign regulatory authorities have also been consulted.

[11] NAT'L ACADS. OF SCIS., ENG'G & MED., *supra* note 1, at 83.
[12] NAT'L ACADS. OF SCIS., ENG'G & MED., *supra* note 1, at 102.
[13] U.S. FOOD & DRUG ADMIN., *supra* note 7, at 10.

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IV. Ethical considerations in cell and gene therapy research using genome editing technologies

The four principles advanced in 1979 by Beauchamp and Childress, namely autonomy, non-maleficence, beneficence and justice, remain central to medical ethics and are the natural starting point for understanding the ethical dimensions of CGT products incorporating genome editing technologies. Read against genome editing, these principles help to identify the ethical imperatives that secure individual rights, dignity, voluntary consent, and the safety and well-being of patients.[14]

A. Principle of autonomy and informed consent

The principle of autonomy secures the right of human participants to decide freely whether to take part in a CGT trial or study. Trial subjects must be given all the information necessary to give a voluntary and informed consent. Respect for individual autonomy and for free and voluntary informed consent are the foremost ethical imperatives in clinical trials, because they ensure that participants understand the benefits and the risks before agreeing to the procedure. In *Jacob Puliyel v. Union of India*,[15] the Supreme Court held that every person has a right to bodily integrity protected by Article 21 of the Constitution, and that no person may be compelled to receive a vaccination without consent. Autonomy grants every person the freedom to decide how to live, including the freedom to refuse medical treatment. Where a CGT product incorporating genome editing is administered to a patient for therapeutic benefit and without malpractice, the enquiry therefore shifts to whether informed consent was freely obtained from the participant.[16]

In *Swasthya Adhikar Manch v. Union of India*,[17] a public interest litigation before the Supreme Court, it was alleged that mentally ill patients had been enrolled in clinical trials without their informed consent, and that trials had been conducted on persons with mental disability without regard to their particular needs. The petition followed reports of deaths at trial sites arising from unethical research conducted by sponsors who lacked the licences required from the Drugs Controller General of India. Following the Court's observations and directions, the Central Drugs Standard Control Organisation introduced more stringent ethical requirements for clinical trials.

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[14] Tom L. Beauchamp & James F. Childress, Principles of Biomedical Ethics 99 (6th ed. 2009).
[15] *Jacob Puliyel v. Union of India*, 2022 INSC 502, ¶ 49 (India).
[16] Naomi Cahn, *CRISPR Parents and Informed Consent*, 23 SMU Sci. & Tech. L. Rev. 3, 30 (2020).
[17] *Swasthya Adhikar Manch, Indore v. Union of India*, W.P. (C) No. 33 of 2012 (India).

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B. Principle of non-maleficence

The principle of non-maleficence in human genome editing gives priority to the duty to avoid harm, and so requires caution in genetic editing in order to protect the well-being of individuals and minimise risk. It sets a standard of conduct towards patients: at the least, do them no harm. Writing on treatment decisions, Beauchamp and Childress observe that the material question is whether a treatment is beneficial or burdensome, not how it is to be classified.[18] On that view, what distinguishes an optional from an obligatory treatment is the balance of benefits and burdens for the patient.

C. Principle of beneficence

The third principle of bioethics is beneficence. Medical professionals should strive to promote the good of their patients; that duty is, in substance, a duty of moral excellence, and doctors should do what they can to help those in their care.[19] Applied to human genome editing, beneficence emphasises the responsibility to advance the well-being of individuals by maximising positive outcomes while minimising risk, with the welfare of those undergoing genetic editing as the governing consideration.

D. Principle of justice

The fourth principle of bioethics is justice. The conception of justice used here follows Aristotle, who treated justice as a moral duty entailing fairness and consideration for all concerned. On that view, doctors, medical professionals and other staff should resist abuse and injustice and do what they can to change the status quo.

The principle of justice in human genome editing stresses the fair distribution of benefits and burdens, argues for equal access to genetic editing technologies, and warns against discrimination on grounds such as race, socio-economic status or geography. Studies in the United States, for example, indicate that black patients and women obtain less access to various forms of health care than white men. Racial and gender inequity in employment affects access to job-based health insurance, and the race and gender of doctors can in turn influence the patient and doctor interaction.[20]

The He Jiankui affair in China, in which a Chinese researcher carried out an unlawful germline genome editing experiment on human embryos, illustrates the stakes. Babies were born with edited genes, and the researcher was convicted and sentenced to three years' imprisonment and

[18] BEAUCHAMP & CHILDRESS, *supra* note 14, at 159.
[19] BEAUCHAMP & CHILDRESS, *supra* note 14, at 197.
[20] BEAUCHAMP & CHILDRESS, *supra* note 14, at 250.

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a fine of three million yuan, then roughly 430,000 United States dollars.[21] The episode drew worldwide criticism of the legal and ethical frameworks governing biotechnology in China, and the conviction raised serious questions about ethical standards, human rights and the legal treatment of germline genome editing. The expert advisory committee convened by the World Health Organization has acknowledged that research on gene editing will cross national borders and carry societal consequences, in somatic cell editing as much as in germline editing, although germline editing is the graver concern. Governance of gene editing technologies is therefore required at both the national and the international level.[22]

V. REGULATION OF CGT PRODUCTS AS NEW DRUGS UNDER THE STATUTE AND RULES

The regulation of CGT products incorporating genome editing technologies in India is spread across statutes, rules, regulations and guidelines. The use of genome editing to develop CGT products is governed by the framework already applicable to cell and stem cell derived products and to gene therapeutic products, and such products are treated as new drugs for the purposes of review and approval.

CGT products are regulated as drugs and evaluated under the Drugs and Cosmetics Act, 1940.[23] They fall within the term "drug" as "substances" under section 3(b)(i) of the Act. The Drugs Rules, 1945[24] contain settled provisions on the licence to manufacture for sale or distribution, and on the form of licences to manufacture drugs, in respect of recombinant DNA (r-DNA) derived drugs within the biologicals category. Until recently there was no corresponding provision for cell or stem cell derived products, gene therapeutic products or xenografts, and amendments were accordingly required to rules 75, 75A, 76 and 76A and to licensing forms 27D, 27DA, 28D and 28DA.

