https://doi.org/10.30895/1991-2919-2023-13-481-tabl5

Таблица 5. *Примеры нормативных документов, регулирующих разработку препаратов на основе технологии редактирования генома соматических клеток*

Table 5. *Examples of regulatory documents applicable to the development of medicinal products based on somatic cell genome-editing technologies*

Регион *Region*	**Нормативные документы** *Regulatory documents*
США *USA*	Фармакопейная конвенция США *The United States Pharmacopeial Convention:* Фармакопея США — Национальный формуляр; 2022 год, издание 1 *USP–NF 2022, Issue 1:* • <1047> Gene therapy products • <1043> Ancillary materials for cell, gene, and tissue-engineered products Управление по контролю за качеством продуктов питания и лекарственных средств *United States Food and Drug Administration (FDA):* • Human gene therapy for hemophilia. Guidance for industry (FDA 2018-D-2238) • Preclinical assessment of investigational cellular and gene therapy products. Guidance for industry (FDA-2012-D-1038) • Considerations for the design of early-phase clinical trials of cellular and gene therapy products. Guidance for industry. (FDA-2013-D-0576) • Human gene therapy for rare diseases. Guidance for industry (FDA-2018-D-2258) • Potency tests for cellular and gene therapy products. Final guidance for industry (FDA-2008-D-0520) • Chemistry, manufacturing, and control (CMC) information for human gene therapy Investigational New Drug Applications (INDs). Guidance for industry (2008-D-0205) • Long-term follow-up after administration of human gene therapy products. Guidance for industry (FDA-2018-D-2173) • Human gene therapy products incorporating human genome editing. Draft guidance for industry (FDA-2021-D-0398)
Страны Европейского союза *EU countries*	Европейская фармакопея, издание 10 *European Pharmacopoeia (Ph. Eur.) 10th Ed.:* • Recombinant DNA technology, Products of (0784) • Nucleic acid amplification technique (20621) • Raw materials of biological origin for the production of cell-based and gene therapy medicinal products (50212) • Gene transfer medicinal products for human use (51400) Европейское агентство по лекарственным средствам *European Medicines Agency (EMA)[1]:* • Guideline on quality, non-clinical and clinical aspects of medicinal products containing genetically modified cells (EMA/CAT/GTWP/671639/2008 Rev. 1 — Corr) • Guideline on quality, non-clinical and clinical requirements for investigational advanced therapy medicinal products in clinical trials (draft) (EMA/CAT/852602/2018) • Guideline on the quality, non-clinical and clinical aspects of gene therapy medicinal products (EMA/CAT/80183/2014) • Guideline on safety and efficacy follow-up and risk management of advanced therapy medicinal products (EMEA/149995/2008 Rev.1) • Reflection paper on management of clinical risks deriving from insertional mutagenesis (EMA/CAT/190186/2012). • Guideline on the non-clinical studies required before first clinical use of gene therapy medicinal products (EMEA/CHMP/GTWP/125459/2006) • Guideline on non-clinical testing for inadvertent germline transmission of gene transfer vectors (EMEA/273974/2005)
Международный совет по гармонизации технических требований к лекарственным средствам для медицинского применения *International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use, ICH*	• General principles to address the risk of inadvertent germline integration of gene therapy vectors. ICH considerations (CHMP/ICH/469991/2006) • General principles to address virus and vector shedding. ICH considerations (EMEA/CHMP/ICH/449035/2009) • On nonclinical biodistribution considerations for gene therapy products (ICH Guideline S12) (EMA/CHMP/ICH/318372/2021)
Канада *Canada*	Министерство окружающей среды и изменения климата Канады *Environment and Climate Change Canada:* • Food and Drugs Act (R.S.C., 1985, c. F-27) • Supplementary guidance document for the notification and testing of new substances: organisms used in cell and gene therapy under Schedule 1 of the New Substances Notification Regulations (Organisms) (December 2021)
Австралия *Australia*	Управление по контролю за оборотом лекарственных средств и изделий медицинского назначения *Therapeutic Goods Administration:* • Australian regulatory guidelines for biologicals • Guidance 21: Medicines produced by genetic manipulation • Therapeutic Goods Act 1989 • Gene Technology Act 2000
Китай *China*	Центр экспертизы лекарственных средств, Национальное управление по изделиям медицинского назначения *Center for Drug Evaluation, National Medical Products Administration:* • Technical guiding principles for quality control of human gene therapy research and preparations
Япония *Japan*	Управление лекарственных средств и медицинских изделий, Министерство здравоохранения, труда и благосостояния *Pharmaceuticals and Medical Devices Agency, Ministry of Health, Labour and Welfare:* • Ensuring the quality and safety of gene therapy products (PSEHB/MDED notification No.0709-2) • Amendment of the enforcement regulations of the Act on Securing Quality, Efficacy and Safety of Pharmaceuticals, Medical Devices, Regenerative and Cellular Therapy Products, Gene Therapy Products, and Cosmetics related to Reprocessed Single-Use Medical Devices (R-SUDs) (PSEHB notification No. 0731-7)
Южная Корея *South Korea* [46]	Институт изучения законодательства Кореи, Министерство безопасности пищевых продуктов и лекарственных средств *Korea Legislation Research Institute, Ministry of Food and Drug Safety:* • Pharmaceutical Affairs Act (Act No. 14328) • Bioethics and Safety Act (Act No. 12844) • Guideline on quality assessment for gene-editing based advanced therapy medicinal products (December, 2018) • Considerations on design and analysis of clinical trials for conditional approval (2018) • Guideline for non-clinical assessment of gene therapy products (2017) • Guideline on design of early-phase clinical trials of cell and gene therapy (2015) • Guideline for follow-up of patients administered with gene therapy products (2016) • Considerations for validating analytical method for biodistribution of gene therapy products using qPCR (2010)
Республика Беларусь *Republic of Belarus*	Государственная фармакопея Республики Беларусь (гармонизирована с Ph. Eur.): • Продукты технологии рекомбинантной ДНК (0784) • Методы амплификации нуклеиновых кислот (20621) • Лекарственные средства для генной терапии для медицинского применения (51400) *State Pharmacopoeia of the Republic of Belarus (harmonised with Ph. Eur.):* • *Recombinant DNA technology, Products of (0784)* • *Nucleic acid amplification technique (20621)* • *Gene transfer medicinal products for human use (51400)*
Российская Федерация *Russian Federation*	• Федеральный закон от 05.07.1996 № 86-ФЗ «О государственном регулировании генно-инженерной деятельности» • Федеральный закон от 23.06.2016 № 180-ФЗ «О биомедицинских клеточных продуктах» • Рекомендации по организации производства, оценке качества, проведению доклинических и клинических исследований генотерапевтических лекарственных препаратов • *Federal Law No. 86-FZ On State Regulation of Genetic Engineering Activities of 05.07.1996* • *Federal Law No. 180-FZ On Biomedical Cellular Products of 23.06.2016* • *Recommendations for the organisation of manufacturing, quality assessment, preclinical and clinical trials of gene therapy products*

[1] The European Medicines Agency. https://www.ema.europa.eu/