The proposal to revise forms 27D, 27DA, 28D and 28DA, and to bring cell or stem cell derived products and gene therapeutic products or xenografts within the Drugs Rules, 1945, was considered by the Drugs Consultative Committee at its sixty-third[25] and sixty-sixth[26] meetings.

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[21] He Jiankui was convicted of the illegal practice of medicine by the Nanshan District People's Court, Shenzhen, in December 2019, and sentenced to three years of imprisonment and a fine of three million yuan. On the governance response to the episode, see WORLD HEALTH ORG., *supra* note 4.

[22] WORLD HEALTH ORG., *supra* note 4, at 51.

[23] Drugs and Cosmetics Act, No. 23 of 1940, § 3(b)(i) (India).

[24] Drugs Rules, 1945 (India).

[25] Drugs Consultative Comm., CENT. DRUGS STANDARD CONTROL ORG., *Minutes of the 63rd Meeting* (Jan. 30, 2024), https://cdsco.gov.in/opencms/opencms/system/modules/CDSCO.WEB/elements/common_download.jsp?num_id_pk=MjIxMQ==.

[26] Drugs Consultative Comm., CENT. DRUGS STANDARD CONTROL ORG., *Minutes of the 66th Meeting* (Jun. 17, 2025), https://cdsco.gov.in/opencms/opencms/system/modules/CDSCO.WEB/elements/common_download.jsp?

The Drugs Technical Advisory Board considered the proposed amendment at its ninety-first meeting.[27]

The Ministry of Health and Family Welfare has since notified the Drugs (Eighth Amendment) Rules, 2026,[28] which amend rules 75, 75A, 76 and 76A and licensing forms 27D, 27DA, 28D and 28DA by inserting the words "Cell or Stem Cell derived products, Gene therapeutic products or Xenografts, etc." after "Recombinant DNA (r-DNA) derived drugs".

The effect of the amendment is to place cell and stem cell based products and gene therapeutic products on the same footing as recombinant DNA derived drugs for the purposes of manufacture, sale and distribution, licensed through the State Licensing Authority and the Central Licence Approving Authority.

The amendment streamlines the regulatory pathway for innovative biological drugs in regenerative and personalised medicine. It also removes the regulatory uncertainty and the technical difficulty that the State and central licensing authorities faced in evaluating CGT products and xenograft models in India.

The New Drugs and Clinical Trials Rules, 2019[29] were made under the Act of 1940 in order to align the regulatory treatment of CGT products in India with international practice. Rule 2(1)(w)(v) brings within the definition of a new drug "a vaccine, recombinant Deoxyribonucleic Acid (r-DNA) derived product, living modified organism, monoclonal anti-body, stem cell derived product, gene therapeutic product or xenografts, intended to be used as drug", and the Explanation to rule 2(1)(w) provides that products of this class shall always be deemed to be new drugs. The 2019 Rules aim to establish a clear and certain regulatory pathway for the approval of clinical trials and of biomedical and health research involving CGT products, after which a successful new drug may be granted approval for commercialisation.

Before a clinical trial of a new drug, or a bioavailability or bioequivalence study, may be conducted in India, the ethics committee of the participating institution must first be registered with the Central Licencing Authority.[30] Registration granted by that authority is valid for five years[31] and is thereafter subject to renewal. The functions of the ethics committee include the review of bioavailability and bioequivalence protocols and the safeguarding of the safety,

num_id_pk=MjYyNg==.

[27] Drugs Tech. Advisory Bd., CENT. DRUGS STANDARD CONTROL ORG., *Minutes of the 91st Meeting* (Aug. 14, 2024), https://cdsco.gov.in/opencms/opencms/system/modules/CDSCO.WEB/elements/common_download.jsp?num_id_pk=MjM3MQ==.

[28] Drugs (Eighth Amendment) Rules, 2026, Gazette of India, G.S.R. 530(E) (Jun. 29, 2026) (India).

[29] New Drugs and Clinical Trials Rules, 2019, Gazette of India, G.S.R. 227(E) (Mar. 19, 2019) (India).

[30] *Id.* r. 8.

[31] *Id.* r. 9.

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welfare and rights of human trial participants.[32] Biomedical and health research conducted in an institution must likewise be reviewed by an ethics committee in accordance with the National Ethical Guidelines 2017.[33]

Where an academic clinical trial is undertaken only to establish a new route of administration, a new indication, a new dosage form or a new dose of an already approved drug, permission from the Central Licencing Authority is not required. Such a trial is instead evaluated under the National Ethical Guidelines of 2017, once the ethics committee has given its permission.[34] Where a participant dies in the course of a clinical trial, the sponsor must pay compensation to the legal heirs of the deceased participant.[35]

Permission from the Central Licencing Authority is required to manufacture a new drug for the purpose of initiating a clinical trial.[36] A prior licence from that authority is likewise required to import a new drug for a clinical trial,[37] and permission is separately required where a new drug is imported for sale or distribution.[38]

The import of an unapproved or experimental new drug may be permitted by the Central Licencing Authority where a government hospital imports it for the treatment of a patient suffering from a life threatening disease, a disease causing serious permanent disability, or a disease requiring therapies for unmet medical needs.[39] Permission to manufacture an unapproved or experimental new drug is available only in the exceptional case where a government hospital seeks to treat patients suffering from a life threatening disease for which no proven therapy exists.[40]

A. Pharmacovigilance under the New Drugs and Clinical Trials Rules

i. Routine pharmacovigilance requirements

Pharmacovigilance is defined under the 2019 Rules as the science and activities relating to the detection, assessment, understanding and prevention of adverse effects or any other drug-related problem, which in this context includes complications arising from CGT products.[41] An adverse event is defined as any untoward medical occurrence during treatment with an investigational

[32] *Id.* r. 11.
[33] *Id.* r. 17.
[34] *Id.* r. 28.
[35] *Id.* r. 39.
[36] *Id.* r. 52.
[37] *Id.* r. 67.
[38] *Id.* r. 75.
[39] *Id.* r. 86.
[40] *Id.* r. 91.
[41] *Id.* r. 2(1)(z).

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drug or a pharmaceutical product in a patient or trial subject, which does not necessarily bear a relationship to the treatment given.[42] A serious adverse event is an untoward medical occurrence during a clinical trial that results in death, permanent disability, hospitalisation, persistent or significant disability or incapacity, congenital anomaly, birth defect or a life threatening event.[43] One important object of a clinical trial is precisely to study a product such as a CGT product in human participants in order to identify its adverse effects.[44]

Where a serious adverse event occurs to a participant in a clinical trial, or in a bioavailability or bioequivalence study, the ethics committee of the institution concerned must prepare and forward a report to the Central Licencing Authority.[45] The ethics committee must also maintain records of serious adverse events occurring during a clinical trial.[46] The Central Licencing Authority may issue a warning to an ethics committee where the conduct of a clinical trial adversely affects the rights of participants,[47] and it may warn, suspend or cancel the registration of an ethics committee for biomedical and health research where a deficiency or defect adversely affects the rights of study participants.[48]

ii. Enhanced pharmacovigilance requirements

A report of a serious adverse event occurring to a study subject during a clinical trial must be forwarded to the Central Licencing Authority within fourteen days of the event,[49] and the same period applies to a serious adverse event occurring during a bioavailability or bioequivalence study.[50] Injury, permanent disability or death is treated as related to the clinical trial or to the bioavailability or bioequivalence study where the event arises from a violation of the protocol, from negligence, or from scientific misconduct on the part of the sponsor or the investigator.[51] For the purpose of compensation, the investigator must report the serious adverse event to the Central Licencing Authority, and an investigator who fails to report within time must submit the reasons for the delay along with the report.[52] In the case of death, the ethics committee must forward the report of the serious adverse event, together with its opinion on the quantum of

[42] *Id.* r. 2(1)(d).
[43] *Id.* r. 2(1)(ff).
[44] *Id.* r. 2(1)(j).
[45] *Id.* r. 11.
[46] *Id.* r. 13(2)(xii).
[47] *Id.* r. 14.
[48] *Id.* r. 18(1)(i).
[49] *Id.* r. 25(x).
[50] *Id.* r. 35(ix).
[51] *Id.* r. 41(b).
[52] *Id.* r. 42(1).

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compensation payable by the sponsor under the formula in the Seventh Schedule, to the Central Licencing Authority within thirty days of receiving the report from the investigator.[53]

iii. Post-marketing surveillance through periodic safety update reports

Periodic safety update reports must be submitted to the Central Licencing Authority every six months for the first two years after approval, and annually for the two years following. The Central Licencing Authority may extend the total period of submission where it considers this necessary in the interest of public health. Cases involving serious unexpected adverse reactions must be reported to the licensing authority within fifteen days of the applicant first receiving the information. Where marketing of the product is delayed by the licensee after commercial approval has been obtained, the data are to be furnished on a deferred basis, beginning from the date on which the product is marketed.[54]

B. Clinical trials involving paediatric, geriatric, pregnant and lactating participants

For a clinical trial under rule 19, and for a bioavailability or bioequivalence study under rule 31, involving paediatric, geriatric, pregnant or lactating patients, the sponsor and the investigator must follow the general principles and practices prescribed in the First Schedule to the 2019 Rules. The principles for the protection of human participants set out in the Third Schedule, together with the Good Clinical Practices guidelines, must be observed in the conduct of the trial.

The Ministry of Health and Family Welfare has recently notified the New Drugs and Clinical Trials (Amendment) Rules, 2026,[55] in consultation with the Drugs Technical Advisory Board, amending the 2019 Rules. The most significant change is the introduction of a prior intimation pathway in relation to rule 52. Before the amendment, approval from the Central Licencing Authority was required for the manufacture of a new drug or an investigational new drug for non-clinical testing.[56] Under the amended rule, applications are to be submitted by way of prior intimation in online Form CT-10, and prior mandatory permission from the Central Licencing Authority is no longer required for low-risk new drugs.[57] High-risk categories, such as narcotic and psychotropic drugs and sex hormones, are excluded from that exemption. Under amended rules 53 and 60, the period for regulatory review of an application to manufacture a new drug or an investigational new drug has been shortened from ninety days to forty-five days.[58] The

[53] *Id.* r. 42(2).
[54] *Id.* Fifth Schedule.
[55] New Drugs and Clinical Trials (Amendment) Rules, 2026, Gazette of India, G.S.R. 46(E) (Jan. 20, 2026) (India).
[56] *Id.* r. 2.
[57] *Id.* r. 2.
[58] *Id.* r. 10.

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shortened review period is intended to accelerate authorisation and to speed the domestic development of CGT products. The amendment is a significant reform, and the combination of a risk-based regulatory strategy with shorter approval periods aims to improve regulatory efficiency while retaining a balanced oversight mechanism for new drugs, including CGT products that use gene editing technologies.

VI. Environmental and biosafety assessment of CGT products

The development of a CGT product is subject to biosafety evaluation under the Rules for the Manufacture, Use, Import, Export and Storage of Hazardous Micro-organisms, Genetically Engineered Organisms or Cells, 1989,[59] made under the Environment (Protection) Act, 1986.[60] The review and biosafety evaluation of CGT products using gene editing technologies is conducted under stringent contained laboratory conditions. At the development stage, regulatory supervision is exercised by the Review Committee on Genetic Manipulation[61] and by Institutional Biosafety Committees,[62] which are among the competent authorities constituted under the 1989 Rules.

The 1989 Rules define gene technology and genetic engineering in comprehensive terms, extending to the modification, deletion or removal of parts of heritable material. On those definitions, the newer human genome editing technologies used to develop CGT products also fall within the scope of the Rules.[63]

Following the recommendations of the Review Committee on Genetic Manipulation at its meeting held on 14 November 2017, the Department of Biotechnology released the Regulations and Guidelines for Recombinant DNA Research,[64] for the evaluation of recombinant DNA research through Institutional Biosafety Committees. Those regulations govern research on, and the large-scale handling of, genetically engineered organisms and their products, including CGT products that use gene editing technologies.[65]

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[59] Rules for the Manufacture, Use, Import, Export and Storage of Hazardous Micro-organisms, Genetically Engineered Organisms or Cells, 1989, Gazette of India, G.S.R. 1037(E) (Dec. 5, 1989) (India).
[60] Environment (Protection) Act, No. 29 of 1986 (India).
[61] Rules for the Manufacture, Use, Import, Export and Storage of Hazardous Micro-organisms, Genetically Engineered Organisms or Cells, 1989, *supra* note 59, r. 4 (constituting the competent authorities, including the Review Committee on Genetic Manipulation).
[62] *Id.* r. 4 (Institutional Biosafety Committees).
[63] Vibha Ahuja, *Regulation of Emerging Gene Technologies in India*, 12 BMC Procs. 1, 5-10 (2018).
[64] Dep't of Biotechnology, Ministry of Science & Technology, Regulations and Guidelines for Recombinant DNA Research and Biocontainment (2017) (India).
[65] *Id.* reg. 1.3.

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VII. UNAPPROVED PRACTICES AND ADVERTISEMENTS IN THE MEDIA

The Drugs and Magical Remedies (Objectionable Advertisements) Act, 1954[66] prohibits misleading advertisements relating to drugs, including cell and gene therapeutic products, and forbids advertisements that make false claims about the efficacy of a drug or therapy.[67] An unproven or experimental cell or gene therapy advertised as a standard treatment, so as to mislead patients or trial participants, contravenes the Act. The Act also provides penalties for contravention: on a first conviction, imprisonment for up to six months, or a fine, or both.[68] Despite these provisions, the Act has proved ineffective against unproven and experimental stem cell therapies advertised as standard treatment or cure, owing to gaps in the regulatory scheme and to weak enforcement.[69]

VIII. REGULATION OF CGT PRODUCTS UNDER THE NATIONAL GENE THERAPY PRODUCT GUIDELINES

The National Gene Therapy Product Guidelines 2019[70] were developed jointly by the Indian Council of Medical Research and the Department of Biotechnology against the background of the existing framework for gene therapeutic products and clinical trials in the country. The guidelines are designed to ensure that all clinical trials of gene therapy products are conducted in a safe, ethical and scientific manner. Under the guidelines, a gene therapy product is defined as any entity which includes a nucleic acid component being delivered by various means for therapeutic benefits. Detailed regulatory requirements are laid down for all such products intended for human application in India, together with review and oversight mechanisms and requirements for preclinical testing, clinical administration and production.

The guidelines set out the duties and responsibilities of ethics committees, institutions, trial sponsors and investigators, and prescribe training and infrastructure requirements directed at safety. They lay down procedures for the preclinical evaluation of gene therapy products and establish a Gene Therapy Advisory and Evaluation Committee for that purpose. They also govern the enrolment of trial participants, safety and risk assessment, and trial design. Trials require the approval of the Central Drugs Standard Control Organisation, the Review

[66] Drugs and Magical Remedies (Objectionable Advertisements) Act, No. 21 of 1954 (India).
[67] *Id.* § 4.
[68] *Id.* § 7.
[69] Vaishnav M., *The Indian Regulatory Framework and the Surge of Unproven Stem Cell Therapies: A Call for Diagnosis*, 12 J.L. & BIOSCIENCES 1, 21 (2025).
[70] INDIAN COUNCIL OF MEDICAL RESEARCH & DEP'T OF BIOTECHNOLOGY, MINISTRY OF SCIENCE & TECHNOLOGY, NATIONAL GUIDELINES FOR GENE THERAPY PRODUCT DEVELOPMENT AND CLINICAL TRIALS (2019) (India).

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Committee on Genetic Manipulation and the Gene Therapy Advisory and Evaluation Committee before they may be conducted. The guidelines apply uniformly to researchers, investigators, clinicians, ethics committees, institutions and others engaged in developing gene therapy products for human application in India. Under the guidelines, the development of gene therapy products using germline cells *in utero* is completely prohibited on ethical and societal grounds.[71]

Gene therapies developed by modifying somatic cells are, however, permitted, because gene edited or modified somatic cells do not pass to the next generation. Such therapies fall into two categories: *ex vivo*, where cells are modified outside the body, and *in vivo*, where cells are modified within it.[72]

Under the guidelines, edited stem cells, edited induced pluripotent stem cells and CAR-T cells fall within the scope of gene therapy products.[73] All *ex vivo* gene modified cells are treated as gene therapy products, and their incorporation or use requires strict oversight and approval.[74]

On risk evaluation, the guidelines proceed from the premise that every gene therapy product carries the possibility of side effects, which may be associated with genome editing and with off-target damage.[75] Sponsors and investigators must therefore follow the prescribed protocols for risk evaluation, and participants must be given information about the disease and must sign informed consent forms.[76]

A. Adverse event reporting and long-term follow-up

Every gene therapy product development programme must incorporate a comprehensive monitoring plan to protect human participants from any adverse event or serious adverse event arising from unintended off-target effects of gene editing.[77] The collection of data on off-target effects on genes other than the targeted genes is also required.[78]

The guidelines make it mandatory to retain patient records relating to clinical trials, and to the administration of gene therapy products, for fifteen years, and place the responsibility for maintaining those records on the institution at which the trial or administration is conducted. Participants in clinical trials are not liable to pay for any procedure related to the trial.[79] The

[71] *Id.* guideline 4.1.
[72] *Id.* guideline 4.2.
[73] *Id.* guideline 2.2.2.
[74] *Id.* guideline 4.2.2.
[75] *Id.* guideline 11.2.
[76] *Id.* guideline 11.2.3.
[77] *Id.* guideline 11.5.1.
[78] *Id.* guideline 11.5.1.6.
[79] *Id.* guideline 7.17.

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informed consent form prescribed by the guidelines requires voluntary consent to participation in the trial and to the storage and analysis of data for fifteen years.[80] Taken together, the guidelines of 2019 give a clear statement of the ethical and scientific requirements for gene therapy product trials in India, and serve as a guidance document for clinicians, sponsors, investigators, institutions and ethics committees on safety, efficacy and clinical standards.

IX. Regulation of cell and stem cell based products under the national stem cell research guidelines

The National Guidelines for Stem Cell Research[81] lay down the basic regulatory framework for the evaluation and review of stem cell research in the country. They require the registration of the institutional ethics committee, mandate the adoption of good manufacturing practice at registered and certified facilities, and require clinicians and physicians conducting trials and health research to be registered with the medical regulator.

The guidelines identify stem cell therapies generated by gene editing methods such as CRISPR-Cas9, human germline engineering and reproductive cloning as the most contested areas of current debate. They also record the risks and ethical concerns attending the use of human embryos to generate human embryonic stem cells, which may lead to the commodification of human life.[82]

On permissible areas of research, *in vitro* work carried out in the laboratory and outside the body is permitted using stem cells separated from human tissues, with the prior approval of the institutional ethics committee.[83] For study purposes it is permissible to generate new embryonic stem cell lines from spare human embryos, or new induced pluripotent stem cell lines from adult somatic cells or spermatogonial stem cells from adult tissues, again with prior authorisation from the committee concerned.[84] Voluntary and informed consent must be obtained from the donor before samples are collected for research,[85] and tissues collected from clinics and hospitals require clearance from the institutional ethics committee of the clinic or hospital concerned.[86]

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[80] National Guidelines for Gene Therapy Product Development and Clinical Trials, *supra* note 70, at 89.
[81] Indian Council of Medical Research & Dep't of Biotechnology, Ministry of Science & Technology, National Guidelines for Stem Cell Research (2017) (India).
[82] *Id.* guideline 1.
[83] *Id.* guideline 8.1.1.
[84] *Id.* guideline 8.1.2.
[85] *Id.* guideline 8.1.2.1.
[86] *Id.* guideline 8.1.3.

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Research falling within the restricted category requires additional and stricter supervision, because it involves contentious issues.[87] All research applying gene editing tools such as CRISPR-Cas9 to stem cells or to human embryos is confined to *in vitro* research.

Such research must be reviewed by the institutional ethics committee and the institutional biosafety committee, and final authorisation must then be obtained from the Review Committee on Genetic Manipulation before the research may begin.[88] Only spare embryos, gametes and germline stem cells may be used for laboratory research.[89] For *in vitro* studies, the source of the somatic stem cells and the minimum number of embryos, germline stem cells and gametes required must be stated clearly.[90] Embryos modified through gene editing may not be cultured for research purposes for more than fourteen days after fertilisation.[91]

The guidelines also prohibit several categories of stem cell research outright, on social and ethical grounds.

Research relating to human germline gene therapy, and all forms of reproductive cloning, are strictly prohibited.[92]

Human embryos may not be cultured *in vitro* for more than fourteen days after fertilisation.[93]

Trials relating to xenogeneic cells are prohibited.[94]

Clinical study or research on xenogeneic and human hybrids is prohibited.[95]

Developmental propagation using genome modified gametes, germline stem cells or human embryos is prohibited.[96]

Members of the institutional ethics committee are expected to keep themselves informed of developments in stem cell research,[97] and all research and trials involving embryonic stem cells, induced pluripotent stem cells and gene editing or alteration for the development of CGT products must be conducted with caution.[98]

[87] *Id.* guideline 8.2.
[88] *Id.* guideline 8.2.8.
[89] *Id.* guideline 8.2.8.1.
[90] *Id.* guideline 8.2.8.2.
[91] *Id.* guideline 8.2.8.3.
[92] *Id.* guideline 8.3.1.
[93] *Id.* guideline 8.3.2.
[94] *Id.* guideline 8.3.3.
[95] *Id.* guideline 8.3.4.
[96] *Id.* guideline 8.3.5.
[97] *Id.* guideline 9.
[98] *Id.* guideline 9.3.

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X. Ethical oversight under the national ethical guidelines

The National Ethical Guidelines 2017,[99] issued by the Indian Council of Medical Research, are binding on all those involved, including investigators, sponsors, ethics committees, institutions, medical professionals and researchers.

On clinical trials using stem cells, the guidelines classify stem cell research into three categories according to the degree of risk to participants: permissible, restricted and prohibited. Research on adult and cord blood cells is permissible; embryonic stem cell research falls within the restricted category; and reproductive cloning is prohibited. Research classified as permissible or restricted may be conducted with the approvals required under the National Guidelines for Stem Cell Research.[100]

A. Compliance with good laboratory, clinical and manufacturing practice

Apart from haemopoietic stem cell transplantation for haematological disorders, every other use of stem cells is categorised as research and must be conducted as a clinical trial with the approvals required from the ethics committee and the institutional committee for stem cell research.[101] The guidelines require every clinical trial to be carried out with clinical grade cells processed in accordance with good laboratory practices, good manufacturing practices and good clinical practices guidelines, in order to protect the safety of human participants.[102] All clinical trials require prior approval and must be registered with the Clinical Trials Registry of India, and the ethics committee must give final approval before a trial begins.[103]

B. Marketing authorisation for commercial use

i. Gene therapies to be treated as research

Under the National Ethical Guidelines of 2017, all gene therapies are treated as research, and all the protections available to human research participants must be in place.[104] Somatic cell gene therapy is permissible for preventing or treating a serious disease where it is the only therapeutic option, and must be restricted to the alleviation of life threatening or seriously disabling genetic disease in individual patients; it may not be used to change normal human traits.[105] Before a gene therapy trial may be initiated, approval must be obtained from the local

[99] Indian Council of Medical Research, National Ethical Guidelines for Biomedical and Health Research Involving Human Participants (2017) (India).
[100] *Id.* guideline 7.9.
[101] *Id.* guideline 7.9.1.
[102] *Id.* guideline 7.9.2.
[103] *Id.* guideline 7.9.4.
[104] *Id.* guideline 10.14.
[105] *Id.* guideline 10.14.1.

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ethics committee and from the Department of Biotechnology for the gene construct.[106] Where the trial concerns a product for commercial use or for marketing, approval must be obtained from the Central Drugs Standard Control Organisation.[107] Every gene therapy trial must provide for long-term surveillance.[108] Informed consent must be obtained, particularly in relation to uncertainty about outcomes.[109] Germline therapy is prohibited in the present state of knowledge,[110] and eugenic genetic engineering to change, select or alter genetic characteristics, and the creation of so-called designer babies, are likewise prohibited.[111]

ii. Therapeutic applications of CRISPR and their limitations

Technologies such as CRISPR have made it possible to read genetic code. The genetic information of each participant or patient is unique and identifies the individual, so digitised medical records must be kept confidential and individual privacy protected through confidentiality.[112]

Therapeutic applications of CRISPR are possible across a wide range of diseases through clinical trials, but CRISPR-based cell and gene therapies carry risks. Inaccurate editing may produce off-target effects, striking genes other than those selected. Germline gene editing raises the prospect of transmitting altered genetic material to later generations and of generating designer traits, and it is for that reason prohibited. A cautious approach is required before genome editing technology comes into wide use.[113]

Open discussion among stakeholders, public engagement and advocacy are also essential to building trust and informing decisions. Capacity building for researchers, policymakers and regulators is equally necessary, so that social and ethical questions can be addressed thoughtfully and systems put in place to secure the safety of therapeutic applications of CRISPR.

[106] *Id.* guideline 10.14.2.
[107] *Id.* guideline 10.14.3.
[108] *Id.* guideline 10.14.4.
[109] *Id.* guideline 10.14.5.
[110] *Id.* guideline 10.14.7.
[111] *Id.* guideline 10.14.8.
[112] *Id.* guideline 10.15.
[113] *Id.* guideline 10.15.3.

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XI. Regulation of medical professionals dealing with CGT products

The Indian Medical Council (Professional Conduct, Etiquette and Ethics) Regulations[114] treat the advertisement of experimental therapies as standard treatment or cure as professional misconduct. Under the regulations, clinical trials and health research involving human participants must be conducted in accordance with the guidelines of the Indian Council of Medical Research. Violation of the ethical norms in those guidelines constitutes professional misconduct, and consent taken for a trial of a drug or therapy otherwise than in accordance with the procedure in the guidelines is likewise treated as misconduct.[115]

XII. The biotechnology policy for economy, environment and employment

The development of cell and gene therapies using genome editing is still at an early stage in India, and the country is pursuing self-reliance in CRISPR and gene editing through a range of policy initiatives.[116] India has recently developed its first indigenous CRISPR-based gene therapy for sickle cell disease under a publicly funded research programme.[117] That therapy, BIRSA 101, is directed at sickle cell anaemia and forms part of the effort to eliminate the disease under the National Sickle Cell Anaemia Elimination Mission by 2047.

The BioE3 Policy on Biotechnology for Economy, Environment and Employment, approved by the Union Cabinet in August 2024, seeks to accelerate the development of advanced therapies and to promote innovation in the medical field, with the aim of transforming India into a world-class biomanufacturing hub.[118] Under the policy a number of research projects are funded to develop CGT products for rare and genetic diseases, and further initiatives are under way to develop genome editing based therapies for such conditions.

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[114] Medical Council of India, Indian Medical Council (Professional Conduct, Etiquette and Ethics) Regulations 2002 (India).

[115] *Id.* reg. 7.22.

[116] Rajya Sabha Unstarred Question No. 3169, *Self Reliance in CRISPR and Gene Editing* (answered Mar. 19, 2026) (India), https://sansad.in/getFile/annex/270/AU3169_UgWuJ0.pdf?source=pqars.

[117] Press Release, Ministry of Science & Technology, *Union Minister Dr. Jitendra Singh Launches India's First Indigenous "CRISPR" Based Gene Therapy for Sickle Cell Disease* (Nov. 19, 2025), https://www.pib.gov.in/PressReleasePage.aspx?PRID=2191740.

[118] Dep't of Biotechnology, Ministry of Science & Technology, BioE3 (Biotechnology for Economy, Environment and Employment) Policy 6-9 (2024) (India).

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XIII. The national policy for rare diseases and access to CGT products

The National Policy for Rare Diseases, 2021[119] was framed by the Ministry of Health and Family Welfare. Sixty-three diseases are at present included as rare diseases under the policy, and registered patients receive financial assistance of up to fifty lakh rupees on the recommendation of the technical committee constituted under it. More than a thousand patients have benefited. The policy recommends legislative measures to promote affordable treatment for patients suffering from rare diseases, and asks the Department for Promotion of Industry and Internal Trade to take steps to accelerate the development, approval and commercialisation of indigenous CGT products at affordable cost by public and private pharmaceutical manufacturers.[120]

XIV. Recent judicial developments in cell and gene therapy research

Since the writ petition in *Swasthya Adhikar Manch v. Union of India*,[121] the Supreme Court has kept the regulatory safeguards governing clinical trials in India under continuing scrutiny. In that matter the Court issued detailed directions requiring audio-video recording of informed consent and a standard compensation mechanism, in order to protect the safety and well-being of clinical trial participants. The judgment thus required the establishment of adequate clinical trial safety standards in India, and the Court's directions were subsequently reflected in the New Drugs and Clinical Trials Rules, 2019. Weak enforcement and monitoring have nonetheless allowed fresh instances of unethical practice to be reported in recent years.

The Supreme Court has recently sought the response of the central government to allegations of illegal clinical trials conducted at the Sheth Vadilal Sarabhai Hospital in Ahmedabad. Counsel appearing for Swasthya Adhikar Manch submitted that a number of clinical trials had been conducted without ethics committee approval, in violation of the 2019 Rules.[122]

Following extensive reporting in the media, the Drugs Controller General of India barred the hospital from conducting clinical trials of new drugs and vaccines. According to those reports,

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[119] Ministry of Health & Family Welfare, National Policy for Rare Diseases 26-28 (2021) (India).
[120] *Id.* at 27.
[121] *Swasthya Adhikar Manch*, *supra* note 17.
[122] *SC Seeks Centre Response on 'Illegal Clinical Trials' in Gujarat*, Times of India (May 1, 2025), https://timesofindia.indiatimes.com/india/sc-seeks-centre-response-on-illegal-clinical-trials-in-gujarat/articleshow/120777030.cms.

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an official investigation found that the hospital had conducted fifty-eight clinical trials between 2021 and 2025, many of them without the mandatory ethical review.[123]

For some years India has also seen a growth in the unlawful promotion by clinics of experimental stem cell therapy as a standard treatment for autism spectrum disorder, in violation of the applicable regulatory norms. The problem has been examined critically in a recent study.[124]

In *Yash Charitable Trust v. Union of India*,[125] the petitioners contended in a public interest litigation that stem cell therapies for autism spectrum disorder remain experimental, yet clinics and hospitals were advertising and selling them as standard treatment. The Court held that consent to undergo treatment is not valid where it rests on inadequate information, and concluded that administering an unproven or experimental stem cell therapy as a standard treatment for autism spectrum disorder does not meet the standard of medical practice expected of medical practitioners. In construing what amounts to inadequate information, the Court relied on *Samira Kohli v. Dr. Prabha Manchanda*,[126] where it had held that consent is valid only if the patient has the competence and the ability to give it voluntarily, and has been given adequate information about the treatment so that the purpose of the consent is understood.

In the same judgment the Court identified the weaknesses of the regulatory mechanism for stem cell research in India. It observed that the case revealed shortfalls and faultlines in that mechanism, that the legal framework governing stem cell research is fragmented and spread across legislations with little harmony between them, and that this fragmentation makes both compliance and enforcement an uphill task. The Court further observed that obscurity in the legal regime enables errant medical practitioners to exploit the vulnerability of patients.[127]

To address these concerns in a meaningful way, the Court suggested the creation of a dedicated authority with clear and well-defined powers of regulatory oversight for stem cell research, and, in order to resolve the regulatory vacuum, the reconstitution of the National Apex Committee for Stem Cell Research and Therapy.[128]

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[123] Farhat Nasim, *58 Unauthorised Clinical Trials Spark DCGI Ban on VS Hospital, Doctors, Pharma Giants Under Fire*, MEDICAL DIALOGUES (June 25, 2025), https://medicaldialogues.in/news/industry/pharma/58-unauthorised-clinical-trials-spark-dcgi-ban-on-vs-hospital-doctors-pharma-giants-under-fire-150599.
[124] Vaishnav, *supra* note 69, at 1.
[125] *Yash Charitable Trust v. Union of India*, 2026 INSC 96, W.P. (C) No. 369 of 2022 (India) (decided Jan. 30, 2026).
[126] *Samira Kohli v. Dr. Prabha Manchanda*, (2008) 2 SCC 1 (India).
[127] *Yash Charitable Trust*, *supra* note 125, at 87.
[128] *Yash Charitable Trust*, *supra* note 125, at 89.

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Following the judgment, the National Medical Commission issued an advisory to all medical colleges and institutions. The advisory records that stem cell therapy in routine practice is permitted only for the disease conditions specified in the list of indications approved by the Ministry of Health and Family Welfare as standard care. Beyond those indications, stem cell therapy is permissible only in the context of research, regulated by the Central Drugs Standard Control Organisation or by the Department of Health Research according to the level of manipulation of the cells, and any treatment falling outside that scheme is to be treated as unlawful.[129]

XV. CONCLUSION AND RECOMMENDATIONS

The starting point must be the Supreme Court's own suggestions in *Yash Charitable Trust* for curbing unproven stem cell research and practice in the treatment of autism spectrum disorder. Legislation on stem cells that consolidates the existing rules, regulations and guidelines, coupled with a dedicated authority holding clear and well-defined powers of regulatory oversight, is the need of the hour. In the absence of strict statutory compliance and enforcement, private clinics and hospitals continue to exploit the vulnerability of patients. Provisions for stringent penalties are required where the health of a patient or trial participant is endangered by unethical conduct.[130]

The Court held that voluntarily signing a consent form for a medical procedure does not produce valid consent where the consent rests on inadequate information, and its earlier decision in *Samira Kohli* illuminates that standard. Unproven or experimental stem cell therapy for autism spectrum disorder cannot be regarded as routine treatment. Written consent therefore does not amount to valid consent where the information given leaves the patient under the false belief, or therapeutic misconception, that an unproven therapy may deliver outcomes comparable to standard medical treatment.[131]

In present circumstances, widespread public awareness of cell and gene therapies is of the first importance, and patients need in particular to understand the complexities of CGT products that use gene editing technologies. Clinical trial participants should be encouraged to consent to audio-visual recording, so as to maintain transparency during medical procedures. A patient-focused approach to communication should be adopted to make the informed consent form and

[129] NATIONAL MEDICAL COMMISSION, *Advisory: Compliance with Hon'ble Supreme Court Judgement Dated 30.01.2026*, No. CDN-20011/105/2026-COORDINATION-NMC (Mar. 25, 2026), https://www.nmc.org.in/MCIRest/open/getDocument?path=/Documents/Public/Portal/LatestNews/Advisory_Merged_Secretary.pdf.

[130] *Yash Charitable Trust*, *supra* note 125, at 89.

[131] *Yash Charitable Trust*, *supra* note 125, at 53-55.

the accompanying disclosure more transparent. To bridge the communication gap between participants or patients and investigators or researchers, the institutional ethics committee may engage a patient expert, or a group of experts with knowledge of CGT products, to safeguard the interests of patients.[132]

Regulatory authorities in India should publish and regularly update a guidance manual for clinical trial participants, drawing on past patient experience. Such a document should be developed in collaboration with all the bodies and agencies involved in the regulation of CGT products. To protect the rights, safety and well-being of patients and human subjects, the procedures for audio-visual recording and the other elements of the informed consent form should be prepared with input from patients and other stakeholders.[133]

The dissolution of the National Apex Committee for Stem Cell Research and Therapy, the only national body for the regulation of stem cell research, was a setback, as the Supreme Court observed in *Yash Charitable Trust*. The order of the Department of Health Research dated 3 March 2024 dissolved the committee, dispensed with registration of institutional committees for stem cell research with it, and left the review of stem cell research involving human participants to ethics committees registered with that Department, which must include stem cell experts.[134] The Supreme Court has appropriately recommended that the central government reinstate the committee, so as to secure effective compliance and nationwide monitoring and oversight of stem cell research.[135]

The allegation that illegal clinical trials of new drugs and vaccines were carried out over a long period at the Sheth Vadilal Sarabhai Hospital in Ahmedabad demonstrates the urgency of strict compliance and monitoring of research and clinical trials in the country. That will require new legislation, or suitable amendment of the existing framework and guidelines. Higher standards of free and voluntary informed consent must be established by law for patients participating in clinical trials of unproven or experimental therapies.[136]

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[132] Varsha Dalal et al., *Redefining Informed Consent Form in Cell and Gene Therapy Trials*, 15 PERSP. CLIN. RES. 4, 9 (2024).

[133] U.S. FOOD & DRUG ADMIN., PATIENT-FOCUSED DRUG DEVELOPMENT: COLLECTING COMPREHENSIVE AND REPRESENTATIVE INPUT, GUIDANCE FOR INDUSTRY, FOOD AND DRUG ADMINISTRATION STAFF, AND OTHER STAKEHOLDERS 21 (2020), https://www.fda.gov/media/139088/download.

[134] DEP'T OF HEALTH RESEARCH, MINISTRY OF HEALTH & FAMILY WELFARE, Order No. T.11016/02/2023-HR, *Dissolving the National Apex Committee for Stem Cell Research and Therapy* (Mar. 3, 2024), https://epms.icmr.org.in/extramuralstaticweb/callforproposal/Dissolution_of_NACSCRT.pdf.

[135] *Yash Charitable Trust*, *supra* note 125, at 89.

[136] *Yash Charitable Trust*, *supra* note 125, at 88.

In the event of death or injury there must be provision for proper compensation, and for immediate interim compensation.[137] Reform of the legal framework and the guidelines must protect the safety and well-being of trial participants. A protective safety net should be provided by law through a rights-based approach and a patient-centred approach, expressed in patient disclosure and a patient-centric consent process.[138]

The regulation of CGT products using genome editing in India is spread across a series of statutes, rules, regulations, guidelines and policies. What the country needs is a uniform approach to updating all of them. To secure patient safety, sound scientific risk assessment and product efficacy, the framework and the guidelines must be revised regularly in step with technological development. Every statute, rule, regulation and guideline applicable to CGT products should expressly add and define the terms relating to cell and stem cell based products and gene therapeutic products, so that the law is clear and certain. Detailed guidelines and guidance documents are needed specifically on CGT products incorporating gene editing, and new strategies must be developed to secure patient access to high-cost CGT products.

Cell and gene therapy development using human gene editing is growing rapidly and is likely to expand further. The draft guidance on next-generation sequencing published by the United States Food and Drug Administration for CGT products using genome editing addresses the safety concerns raised by editing, and the sequencing and bioinformatics methods it describes are more comprehensive tools that may assist sponsors designing non-clinical studies to screen for off-target edits and loss of genome integrity.[139] Those methods are capable of tracing off-target editing and chromosomal translocation, either of which can compromise the genomic integrity of the edited cell.[140]

Personalised cell and gene therapies using genome editing hold real promise for rare and genetic diseases affecting very small numbers of patients, but regulatory approval is difficult to obtain because of the demands of traditional development models. The United States Food and Drug Administration has recently published draft guidance on a plausible mechanism framework, which recommends an accelerated approval pathway for CGT products where a randomised clinical trial is not feasible.[141] The object of that guidance is to establish how evidence of

[137] *Yash Charitable Trust*, *supra* note 125, at 88.
[138] *Yash Charitable Trust*, *supra* note 125, at 88.
[139] U.S. FOOD & DRUG ADMIN., SAFETY ASSESSMENT OF GENOME EDITING IN HUMAN GENE THERAPY PRODUCTS USING NEXT-GENERATION SEQUENCING: DRAFT GUIDANCE FOR INDUSTRY 2 (Apr. 2026), https://www.fda.gov/media/191966/download.
[140] *Id.* at 15.
[141] Vinay Prasad & Martin A. Makary, *FDA's New Plausible Mechanism Pathway*, 393 NEW ENG. J. MED. 2365, 2365 (2025).

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therapeutic effectiveness and of safety may be demonstrated for a personalised therapy on the basis of a plausible mechanism.[142]

The draft guidance on leveraging prior knowledge is directed at accelerating the development of CGT products incorporating gene editing. It recommends how sponsors may draw on existing data to maximise the efficiency of review, and how non-clinical and clinical prior knowledge relating to chemistry, manufacturing and controls may be used.[143]

The regulation of CGT products under the present framework and guidelines in India applies rigorous standards of safety assessment, but specific legal provisions and detailed guidelines on human genome editing are missing. The existing guidelines do no more than mark out the permissible and prohibited areas of research. Proper guidance documents on CGT products using gene editing need to be issued by the regulatory bodies. Documents addressing the design, manufacture, testing and preclinical safety assessment of such products, together with guidance on clinical trial design, would help all stakeholders to understand the regulators' thinking in this field.[144] It is genuinely important that the Indian regulatory authorities issue the necessary guidance for the development of CGT products incorporating genome editing technologies, and the guidance documents published in recent years by foreign regulators will help stakeholders to understand contemporary developments.

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[142] U.S. FOOD & DRUG ADMIN., CONSIDERATIONS FOR THE USE OF THE PLAUSIBLE MECHANISM FRAMEWORK TO DEVELOP INDIVIDUALIZED THERAPIES THAT TARGET SPECIFIC GENETIC CONDITIONS WITH KNOWN BIOLOGICAL CAUSE: DRAFT GUIDANCE FOR INDUSTRY (2026), https://www.fda.gov/regulatory-information/search-fda-guidance-documents/considerations-use-plausible-mechanism-framework-develop-individualized-therapies-target-specific.

[143] U.S. FOOD & DRUG ADMIN., LEVERAGING PRIOR KNOWLEDGE IN THE DEVELOPMENT OF HUMAN GENE THERAPY PRODUCTS INCORPORATING GENOME EDITING: DRAFT GUIDANCE FOR INDUSTRY 1 (2026), https://www.fda.gov/regulatory-information/search-fda-guidance-documents/leveraging-prior-knowledge-development-human-gene-therapy-products-incorporating-genome-editing.

[144] U.S. FOOD & DRUG ADMIN., *supra* note 7, at 1.

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